Annual Report 2025

uniQure N.V.

Amsterdam, April 24, 2026

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A Report of the Board of Directors

1 Introduction

a) Forward-looking statements

This Report, the Financial Statements and Other Information (the "Annual Report") contains "forward-looking statements" within the meaning of U.S. federal securities laws. Forward-looking statements are based on our current expectations of future events and many of these statements can be identified using terminology such as "believes," "expects," "anticipates," "plans," "may," "will," "projects," "continues," "estimates," "potential," "opportunity" and similar expressions. These forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. These forward-looking statements include, without limitation, statements concerning: our financial position and performance, revenues, costs, expenses, liquidity, uses of cash and capital requirements; our need for additional financing or the time period for which our existing cash resources will be sufficient to meet our operating requirements; the success, progress, number, scope, cost, duration, timing or results of our research and development activities, preclinical and clinical trials, including the timing for initiation, completion or availability of results from any preclinical studies and clinical trials or for the submission, review or approval of any regulatory filing, including the timing of the Company's Biologics License Application for AMT-130 to the United States Food and Drug Administration (the "FDA"); the anticipated or ultimate outcome, and timing, of interactions with regulatory agencies, including the FDA; the timing of, and our ability to, obtain and maintain regulatory approvals for any of our product candidates; the potential benefits that may be derived from any of our product candidates; our strategies, prospects, plans, goals, expectations, forecasts or objectives; the success of our collaborations with third parties; our ability to identify and develop new product candidates and technologies; our intellectual property position; our commercialization, marketing and manufacturing capabilities and strategy, including plans for potential commercialization of AMT-130; our ability to achieve long-term growth; and developments and projections relating to our competitors in the industry. These forward-looking statements may be found in Part 2, Part 3, Part 4 and other sections of this Annual Report.

Although we believe that we have a reasonable basis for each forward-looking statement contained in this Annual Report, such statements are only predictions based on management's current views and assumptions and involve risks and uncertainties, and actual results could differ materially from those projected or implied. The most significant factors known to us that could materially adversely affect our business, operations, industry, financial position or future financial performance include those described under "Risk Factors" and elsewhere in this Annual Report. Furthermore, we refer to our Annual Report on Form 10-K for the year ended December 31, 2025, filed with the U.S. Securities and Exchange Commission (the "SEC") on March 2, 2026, or in the documents where such forward-looking statements appear. You should carefully consider that information before you make an investment decision.

You should not place undue reliance on these statements, which speak only as of the date that they were made. Our actual results or experience could differ significantly from those anticipated in the forward-looking statements and from historical results, due to the risks and uncertainties described under "Risk Factors" and elsewhere in this Annual Report as well as others that we may consider immaterial or do not anticipate at this time. These cautionary statements should be considered in connection with any written or oral forward-looking statements that we may make in the future. We do not undertake any obligation to release publicly any revisions to these forward-looking statements after completion of this Annual Report to reflect later events or circumstances or to reflect the occurrence of unanticipated events. All forward-looking statements attributable to us are expressly qualified in their entirety by these cautionary statements.

In addition, with respect to all of our forward-looking statements, we claim the protection of the safe harbor for forward-looking statements contained in the U.S. Private Securities Litigation Reform Act of 1995.

b) History and development of uniQure

We are a leader in the field of gene therapy and seek to deliver to patients suffering from rare and other devastating diseases single treatments with potentially curative results. Our business was founded in 1998 and was initially operated through our predecessor company, Amsterdam Molecular Therapeutics Holding N.V ("AMT"). We were incorporated on January 9, 2012 as a private company with limited liability (besloten vennootschap met beperkte

aansprakelijkheid) under the laws of the Netherlands. In 2012, AMT undertook a corporate reorganization, pursuant to which uniQure B.V. acquired the entire business and assets of AMT and completed a share-for-share exchange with the shareholders of AMT. Effective February 10, 2014, in connection with our initial public offering, we converted into a public company with limited liability (*naamloze vennootschap*) and changed our legal name from uniQure B.V. to uniQure N.V.

We are registered in the trade register of the Dutch Chamber of Commerce (Kamer van Koophandel) in Amsterdam, the Netherlands under number 54385229. Our headquarters are in Amsterdam, the Netherlands, and our registered office is located at Paasheuvelweg 25, Amsterdam 1105 BP, the Netherlands and our telephone number is +31 20 240 6000. Our website address is www.uniqure.com. Our ordinary shares are listed on the Nasdaq Global Select Market ("Nasdaq") and trade under the symbol "QURE".

Unless the context requires otherwise, references in this report to "uniQure," "Company," "we," "us" and "our" and similar designations refer to uniQure N.V. and our subsidiaries.

c) Business overview

We are advancing a focused pipeline of innovative gene therapies, including our clinical candidates for the treatment of Huntington's disease, refractory mesial temporal lobe epilepsy ("MTLE"), and Fabry disease. We also continue to advance early-stage research candidates with a focus on gene therapies for severe diseases.

Recent Product Candidate Developments

Huntington's disease program (AMT-130)

In September 2025, we announced positive topline data from the three-year analysis of the ongoing Phase I/II studies of AMT-130 for the treatment of Huntington's disease. We analyzed clinical outcomes for 29 patients treated with AMT-130 (n=17 high-dose; n=12 low-dose) of which 12 patients per dose group had attained 36 months of follow-up and were evaluated at that time point. Outcomes for each dose group were compared to a propensity score-matched external control drawn from the Enroll-HD natural history data set (n=940 for high-dose; n=626 for low-dose).

Topline 36-month efficacy results for patients receiving high-dose AMT-130 were as follows (data cutoff as of June 30, 2025):

- A statistically significant 75% slowing of disease progression as measured by composite Unified Huntington's Disease Rating Scale ("cUHDRS") (p=0.003), which met the primary endpoint of the study. Treated patients had a mean change in cUHDRS from baseline of -0.38 compared to a change of -1.52 for patients in the propensity score-matched external control.

- A statistically significant 60% slowing of disease progression as measured by Total Function Capacity ("TFC") (p=0.033), which met a key secondary endpoint of the study. Treated patients had a mean change in TFC from baseline of -0.36 compared to a change of -0.88 for patients in the propensity score-matched external control.

- Favorable trends in other secondary endpoint measures of motor and cognitive function, including Symbol Digit Modalities Test ("SDMT"), Stroop Word Reading Test ("SWRT") and Total Motor Score ("TMS").

    - An 88% slowing of disease progression as measured by SDMT (p=0.057), with a mean change in SDMT from baseline of -0.44 compared to a change of -3.73 for patients in the propensity score-matched external control.

    - A 113% slowing of disease progression as measured by SWRT (nominal p=0.002), with a mean change in SWRT from baseline of 0.88 compared to a change of -6.98 for patients in the propensity score-matched external control.

- A 59% slowing of disease progression as measured by TMS (nominal p=0.174), with a mean change in TMS from baseline of 2.01 compared to a change of 4.88 for patients in the propensity score-matched external control.

A mean reduction from baseline in cerebrospinal neurofilament light protein ("CSF NfL") of -8.2% was observed at 36 months in the high-dose of AMT-130 of the Phase I/II studies. CSF NfL is a well-characterized, supportive biomarker of neurodegeneration. Elevation in CSF NfL has been shown to be strongly associated with greater clinical severity of Huntington's disease.

We believe that the consistently favorable results in functional, motor and cognitive endpoints at 36 months observed in the high-dose group, compared to the variable trends observed in the low-dose group, reflect a dose-dependent response to AMT-130.

Various other supportive analyses of the results from the AMT-130 high-dose treatment group, including those using a propensity score-weighted external control and comparisons to the TRACK-HD and PREDICT-HD datasets, were consistent with the primary analysis.

AMT-130 was generally well-tolerated in the Phase I/II studies, with a manageable safety profile at both doses with no new drug-related serious adverse events ("SAEs") observed since December 2022. The most common adverse events in the treatment groups were related to the administration procedure.

In 2025, we treated six patients with the high-dose of AMT-130 in a fourth cohort to evaluate the safety and efficacy of AMT-130 in patients with lower striatal volumes compared to patients in previous cohorts.

From November 2024 through April 2025, we held three Type B meetings with the FDA. As part of these interactions, the FDA agreed that data from the ongoing Phase I/II studies, compared to a natural history external control, may serve as the primary basis of a Biologics License Application ("BLA") submission under the FDA's accelerated approval pathway. The FDA also agreed that cUHDRS may be used as an intermediate clinical endpoint and reductions in CSF NfL may serve as supportive evidence of therapeutic benefit in the application for such accelerated approval.

In April 2025, we announced that the FDA granted Breakthrough Therapy designation to AMT-130 for the treatment of Huntington's disease. The Breakthrough Therapy designation was supported by clinical data from the ongoing Phase I/II trials of AMT-130 for the treatment of Huntington's disease showing the potential for meaningful slowing of disease progression. Breakthrough Therapy designation is intended to expedite the development and review of investigational therapeutic candidates that are intended to treat a serious condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on a clinically significant endpoint(s). In general, the preliminary clinical evidence should show a clear advantage over available therapy.

In addition to the Breakthrough Therapy designation, the FDA previously granted Regenerative Medicine Advance Therapy ("RMAT") designation, Orphan Drug designation and Fast Track designation to AMT-130.

In October 2025, we met with the FDA at a pre-Biologics License Application meeting to discuss the application for AMT-130. In December 2025, we announced that in the final meeting minutes, the FDA conveyed that data submitted from the Phase I/II studies of AMT-130 are currently unlikely to provide the primary evidence to support a BLA submission.

In January 2026, we met with the FDA at a Type A meeting to discuss AMT-130. In March 2026, following receipt of the final meeting minutes from the Type A meeting, we announced that the FDA stated that it cannot agree that data from the Phase I/II studies, compared to an external control, are sufficient to provide the primary evidence of effectiveness required to support a marketing application for AMT-130. The FDA strongly recommended we conduct a prospective, randomized, double-blind, sham surgery-controlled study.

Temporal lobe epilepsy program (AMT-260)

In May 2025, we presented initial safety and exploratory efficacy data from the first treated patient in our Phase I/IIa clinical trial ("GenTLE") of AMT-260 for the treatment of MTLE in the United States ("U.S."). As of the

April 17, 2025 cutoff date, the patient showed a 92% reduction in seizure frequency observed through the first five months of follow-up with no serious safety events as per the data announcement.

In September 2025, we completed enrollment of the first three patients in the first cohort administering AMT-260 to the non-dominant hemisphere of the brain. Following a positive review by the independent data monitoring committee ("IDMC"), we expanded the first cohort into MTLE in the dominant hemisphere. We completed enrollment of the first cohort in 2025. We also initiated enrollment of a second cohort in 2025, which is expected to include an additional six patients.

Fabry disease program (AMT-191)

In February 2025, we announced a favorable recommendation from the IDMC following a review of safety data from the first two patients treated in the Phase I/II clinical trial of AMT-191 for the treatment of Fabry disease. The IDMC's review did not identify any significant safety concerns and recommended proceeding with enrollment in the second cohort.

During 2025, we enrolled three patients in a second, lower dose cohort in our Phase I/II clinical trial of AMT-191 for the treatment of Fabry disease, as well as enrolled four patients in a third cohort.

In September 2025, we announced initial safety and exploratory efficacy data from the first cohort. The data presented in respect of the July 24, 2025 cutoff date, at which date all patients had less than three months of follow-up, was as follows:

- All patients in the first dose cohort showed sustained increases in α-galactosidase A ("α-Gal A") enzyme activity, ranging from 27- to 208-fold above the mean normal range (1.38-8.66 nmol; mean normal of 3.57 nmol). All four patients were withdrawn from enzyme replacement therapy ("ERT") and maintained stable plasma lyso-Gb3 levels through the cutoff date.

- AMT-191 showed a manageable safety profile. At the high dose, two SAEs unrelated to AMT-191 (stroke, diplopia), two related SAEs (chest pain, increased troponin), and one possibly related SAE (leptomeningeal enhancement) were observed in two patients. Additionally, one patient experienced an asymptomatic Grade 3 liver enzyme elevation that resolved with corticosteroid therapy. This event, classified as a dose-limiting toxicity per protocol, was not considered serious and did not require hospitalization. No loss of α-Gal A expression was observed in this patient.

In February 2026, we announced updated preliminary data from the Phase I/II study of AMT-191 for Fabry disease. Updated preliminary safety and exploratory efficacy data included the following, with a data cutoff date as of January 8, 2026:

- Dose-dependent elevations were observed across 11 patients in three dose levels with α-Gal A activity ranging from 0.34- to 82.2-fold above mean normal range (1.38-8.66 nmol; mean normal of 3.57 nmol) at the lowest dose, 1.6- to 312.52-fold at the mid dose, and 27.7- to 223.7-fold at the highest dose. These increases were durable for the measured time period ranging from the longest follow-up period of more than a year in a treated patient (high-dose cohort) to the shortest follow-up period of four-months in a treated patient (mid-dose cohort).

- Six of 11 dosed patients were withdrawn from ERT.

- Plasma lyso-Gb3 levels were stable post-dose across all dose cohorts, regardless of ERT status through the cutoff date.

As of February 18, 2026, all 11 dosed patients were withdrawn from ERT.

AMT-191 continued to show a manageable safety profile. No SAEs related to AMT-191 were observed at the $4x10^{13}$ gc/kg and $2x10^{13}$ gc/kg doses. No additional SAEs were observed at the $6x10^{13}$ gc/kg dose beyond the five previously reported in September 2025 in two patients. Per protocol, additional dosing in the mid- and high-dose cohorts has been paused pending further evaluation following asymptomatic Grade 3 liver enzyme elevations observed in two patients in the mid-dose cohort, which were confirmed dose-limiting toxicity.

Amyotrophic Lateral Sclerosis (AMT-162)

EPISOD1 is a Phase I/II multi-center, open-label trial of AMT-162 for the treatment of amyotrophic lateral sclerosis caused by mutations in superoxide dismutase 1 ("SOD1-ALS") in the U.S. In September 2025, we voluntarily paused enrollment in EPISOD1 upon the recommendation of the IDMC following a review of available preliminary data related to the safety and efficacy of AMT-162 in the context of a dose limiting toxicity, which resulted in an SAE determined to be related to AMT-162, that was observed in one patient in the second cohort. Following review of the preliminary efficacy and safety data generated from EPISOD1, we have decided to discontinue development of AMT-162. We will continue to collect safety data from the five patients dosed in EPISOD1, consistent with applicable safety and regulatory requirements.

Other Business Developments

Public Offerings

In January 2025, we received net proceeds of $70.1 million, after deducting underwriting discounts and commissions and issuance costs payable by us, through a follow-on public offering of 4.4 million ordinary shares at a public offering price of $17.00 per ordinary share. In February 2025, we received an additional $10.4 million in net proceeds, after related issuance costs, upon the underwriters' exercise of their option to purchase an additional 0.7 million ordinary shares at the public offering price.

In September 2025, we received net proceeds of $323.7 million, after deducting underwriting discounts and commissions and issuance costs payable by us, through a follow-on public offering of 6.7 million ordinary shares at a public offering price of $47.50 per ordinary share, and, in lieu of ordinary shares to certain investors, pre-funded warrants to purchase 0.5 million of our ordinary shares at the public offering price per share less the $0.0001 per share exercise price of each pre-funded warrant.

Hercules Loan Amendment

In September 2025, we entered into a $175.0 million senior secured term loan facility (the "2025 Amended Facility") with Hercules Capital, Inc. ("Hercules"), which replaced the loan facility amended in 2024 (together the "Hercules Loan Facilities". The 2025 Amended Facility consists of three tranches including a first tranche of $50.0 million replacing the outstanding borrowing as of the loan amendment date, an additional term loan tranche of $100.0 million, which can be drawn at our option, subject to a BLA approval of AMT-130 prior to June 2027, provided that confirmatory trials to the extent and in the manner required to support full approval (if applicable) remain ongoing or are being planned, and a third tranche of up to $25.0 million, subject to Hercules' approval. All tranches have a floating interest rate of the greater of 9.45% and the prime rate plus 2.45%, reflecting a floating rate of 9.45% as of December 31, 2025. The tranches mature on October 1, 2030.

Our Mission and Strategy

Our mission is to deliver curative, one-time administered genomic medicines that transform the lives of patients. We aim to build an industry-leading, fully integrated, and global company that leverages its technology and proprietary manufacturing platform to deliver these medicines to patients with serious unmet medical needs. Our strategy to achieve this mission is to:

Advance the development and potential commercialization of AMT-130, a potential one-time gene-therapy approach for the treatment of Huntington's disease. AMT-130 is the first Adeno-Associated Virus-based gene therapy for the treatment of Huntington's disease to have entered into clinical development.

Advance our pipeline of clinical-stage gene therapy candidates and research programs. We have dosed patients in the clinical studies of our product candidates for the treatment of MTLE (AMT-260) and Fabry disease (AMT-191). We intend to generate clinical data to assess the safety, tolerability and potential efficacy of these product candidates. We also continue to advance early-stage research candidates with a focus on gene therapies for severe diseases.

Develop next-generation delivery and cargo technologies. We are developing technologies that have the potential to augment the safety and efficacy of our product candidates and broaden the applicability of our gene therapies to a wider range of diseases and patients. These technologies include next-generation delivery approaches, such as smart Adeno-Associated Virus ("AAV") capsids potentially capable of improved central nervous system ("CNS") transduction and crossing the blood-brain barrier after systemic delivery, as well as novel cargo technologies such as miQURE®, our one-time administered gene silencing platform, LinQURE®, a single AAV carrying multiple micro ribonucleic acids ("miRNAs") designed to suppress different genes, and GoQURE® for simultaneous silencing of a disease gene and replacement with a healthy gene.

Central Nervous System Diseases

Huntington's Disease

Huntington's Disease and Market Background

Huntington's disease is a severe genetic neurodegenerative disorder causing loss of muscle coordination, behavioral abnormalities and cognitive decline, often resulting in complete physical and mental deterioration over a 12 to 15-year period. The median survival time after onset is 15 to 18 years (range: 5 to >25 years). Huntington's disease is caused by an inherited defect in a single gene that codes for a protein called Huntingtin ("HTT"). It is estimated that approximately 75,000 people in the U.S., European Union ("EU"), and United Kingdom ("UK") have symptomatic Huntington's disease, of which approximately 40,000 are in the U.S. In addition to this, there are hundreds of thousands of others at risk of inheriting the disease. Huntington's disease mutation carriers can be identified decades before onset. There is currently no available therapy that can delay onset or slow progression of the disease. Although some symptomatic treatments are available, they are only transiently effective and are often associated with significant side effects.

Our Development of AMT-130 for Huntington's Disease

AMT-130 is our novel gene therapy candidate for the treatment of Huntington's disease, which utilizes our proprietary, gene-silencing miQURE platform and incorporates a miRNA, specifically designed to silence the huntingtin gene and the potentially highly toxic exon 1 protein fragment.

Our goal for AMT-130 is to develop a gene therapy with the following profile:

(1) one-time administration of a disease-modifying therapy into the striatum, the area of the brain where Huntington's disease is known to manifest;
(2) biodistribution of the therapy in both the deep and cortical structures of the brain via transport of the AAV vector and through secondary exosome-mediated delivery; and,
(3) safe, on-target and durable knockdown of HTT and exon 1 HTT.

We are currently conducting a multi-center randomized, controlled Phase I/II clinical trial for AMT-130 in the U.S. (the "U.S. study"), as well as an open-label Phase Ib/II study in Europe (the "European study") with the same early-manifest criteria for Huntington's disease as the U.S. study.

We completed the enrollment of all 26 patients in the first two cohorts of the U.S. study in March 2022 and the enrollment of 13 patients in the first two cohorts of the European study in June 2023. In the U.S. study, 26 patients with early manifest Huntington's disease were randomized to treatment (six low-dose patients, 10 high-dose patients) or an imitation (sham) surgical procedure (10 patients). The U.S. study consists of a blinded 12-month core patient study period followed by an unblinded long-term follow-up period of five years. An additional four control patients crossed over to treatment. In the European study, 13 patients were enrolled and treated with AMT-130 (six low-dose patients and seven high-dose patients).

In 2023, we initiated patient dosing in a third cohort consisting of up to 12 patients to further investigate both doses of AMT-130 together with perioperative immunosuppression using the current, established stereotactic administration procedure. In February 2025, we completed enrollment of all 12 patients in the third cohort.

In 2025, we treated six patients with the high-dose of AMT-130 in a fourth cohort to evaluate the safety and efficacy of AMT-130 in patients with lower striatal volumes compared to patients in previous cohorts.

Temporal Lobe Epilepsy Program (AMT-260)

Temporal Lobe Epilepsy Disease and Market Background

Temporal Lobe Epilepsy ("TLE") affects approximately 0.5 million people in the U.S. alone, of which approximately 0.3 million U.S. patients are inadequately treated through anti-seizure medications and are considered refractory. 240,000 of U.S. refractory TLE patients have a lesion in the mesial temporal lobe (hippocampus), which is expressed as sclerosis, atrophy or scarring. MTLE is often caused by brain injury, infections or prolonged febrile seizures which can lead to hyperexcitability of the hippocampus and repeated seizures which can further damage the hippocampus over time. Refractory MTLE patients have a poor quality of life and a reduced lifespan. Surgical treatment for refractory patients is lobectomy or laser tissue ablation but only approximately 1% of eligible patients undergo surgery annually.

Our Development of AMT-260 for Temporal Lobe Epilepsy

In July 2021, we acquired uniQure France SAS (the "uniQure France Acquisition") and its lead program, now known as AMT-260, to treat refractory MTLE. AMT-260 is being developed based on exclusive licenses to certain patents that uniQure France obtained following its formation in 2019 from two French research institutions.

AMT-260 is comprised of an AAV9 vector that locally delivers two engineered miRNAs designed to degrade the GRIK2 gene and suppress the aberrant expression of glutamate receptor subtype GLUK2 that is believed to trigger seizures in patients with refractory MTLE. The use of AAV9 to deliver any sequence that affects the expression of the GRIK2 gene in humans has been exclusively licensed from Regenxbio Inc. ("Regenxbio").

We initiated a Phase I/IIa clinical trial in November 2024 that is being conducted in the U.S. and is intended to consist of two parts. The first active part of the clinical trial is a multicenter, open-label trial with two dosing cohorts of six patients each to assess safety, tolerability, and initial efficacy of AMT-260 in patients with refractory MTLE. The second part is expected to be a randomized, controlled trial to generate proof-of-concept data. The FDA-approved study protocol provides that the first three patients to be enrolled in the study were required to have Magnetic Resonance Imaging ("MRI")-confirmed unilateral, hippocampal sclerosis.

In September 2025, we completed enrollment of the first three patients in the first cohort administering AMT-260 to patients with lesions in the non-dominant hemisphere of the brain. Following a positive review by the IDMC, we expanded the first cohort into MTLE in the dominant hemisphere and initiated a second cohort. We completed enrollment of the six patients into the first cohort in 2025. We also initiated enrollment of a second cohort in 2025, which is expected to include an additional six patients.

Amyotrophic Lateral Sclerosis ("ALS")

ALS Disease and Market Background

ALS, commonly known as Lou Gehrig's disease, is a progressive and fatal neuromuscular disease with the majority of ALS patients dying within 2 to 5 years of receiving a diagnosis. Familial ALS, a hereditary form of the disease, accounts for 5-10% of cases, whereas the remaining cases (sporadic ALS) have no clearly defined etiology. ALS affects persons of all races and ethnicities; however, persons of certain demographics (Caucasians, males, non-Hispanics and persons aged 60 years or older) and those with a family history of ALS are more likely to develop the disease.

ALS affects up to approximately 35,000 adults in the U.S. Evidence from prevalence studies suggests that prevalence and incident rates can vary significantly between regions and ethnicities. Most cases are sporadic ("sALS") but approximately 10% are found to have a familial, i.e., dominant genetic causation ("fALS"). fALS can be caused by mutations in various genes including chromosome 9 open reading frame 72, SOD1, tyrosyl-DNA phosphodiesterase 2 and others.

One genetic mutation that causes ALS is pathogenic mutations in the superoxide dismutase enzyme 1 ("SOD1"). SOD1 is an enzyme that is responsible for catalyzing toxic superoxide to hydrogen peroxide and dioxygen. While the exact mechanism for the disease is not known, it is believed that a toxic gain of function in SOD1 results in oxidative stress and cell death of motor neurons. More than 100 pathogenic SOD1 have been identified. Mutations are concentrated in a few regions of the protein. Mutations can be both dominant and recessive. The most common mutations in the SOD1 gene are D90A, G93A, A4H and D46R.

Patients with different mutations progress at different rates. It is estimated that there are approximately 300 to 600 patients with SOD1-ALS in the U.S.

Our Development of AMT-162 for ALS – SOD1

In January 2023 we announced that we entered into a global licensing agreement with Apic Bio for a novel, one-time, intrathecally administered gene therapy for ALS caused by SOD1 mutations (AMT-162 formerly APB-102). The APB-102 had an active Investigational New Drug ("IND") application and had been granted Orphan Drug and Fast Track designation by the FDA. APB-102 is comprised of a recombinant AAVrh10 vector that expresses a miRNA designed to knock down the expression of SOD1 with the goal of slowing down or potentially reversing the progression of ALS in patients with SOD1 mutations. The FDA cleared an updated IND application for AMT-162.

EPISOD1 is a Phase I/II multi-center, open-label trial of AMT-162 for the treatment of SOD1-ALS. In September 2025, we voluntarily paused enrollment in EPISOD1 upon the recommendation of the IDMC following a review of available preliminary data related to the safety and efficacy of AMT-162 in the context of a dose limiting toxicity, which resulted in an SAE determined to be related to AMT-162, that was observed in one patient in the second cohort. Following review of the preliminary efficacy and safety data generated from EPISOD1, we have decided to discontinue development of AMT-162. We will continue to collect safety data from the five patients dosed in EPISOD1, consistent with applicable safety and regulatory requirements.

Liver-directed diseases

Fabry disease program (AMT-191)

Fabry Disease and Market Background

Fabry disease is a progressive, inherited, multisystemic lysosomal storage disease characterized by specific neurological, cutaneous, renal, cardiovascular, cochleo-vestibular, and cerebrovascular manifestations. Fabry disease is caused by a defect in a gene that encodes for a protein called α-galactosidase A ("GLA"). The GLA protein is an essential enzyme required to breakdown globotriaosylsphingosine ("Gb3") and lyso-globotriaosylsphingosine ("lyso-Gb3"). In patients living with Fabry disease, Gb3 and lyso-Gb3 accumulate in various cells throughout the body causing progressive clinical signs and symptoms of the disease. Current treatment options, which consist of bi-weekly intravenous enzyme replacement therapy, typically have no therapeutic benefit in patients with advanced renal or

cardiac disease. Studies have also shown that a majority of male patients develop antibodies that inhibit the GLA protein and interfere with therapeutic efficacy.

Fabry disease has two major disease phenotypes: the type 1 "classic" and type 2 "later-onset" subtypes. Both lead to renal failure, and/or cardiac disease, and early death. Type 1 males have little or no functional a-Gal A enzymatic activity (<1% of normal mean) and marked accumulation of GL-3/Gb3 and related glycolipids in capillaries and small blood vessels which cause the major symptoms in childhood or adolescence. In contrast, males with the type 2 "later-onset" phenotype (previously called cardiac or renal variants) have residual a-Gal A activity, lack GL-3/Gb3 accumulation in capillaries and small blood vessels, and do not manifest the early manifestations of type 1 males. They experience an essentially normal childhood and adolescence. They typically present with renal and/or cardiac disease in the third to seventh decades of life. Most type 2 later-onset patients have been identified by enzyme screening of patients in cardiac, hemodialysis, renal transplant, and stroke clinics and recently by newborn screening. Fabry disease occurs in all racial and ethnic populations and affects males and females. It is estimated that type 1 classic Fabry disease affects at least one in 40,000 males and approximately one in 20,000 females. In the U.S., there may be approximately 7,000 type 1 Fabry patients. The type 2 later-onset phenotype is more frequent, and in some populations may occur as frequently as about 1 in 1,500 to 4,000 males.

Our Development of AMT-191 for Fabry Disease

AMT-191 is our investigational gene therapy candidate for the treatment of Fabry disease. AMT-191 is comprised of an AAV5 capsid that incorporates the GLA transgene and a proprietary, highly potent, liver-specific promoter. In 2023 we announced that the FDA had cleared the IND for AMT-191, and in 2024 we announced that the FDA granted Orphan Drug Designation and Fast Track Designation to AMT-191. We are conducting a Phase I/II clinical trial in the U.S. The multicenter, open-label clinical trial consists of three dose-ranging cohorts of three or more patients each to assess safety, tolerability, and efficacy of AMT-191 in patients with Fabry disease.

In February 2025, we announced the completion of enrollment in the first cohort of the Phase I/II trial. Additionally, the IDMC reviewed safety data from the initial two patients enrolled in the first cohort. The IDMC's review did not identify any significant safety concerns and recommended proceeding with enrollment in the second cohort.

In 2025, we enrolled three patients in a second cohort and four patients in a third cohort in our Phase I/II clinical trial of AMT-191.

Per protocol, additional dosing in the mid- and high-dose cohorts has been paused pending further evaluation following asymptomatic Grade 3 liver enzyme elevations observed in two patients in the mid-dose cohort, which were confirmed dose-limiting toxicity.

Hemophilia B (HEMGENIX® or etranacogene dezaparvovec)

Hemophilia B Disease and Market Background

Hemophilia B is a rare, lifelong bleeding disorder caused by a single gene defect, resulting in insufficient production of factor IX, a protein primarily produced by the liver that helps blood clots form. Treatments for moderate to severe hemophilia B include prophylactic infusions of factor IX replacement therapy to temporarily replace or supplement low levels of blood-clotting factor and, while these therapies are effective, those with hemophilia B must adhere to strict, lifelong infusion schedules. They may also still experience spontaneous bleeding episodes as well as limited mobility, joint damage or severe pain as a result of the disease. For appropriate patients, HEMGENIX allows people living with hemophilia B to produce their own factor IX, which can lower the risk of bleeding.

CSL Behring collaboration

In June 2020, we entered into a commercialization and license agreement with CSL Behring LLC (the "CSL Behring Agreement") pursuant to which CSL Behring LLC ("CSL Behring") received exclusive global rights to HEMGENIX.

We and CSL Behring also entered into a development and commercial supply agreement, pursuant to which, among other things, we agreed to supply the product to CSL Behring until such time that these capabilities are transferred to CSL Behring or its designated contract manufacturing organization (the "CSL Behring CSA"). CSL Behring in September 2022 informed us about their intent to transfer contract manufacturing to a designated third party. In July 2024 as part of our divestment of our commercial manufacturing operations to Genezen Holdings Inc. and its subsidiary Genezen MA, Inc. (together "Genezen"), we entered into a commercial supply agreement to transfer the manufacturing and supply activities to Genezen such that Genezen manufactures and supplies CSL Behring's commercial demand for HEMGENIX on our behalf (the "Genezen CSA").

In April 2026, we entered into agreements with CSL Behring and Genezen that provide for (i) the termination of our obligation to supply HEMGENIX and any minimum purchase commitments under the Genezen CSA, once the contractually specified batches have been supplied to CSL Behring, which we expect to occur in mid-2026, and (ii) the designation of Genezen as CSL Behring's contract manufacturing organization. In addition, the CSL Behring Agreement was amended to terminate certain manufacturing-related terms associated with both the CSL Behring CSA and the Genezen CSA, as well as our development support that CSL Behring could request from time to time with respect to HEMGENIX. All other terms of the CSL Behring Agreement remain in full force and effect.

New Technology Development

We are seeking to develop next-generation technologies with the goal of further improving the potential of AAV-based gene therapies to treat patients suffering from debilitating diseases. We are focused on innovative technologies across each of the key components of an AAV-based gene therapy, including: (i) the capsid, or the outer viral protein shell that encloses the target deoxyribonucleic acid ("DNA"); (ii) the cargo, including the transgene or therapeutic gene, and promoters, or the DNA sequence that drives or regulates the expression of the transgene; and (iii) administration techniques.

We dedicate significant effort to designing and screening novel AAV capsids with the potential for (i) higher biological potency; (ii) improved biodistribution including greater cell transduction and increased cellular specificity; (iii) enhanced safety; and (iv) manufacturing efficiency. We believe we have significant expertise in vector engineering and have created promising genetically engineered capsids using both rational and directed evolution approaches.

We have also demonstrated the ability to deliver engineered DNA constructs that can silence or suppress disease-causing genes. Our miQURE gene silencing platform, based on exclusively licensed technology from Cold Spring Harbor Laboratory ("CSHL"), is designed to degrade mutated genes without producing a "passenger strand" thereby mitigating potential off-target toxicity and induce silencing of the mutated gene in the entire target organ through secondary exosome-mediated delivery. miQURE-based gene therapy candidates, such as AMT-130, incorporate proprietary, therapeutic miRNA constructs that can be delivered using AAVs to potentially provide long-lasting activity. Preclinical studies of miQURE-based gene therapies have demonstrated several important advantages, including enhanced tissue-specificity, improved nuclear and cytoplasmic gene lowering and no off-target effects associated with impact to the cellular miRNA or messenger RNA transcriptome. The existing miQURE gene silencing strategy was expanded by linking several miRNA molecules in a single construct, resulting in the new linQURE platform.

Our Intellectual Property

We strive to protect the proprietary technologies that we believe are important to our business, including by seeking and maintaining patent protection in the U.S., Europe, and other jurisdictions for novel components of our gene therapies, the chemistries of and processes for manufacturing these gene therapies, the use of these components in gene therapies, our technology platform, and other inventions and technology. To the extent applicable, we also rely on trade secrets, know-how protection measures and monitoring of our proprietary information to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection.

We expect that our probability of success will be enhanced by our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business, defend and enforce our patents, maintain our licenses to use intellectual property owned by third parties, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and other proprietary rights of third parties. We also rely on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position in the field of AAV-based gene therapies.

In some cases, we are dependent on the patented or proprietary technology of third parties to develop and commercialize our products. We must obtain licenses from such third parties on commercially reasonable terms, or our business could be harmed, possibly materially. For example, we license from third parties essential parts of the therapeutic gene cassettes as well as the principal AAV vectors we use and key elements of our manufacturing process. We anticipate that we will require licenses to additional technology in the future.

Because most patent applications throughout the world are confidential for 18 months after the earliest claimed priority date, and since the publication of discoveries in scientific and patent literature often lags actual discoveries, we cannot be certain that we were the first to invent or file applications for the inventions covered by our pending patent applications. Moreover, we may have to participate in post-grant proceedings in the patent offices of the U.S. or foreign jurisdictions, such as oppositions, reexaminations, or interferences, in which the patentability or priority of our inventions are challenged. Such proceedings could result in substantial cost, even if the eventual outcome is favorable to us. For more information regarding the risks related to our intellectual property, please see Item 1A., *Risk factors—Risks Related to Our Intellectual Property*, in this Annual Report on Form 10-K.

Our intellectual property portfolio consists of owned and in-licensed patents, copyrights, licenses, trademarks, trade secrets and other intellectual property rights.

Patent Portfolio

Our gene therapy programs are protected by patents and patent applications directed to various aspects of our technology. For example, our gene therapy programs are protected by patents and patent applications with composition of matter or method of use claims that cover the therapeutic gene, the promoter, the viral vector capsid, or other specific parts of these technologies. We also seek protection of core aspects of our manufacturing process, particularly regarding our baculovirus expression system for AAV vectors in insect cells. In addition, we have filed manufacturing patent applications with claims directed to alternative compositions of matter and manufacturing processes to seek better protection from competitors.

We file the initial patent applications for our commercially important technologies in Europe and/or the U.S. For the same technologies, we typically file international patent applications under the Patent Cooperation Treaty ("PCT") within one year. After the PCT phase, we file patent applications in Europe or the U.S., as applicable based on the jurisdiction in which we file the first patent application. We also may seek, usually on a case-by-case basis, local patent protection in other jurisdictions, including Canada, Australia, Japan, China, India, Israel, South Africa, New Zealand, South Korea, and Eurasia, as well as South American jurisdictions, such as Brazil and Mexico. During prosecution of the patent applications, we may file divisional applications at the European Patent Office ("EPO") or divisionals or continuations in the U.S. prior to the grant or issue of the patent. For patent families which are considered particularly important, we may also file divisional applications in other jurisdictions.

As of December 31, 2025, our fully owned intellectual property portfolio included 147 issued patents (including 32 U.S. patents and 21 patents granted by the EPO) and 173 (published) pending patent applications (including 36 U.S. patent applications and 31 patent applications pending at the EPO). These patents relate to a variety of technologies including our product candidates that are in development as well as to our manufacturing and technology platforms.

We own three published patent families directed to gene therapy treatment of Huntington's disease, including AMT-130 and its formulation. AMT-130 is a miQURE®-based gene-transcript silencing technology designed to degrade disease-causing gene transcripts without off-target toxicity while inducing silencing throughout the entire target organ through secondary exosome-mediated delivery. We co-own two patent families, and fully own one patent family, directed to the gene therapy-mediated treatment of TLE, including our product candidate AMT-260. Of the two co-owned patent families, the co-owners have exclusively licensed their rights to us. Additionally, we are the exclusive licensee to two other patent families directed to the Gluk2/Gluk5 antagonists and their use in TLE. We have obtained an exclusive license to two patent families directed to gene therapy treatment of ALS, including AMT-162. We own a patent family directed to potent liver-specific promoters, including the promoter present in our product candidate AMT-191. Additionally, we own a patent family directed to the formulation of AMT-191 for intravenous infusion.

Licenses

We have obtained exclusive or non-exclusive rights from third parties under a range of patents and other technology that we use in our product and development programs, as described below. Our agreements with these third parties generally grant us a license to make, use, sell, offer to sell, and import products covered by the licensed patent rights in exchange for our payment of some combination of an upfront amount, annual fees, royalties, a percentage of amounts we receive from our licensees and payments upon the achievement of specified development, regulatory or commercial milestones. Some of the agreements specify the extent of the efforts we must use to develop and commercialize licensed products. The agreements generally expire upon expiration of the last-to-expire valid claim of the licensed patents. Each licensor may terminate the applicable agreement if we materially breach our obligations and fail to cure the breach within a specified cure period.

Licensed Technology Used for Multiple Programs

We are exploiting technology from third-party sources described below in more than one of our programs.

Cold Spring Harbor Laboratory

In 2015, we entered into a license agreement with CSHL in which CSHL granted to us an exclusive, sublicensable license to develop and commercialize certain of CSHL's patented RNAi-related technology for use in connection with the treatment or prevention of Huntington's disease. We expanded the scope of the license agreement with CSHL in 2018 beyond Huntington's disease. In addition, pursuant to the 2018 amendment, we were granted a license to use the RNAi-related technology for research in the fields of liver diseases, neuromuscular diseases, and cardiovascular diseases.

Under this license agreement, as amended, annual fees, development milestone payments and future single-digit royalties on net sales of a licensed product are payable to CSHL. The standard 20-year patent term for the licensed patents expires in 2031.

Protein Sciences

In 2016, we revised our existing license contract with Protein Sciences Corporation for the use of its *expresSF+* insect cell line and associated technology for human therapeutic and prophylactic uses (except influenza) to provide us with a royalty free, perpetual right and license to the technology in the field of AAV-based gene therapy.

Technology Used for Specific Development Programs

Temporal Lobe Epilepsy (AMT-260)

Regenxbio

In June 2020, uniQure France entered into an agreement, subsequently amended in June 2021, with Regenxbio for an exclusive (in the field of using AAV9 to expression of the *GRIK2* gene in humans (the "Field")), sublicensable, royalty-bearing, worldwide license under Regenxbio's interest in EU patent application 19185533.7 (the "Foreground Patents") and related patents, as well as patents covering inventions developed during the collaboration and certain patents and know-how relating to AAV9. The license also includes non-exclusive rights to exploit the licensed Foreground Patents and certain related patents know-how developed in collaboration pursuant to the license agreement outside the Field. The license also includes retained and license back rights that permit Regenxbio and its upstream licensors to exploit for any research, development, commercialization, or other purposes certain patents, inventions and know-how (other than the Foreground Patents) subject to or created pursuant to the license agreement.

Payment obligations under the agreement provide for royalty payments on net sales in the mid-single digit to low-double digits, and milestone payments to Regenxbio in the mid-tens of millions of dollars related to clinical trials, commercialization, and net sales. The agreement also calls for sublicense fees in the low-double digit range. The royalty is paid on sales of licensed products using any of licensed patents or know-how for as long as the agreement is in effect. Royalty and milestone payments may continue to be owed under the license, following termination of the agreement if licensed products are sold following termination of the license. Under the agreement, uniQure France has certain diligence obligations and Regenxbio has certain obligations related to the pre-clinical development of manufacturing technology.

Inserm Transfert

In January 2020, uniQure France entered into a license agreement with Inserm Transfert SA (also acting as a delegate for the French National Institute of Health and Medical Research) and La société SATT Aquitaine (the counterparties collectively referred to as "Inserm Transfert"). Under the license agreement, uniQure France is granted an exclusive, sublicensable, royalty-bearing, worldwide license under European Patent ("EP") patent application 13306265.3 in the field of the prevention and treatment of epilepsy, and in Inserm Transfert's share in EP patent application 19185533.7 (which is co-owned by Regenxbio) in the field of all human use. uniQure France is also granted a non-exclusive, sublicensable, royalty-bearing, worldwide license under certain know-how in the fields that may be developed by Inserm pursuant to the agreements. Under the agreements, Inserm Transfert retains certain rights for teaching, academic and/or research purposes.

Payment obligations under the agreements include a royalty on the net sales of license products in the low single digits, milestone payments associated with clinical trial and regulatory approval milestones of multiple licensed products totaling in the low-single digit millions of Euros. The agreement also calls for sublicense fees in the low to mid double-digit range depending on the timing of such sublicense. The obligation to pay royalties extends until the later of the expiration of the patent rights, any regulatory exclusivity period, and 10 years from the first commercial sale of a licensed product.

Amyotrophic Lateral Sclerosis (AMT-162)

In January 2023, we announced that we had entered into a global licensing agreement with Apic Bio for a one-time, intrathecally administered investigational gene therapy for ALS caused by mutations in SOD-1, pursuant to which we acquired an exclusive global license (including a sublicense of rights granted to Apic Bio pursuant to an exclusive license agreement with a certain U.S.-based academic institution) to Apic Bio's rights under certain licensed technology to develop, manufacture, and commercialize any product incorporating a licensed construct (including APB-102, certain constructs expressing a SOD1-targeting miRNA or AAV that codes for a miRNA that silences SOD1 expression), in any dosage strength, formulation, concentration or method of delivery in the applicable field. We made an initial cash payment of $10.0 million to Apic Bio. In addition, we will pay Apic Bio up to $43.0 million in milestones upon achievement of regulatory approvals in the U.S. and Europe and pre-specified annual net sales, and a tiered royalty on net sales ranging from the mid-single digits to low-double digits.

Trade Secrets

In addition to patents and licenses, we rely on trade secrets and know-how to develop and maintain our competitive position. For example, significant aspects of the process by which we manufacture certain of our gene therapies are based on unpatented trade secrets and know-how. We seek to protect our proprietary technology and processes and obtain and maintain ownership of certain technologies, in part, through confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and commercial collaborators. We also seek to preserve the integrity and confidentiality of our data, trade secrets and know-how by maintaining physical security of our premises and physical and electronic security of our information technology systems.

Trademarks

We have a number of material registered trademarks, including "uniQure", that we have registered in various jurisdictions including the U.S. and the EU. We may seek trademark protection for other product candidates and technologies as and when appropriate.

Competition

The biotechnology and pharmaceutical industries, including in the gene therapy field, are characterized by rapidly advancing technologies, intense competition, and a strong emphasis on intellectual property. We face substantial competition from many different sources, including large and specialty pharmaceutical and biotechnology companies, academic and other research institutions and governmental agencies and public and private research institutions that are developing and commercializing pharmaceutical products that may be competitive with ours.

Our key competitors, focused on developing therapies in various indications, include among others, Novartis, Roche, Wave Life Sciences, Alnylam Pharmaceuticals, Regeneron Pharmaceuticals, Skyhawk Therapeutics and Sarepta Therapeutics (for Huntington's disease), Neurona Therapeutics, EpilepsyGTx and Xenon Pharmaceuticals (for TLE), Biogen, Ionis Pharmaceuticals, Neurimmune, Regeneron Pharmaceuticals, Alnylam Pharmaceuticals, Coya Therapeutics, Amylyx Pharmaceuticals, NeuroSense Therapeutics, AL-S Pharma and Voyager Therapeutics (for ALS) and Amicus Therapeutics, Sanofi, Takeda, Chiesi, Idorsia, Sangamo Therapeutics, 4D Molecular Therapeutics, Skyline Pharmaceuticals and CANbridge (for Fabry disease).

We also compete with existing standards of care, therapies, and symptomatic treatments, as well as any new therapies and novel technologies, which may become available in the future for the indications we are targeting.

Many of our current or potential competitors, either alone or with their collaborators, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials regulatory affairs, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical, biotechnology and gene therapy industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

The key competitive factors affecting the success of all our programs are likely to be their efficacy, safety, convenience, price, and the availability of reimbursement from government and other third-party payers. We also believe that, due to the small size of the patient populations in the orphan indications we target, being first to market will be a significant competitive advantage. We believe that our advantages in vector and manufacturing technology will enable us to reach market in a number of indications ahead of our competitors, and to potentially capture the markets in these indications either by being first or in those markets with larger populations having a differentiated product.

Government Regulation and Reimbursement

Government authorities in the U.S., EU and other countries extensively regulate, among other things, the approval, research, development, nonclinical and clinical testing, manufacture (including any manufacturing changes), packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting, reimbursement, and import and export of pharmaceutical products, biological products, and medical devices. We believe that all our product candidates will be regulated as biological products, or biologics, and in particular, as gene therapies, and will be subject to such requirements and regulations under U.S. and foreign laws. If we fail to comply with applicable regulatory requirements, we may be subject to, among other things, civil penalties, refusal to approve pending applications, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions, and criminal prosecution.

Regulation in the United States

In the U.S., the FDA regulates biologics under the Public Health Service Act ("PHSA") and the Federal Food, Drug, and Cosmetic Act ("FDCA") and regulations and guidance implementing these laws. These laws and regulatory guidance are continually evolving. By example, various actions have been taken by the U.S. Congress and President over recent years with respect to drug shortage prevention and reporting, supply chain security, and the promotion of U.S. domestic manufacturing. The FDA also continually issues nonbinding guidance documents that provide the FDA's interpretation of its laws and regulations, as well as the FDA's approach to scientific issues and questions.

Obtaining regulatory approvals and ensuring compliance with applicable statutes and regulatory requirements entails the expenditure of substantial time and financial resources, including payment of user fees for applications to the FDA. All our current product candidates are subject to regulation by the FDA as biologics. An applicant seeking approval to market and distribute a new biologic in the U.S. must typically undertake the following:

- completion of nonclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's current Good Laboratory Practice regulations;

- submission to the FDA of an IND application which allows human clinical trials to begin unless the FDA objects within 30 days; the sponsor of an IND or its legal representative must be based in the U.S.;

- approval by an independent institutional review board ("IRB") and, for some studies, Institutional Biosafety Committee ("IBC") before each clinical trial may be initiated;

- performance of adequate and well-controlled human clinical trials in accordance with the FDA's current Good Clinical Practice regulations ("cGCP") to establish substantial evidence of the safety and efficacy for the proposed biological product for each indication;

- preparation and submission to the FDA of a BLA;

- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities at which the product, or components thereof, are produced to assess compliance with current Good Manufacturing Practice ("cGMP") requirements and to assure that the facilities, methods, and controls are adequate to preserve the product's identity, strength, quality, and purity, as well as selected clinical trial sites and investigators to determine cGCP compliance;

- approval of the BLA by the FDA, in consultation with an FDA advisory committee, if deemed appropriate by the FDA; and

- compliance with any post-approval commitments, including Risk Evaluation and Mitigation Strategies ("REMS"), and post-approval studies required by the FDA.

Human Clinical Studies in the United States under an IND

Before initiating clinical studies in the U.S. or under an IND, investigational product sponsors must first complete nonclinical studies. Nonclinical studies include laboratory evaluation of chemistry, pharmacology, toxicity, and product formulation, as well as animal studies to assess potential safety and efficacy. Such studies must generally be conducted in accordance with the FDA's Good Laboratory Practices. In 2025, however, the FDA announced a plan to reduce the need for animal testing, with an initial focus on monoclonal antibodies.

Clinical trials involve the administration of the investigational biologic to human subjects under the supervision of qualified investigators in accordance with cGCP requirements, which includes requirements for informed consent, study conduct, and IRB review and approval. Special clinical trial ethical considerations also must be considered if a study involves children. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of an IND. INDs include nonclinical study reports, together with manufacturing information, analytical data, any available clinical data, or literature, and proposed clinical study protocols among other things. A clinical trial may not proceed in the U.S. unless and until an IND becomes effective, which is 30 days after its receipt by the FDA. The FDA may raise concerns or questions related to one or more components of an IND and place the IND on clinical hold if during its review the FDA determines that study subjects would be exposed to significant risk of illness or injury. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. Clinical holds also may be imposed by the FDA at any time before or during trials due to safety concerns or non-compliance.

The protocol and informed consent documents, as well as other subject communications must also be approved by an IRB that continues to oversee that trial. In the case of gene therapy studies, an IBC at the local level may also review and maintain oversight over the particular study, in addition to the IRB. The FDA, an IRB, and IBC, or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk or that research requirements are not being met.

Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor that regularly reviews accumulated data and advises the study sponsor regarding the continuing safety of the trial. This group may also review interim data to assess the continuing validity and scientific merit of the clinical trial. This group receives special access to unblinded data during the clinical trial and may advise the sponsor to halt, pause, or otherwise modify the clinical trial.

Information about certain clinical trials, including results, must be submitted within specific timeframes for listing on the ClinicalTrials.gov website. Sponsors or distributors of investigational products for the diagnosis, monitoring, or treatment of one or more serious diseases or conditions must also have a publicly available policy on evaluating and responding to requests for expanded access. Investigators must also provide certain information to the clinical trial sponsors to allow the sponsors to make certain financial disclosures to the FDA.

Subsequent clinical protocols and amendments must also be submitted to an active IND but are not subject to the 30-day review period imposed on an original IND. Progress reports detailing the results of the clinical trials must also be submitted at least annually to the FDA and the IRB and more frequently if serious adverse events or other significant safety information is found. There is a risk that once a new protocol or amendment is submitted to an active IND there may be an extended period before the FDA may comment or provide feedback. This may result in a need to modify an ongoing clinical trial to incorporate this feedback or even a clinical hold of the trial. There is also risk that the FDA may not provide comments or feedback but may ultimately disagree with the design of the study once a BLA is submitted.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- Phase I: The biological product is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early understanding of its effectiveness.

- Phase II: The biological product is administered to a limited patient population to further identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.

- Phase III: The biological product is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in adequate and well-controlled clinical trials to generate sufficient data to statistically confirm the potency and safety of the product for approval, to establish the overall risk-benefit profile of the product and to provide adequate information for the labelling of the product. Typically, two Phase III trials are required by the FDA for product approval. Under some limited circumstances, however, the FDA may approve a BLA based upon a single Phase III clinical study plus confirmatory evidence or a single large multicenter trial without confirmatory evidence.

Legislation further established a new program that may be used to facilitate future marketing applications and development programs following a first product approval. Specifically, the Consolidated Appropriations Act, 2023 established a program whereby a platform technology that is incorporated within or utilized by an approved drug or biologic product may be designated as a platform technology, provided that certain conditions are met, in which case development and approval of subsequent products using such technology may be expedited.

In addition, under the Pediatric Research Equity Act of 2003 (the "PREA"), a BLA or BLA supplement for a new active ingredient, indication, dosage form, dosage regimen, or route of administration, must contain data that are adequate to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Orphan products are also exempt from the PREA requirements.

The manufacture of investigational drugs and biologics for the conduct of human clinical trials is subject to cGMP requirements. Investigational drugs and biologics and active ingredients and therapeutic substances imported into the U.S. are also subject to regulation by the FDA. Further, the export of investigational products outside of the U.S. is subject to regulatory requirements of the receiving country as well as U.S. export requirements under the FDCA.

Concurrent with clinical trials, companies usually complete additional nonclinical animal studies and must also develop additional information about the chemistry and physical characteristics of the product candidate as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, manufacturers must develop methods for testing the identity, strength, quality, potency, and purity of the final product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

Regulation and FDA Guidance Governing Gene Therapy Products

The FDA has and continues to issue various guidance documents with respect to the development and commercialization of gene therapies, including for specific diseases, such as neurodegenerative diseases, rare diseases, and hemophilia, as such products may face special challenges.

Certain gene therapy studies are also subject to the National Institutes of Health's Guidelines for Research Involving Recombinant DNA Molecules, ("NIH Guidelines"). The NIH Guidelines include the review of the study by an IBC. The IBC assesses the compliance of the research with the NIH Guidelines, assesses the safety of the research and identifies any potential risk to public health or the environment.

Compliance with cGMP Requirements

Manufacturers of biologics must comply with applicable cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Manufacturers and others involved in the manufacture and distribution of such products must also register their establishments with the FDA and certain state agencies and provide the FDA a list of products manufactured at the facilities. Sponsors must further submit information to the FDA regarding manufactured products, including the volume of drugs produced during the prior

year. Establishments may be subject to periodic unannounced inspections by government authorities to ensure compliance with cGMPs and other laws. Discovery of non-compliance may result in the FDA issuing a Form 483, placing restrictions on a product, manufacturer, or holder of an approved BLA, and may extend to requiring withdrawal of the product from the market, recall, shutdown, or enforcement letters, among other consequences. Noncompliance with the applicable manufacturing requirements may also require costly corrective and preventative actions. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are following cGMP requirements and adequate to assure consistent production of the product within required specifications.

FDA Programs to Expedite Product Development

The FDA has several programs to expedite product development, including fast track designation and breakthrough therapy designation. These are outlined in specific FDA guidance. Under the fast track program, the sponsor of a biologic candidate may request the FDA to designate the product for a specific indication as a fast track product concurrent with or after the filing of the IND for the product candidate. To be eligible for a fast track designation, the FDA must determine that a product candidate is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address an unmet medical need. This may be demonstrated by clinical or nonclinical data. If granted, the benefits include more frequent interactions with the FDA and potentially rolling review of sections of the BLA. In some cases, a fast track product may be eligible for accelerated approval or priority review.

Moreover, under the provisions of the Food and Drug Administration Safety and Innovation Act, enacted in 2012, a sponsor can request designation of a product candidate as a breakthrough therapy. A breakthrough therapy is defined as a product that is intended, alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Products designated as breakthrough therapies are potentially eligible for rolling review, as well as intensive guidance on an efficient development program beginning as early as Phase I trials, and a commitment from the FDA to involve senior managers and experienced review staff in a proactive collaborative, cross disciplinary review.

Biologics studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval, which means the FDA may approve the product based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on an intermediate clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. A biologic candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. By the date of approval of an accelerated approval product, FDA must specify the conditions for the required post approval studies, including enrollment targets, the study protocol, milestones, and target completion dates. FDA may also require, and frequently does require, that the confirmatory Phase 4 studies be commenced prior to FDA granting a product accelerated approval. Reports on the progress of the required Phase 4 confirmatory studies must be submitted to FDA every 180 days after approval. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow the FDA to withdraw the drug or biologic from the market on an expedited basis using a statutorily defined streamlined process. Failure to conduct the required Phase 4 confirmatory studies or to conduct such studies with due diligence, as well as failure to submit the required update reports can subject a sponsor to penalties. All promotional materials for drug or biologic candidates approved under accelerated regulations are subject to prior review by the FDA. In recent years, the accelerated approval pathway has come under significant FDA and public scrutiny. Accordingly, the FDA may be more conservative in granting accelerated approval or, if granted, may be more apt to withdrawal approval if clinical benefit is not confirmed.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Submission of a BLA

The results of the nonclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls, and proposed labeling, among other things, are submitted to the FDA as part of a BLA requesting a license to market the product for one or more indications. The submission of a BLA is subject to an application user fee, though products with orphan designation are exempt from the BLA filing fee. The sponsor of an approved BLA is also subject to annual program user fees. Orphan products may also be exempt from program fees provided that certain criteria are met. These fees are typically increased annually. Under the Prescription Drug User Fee Act ("PDUFA") the FDA has agreed to specified performance goals in the review of BLAs.

Most such applications are meant to be reviewed within ten months from the filing acceptance date (typically 60 days after date of filing), and most applications for priority review products are meant to be reviewed within six months of the filing acceptance date (typically 60 days after date of filing). Priority review designation may be assigned to product candidates that are intended to treat serious conditions and, if approved, would provide significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of the serious condition.

The FDA may refuse to file an application and request additional information. In this event, the application must be refiled with the additional information. The refiled application is also subject to assessment of content before the FDA accepts it for review. Once the submission is accepted, the FDA begins an in-depth substantive review. The FDA will assign a date for its final decision for the product (the "PDUFA action date") but can extend this date to complete review of a product application or to consider additional information submitted during the application review period. The PDUFA action date is only a goal, thus, the FDA does not always meet its PDUFA dates.

The FDA may also refer certain applications to an advisory committee. An advisory committee is typically a panel that includes clinicians and other experts, which reviews, evaluates, and makes recommendations as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

The FDA reviews applications to determine, among other things, whether a product candidate meets the agency's approval standards and whether the manufacturing methods and controls are adequate to assure and preserve the product's identity, strength, quality, potency, and purity. Before approving a marketing application, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities, including contract manufacturers and subcontractors, are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a marketing application the FDA will inspect one or more clinical trial sites to assure compliance with cGCPs.

After evaluating the marketing application and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the biological product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information for the FDA to reconsider the application. In 2025, the FDA began publicly releasing complete response letters after issuance, for both products that eventually obtained approval and products that have not yet received approval. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the BLA, the FDA will issue an approval letter. Many drug applications receive complete response letters from the FDA during their first cycle of FDA review.

If the FDA approves a product, it may limit the approved indications for use of the product; require that contraindications, warnings, or precautions be included in the product labeling, including boxed warnings; require that post-approval studies, including Phase 4 clinical trials and trials to ensure that population representative data is collected, be conducted to further assess a biologic's efficacy and safety after approval; or require testing and surveillance programs to monitor the product after commercialization. The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs. The FDA may also not approve label statements that are necessary for successful commercialization and marketing.

In addition to the above conditions of approval, the FDA also may require submission of a REMS to ensure that the benefits of the product candidate outweigh the risks. The REMS plan could include medication guides, physician communication plans, and elements to assure safe use, such as restricted distribution methods, patient registries, or other risk minimization tools. An assessment of the REMS must also be conducted at set intervals. Following product approval, a REMS may also be required by the FDA if new safety information is discovered, and the FDA determines that a REMS is necessary to ensure that the benefits of the product outweigh the risks. During the review of a BLA for a gene therapy, the FDA will assess whether a REMS is necessary. Several gene therapy products that have been approved by the FDA have required substantial REMS, which included requirements for dispensing hospital and clinic certification, training, adverse event reporting, documentation, and regulatory audits and monitoring conducted by the sponsor, among other conditions. REMS, such as these, can be expensive and burdensome to implement, and burdensome for hospitals, clinics, and healthcare providers to comply with.

Biosimilars and Exclusivity

The Biologics Price Competition and Innovation Act of 2009 ("BPCIA") which amended the PHSA authorized the FDA to approve biosimilars under Section 351(k) of the PHSA. Under the BPCIA, a manufacturer may apply for licensure of a biologic product that is biosimilar to or interchangeable with a previously approved biological product or reference product. For the FDA to approve a biosimilar product, it must find that it is highly similar to the reference product notwithstanding minor differences in clinically inactive components and that there are no clinically meaningful differences between the reference product and proposed biosimilar product in safety, purity or potency. A finding of interchangeability requires that a product is determined to be biosimilar to the reference product, and that the product can be expected to produce the same clinical results as the reference product and, for products administered multiple times, the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic.

An application for a biosimilar product may not be submitted to the FDA until four years following approval of the reference product, and it may not be approved until 12 years following approval of the reference product. These exclusivity provisions only apply to biosimilar companies and not companies that rely on their own data and file a full BLA. Moreover, this exclusivity is not without limitation. Certain changes and supplements to an approved BLA, and subsequent applications filed by the same sponsor, manufacturer, licensor, predecessor in interest, or other related entity do not qualify for the twelve-year exclusivity period. Further, the twelve-year exclusivity period in the U.S. for biologics has been controversial and may be shortened in the future.

The PHSA also includes provisions to protect reference products that have patent protection. The biosimilar product sponsor and reference product sponsor may exchange certain patent and product information for the purpose of determining whether there should be a legal patent challenge. Based on the outcome of negotiations surrounding the exchanged information, the reference product sponsor may bring a patent infringement suit and injunction proceedings against the biosimilar product sponsor. The biosimilar applicant may also be able to bring an action for declaratory judgment concerning the patent.

The FDA maintains a list of approved biological products, which is commonly referred to as the Purple Book. This list includes product names, the date of licensure, and any periods of regulatory exclusivity. Following the exchange of patent information between the biosimilar and reference product sponsor, the reference product sponsor must also provide the exchanged patent information and patent expiry dates to the FDA. The FDA then publishes this information in the Purple Book.

To increase competition in the drug and biologic product marketplace, Congress, the executive branch, and the FDA have taken certain legislative and regulatory steps. For example, the FDA finalized certain guidance documents to facilitate biologic product importation. Moreover, the 2020 Further Consolidated Appropriations Act included provisions requiring that sponsors of approved biologic products, including those subject to REMS, provide samples of the approved products to persons developing biosimilar products within specified timeframes, in sufficient quantities, and on commercially reasonable market-based terms. Failure to do so can subject the approved product sponsor to civil actions, penalties, and responsibility for attorney's fees and costs of the civil action. This same legislation also includes provisions with respect to shared and separate REMS programs.

Orphan Drug Exclusivity

Under the Orphan Drug Act of 1983, the FDA may designate a biological product as an orphan drug if it is intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the U.S., or more in cases in which there is no reasonable expectation that the cost of developing and making a biological product available in the U.S. for treatment of the disease or condition will be recovered from sales of the product, and where there is a medically plausible basis for the use of the drug for the rare disease or condition. Additionally, sponsors must present a plausible hypothesis for clinical superiority to obtain orphan drug designation if there is a product already approved by the FDA that is considered by the FDA to be the same as the already approved product and is intended for the same indication. This hypothesis must be demonstrated to obtain orphan exclusivity. With respect to gene therapies, the FDA has issued a specific guidance on how the agency interprets its sameness regulations. Specifically, whether two products are deemed to be the same by the FDA will depend on the products' transgene expression, viral vectors groups and variants, and additional product features that may contribute to therapeutic effect. Minor product differences will not, generally, result in a finding that two products are different and there are some factors that the FDA will consider on a case-by-case basis. Any of the FDA sameness determinations could impact our ability to receive approval for our product candidates and to obtain or retain orphan drug exclusivity.

If a product with orphan designation receives the first FDA approval, it may be granted seven years of marketing exclusivity, which means that the FDA may not approve any other applications for the same product for the same indication for seven years, unless clinical superiority is demonstrated. Competitors may receive approval of different products for the indication for which the orphan product has exclusivity and may obtain approval for the same product but for a different indication. Orphan drug designation does not change the FDA's standard for product approval.

Pediatric Exclusivity

Under the PREA, pediatric exclusivity provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity in the US, including orphan exclusivity and reference biologic exclusivity. This six-month exclusivity may be granted if the FDA issues a written request to the sponsor for the pediatric study, the sponsor submits a final study report after receipt of the written request and meets the terms and timelines in the FDA's written request.

Regenerative Medicine Advanced Therapy Designation

The 21st Century Cures Act became law in December 2016 and created a new program under Section 3033 in which the FDA has authority to designate a product as a RMAT. A drug is eligible for a RMAT designation if: 1) it is a regenerative medicine therapy which is a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, except those products already regulated under Section 361 of the PHSA; 2) the drug is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and 3) preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such disease or condition. A RMAT designation request must be made with the submission of an IND or as an amendment to an existing IND. The FDA will determine if a product is eligible for RMAT designation within 60 days of submission. Advantages of the RMAT designation include all the benefits of the fast track and breakthrough therapy designation programs, including early interactions with the FDA. These early interactions may be used to discuss potential surrogate or intermediate endpoints to support accelerated approval. In 2019 the FDA stated in guidance that human gene therapies, including genetically modified cells, that lead to a sustained effect on cells or tissues, may meet the definition of a regenerative therapy.

FDA Regulation of Companion Diagnostics and Other Combination Products

We may seek to develop companion diagnostics for use in identifying patients who we believe are more likely to respond to our gene therapies. Similarly, our product candidates may require delivery devices. A biologic product may be regulated as a combination product if it is intended for use in conjunction with a medical device, such as a drug delivery device or an in vitro diagnostic device. For combination products, the biologic and device components must, when used together, be safe and effective and the product labeling must reflect their combined use. In some cases, the medical device component may require a separate premarket submission. Moreover, clinical trial sponsors using investigational devices in their studies must comply with the FDA's investigational device exemption regulations. If the device component (e.g., in vitro diagnostic device) is not packaged with the drug component and

authorized by the FDA as a combination product, or approved or cleared as a medical device, the device component must comply with the FDA general controls applicable to a medical device, including establishment registration, device listing, device labeling, unique device identifier, quality system regulation, medical device reporting, and reporting of corrections and removals requirements. If the device component is packaged with the drug component (e.g., drug delivery device), then only certain FDA general controls applicable to a medical device will apply (assuming the manufacturer's quality system complies with the cGMPs).

If the safety or effectiveness of a biologic product is dependent on the results of a diagnostic, the FDA may require that the in vitro companion diagnostic device and biologic product be contemporaneously authorized by the FDA, with labeling that describes the use of the two products together. The type of premarket submission required for a companion diagnostic device will depend on the FDA device classification. A premarket approval ("PMA"), application is required for high-risk devices classified as Class III; a 510(k) premarket notification is generally required for moderate risk devices classified as Class II. Low risk devices classified as Class I generally do not require any FDA premarket authorization, with limited exceptions. A de novo request may be used for novel devices not previously classified by the FDA (and hence are automatically Class III) but are low or moderate risk (due to the application of special controls) and thus can be re-classified as Class II or Class I. Except in some limited circumstances, the FDA generally will not approve a biologic that is dependent upon the use of a companion diagnostic device if the device is not contemporaneously FDA-approved or -cleared. It is also possible that a diagnostic test could be considered outside the scope of the FDA's regulatory authority if it meets the definition of a Laboratory Developed Test ("LDT"). The FDA attempted to actively regulate all LDTs as medical devices through a final rule in April 2024, but this rule was challenged in the US District Court for the Eastern District of Texas, which found that LDTs are not medical devices and thus not subject to the FDA's device authority. Nonetheless, LDTs remain subject to the regulatory requirements under the Clinical Laboratory Improvement Amendments.

Post-approval Requirements

Any products manufactured or distributed pursuant to the FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements related to manufacturing, recordkeeping, and reporting, including adverse experience reporting, deviation reporting, shortage reporting, and periodic reporting, product sampling and distribution, long term follow-up, advertising, marketing, promotion, certain electronic records and signatures, and post-approval obligations imposed as a condition of approval, such as Phase 4 clinical trials, REMS, and surveillance to assess safety and effectiveness after commercialization.

After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing annual program user fee requirements for approved products, excluding orphan products provided that certain criteria are met. Regulatory authorities may withdraw product approvals, require label modifications, or request product recalls, among other actions, if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

Changes to the manufacturing process are strictly regulated and often require prior FDA approval or notification before being implemented. For example, in 2023, the FDA issued a guidance specifically on demonstrating product comparability, and the management and reporting of manufacturing changes for investigational and licensed cellular and gene therapy products. FDA regulations also require investigation and correction of any deviations from cGMP and specifications and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in production and quality control to maintain cGMP compliance.

In addition to the above manufacturing requirements, the FDA and the current U.S. presidential administration have taken actions to encourage the use of U.S. domestic manufacturing and supply chains. This includes executive orders intended to facilitate and streamline the development of U.S. manufacturing, enhance the inspection of foreign manufacturing facilities, and increase foreign manufacturing facility user fees.

The FDA also strictly regulates marketing, labeling, advertising, and promotion of products that are placed on the market. A company can make only those advertising and promotional claims relating to a product that are consistent with the label approved by the FDA. Physicians, in their independent professional medical judgment, may prescribe legally available products for unapproved indications that are not described in the product's labeling and that differ from those tested and that have been approved by the FDA. Biopharmaceutical companies, however, are

required to promote their products only for the approved indications and in a manner that is consistent with the provisions of the approved label. Companies must also provide adequate balancing information on a product's risks in its advertising and promotional pieces. In 2023, the FDA took certain regulatory actions in the advertising and promotional spaces, including issuing a final rule and a guidance on risk and efficacy disclosures in direct-to-consumer advertising. At the beginning of 2025, the FDA also finalized guidance on communication of off-label scientific information about approved products. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability, including, but not limited to, criminal fines and civil penalties under the FDCA and False Claims Act, exclusion from participation in federal healthcare programs, mandatory compliance programs under corporate integrity agreements, suspension and debarment from government procurement and non-procurement programs, and refusal of orders under existing government contracts.

Moreover, the enacted Drug Quality and Security Act ("DQSA"), imposes obligations on sponsors of biopharmaceutical products related to product tracking and tracing. Among the requirements of this legislation, sponsors are required to provide certain information regarding the products to individuals and entities to which product ownership is transferred, are required to label products with a product identifier, and are required to keep certain records regarding the product. The transfer of information to subsequent product owners by sponsors is also required to be done electronically and allow interoperable electronic product tracing at the package level. Sponsors must also verify that purchasers of the sponsors' products are appropriately licensed. Further, under this legislation, manufacturers have product verification responsibilities, as well as investigation, quarantine, disposition, and notification responsibilities related to counterfeit, diverted, stolen, and intentionally adulterated products that would result in serious adverse health consequences or death to humans, as well as products that are the subject of fraudulent transactions or which are otherwise unfit for distribution such that they would be reasonably likely to result in serious health consequences or death. Similar requirements are also imposed on other entities in the biopharmaceutical supply chain, including distributors and dispensers, as well as certain sponsor licensees and affiliates.

Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements before or after approval, may result in significant regulatory actions. Such actions may include refusal to approve pending applications, license or approval suspension or revocation, imposition of a clinical hold or termination of clinical trials, warning letters, untitled letters, cyber letters, modification of promotional materials or labeling, provision of corrective information, imposition of post-market requirements including the need for additional testing, imposition of distribution or other restrictions under a REMS, product recalls, product seizures or detentions, refusal to allow imports or exports, total or partial suspension of production or distribution, FDA debarment, injunctions, consent decrees, corporate integrity agreements, suspension and debarment from government procurement and non-procurement programs, refusal of orders under existing government contracts, exclusion from participation in federal and state healthcare programs, restitution, disgorgement, civil penalties, criminal prosecution, including fines and imprisonment, and adverse publicity, among other adverse consequences.

Additional Controls for Biologics

To help reduce the increased risk of the introduction of adventitious agents, the PHSA emphasizes the importance of manufacturing controls for products whose attributes cannot be precisely defined. The PHSA also provides authority to the FDA to immediately suspend licenses in situations where there exists a danger to public health, to prepare or procure products in the event of shortages and critical public health needs, and to authorize the creation and enforcement of regulations to prevent the introduction or spread of communicable diseases in the U.S. and between states.

After a BLA is approved, the product may also be subject to official lot release as a condition of approval. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing the results of all the manufacturer's tests performed on the lot. The FDA may also perform certain confirmatory tests on lots of some products before releasing the lots for distribution by the manufacturer.

In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products.

Patent Term Restoration

If approved, biologic products may also be eligible for periods of U.S. patent term restoration. If an application for patent term restoration is timely filed with the U.S. Patent and Trademark Office and granted, patent term restoration extends the patent life of a single unexpired patent, that has not previously been extended, for a maximum of five years. The total patent life of the product with the extension cannot exceed fourteen years from the product's approval date. Subject to the prior limitations, the period of the extension is calculated by factoring in half of the time from the effective date of an IND to the initial submission of a complete marketing application, all the time between the submission of the marketing application and its approval, and any period that the applicant did not act with due diligence.

Anti-Kickback Provisions and other Fraud and Abuse Requirements

The federal Anti-Kickback Statute ("AKS") is a criminal statute that prohibits, among other things, knowingly and willfully offering, paying, soliciting, or receiving remuneration directly or indirectly, overtly or covertly, in cash or in kind, to induce or in return for purchasing, leasing, ordering, or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs, in whole or in part. The term "remuneration" has been interpreted broadly to include anything of value. The AKS has been interpreted to apply to arrangements between biopharmaceutical industry members on the one hand and prescribers, purchasers, and formulary managers on the other. The Beneficiary Inducement Civil Monetary Penalties Law imposes similar restrictions on interactions between the biopharmaceutical industry and federal healthcare program beneficiaries. There are certain statutory exceptions and regulatory safe harbors to the AKS protecting some common activities from prosecution. The exceptions and safe harbors are drawn narrowly, and practices that involve remuneration that may be alleged to be intended to induce or reward prescribing, purchases, or recommendations may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the AKS. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all its facts and circumstances.

Several courts have interpreted the statute's intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce or reward referrals of federal healthcare program business, including purchases of products paid by federal healthcare programs, the statute has been violated. The Patient Protection and Affordable Care Act, of 2010, as amended, (the "ACA") modified the intent requirement under the AKS to a stricter standard, such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it to have committed a violation. In addition, the ACA also provided that a violation of the AKS is grounds for the government or a whistleblower to assert that a claim for reimbursement submitted to a federal healthcare program for payment of items or services resulting from such violation constitutes a per se false or fraudulent claim for purposes of the federal civil False Claims Act. The Department of Health and Human Services promulgated regulations in November 2020 with respect to the safe harbors that are effective in two phases. First, the regulation excludes from the definition of "remuneration" limited categories of (a) Pharmacy Benefit Manager ("PBM") rebates or other reductions in price to a plan sponsor under Medicare Part D or a Medicaid Managed Care Organization plan reflected in point-of-sale reductions in price and (b) PBM service fees. Second, the regulation expressly provides that rebates to plan sponsors under Medicare Part D, either directly to the plan sponsor under Medicare Part D or indirectly through a PBM, will not be protected under the AKS discounts safe harbor. The Inflation Reduction Act of 2022 ("IRA") extended a moratorium on the implementation, administration or enforcement of this final rule until January 1, 2032.

The federal civil False Claims Act prohibits any person from knowingly presenting, or causing to be presented, a false or fraudulent claim for payment to, or approval by, the federal government, knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government, or avoiding, decreasing, or concealing an obligation to pay money to the federal government. A claim includes "any request or demand" for money or property presented to the U.S. government. The civil False Claims Act has been used to assert liability on the basis of kickbacks and other improper referrals, improperly reported government pricing metrics such as Best Price or Average Manufacturer Price, improper use of Medicare provider or supplier numbers when detailing a provider of services, improper promotion of off-label uses not expressly approved by the FDA in a product's label, and allegations as to misrepresentations with respect to products, contract requirements, and services rendered. In addition, private payers have been filing follow-on lawsuits alleging fraudulent misrepresentation, although establishing liability and damages in these cases is more difficult than under the False Claims Act. Intent to deceive is not required to establish liability under the civil False Claims Act. Rather, a claim

may be false for deliberate ignorance of the truth or falsity of the information provided or for acts in reckless disregard of the truth or falsity of that information. Civil False Claims Act actions may be brought by the government or may be brought by private individuals on behalf of the government, called "qui tam" actions. If the government decides to intervene in a qui tam action and prevails in the lawsuit, the individual will share in the proceeds from any damages, penalties or settlement funds. If the government declines to intervene, the individual may pursue the case alone. The civil False Claims Act provides for treble damages and a civil penalty for each false claim, such as an invoice or pharmacy claim for reimbursement, which can aggregate into tens and even hundreds of millions of dollars. For these reasons, since 2004, False Claims Act lawsuits against biopharmaceutical companies have increased significantly in volume and breadth, leading to several substantial civil and criminal settlements, as much as $3.0 billion, regarding certain sales practices and promoting off label uses. Civil False Claims Act liability may further be imposed for known Medicare or Medicaid overpayments, for example, overpayments caused by understated rebate amounts, that are not refunded within 60 days of the identification of the overpayment, even if the overpayment was not caused by a false or fraudulent act. In addition, civil judgment for violating the False Claims Act may result in exclusion from federal healthcare programs, suspension and debarment from government procurement and non-procurement programs, and refusal of orders under existing government contracts. The majority of states also have statutes similar to the AKS and civil False Claims Act, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer.

The government may further prosecute conduct constituting a false claim under the criminal False Claims Act. The criminal False Claims Act prohibits the making or presenting of a claim to the government knowing such claim to be false, fictitious, or fraudulent and, unlike the civil False Claims Act, requires proof of intent to submit a false claim.

The Civil Monetary Penalties Law is another potential statute under which biopharmaceutical companies may be subject to enforcement. Among other things, the civil monetary penalties statute imposes fines against any person who is determined to have knowingly presented, or caused to be presented, claims to a federal healthcare program that the person knows, or should know, is for an item or service that was not provided as claimed or is false or fraudulent.

Payment or reimbursement of prescription therapeutics by Medicaid or Medicare requires sponsors to submit certified pricing information to the Centers for Medicare and Medicaid Services ("CMS"). The Medicaid Drug Rebate statute requires sponsors to calculate and report price points, which are used to determine Medicaid manufacturer rebate payments shared between the states and the federal government and Medicaid payment rates for certain therapeutics. For therapeutics paid under Medicare Part B, sponsors must also calculate and report their Average Sales Price, which is used to determine the Medicare Part B payment rate. In addition, therapeutics covered by Medicaid are subject to an additional inflation penalty which can substantially increase rebate payments. For certain products, including those approved under a BLA (including biosimilars), the Veterans Health Care Act (the "VHCA") requires sponsors to calculate and report to the Department of Veterans Affairs ("VA") a different price called the Non-Federal Average Manufacturer Price, which is used to determine the maximum price that can be charged to certain federal agencies, referred to as the Federal Ceiling Price ("FCP"). Like the Medicaid rebate amount, the FCP includes an inflation penalty. A Department of Defense regulation requires sponsors to provide this discount on therapeutics dispensed by retail pharmacies when paid by the TRICARE Program. All these price reporting requirements create risk of submitting false information to the government, potential False Claims Act liability and exclusion from certain of these programs.

The VHCA also requires sponsors of covered therapeutics participating in the Medicaid program to enter into Federal Supply Schedule contracts with the VA through which their covered therapeutics must be sold to certain federal agencies at FCP. This necessitates compliance with applicable federal procurement laws and regulations, including submission of commercial sales and pricing information, and subjects companies to contractual remedies as well as administrative, civil, and criminal sanctions. In addition, the VHCA requires sponsors participating in Medicaid to agree to provide different mandatory discounts to certain Public Health Service grantees and other safety net hospitals and clinics under the 340B program based on the sponsor's reported Medicaid pricing information. The 340B program has its own regulatory authority to impose sanctions for non-compliance, adjudicate overcharge claims against sponsors by the purchasing entities, and impose civil monetary penalties for instances of overcharging.

The federal Health Insurance Portability and Accountability Act of 1996, ("HIPAA"), also created federal criminal statutes that prohibit, among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any of the

money or property owned by, or under the custody or control of, a healthcare benefit program, regardless of whether the payer is public or private, in connection with the delivery of or payment for healthcare benefits, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense and knowingly and willfully falsifying, concealing, or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items, or services relating to healthcare matters. Additionally, the ACA amended the intent requirement of certain of these criminal statutes under HIPAA so that a person or entity no longer needs to have actual knowledge of the statute, or the specific intent to violate it, to have committed a violation.

In addition, as part of the ACA, the federal government enacted the Physician Payment Sunshine Act. Manufacturers of drugs, biologics and devices for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program (with certain exceptions) are required to annually report to CMS certain payments and other transfers of value made to or at the request of covered recipients, which are physicians (as defined under the Social Security Act), physician assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists and certified nurse midwifes licensed in the U.S. and U.S. teaching hospitals, as well as ownership and investment interests held by physicians and members of their immediate family. Payments made to principal investigators and research institutions for clinical trials are also included within this law. Reported information is made publicly available by CMS. Failure to submit required information may result in civil monetary penalties. If not preempted by this federal law, several states currently also require reporting of marketing and promotion expenses, as well as gifts and payments to healthcare professionals and organizations. State legislation may also prohibit gifts and various other marketing related activities or require the public posting of information. Certain states also require companies to implement compliance programs.

Further, we may be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, ("HITECH Act"), and their respective implementing regulations impose certain requirements on covered entities relating to the privacy, security, and transmission of certain individually identifiable health information known as protected health information. Among other things, the HITECH Act, and its implementing regulations, made HIPAA's security standards and certain privacy standards directly applicable to business associates, defined as persons or organizations, other than members of a covered entity's workforce, that create, receive, maintain, or transmit protected health information on behalf of a covered entity for a function or activity regulated by HIPAA. The HITECH Act also strengthened the civil and criminal sanctions that may be imposed against covered entities, business associates, and individuals, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions. HIPAA privacy rules governing disclosures of protected health information by covered entities for research purposes may apply to gene therapy studies.

In addition, other federal and state laws, including state health information privacy laws, state security breach notification laws, and state consumer privacy laws (e.g., the California Consumer Privacy Act), may govern the privacy and security of health and other information in certain circumstances. Some states, like Washington State, have passed healthcare-specific privacy laws. For example, the My Health My Data Act became effective March 31, 2024 and restricts how entities collect, use, and process "consumer health data," defined broadly as personal information that is linked or reasonably linkable to a consumer and that identifies the consumer's health status. While HIPAA-regulated entities may be exempt from this act, the exemption is based on the data collected and used rather than on the entity's status as a HIPAA covered entity or business associate. As such, some data may be subject to this act and HIPAA, while other data may only be subject to HIPAA. State laws may be more stringent, broader in scope or offer greater individual rights with respect to health-related information or other personal information than HIPAA. Complying with these various state laws and regulations, which may differ from state to state, requires significant resources and may complicate our compliance efforts. Penalties for violation of any of these laws and regulations may include civil and/or criminal penalties.

Additionally, U.S. federal and state consumer protection laws may, among other things, require us to publish statements that describe how we handle personal information and choices individuals may have about the way we handle their personal information.

Many states have also adopted laws similar to each of the above federal laws, which may be broader in scope and apply to items or services reimbursed by any third-party payer, including commercial insurers. Certain state laws also regulate sponsors' use of prescriber-identifiable data. Certain states also require implementation of commercial

compliance programs and compliance with the pharmaceutical industry's voluntary compliance guidelines and the applicable compliance program guidance promulgated by the federal government, or otherwise restrict payments or the provision of other items of value that may be made to healthcare providers and other potential referral sources; impose restrictions on marketing practices; or require sponsors to track and report information related to payments, gifts, and other items of value to physicians and other healthcare providers and entities. Recently, states have enacted or are considering legislation intended to make drug prices more transparent and deter significant price increases that impose reporting requirements on biopharmaceutical companies. These laws may affect our future sales, marketing, and other promotional activities by imposing administrative and compliance burdens. Such laws also typically impose significant civil monetary penalties for each instance of reporting noncompliance that can quickly aggregate into the tens of millions of dollars.

If our operations are found to be in violation of any of the laws or regulations described above or any other laws that apply to us, we may be subject to penalties or other enforcement actions, including significant civil monetary penalties, damages, criminal fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, corporate integrity agreements, suspension and debarment from government procurement and non-procurement programs, refusal of orders under existing government contracts, reputational harm, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our business.

U.S. Foreign Corrupt Practices Act

The U.S. Foreign Corrupt Practices Act, to which we are subject, imposes certain recordkeeping requirements and prohibits corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party, or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity.

Coverage, Pricing and Reimbursement

The containment of healthcare costs has become a priority of federal, state, and foreign governments, and the prices of drugs have been a focus in this effort. Third-party payers and independent non-profit healthcare research organizations such as the Institute for Clinical and Economic Review are also increasingly challenging the prices charged for medical products and services and examining the medical necessity, budget-impact, and cost-effectiveness of medical products and services, in addition to their safety and efficacy. If these third-party payers do not consider a product to be cost-effective compared to other available therapies and/or the standard of care, they may not cover the product after approval as a benefit under their plans or, if they do, measures including prior authorization and step-throughs could be required, manufacturer rebates may be negotiated or required and/or the level of payment may not be sufficient to allow a company to sell its products at a profit. The U.S. federal and state governments and foreign governments have shown significant interest in implementing cost containment programs to limit the growth of government-paid healthcare costs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products for branded prescription drugs. In this regard, for example, on November 27, 2020, CMS issued an interim final rule implementing a Most Favored Nation payment model under which reimbursement for certain Medicare Part B drugs and biologicals will be based on a price that reflects the lowest per capital Gross Domestic Product-adjusted ("GDP-adjusted") price of any non-U.S. member country of the Organization for Economic Co-operation and Development ("OECD") with a GDP per capita that is at least sixty percent of the U.S. GDP per capita. Although this rule was subsequently rescinded, in November 2025, CMS introduced the GENEROUS Model, which is expected to launch in 2026, and which requires manufacturers to provide rebates aligned with Most Favored Nation pricing to participating state Medicaid programs that opt into the program. Further, government negotiation of certain Medicare drug pricing continues to be the focus of recent proposed legislation. The Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. Failure of the Joint Select Committee on Deficit Reduction to reach required deficit reduction goals triggered the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers up to 2% per fiscal year. While President Biden previously signed legislation to eliminate this reduction through the end of 2021, a 1% payment adjustment was implemented from April 1 – June 30, 2022, and a 2% payment adjustment took effect beginning July 1, 2022. The IRA requires manufacturers of selected drugs to negotiate discounted prices with the Secretary of the Department of Health and Human Services. Failure to reach an agreement can subject manufacturers to an excise tax or withdrawal of all drug products from coverage under Medicare and Medicaid. In addition to entering into direct agreements with manufacturers, on December 1, 2025, the

U.S. entered into a trade agreement with the UK to permit tariff-free imports of pharmaceutical products in exchange for reductions in certain rebates paid by manufacturers to the UK Department of Health and Social Care. Adoption of additional healthcare reform controls and measures and tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceuticals.

As a result, the marketability of any product which receives regulatory approval for commercial sale may suffer if the government and third-party payers choose to provide low coverage and reimbursement. In addition, an increasing emphasis on managed care in the U.S. has increased and will continue to increase the pressure on drug pricing. Decisions regarding whether to cover any of our products, the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. Further, no uniform policy for coverage and reimbursement exists in the U.S., and coverage and reimbursement can differ significantly from payer to payer. Coverage policies, third-party reimbursement rates and drug pricing regulation may change at any time. In particular, the ACA contains provisions that may reduce the profitability of drug products, including, for example, increased rebates for drugs sold to Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies' share of sales to federal healthcare programs. Multiple other current and proposed legislative and regulatory efforts require and likely will in the future require payment of increased manufacturer rebates and implement mechanisms to reduce drug prices. Even if favorable coverage and reimbursement status is attained for one or more products that receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Regulation in the European Union and United Kingdom

The regulatory framework for medicinal products in the EU and the UK is broadly similar to that of the U.S., and many of the considerations described above apply in these jurisdictions. Pharmaceutical products in the EU and UK are also subject to national pricing and reimbursement controls.

Following the UK's withdrawal from the EU in January 2020 ("Brexit") and the end of the transition period in December 2020, the Medicines and Healthcare products Regulatory Agency ("MHRA") became the UK's standalone medicines regulator as of January 1, 2021. Under the Northern Ireland Protocol, different regulatory rules apply in Northern Ireland than in Great Britain, with Northern Ireland generally continuing to follow the EU regulatory regime.

On January 1, 2025, the Windsor Framework came into effect to simplify the supply of medicines between Great Britain and Northern Ireland, under which EU pharmaceutical legislation does not apply in all circumstances in Northern Ireland. EU pharmaceutical legislation adopted after the end of the Brexit transition period generally does not apply in Great Britain (but may apply in Northern Ireland).

Clinical Trials

The Clinical Trial Regulation EU 536/2014 ("CTR"), which replaced the Clinical Trials Directive 2001/20/EC, as amended ("CTD"), on January 31, 2022, provides a system for the approval of clinical trials in the EU. The CTR is directly applicable in all member states without the need for national implementation. Applicants make a single harmonized electronic submission and have a single assessment process for clinical trials conducted in multiple member states. Furthermore, a clinical trial may only be started after a competent ethics committee has issued a favorable opinion on the Clinical Trial Application ("CTA"), which must be supported by an investigational medicinal product dossier with supporting information prescribed by the CTR and corresponding national laws of the member states and further detailed in applicable guidance documents. In the case of Advanced Therapy Investigational Medicinal Products ("ATIMPs") consisting of or containing Genetically Modified Organisms ("GMOs"), as is the case for our products, an additional approval for the environmental and biosafety aspects of the use and release of the GMO is required by the GMO competent authorities. Unlike EU Regulations, EU Directives require national implementation and GMO Directives have been implemented in different ways by Member States; either following the Directive for "Contained use" (Directive 2009/41/EC) or "deliberate release" (Directive 2001/18/EC). As a consequence, in some EU member states the GMO application must be approved before the CTA is submitted, in some after approval of the CTA, and in some, in parallel.

The sponsor of a clinical trial, or its legal representative, must be based in the European Economic Area ("EEA"). European regulators and ethics committees also require the submission of adverse event reports during a

study and a copy of the final study report. Under the CTR, member states may dispense with the requirement for a legal representative for a non-EU resident sponsor provided there is a contact person based in the EEA.

Under the CTR, the introduction of a new database called the Clinical Trial Information System ("CTIS"), requires sponsors to upload and submit all data, including initial clinical trial application data and documentation, to the CTIS, with such data being publicly available, with few exceptions and with limited ability to redact the documents. This means there is data transparency throughout the development process with the onus on sponsors to protect patient and commercial confidentiality at the point of submission.

The CTR does not apply in the UK. Instead, the regulatory framework in relation to clinical trials is currently derived from the CTD (as implemented into UK law, through the Medicines for Human Use (Clinical Trials) Regulations 2004, as amended). However, in April 2025, new Regulations – the Medicines for Human Use (Clinical Trials) (Amendment) Regulations 2025 – were finalized that will replace the current regulatory framework for clinical trials. The new regulations aim to provide a more flexible regime to make it easier to conduct clinical trials in the UK, increase the transparency of clinical trials conducted in the UK and make clinical trials more patient centered. The new regulations will take full effect in April 2026.

Marketing approval

Marketing approvals under the EU regulatory system may be obtained through a centralized or decentralized procedure. The centralized procedure results in the grant of a single marketing authorization that is valid in all 27 EU member states. Pursuant to Regulation (EC) No 726/2004, as amended, the centralized procedure is mandatory for drugs developed by means of specified biotechnological processes, and advanced therapy medicinal products as defined in Regulation (EC) No 1394/2007, as amended. Drugs for human use containing a new active substance for which the therapeutic indication is the treatment of specified diseases, including but not limited to acquired immune deficiency syndrome, neurodegenerative disorders, auto-immune diseases and other immune dysfunctions, as well as drugs designated as orphan drugs pursuant to Regulation (EC) No 141/2000, as amended, also fall within the mandatory scope of the centralized procedure. Because of our focus on gene therapies, which fall within the category of advanced therapy medicinal products ("ATMPs") and orphan indications, our products and product candidates will need to go through the centralized procedure.

In the marketing authorization application ("MAA") the applicant must properly and sufficiently demonstrate the quality, safety, and efficacy of the drug. The Committee for Medicinal Products for Human Use ("CHMP") is the European Medicines Agency's ("EMA") committee responsible for human medicines. Guidance on the factors that the EMA will consider in relation to the development and evaluation of ATMPs have been issued and include, among other things, the nonclinical studies required to characterize ATMPs; the manufacturing and control information that should be submitted in an MAA; and post-approval measures required to monitor patients and evaluate the long-term efficacy and potential adverse reactions of ATMPs. Although these guidelines are not legally binding, we believe that our compliance will effectively be necessary to gain and maintain approval for any of our product candidates. The maximum timeframe for the evaluation of an MAA under the centralized procedure is 210 days after receipt of a valid application subject to clock stops during which the applicant deals with CHMP questions. Initially, a standard marketing authorization ("MA") is valid for 5 years, but can be renewed for unlimited validity.

The EU also provides for a system of regulatory data and market exclusivity. According to Article 14(11) of Regulation (EC) No 726/2004, as amended, and Article 10 of Directive 2001/83/EC, as amended, as well as European case law and guidance, upon receiving an MA, new active substances approved on the basis of complete independent data package benefit from eight years of data exclusivity and an additional two years of market exclusivity. Data exclusivity prevents regulatory authorities in the EU from referencing the innovator's data to assess a generic (abbreviated) application during the eight-year period from the date of notification of the MA. During the additional two-year period of market exclusivity, a generic marketing authorization can be submitted, and the innovator's data may be referenced, but no generic medicinal product can be placed on the market until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of eleven years if, during the first eight years of those ten years, the MA holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be a new active substance and the innovator can gain the period of data exclusivity, another company nevertheless could also market another version of the drug if such company obtained marketing authorization based on an MAA with a complete independent data package of pharmaceutical test, preclinical tests, and clinical trials that does not reference the data within the original dossier. The

EMA has also issued guidelines for a comprehensive comparability exercise for biosimilars, and for specific classes of biological products and has recently conducted a consultation on streamlining this procedure. In addition, under recently agreed revisions to the EU's pharmaceutical legislation, the standard period of data protection will be reduced, but this may be extended if particular criteria are fulfilled. The final text of the amendments is not yet available, so it is not certain when and in what form the new legislation will be adopted or how it will impact the development of our products or the protections they may receive.

Under Regulation (EC) No 141/2000 article 3 as amended (Orphan Drug Regulation, ("ODR")) a product can benefit from orphan drug status if it is intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 people in the European Community when the application is made and there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the European Community or, if such method exists, that the medicinal product will be of significant benefit to those affected by that condition. The principal benefit of such status, if confirmed at the time of marketing authorization, is 10 years' market exclusivity once the MA is granted, during which the authorization shall not accept another application for a MA, or grant a MA or accept an application to extend an existing marketing authorization, for the same therapeutic indication, in respect of a similar medicinal product. This may be reduced to six years under certain circumstances including if the product is sufficiently profitable not to justify maintenance of market exclusivity. Under the above-mentioned proposed new legislation, the current 10-year period could be reduced for certain products, but may be extended by one year if certain conditions are met.

Additional rules apply to medicinal products for pediatric use under Regulation (EC) No 1901/2006, as amended. This includes obligations to carry out pediatric research. Potential incentives include a six-month extension of any supplementary protection certificate granted pursuant to Regulation (EC) No 469/2009; however, this is not available in cases in which the relevant product is designated as an orphan medicinal product pursuant to the ODR. Instead, medicinal products designated as orphan medicinal product may enjoy an extension of the ten-year market exclusivity period granted under Regulation (EC) No 141/2000, as amended, to twelve years subject to the conditions applicable to orphan drugs. The proposed new legislation will retain the 6-month SPC extension but the current separate reward of two years market exclusivity for pediatric indications of orphan products would no longer apply.

The position in the UK is broadly similar, although based on UK legislation. In order to obtain a UK MA to commercialize products in the UK, an applicant must be established in the UK and must follow one of the UK national authorization procedures or one of the post-Brexit international recognition procedures. Applications are governed by the Human Medicines Regulations (SI 2012/1916). While the review process and documentation are similar to the EU, the MHRA has introduced changes to national licensing procedures, including procedures to prioritize access to new medicinal products that will benefit patients, such as a 150-day assessment (subject to clock-stops) and a rolling review procedure. In addition, an international recognition procedure ("IRP") has been in place since January 1, 2024. Pursuant to the IRP, the MHRA will take into account the expertise and decision-making of trusted regulatory partners (i.e., the regulators in Australia, Canada, Switzerland, Singapore, Japan, the U.S. and the EU). The MHRA will conduct a targeted assessment of IRP applications but retain the authority to reject applications if the evidence provided is considered insufficiently robust. The IRP allows medicinal products approved by such trusted regulatory partners that meet certain criteria to undergo a fast-tracked MHRA review to obtain and/or update an MA in the UK. There are also procedures in the UK for conditional approvals or approvals under exceptional circumstances that are similar to the procedures in the EU.

Regulatory data and market exclusivity operate in broadly the same way as in the EU. However, there is no pre-MA orphan designation in the UK. Instead, the MHRA reviews applications for orphan designation in parallel to the corresponding MA application. The criteria are essentially the same, but have been tailored for the market, i.e., the prevalence of the condition in the UK, rather than the EU, must not be more than five in 10,000. Should an orphan designation be granted, the period or market exclusivity will be set from the date of first approval of the product in the UK. Once finalized, the changes to the framework in the EU will not apply in the UK, although may apply to certain products, depending on what basis they are placed on the market in the UK.

In addition, the UK has announced its intention to introduce a new regulatory pathway for orphan medicinal products to make it quicker and easier to get these therapies tested, manufactured and approved in the UK. Consultation on this framework will take place in 2026.

Programs to expedite authorization in the EU and UK

In the EU, there is the possibility to obtain approval via an accelerated assessment whereby, on request, the CHMP can reduce the timeframe to 150 days if the applicant provides sufficient justification for an accelerated assessment by demonstrating that the medicinal product is expected to be of major public health interest, particularly from the point of view of therapeutic innovation.

Market access can also be expedited through various mechanisms, including the grant of conditional authorization on the basis of less comprehensive data for a medicine that may fulfil unmet needs, and which may be granted provided that the benefit-risk balance of the product is positive. The benefit-risk balance is likely to be positive if the applicant can provide data that the benefit to public health of the medicinal product's immediate availability on the market outweighs the risks due to need for further data. Such authorizations are valid for one year and can be renewed annually. The holder will be required to complete specific obligations (ongoing or new studies, and in some cases additional activities) with a view to providing comprehensive data confirming that the benefit-risk balance is positive. Once comprehensive data on the product have been obtained, the conditional authorization may be converted into a standard MA (not subject to specific obligations). As noted above, a standard MA is initially valid for 5 years, but can be renewed for unlimited validity. Applicants for conditional authorizations can benefit from early dialogue with CHMP through scientific advice or protocol assistance and discuss their development plan well in advance of the submission of an MAA. Other stakeholders (e.g., health technology assessment bodies) can be included.

In addition, the priority medicines ("PRIME") scheme for medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients without treatment options based on early clinical data, is intended to support the development of medicines that target an unmet medical need. This voluntary scheme is based on enhanced interaction and early dialogue with developers of promising medicines, to optimize development plans and speed up evaluation so these medicines can reach patients earlier. Early dialogue and scientific advice also ensure that patients only participate in trials designed to provide the data necessary for an application, making the best use of limited resources.

MHRA has also introduced procedures to prioritize access to new medicinal products that will benefit patients, such as an expedited 150-day assessment (subject to clock-stops) and a rolling review procedure. Further, similar to the position in the EU, the MHRA offers a national conditional marketing authorization scheme for new medicinal products that is available where comprehensive clinical data are not yet complete, but it is judged that such data will become available soon. The criteria is similar to the position in the EU, and a conditional MA will be initially valid for one year and will be renewable annually.

There are also various schemes and pilots that are aimed at joining up the various steps in the authorization and access pathway in the UK, such as the Innovative Licensing and Access Pathway ("ILAP") that provides coordinated advice from the MHRA, reimbursement authorities and the national healthcare system to try to speed up the time for authorized products to be accessed by patients. Similarly, there are frameworks to provide patients with life-threatening or seriously debilitating conditions access to promising new medicines before they receive an MA, such as the Early Access to Medicines Scheme ("EAMS"). Each of these schemes have different access criteria and requirements to be met, but are aimed at speeding up development and access to innovative products.

Manufacturing and promotion

Pursuant to European Commission Directive 2003/94/EC as transposed into the national laws of the member states, the manufacturing of investigational medicinal products and approved drugs is subject to a separate manufacturer's license and must be conducted in strict compliance with cGMP requirements, which mandate the methods, facilities, and controls used in manufacturing, processing, and packing of drugs to assure their quality, safety and identity. Manufacturers must have at least one qualified person permanently and continuously at their disposal. The qualified person is ultimately responsible for certifying that each batch of finished product released onto the market has been manufactured in accordance with cGMP and the specifications set out in the marketing authorization or investigational medicinal product dossier. cGMP requirements are enforced through mandatory registration of facilities and inspections of those facilities. Failure to comply with these requirements could interrupt supply and result in delays, unanticipated costs, and lost revenues, and subject the applicant to potential legal or regulatory action, including but not limited to warning letters, suspension of manufacturing, seizure of product, injunctive action, or possible civil and criminal penalties. The same principles apply in the UK.

Advertising

In the EU, the promotion of prescription medicines is subject to intense regulation and control, including a prohibition on direct-to-consumer advertising. All medicines advertising must be consistent with the product's approved summary of products characteristics, and be factual, accurate, balanced and not misleading. Advertising of medicines pre-approval or off-label is prohibited. Some jurisdictions require that all promotional materials for prescription medicines be subjected to either prior internal or regulatory review and approval. The same principles apply in the UK.

Other Regulatory Requirements

A holder of a MA for a medicinal product is legally obliged to fulfill several obligations by virtue of its status as a marketing authorization holder ("MAH"). The MAH can delegate the performance of related tasks to third parties, such as distributors or marketing collaborators, provided that this delegation is appropriately documented and the MAH maintains legal responsibility and liability.

The obligations of an MAH in the EU and the UK include:

- *Manufacturing and Batch Release.* MAHs should guarantee that all manufacturing operations comply with relevant laws and regulations, applicable GMP, with the product specifications and manufacturing conditions set out in the marketing authorization and that each batch of product is subject to appropriate release formalities.

- *Pharmacovigilance.* MAHs are obliged to establish and maintain a pharmacovigilance system, including a qualified person responsible for oversight, to submit safety reports to the regulators and comply with the good pharmacovigilance practice guidelines adopted by the EMA (as adapted by the MHRA in the UK).

- *Advertising and Promotion.* MAHs remain responsible for all advertising and promotion of their products, including promotional activities by other companies or individuals on their behalf and in some cases, must conduct internal or regulatory pre-approval of promotional materials.

- *Medical Affairs/Scientific Service.* MAHs are required to establish a scientific service that can disseminate scientific and medical information on their medicinal products to healthcare professionals, regulators, and patients.

- *Legal Representation and Distributor Issues.* MAHs are responsible for regulatory actions or inactions of their distributors and agents.

- *Preparation, Filing and Maintenance of the Application and Subsequent Marketing Authorization.* MAHs must maintain appropriate records, comply with the MA's terms and conditions, fulfill reporting obligations to regulators, submit renewal applications and pay all appropriate fees to the authorities.

We may hold any future marketing authorizations granted for our product candidates in our own name or appoint an affiliate or a collaborator to hold MAs on our behalf. Any failure by an MAH to comply with these obligations may result in regulatory action against an MAH and ultimately threaten our ability to commercialize our products.

Reimbursement

In the EU and the UK, the pricing and reimbursement mechanisms by private and public health insurers vary largely by country and even within countries. In respect of the public systems, reimbursement for standard drugs is determined by guidelines established by the legislature or responsible national authority. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. Other member states allow companies to determine the prices for their medicines but monitor and control company profits and may limit or restrict reimbursement and can include retrospective rebates to the government. The downward pressure on healthcare costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products and some of EU countries and the UK

require the completion of studies that compare the cost-effectiveness of a particular product candidate to currently available therapies to obtain reimbursement or pricing approval. Special pricing and reimbursement rules may apply to orphan drugs.

Inclusion of orphan drugs in reimbursement systems tend to focus on the medical usefulness, need, quality and economic benefits to patients and the healthcare system as for any drug. Acceptance of any medicinal product for reimbursement may come with cost, use and often volume restrictions, which again can vary by country. In addition, results-based rules or agreements on reimbursement may apply. Recently, in the EU, a process has been formalized that allows sponsors to receive parallel advice from EMA and relevant national health technology assessment ("HTA") bodies for pivotal clinical studies designed to support marketing approval. This process was followed for etranacogene dezaparvovec. EU Regulation (EU) 2021/2282 also creates a framework for the joint clinical assessment of products where that assessment can be used for national pricing and reimbursement decisions.

Orphan Drug Regulation

We have been granted orphan drug exclusivity for AMT-130 for the treatment of Huntington's disease subject to the conditions applicable to orphan drug exclusivity in the EU. No pre-marketing authorization orphan designation step is required in the UK, but similar criteria will apply at the time of MA. The ODR states that a drug will be designated as an orphan drug if its sponsor can establish:

- that it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in ten thousand persons in the community when the application is made, or that it is intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition in the EU and that without incentives it is unlikely that the marketing of the drug in the EU would generate sufficient return to justify the necessary investment; and
- that there exists no satisfactory method of diagnosis, prevention, or treatment of the condition in question that has been authorized in the EU or, if such method exists, that the drug will be of significant benefit to those affected by that condition.

Regulation (EC) No 847/2000 sets out further provisions for implementation of the criteria for designation of a drug as an orphan drug. An application for the designation of a drug as an orphan drug must be submitted at any stage of development of the drug before filing of an MAA and the criteria are also reassessed at the time of marketing authorization.

If an EU-wide community marketing authorization in respect of an orphan drug is granted pursuant to Regulation (EC) No 726/2004, as amended, the EU and the member states will not, for a period of 10 years, accept another MAA, or grant a MA or accept an application to extend an existing MA, for the same therapeutic indication, in respect of a similar drug. Similar orphan exclusivity will be available in the UK if relevant criteria are met at the time of MA.

This period may however be reduced to six years if, at the end of the fifth year, it is established, in respect of the drug concerned, that the criteria for orphan drug designation are no longer met, in other words, when it is shown on the basis of available evidence that the product is sufficiently profitable not to justify maintenance of market exclusivity. Notwithstanding the foregoing, a MA may be granted, for the same therapeutic indication, to a similar drug if:

- the holder of the MA for the original orphan drug has given its consent to the second applicant;
- the holder of the MA for the original orphan drug is unable to supply sufficient quantities of the drug; or
- the second applicant can establish in the application that the second drug, although similar to the orphan drug already authorized, is safer, more effective, or otherwise clinically superior.

Regulation (EC) No 847/2000 sets forth definitions of the concepts of similar drug and clinical superiority, which concepts have been expanded upon in subsequent European Commission guidance and case law. Other incentives available to orphan drugs in the EU include financial incentives such as a reduction of fees or fee waivers and protocol assistance. Orphan drug designation does not shorten the duration of the regulatory review and approval process. As described above, the above proposed legislation would, if implemented, impact orphan protection in the EU in the future.

Human Capital Resources

As of December 31, 2025, we had a total of 221 employees, 126 of whom are based in The Netherlands, 83 in the United States of America, and 12 in other European countries. As of December 31, 2025, 72 of our employees had an M.D. or Ph.D. degree, or the foreign equivalent. During 2017, we established a works council in the Netherlands. None of our employees are subject to collective bargaining agreements or other labor organizations. We believe that we have good relations with all our employees and with the works council in the Netherlands.

We pride ourselves on our dedication to teamwork and uphold values that are:

- Passionate - Why we are here. We are driven by a deep passion for making a difference in patients' lives, channeling our energy and commitment into every project and celebrating the impact of our work together;

- Tenacious - How we move forward. We push through challenges with grit, focus, and heart, relentlessly pursuing continuous improvement and scientific breakthroughs. We never give up — because patients are counting on us;

- Honorable - How we behave. We build trust with each other, and the wider patient community we serve, through honesty, integrity, and support for one another. By acting with respect and compassion, we create a foundation for lasting success; and,

- Exceptional - What we deliver. We give our best in everything we do, setting ambitious goals and holding ourselves to the highest standards. Our patients deserve nothing less.

Our people are a critical component in our continued success. We strive to maximize the potential of our human capital resources by creating a respectful, rewarding and inclusive work environment that enables our employees to further our values. Development of our culture is reflected as part of our annual corporate goals. We invest in numerous learning opportunities focused on individual, management and team development and other initiatives to support our employees and build our culture.

2 Financial results

Financing

Public offerings

In January 2025, we raised $70.1 million of net proceeds, after deducting underwriting discounts and commissions and issuance costs payable by us, through a follow-on public offering of 4.4 million ordinary shares at a price to the public of $17.00 per ordinary share. In February 2025, we raised an additional $10.4 million in net proceeds upon the underwriters' exercise of their option to purchase an additional 0.7 million ordinary shares at the public offering price.

In September 2025, we received net proceeds of $323.7 million, after deducting underwriting discounts and commissions and other issuance costs payable by us, through a follow-on public offering of 6.7 million ordinary shares at a public offering price of $47.50 per ordinary share, and, in lieu of ordinary shares to certain investors, pre-funded warrants to purchase 0.5 million of our ordinary shares at the public offering price per share less the $0.0001 per share exercise price of each pre-funded warrant.

Hercules loan repayment and amendment

In September 2025, we entered into the 2025 Amended Facility with Hercules for a $175.0 million senior secured term loan facility. The 2025 Amended Facility consists of three tranches including a first tranche of $50.0 million replacing the debt outstanding as per the loan amendment date, an additional term loan tranche of $100.0 million, which can be drawn at our option, subject to the BLA approval of AMT-130 prior to June 2027, provided that confirmatory trials to the extent and in the manner required to support full approval (if applicable) remain ongoing or are being planned, and a third tranche of up to $25.0 million, subject to Hercules' approval. All tranches have a floating interest rate of the greater of 9.45% and the prime rate plus 2.45%, reflecting a floating rate of 9.45% as of December 31, 2025. The tranches mature on October 1, 2030. In the year ended December 31, 2025, we recognized a gain of $4.9 million in connection with the modification of the 2025 Amended Facility in accordance with IFRS 9, *Financial Instruments*.

Results of operations

The following table presents a comparison of the results of operations for the twelve months ended December 31, 2025, and 2024:

	Years ended December 31,		
	2025	2024	2025 vs 2024
	$ in thousands		
Total revenues	**16,098**	**27,119**	**(11,021)**
Cost of revenues	(1,686)	(18,327)	16,641
Gross profit	**14,412**	**8,792**	**5,620**
Operating expenses:			
Research and development expenses	(136,076)	(135,180)	(896)
Selling, general and administrative expenses	(64,470)	(50,553)	(13,917)
Total operating expenses	**(200,546)**	**(185,733)**	**(14,813)**
Other income	14,410	11,143	3,267
Other expense	(7,454)	(4,201)	(3,253)
Loss from operations	**(179,178)**	**(169,999)**	**(9,179)**
Finance income	61,226	21,415	39,811
Finance expense	(35,993)	(72,650)	36,657
Finance income / (expense), net	**25,233**	**(51,235)**	**76,468**
Loss before income tax expense	**(153,945)**	**(221,234)**	**67,289**
Income tax expense	(5,650)	(2,687)	(2,963)
Net loss	**(159,595)**	**(223,921)**	**64,326**

Revenues and cost of revenues

Our revenues and associated costs for the years ended December 31, 2025 and 2024 were as follows:

	Years ended December 31,		
	2025	2024	2025 vs 2024
	$ in thousands		
License revenues	15,934	10,133	5,801
Collaboration revenues	164	10,872	(10,708)
Contract manufacturing revenues	—	6,114	(6,114)
Total revenues	**16,098**	**27,119**	**(11,021)**
Cost of license revenues	(1,686)	(1,267)	(419)
Cost of contract manufacturing revenues	—	(17,060)	17,060
Total cost	**(1,686)**	**(18,327)**	**16,641**

Revenues

CSL Behring

We sold the exclusive global rights to HEMGENIX to CSL Behring in 2021 (the "License Sale"). We recognize license revenue in relation to the License Sale when it becomes probable that regulatory and sales milestone events will be achieved as well as when royalties on sales of HEMGENIX have been earned. We recognized $15.9 million and $10.1 million of license revenue related to royalty payments for the years ended December 31, 2025 and 2024.

In 2008, we entered into a license agreement with St. Jude Children's Research Hospital ("St. Jude"), which we amended in 2012. Under this license agreement, St. Jude has granted us an exclusive license that requires us to make royalty payments related to sales of HEMGENIX. The majority of the U.S. patent rights will expire in 2028 and the European patent rights (along with one U.S. patent) expired in 2025.

With respect to our collaboration with CSL Behring, we have agreed with St. Jude on an apportionment of certain amounts we receive from CSL Behring as sublicensing revenue that is equivalent to a low-single digit percentage of such amounts. The agreement will remain in effect until no further payment is due relating to any licensed product under this agreement or either we or St. Jude exercise our rights to terminate it. St. Jude may terminate the agreement in specified circumstances relating to our insolvency. We may terminate the agreement for convenience at any time subject to a specified notice period.

We treat license payments to St. Jude as contract fulfillment costs associated with the license revenue we receive from CSL Behring and recognize these as costs of license revenues. We incurred $1.7 million and $1.3 million of such cost in the years ended December 31, 2025 and 2024, respectively.

We recognize collaboration revenues associated with services we provide to CSL Behring. Collaboration revenue is recognized when performance obligations are satisfied. We recognized $0.2 million and $10.9 million of collaboration revenue for the years ended December 31, 2025 and 2024, respectively. The $10.7 million decrease in collaboration revenue in 2025 compared to 2024 was primarily related to a reduction in services requested by CSL Behring following the July 2024 closing (the "Closing") of the sale of our commercial manufacturing activities located in Lexington, MA to Genezen (the "Lexington Transaction").

We provided contract manufacturing services to CSL Behring between April 2022 and July 2024. We transferred these activities to Genezen as part of the Lexington Transaction. Following the Closing of the Lexington Transaction, title to HEMGENIX supply directly passes from the contract manufacturer, Genezen, to CSL Behring. We do not control HEMGENIX before it is transferred to CSL Behring. We arrange for HEMGENIX to be provided by Genezen to CSL Behring. We determined that we are an agent in the sale of HEMGENIX to CSL Behring.

As a result of our being an agent, we recognize corresponding costs related to the purchase of HEMGENIX from Genezen net of income from the sales of HEMGENIX to CSL Behring and income related to the release of the onerous contract provision associated with expected net losses in Other expense within the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss. We recognized contract manufacturing revenues related to contract manufacturing HEMGENIX for CSL Behring when earned upon sales of HEMGENIX to CSL Behring. We recognized nil and $6.1 million contract manufacturing revenues in the years ended December 31, 2025 and 2024, respectively.

We incurred nil cost of contract manufacturing revenues related to the manufacture of HEMGENIX in the year ended December 31, 2025 and $17.1 million of cost of contract manufacturing revenues, in the year ended December 31, 2024. Costs of contract manufacturing revenues decreased in the year ended December 31, 2025 compared to the year ended December 31, 2024 by $17.1 million as a result of the Lexington Transaction.

Research and development expenses

We expense research and development ("R&D") expenses as incurred. R&D expenses include costs which relate to our primary activities of biopharmaceutical research and development. Our R&D expenses generally consist of costs incurred for the development of our target candidates, which include:

- employee-related expenses, including salaries, benefits, travel and share-based compensation expense;
- costs incurred for laboratory research, preclinical and nonclinical studies, clinical trials, statistical analysis and report writing, and regulatory compliance costs incurred with clinical research organizations and other third-party vendors;
- costs incurred to conduct consistency and comparability studies;
- costs incurred for the development and improvement of our manufacturing processes and methods;
- costs associated with research activities for enabling technology platforms;
- costs associated with the rendering of collaboration services;
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance, and other supplies; and,
- changes in the fair value of liabilities recorded in relation to the uniQure France Acquisition.

Our R&D expenses may vary substantially from period to period based on the timing of our research and development activities, including regulatory submissions, and enrollment of patients in clinical trials. The successful development of our product candidates is highly uncertain. Estimating the nature, timing, or cost of the development of any of our product candidates involves considerable judgement due to numerous risks and uncertainties associated with developing gene therapies, including the uncertainty of:

- the scope, rate of progress and expense of our research and development activities;
- clinical trial protocols, speed of enrollment and resulting data;
- the effectiveness and safety of our product candidates; and,
- the timing of regulatory approvals.

A change in the outcome of any of these variables with respect to our product candidates that we may develop could mean a significant change in the expenses and timing associated with the development of such product candidate

Research and development expenses for the year ended December 31, 2025 were $136.1 million, compared to $135.2 million for the year ended December 31, 2024. Other research and development expenses, which are separately classified in the table below, are not allocated to projects as they are deployed across multiple projects under development.

	Year ended December 31,		
	2025	2024	2025 vs 2024
	$ in thousands		
Huntington's disease (AMT-130)	42,462	17,989	24,473
Temporal lobe epilepsy (AMT-260)	8,251	12,053	(3,802)
Amyotrophic lateral sclerosis (AMT-162)	6,827	7,306	(479)
Fabry disease (AMT-191)	6,336	4,814	1,522
Programs in preclinical development and platform related expenses	3,611	3,927	(316)
Total direct research and development expenses	**67,487**	**46,089**	**21,398**
Employee and contractor-related expenses	35,268	49,818	(14,550)
Facility expenses	13,047	19,176	(6,129)
Share-based compensation expense	6,608	3,154	3,454
Fair value changes related to contingent consideration	6,247	(1,817)	8,064
Disposables	2,585	5,084	(2,499)
Severance costs	—	3,967	(3,967)
Other expenses	4,834	9,709	(4,875)
Total other research and development expenses	**68,589**	**89,091**	**(20,502)**
Total research and development expenses	**136,076**	**135,180**	**896**

Direct research and development expenses

Huntington's disease (AMT-130)

In the years ended December 31, 2025 and 2024, we incurred $42.5 million and $18.0 million external cost for the development of AMT-130, respectively. The increase of $24.5 million in external cost in 2025 compared to 2024 includes $19.4 million of costs incurred in relation to the preparation of the BLA submission for AMT-130 in the U.S., including manufacturing process validation-related costs as well as expenses to support the process performance qualification campaign and other activities. In addition, in 2025 we incurred costs in relation to treating all six patients in the fourth cohort.

Temporal lobe epilepsy (AMT-260)

In the years ended December 31, 2025 and 2024 we incurred $8.3 million and $12.1 million of external costs, respectively, for the development of AMT-260. In the year ended December 31, 2025 we incurred costs of $7.5 million related to our Phase I/IIa clinical trial which enrolled the first patient in November 2024 as well as $0.7 million related to CMC development (2024: $7.4 million and $4.3 million, respectively).

Amyotrophic Lateral Sclerosis caused by mutations in SOD1 (AMT-162)

In the years ended 31 December 2025 and 2024, the Company incurred external costs of $6.8 million and $7.3 million, respectively, for the development of AMT-162. Of the costs incurred in 2025, $6.0 million related to the Phase I/II clinical trial, which commenced with the first patient enrolled in October 2024, and $0.3 million related to CMC development (2024: $5.2 million and $1.9 million, respectively).

Fabry disease (AMT-191)

In the years ended December 31, 2025 and 2024, we incurred $6.3 million and $4.8 million external cost for the development of AMT-191. $6.0 million of cost incurred in 2025 related to our Phase I/II clinical trial with the first patient dosed in August 2024, as well as $0.7 million related to CMC development (2024: $4.6 million and $0.2 million, respectively).

Preclinical programs & platform development

In the years ended December 31, 2025 and 2024, we incurred $3.6 million and $3.9 million of expenses, respectively, related to our preclinical activities associated with product candidates for various other research programs and technology innovation projects.

Other research & development expenses

- We incurred $35.3 million in employee and contractor expenses in the year ended December 31, 2025 compared to $49.8 million in 2024. Our cost decreased in 2025 by $14.6 million compared to 2024 primarily related to the Lexington Transaction as well as the August 2024 reorganization;
- We incurred $13.0 million in operating expenses and depreciation expenses related to our rented facilities in Amsterdam and Lexington, Massachusetts in the year ended December 31, 2025, compared to $19.2 million in 2024. The decrease of $6.1 million in 2025 compared to 2024 is primarily related to the Lexington Transaction;
- We incurred $6.6 million in share-based compensation expenses in the year ended December 31, 2025 compared to $3.2 million in 2024. The increase in 2025 of $3.5 million, compared to 2024, is primarily a result of an increase in fair value of long-term incentive awards granted during the year. This increase was partially offset by the impact of the Lexington Transaction and the August 2024 reorganization;
- We incurred a $6.2 million loss in the year ended December 31, 2025 related to an increase in the fair value of contingent consideration associated with the uniQure France Acquisition, compared to a gain of $1.8 million in 2024. The increase in fair value of the contingent consideration in 2025 was primarily due to changes in the discount rate used in the valuation of the liability;
- We incurred $2.6 million in disposables costs in the year ended December 31, 2025 compared to $5.1 million in 2024. The decrease of $2.5 million in 2025 primarily related to the Lexington Transaction;
- We incurred nil severance costs in the year ended December 31, 2025. We incurred $4.0 million of severance costs in the year ended December 31, 2024 related to the Lexington Transaction and the August 2024 reorganization; and,
- We incurred $4.8 million in other expenses in the year ended December 31, 2025 compared to $9.7 million in 2024. The $4.9 million decrease in other expenses in 2025, compared to 2024, is primarily due to a decrease in information technology expenses and consultant-related expenses.

Selling, general and administrative expenses

Our selling, general and administrative expenses consist principally of employee, office, consulting, legal and other professional and administrative expenses. We incurred expenses associated with operating as a public company, including expenses for personnel, legal, accounting and audit fees, board of directors' costs, directors' and officers' liability insurance premiums, Nasdaq listing fees, expenses related to investor relations and fees related to business development and maintaining our patent and license portfolio. As from 2025, we also incurred material expenses associated with activities to prepare for a potential launch of AMT-130 in the U.S., and present expenses related to medical affairs, new product planning and patient advocacy within selling, general and administrative expenses.

Selling, general and administrative expenses for the year ended December 31, 2025 were $64.5 million, compared to $50.6 million for the year ended December 31, 2024.

	Year ended December 31,		
	2025	2024	2025 vs 2024
	$ in thousands		
Employee and contractor-related expenses	24,941	21,343	3,598
Professional fees	16,638	7,261	9,377
Share-based compensation expense	9,432	10,069	(637)
Intellectual property fees	1,944	1,744	200
Depreciation and facility costs	1,592	1,868	(276)
Information technology costs	1,129	1,101	28
Severance costs	—	1,223	(1,223)
Other expenses	8,794	5,944	2,850
Total selling, general and administrative expenses	**64,470**	**50,553**	**13,917**

- We incurred $24.9 million in employee and contractor-related expenses in the year ended December 31, 2025 compared to $21.3 million in 2024. The $3.6 million increase in 2025, compared to 2024, was primarily due to higher personnel-related expenses incurred in preparation for a potential commercial launch of AMT-130 in the U.S;
- We incurred $16.6 million in professional fees in the year ended December 31, 2025 compared to $7.3 million in 2024. We regularly incur accounting, audit and legal fees associated with operating as a public company. The $9.4 million increase to professional fees in 2025 included $6.5 million of expenses incurred in relation to the preparation of a potential commercial launch of AMT-130 in the U.S;
- We incurred $9.4 million of share-based compensation expenses in the year ended December 31, 2025 compared to $10.1 million in 2024. The decrease in 2025 of $0.6 million, compared to 2024, primarily related to the Lexington Transaction and the August 2024 reorganization, partially offset by an increase in fair value of long-term incentive awards granted during the year;
- We incurred $1.9 million in intellectual property fees including registration and professional fees in the year ended December 31, 2025 compared to $1.7 million in 2024;
- We incurred nil severance costs in the year ended December 31, 2025. We incurred $1.2 million of severance costs in the year ended December 31, 2024 related to the Lexington Transaction and the August 2024 reorganization; and,
- We incurred $8.8 million in other expenses in the year ended December 31, 2025 compared to $5.9 million in 2024. The $2.9 million increase in other operating expenses in 2025, compared to 2024, was primarily due to an increase in non-income tax expenses recorded in 2025, partially offset by a decrease in other expenses relating to the Lexington Transaction in 2024.

Other income / Other expense

In the year ended December 31, 2025, we recognized $6.0 million in income related to a one-time sale of critical reagents to Genezen, compared to nil in the same period of 2024.

In the year ended December 31, 2025, we recognized $5.7 million in income related to payments received from European authorities to subsidize our research and development efforts in the Netherlands and France compared to $5.6 million in 2024.

In the year ended December 31, 2024, we recognized a $4.4 million net gain related to the Lexington Transaction, with no such amounts applicable in 2025.

Other income for the years ended December 31, 2025 and 2024 includes income from the subleasing of a portion of our Amsterdam facility and Lexington research and development facility. We present expenses related to such income as other expense.

We recognized $5.7 million and $2.8 million in net other expenses during the year ended December 31, 2025 and 2024, related to the purchase of HEMGENIX from Genezen, net of income from the sales of HEMGENIX to CSL Behring, amortization of the intangible asset for our favorable supply terms under the Genezen CSA in connection with the Lexington Transaction, and credits from the release of the onerous contract provision related to the unavoidable excess cost of meeting our obligations under the Genezen CSA over the economic benefits that we expect to receive from our customer contract with CSL Behring.

Finance income / (expense), net

Our finance income / (expense), net, for the years ended December 31, 2025, and 2024 was as follows:

	Years ended December 31,		
	2025	2024	2025 vs 2024
		$ in thousands	
Finance income:			
Foreign exchange gains, net	25,235	—	25,235
Interest income on cash and cash equivalents and investment securities	18,658	21,008	(2,350)
Fair value gain on financial liability at fair value through profit or loss ("FVTPL")	10,905	—	10,905
Gain on modification of Loan Facilities (see Note 8, "*Borrowings*")	4,896	—	4,896
Fair value gains and other adjustments on financial assets at FVTPL	1,532	407	1,125
Total finance income:	**61,226**	**21,415**	**39,811**
Finance expense:			
Interest expenses on royalty financing agreement	(25,040)	(44,186)	19,146
Interest expenses on Hercules Loan Facilities	(7,433)	(15,185)	7,752
Interest expense on leases	(1,965)	(3,142)	1,177
Interest expense on unwinding of discount on onerous contract provision	(588)	(377)	(211)
Interest expense - other	(967)	—	(967)
Foreign exchange losses, net	—	(9,760)	9,760
Total finance expense:	**(35,993)**	**(72,650)**	**36,657**
Finance income (expense), net	**25,233**	**(51,235)**	**76,468**

We hold monetary items and enter into transactions in foreign currencies, predominantly in euros and U.S. dollars. We recognize foreign exchange results related to changes in these foreign currencies. In 2025, we recognized a net foreign currency gain of $25.2 million related to our borrowings from Hercules, the May 2023 royalty financing agreement entered into with HemB SPV L.P. (the “Royalty Financing Agreement”) and our cash and cash equivalents and investment securities as well as loans between entities within the uniQure group, compared to a net loss of $9.8 million in 2024.

We recognize interest income associated with our cash, cash equivalents, and investment securities. We recognized interest income of $18.7 million in 2025 and $21.0 million in 2024. Our interest income decreased by $2.4 million in 2025 compared to 2024, primarily driven by lower average market interest rates on our cash and cash equivalent balances.

As part of the September 2025 follow-on public offering, we issued pre-funded warrants. We recognized $10.9 million and nil fair value gain in connection with the liability related to pre-funded warrants in the years ended December 31, 2025 and 2024, respectively.

In connection with the 2025 Amended Loan Facility, we recognized a $4.9 million gain on modification of the Hercules Loan Facilities in accordance with IFRS 9, *Financial Instruments*, compared to nil in 2024.

We recognize a fair value gain of $1.5 million on financial assets related to our series C preferred stock and convertible promissory note in 2025, compared to $0.4 million in 2024.

We recognized non-cash interest expense related to the Royalty Financing Agreement of $25.0 million in 2025 and $44.2 million in 2024. The $19.1 million decrease of non-cash interest expense in 2025 compared to 2024 was primarily driven by updates to estimated future cash flows, including a shift in the expected timing of those cash flows to future periods.

We recognized $7.4 million interest expense in 2025 and $15.2 million in 2024 related to the Hercules Loan Facilities. Interest expense decreased by $7.8 million in 2025 compared to 2024 primarily due to the $50.0 million repayment of Hercules debt in July 2024 and a decrease in market interest rates.

We recognized $2.0 million interest expense in 2025 and $3.1 million interest expense in 2024 related to the lease liabilities. Interest expense decreased by $1.2 million in 2025 compared to 2024 as a result of the derecognition of the lease liabilities related to the lease derecognized as part of the Lexington Transaction.

Income tax expense

We recognized total income tax expense of $5.7 million in 2025, which comprised of $1.6 million of deferred tax expense and $4.0 million of current tax expense. In 2024, we recognized total income tax expense of $2.7 million, which comprised of $2.5 million of deferred tax expense and $0.2 million of current tax expense. The $3.0 million increase in 2025 compared to 2024 is a result of recognizing $3.9 million of current income tax expense related to our agreement with the Dutch tax authorities in 2025, to treat the $375.0 million upfront payment received in 2023 under the Royalty Financing Agreement as taxable income in 2023. This increase in current income tax was offset by a $0.9 million reduction of deferred tax expense. Such deferred tax expenses primarily result from the consumption of net operating tax losses in the US.

Cash Flow and Cash Position

As of December 31, 2025, we had cash and cash equivalents, restricted cash and investment securities of $624.1 million. We believe our cash and cash equivalents, restricted cash, and investment securities will be sufficient to fund our projected operating expenses into the second half of 2029. Such operating expenses include expenses to fund the ongoing clinical trials of AMT-130, AMT-191 and AMT-260, as well as the potential investment of a material portion of the proceeds from our September 2025 public follow-on offering primarily into advancing certain of our clinical candidates into late stage development. The amount and timing of our actual expenditures may vary significantly depending on when we commence, as well as the design of, any Phase III trials for AMT-130, AMT-191, and AMT-260 that we may conduct. We have based our estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect, and as such, we may require additional funding.

If the BLA for AMT-130 is approved prior to June 15, 2027, provided that confirmatory trials to the extent and in the manner required to support full approval (if applicable) remain ongoing or are being planned, we could draw down up to $100.0 million under our senior secured term loan facility with Hercules. Additional funding could also include a combination of public equity offerings, collaborations, strategic alliances, licensing arrangements or marketing and distribution arrangements, which may not be possible. If adequate funds are not available to us on acceptable terms when we need them, we may be unable to pursue further development of our clinical product candidates.

The table below summarizes our consolidated cash flow data for the years ended December 31:

	Years ended December 31,		
	2025	2024	2025 vs 2024
		$ in thousands	
Cash and cash equivalents at the beginning of the period	158,930	241,360	(82,430)
Net cash used in operating activities	(173,967)	(179,366)	5,399
Net cash (used in) / generated from investing activities	(323,119)	156,149	(479,268)
Net cash generated from / (used in) financing activities	412,843	(54,293)	467,136
Foreign exchange impact	5,553	(4,920)	10,473
Cash and cash equivalents at the end of the period	**80,240**	**158,930**	**(78,690)**

We have previously incurred losses and cumulative negative cash flows from operations since our business was founded by our predecessor entity AMT Holding N.V. in 1998, with the exception of generating income in 2021 after receiving the upfront payment upon closing of the CSL Behring Agreement. We continued to incur losses in the current period. We recorded a net loss of $159.6 million for the year ended December 31, 2025, and net loss of $223.9 million in 2024. As of December 31, 2025, we had an accumulated deficit of $1,279.5 million.

Net Cash used in operating activities

Net cash used in operating activities was $174.0 million for the year ended December 31, 2025, and consisted of a net loss of $159.6 million adjusted for non-cash items, including depreciation, amortization and impairment expense of $17.5 million, amortization of the discount on investment securities of $10.5 million, share-based compensation expense of $16.0 million, $37.0 million of interest expense, a change in deferred taxes of $1.6 million, $6.2 million change in the fair value of contingent consideration and unrealized foreign exchange gains of $22.6 million. Net cash used in operating activities also included unfavorable changes in operating assets and liabilities of $7.9 million. There was a net increase in accounts receivable, prepaid expenses, and other current assets of $8.6 million. There was a net increase in accounts payable, accrued expenses, other liabilities, and operating leases of $0.7 million, primarily related to a decrease of $2.7 million in accounts payable and an increase of $3.4 million related to various accruals. Net cash used in operating activities also includes interest paid of $26.7 million, interest received of $5.5 million and income taxes paid of $7.5 million.

Net cash used in operating activities was $179.4 million for the year ended December 31, 2024, and consisted of a net loss of $223.9 million adjusted for non-cash items, including depreciation, amortization and impairment expense of $16.2 million, amortization of the discount on investment securities of $10.9 million, share-based compensation expense of $14.2 million, $61.2 million of interest expense, a change in deferred taxes of $2.7 million, $1.8 million change in the fair value of contingent consideration and unrealized foreign exchange losses of $14.6 million. Net cash used in operating activities also included unfavorable changes in operating assets and liabilities of $23.2 million. There was a net increase in accounts receivable, prepaid expenses, and other current assets of $2.2 million. There was a decrease in inventory balances of $2.4 million. There was a net decrease in accounts payable, accrued expenses, other liabilities, and operating leases of $3.8 million, primarily related to an increase of $1.5 million in accounts payable and a decrease of $5.3 million related to various accruals. Net cash used in operating activities also includes a payment for a contingent consideration milestone of $19.6 million, interest paid of $23.7 million and interest received of $10.6 million.

Net cash (used in) / generated from investing activities

In 2025, we used $323.1 million in our investing activities, compared to $156.1 million generated from investing activities in 2024.

	Years ended December 31,		
	2025	2024	2025 vs 2024
		$ in thousands	
Proceeds from maturity of investment securities	337,195	534,498	(197,303)
Investment in investment securities	(658,375)	(359,841)	(298,534)
Divestment of commercial manufacturing facility	—	(8,321)	8,321
Capital expenditure	(439)	(3,368)	2,929
Purchase of intangible assets	(1,500)	—	(1,500)
Contingent consideration milestone payment	—	(8,559)	8,559
Receipt of bank deposit	—	1,740	(1,740)
Net cash (used in) / generated from investing activities	**(323,119)**	**156,149**	**(479,268)**

In December 2024, following the dosing of the first patient in Phase I/IIa clinical trial for AMT-260, we made a payment of EUR 30.0 million ($31.5 million) to the former shareholders of uniQure France SAS based on contractually defined milestones. EUR 26.8 million ($28.2 million) of this payment related to contingent consideration of which EUR 8.2 million ($8.6 million) was presented within cash generated from investing activities and EUR 18.7 million ($19.6 million) was presented within net cash used in operating activities. We had no such payments in 2025.

Net cash generated from / (used in) financing activities

In 2025, we generated $412.8 million from our financing activities, compared to $54.3 million used in financing activities in 2024.

	Years ended December 31,		
	2025	2024	2025 vs 2024
		$ in thousands	
Proceeds from follow-on public offerings of ordinary shares, net of issuance costs	380,737	—	380,737
Proceeds from issuance of pre-funded warrants, net of issuance costs	23,499	—	23,499
Proceeds from issuance of ordinary shares related to employee stock option and purchase plans	11,162	2,123	9,039
Repayment of long-term debt	—	(53,050)	53,050
Payments for principal portion of lease liability	(2,555)	(3,366)	811
Net cash generated from / (used in) financing activities	**412,843**	**(54,293)**	**467,136**

Shareholders' Equity

Shareholders' equity at December 31, 2025, amounted to $244.3 million compared to $2.5 million at December 31, 2024; a total of 62.3 million ordinary shares were issued and outstanding as of December 31, 2025.

We had a net loss of $159.6 million in 2025 and $223.9 million in 2024. As of December 31, 2025, we had an accumulated deficit of $1,279.5 million.

Outlook 2026

Huntington's disease program (AMT-130)

In January 2026, we met with the FDA at a Type A meeting to discuss AMT-130. In March 2026, following receipt of the final meeting minutes from the Type A meeting, we announced that the FDA stated that it cannot agree that data from the Phase I/II studies, compared to an external control, are sufficient to provide the primary evidence of effectiveness required to support a marketing application for AMT-130. The FDA strongly recommended we conduct a prospective, randomized, double-blind, sham surgery-controlled study.

We intend to continue engaging with the FDA regarding Phase III development considerations and plan to request a Type B meeting in the second quarter of 2026 to further discuss potential study design approaches. In addition, we plan to continue progressing discussions with other regulatory agencies regarding AMT-130, including in the European Union and United Kingdom. We also intend to update our Phase I/II statistical analysis plan for AMT-130 to incorporate a 4-year analysis that we expect to conduct in the third quarter of 2026.

Continued clinical progress in pipeline programs

AMT-260 for the treatment of refractory mesial temporal lobe epilepsy (MTLE) – In May 2025, we presented initial safety and exploratory efficacy data from the first treated patient in our Phase I/IIa clinical trial of AMT-260 for the treatment of MTLE in the U.S. In 2025, we completed enrollment in the first dose cohort of six patients in the Phase I/IIa study. We also initiated enrollment of a second cohort in 2025, which is expected to include an additional six patients.

AMT-191 for the treatment of Fabry disease – In February 2026, we presented updated safety and exploratory efficacy data from three dose cohorts from the Phase I/II study of AMT-191 in Fabry disease. Per protocol, additional dosing in the mid- and high-dose cohorts has been paused pending further evaluation of asymptomatic Grade 3 liver enzyme elevations reported in two patients from the mid-dose cohort, which were confirmed as dose-limiting toxicities. We expect to complete enrollment of the Phase I/II study in 2026.

AMT-162 for the treatment of SOD1 amyotrophic lateral sclerosis (ALS) – Following review of the preliminary efficacy and safety data generated from EPISOD1, we have decided to discontinue development of AMT-162. We will continue to collect safety data from the five patients dosed in EPISOD1, consistent with applicable safety and regulatory requirements.

3 Risk management

Risk appetite

We are developing gene therapy product candidates for the treatment of rare and other devastating diseases. We operate in a dynamic and rapidly changing industry that involves numerous risks and uncertainties. Our Board, in its advisory capacity, and management regularly review our strategic plan, which includes, among other things, the various business, clinical, developmental, financial, and other market risks confronting, and opportunities available to, us at any given time. Specifically, pursuant to the Company's Corporate Governance Guidelines and Rules for the Board, the Board is charged with assessing major risks we face and reviewing options to mitigate such risks. The Board performs this oversight role by using several different levels of review. In connection with its reviews of our operations and corporate functions, the Board addresses the primary risks associated with those operations and corporate functions. In addition, the Board reviews the risks associated with our business strategies periodically throughout the year as part of its consideration of undertaking any such business strategies.

In consideration of our business and industry, below is a summary of principal risks associated with the Company. If any of the risks discussed below actually occur, our business, financial condition, operating results, or cash flows could be materially adversely affected. In addition, the development and commercialization of gene therapies is a multi-year process involving many risks that can result in delays or even termination of product development efforts. Advancing our product candidates through development efforts and, if approved, through commercialization requires considerable financial resources. We expect that our existing, cash equivalents and investments will be sufficient to fund our operating expenses and capital requirements into the second half of 2029. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. The additional funding we expect to require depends on when we commence as well as the design of any Phase III trials for AMT-130, AMT-191, and AMT-260. As a development-stage biotechnology company, acceptance of these industry risks forms part of our strategy.

We strive to mitigate these business and industry risks by ensuring that we strictly comply with the rules and regulations governing our highly regulated industry. We have established and maintain adequate internal controls over financial reporting to provide reasonable assurance regarding the reliability of our financial reporting as well as processes to ensure that we comply with the rules and regulations defined by the SEC as well as Nasdaq listing standards. Compliance with these rules and regulations is necessary for us to access the capital markets to fund our business, which may be critical for us.

a) Summary Risk Factors

Below is a summary of the principal risks associated with the Company.

- We are dependent on the success of our lead product candidate, AMT-130, for the treatment of Huntington's disease. A failure of AMT-130 in clinical development, including inability to demonstrate sufficient safety or efficacy, or challenges associated with its regulatory approval, manufacturing or commercialization could adversely affect our business.

- We have encountered and may encounter future delays in and impediments to the progress of our clinical trials or fail to demonstrate the safety and efficacy of our product candidates.

- Our progress in early-stage clinical trials may not be predictive of long-term efficacy in late-stage clinical trials, and our progress in trials for one product candidate may not be predictive of progress in trials for other product candidates.

- If we are unable to successfully commercialize our product candidates or experience significant delays in doing so, including as result of a BLA or other key regulatory filings being delayed or rejected, our business could be materially harmed.

- Any approved gene therapy we seek to commercialize may fail to achieve the degree of market acceptance by physicians, patients, third party payers and others in the medical community necessary for commercial success.

- Interim, topline or preliminary results from our clinical trials may change as more data become available, as such data are subject to regulatory audit and verification procedures, and/or regulatory review, which could result in material changes in the final results and conclusions.

- We cannot predict when or if we will obtain marketing approval to commercialize our product candidates.

- Disruptions or changes at the FDA or other government agencies resulting from funding cuts, personnel losses, leadership changes, regulatory changes or reform, government shutdowns, or other developments could hinder the timing of, or our ability to obtain, further guidance from the FDA regarding our clinical development programs and to secure approval of our product candidates in a timely manner, which could adversely affect our business. The risks associated with the marketing approval process are heightened by the status of our products as gene therapies.

- We may leverage certain specialized regulatory pathways and designations, such as the FDA's accelerated approval pathway, RMAT designation and Breakthrough Therapy designation, to develop our product candidates or to seek licensure. Even if one or more of our product candidates receives such a designation or is permitted to pursue such a pathway, we may be unable to obtain and maintain the benefits associated with such designations and pathways.

- Negative public opinion and increased regulatory scrutiny of gene therapy and genetic research may damage the public perception of our product candidates or adversely affect our ability to conduct our business or obtain marketing approvals for our product candidates.

- Our business development and strategic initiatives, acquisitions, partnerships, collaborations or other transactions, may not achieve their intended benefits or goals and may result in additional risks to our business.

- We may not be successful in obtaining rights from external parties to new product candidates and key technologies, or in securing partnerships to support the development or commercialization of our product candidates.

- Gene therapies are complex, expensive and difficult to manufacture. Genezen Holdings Inc. and its subsidiary Genezen MA, Inc. (together "Genezen") or any third-party manufacturer that we engage could experience capacity, production or technology transfer challenges that could result in delays in our development or commercialization schedules or otherwise adversely affect our business.

- The manufacturing of our products and product candidates is subject to significant government regulations and approvals. We currently rely and expect to continue to rely on third parties to manufacture our product candidates, and these third parties may not perform satisfactorily or may fail to comply with these regulations or maintain these approvals.

- We had net losses in the years ended December 31, 2025 and 2024, have incurred significant losses in previous years and expect to incur losses in the future, and may never achieve or maintain profitability.

- There may be future changes in legal and regulatory requirements or standards that may materially impact our results of operations.

- The market price of our ordinary shares has been and may in the future be volatile and fluctuate substantially.

- We may need to raise additional funding in order to advance the development of our product candidates (including AMT-130) and support the potential commercialization of AMT-130, which may not be available on acceptable terms, or at all. Failure to obtain capital, if and when needed, may force us to delay, limit or terminate our product development efforts or other operations, which could have a material adverse effect on our business, financial condition, results of operations and cash flows.

- Our future success depends on our ability to retain key executives, technical staff, and other employees and to attract, retain and motivate qualified personnel.

- We face substantial competition, and others may discover, develop, or commercialize competing products before or more successfully than we do.

b) Internal risk management and control system

We have developed an internal risk management and control system that is tailored to the risk factors that are relevant to us. Our controls frequently entail involvement of the Board and Senior Management. Our internal risk management and control systems are regularly discussed with the Board.

The Board is responsible for designing, implementing and operating our internal risk management and control systems. The objective of these systems is to manage in an effective and efficient manner the significant risks to which we are exposed. Our internal risk management and control systems are designed to provide reasonable assurance that these objectives are met. Such systems can never provide absolute assurance regarding achievement of the our objectives, nor can they provide absolute assurance that material errors, losses, fraud, and the violation of laws or regulations will not occur. A summary of the risks that could have prevented us from realizing our objectives is included in the section 'Risk Factors' of this Annual Report.

Our internal risk management and control systems make use of various measures including:

- Annual strategic evaluations of our business;
- Periodic operational review meetings of our Leadership Team comprising our executive director of the Board and Senior Management;
- Quarterly review of the financial position and prospects as part of our Board meetings;
- A planning and control cycle consisting of annual, quarterly and monthly procedures, including subsequent follow-up on achievements of targets set;
- A system of internal controls and procedures; and
- The delegation of certain risk oversight responsibilities to committees of the Board, with such committees regularly meeting with executives and senior management, and with respect to the Audit Committee, the independent auditors. For example:
    - The Audit Committee is delegated the responsibility to regularly review and discuss with management the Company's major financial risk exposures and the steps management has taken to monitor and control for such exposure;
    - The Nominating and Corporate Governance Committee regularly reviews our corporate governance principles and recommends to the Board any proposed changes it may deem appropriate;
    - The Compensation Committee considers risks related to the attraction and retention of professional talent and the implementation and administration of compensation and benefit plans affecting our employees;
    - The Research & Development Committee is charged with reviewing our research and development strategy as well as our technology and patent strategies and related risks; and
    - The Commercial Committee is charged with reviewing the Company's commercial strategy and related operations.

We maintain controls and procedures designed to:

- Ensure that records are maintained, in reasonable detail, accurately and fairly reflect the transactions and disposition of the assets of the Company;
- Provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with International Financial Reporting Standards ("IFRS"), and that receipts and expenditures of the Company are being made only by authorized employees in accordance with documented authorizations; and

- Provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of the Company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness for future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with policies or procedures may deteriorate.

Based on the evaluation of our disclosure controls and procedures as of December 31, 2025, our Chief Executive Officer ("CEO") and Chief Financial Officer ("CFO") concluded that our disclosure controls and procedures were effective.

Director's Annual Report on Internal Control Over Financial Reporting

The Board is responsible for establishing and maintaining adequate internal control over financial reporting, as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934, as amended ("Exchange Act"), and in accordance with the Dutch Corporate Governance Code 2025 ("DCGC"). This rule defines internal control over financial reporting as a process designed by, or under the supervision of, a company's chief executive officer and chief financial officer and effected by its board of directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles and includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of our assets that could have a material effect on the financial statements.

We assessed the effectiveness of our internal control over financial reporting as of December 31, 2025. This assessment was performed under the direction and supervision of our CEO and CFO and based on criteria established in *Internal Control - Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission ("COSO"). Our management's assessment of the effectiveness of our internal control over financial reporting included testing and evaluating the design and operating effectiveness of our internal controls. In our management's opinion, we have maintained effective internal control over financial reporting as of December 31, 2025, based on criteria established in the COSO 2013 framework.

The DCGC also requires the Board to confirm, and the Board hereby confirms, that:

i. The Report of the Board provided sufficient insights into any failings in the effectiveness of the internal risk management and control systems;

ii. The aforementioned systems provided reasonable assurance that the financial reporting does not contain any material inaccuracies;

iii. Based on the current state of affairs, it is justified that the financial reporting is prepared on a going concern basis; and

iv. The Report states those material risks and uncertainties that are relevant to the expectations of the Company's continuity for the period of twelve months after the preparation of the Report.

Additional information

In addition to the information contained in this Annual Report, we also filed Consolidated Financial Statements for 2025 of uniQure N.V. prepared in accordance with generally accepted accounting principles in the United States ("U.S. GAAP") with the SEC on March 2, 2026, as part of our Annual Report on Form 10-K for the year ended December 31, 2025, which is available on our website.

4 Governance and compliance

uniQure N.V. recognizes the importance of complying with both Dutch and U.S. corporate governance regulations, including the DCGC, as well as U.S. federal and state securities laws. For the fiscal year covered by this report, the Company applied the provisions of the DCGC. Additionally, as a company listed solely on Nasdaq, the Company is required to adhere to all applicable U.S. securities laws and regulations, including those promulgated by the SEC under the Exchange Act and the Sarbanes-Oxley Act of 2002, along with the applicable Nasdaq listing rules.

The Company's Board of Directors ("Board") believes that certain deviations from the provisions of the DCGC are justified due to the Company's international business model, organizational size, and specific operational circumstances. These deviations are in line with the "comply or explain" principle, which is detailed in this report. Deviations from specific aspects of the DCGC, when deemed necessary, are disclosed in our Annual Report. These deviations largely stem from the Company's international focus, particularly as a Nasdaq-listed company with a significant portion of its investors outside of the Netherlands. As such, the Company primarily complies with Nasdaq's corporate governance standards, except where Dutch corporate governance practices take precedence, as explained below. This alignment with Nasdaq practices is more familiar to the Company's investors, who are accustomed to these rules over those of the DCGC.

The Company's corporate governance structure is based on the requirements of the Dutch Civil Code, the Company's articles of association (as last amended by deed on June 22, 2021) ("Articles of Association"), the principles and best practices of the DCGC, and the rules applicable to companies listed on Nasdaq. This structure includes a robust risk management and control system, along with mechanisms to ensure compliance with all relevant laws and regulations.

This report outlines the Company's corporate governance framework, including how it applies the principles and best practices of the DCGC. The Company believes that its Board, composed of one executive and seven non-executive directors, effectively implements the principles and provisions applicable to the Company's governance structure, in line with best practices followed by the Company.

The full text of the DCGC can be accessed on the website of the Monitoring Commission Corporate Governance Code (www.mccg.nl). An overview of the Company's governance documents, such as its audit committee charter, compensation committee charter, nominating and corporate governance committee charter, and code of conduct, are available on the Company's website (www.uniqure.com).

When in this chapter a reference is made to Articles of Association, this shall be a reference to the Company's Articles of Association.

a) Board

All members of the Board, both the executive director and the non-executive directors, are collectively responsible for the management of the Company, the implementation of its strategy, and the general course of business. The executive director is charged with the day-to-day management of the Company, subject to the supervision of the non-executive directors, and is supported by the leadership team. The leadership team is identified as the chief operating decision-maker and regularly reviews the consolidated operating results to assess overall performance, allocate resources, and drive technological progress. The non-executive directors focus on the supervision of the policy and functioning of the executive director, as well as the general state of affairs within the Company. Their responsibilities include overseeing corporate strategy, financial reporting, risk management, and regulatory compliance. The division of tasks and responsibilities, the decision-making process and other matters concerning the Board are set out in the Corporate Governance Guidelines and Rules for the Board, which have been effective as of April 14, 2017 and are available on the Company's website. The Board is supported by a corporate secretary, who is appointed by the Board. The corporate secretary ensures that the governance process run effectively and that the Board operates in accordance with applicable regulations and internal policies.

b) Appointment and Composition

The executive director and non-executive directors are appointed by the General Meeting of the Shareholders (the "General Meeting") based on the binding nomination by the non-executive directors. In preparing such nominations, the Board takes into account the profile for the Board, including expertise, experience, independence

and diversity considerations, in accordance with the DCGC. The General Meeting may at any time override a binding nomination by a resolution passed with at least two-thirds majority of the votes cast, provided such majority represents more than half of the issued share capital. The Board determines the number of executive and non-executive directors, provided that the number of executive directors is at all times less than the number of non-executive directors. Only natural persons may be non-executive directors. The General Meeting may at any time suspend or dismiss a Board member by a resolution adopted with a two-thirds majority of the votes cast, provided such majority represents more than half of the issued share capital. The Board may also suspend (but not dismiss) an executive director.

The Board appoints an executive director as Chief Executive Officer. The Board also appoints a non-executive director to be Chair of the Board for a period determined by the Board.

The Board is authorized to represent the Company. In addition, the Company may be represented by two directors acting jointly or, if only one executive director is in office, by such executive director acting individually, in accordance with the Articles of Association.

Under the Company's Articles of Association, members of the Board are appointed for a maximum term of four (4) years, provided that, unless a Board member resigns earlier, his or her term shall end at the close of the annual General Meeting to be held in the fourth year after the date of his or her appointment. A Board member may be reappointed with due observance of the preceding sentence. The Articles of Association provide that the Board shall draw up a retirement schedule for the directors of the Board, designed to ensure continuity while avoiding, to the extent possible, a concentration of reappointments in any given year.

The current practice of the Board is to nominate members for terms of three years. Pursuant to that practice, all Board members are currently serving three-year terms. The following table sets out information with respect to the executive and non-executive directors of the Board, and their ages and position at the Company as of the date of this annual report. The business address of the executive and non-executive directors of the Board is our registered office address at Paasheuvelweg 25, 1105 BP, Amsterdam, The Netherlands.

Name	Age[1]	Nationality	Position	Member Since	Term Expires
Mr. Matthew Kapusta[3]	53	American	Executive Director	2016	2028
Mr. David Meek[2]	62	American	Non-Executive Director, Chairman of the Board	2018	2027
Mr. Madhavan Balachandran	75	American	Non-Executive Director	2017	2026
Mr. Robert Gut[3]	61	American	Non-Executive Director	2018	2028
Ms. Rachelle Jacques[2]	54	American	Non-Executive Director	2021	2027
Mr. Jack Kaye	82	American	Non-Executive Director	2016	2026
Mr. Leonard Post	73	American	Non-Executive Director	2020	2026
Mr. Jeremy P. Springhorn[3]	63	American	Non-Executive Director	2017	2028

[1] As of March 31, 2026.
[2] Reappointed at AGM held on June 18, 2024.
[3] Reappointed at AGM held on June 11, 2025.

MATTHEW KAPUSTA has served as the Company's Chief Executive Officer since December 2016 and currently serves on the Company's Board. Mr. Kapusta also served as the Company's Chief Financial Officer from January 2015 until June 2021. Mr. Kapusta also serves as a member of the Board of Directors and Executive Committee of the Alliance of Regenerative Medicine. Prior to joining uniQure, Mr. Kapusta held executive roles at AngioDynamics (Nasdaq: ANGO) from 2011 to 2015 and Smith & Nephew (NYSE: SNN) from 2009 to 2011. Mr. Kapusta's career also includes more than a decade of investment banking experience focused on emerging life-sciences companies. Mr. Kapusta was Managing Director, Healthcare Investment Banking at Collins Stewart, and held various positions at Wells Fargo Securities, Robertson Stephens, and PaineWebber. In addition, Mr. Kapusta has served as a member of the board of directors of Genezen, a private contract development and manufacturing organization, since July 2024, and previously served as a director of Decibel Therapeutics (Nasdaq: DBTX) from March 2023 to September 2023 prior to its acquisition by Regeneron Pharmaceuticals. Mr. Kapusta holds an MBA from New York University's Stern School of Business, a B.B.A. from University of Michigan's Ross School of Business and earned his C.P.A license while at Ernst & Young. The Company believes that Mr. Kapusta is qualified to serve as its Chief Executive Officer and as an Executive Director due to his broad expertise in the life science and finance industries.

DAVID MEEK has served as a member of the Company's Board since June 2018 and as Chair of the Board since June 2021. Mr. Meek has more than 30 years of experience in the biopharmaceutical industry where he has held various global executive positions in major pharmaceutical and biotechnology companies. Since June 2025, Mr. Meek has been Chief Executive Officer of Genetix Biotherapeutics, a private commercial-stage biotechnology company. Mr. Meek previously served as Chief Executive Officer and Director of Mirati Therapeutics, Inc. (Nasdaq: MRTX), a publicly traded commercial-stage oncology biotech company, from September 2021 to August 2023. Mirati has since been acquired by Bristol Myers Squibb. From January 2020 to March 2021, Mr. Meek served as President, Chief Executive Officer and Director of FerGene, Inc., a gene therapy biotechnology company focused on the treatment of cancer. From July 2016 to January 2020, Mr. Meek served as Chief Executive Officer and Director of Ipsen, a French publicly traded global biopharmaceutical company. From July 2014 to June 2016, he was Executive Vice-President and President of the oncology division of Baxalta Incorporated prior to its acquisition by Shire plc. He spent two years as the Chief Commercial Officer of Endocyte from August 2012 to July 2014. Mr. Meek also spent eight years at Novartis as a global franchise head from January 2005 to June 2007, CEO of Novartis Canada from July 2007 to December 2009, and region head of oncology for northern, central and Eastern Europe from January 2010 to August 2012. Mr. Meek has served as Chairman of the board or directors of Sobi, a Swedish publicly listed biopharmaceutical company focused on rare diseases since December 2024, and also serves as a member of the boards of directors of Cullinan Therapeutics (Nasdaq: CGEM) since May 2024. He began his biopharma career at Johnson & Johnson and Janssen Pharmaceuticals where he worked from July 1989 to December 2004 and where he held increasingly senior levels of executive roles. Mr. Meek holds a B.A. from the University of Cincinnati. The Company believes Mr. Meek is qualified to serve as a Non-Executive Director due to his extensive experience in the biotechnology industry.

MADHAVAN BALACHANDRAN has served as a member of the Company's Board since September 2017. Mr. Balachandran was Chief Operating Officer of Nutcracker Therapeutics, a developer of mRNA therapeutics, from September 2020 to March 2022. Mr. Balachandran was Executive Vice President, Operations of Amgen Inc., a global biotechnology company, from August 2012 until July 2016 and retired as an Executive Vice President in January 2017. Mr. Balachandran joined Amgen in 1997 as Associate Director, Engineering. He became Director, Engineering in 1998, and, from 1999 to 2001, he held the position of Senior Director, Engineering and Operations Services before moving to the position of Vice President, Information Systems from 2001 to 2002. Thereafter, Mr. Balachandran was Vice President, Puerto Rico Operations from May 2002 to February 2007. From February 2007 to October 2007, Mr. Balachandran was Vice President, Site Operations, and from October 2007 to August 2012, he held the position of Senior Vice President, Manufacturing. Prior to his tenure at Amgen, Mr. Balachandran held leadership positions at Copley Pharmaceuticals, now a part of Teva Pharmaceuticals Industries Ltd., and Burroughs Wellcome Company, a predecessor before mergers of GlaxoSmithKline plc. He currently serves on the board of directors of Incog, Inc., ADRX, Inc., A2 Biotherapeutics, Inc., Stevanato Group (NYSE: STVN), and Replimune Group, Inc. (Nasdaq: REPL). Mr. Balachandran previously served as a director of Catalent, Inc. (NYSE: CTLT) from May 2017 to January 2024. Mr. Balachandran holds a Master of Science degree in Chemical Engineering from The State University of New York at Buffalo, a Bachelor's degree in Chemical Engineering from the Indian Institute of Technology, Bombay, and an MBA from East Carolina University. The Company believes Mr. Balachandran is qualified to serve as a Non-Executive Director due to his extensive experience in the biotechnology industry.

ROBERT GUT, PH.D. has served as a Non-Executive Director on the Company's Board since June 2022. Dr. Gut first joined the Board in June 2018 and previously served as both a Non-Executive and an Executive Director, having served as the Company's Chief Medical Officer from August 2018 until October 2020. As the Company's Chief Medical Officer, Dr. Gut led clinical development, clinical operation, and medical team activities that successfully initiated and executed our HOPE-B pivotal trial of etranacogene dezaparvovec for hemophilia B and our Phase 1/2 clinical trial of AMT-130 for the treatment of Huntington's disease. In October 2020, he resigned as Chief Medical Officer and as Executive Director (because under Dutch law, the Executive Directors must hold an executive position with the Company). In December 2020, he was reappointed to the Board as a Non-Executive Director. Dr. Gut has more than 25 years of experience in the biopharmaceutical industry, leading clinical development and medical affairs activities in rare disorders and other therapeutic areas. For most of his career, Dr. Gut worked at Novo Nordisk Inc. (NYSE: NVO), where he headed the company's U.S. Biopharm Medical organization with leading products in hemophilia, endocrinology, and women's health (NovoSeven®, Norditropin®, and Vagifem®), totaling approximately$1.6 billion in U.S. revenue. Over his career, Dr. Gut has worked on many INDs and BLAs submissions, early-stage and late-stage drug development. He helped to achieve 11 different FDA and EMA approvals and the successful launches of those products, overseeing medical activities, including medical science liaisons and health economics and outcomes teams. He has also served for the FDA's Center for Drug Evaluation and Research as a member of the Advisory Committees for Reproductive Health Drugs and Drug Safety and Risk Management. Dr. Gut was the Chief Medical Officer of Versartis, Inc. in 2017. He received his Doctor of Medicine degree from the Medical University of Lublin and his Doctorate from the Lublin Institute of Medicine, Poland. He attended numerous postgraduate programs at Wharton, Stanford, and Harvard Business School. The Company believes Dr. Gut is qualified to serve as a Non-Executive Director due to his extensive experience in the biotechnology industry.

RACHELLE JACQUES has served as a member of the Company's Board since October 2021. Ms. Jacques has more than 25 years of industry experience, with strong global experience in strategic, cross-functional leadership roles spanning finance, business operations, manufacturing and commercial, including the successful launches of several novel therapies for rare diseases. Ms. Jacques is currently the Chief Executive Officer of Vasque Bio, a private pre-clinical stage biopharmaceutical company. Ms. Jacques previously served as President and Chief Executive Officer and a board member of Akari Therapeutics, plc (Nasdaq: AKTX), a late-stage biopharmaceutical company focused on innovative therapeutics to treat orphan autoimmune and inflammatory diseases, from March 2022 to May 2024. From February 2019 to March 2022, Ms. Jacques served as the Chief Executive Officer of Enzyvant Therapeutics, Inc., focusing on the development of transformative regenerative therapies for rare diseases. From August 2017 to February 2019, she served as Senior Vice President and Global Complement Franchise Head at Alexion Pharmaceuticals, Inc. where she was responsible for global franchise strategy development and execution across the therapeutic areas of hematology, nephrology, and neurology. From January 2016 to June 2017, she served as Vice President of U.S. Hematology Marketing at Baxalta Incorporated and then Shire plc, following Shire's acquisition of Baxalta in 2016. From July 2015 to June 2016, Ms. Jacques served as Vice President of Business Operations at Baxalta after its spinoff from Baxter International. Ms. Jacques held multiple leadership positions at Baxter, including Vice President of Finance, U.S. BioScience Business. Earlier in her career, Ms. Jacques served in various roles at Dow Corning Corporation, including operational management positions in the U.S., Europe, and China. Ms. Jacques received her B.A. in business administration from Alma College. Earlier in her career Ms. Jacques served as a financial auditor for Ernst & Young and Deloitte & Touche. Ms. Jacques is currently the chair of the board and has served on the board of directors of Corbus Pharmaceuticals (Nasdaq: CRBP) since April 2019 and previously served on the board of directors of Viela Bio from April 2020 to February 2021. She is a founding member of the Alliance for Regenerative Medicine Action for Equality Task Force, and was a member of the board of trustees of Alma College from 2022 to 2026. The Company believes Ms. Jacques is qualified to serve as a Non-Executive Director due to her extensive experience in the biotechnology industry.

JACK KAYE has served as a member of the Company's Board since June 2016. Mr. Kaye has served on the board of directors of Dyadic International, Inc. (OTC: DYAI) since February 2015, and on the board of directors of TDA Industries, Inc., a private company, since February 2024. At Dyadic, Mr. Kaye serves as Chair of the company's audit committee and as a member of the compensation committee. He has also served as Chairman of the audit committee and as a member of the compensation committee of Keryx Biopharmaceuticals, Inc. (Nasdaq: KERX) from 2006 to 2016. Mr. Kaye began his career at Deloitte LLP in 1970, and was as a partner in the firm from 1978 until May 2006. At Deloitte, he was responsible for servicing a diverse client base of public and private, global, and domestic companies in a variety of industries. Mr. Kaye has extensive experience consulting with clients on accounting and reporting matters, private and public debt financings, SEC rules and regulations, corporate governance, and Sarbanes-Oxley matters. Prior to retiring, Mr. Kaye served as Partner-in-Charge of Deloitte's Tri-State Core Client practice, a position he held for more than 20 years. Mr. Kaye has a Bachelor of Business Administration from Baruch College and is a Certified Public Accountant. The Company believes that Mr. Kaye is qualified to serve as a Non-Executive Director due to his extensive accounting and financial experience.

LEONARD POST, PH.D. has served as a member of the Company's Board since June 2020. Dr. Post has over 35 years of experience in the pharmaceutical industry, where he has held various global executive positions and has extensive experience in the research and development of product candidates. From 2016 to January 2024, Dr. Post served as Chief Scientific Officer of Vivace Therapeutics, an oncology company working on small molecules targeting the hippo pathway and, from 2018 to January 2024, as Chief Scientific Officer of its sister company Virtuoso Therapeutics, a company working on bispecific antibodies for oncology. Since 2018, Dr. Post has served as a director on the Board of CG Oncology (Nasdaq: CGON). From February 2010 until June 2016, Dr. Post worked at BioMarin (Nasdaq: BMRN), in various positions including Chief Scientific Officer. During that time, he oversaw the initiation of BioMarin's first gene therapy project for hemophilia A. Prior to that, Dr. Post served as Chief Scientific Officer of LEAD Therapeutics, Senior Vice President of Research & Development at Onyx Pharmaceuticals, and Vice President of Discovery Research at Parke-Davis Pharmaceuticals. He is also currently an advisor to Canaan Partners. Dr. Post is a virologist by training and did early work on the engineering of the herpes simplex virus as a post-doctoral fellow. He has a Bachelor of Science degree in Chemistry from the University of Michigan, and a Doctorate degree in Biochemistry from the University of Wisconsin. The Company believes Dr. Post is qualified to serve as a Non-Executive Director due to his extensive experience in the biotechnology industry.

JEREMY SPRINGHORN, PH.D. has served as a member of the Company's Board since September 2017. From April 2021 until January 2026, Dr. Springhorn was Chief Executive Officer of Nido Biosciences, a developer of small molecule therapeutics. Prior to taking his position at Nido, Dr. Springhorn was Chief Business Officer of Syros Pharmaceuticals, Inc. (Nasdaq: SYRS) from November 2017 until April 2021. Prior to taking his position at Syros, Dr. Springhorn served as Partner, Corporate Development at Flagship Pioneering from March 2015 until June 2017 where he worked with VentureLabs in helping companies in various strategic and corporate development capacities, creating next generation startups, and working with Flagship's Corporate Limited Partners. Prior to joining Flagship, Dr. Springhorn was one of the original scientists at Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) and was one of the original inventors of the drug Soliris®. At Alexion Pharmaceuticals, Dr. Springhorn was Vice President of Corporate Strategy and Business Development from 2006 until March 2015. Dr. Springhorn started at Alexion in 1992, where he served in various leadership roles in R&D before switching to Business Development in 2006. Prior to 1992, Dr. Springhorn received his Ph.D. from Louisiana State University Medical Center in New Orleans and his BA from Colby College. The Company believes Dr. Springhorn is qualified to serve as a Non-Executive Director due to his extensive experience in the biotechnology industry.

Meetings and board committees

The Company has established an Audit Committee ("Audit Committee"), a Compensation Committee ("Compensation Committee"), a Nominating and Corporate Governance Committee ("Nominating and Corporate Governance Committee"), a Research and Development Committee ("Research and Development Committee"), and a Commercial Committee ("Commercial Committee") (collectively, the "Committees"). The charter of the Compensation Committee and the Nominating and Corporate Governance Committee were amended in June 2024, and the charter of the Audit Committee was amended in February 2024. The charter of the Research and Development Committee was established in December 2019, and the charter of the Commercial Committee was established in July 2025. The charters are published on the Company's website.

Meetings

In 2025, the Board held thirteen (13) meetings in person or by means of a video conference call. During these meetings and also in informal communications among its members, extensive discussions were held to ensure the continuity of high-level management of the Company. The Chair sets the agenda and ensures that the directors receive accurate information in time. During these formal meetings and discussions, the Board primarily focuses on the objectives and strategy of uniQure, the main risks of its business, the assessment made by the executive directors of the design and effectiveness of the internal risk management and control systems, the progress made on clinical development, corporate governance, the financial budgets, the operational plan and the annual and quarterly consolidated financial statements. Specifically, pursuant to the Company's Corporate Governance Guidelines and Rules for the Board, the Board is charged with assessing major risks facing the Company and reviewing options to mitigate such risks. The Board performs this oversight role by using several different levels of review. In connection with its reviews of the operations and corporate functions of the Company, the Board addresses the primary risks associated with those operations and corporate functions. In addition, the Board reviews the risks associated with the Company's business strategies periodically throughout the year as part of its consideration of undertaking any such business strategies.

The Board has delegated certain risk oversight responsibilities to the Committees. Each of our Committees also oversees the management of the Company's risk that falls within each Committee's areas of responsibility. In performing this function, each Committee has full access to management, as well as the ability to engage advisors. For example, the Audit Committee is required to regularly review and discuss with management the Company's major financial risk exposures and the steps management has taken to monitor and control such exposures. In addition, the Audit Committee is responsible for the oversight of risks from cybersecurity threats and receives regular updates from senior management, including leaders from our information technology, legal and compliance teams regarding matters of cybersecurity. The Nominating and Corporate Governance Committee is required to regularly review the corporate governance principles of the Company and recommend to the Board any proposed changes it may deem appropriate. The Compensation Committee considers risks related to the attraction and retention of professional talent and the implementation and administration of compensation and benefit plans affecting the Company's employees. The Research and Development Committee serves as an advisory body to the Board in matters related to the Company's technology and evaluates the function and effectiveness of the Company's research, development, manufacturing operations, clinical operations, and other technical, scientific and medical operations. The Commercial Committee is charged with reviewing the Company's commercial strategy and related operations. All committees are required, pursuant to their respective charters, to report regularly to the Board. The activities of the Audit, Nominating and Corporate Governance and Compensation as well as the Research and Development and Commercial Committees are more fully described below.

Throughout 2025, the Board actively reviewed progress on the advancement and expansion of our pipeline of gene therapy product candidates, including AMT-130 for the treatment of Huntington's disease, AMT-260 for the treatment of Temporal Lobe Epilepsy, AMT-162 for the treatment of SOD-1 Amyotrophic Lateral Sclerosis and AMT-191 for the treatment of Fabry disease.

Attendance at the Board meetings during 2025 was as follows:

	Number of meetings	Meetings attended
David Meek (Chair)	13	13
Matthew Kapusta	13	13
Jack Kaye	13	12
Jeremy Springhorn	13	13
Madhavan Balachandran	13	10
Robert Gut	13	13
Rachelle Jacques	13	12
Leonard Post	13	13

Audit Committee

The Audit Committee currently consists of the Company's non-executive directors Mr. Kaye, Ms. Jacques and Dr. Springhorn. Mr. Kaye serves as the Chair of the Audit Committee. Each member satisfies the independence requirements of Nasdaq listing standards, and Ms. Jacques and Mr. Kaye qualify as an audit committee financial expert pursuant to Section 407 of the U.S. Sarbanes-Oxley Act of 2002 and as determined by the Board. The Audit Committee oversees the Company's accounting and financial reporting processes, the funding of the Company and the audits of its consolidated financial statements. In addition to the risk oversight responsibilities discussed above, the Audit Committee assists the Board and is responsible for:

- Recommending the selection of the Company's independent registered public accounting firm;
- Reviewing with the Company's independent registered public accounting firm the procedures for and results of their audits;
- Reviewing with the independent accountants and management the Company's financial reporting, internal controls and internal audit procedures;
- Reviewing and approving related party transactions; and
- Reviewing matters relating to the relationship between the Company and its independent registered public accounting firm, including the selection of and engagement fee for the independent registered public accounting firm, and assessing the independence of the independent registered public accounting firm.

The Audit Committee has the authority to engage independent legal, accounting and other advisers, as it determines necessary to carry out its duties. The Audit Committee reviews regularly and discusses with management the Company's major financial, income tax and information technology related risk exposures and the steps management has taken to monitor and control such exposures. The Audit Committee met eight (8) times during 2025. During these meetings, the committee discussed the internal controls, related party transactions, the whistle blower hotline, the (interim) financial statements, the actual financial results of each of the quarters, securities filings and financial press releases as well as the audit approach and the budget for 2025 and 2026. The Audit Committee held quarterly discussions with the independent auditor as well as an annual meeting with the independent auditor without management present.

The Audit Committee annually reviews the independent registered public accounting firm's independence, including reviewing all relationships between the independent registered public accounting firm and us and any disclosed relationships or services that may impact the objectivity and independence of the independent registered public accounting firm, and the independent registered public accounting firm's performance.

Compensation Committee

The Company's Compensation Committee currently consists of the Company's non-executive directors Mr. Balachandran, Mr. Kaye and Mr. Meek. Mr. Balachandran serves as Chair of the Compensation Committee. Each member satisfies the independence requirements of Nasdaq listing standards. The Compensation Committee assists the Board in reviewing and approving or recommending our compensation structure, including all forms of compensation relating to our non-executive and executive directors as well as senior management. Members of the Company's senior management (other than the Chief Legal Officer) may not be present at any committee meeting while the compensation of that person is deliberated, and the practice of the committee is to hold discussions on the compensation of any executive director as well as other compensation matters in executive session. The Compensation Committee has the authority to retain compensation consultants and other outside advisors to assist in the evaluation of executive officer compensation.

Subject to the terms of the compensation policy approved by our General Meeting and as required by Dutch law, the Compensation Committee assists the Board and is responsible for:

- Reviewing and approving or recommending to the Board for approval, as appropriate, the compensation of the Company's executive officers following consideration of corporate goals and objectives relevant to such executive officers;
- Overseeing the evaluation of the Company's senior executives;
- Reviewing and making recommendations to the Board regarding incentive compensation and equity-based plans; and
- Administering the Company's stock option plans.

The Compensation Committee met eight (8) times during 2025. The committee discussed the long-term incentive grant guidelines, the compensation terms of our newly recruited executives, the terms and conditions of our executive compensation, and assessed the Company's 2025 corporate goals. The remuneration policy provides for fixed pay, incentives and benefits. The fixed pay is in cash and is paid monthly. The fixed pay is set at the median of the appropriate peer group. Benefits include provisions of death, disability and medical insurance cover, directors' liability insurance and tax returns preparation costs. The Company has established a long-term incentive plan and sets incentives on a year-to-year delivery basis in support of the strategic and corporate goals as part of the ongoing enhancement of shareholders value. The target annual bonus of the CEO is 60% of the fixed pay adjusted by the corporate factor. The corporate factor is the outcome of the assessment of the achievement of the corporate goals. Over 2025 the Board assessed the corporate factor at 85%.

Nominating and Corporate Governance Committee

The Company's Nominating and Corporate Governance Committee consists of the Company's non-executive directors Dr. Springhorn, Ms. Jacques and Mr. Meek. Dr. Springhorn serves as Chair of the Nominating and Corporate Governance Committee. Each member satisfies the independence requirements of Nasdaq listing standards. The Nominating and Corporate Governance Committee assists the Board in selecting individuals qualified to serve as an executive or non-executive director of the Board and in determining the composition of the board and its committees.

The Nominating and Corporate Governance Committee assists the Board and is responsible for, among other things:

- Identifying individuals qualified to become Board members and to recommend to the Board the nominees for director at annual General Meetings;
- Recommending to the Board nominees for each committee;
- Developing and recommending to the Board corporate governance principles applicable to the Company; and
- Leading the Board in its annual review of the Board's performance.

The Nominating and Corporate Governance Committee met six (6) times during 2025. The committee discussed the composition of the committees, the selection of new non-executive members, the Board's annual performance review, and conducted a review of the Company's policies related to corporate governance.

Research and Development Committee

The Company's Research and Development Committee consists of the Company's non-executive directors Dr. Post, Dr. Gut and Dr. Springhorn. Dr. Post serves as Chair of the Research and Development Committee. The members of this committee are not subject to independence requirements of Nasdaq listing standards. The Research and Development Committee serves as an advisory body to the Board in matters related to the Company's technology, research and development activities, product pipeline, and manufacturing platform (the "Company's Technology").

The Research and Development Committee assists the Board and is responsible for, among other things:

- Advising the Board on the strategic direction of the Company with respect to the Company's Technology;
- Evaluating the function and effectiveness of the Company's research, development, manufacturing operations, clinical operations, and other technical, scientific and medical operations;
- Conferring with officers and employees of the Company as needed on matters related to the Company's technology; and
- Performing other tasks customarily performed by research and development committees as may be reasonably required to effectively advise the Board on matters associated with the Company's Technology.

The Research and Development Committee met five (5) times during 2025. The committee discussed the status of various programs, reviewed potential business development transactions, evaluated the Company's manufacturing, quality and clinical operations, and reviewed the Company's research and development pipeline.

Commercial Committee

The Company's Commercial Committee, established by the Board in June 2025, consists of the Company's non-executive directors Ms. Jacques, Mr. Meek, Mr. Balachandran, and Dr. Springhorn. Ms. Jacques serves as Chair of the Commercial Committee. The members of this committee are not subject to independence requirements of Nasdaq listing standards. Commercial Committee serves as an advisory body to the Board in matters related to the Company's commercial strategy and operations.

The Commercial Committee assists the Board and is responsible for, among other things:

- Advising the Board with respect to commercial launch preparations and execution for the Company's programs; and
- Advising the Board on strategic commercial decisions.

The Commercial Committee met four (4) times during 2025. The committee discussed the status of commercialization preparations and strategy for the Company's investigational gene therapy for Huntington's disease, AMT-130.

b) Corporate governance

In addition to U.S. securities laws, Nasdaq listing standards and rules and regulations as promulgated by the SEC, as a Dutch company, the Company's governance practices are governed by the DCGC. The DCGC contains a comply-or-explain principle, offering the possibility to deviate from the DCGC and still be compliant, provided that any deviations are adequately explained in this report. The Company, as a domestic filer under SEC and Nasdaq rules, recognizes that the DCGC and the SEC rules do not always align. In the event of non-alignment between applicable U.S. rules and the DCGC, it is permissible for the Company to deviate from the DCGC in accordance with the "comply or explain" principle, provided that deviations are disclosed and explained. In line with this principle, the Company complies with most of the principles and best practice provisions of the DCGC. Deviations from the DCGC are disclosed in this report or in the Company's Annual Report, as appropriate.

The Company conducts its operations in accordance with internationally accepted principles of good governance and best practice, while ensuring compliance with the corporate governance requirements applicable in the countries in which the Company operates. There is considerable overlap between the requirements the Company must meet under U.S. rules and regulations and the provisions of the DCGC, and the Company applies most of the provisions of the DCGC. For further clarity, the Company has listed below deviations from the DCGC and its reasons for deviating.

1.3.6 Absence of an internal audit department

The Audit Committee meets with the executive director of the Board prior to the release of the publicly disclosed financial reports, which enables the Audit Committee to monitor the quality and the completeness of such reports.

The Board is responsible for establishing and maintaining adequate internal control over financial reporting, as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act and in accordance with the DCGC. These Rules define internal control over financial reporting as a process designed by, or under the supervision of, a company's chief executive officer and chief financial officer and effected by the Board, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. Under the direction and supervision of our CEO and CFO and based on criteria established in the Internal Control—Integrated Framework (2013) issued by COSO, an assessment on the effectiveness of the Company's internal control over financial reporting was performed. This included testing and evaluating the design and operating effectiveness of the Company's internal controls.

The non-executive directors have adopted the recommendation of the audit committee, that due to the limited size and complexity of the Company and the retaining of the support services of an external professional services provider, no internal audit department had to be established in 2025.

1.4.2 – 1.4.3 Reporting on Risk Management and Statement by the Board

Best practice provisions 1.4.2 and 1.4.3 require the Board to include a substantiated declaration regarding the effectiveness of the internal risk management and control systems. The Company does not include all of the specific declarations as described in best practice provisions 1.4.2 and 1.4.3 and has therefore deviated from best practice provisions 1.4.2 (iii) and 1.4.3 (iii) – (iv). The Company is listed on the Nasdaq and is subject to the U.S. Sarbanes-Oxley Act of 2002 ("SOX"), including the provisions of Section 404(a) and 404(b) relating to internal control over financial reporting. In this context, the Company has designed, implemented, and evaluated a comprehensive system of internal control over financial reporting in accordance with SOX requirements. The Company's broader risk management framework further comprises an enterprise risk management process covering strategic, operational, financial and compliance risks, documented internal controls over financial reporting, and ongoing oversight by the Company's Audit Committee, which the Company believes allows for appropriate transparency regarding the Company's risk management and control environment. Through these alternative measures the Company aims to achieve the underlying objectives of best practice provisions 1.4.2 and 1.4.3.

2.1.5 – 2.1.6 Commitment to Diversity and Inclusion

The Company is committed to fostering a diverse and inclusive corporate culture at all levels of the organization. The Board applies the criteria for nominating executive and non-executive members as defined in the charter of the Nominating and Governance Committee. Candidates are selected based on their experience, background, skills, knowledge, and insight, ensuring a composition that aligns with the Company's strategic goals and stakeholder interests. The Company publishes biographical information regarding its Board members on its website and in its filings with the SEC. Currently, the Board consists of eight members, one of whom identifies as female. The Board is committed to ensuring that no candidate is discriminated against based on race, religion, sexual orientation, sex, physical abilities, socio-economic background or national origin. It is the Board's ambition to meet the gender diversity objectives set forth in the DCGC, while respecting the criteria for nominating executive and non-executive members as defined in the charter of the Nominating and Governance Committee.

The Company's non-discrimination practices extend to recruitment, selection, and retention processes, as well as its policies on compensation and benefits, professional development and training, and workplace culture. The Company is committed to maintaining a safe, respectful, and inclusive work environment, free from harassment and discrimination, ensuring that every employee is valued and empowered to contribute to the Company's success.

2.5.1 Corporate Responsibility

The Company is committed to making a difference to all stakeholders – patients, employees, communities and the earth. The Company's approach and efforts toward corporate responsibility continue to expand, keeping pace with the Company's growth in a changing world. In 2022, the Company established an environmental, social and

governance ("ESG") committee to provide oversight and to ensure that the right programs and policies are developed across the organization.

In the Company's corporate responsibility report —first published in 2023 and updated as appropriate — the Company aims to show how corporate efforts favorably impact all stakeholders. Accordingly, to the extent that the DCGC prescribes annual reporting on such matters, the Company's approach may constitute a deviation from the DCGC.

2.2.2 Appointment and Reappointment Periods.

According to this best practice provision, non-executive directors are appointed for a period of four years. However, the current practice of the Company's Board is to nominate directors for terms of three years.

3.1.2 Remuneration Policy.

> *vi. If shares are awarded, the terms and conditions govern this. Shares should be held for at least five years after they are awarded.*
> *vii. If share options are awarded, the terms and conditions govern this, as well as the conditions subject to which the share options can be exercised. Share options cannot be exercised during the first three years after they are awarded.*

The stock options and restricted share units the Company grants to its executive and non-executive directors of the Board and to its senior management are issued under the Company's 2014 Share Incentive Plan, as amended, and are exercisable pursuant to a vesting schedule that may allow vesting (in whole or in part) before the third anniversary of the date of grant, which is contrary to best practices provision 3.1.2 of the DCGC.

The Company believes its vesting schedules are in line with the practices of its peer group used for executive compensation purposes and necessary to attract, retain and incentivize talent adequately.

3.2.3 Severance Payment.

The remuneration in the event of a dismissal of the executive director of the Board exceeds one year's salary. The terms and conditions triggering a higher severance amount have been approved following a review and recommendation by the Compensation Committee. In addition, the Company believes it is in line with the practice of its peer group used for executive compensation.

3.3 Remuneration of the non-executive Members of the Board.

The non-executive members of the Board are eligible to receive restricted share units and options grants which will vest on the first anniversary of the grant date. The Company believes it is in line with the practice of its peer group used for non-executive compensation.

The Remuneration Policy provides guidelines for the compensation of non-executive directors. The non-executive directors are compensated for their services on the Board as follows:

- Each non-executive director receives an annual retainer of $45,000, pro-rated for service over the course of the year.
- The chairman of the Board receives an additional annual retainer of $35,000, and as such receives a total annual retainer of $80,000.
- Each non-executive director who serves as a member of a committee of the Board receives additional compensation as follows:

    - Compensation Committee: members receive an annual retainer of $7,500; the chair receives an annual retainer of $15,000 in total.
    - Nominating and Corporate Governance Committee: members receive an annual retainer of $5,000; the chair receives an annual retainer of $10,000 in total.
    - Audit Committee: members receive an annual retainer of $10,000; the chair receives an annual retainer of $20,000 in total.

- Research and Development Committee: members receive an annual retainer of $7,500; the chair receives an annual retainer of $15,000 in total.
- Commercial Committee: members receive an annual retainer of $10,000; the chair receives an annual retainer of $20,000 in total.
- Non-executive director receives an annual equity grant vesting after one year. The total fair value of the grant is divided equally by fair value between options to acquire our ordinary shares as well as restricted stock units. The size of the annual equity grant is determined by reference to our peer group companies.

Each annual retainer for Board and committee services is payable semi-annually.

Each member of the Board is entitled to be reimbursed for reasonable travel and other expenses incurred in connection with attending meetings of the Board and any committee of the Board on which she or he serves.

3.4.1 Remuneration report.

The Company has not posted a comprehensive report on its website, but remuneration information is reported publicly in the Company's filings with the SEC. The Company has engaged an independent, third-party compensation expert to benchmark its remuneration of non-executive directors, executive directors and senior management compared to the Company's peer group. Based on this evaluation, the Company believes its compensation is in line with market practice. The remuneration of the Board is disclosed in Note 23, "*Key management compensation*" to the consolidated financial statement.

3.4.2 Agreement of the executive member of the Board.

The Company does not disclose the main elements of the agreement with the executive director of the Board at the Company's website. As for the year ended December 31, 2025, the Company was a listed company on Nasdaq. The disclosures made by the Company under the applicable listing rules, and which are published at http://www.sec.gov are deemed to be appropriate in this respect.

4.2.2 Policy on bilateral contacts with shareholders

The Company has not formulated a policy on bilateral contacts with shareholders. The Company regularly meets with shareholders in one-on-one situations, which it considers to be in the best interests of the Company and its stakeholders. In such meetings no price-sensitive or material, non-public financial information shall be disclosed.

The Company announces by press release most corporate presentations held at investor conferences and provides for real time participation via webcast. However, considering the Company's size, it would create an excessive burden to establish and maintain formal bilateral contacts with shareholders. The Company endeavors to facilitate its shareholders by announcing its business updates on its website and follow such updates, to the extent possible, via webcast. The Company does not issue press releases as a standard practice. The Company will undertake that presentations are posted on its website immediately after the meetings in question.

c) Related party transactions

Details of transactions between the Company and members of the Board are set out in Note 22, "*Related party transactions*" to the consolidated financial statements. There have been no material transactions with shareholders having a significant influence over the Company.

d) Functioning of the Board of Directors

The members of the Board have discussed their individual functioning, as well as that of the Board as a whole, on a continuing basis. The Board undertakes a self-assessment of its performance annually to identify, discuss and act on any areas of potential deficiency as well as for overall improvement. Additionally, the Nominating and Governance Committee addresses, on a regular basis, the composition of the Board to determine whether the existing members of the Board collectively have the proper profile for the needs of the Company both at the present time and as are anticipated for the future, which are reported to and discussed with the full Board on a regular basis. In these discussions, consideration was also given to the composition and profile of the Board, as well as the functioning of its members and committees and the Board's tasks. The profile sets out the types of expertise the Board must possess. Annually, following the completion of the annual review of the Board's performance, the Board considers and discusses the recommendations by the Nominating and Corporate Governance Committee. The chair of the Board reviews and assesses the performance of the executive director. As the Board devotes time and addresses the issues reported at regular scheduled Board meetings, the Board, in the Company's view satisfies the defined requirements, and the composition is considered to be adequate for the proper performance of its duties. The Board has appointed from among its members five separate committees with special tasks, the Audit Committee, the Compensation Committee, the Nominating and Corporate Governance Committee, the Research and Development Committee, and the Commercial Committee. These committees prepare the decision making of the Board on the relevant matters. The following regulations can be found on the Company's website: Corporate Governance Guidelines and Rules for the Board, Disclosure Policy, Expanded Access Policy, Insider Trading Policy, Code of Conduct, Related Party Transaction Policy, Audit Committee Charter, Compensation Committee Charter, Nominating and Corporate Governance Committee Charter, Research and Development Committee Charter, Commercial Committee Charter, Remuneration Policy and the Articles of Association of the Company.

e) Compensation of the executive director

This report sets out the remuneration policy operated by the Company in respect of its executive director.

In summary, the Company's compensation program is designed to be straightforward in nature with five core elements, the first three of which are compensation related and the last two are benefits reflecting local market practices for each executive officer.

Element	Purpose	Key Features
Base Salary	Provide market-competitive fixed compensation Attract exceptional talent in the relevant market	• Fixed cash compensation • Reviewed annually • Value informed by market levels for executives with comparable qualifications, experience, and responsibility, coupled with the nature, scope and impact of the role • Target approximately 50th percentile of market peers, considering the factors noted above
Short-Term Incentive (Annual Cash Bonus)	Reward for achievement of pre-defined criteria in areas of strategic importance to the Company Align compensation with Company performance	• Subject to the approval of the Board in its discretion • Discretionary variable cash compensation of 60% of annual Base Salary in 2025 • Maximum opportunity capped at 150% of target • Weighting is based solely on performance against corporate goals for the Chief Executive Officer • Corporate and individual targets established in the beginning of each year • Assessment against the predetermined targets informs actual cash bonus that is awarded • Target opportunity informed by levels in the market, with reference to the 50th percentile
Long-Term Incentives (Equity Awards)	Align long-term interests with shareholders Reward sustainable value creation Encourage retention	• Annual awards subject to the approval of the Board in its discretion • Annual awards in 2025 were a mix of stock options and restricted stock units • Stock options have a ten-year term, with 25% vesting after one year and then ratably on a quarterly basis • Restricted stock units vest ratably on an annual basis over three years • Target opportunity informed by prior year performance and levels in the market with reference to the 50th percentile
Pension and Retirement Savings Plans	Provide market-competitive retirement benefits	• Based on local market practice • U.S.-based employees eligible to participate in a qualified 401(k) Plan with matching of up to 3% of base salary

Other Benefits	Provide market competitive benefits focused on well-being	• Medical, dental and vision health care plans with premiums paid by the company • Up to four weeks of paid time off

f) Financial statements

The Annual Accounts have been prepared by the Company's executive director and discussed within the full Board. The Report of the Independent Auditor, KPMG Accountants N.V., is included in 'D Other Information.' The financial statements are being presented for adoption by the General Meeting. The Board recommends that the General Meeting adopts these financial statements.

g) Shareholders and the General Meeting

The annual General Meeting shall be held within six months after the end of each financial year. The Company's financial year corresponds to a calendar year. The Board or those who are authorized by law or pursuant to the Articles of Association of the Company may convene the General Meeting. The Articles of Association provide that, unless a different majority or quorum is required by law or the Articles of Association, all resolutions of the General Meeting shall be adopted by a simple majority of the votes cast, provided that more than one-third (33⅓%) of the issued share capital is represented.

An Extraordinary General Meeting of Shareholders may be convened by the Board or by those who are authorized by law or pursuant to the Articles of Association of the Company.

In accordance with Dutch law and the Articles of Association, shareholders representing alone or in aggregate at least one-tenth of the Company's issued and outstanding share capital may petition a Dutch court for authorization to convene a General Meeting if the Company does not convene such meeting upon their request in a timely fashion.

A record date shall be applied to determine which shareholders are entitled to attend and vote at the General Meeting. The record date (if set) will, in line with the Dutch Civil Code, always be the twenty-eighth day prior to the date of the meeting.

Each of the Company's shares carries the right to cast one vote. Shareholders may vote by proxy. No votes may be cast at the General Meeting in respect of Shares held by the Company or any of its Subsidiaries as long as they are held in treasury.

Amendment of the Articles of Association

The General Meeting may only resolve to amend the Articles of Association at the proposal of the Board.

Issuance of ordinary shares, options, restricted share units and performance share units

The General Meeting is authorized to issue shares or grant rights to subscribe for ordinary shares. Under Dutch law and under the Company's Articles of Association, following a proposal by the Board, the General Meeting can delegate this authority to the Board for a specific period not exceeding five years.

On June 11, 2025, the General Meeting delegated the authority to the Board to issue ordinary shares in the share capital of the Company and to grant rights to subscribe for ordinary shares and to limit or exclude pre-emptive rights in connection therewith for a period of 18 months following June 11, 2025, up to a maximum of 10,750,000 ordinary shares in connection with any single issuance or series of related issuances.

Acquisition of own shares

The Company may not subscribe for newly issued ordinary shares in its own share capital. The Company may, however, subject to certain restrictions under Dutch law and the Articles of Association, acquire shares in its own share capital. The Company may acquire fully paid-up shares in its own share capital at any time for no consideration.

Furthermore, subject to certain provisions of Dutch law and the Articles of Association, the Company may repurchase fully paid-up shares in its own share capital if (i) the Company's shareholders' equity (*eigen vermogen*) less the payment required to make the acquisition does not fall below the sum of paid-in and called-up share capital plus any reserves required by Dutch law or the Articles of association and (ii) the aggregate nominal value of shares of the Company which the Company acquires, holds or on which the Company holds a pledge (*pandrecht*) or which are held by a subsidiary of the Company, would not exceed 50% of its then-current issued share capital.

Other than shares acquired for no consideration, ordinary shares may only be acquired following a resolution of the Board, acting pursuant to an authorization for the repurchase of shares granted by the General Meeting. An authorization by the General Meeting for the repurchase of shares can be granted for a maximum period of 18 months. Such authorization must specify the number of shares that may be acquired, the manner in which these shares may be acquired and the price range within which the shares may be acquired. The Board has been authorized, for a period of 18 months to be calculated from the date of the annual general meeting of shareholders held on June 11, 2025, to cause the repurchase of ordinary shares by the Company of up to 10% of the Company's issued share capital, for a price per share between the nominal value of the ordinary shares and an amount of 110% of the highest price of the ordinary shares officially quoted on any of the official stock markets on which the Company is listed during any of 30 banking days preceding the date the repurchase is effected or proposed.

No authorization of the General Meeting is required if fully paid-up ordinary shares are acquired by the Company with the intention of transferring such ordinary shares to the Company's employees under an applicable employee stock purchase plan, provided such ordinary shares are officially quoted on any of the official stock markets.

Reduction of share capital

Subject to Dutch law, the General Meeting may resolve to reduce the Company's issued and outstanding share capital by (i) amending the Articles of Association to reduce the nominal value of the shares or (ii) canceling:

- shares which the Company holds itself in the Company's share capital, or
- all issued shares against repayment of the amount paid on those shares.

Dividends and other distributions

The Board may determine which part of the profits shall be added to the reserves. The part of the profit remaining after reservation shall be at the disposal of the General Meeting, which may resolve to carry it to the reserves or to distribute it among the shareholders.

Under the Articles of Association, the Company may make distributions of profit to the Company's shareholders after adoption of the Company's annual accounts demonstrating that such distributions are legally permitted. With due observance of applicable law and the Articles of Association, the Board may resolve to make interim distributions to the shareholders.

The General Meeting may, at the proposal of the Board, resolve to distribute to the shareholders a dividend in the form of shares in the share capital of the Company. Each of the Company's shares entitles its holder to equal rights to dividends and other distributions.

h) Anti-takeover and/or Protective measures

Under Dutch law, various protective measures are possible and permissible within the boundaries set by Dutch statutory and case law. Certain provisions of the Articles of Association may make it more difficult for a third party to acquire control of the Company or to effect a change in the composition of the Board.

These provisions include:

- *Staggered Board Terms*: The non-executive directors of the Company serve staggered three-year terms, meaning that only approximately one-third of the non-executive directors may be subject to election or re-election in any given year. This staggered approach provides continuity and stability in the Board's composition.

- *Dismissal and Suspension of Directors*: A provision in the Articles of Association states that the directors of the Company may only be dismissed or suspended by the General Meeting with a two-thirds majority of votes cast, provided such majority represents more than half of the Company's issued share capital. This ensures that a significant majority of shareholders must agree before a director can be removed.

- *Binding Nomination for Directors*: Executive Directors and Non-Executive Directors can only be appointed upon a binding nomination from the Non-Executive Directors. Such nomination can only be overruled by the General Meeting with a two-thirds majority of votes cast, representing more than half of the issued share capital.

- *Proposal Requirement for Amendments*: A resolution to amend the Articles of Association or to dissolve the Company may only be adopted by the General Meeting at the proposal of the Board. This provision helps maintain the stability and consistency of the company's governance structure.

Additionally, in accordance with the DCGC, shareholders who have the right to propose agenda items or request the convening of a General Meeting must first consult with the Board. If such rights are exercised and could lead to a change in strategy (such as the dismissal of one or more directors), the Board is entitled to invoke a response period of up to 180 days. During this period, the Board will engage in further deliberation and consultation with the concerned shareholders, exploring potential alternatives. The response period may only be invoked once per General Meeting and is not applicable if the response period or a statutory cooling-off period (discussed below) has already been invoked for the same matter or if a shareholder holds at least 75% of our issued share capital as a consequence of a successful tender offer for our shares.

Moreover, the Board can invoke a cooling-off period of up to 250 days in the case of an unsolicited takeover bid or shareholder activism that conflicts with the company's interests. This period allows the Board to gather relevant information and consult with shareholders holding at least 3% of the Company's issued share capital. The cooling-off period can be extended only if necessary for careful decision-making and after consultation with relevant stakeholders, including the Dutch works council (if applicable). Shareholders representing at least 3% of the company's issued share capital may request the early termination of the cooling-off period through the Enterprise Chamber (Ondernemingskamer) of the Amsterdam Court of Appeal.

These protective measures are designed to ensure that decisions are made with careful consideration of the long-term interests of the Company and its stakeholders, which includes not only shareholders but also employees and other relevant parties.

(i) Company culture and Code of Conduct

The Company has established the following values:

- Passionate - We are driven by a deep commitment to improving patients' lives and channel our energy and dedication into advancing our mission.

- Tenacious - We pursue scientific progress and operational excellence with determination, resilience and focus, recognizing that patients depend on our perseverance.

- Honorable - We act with integrity, respect and transparency, fostering trust among colleagues, patients, partners and other stakeholders; and

- Exceptional - We strive for the highest standards in everything we do, setting ambitious goals and delivering quality outcomes for patients and stakeholders.

These values were established to guide the Company and its employees in order to effectively execute the Company's mission and strategy. In order to assure that employees live up to these principles we have implemented various training and evaluation programs. By virtue of these programs and evaluations we create an environment in which the employees, with more than 20 different nationalities represented, can contribute to the growth and values of the Company.

The Company has a Code of Conduct in place which sets forth the legal and ethical standards of conduct for employees and directors. The DCGC of Conduct is provided to every new employee and the Company annually requires confirmation from all employees and directors of their adherence.

i) Non-Executive Board Report

The personal information of the non-executive directors is detailed starting at page 54.

The non-executive directors have assessed and considered if it is necessary to establish an internal audit department. The non-executive directors following the recommendation of the audit committee, that due to the limited size and complexity of the Company and retaining of the support services of an external professional services provider no internal audit department had to be established in 2025, have adopted the recommendation.

The evaluation accountability is incorporated in the section "*Nominating and Corporate Governance Committee"* starting at page 59 and in the section "*Functioning of the Board of Directors*" staring at page 64.

Details of the reporting of the Committees and on the attendance of the Board and Committee meetings are reflected in each Committee section starting at page 57.

(1) In the opinion of the non-executive directors, the independence requirements referred to in best practice provision 2.1.7 to 2.1.9 have been fulfilled.

5 Statement of the Board of Directors

The Board of Directors is responsible for the preparation of the Annual Accounts and the Annual Report of uniQure N.V. for the year ended December 31, 2025, in accordance with applicable Dutch law and IFRS Accounting Standards as endorsed by the European Union (EU-IFRS).

RESPONSIBILITY STATEMENT PURSUANT TO SECTION 5:25C PARAGRAPH 2(C) OF THE DUTCH FINANCIAL MARKETS SUPERVISION ACT (*'Wet op het financieel toezicht'*)

Each of the Directors of the Board confirms that to the best of his or her knowledge:

- the uniQure N.V. 2025 Annual Accounts give a true and fair view of the assets, liabilities, financial position and profit or loss of uniQure N.V. and the entities included in the consolidation;
- the uniQure N.V. 2025 Annual Report gives a true and fair view of the state of affairs on December 31, 2025, the course of business during the financial year of uniQure N.V. and of the entities affiliated to it whose data are included in the 2025 Annual Accounts and that the 2025 Annual Report describes the substantial risks with which uniQure N.V. is confronted.

Amsterdam, April 24, 2026

Executive Director

/s/ Matthew Kapusta
Matthew Kapusta, Chief Executive Officer

Non-Executive Directors

/s/ David Meek
David Meek, Chairman

/s/ Madhavan Balachandran
Madhavan Balachandran, Member

/s/ Robert Gut
Robert Gut, Member

/s/ Rachelle Jacques
Rachelle Jacques, Member

/s/ Jack Kaye
Jack Kaye, Member

/s/ Leonard Post
Leonard Post, Member

/s/ Jeremy P. Springhorn
Jeremy P. Springhorn, Member

B Consolidated Financial Statements of uniQure N.V. for the year ended December 31, 2025

uniQure N.V.

Consolidated Statement of Financial Position

	Note	December 31, 2025	December 31, 2024
		$ in thousands	
Current assets			
Cash and cash equivalents	4	80,240	158,930
Current investment securities	4	542,301	208,591
Accounts receivable	4, 14	5,863	5,881
Prepaid expenses		20,506	9,281
Other current assets and receivables		7,073	7,602
Total current assets		**655,983**	**390,285**
Non-current assets			
Property, plant and equipment, net	5	13,800	20,424
Other investments	3, 4	32,665	28,211
Right-of-use assets	20	12,225	12,980
Intangible assets other than goodwill, net	6	80,799	77,552
Goodwill	6	25,355	22,414
Deferred tax assets, net	18	8,761	9,982
Other non-current assets		5,561	1,399
Total non-current assets		**179,166**	**172,962**
Total assets		**835,149**	**563,247**
Current liabilities			
Accounts payable		5,170	7,227
Accrued expenses and other current liabilities	7	36,190	27,643
Liability related to pre-funded warrants	4	12,595	—
Provisions	3	5,102	1,582
Lease liabilities - current	20	4,604	4,156
Total current liabilities		**63,661**	**40,608**
Non-current liabilities			
Borrowings	8	44,478	50,494
Liability from royalty financing agreement	9	440,706	431,496
Lease liabilities - non-current	20	11,631	12,318
Contingent consideration	4	18,736	10,860
Provisions	3	901	4,856
Deferred tax liability, net	18	7,967	7,043
Other non-current liabilities		2,753	3,086
Total non-current liabilities		**527,173**	**520,153**
Total liabilities		**590,834**	**560,761**
Shareholders' equity			
Share capital		3,688	2,945
Share premium		1,346,943	955,319
Other reserves		173,183	164,125
Accumulated deficit		(1,279,499)	(1,119,903)
Total shareholders' equity	11	**244,315**	**2,486**
Total liabilities and shareholders' equity		**835,149**	**563,247**

After appropriation of the result for the year.

The accompanying notes are an integral part of these consolidated financial statements.

uniQure N.V.

Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss

	Note	Years ended December 31, 2025	2024
		$ in thousands, except for per share data (in $)	
License revenues	14	15,934	10,133
Contract manufacturing revenues	14	—	6,114
Collaboration revenues	14	164	10,872
Total revenues		**16,098**	**27,119**
Cost of license revenues		(1,686)	(1,267)
Cost of contract manufacturing revenues		—	(17,060)
Gross profit		**14,412**	**8,792**
Operating expenses:			
Research and development expenses	13	(136,076)	(135,180)
Selling, general and administrative expenses	13	(64,470)	(50,553)
Total operating expenses		**(200,546)**	**(185,733)**
Other income	15	14,410	11,143
Other expense	16	(7,454)	(4,201)
Loss from operations		**(179,178)**	**(169,999)**
Finance income	17	61,226	21,415
Finance expense	17	(35,993)	(72,650)
Finance expense, net		**25,233**	**(51,235)**
Loss before income tax expense		**(153,945)**	**(221,234)**
Income tax expense	18	(5,650)	(2,687)
Net loss		**(159,595)**	**(223,921)**
Total other comprehensive loss, net of income tax:			
Items that may be reclassified subsequently to profit or loss			
Foreign currency translation adjustments		(7,754)	(165)
Defined benefit pension gain, net of taxes		771	327
Total comprehensive loss		**(166,578)**	**(223,759)**
Loss per share			
Basic and diluted loss per ordinary share	19	(2.78)	(4.60)

The accompanying notes are an integral part of these consolidated financial statements.

uniQure N.V.

Consolidated Statement of Changes in Equity

	Note	Share Capital No. of shares	Share Capital Amount	Share Premium	Other Reserves	Accumulated Deficit	Total Equity
				$ in thousands (except number of shares)			
Balance at January 1, 2024		**47,833,830**	**2,883**	**953,258**	**149,716**	**(895,982)**	**209,875**
Net loss		—	—	—	—	(223,921)	(223,921)
Other comprehensive income		—	—	—	162	—	162
Total comprehensive income / (loss)		—	—	—	162	(223,921)	(223,759)
Exercises of share options	12	169,898	9	2,065	—	—	2,074
Restricted and performance share units distributed during the period	12	974,209	52	(52)	—	—	—
Share-based compensation expense	12	—	—	—	14,247	—	14,247
Issuance of ordinary shares relating to employee stock purchase plan		10,150	1	48	—	—	49
Balance at December 31, 2024		**48,988,087**	**2,945**	**955,319**	**164,125**	**(1,119,903)**	**2,486**
Net loss		—	—	—	—	(159,595)	(159,595)
Other comprehensive loss		—	—	—	(6,983)	—	(6,983)
Total comprehensive loss		—	—	—	(6,983)	(159,595)	(166,578)
Follow-on public offerings		11,810,370	655	380,082	—	—	380,737
Tax benefit related to historical share issuance costs		—	—	468	—	—	468
Exercises of share options	12	694,879	42	11,120	—	—	11,162
Restricted and performance share units distributed during the period	12	843,381	46	(46)	—	—	0
Share-based compensation expense	12	—	—	—	16,040	—	16,040
Balance at December 31, 2025		**62,336,717**	**3,688**	**1,346,943**	**173,182**	**(1,279,498)**	**244,315**

The accompanying notes are an integral part of these consolidated financial statements.

uniQure N.V.

Consolidated Statement of Cash Flows

	Note	Years ended December 31, 2025	2024
		$ in thousands	
Cash flows from operating activities			
Net loss		(159,595)	(223,921)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation, amortization and impairment of intangible assets and property, plant and equipment, and right-of-use assets	5, 6, 20	17,497	16,194
Amortization of discount on investment securities	4, 17	(10,463)	(10,901)
Share-based compensation expense	12	16,040	14,247
Interest expense, net	8, 9, 17, 20	37,016	61,233
Change in fair value of contingent consideration	4	6,247	(1,817)
Changes in fair value of liability related to pre-funded warrants	4	(12,405)	—
Unrealized foreign exchange (gains) / losses, net		(22,604)	14,568
Deferred tax expense	18	1,608	2,687
Other items, net		(10,715)	(15,319)
Changes in operating assets and liabilities:			
Accounts receivable, prepaid expenses and other current assets	14	(8,597)	(2,243)
Inventories		—	2,421
Accounts payable		(2,667)	1,520
Accrued expenses and other liabilities	7	3,397	(5,281)
Contingent consideration milestone payment	4	—	(19,608)
Cash (used in) operating activities		(145,241)	(166,220)
Interest paid	9, 17	(26,730)	(23,709)
Interest received	17	5,546	10,584
Income tax paid	18	(7,542)	(21)
Net cash (used in) operating activities		(173,967)	(179,366)
Cash flows from investing activities			
Proceeds from maturity of investment securities	4	337,195	534,498
Investment in investment securities	4	(658,375)	(359,841)
Divestment of commercial manufacturing facility	3	—	(8,321)
Purchase of property, plant and equipment	5	(439)	(3,368)
Purchase of intangible assets	6	(1,500)	—
Change in deposits		—	1,740
Contingent consideration milestone payment	4	—	(8,559)
Net cash (used in) / generated from investing activities		(323,119)	156,149
Cash flows from financing activities			
Proceeds from follow-on public offerings of ordinary shares, net of issuance costs	11	380,737	—
Proceeds from issuance of pre-funded warrants, net of issuance costs	4	23,499	—
Proceeds from issuance of ordinary shares related to employee stock option and purchase plans	12	11,162	2,123
Repayment of borrowings	8	—	(53,050)
Payments for principal portion of lease liability	20	(2,555)	(3,366)
Net cash generated from / (used in) financing activities		412,843	(54,293)
Currency effect cash and cash equivalents		5,553	(4,920)
Net (decrease) in cash and cash equivalents		(78,690)	(82,430)
Cash and cash equivalents at the beginning of the year		158,930	241,360
Cash and cash equivalents at the end of the year		**80,240**	**158,930**

The accompanying notes are an integral part of these consolidated financial statements.

Notes to the Consolidated Financial Statements

1. General information

uniQure N.V.

uniQure N.V. (the "Company") was incorporated on January 9, 2012 as a private company with limited liability (besloten vennootschap met beperkte aansprakelijkheid) under the laws of the Netherlands. The Company's business was founded in 1998 and was initially operated through its predecessor company, Amsterdam Molecular Therapeutics (AMT) Holding N.V ("AMT"). In 2012, AMT undertook a corporate reorganization, pursuant to which uniQure B.V. acquired the entire business and assets of AMT and completed a share-for-share exchange with the shareholders of AMT. Effective February 10, 2014, in connection with its initial public offering, the Company converted into a public company with limited liability (naamloze vennootschap) and changed its legal name from uniQure B.V. to uniQure N.V. Unless the context indicates otherwise, all references to "uniQure" or the "Company" refer to uniQure and its consolidated subsidiaries.

The Company is registered in the trade register of the Dutch Chamber of Commerce (Kamer van Koophandel) under number 54385229. The Company's headquarters are in Amsterdam, the Netherlands, and its registered office is located at Paasheuvelweg 25, Amsterdam 1105 BP, the Netherlands and its telephone number is +31 20 240 6000. The Company's website address is www.uniqure.com.

The Company's ordinary shares are listed on Nasdaq and trade under the symbol "QURE".

This Annual Report and the Consolidated Financial Statements (this "Annual Report") were authorized for issue by the board of directors on April 24, 2026 and will be filed at the trade register of the Chamber of Commerce in Amsterdam, the Netherlands within eight days after adoption by the 2026 general meeting of shareholders.

The Company is a leader in the field of gene therapy, seeking to deliver to patients suffering from rare and other devastating diseases single treatments with potentially curative results. The Company is advancing a focused pipeline of innovative gene therapies, including clinical candidates for the treatment of Huntington's disease, refractory mesial temporal lobe epilepsy ("MTLE"), Fabry disease and amyotrophic lateral sclerosis ("ALS").

Organizational structure of uniQure

uniQure N.V. is the ultimate parent of the following entities:

Entity name
uniQure biopharma B.V.
uniQure IP B.V.
uniQure Inc.
uniQure France SAS (formerly Corlieve Therapeutics SAS)
uniQure Switzerland GmbH (formerly Corlieve Therapeutics AG)

2. Summary of Significant Accounting Policies

The principal accounting policies applied in the preparation of these consolidated financial statements are set out below. These policies have been consistently applied to all the years presented, unless otherwise stated.

2.1 Basis of Preparation

The Company prepared its consolidated financial statements in compliance with IFRS Accounting Standards as endorsed by the European Union (EU-IFRS) and with Part 9 of Book 2 of the Dutch Civil Code, effective as of December 31, 2025.

The consolidated financial statements have been prepared on a historical cost basis, except for contingent consideration, the series C preferred stock and convertible promissory note and the liability related to pre-funded warrants, which are recorded at fair value through profit or loss.

The functional currency of the Company and each of its entities (with the exception of uniQure Inc. and uniQure Switzerland GmbH) is the euro (€, or EUR). This represents the currency of the primary economic environment in which the entities operate. The functional currency of uniQure Inc. is the U.S. dollar ($) and the functional currency of uniQure Switzerland GmbH is the Swiss Franc (CHF).

The Company files consolidated financial statements with the SEC in accordance with U.S. generally accepted accounting principles, presented in U.S. dollars. To consistently report financial information, the Company is also presenting its consolidated financial statements in accordance with IFRS Accounting Standards in U.S. dollars, except where otherwise indicated. Transactions denominated in currencies other than U.S. dollars are presented in the transaction currency with the U.S. dollar amount included in parenthesis, converted at the foreign exchange rate as of the transaction date.

Foreign currency transactions are translated into the functional currency using the exchange rates prevailing at the dates of the transactions. Foreign exchange gains and losses resulting from the settlement of such transactions and from the remeasurement at exchange rates prevailing at reporting date of monetary assets and liabilities denominated in foreign currencies are recognized in profit or loss.

Upon consolidation, the assets and liabilities of foreign operations are translated into the functional currency of the shareholding entity at the exchange rates prevailing at the reporting date; items of income and expense are translated at monthly average exchange rates. The consolidated assets and liabilities are translated from uniQure N.V.'s functional currency into the presentation currency U.S. dollar at the exchange rates prevailing at the reporting date; items of income and expense are translated at monthly average exchange rates. Issued capital and share premium are translated at historic rates with differences to the reporting date rate, recorded as translation adjustments in other reserves. The exchange differences arising on translation for consolidation are recognized in other comprehensive income. On disposal of a foreign operation, the component of other comprehensive income relating to the foreign operation is recognized in profit or loss.

The consolidated financial statements presented have been prepared on a going concern basis based on the Company's cash and cash equivalents as of December 31, 2025, and the Company's budgeted cash flows for the twelve months following the issuance date.

The financial information of the Company is included in the consolidated financial statements. For this reason, pursuant to Section 2:402 of the Dutch Civil Code, the Statement of Profit or Loss in the separate financial statements exclusively states the share of the result of participating interests and other income and expenses. For an appropriate interpretation of these statutory financial statements, the consolidated financial statements of the Company should be read in conjunction with the separate financial statements, as included in section C "Company-only Financial Statements".

2.2 Use of judgments and estimates

In preparing these consolidated financial statements, management made judgments, estimates and assumptions that affect the application of the Company's accounting policies and the reported amounts of assets, liabilities, disclosure of contingent assets and liabilities at the date of the consolidated financial statements and reported amounts of revenues and expenses during the reporting period. Actual results may differ from these estimates. Estimates and underlying assumptions are reviewed on an ongoing basis. Revisions to estimates are recognized prospectively.

Estimates and assumptions are primarily made in relation to contingent consideration related to the July 2021 acquisition of uniQure France SAS (the "uniQure France Acquisition"). If actual results differ from the Company's estimates, or to the extent these estimates are adjusted in future periods, the Company's results of operations could either benefit from, or be adversely affected by, any such change in estimate.

2.3 New standards, amendments and interpretations

New and amended standards adopted by the Company in 2025

There were no new IFRS Accounting Standards adopted by the Company in 2025.

New and amended standards not yet adopted by the Company

IFRS 18, *Presentation and Disclosure in Financial Statements*, was issued by the International Accounting Standards Board ("IASB") in April 2024. This standard supersedes IAS 1 and introduces enhanced requirements for the presentation and disclosure of financial statements, focusing particularly on the statement of profit or loss. IFRS 18 becomes effective for annual reporting periods beginning on or after January 1, 2027, with earlier adoption permitted. The Company has not yet adopted IFRS 18.

There are no other IFRS Accounting Standards or International Financial Reporting Interpretations Committee ("IFRIC") Interpretations that are not yet effective or that could have been early adopted that would have a material impact on the Company in the current or future reporting periods and on foreseeable future transactions.

2.4 Consolidation

The consolidated financial statements include the financial statements of the Company and subsidiaries controlled by the Company, as investees. Control is achieved when the Company:

- Has power over the investee;
- Is exposed, or has rights, to variable returns from its involvement with the investee; and,
- Has the ability to use its power to affect its returns.

The Company reassesses whether or not it controls an investee if facts and circumstances indicate that there are changes to one or more of these three elements of control.

Intra-group transactions, balances, income and expenses on transactions between uniQure entities are eliminated in consolidation. Profits and losses resulting from intra-group transactions that are recognized in assets are also eliminated. Accounting policies of subsidiaries have been changed where necessary to ensure consistency with the policies adopted by the Company.

2.5 Current versus non-current classification

The Company classifies assets and liabilities as current when they are expected to be realized or settled within twelve months after the end of the reporting period (except for liabilities for which the Company does not have an unconditional right to defer settlement of that liability for at least twelve months after the end of the reporting period), when they are realized or settled within the Company's normal operating cycle or when they are primarily held for trading purposes. Cash and cash equivalents are presented as current unless it is restricted from being exchanged or used to settle a liability for at least twelve months after the reporting period. The Company classifies the pre-funded warrants as current since they are exercisable at the option of the holder and may be settled at any time. Refer to Note 2.16, "*Financial Instruments*".

Deferred tax assets and liabilities, if any, are classified as non-current.

2.6 Fair value measurement

The Company measures financial instruments and non-financial assets at fair value at each reporting date using valuation techniques that are appropriate in the circumstances and for which sufficient data are available as disclosed in Note 4.3, "*Fair value measurement"*.

2.7 Business Combinations

a. Goodwill

Goodwill represents the excess of the fair value of the consideration transferred over the fair value of the net assets assumed in a business combination. Goodwill is not amortized but is evaluated for impairment on an annual basis and between annual tests if the Company becomes aware of any events occurring or changes in circumstances that would more likely than not reduce the fair value of a reporting unit below its carrying amount.

b. Acquired research and development

An in-process research and development intangible asset ("IPR&D Intangible Asset") is considered to be indefinite lived until the completion or abandonment of the associated research and development efforts and is not amortized. If and when development is completed, which generally occurs when regulatory approval to market a product is obtained, the associated asset would be deemed finite-lived and would then be amortized based on its respective useful life at that point in time.

c. Contingent consideration

Contingent consideration is remeasured at fair value at each reporting date and subsequent changes in the fair value of the contingent consideration are recognized in profit or loss, within research and development expenses. Changes in contingent consideration result from changes in assumptions regarding the probabilities of achieving the relevant milestones, or probability of success ("POS"), the estimated timing of achieving such milestones, and the interest rate to discount the payments.

The Company presents contingent consideration payments reflecting the settlement of the fair value of the contingent consideration recognized on initial recognition as investing activities in the Consolidated Statement of Cash Flows. Contingent consideration payments in excess of the fair value at which the contingent consideration was initially recognized are presented in operating activities in the Consolidated Statement of Cash Flows.

2.8 Notes to the Consolidated Statement of Cash Flows

The Consolidated Statement of Cash Flows have been prepared using the indirect method. Cash and cash equivalents include bank balances, demand deposits and other short-term highly liquid investments (with maturities of less than three months at time of purchase) that are readily convertible into a known amount of cash and are subject to an insignificant risk of fluctuation in value.

Cash flows denominated in foreign currencies are translated at the average exchange rates for the reporting period. Exchange differences, if any, affecting cash and cash equivalents are presented separately in the consolidated statements of cash flows.

2.9 Segment reporting

The Company's chief operating decision-maker regularly reviews and determines whether a particular component of uniQure's activities constitutes a separate operating segment by identifying and reviewing the allocation of resources to that component of uniQure's activities and/or assessing the performance of that particular component of uniQure's activities. The leadership team is identified as the chief operating decision-maker and reviews the consolidated operating results regularly to make decisions about the resources and to assess overall performance. The leadership team regularly reviews total cash operating expenditures by departmental area. The leadership team has determined that the activities of uniQure are one segment, which comprises the discovery, development and commercialization of innovative gene therapies, and the segmental analysis is the same as the analysis for uniQure as a whole.

2.10 Impairment

Non-financial assets

Goodwill impairment reviews are undertaken annually or more frequently if events or changes in circumstances indicate a potential impairment.

Non-financial assets, other than goodwill, that have been previously impaired are reviewed for possible reversal of the impairment at each subsequent reporting date.

2.11 Accounts receivable

Accounts receivables include amounts due from services provided to the Company's licensing and collaboration partners as well as unconditional rights to consideration from its licensing and collaboration partners.

2.12 Other (non) current assets

Deposits paid are either presented as other current assets or as other non-current assets based on duration of the underlying contractual arrangement. Deposits are classified as restricted cash and primarily relate to facility leases.

Contract assets are presented in current assets or as non-current assets based on the timing of the right to consideration.

2.13 Property, plant and equipment

Property, plant and equipment comprise mainly of laboratory equipment, leasehold improvements, construction-in-progress ("CIP"), and office equipment. All property, plant and equipment is stated at cost less accumulated depreciation. CIP consists of capitalized expenses associated with construction of assets not yet placed into service. Depreciation commences on CIP once the asset is placed into service based on its useful life determined at that time.

Subsequent costs are included in the asset's carrying amount or recognized as a separate asset, as appropriate, when it is probable that future economic benefits associated with the item will flow to uniQure and the cost of the item can be measured reliably. All other repairs and maintenance costs are expensed as incurred. Upon disposal, the related cost and accumulated depreciation is removed from the accounts and any resulting gain or loss on the transaction is recognized in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss.

Depreciation is calculated using the straight-line method over the estimated useful lives of the assets (or in the case of leasehold improvements a shorter lease term), which are as follows:

Leasehold improvements	Between 3 – 15 years
Laboratory equipment	5 years

- Office equipment Between 3 – 5 years

The assets' residual values and useful lives are reviewed, and adjusted if appropriate, at each reporting date. An asset's carrying amount is written down immediately to its recoverable amount if the asset's carrying amount is greater than its estimated recoverable amount.

2.14 Intangible Assets

(a) Licenses

Acquired licenses have either an indefinite or a finite useful life. Acquired licenses with a finite or indefinite useful life are carried at cost. An intangible asset with an indefinite life is tested annually for impairment. The cost of an intangible asset with a finite life is adjusted by accumulated amortization and impairment losses. Amortization is calculated using the straight-line method to allocate the cost of licenses over their estimated useful lives (generally 20 years unless a license expires prior to that date). Amortization is included in research and development expenses.

(b) Research and development

Research and development expenditures are expensed as incurred. Development expenses are capitalized prospectively following regulatory approval for commercial production of a target.

(c) Goodwill

Goodwill represents the excess of the consideration transferred over the fair value of the identifiable net assets acquired in a business combination. Goodwill is not amortized but is allocated to cash-generating units and tested for impairment annually and whenever there is an indication that the carrying amount may not be recoverable.

(d) Right to purchase

In connection with the sale of the Company's commercial manufacturing activities located in Lexington, MA (the "Lexington Transaction") to Genezen Holdings Inc. and its subsidiary Genezen MA, Inc. (together "Genezen"), which closed on July 22, 2024 (the "Closing"), the Company entered into a Commercial Supply Agreement (the "Genezen CSA") with Genezen. The Genezen CSA provides the Company with rights to purchase HEMGENIX® at terms considered favorable to market terms. In accordance with IFRS 3, *Business Combinations,* the Company recorded an intangible asset at its fair value with respect to these favorable terms at Closing. The intangible asset is stated at historical cost less accumulated amortization. The Company amortizes the intangible asset on a straight-line basis over a three-year term. The Company presents the amortization expense within Other expense in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss.

2.15 Leases

At inception of a contract, the Company assesses whether a contract is, or contains, a lease. A contract is, or contains, a lease if the contract conveys the right to control the use of an identified assets for a period of time in exchange for consideration. At commencement, the Company allocates the consideration in the contract to each lease component on the basis of its relative stand-alone prices. The Company recognizes a right-of-use asset and a lease liability at the lease commencement date.

Lease liabilities are initially measured at the present value of minimum lease payments and a right to use asset is recorded for the same amount. Lease liabilities are measured at the present value of the lease payments that are not paid at that date including:

- fixed payments less any lease incentives receivable; and,

- variable lease payments that are based on an index or a rate.

The lease payments are discounted using the interest rate implicit in the lease. If that rate cannot be determined, the Company's incremental borrowing rate at the lease commencement date is used, which is based on an assessment of the interest rate the Company would have to pay to borrow funds, including the considerations of factors such as the nature of the asset and location, collateral, market terms and conditions, as applicable. After the commencement date, the amount of lease liabilities is increased to reflect the accretion of interest (presented as finance expense in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss) and reduced for the lease payments made.

The interest element of the finance expense is determined so as to produce a constant periodic rate of interest on the remaining balance of the liability for each period during the lease term. The interest element is presented within cash flows from operating activities and the repayment of the liability is presented within cash flows from financing activities in the Consolidated Statement of Cash Flows. In addition, the carrying amount of lease liabilities is remeasured if there is a modification, a change in the lease term, a change in the in-substance fixed lease payments, or a change in future lease payments arising from a change in an index. When the lease liability is remeasured, a corresponding adjustment is made to the carrying amount of the right-of-use asset.

Right-of-use assets are measured at cost comprising the following:

- the amount of the initial measurement of lease liability;

- any lease payment made at or before the commencement date less any lease incentives received; and

- any initial direct costs.

The right-of-use assets are subsequently accounted for using principles for property, plant and equipment. Right-of-use assets are depreciated using the straight-line method from the commencement date to the end of the lease term. Depreciation expense related to right-of-use assets are presented within operating expenses.

Payments associated with short-term leases and leases of low value assets are recognized on a straight-line basis as an expense in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss. Short-term leases are leases with a term of 12 months or less. The Company determines the lease term as the non-cancellable term of the lease, together with any periods covered by an option to the extend the lease if it is reasonably certain to be exercised, or any periods covered by an option to terminate the lease, if it is reasonably certain not to be exercised. The Company applies judgement in evaluating whether it is reasonably certain to exercise an option to renew.

2.16 Financial instruments

Initial recognition and measurement

Financial assets and financial liabilities are initially recognized when the Company becomes a party to the contractual provisions of the instrument.

A financial asset (unless it is accounts receivable without a significant financing component) or a financial liability is initially measured at fair value plus, for an item not at fair value through profit or loss, transaction costs that are directly attributable to its acquisition or issue.

Financial assets

(a) Investment securities

The Company classifies its investment securities as measured at amortized cost. A financial asset is measured at amortized cost if it meets both of the following conditions:

- It is held within a business model whose objective is to hold assets and collect contractual cash flows; and,
- Its contractual terms give rise on specified dates to cash flows that are solely payments of principal and interest ("SPPI") on the principal amount outstanding.

Financial assets at amortized cost are subsequently measured at amortized cost using the effective interest method. The amortized cost is reduced by impairment losses. Interest income, foreign exchange gains and losses and impairment are recognized in profit or loss. Any gain or loss on derecognition is recognized in profit or loss.

The Company recognizes loss allowances for expected credit losses on financial assets measured at amortized cost. The Company measures loss allowances at an amount equal to lifetime expected credit losses, except for debt securities that are determined to have low credit risk at the reporting date which are measured at 12-month expected credit losses.

The Company applies a simplified approach to measurement of lifetime expected credit losses based on the probability of default of its counterparties. The probability of default is derived from applicable external credit ratings. The Company considers a debt security to have a low credit risk when its credit risk rating is equivalent to the globally understood definition of 'investment grade'.

Loss allowances for financial assets measured at amortized cost are deducted from the gross carrying amount of the assets. Loss allowances are presented within Other expense in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss, unless material.

(b) Series C preferred stock

The Company recorded the Series C preferred stock in Genezen Holdings Inc. at its fair value. The Company classified this investment as FVTPL – equity investment and subsequently measures it at fair value through profit or loss, with changes in fair value presented within Finance income or Finance expense, as appropriate, on the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss. The series C preferred stock is presented within Other investments on the Consolidated Statement of Financial Position.

(c) Convertible promissory note

The Company recorded the convertible promissory note at its fair value. The convertible promissory note is classified as FVTPL – debt investment and subsequently measured at fair value through profit or loss, with changes in fair value presented within Finance income or Finance expense, as appropriate, on the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss. The convertible promissory note is presented within Other investments on the Consolidated Statement of Financial Position.

(d) Other financial assets

All other financial assets are recognized when the Company becomes a party to the contractual provisions of the instrument.

The Company derecognizes a financial asset when the contractual rights to the cash flows from the financial asset expire.

Financial liabilities

(a) Borrowings

The Company classifies and measures borrowings initially at fair value and subsequently measures borrowings at amortized cost using the effective interest method. The Company recognizes interest expense and foreign exchange gains and losses in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss. The Company derecognizes borrowings when its contractual obligations are discharged or cancelled, expire, or its terms are modified and the cash flows of the modified liability are substantially different. The Company also recognizes any gain or loss on derecognition in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss. If the cash flows of the borrowings do not substantially differ before and after modification, then the Company continues to apply the originally effective interest rate and recognizes the difference in net present value as at modification date in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss. Any fees incurred are capitalized.

(b) Liability related to pre-funded warrants

As part of the Company's September 2025 follow-on public offering, the Company issued pre-funded warrants (the "Pre-Funded Warrants"), denominated in U.S. dollars. As the Pre-Funded Warrants are not denominated in the Company's functional currency, they are not indexed to the Company's ordinary shares in accordance with IAS 32, *Financial Instruments, Presentation*. The obligation to issue ordinary shares at an offering price of $47.50 per ordinary share, less a $0.0001 per ordinary share exercise price, is classified as a financial liability and presented as liability related to pre-funded warrants on the Consolidated Statement of Financial Position. The liability related to pre-funded warrants is measured at fair value in accordance with IFRS 9, *Financial Instruments*. Changes in the fair value of the liability related to pre-funded warrants are recognized through profit or loss within Finance income in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss. The liability related to pre-funded warrants is classified as current due to the Pre-Funded Warrant holder's right to exercise at any time. In accordance with IAS 33, *Earnings Per Share*, the Pre-Funded Warrants reflect potential ordinary shares and as such are excluded from the denominator in the computation of basic net loss per ordinary share.

(c) Other financial liabilities

The Company recognizes other financial liabilities on the trade date when the entity becomes a party to the contractual provisions of the instrument and derecognizes the financial liability when its contractual obligations are discharged or cancelled, or expire. Other financial liabilities are initially measured at fair value less any directly attributable transaction costs. Following the initial recognition, these liabilities are measured at amortized cost using the effective interest method.

2.17 Equity

An equity instrument is defined as any contract that evidences a residual interest in the assets of an entity after deducting all of its liabilities. An instrument is an equity instrument only if the issuer has an unconditional right to avoid settlement in cash or another financial asset.

Incremental costs directly attributable to the issue of new ordinary shares or options are shown in equity as a deduction, net of tax, from the proceeds.

Ordinary shares are classified as equity.

Dividend distributions to the Company's shareholders are recognized as a liability in uniQure's Consolidated Statement of Financial Position in the period in which the dividends are approved by its shareholders. To date, uniQure has not paid dividends.

2.18 Income taxes

Income tax comprises current and deferred tax. Income tax is recognized in profit or loss. Tax consequences related to items recognized in other comprehensive income are recognized in other comprehensive income as well.

Current tax comprises the expected tax payable or receivable on the taxable income or loss for the year and any adjustment to the tax payable or receivable in respect of previous years.

Deferred tax is recognized in respect of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for taxation purposes. Deferred tax is not recognized for temporary differences on the initial recognition of assets or liabilities in a transaction that is not a business combination and that affects neither accounting nor taxable profit or loss, temporary differences related to investments in subsidiaries to the extent that the Company is able to control the timing of the reversal of the temporary differences and it is probable that they will not reverse in the foreseeable future and taxable temporary differences arising on the initial recognition of goodwill.

Deferred tax assets are recognized for unused tax losses, unused tax credits and deductible temporary differences to the extent that it is probable that future taxable profits will be available against which they can be used at the individual tax filing entity level. Unrecognized deferred tax assets are reassessed at each reporting date and recognized to the extent that it has become probable that future taxable profits will be available against which they can be used at the individual tax filing entity level.

The Company's management periodically evaluates positions taken in tax returns with respect to situations in which applicable tax regulation is subject to interpretation. It establishes provisions where appropriate.

Income tax is measured using tax rates enacted or substantively enacted at the reporting date. Deferred tax is measured at the tax rates that are expected to be applied to temporary differences when they reverse.

Income tax assets and liabilities are offset when there is a legally enforceable right to offset current tax assets against current tax liabilities and when the deferred income taxes assets and liabilities relate to income taxes levied by the same taxation authority on either the taxable entity or different taxable entities where there is an intention to settle the balances on a net basis.

2.19 Employee benefits

The Company operates a defined contribution pension plan for all employees at its Amsterdam facility in the Netherlands, which is funded by the Company through payments to an insurance company. The Company has no legal or constructive obligation to pay further contributions if the plan does not hold sufficient assets to pay all employees the benefits relating to employee service in the current and prior periods. The contributions are recognized as employee benefit expense when they are due. Prepaid contributions are recognized as an asset to the extent that a cash refund or a reduction in the future payments is available.

The Company maintains a qualified 401(k) Plan for all employees located in the U.S. The 401(k) Plan offers both a pre-tax and post-tax (Roth) component. Employees may contribute up to the U.S. Internal Revenue Service ("IRS") statutory limit each calendar year. The Company matches $0.50 for every $1.00 contributed to the plan by participants, up to 6% of base compensation. Employer contributions are recognized as they are contributed, as long as the employee is rendering services in that period. If employer contributions are made in periods after an individual retires or terminates, the estimated cost is accrued during the employee's service period.

The Company maintains defined benefit plans for its Swiss employees, including retirement benefit plans required by applicable local law. The liability in respect to defined benefit pension plans is the defined benefit obligation calculated annually by independent actuaries using the projected unit credit method.

The defined benefit obligation as of December 31, 2025 and December 31, 2024 represents the actuarial present value of the estimated future payments required to settle the obligation that is attributable to employee services rendered before that date. Service cost is reported in research and development and general and administrative expenses. All other components of net period costs are reported in interest expense in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss. Plan assets are recorded at their fair value. Gains or losses arising from plan curtailments or settlements are accounted for at the time they occur. Actuarial gains and losses arising from differences between the actual and the expected return on plan assets are recognized in other reserves.

2.20 Share-based compensation

Employee share-based compensation plans

The fair value of services received in exchange for equity instruments granted is recognized as an expense, with a corresponding adjustment to Other Reserves in equity. The total amount to be expensed over the vesting period is determined by reference to the fair value of the instruments granted and based on the share price and vesting conditions. For share-based payments that do not vest until the employees have completed a specified period of service, uniQure recognizes the services received as the employees render services during the service period. For the allocation of the expenses to be recognized, the Company treats each installment of a graded vesting award as a separate share grant.

For performance share units ("PSUs) which depend on the achievement of a defined performance condition, the Company recognizes an amount for the services received during the vesting period based on the best available estimate of the number of equity instruments expected to vest. The Company will revise that estimate if subsequent information indicates that the number of equity instruments expected to vest differs from previous estimates.

Options

The fair value of options granted is determined at the grant date.

Restricted share units ("RSUs")

The fair value of RSUs granted is determined at the grant date by reference to the share-price.

Performance share units ("PSUs")

Awards of PSUs are subject to the achievement of specified performance objectives. The fair value of PSUs that have been granted is determined at the grant date by reference to share-price. The fair value of PSUs awarded and for which the service period precedes the grant date, is determined by reference to the Company's share-price as of the reporting date.

2.21 Provisions

Provisions are recognized when the Company has a present legal or constructive obligation as a result of past events, it is probable that an outflow of resources will be required to settle the obligation, and the amount can be reliably estimated.

Provisions are measured at the present value of amounts expected to be required to settle the obligation using a pre-tax rate that reflects current market assessments of the time value of money and the risks specific to the obligation. The increase in the provision due to the passage of time is recognized as interest expense.

Purchase commitment provision

The Genezen CSA includes a minimum term of three years and minimum purchase commitments of HEMGENIX® over the first three years, unless certain contractual provisions are triggered. The Company expects to resell HEMGENIX® purchased from Genezen via its minimum commitments to CSL Behring at a loss. In accordance with IAS 37, *Provisions, Contingent Liabilities and Contingent Assets,* the Company recognized an onerous contract provision to reflect the unavoidable excess cost of meeting the Company's obligations under the Genezen CSA over the economic benefits that the Company expects to receive from its customer contract with CSL Behring. The Company subsequently adjusts the provision for any changes to the losses expected to be incurred. The carrying amount of the provision increases in each period to reflect the unwind of the discount which is presented as Finance expense in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss.

2.22 Revenue recognition

The Company primarily generates revenue from its commercialization and license agreement with CSL Behring (the "CSL Behring Agreement").

(a) License revenue

In June 2020 the Company entered into a commercialization and license agreement pursuant to which CSL Behring received exclusive global rights to HEMGENIX® which became fully effective in May 2021. The Company concluded that CSL Behring is a customer in accordance with IFRS 15, *Revenue from Contracts with Customers*. The Company recognized the revenue related to its performance obligation to sell the exclusive global rights to HEMGENIX® in May 2021. The Company also allocated the following consideration to this performance obligation:

i) Variable milestone payments; and,
ii) Sales milestone payments and royalties.

The Company recognizes license revenue in relation to the regulatory and sales milestone payments when it becomes probable that these will be achieved as well as when royalties on sales of HEMGENIX® have been earned.

Refer to Note 14, "*Collaboration arrangements and concentration of credit risk*" for further detail.

(b) Contract manufacturing revenue

In the period between April 2022 and July 2024, the Company manufactured HEMGENIX® for CSL Behring in accordance with a June 2020 Development and Commercial Supply Agreement between the Company and CSL Behring (the "CSL Behring CSA"). Following the July 2024 closing (the "Closing") of the sale of our commercial manufacturing activities located in Lexington, MA to Genezen (the "Lexington Transaction"), title to HEMGENIX® drug product supply directly passes from the contract manufacturer, Genezen, to CSL Behring. The Company does not control HEMGENIX® before it is transferred to CSL Behring. The Company arranges for HEMGENIX® to be provided by Genezen to CSL Behring. The Company determined that it is an agent in the sale of HEMGENIX® to CSL Behring with related accounts receivable presented in other receivables in the consolidated balance sheets.

The Company recognized contract manufacturing revenue when ownership transferred to CSL Behring.

(c) Collaboration revenue

Collaboration revenue related to contracted services is recognized when performance obligations are satisfied.

2.23 Other income, other expense

The Company receives certain government and regional grants, which support its research efforts in defined projects, and include contributions towards the cost of research and development. These grants generally provide for reimbursement of approved costs incurred as defined in the respective grants and are deferred and recognized in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss over the period necessary to match them with the costs they are intended to compensate, when it is probable that the Company complied with any conditions attached to the grant and will receive the reimbursement.

Other income also consists of income from subleasing part of the Company's Amsterdam facility. Other expense consists of expenses incurred in relation to the subleasing income.

Other expense includes expenses related to the purchase of HEMGENIX® from Genezen, net of income from the sales of HEMGENIX® to CSL Behring, amortization of the intangible asset for the favorable supply terms under the Genezen CSA and credits from the release of provisions related to expected net losses associated with minimum purchase commitments under the Genezen CSA.

2.24 Finance income, finance expense

Finance income consists of interest income on cash and cash equivalents and investment securities, fair value changes on financial assets measured at FVPL in accordance with IFRS 9, *Financial Instruments,* gains related to the modification of the Company's borrowings, and foreign exchange gains related to financing.

Finance expense consists of interest expense related to borrowings, lease liabilities, fair value changes on financial liabilities measured at FVPL in accordance with IFRS 9, *Financial Instruments* and foreign exchange losses related to financing

3. Divestiture of commercial manufacturing activities

On June 29, 2024, affiliates of the Company agreed with Genezen to sell the Company's commercial manufacturing activities located in Lexington, MA (the "Lexington Transaction") which closed on July 22, 2024 ("Closing").

Genezen extended offers of employment to a significant majority of the Company's employees located at the Lexington facility (the "Lexington Facility"), with the remaining employees terminated effective August 30, 2024.

uniQure Inc. and uniQure B.V., both wholly owned subsidiaries of the Company, entered into an Asset Purchase Agreement ("APA") with Genezen on June 29, 2024. Pursuant to the APA, Genezen agreed to acquire the manufacturing facility including equipment and related manufacturing operations with a carrying value of $15.2 million, inventory with a carrying value of $8.8 million and certain other assets (including allocated goodwill) with a carrying value of $2.8 million associated with the Lexington Facility on Closing.

As consideration, the Company received (i) shares of newly issued Series C preferred stock of Genezen Holdings Inc. which are convertible into Genezen common stock and will accrue an 8.0% per annum cumulative dividend, (ii) a convertible promissory note with a nominal amount of $12.5 million, bearing interest at 8.0% per annum and maturing 63 months following the date of issuance (refer to Note 4, "*Financial risk management*") and (iii) a right to purchase HEMGENIX® at terms considered favorable to market terms. Refer to Note 6, "*Intangible assets*".

uniQure Inc., Genezen and the landlord of the Lexington Facility entered into an agreement for uniQure to assign and Genezen to assume the existing lease agreement between uniQure and the landlord at Closing. The Company also amended its original July 2013 guarantee to continue guaranteeing rental payments owed by Genezen until the end of the current term on May 31, 2029. In the event of Genezen's default related to rental payments owed to the landlord, the Company is entitled to terminate the assignment agreement and step into the original lease agreement. On Closing, the Company derecognized its right of use asset with a carrying amount of $8.5 million and related lease liability for the facility of $17.7 million. Following the Closing, $1.7 million of deposits were released and reclassified from restricted cash into cash.

At Closing, uniQure Inc. entered into the Genezen CSA. Pursuant to the terms of the Genezen CSA, the parties agreed to subcontract the manufacturing of HEMGENIX® to Genezen. The Genezen CSA includes a minimum term of three years and minimum purchase commitments of HEMGENIX® commercial supplies of $43.3 million over the first three years, unless certain contractual provisions are triggered. The Genezen CSA provides the Company with rights to purchase HEMGENIX® at terms considered favorable to market terms. In accordance with IFRS 3, *Business Combinations*, the Company recorded an intangible asset valued at $16.7 million with respect to these favorable terms. The intangible asset will be amortized on a straight-line basis over a three-year term, commencing at Closing. Amortization expense of $5.6 million and $2.5 million was recorded during the years ended December 31, 2025 and 2024, respectively.

The Company's obligations with respect to the supply of HEMGENIX® to CSL Behring pursuant to the CSL Behring CSA remain in effect notwithstanding the subcontracting to Genezen. The Company expects to resell HEMGENIX® material purchased from Genezen via its minimum commitments to CSL Behring at a loss. In accordance with IAS 37, *Provisions, Contingent Liabilities and Contingent Assets*, the Company, at Closing, recognized an onerous contact provision of $8.8 million related to the excess of the unavoidable cost of meeting the Company's obligations under the Genezen CSA over the economic benefits that the Company expects to receive from its customer contract with CSL Behring. The provision was accounted for as a reduction of the consideration received. During the year ended December 31, 2025 the Company recorded a decrease in the provision of $1.0 million offset by a $0.6 million increase related to the unwinding of the discount. During the year ended December 31, 2024 the Company recorded a decrease in the provision of $2.8 million offset by a $0.4 million increase related to the unwinding of the discount. As of December 31, 2025, the Company classified $5.1 million (December 31, 2024: $1.6 million) of the provision as current and $0.9 million (December 31, 2024: $4.8 million) as non-current in the Consolidated Statement of Financial Position.

The movement in the provision during the years ended December 31, 2025, and 2024, is as follows:

	Years ended December 31,	
	2025	2024
	$ in thousands	
Balance at January 1	**6,438**	—
Initial recognition (at Closing)	—	8,772
Utilization of provision (reductions)	(1,023)	(2,710)
Unwinding of discount (Finance expense)	588	376
Balance at December 31	**6,003**	**6,438**
Current portion	5,102	1,582
Non-current portion	901	4,856
Total Provision	**6,003**	**6,438**

The Lexington Transaction was accounted for as a divestment of the Company's commercial manufacturing activities. The total net consideration received of $25.4 million, less costs associated with the sale of $3.3 million, exceeded the $17.7 million fair market value of the net assets transferred (including allocated goodwill) by $4.4 million. The excess over the fair market value was recognized as a net gain in Other income in the Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss.

At Closing, the Company paid a total of $8.3 million to Genezen and CSL related to adjustments of working capital and to obtain consent to proceed with the divestiture.

Additionally, uniQure biopharma B.V. entered into a Development, Manufacturing and Supply Agreement ("DMSA") with Genezen at Closing. Pursuant to the DMSA, the Company is entitled to receive, as a preferred customer, manufacturing and development services to support the Company's investigational gene therapy programs and other services related to HEMGENIX®. The DMSA has a minimum term of three years and requires the Company to purchase services for a total minimum of $14.0 million. As of December 31, 2025, the remaining minimum purchase commitments amount to $7.3 million.

4. Financial Risk Management

4.1 Financial Risk Factors

uniQure is exposed to a variety of financial risks, including credit risk, market risk (e.g., currency risk, interest rate risk and other price risk) and liquidity risk. The Company's overall management program focuses on preservation of capital and the unpredictability of financial markets and has sought to minimize potential adverse effects on its financial performance and position.

uniQure's risk management policies are established to identify and analyze the risks faced by the Company and are reviewed regularly to reflect changes in market conditions and its activities. Financial risk management is carried out by the finance department, which identifies and evaluates financial risks and hedges these risks if deemed appropriate.

uniQure does not engage in speculative transactions, nor does it issue or hold financial instruments for trading purposes.

a) Credit Risk

Credit risk is managed on a consolidated basis. Credit risk arises from cash and cash equivalents and deposits with banks and financial institutions, as well as from outstanding receivables related to (i) license arrangements, (ii) collaboration services provided to partners, and (iii) other contractual arrangements with counterparties.

As of December 31, 2025, the Company's primary credit exposures relate to the collection of license receivables from CSL Behring, as well as advance payments made to Genezen in connection with manufacturing and critical reagent supply arrangements. In addition, the Company holds a convertible promissory note issued by Genezen with a nominal amount of $12.5 million, bearing interest at 8.0% per annum and maturing 63 months following the date of issuance. Refer to Note 3, "*Divestiture of commercial manufacturing activities"* and Note 4.3, "*Fair value measurement"*, for further information. The Company recorded prepaid expenses to Genezen in relation to the Genezen CSA and DMSA of $13.9 million and nil as of December 31, 2025 and 2024, respectively. These balances give rise to concentrations of credit risk. The Company's maximum exposure to credit risk at the reporting date is the carrying amount of these financial assets. No loss allowance for expected credit losses was recognized related to these prepayments or the convertible promissory note during the years ended December 31, 2025 or 2024.

Restricted cash includes deposits made in relation to facility leases in Amsterdam, the Netherlands and Lexington, Massachusetts, and Basel, Switzerland. The deposits are neither impaired nor past due.

The Company's cash and cash equivalents include bank balances, demand deposits and other short term highly liquid investments (with maturities of less than three months at the time of purchase) that are readily convertible into a known amount of cash and are subject to an insignificant risk of fluctuation in value. Restricted cash includes deposits made in relation to facility leases. The Company also has short-term investment securities in U.S. and European government bonds maturing within one to nine months. Refer to Note 2.16, "*Summary of significant accounting policies – Financial Instruments*". The Company's investment policy requires it to invest with counterparties with a high investment credit rating. Due to the high credit quality of its counterparties, the Company believes there is no material exposure to credit risk in its portfolio of investment securities.

Cash and cash equivalents and restricted cash were placed at the following banks and accounts receivable were receivable from the following trade customers:

	As of December 31,			
	2025		2024	
	Amount	Credit rating	Amount	Credit rating
	$ in thousands		$ in thousands	
Cash, cash equivalents and restricted cash				
Bank of America	41,192	Aa2	37,730	Aa1
Rabobank	40,021	Aa2	106,955	Aa2
BNP Paribas	376	A1	236	A1
UBS	212	Aa3	139	Aa3
Investment in debt securities	—	n/a	15,269	Aaa
Total	**81,801**		**160,329**	
Investment in debt securities				
Investment in debt securities	542,301	Aaa - Aa1	208,591	Aaa
Total	**542,301**		**208,591**	
Accounts receivable				
CSL Behring	5,863	A3	5,881	A3
Total	**5,863**		**5,881**	
Other current assets and receivables				
CSL Behring	187	A3	5,636	A3
Genezen	403	n/a	46	n/a
Total	**590**		**5,682**	
Other investments				
Genezen	15,953	n/a	13,660	n/a
Total	**15,953**		**13,660**	

Ratings are by Moody's. The credit exposure related to accounts receivable from CSL Behring is not considered material. Refer to Note 14, "*Collaboration arrangements and concentration of credit risk* ".

Investment securities with original maturities of 90 days or less when purchased are presented within cash and cash equivalents and measured at amortized cost (December 31, 2025: nil, December 31, 2024: $15.3 million).

b) Market Risk

The Company's market risks did not substantially change during the twelve months ended December 31, 2025 compared to the twelve months ended December 31, 2024.

(i) Currency risk

The Company primarily operates from Amsterdam, the Netherlands and Lexington, MA in the United States of America. The Company is exposed to foreign exchange risk arising from various currencies, primarily due to the U.S. dollar and the euro and to a lesser extent to the British pound and the Swiss franc. As the Company's U.S. operations are primarily conducted in U.S. dollar, the exposure of the Company's US entity to changes in foreign currency is insignificant. Similarly, the exposure to changes in foreign currencies of the Company's Swiss and French entities are insignificant as well.

The Company's Dutch operations hold significant amounts of U.S. dollars in cash and cash equivalents and investment securities, and has debt and interest obligations to Hercules Capital, Inc. ("Hercules") which are also U.S. dollar denominated. Refer to Note 8, "*Borrowings"*. The liability resulting from the 2023 agreement entered into between uniQure biopharma and HemB SPV, L.P. to sell certain current and future royalties due to the Company from the net sales of HEMGENIX® (the "Royalty Financing Agreement") is also denominated in U.S. dollars. Refer to Note 9, "*Royalty Financing Agreement*". The Company generates collaboration revenue denominated in U.S. dollars, receives services from vendors denominated in U.S. dollars and occasionally British pounds, and compensates the

operations of its U.S. operating entity for services received in U.S. dollars. Foreign currency denominated account receivables and account payables are short term in nature (generally 30 to 45 days).

Variations in exchange rates impact earnings and other comprehensive income or loss. On December 31, 2025, if the euro had weakened 10% against the U.S. dollar with all other variables held constant, pre-tax loss for the year would have been $0.5 million lower (December 31, 2024: pre-tax loss $23.6 million higher), and other comprehensive income would have been $1.6 million lower (December 31, 2024: other comprehensive income $11.0 million higher). Conversely, if the euro had strengthened 10% against the US dollar with all other variables held constant, pre-tax loss for the year would have been $0.5 million higher (December 31, 2024: pre-tax loss $23.6 million lower), and other comprehensive income would have been $3.1 million higher (December 31, 2024: other comprehensive income $12.1 million lower).

The Company strives to mitigate foreign exchange risk through holding sufficient funds in euro and U.S. dollars to finance budgeted cash flows for generally 18 months forward.

The sensitivity in other comprehensive income to fluctuations in exchange rates primarily relates to the translation of the net assets of the Company's Dutch entities from their functional currency euro into the Company's reporting currency, the U.S. dollar.

(ii) Interest rate risk

The Company's interest rate risk arises from short- and long-term debt, investment securities and cash on hand.

In June 2013, the Company entered into a venture debt loan facility with Hercules, which was last amended in September 2025, and under which the Company's borrowings bear interest at a variable rate with a fixed floor. Long-term debt issued at fixed rates expose the Company to fair value interest rate risk. As of December 31, 2025, the loan bore a nominal interest rate of 9.45%.

On December 31, 2025 if interest rates on borrowings had been 1.0% higher with all other variables held constant, pre-tax results for the year would have been $0.5 million lower (December 31, 2024: $0.8 million lower). This is partially offset by interest income the Company generates from investments in government bonds and cash on hand.

The Company invests in government debt in accordance with its investment policy. The Company is exposed to interest rate risk as market interest rates could differ from the interest rates that it fixes at the time of acquiring these investment securities. As the Company intends to hold these to maturity, it does not recognize changes in the fair value of its investment which are caused by changes in market interest rates, and as result, a change in prevailing interest rates may cause the fair value of the investment to fluctuate. For example, if the Company holds a security that was issued at a fixed interest rate at the then-prevailing rate and the prevailing interest rate later rises, the fair value of the investment will probably decline.

The contractual maturities of the Company's investment securities held as of December 31, 2025 ranged between one and nine months. Due to the relatively short-term nature of these financial instruments and the Company's ability and intention to hold these investments to maturity, the Company believes there is no material exposure to interest rate risk.

(iii) Other price risk

uniQure is not exposed to significant price risk.

c) Liquidity risk

Liquidity risk is the risk that the Company will encounter difficulty in meeting the obligations associated with its financial liabilities that are settled by delivering cash or another financial asset. The Company's approach to managing liquidity is to ensure, as far as possible, that it will have sufficient liquidity to meet its liabilities when they are due, under both normal and stressed conditions, without incurring unacceptable losses or risking damage to the Company's reputation.

The table below analyzes our financial liabilities in relevant maturity groupings based on the length of time until the contractual maturity date, as of the balance sheet dates. Disclosed in the table below are the contractual undiscounted cash flows. Balances due within 12 months equal their carrying value balances as the impact of discounting is not significant.

	Undefined	Less than 1 year	Between 1 - 3 years	Between 3 - 5 years	Over 5 years
			$ in thousands		
At December 31, 2025					
Borrowings (including interest payments)	—	4,791	15,659	51,472	—
Accounts payable, accrued expenses and other current liabilities	—	41,360	—	—	—
Commitments related to uniQure France Acquisition (maximum nominal amount)[(1)]	188,000	—	—	—	—
Total	**188,000**	**46,151**	**15,659**	**51,472**	**—**
At December 31, 2024					
Borrowings (including interest payments)	—	8,616	57,403	—	—
Accounts payable, accrued expenses and other current liabilities	—	34,870	—	—	—
Commitments related to uniQure France Acquisition (maximum nominal amount)[(2)]	166,195	—	—	—	—
Total	**166,195**	**43,486**	**57,403**	**—**	**—**

(1) Payments are due in EUR and have been translated at the foreign exchange rate as of December 31, 2025, of $1.18 / €1.00

(2) Payments are due in EUR and have been translated at the foreign exchange rate as of December 31, 2024, of $1.04 / €1.00

In relation to the uniQure France Acquisition, the Company entered into commitments to make payments to the former shareholders upon the achievement of certain contractual milestones. The commitments include payments related to post-acquisition services that the Company agreed to as part of the transaction. The timing of achieving these milestones, as well as whether the milestone will be achieved at all, and consequently the timing of payments is generally uncertain. The Company expects these obligations will become payable between 2028 and 2033. If and when due, up to 25% of the milestone payments can be settled with the Company's ordinary shares.

4.2 Capital risk management

The Company's objectives in managing capital are to safeguard uniQure's ability to continue as a going concern and to minimize the cost of capital to provide returns for shareholders and benefits for other stakeholders.

The Company has no firm sources of additional financing. Until such time, if ever, that uniQure can generate substantial cash flows from successfully commercializing its proprietary product candidates, uniQure expects to finance its cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution and licensing arrangements.

In September 2025, the Company entered into a $175.0 million senior secured term loan facility (the "2025 Amended Facility") with Hercules. The 2025 Amended Facility consists of three tranches including a first tranche of $50.0 million replacing the debt outstanding as per the loan amendment date, an additional term loan tranche of $100.0 million, which can be drawn at its option, subject to the Biologics License Application ("BLA") approval of AMT-130 prior to June 2027, provided that confirmatory trials to the extent and in the manner required to support full approval (if applicable) remain ongoing or are being planned, and a third tranche of up to $25.0 million, subject to Hercules' approval. All tranches have a floating interest rate of the greater of 9.45% and the prime rate plus 2.45%,

reflecting a floating rate of 9.45% as of December 31, 2025. The tranches mature on October 1, 2030. The Company is subject to covenants under the 2025 Amended Facility, and may become subject to covenants under any future indebtedness that could limit the Company's ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, which could adversely impact the Company's ability to conduct its business. In addition, its pledge of assets as collateral to secure its obligations under the 2025 Amended Facility may limit the Company's ability to obtain debt financing.

If financing is not available when needed, including through debt financings or equity offerings, or is available only on unfavorable terms, the Company may be unable to meet its cash needs. If the Company raises additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, uniQure may have to relinquish valuable rights to its technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to it. If uniQure is unable to raise additional funds through equity or debt financings when needed, uniQure may be required to delay, limit, reduce or terminate its product development or future commercialization efforts or grant rights to develop and market product candidates that the Company would otherwise prefer to develop and market itself, which could have a material adverse effect on its business, financial conditions, results of operations and cash flows.

The amount of total shareholders' equity as recorded in the Consolidated Statement of Financial Position is managed as capital by the Company.

4.3 Fair value measurement

Financial instruments measured at fair value are categorized as follows:

- Level 1: quoted prices (unadjusted) in active markets for identical assets or liabilities.
- Level 2: inputs other than quoted prices included in Level 1 that are observable for the asset or liability, either directly (i.e., as prices) or indirectly (i.e., derived from prices).
- Level 3: inputs for the asset or liability that are not based on observable market data (unobservable inputs).

The carrying amounts of financial assets and financial liabilities, measured at amortized cost, are a reasonable approximation of their fair value and therefore information about the fair values of each class is not disclosed.

Financial assets measured at fair value using Level 2 and 3 inputs as of December 31, 2025 and December 31, 2024 consisted of the Company's investment in Series C preferred stock of Genezen Holdings Inc. and a convertible promissory note.

Liabilities measured at fair value using Level 2 inputs as of December 31, 2025 consisted of the pre-funded warrants, given that they are not exchange-quoted and are measured using a valuation technique whose significant inputs are observable. Liabilities measured at fair value using Level 3 inputs as of December 31, 2025 and December 31, 2024 consisted of contingent consideration.

a) Other investments

Convertible promissory note

As of December 31, 2025, the Company holds an investment in a convertible promissory note with a fair value of $16.0 million (December 31, 2024: $13.7 million). The convertible promissory note was issued in July 2024 and matures in October 2029. The convertible promissory note was recognized at its fair value of $13.3 million upon Closing of the Lexington Transaction. Refer to Note 3, "*Divestiture of commercial manufacturing activities*". The fair value of the investment as of December 31, 2025 and 2024 was determined using Level 3 inputs and is presented within Other investments in the Company's Consolidated Statement of Financial Position. In the years ended December 31, 2025 and 2024, the Company recognized fair value gains of $0.5 million and $0.4 million, related to the convertible promissory note, respectively.

The fair value of the investment in the convertible promissory note is measured using an option pricing model based on the Black-Scholes framework. The unobservable inputs that are utilized in the calculation of the fair value of the convertible promissory note include the underlying common stock price, expected volatility, and the discount

rate. As of December 31, 2025, the quantitative unobservable inputs utilized to develop the valuation range for the convertible promissory note include an expected volatility ranging from 80.0% to 85.0%, and a discount rate of 12.4%. The recognized fair value represents the midpoint of this calculated valuation range.

Series C preferred stock

As of December 31, 2025, the Company holds an investment in Series C preferred stock in Genezen Holdings Inc. with a fair value of $14.4 million (December 31, 2024: $12.5 million). The Company recorded the Series C preferred stock at Closing at its fair market value of $12.5 million. The Series C preferred stock is presented within Other investments in the Company's Consolidated Statement of Financial Position. In the years ended December 31, 2025 and 2024, the Company recognized fair value gains of $1.1 million and nil, related to the Series C preferred stock, respectively.

The fair value of the investment in the series C preferred stock was determined using an option pricing model, utilizing an as-converted plus risky put framework to capture the minimum contractual payment subject to credit risk. The unobservable inputs utilized for the calculation of the fair value of the Series C preferred stock include the underlying enterprise value, expected volatility, and the discount rate. As of December 31, 2025, the quantitative unobservable inputs utilized to develop the valuation range for the Series C preferred stock include an expected volatility ranging from 75.0% to 80.0%, a discount rate ranging from 14.7% to 17.7%, and an estimated time to liquidity of 5.0 years. The recognized fair value represents the midpoint of this calculated valuation range.

The movement in the fair value of Other investments during the years ended December 31, 2025, and 2024, is as follows:

	Other Investments [1]	Convertible Note	Total
		$ in thousands	
Fair value at January 1, 2024	**2,179**	**—**	**2,179**
Amount recognized on Closing of Lexington Transaction	12,500	13,253	25,753
Fair value gains and other adjustments in profit or loss	—	407	407
Currency translation effects	(128)	—	(128)
Fair value at December 31, 2024	**14,551**	**13,660**	**28,211**
Fair value gains and other adjustments in profit or loss	1,052	480	1,532
Currency translation effects	1,109	1,813	2,922
Fair value at December 31, 2025	**16,712**	**15,953**	**32,665**

[1] As of December 31, 2025 and 2024, the other investments balance includes Series C preferred stock and other investments.

b) Contingent consideration

The Company is required to pay up to EUR 143.1 million ($168.1 million at the December 31, 2025 foreign exchange rate) to the former shareholders of uniQure France SAS (formerly Corlieve Therapeutics SAS) upon the achievement of the remaining contractually defined milestones in connection with the Company's acquisition of uniQure France in 2021.

The fair value of the contingent consideration liability related to this acquisition was $18.7 million as of December 31, 2025, using a discount rate of 11.8%, and $10.9 million as of December 31, 2024, using discount rates ranging from 15.3% to 16.2%.

In December 2024, a milestone payment of EUR 30.0 million ($31.5 million) was paid, of which EUR 26.8 million ($28.2 million) related to contingent consideration. No milestones were achieved or paid during the year ended December 31, 2025.

If, as of December 31, 2025, the Company had assumed a 100% likelihood of AMT-260 advancing into a Phase III clinical study, then the fair value of the contingent consideration would have increased to EUR 51.4 million ($60.4 million). If, as of December 31, 2025 the Company had assumed that it would discontinue development of the AMT-260 program, then the contingent consideration would have been released to income.

As of December 31, 2025, the Company classified none of the total contingent consideration of $18.7 million as current liabilities. The balance sheet classification between current and non-current liabilities is based upon the Company's best estimate of the timing of settlement of the remaining relevant milestones.

The movement in the fair value of the contingent consideration liability during the years ended December 31, 2025, and 2024, is as follows:

	Contingent Consideration
	$ in thousands
Fair value at January 1, 2024	**43,006**
Contingent consideration milestone payment	(28,167)
Currency translation effects	(2,162)
Fair value gain	(1,817)
Fair value at December 31, 2024	**10,860**
Fair value loss	6,247
Contingent consideration milestone payment	—
Currency translation effects	1,629
Fair value at December 31, 2025	**18,736**

c) Liability related to pre-funded warrants

As of December 31, 2025, there were 526,316 Pre-Funded Warrants outstanding to purchase the Company's ordinary shares at the public offering price of $47.50 per ordinary share, less a $0.0001 per ordinary share exercise price for each Pre-Funded Warrant.

The Pre-Funded Warrants are not exchange-quoted and are measured using a valuation technique whose significant inputs are observable, being the share price of the Company's exchange quoted shares; therefore, the fair value measurement is classified within Level 2 of the fair value hierarchy. During the year ended December 31, 2025 no warrants were exercised.

	Liability related to pre-funded warrants
	$ in thousands
Fair value at December 31, 2024	**—**
Issuance of pre-funded warrants in connection with the September 2025 follow-on public offering	25,000
Fair value gain on financial liability at FVPTL	(12,405)
Fair value at December 31, 2025	**12,595**

d) Investment securities

The following table summarizes the Company's investments in sovereign debt as of December 31, 2025 and 2024:

	December 31,			
	2025		2024	
	Carrying Amount	Fair Value	Carrying Amount	Fair Value
	$ in thousands			
Current investments:				
Government bonds - at amortized cost	542,301	542,609	208,591	208,577
	542,301	**542,609**	**208,591**	**208,577**

The Company invests in short-term U.S. and European government debt securities with a high investment credit rating. The U.S. and European government bonds are U.S. dollar and euro denominated, respectively.

As of December 31, 2025, investments in government debt securities measured at amortized cost have remaining maturities between one and nine months

Inputs to the fair value of the investments are considered Level 2 inputs.

e) Pension plan assets

Refer to Note 10, "*Retirement benefits"* for the fair value of the plan assets as of December 31, 2025.

5. Property, plant and equipment

	Leasehold improvements	Laboratory equipment	Office equipment	Construction in-progress	Total
			$ in thousands		
Cost	46,512	43,657	6,383	5,668	102,220
Accumulated depreciation	(25,606)	(25,897)	(4,169)	—	(55,672)
Carrying amount January 1, 2024	**20,906**	**17,760**	**2,214**	**5,668**	**46,548**
Additions	—	—	—	2,736	2,736
Reclassifications	911	3,867	794	(5,572)	—
Disposals - cost	(21,142)	(22,732)	(3,077)	(2,809)	(49,760)
Disposals - accumulated depreciation	14,787	15,302	2,330	—	32,419
Depreciation expense	(4,633)	(4,855)	(770)	—	(10,258)
Currency translation effects	(605)	(590)	(43)	(23)	(1,261)
Carrying amount December 31, 2024	**10,224**	**8,752**	**1,448**	**—**	**20,424**
Cost	24,808	23,359	3,910	—	52,077
Accumulated depreciation	(14,584)	(14,607)	(2,462)	—	(31,653)
Carrying amount December 31, 2024	**10,224**	**8,752**	**1,448**	**—**	**20,424**
Additions	—	—	—	340	340
Reclassifications	243	66	30	(340)	—
Disposals - cost	—	(1,441)	(6)	—	(1,447)
Disposals - accumulated depreciation	—	1,442	5	—	1,447
Depreciation expense	(4,323)	(4,252)	(486)	—	(9,060)
Currency translation effects	1,000	965	131	—	2,097
Carrying amount December 31, 2025	**7,144**	**5,533**	**1,123**	**—**	**13,800**
Cost	28,188	24,988	4,357	—	57,534
Accumulated depreciation	(21,044)	(19,455)	(3,235)	—	(43,734)
Carrying amount December 31, 2025	**7,144**	**5,533**	**1,123**	**—**	**13,800**

Total depreciation expense was $9.1 million for the year ended December 31, 2025 (December 31, 2024: $10.3 million). Depreciation expense is allocated to research and development expenses and cost of contract manufacturing to the extent it related to the Company's commercial manufacturing facility and equipment and laboratory equipment. All other depreciation expenses are allocated to selling, general and administrative expense.

In July 2024, the Company sold property, plant and equipment with a carrying value of $17.4 million, including leasehold improvements with a carrying value of $6.4 million, laboratory and office equipment with a carrying value of $8.2 million as well as assets under construction with a carrying value of $2.8 million as part of the Lexington Transaction (refer to Note 3, "*Divestiture of commercial manufacturing activities"*).

The carrying amount of property, plant and equipment by location is set out below:

	December 31, 2025	December 31, 2024
	$ in thousands	
Dutch sites	13,154	19,463
Lexington (United States of America)	461	730
Other	185	231
Carrying amount	**13,800**	**20,424**

6. Intangible assets and goodwill

	Acquired licenses	Acquired IPR&D	Favorable Supply Terms[1]	Goodwill	Total
			$ in thousands		
Cost	2,761	65,628	—	26,379	94,768
Accumulated amortization	(1,399)	—	—	—	(1,399)
Carrying amount January 1, 2024	**1,362**	**65,628**	**—**	**26,379**	**93,369**
Additions (see Note 3, "*Divestiture of commercial manufacturing activities*")	—	—	16,700	—	16,700
Disposal (see Note 3, "*Divestiture of commercial manufacturing activities*")	—	—	—	(2,500)	(2,500)
Amortization expense	(127)	—	(2,454)	—	(2,581)
Currency translation effects	(76)	(3,481)	—	(1,465)	(5,022)
Carrying amount December 31, 2024	**1,159**	**62,147**	**14,246**	**22,414**	**99,966**
Cost	2,276	62,147	16,700	22,414	103,537
Accumulated amortization	(1,117)	—	(2,454)	—	(3,571)
Carrying amount December 31, 2024	**1,159**	**62,147**	**14,246**	**22,414**	**99,966**
Additions	1,500	—	—	—	1,500
Amortization expense	(137)	—	(5,567)	—	(5,704)
Currency translation effects	152	7,299	—	2,941	10,392
Carrying amount December 31, 2025	**2,674**	**69,446**	**8,679**	**25,355**	**106,154**
Cost	3,928	69,446	16,700	25,355	115,429
Accumulated amortization	(1,254)	—	(8,021)	—	(9,275)
Carrying amount December 31, 2025	**2,674**	**69,446**	**8,679**	**25,355**	**106,154**

(1) Relates to the favorable supply terms under the Genezen CSA.

a. Acquired licenses

Intangible assets are owned by uniQure biopharma B.V and uniQure France SAS, both of which are wholly owned subsidiaries of the Company.

b. Acquired in-process research and development

In 2021, the Company identified certain intangible assets related to an IPR&D Intangible Asset as part of the uniQure France Acquisition, which had a carrying value of EUR 53.6 million ($62.9 million) as of December 31, 2025 and EUR 53.6 million ($55.6 million) as of December 31, 2024. As of December 31, 2025 and December 31, 2024, the Company also held a $6.5 million IPR&D Intangible Asset acquired as part of the global licensing agreement with Apic Bio.

c. Goodwill

Goodwill results from business combinations. The Company identified a single cash-generating unit ("CGU"). In 2025, the Company estimated the recoverable amount of the single CGU to which the carrying amount of the goodwill, as well as the carrying amounts of the IPR&D Intangible Assets, are allocated. The estimated recoverable amount of the single CGU is based on the fair value, less cost to sell, determined using an observable market price. The estimated recoverable amount exceeded its carrying amount.

7. Accrued expenses and other current liabilities

Accrued expenses and other current liabilities include the following items:

	December 31, 2025	December 31, 2024
	$ in thousands	
Accruals for services provided by vendors-not yet billed	20,490	10,109
Personnel related accruals and liabilities	10,177	12,583
Accrued royalties payable (see Note 9, "*Royalty financing agreement*")	5,523	4,951
Total	**36,190**	**27,643**

8. Borrowings

On June 14, 2013, the Company entered into a venture debt loan facility with Hercules. The facility has been amended at various times, including on July 22, 2024 (the "2024 Amended Facility") and most recently on September 22, 2025 with respect to the 2025 Amended Facility, upon which date the amendment closed (the "Amendment Closing Date").

On the Amendment Closing Date, the Company drew down $50.0 million of the 2025 Amended Facility to repay the $50.0 million then outstanding under the 2024 Amended Facility. A facility charge of $0.2 million was incurred on the Amendment Closing Date. The 2024 Amended Facility was due on January 7, 2027 and bore interest at a rate equal to the greater of (i) 7.95% and (ii) 7.95% plus the prime rate less 3.25% per annum, reflecting a floating rate of 11.95% at the Amendment Closing Date.

Pursuant to the 2025 Amended Facility, additional term loans of $125.0 million were made available. The Company may draw down (i) an additional $100.0 million in two increments of at least $25.0 million, following the approval of a BLA for AMT-130 prior to June 15, 2027, provided that confirmatory trials to the extent and in the manner required to support full approval (if applicable) remain ongoing or are being planned, and (ii) an additional $25.0 million in a single advance, subject to approval by Hercules. Advances under the 2025 Amended Facility bear interest at a rate equal to the greater of (i) 9.45% and (ii) the sum of the prime rate and 2.45%, reflecting a floating rate of 9.45% as of December 31, 2025.

The 2025 Amended Facility matures on October 1, 2030, upon which date the Company is required to repay the principal balance and any unpaid interest on advances received. The interest-only period of the 2025 Amended Facility is effective through October 1, 2028. The interest-only period will be extended to October 1, 2029 if the BLA for AMT-130 is approved prior to June 15, 2027. The 2025 Amended Facility will be interest-only if certain commercial milestones are met prior to December 31, 2028.

The Company recognized a $4.9 million gain on modification of the 2024 Amended Facility in the year ended December 31, 2025, reflecting the difference between the amortized cost as of September 22, 2025 of the original liability and the present value of the revised cash flows, discounted at the original effective interest rate of 15.2% established in 2025 in accordance with IFRS 9, *Financial Instruments*. The Company recognized no such gain in 2024.

The Company may prepay all or part of the outstanding principal together with a prepayment charge of 1.50% if repayment occurs within the first 30 months following the Amendment Closing Date, and thereafter, with a prepayment charge of 0.75%. An end-of-term charge of 2.75% of the aggregate principal outstanding is due if repaid within 18 months of the Amendment Closing Date, otherwise 5.50% if repaid thereafter.

The total principal outstanding as of December 31, 2025 under the 2025 Amended Facility was $50.0 million. The amortized cost, including interest due presented as part of Accrued expenses and other current liabilities, was $44.9 million as of December 31, 2025, compared to $51.0 million as of December 31, 2024. The foreign currency gain on the loan was $9.3 million in 2025 (2024: loss of $4.1 million). The fair value of the loan approximates its carrying amount. Inputs to the fair value of the loan are considered Level 3 inputs.

The movement in the amortized cost of the borrowing during the years ended December 31, 2025, and 2024, is as follows:

	Borrowings
	$ in thousands
At January 1, 2024	**99,746**
Repayment of borrowings and back-end fee	(53,050)
Loss on repayment of borrowings	2,845
Non-cash changes recognized in profit or loss	1,484
At December 31, 2024 (includes $0.5 million presented as 'Accrued expenses and other current liabilities')	**51,025**
Modification gain	(4,896)
Payment of back-end fee	(2,625)
Non-cash changes recognized in profit or loss	1,381
At December 31, 2025 (includes $0.4 million presented as 'Accrued expenses and other current liabilities')	**44,885**

Interest expense recorded during the years ended December 31, 2025 and 2024 was as follows:

Years	Amount
	$ in thousands
2025	7,433
2024	15,185

Under the 2025 Amended Facility, the Company must remain current in its periodic reporting requirements. Unless the Company maintains a minimum market capitalization threshold of at least $1,200.0 million, the Company, beginning January 1, 2027, is required at all times to keep a minimum cash balance deposited in bank accounts in the U.S. of at least the lesser of (i) 65% of the outstanding balance of aggregate principal due, provided that such percentage will be reduced to 50% upon the occurrence of the approval of a BLA for AMT-130 prior to June 15, 2027, and further reduced to 35% upon the occurrence of the contractually defined commercial milestones or (ii) 100% of worldwide cash and cash equivalents. Should the second tranche of the 2025 Amended Facility be drawn, a certain performance covenant related to AMT-130 net product revenue would apply nine months past a potential BLA approval of AMT-130, unless the Company maintains a minimum market capitalization threshold of at least $1,200.0 million. The 2025 Amended Facility restricts the Company's ability, among other things, to incur future indebtedness and obtain additional debt financing, make investments in securities or in other companies, transfer assets, perform certain corporate changes, own or hold certain digital assets, make loans to employees, officers, and directors, and make dividend payments and other distributions to its shareholders. The 2025 Amended Facility is secured by a direct or indirect pledge of the Company's total assets of $835.2 million, less $35.5 million of cash and cash equivalents and other current assets held by the Company, and $88.8 million of other current assets and investments held by uniQure France SAS as well as receivables sold to HemB SPV, L.P. as described in Note 9, "*Royalty Financing Agreement*". In accordance with the 2025 Amended Facility, the Company was in compliance with all applicable covenants as of December 31, 2025.

9. Royalty Financing Agreement

In May 2023, we entered into a royalty purchase agreement (the "Royalty Financing Agreement") with HemB SPV, L.P. (the "Purchaser"). Under the terms of the Royalty Financing Agreement, the Company received an upfront payment of $375.0 million in exchange for its rights to the lowest royalty tier on CSL Behring's worldwide net sales of HEMGENIX® for certain current and future royalties due to the Company. The Company will be obligated to pay $25.0 million of the first worldwide sales milestone payment from CSL Behring, if received, to HemB SPV, L.P. (the "Purchaser").

The Purchaser will receive 1.85 times the upfront payment (or $693.8 million) until June 30, 2032 ("First Hard Cap Date") if such thresholds are met or, if such cap is not met by June 30, 2032, up to 2.25 times of the upfront payment through December 31, 2038 ("Second Hard Cap Date"). If, on or prior to the defined dates for each cap amount, the total amount of royalty payments received by the Purchaser equals or exceeds the cap amount applicable to such date, the Royalty Financing Agreement will automatically terminate and all rights to the HEMGENIX® royalty payments will revert back to the Company. The Company has no obligation to repay any amounts received from the

Purchaser in the event that the applicable cap amount is not reached during the term of the Royalty Financing Agreement.

The Company has retained the rights to all other royalties, as well as commercial milestones totaling up to $1.3 billion, under the terms of the CSL Behring Agreement.

Net proceeds from the Royalty Financing Agreement, after deducting professional and financial advisory fees related to the transaction of $4.9 million, were $370.1 million. The Company initially recorded these net proceeds as "Liability from royalty financing agreement" at their fair market value on its Consolidated Statement of Financial Position as of closing of the transaction on June 5, 2023. Following the initial recognition, the Company records the debt at amortized cost.

As of December 31, 2025 the Company expects to satisfy its commitment to the Purchaser prior to the Second Hard Cap Date. The Company will record the difference of $473.7 million between the total expected payments of $843.8 million to the Purchaser and the $370.1 million net proceeds as interest expense using the effective interest rate method. The Company determined the effective interest rate based on the projected cash flows up to the Second Hard Cap Date.

At December 31, 2025 and 2024, the Company adjusted the amortized cost of the financial liability to reflect revised estimated future cash flows. The Company recalculated the amortized cost of the financial liability as the present value of the revised estimated future cash flows, determined at December 31, 2025 and 2024, discounted at the original effective interest rate of 12.2%. The reduction of the financial liability of $29.1 million as of December 31, 2025 was recognized in profit or loss as interest expense (December 31, 2024: $ 6.7 million).

The following table presents the movement in the liability related to the Royalty Financing Agreement during the years ended December 31, 2025 and 2024:

	Amount of liability $ in thousands
Carrying amount of liability at December 31, 2023 (includes $1.4 million presented as 'Accrued expenses and other current liabilities')	**398,923**
Royalty payments to Purchaser	(6,662)
Interest expense for the year ended December 31, 2024	44,186
Carrying amount of liability at December 31, 2024 (includes $5.0 million presented as 'Accrued expenses and other current liabilities')	**436,447**
Royalty payments to Purchaser	(15,258)
Interest expense for the year ended December 31, 2025	25,040
Carrying amount of liability at December 31, 2025 (includes $5.5 million presented as 'Accrued expenses and other current liabilities')	**446,229**

10. Retirement Benefits

The Company operates a defined benefit pension plan for its Swiss employees (the "Swiss Plan") in accordance with local regulations and practices. The normal retirement age under the Swiss Plan is 65. All benefits are immediately vested. Under the Swiss Plan, a percentage of pensionable salary is contributed as a retirement credit with additional contributions being made for death and disability benefits. Under Swiss pension law, participants who are covered by the pension plan of another employer are required to transfer the termination benefit of that pension plan into the plan of the Company. When employment at the Company ends before reaching retirement, the termination benefit is transferred out of the defined benefit pension plan. At time of retirement the accumulated retirement credit can be converted into a life-long annuity or be paid-out as a lump-sum. Participants are also permitted to withdraw a part of the accumulated termination benefit in special circumstances before reaching retirement age for example for payments related to obtain home ownership.

A summary of the changes in the defined benefit obligation ("DBO") and fair value of plan assets is presented below:

	December 31, 2025	December 31, 2024
	$ in thousands	
Beginning defined benefit obligation	11,495	11,499
Service cost	674	551
Interest cost	123	167
Employee contributions	633	643
Actuarial losses	(600)	509
Liabilities assumed from participants joining and leaving the plan, net	1,105	(984)
Foreign currency exchange rate changes	1,725	(890)
Defined benefit obligation	**15,157**	**11,495**

	December 31, 2025	December 31, 2024
	$ in thousands	
Beginning fair value of plan assets	9,500	8,946
Return on plan assets	125	786
Interest income	104	131
Contributions by the employer	633	643
Contributions by the employee	633	643
Assets distributed to participants leaving the plan, net	1,105	(984)
Foreign currency exchange rate changes	1,473	(665)
Fair value of plan assets	**13,573**	**9,500**

The funded status of the Swiss Plan as of December 31, 2025 and 2024, respectively, is as follows:

	December 31, 2025	December 31, 2024
	$ in thousands	
Fair value of plan assets	13,574	9,500
Present value of defined benefit obligation	(15,157)	(11,495)
Funded status: (net liability)	**(1,583)**	**(1,995)**
Accumulated benefit obligation as at December 31	**14,870**	**11,075**

Amounts recorded in accumulated other comprehensive (income):

	December 31, 2025	December 31, 2024
	$ in thousands	
Actuarial loss DBO – Financial	(857)	436
Actuarial (gain) DBO – Experience	318	(33)
Return on plan assets excluding interest income	(19)	(786)
Total	**(558)**	**(383)**

Net pension expense related to the defined benefit plan includes the following components:

	December 31, 2025	December 31, 2024
	$ in thousands	
Service cost	674	551
Interest cost	123	167
Interest income	(103)	(131)
Net pension expense	**695**	**587**

The assumptions related to the Swiss Plan are as follows:

Actuarial assumptions (% p.a.)	December 31, 2025	December 31, 2024
Discount rate	1.30%	0.90%
Expected inflation rate	0.70%	0.70%
Interest credit rate	1.25%	1.25%
Long-term expected rate of salary increases	0.70%	0.70%
Pension increase	0.00%	0.00%

The Company expects to pay $0.6 million in contributions to the defined benefit plan in 2026.

At December 31, 2025 the weighted average duration of the DBO was 14.7 years (December 31, 2024: 15.9 years).

The Company's investment strategy for its pension plan is to optimize the long-term investment return on plan assets in relation to the liability structure to maintain an acceptable level of risk while minimizing the cost of providing pension benefits and maintaining adequate funding levels in accordance with the applicable rules in each jurisdiction. The Company does not manage any assets internally. The plan assets relate to assets being held by the Swiss pension foundations in which the Company's pension plan is set-up.

The allocation of plan assets is presented below:

	December 31, 2025	December 31, 2024
Bonds	61.00%	60.50%
Equities	26.00%	25.60%
Real estate	10.00%	10.30%
Others	3.00%	3.60%
Total	**100%**	**100%**

Reasonably possible changes at the reporting date to each one of the relevant actuarial assumptions, holding other assumptions constant, would have affected the defined benefit obligation by the amounts shown below:

	December 31, 2025	
	Increase	Decrease
	$ in thousands	
Discount rate (0.5% movement)	(1,120)	1,266
Future salary growth (0.5% movement)	220	(211)
Future pension growth (0.5% movement)	641	—

	December 31, 2024	
	Increase	Decrease
	$ in thousands	
Discount rate (0.5% movement)	(921)	1,049
Future salary growth (0.5% movement)	322	(309)
Future pension growth (0.5% movement)	497	—

11. Total shareholders' equity

As of December 31, 2025, the Company's reserve for a currency translation adjustment was a loss of $61.6 million (December 31, 2024: loss of $53.8 million) as a result of $7.0 million presented in the Consolidated Statement of Profit or Loss and Other Comprehensive Income as Other comprehensive loss (2024: $0.2 million Other comprehensive loss). The reserve for the currency translation adjustment is reflected in the Company's equity, under Other Reserves.

As of December 31, 2025, the Company's authorized share capital is €4.0 million (or $4.7 million when translated at an exchange rate as of December 31, 2025, of $1.175 / €1.00), divided into 80,000,000 ordinary shares, each with a nominal value of €0.05.

All ordinary shares issued by the Company were fully paid. Under the Company's Articles of Association, the Company is required to maintain a minimum amount of share capital in reserve. In addition, the Company is only permitted to make distributions on its ordinary shares to the extent that its shareholders' equity exceeds the sum of the paid-up and called-up capital and the reserves it is required to maintain under Dutch law. There are no other distribution restrictions applicable to the Company's shares.

12. Share-based compensation

Share-based compensation expense recognized by classification included in the Consolidated Statement of Profit or Loss and Other Comprehensive Profit or Loss was as follows:

	Years ended December 31,	
	2025	2024
	$ in thousands	
Cost of manufacturing services revenue	—	1,033
Research and development	6,608	3,154
Selling, general and administrative	9,432	10,060
Total	**16,040**	**14,247**

Share-based compensation expense recognized by award type was as follows:

	Years ended December 31,	
	2025	2024
Award type	$ in thousands	
Share options	5,942	5,833
Restricted share units	9,293	7,824
Performance share units	776	579
Employee share purchase plan	29	11
Total	**16,040**	**14,247**

The Company satisfies the exercise of share options and vesting of RSUs and PSUs through newly issued ordinary shares.

The Company's share-based compensation plans include the amended and restated 2014 Share Incentive Plan (as amended, the "2014 Plan") and inducement grants under Rule 5653(c)(4) of the Nasdaq Global Select Market with terms similar to the 2014 Plan (together the "2014 Plans"). At the annual general meeting of shareholders in June 2025, the Company's shareholders authorized an additional 2,400,000 shares for issuance under the 2014 Plan. As of December 31, 2025, a total of 4,285,461 ordinary shares remain available for issuance under the 2014 Plan.

Share options

Share options are priced on the date of grant and, except for certain grants made to non-executive directors, vest over a period of four years. The first 25% vests after one year from the initial grant date and the remainder vests in equal quarterly installments over years two, three and four. Certain grants to non-executive directors vest in full after one year. Any options that vest must be exercised by the tenth anniversary of the initial grant date.

2014 Plan

The following table summarizes option activity under the Company's 2014 Plan for the years ended December 31, 2025 and 2024:

	Options		
	Number of ordinary shares	Weighted average exercise price	Weighted average remaining contractual life in years
Outstanding at January 1, 2024	**4,974,030**	**$ 23.25**	**6.71**
Granted	1,093,080	$ 5.47	
Forfeited	(316,056)	$ 16.69	
Expired	(613,996)	$ 25.04	
Exercised	(169,898)	$ 13.39	
Outstanding at January 1, 2025	**4,967,160**	**$ 19.87**	**5.91**
Granted	1,220,410	$ 12.63	
Forfeited	(126,365)	$ 13.11	
Expired	(834,335)	$ 29.87	
Exercised	(694,879)	$ 16.06	
Outstanding at December 31, 2025	**4,531,991**	**$ 17.04**	**7.16**
Fully vested and exercisable at December 31, 2025	2,532,676	$ 21.17	5.98
Outstanding and expected to vest after December 31, 2025	1,999,315	$ 11.81	8.67
Outstanding and expected to vest after December 31, 2024	1,838,603	$ 11.46	8.34
Total weighted average grant date fair value of options issued during 2025 (in $ millions)		$ 9.4	
Granted to directors and officers during 2025 (options, grant date fair value $ in millions)	532,410	$ 3.8	

The weighted-average share price of options exercised during the year ended December 31, 2025 at the date of exercise was $44.10.

The following table summarizes information about the weighted average grant-date fair value of options granted during the years ended December 31:

	Granted during the year	Weighted average grant date fair value (in $)
2025	1,220,410	7.68
2024	1,093,080	3.19

Share options outstanding at the end of the year have the following weighted-average remaining contractual life and ranges of exercise prices:

Weighted average remaining contractual life	Range exercise price per share	Number of options
0 to 5 years	$3.17 - $45.94	578,735
6 years	$15.86 - $21.71	293,896
7 years	$7.30 - $13.77	626,502
8 years	$3.92 - $11.74	979,343
9 years	$2.66 - $10.00	2,053,515
At December 31, 2025		**4,531,991**

Weighted average remaining contractual life	Range exercise price per share	Number of options
0 to 5 years	$5.37 -$78.01	1,764,204
6 years	$28.24 - $37.00	409,046
7 years	$14.08 - $23.73	738,760
8 years	$6.73 - $20.83	1,123,720
9 years	$4.57 - $5.59	931,430
At December 31, 2024		**4,967,160**

The fair value of each option issued was estimated at the date of grant using the Hull & White option pricing model with the following weighted-average assumptions:

	Years ended December 31,	
Assumptions	2025	2024
Options with change of control and service-based vesting conditions	4,531,991	4,967,160
Share price [1)]	$4.57 - $78.01	$4.57- $78.01
Estimated fair value per option as of grant date	$10.00 - $6.71	$20.60 - $45.90
Expected volatility	70.0% - 80.0%	70.0%
Expected term	10 years	10 years
Exercise price	$4.57 - $78.01	$4.57- $78.01
Expected dividend yield [2)]	0.0%	0.0%
Risk-free rate [3)]	4.36% - 4.64%	0.16% - 4.8%

(1) Closing share price on the grant dates.

(2) The Company currently does not pay dividends and has no plans to do so.

(3) Based on Government bonds with a term that is commensurate with the expected term of each option tranche. Also considered is the risk- free rate over the performance period for each option tranche.

Expected option term

The Hull & White option model captures early exercises by assuming that the likelihood of exercises will increase when the share price reaches defined multiples of the strike price. This analysis is included for the full contractual term.

Expected volatility

The historical volatility assumption was based on the stock price over the 10-year period preceding the grant date.

Restricted share units

The movement in the number of RSUs issued under the 2014 Plan is as follows:

	RSU		
	Number of Ordinary shares		Weighted average grant-date fair value
Non-vested at January 1, 2024	**2,264,369**	**$**	**18.07**
Granted	1,321,360	$	5.57
Distributed	(931,450)	$	19.49
Forfeited	(994,381)	$	12.44
Non-vested at January 1, 2025	**1,659,898**	**$**	**10.71**
Granted	1,751,190	$	13.18
Distributed	(776,882)	$	12.04
Forfeited	(238,786)	$	10.04
Non-vested at December 31, 2025	**2,395,420**	**$**	**12.15**
Total weighted average grant date fair value of RSUs granted during 2025 (in $ millions)		$	23.1
Granted to directors and officers during 2025 (shares, $ in millions)	300,790	$	3.6

RSUs vest over one to three years. RSUs granted to non-executive directors will vest one year from the date of grant. In determining the fair values no payments of dividends were assumed during the service periods.

Performance share units

The movement in the number of PSUs issued under the 2014 Plan is as follows:

	PSU		
	Number of Ordinary shares		Weighted average grant-date fair value
Non-vested at January 1, 2024	**222,550**	**$**	**28.09**
Granted	65,000	$	15.40
Forfeited	(42,759)	$	29.35
Distributed	(94,671)	$	28.63
Non-vested at January 1, 2025	**150,120**	**$**	**21.65**
Forfeited	(61,120)	$	26.70
Distributed	(66,500)	$	18.46
Non-vested at December 31, 2025	**22,500**	**$**	**15.40**

The Company granted PSUs to certain employees in 2021 and 2022 that were earned upon the achievement of defined milestones. Earned shares vested upon the latter of a minimum service period of three years, or the achievement of defined milestones, subject to the grantee's continued employment. In addition, the December 2021 PSUs granted to executives and other members of senior management were subject to the achievement of a minimum total shareholder return relative to the Nasdaq biotechnology index. As of December 31, 2025, there are no remaining milestones to be achieved in relation to the PSUs granted in 2021 and 2022.

The Company granted PSUs to certain employees in December 2024 that will vest shortly after the achievement of specified performance conditions, subject to the grantee's continued employment. A portion of this grant was retired as of December 31, 2025, as the specified performance conditions of other PSUs had not been met. As of December 31, 2025, the Company estimates that the specified performance conditions associated with the one grant outstanding will be partially achieved in 2026.

Employee Share Purchase Plan ("ESPP")

In June 2018, the Company's shareholders adopted and approved an ESPP allowing the Company to issue up to 150,000 ordinary shares. The ESPP is intended to qualify under Section 423 of the Internal Revenue Code of 1986. Under the ESPP, employees are eligible to purchase ordinary shares through payroll deductions, subject to any plan limitations. The purchase price of the shares on each purchase date is equal to 85% of the lower of the closing market price on the offering date or the closing market price on the purchase date of each three-month offering period. During the year ended December 31, 2025, nil shares were issued in relation to the ESPP (December 31, 2024: 10,150 ordinary shares). As of December 31, 2025, a total of 86,712 ordinary shares remains available for issuance under the ESPP plan.

13. Expenses by nature

Operating expenses excluding expenses presented in Other expenses within the Company's Consolidated Statement of Profit or Loss and Other Comprehensive Income or Loss included the following expenses by nature:

	Years ended December 31,	
	2025	2024
	$ in thousands	
Employee-related expenses	76,249	89,388
Laboratory and development expenses	60,868	49,497
Legal and advisory expenses	17,958	11,431
Commercial advisory expenses	7,119	—
Depreciation and amortization	9,192	9,751
Fair value loss / (gain) - contingent consideration liability	6,247	(1,817)
Office and housing expenses	4,183	8,586
Patent and license expenses	3,315	2,432
Expenses related to lease arrangements	2,738	3,553
Other operating expenses	12,677	12,912
Total	**200,546**	**185,733**

The Company employed an average of 215 employees during the year ended December 31, 2025 (December 31, 2024: 346) including an average of 64 employees outside of Europe (December 31, 2024: 134). The average number of employees by function during the years ended December 31, 2025 and 2024 is summarized as follows:

	2025	2024
Manufacturing	—	51
Research and development	140	216
Selling, general and administrative	75	79
Total	**215**	**346**

Details of employee-related expenses for the years ended December 31, 2025 and 2024 are as follows:

	Years ended December 31,	
	2025	2024
	$ in thousands, except for employee numbers	
Wages and salaries	44,609	53,860
Share-based compensation expenses	16,040	13,036
Social security costs	3,750	4,672
Contractor expenses	4,129	3,608
Health insurance	1,492	2,942
Costs related to pension plans	2,618	2,461
Severance costs	—	5,190
Other employee expenses	3,611	3,619
Total	**76,249**	**89,388**
Number of employees at the end of the period	221	209

14. Collaboration arrangements and concentration of credit risk

Genezen

Refer to Note 3, "*Divestiture of commercial manufacturing activities*" and Note 4, "*Financial risk management*" for further information.

CSL Behring collaboration

Revenues recorded for the years ended December 31, 2025 and 2024 are as follows:

	Years ended December 31,	
	2025	2024
	$ in thousands	
License revenues (royalty payments)	15,934	10,133
Contract manufacturing revenues	—	6,114
Collaboration revenues	164	10,872
Total	**16,098**	**27,119**

Contract manufacturing revenues from CSL Behring

The Company is required to supply HEMGENIX® to CSL Behring pursuant to the CSL Behring CSA until such time that these capabilities are transferred to CSL Behring or a contract manufacturing organization designated by CSL Behring. On September 6, 2022, CSL Behring notified the Company of its intent to transfer manufacturing technology related to HEMGENIX® in the coming years to a third-party contract manufacturer designated by CSL Behring.

The Company generated nil and $6.1 million of contract manufacturing revenue from sales to CSL Behring during the years ended December 31, 2025 and 2024. The Company incurred nil and $17.1 million of costs in relation to its contract manufacturing activities during the years ended December 31, 2025 and 2024.

Collaboration revenues from CSL Behring

The Company provides on-demand development services and other services to CSL Behring. These activities are reimbursed at an agreed full-time employee rate, and CSL Behring also reimburses agreed third-party expenses incurred in connection with the performance of these activities.

The Company recognized $0.2 million of collaboration revenue in the year ended December 31, 2025, compared to $10.9 million in 2024.

Accounts receivable

As of December 31, 2025, accounts receivable totaled $5.9 million, and related to royalty revenue due from CSL Behring. As of December 31, 2024, the $5.7 million accounts receivable balance related to collaboration services, contract manufacturing revenue, and royalty revenue due from CSL Behring.

15. Other income

Other income during the year ended December 31, 2025 was $14.4 million compared to $11.1 million during the same period in 2024.

	Years ended December 31,	
	2025	2024
	$ in thousands	
Sale of critical reagents to Genezen	6,000	—
Research and development grants from Dutch and French authorities	5,681	5,602
Sublease income	2,101	1,073
Gain on sale of commercial manufacturing facility (see Note 3, "*Divestiture of commercial manufacturing activities*")	—	4,367
Other	628	101
Total	**14,410**	**11,143**

16. Other expense

Other expense during the year ended December 31, 2025 was $7.5 million compared to $4.2 million during the same period in 2024.

	Years ended December 31,	
	2025	2024
	$ in thousands	
Supply of HEMGENIX® to CSL Behring	5,663	2,842
Sublease expense	1,791	1,359
Total	**7,454**	**4,201**

During the year ended December 31, 2025, the Company recognized a net expense of $5.7 million within other expense, compared with $2.8 million in 2024, related to HEMGENIX® supply arrangements with Genezen and CSL Behring, including the amortization of favorable supply terms and the release of the onerous contract provision.

17. Finance income / (expense), net

	Years ended December 31,	
	2025	2024
	$ in thousands	
Finance income		
Foreign exchange gains, net	25,235	—
Interest income on cash and cash equivalents and investment securities	18,658	21,008
Fair value gain on financial liability at FVTPL (see Note 4, "*Financial risk management*")	10,905	—
Gain on modification of Hercules borrowing (see Note 8, "*Borrowings*")	4,896	—
Fair value gain and other adjustments on financial assets at FVTPL (see Note 4, "*Financial risk management*")	1,532	407
Total finance income	**61,226**	**21,415**
Finance expense		
Interest expense on Royalty Finance Agreement (see Note 9, "*Royalty Financing Agreement*")	(25,040)	(44,186)
Interest expense on Hercules borrowing (see Note 8, "*Borrowings*")	(7,433)	(15,185)
Interest expense on leases (see Note 20, "*Leases*")	(1,965)	(3,142)
Interest expense on unwinding of discount on onerous contract provision (see Note 3, "*Divestiture of commercial manufacturing activities*")	(588)	(377)
Interest expense - other	(967)	—
Foreign exchange losses, net	—	(9,760)
Total finance expense	**(35,993)**	**(72,650)**
Finance income / (expense), net	**25,233**	**(51,235)**

Foreign exchange losses and gains, net include foreign currency gains and losses on cash and cash equivalents and investment securities, Hercules borrowing and the Royalty Financing Agreement, as well as loans between entities within the uniQure group and other foreign currency monetary items.

18. Income taxes

Due to the uncertainty surrounding the realization of favorable tax attributes in future fiscal years, the Company has not recognized net deferred tax assets in the Netherlands and partly recognized net deferred tax assets in France.

There are no significant unrecognized tax benefits as of December 31, 2025 and 2024.

The reconciliation of the Dutch statutory income tax rate to the Company's effective tax rate for the years ended December 31, 2025, and 2024 (-3.7% in 2025 and -1.2% in 2024) is as follows:

	Years ended December 31,			
	2025		2024	
	%	$ in thousands	%	$ in thousands
Loss before income tax expense		(153,945)		(221,234)
Expected tax benefit at the 26% tax rate enacted in the Netherlands	25.8%	39,718	25.8%	57,078
Difference in tax rates between the Netherlands and foreign jurisdictions	(0.1)%	(134)	(0.1)%	(274)
Tax effect of:				
Non-deductible expenses	(3.7)%	(5,664)	(1.8)%	(3,967)
Non-taxable income	2.4%	3,751	0.3%	680
Current-year losses for which no deferred tax asset is recognized	(37.4)%	(57,553)	(18.5)%	(40,967)
Current-year changes in unrecognized temporary differences, net	11.8%	18,139	(6.9)%	(15,237)
Current tax expense related to prior years	(2.5)%	(3,907)		
Income tax expense	(3.7)%	**(5,650)**	(1.2)%	**(2,687)**
Income tax expense				
Current tax expense		(4,042)		(145)
Deferred tax expense		(1,608)		(2,542)
Income tax expense recorded in the period		**(5,650)**		**(2,687)**

Non-deductible expenses relate to share-based compensation expenses of $4.1 million in 2025 (2024: $3.5 million) and a fair value loss on contingent consideration of EUR 1.5 million ($1.6 million) in 2025 (2024: nil).

Non-taxable income primarily relates to a fair value gain on the liability related to pre-funded warrants of $3.1 million in 2025 (2024: nil), a fair value gain on the convertible promissory note of $0.3 million (2024: nil), a fair value gain on the Series C preferred shares of $0.3 million (2024: $0.2 million), and a fair value gain on contingent consideration of nil in 2025 (2024: EUR 0.4 million ($0.5 million)).

Current-year losses for which no deferred tax is recognized related to EUR 51.1 million ($57.6 million) of taxable losses in the Netherlands and France (2024: EUR 38.2 million ($41.0 million)).

Current-year changes in unrecognized temporary differences primarily relate to the alignment of the Dutch corporate income tax treatment of the Royalty Financing Agreement. In 2025, the Company revised its Dutch filing position to treat the $375.0 million upfront payment received in 2023 as taxable income in that year, which resulted in taxable profit in 2023 rather than a tax loss and consumption of Dutch net operating loss carryforwards, resulting in deductible temporary differences for which no deferred tax asset had previously been recognized as recovery had not been considered probable. This adjustment of the filing position also resulted in the recognition of EUR 3.3 million ($3.9 million) in current tax expenses in 2025.

Movement in deferred tax balances:

	At January 1, 2025	Recognized in profit or loss	Other comprehensive income	Recognized in equity	At December 31, 2025
			$ in thousands		
Movement in deferred tax balances					
Net operating loss carryforwards	13,122	(1,079)	949	466	13,458
Lease liabilities	4,728	(3,483)	—	—	1,245
Defined pension liability	260	(22)	(32)	—	206
Accrued expenses and other current liabilities	3,069	(34)	—	—	3,035
Intangible assets and other	947	(474)	—	—	473
Total deferred tax assets	22,126	(5,092)	917	466	18,417
IPR&D asset	(14,318)	—	(1,920)	—	(16,238)
Right-of-use assets	(3,847)	2,713	—	—	(1,134)
Prepaid expenses and other	(857)	705	—	—	(152)
Property, plant and equipment	(165)	66	—	—	(99)
Total deferred tax liabilities	(19,187)	3,484	(1,920)	—	(17,623)
Net deferred tax asset	**2,939**	**(1,608)**	**(1,003)**	**466**	**794**

	At January 1, 2024	Recognized in profit or loss	Other comprehensive income	At December 31, 2024
		$ in thousands		
Movement in deferred tax balances				
Net operating loss carryforwards	16,026	(2,489)	(415)	13,122
Lease liabilities	10,125	(5,397)	—	4,728
Defined pension liability	406	(1)	(145)	260
Accrued expenses and other current liabilities	1,435	1,634	—	3,069
Property, plant and equipment	—	—	—	—
Intangible assets and other	960	(13)	—	947
Inventory	497	(497)	—	—
Total deferred tax assets	29,449	(6,763)	(560)	22,126
IPR&D asset	(15,242)	—	924	(14,318)
Right-of-use assets	(7,369)	3,522	—	(3,847)
Prepaid expenses and other	(1,002)	145	—	(857)
Property, plant and equipment	(719)	554	—	(165)
Total deferred tax liabilities	(24,332)	4,221	924	(19,187)
Net deferred tax asset	**5,117**	**(2,542)**	**364**	**2,939**

Unrecognized temporary differences:

Deferred tax assets and liabilities have not been recognized in respect of the following items, because it is not probable that future taxable profit will be available against which the Company can use the benefits therefrom.

	Years ended December 31,			
	2025		2024	
	Gross Amount	Tax Effect	Gross Amount	Tax Effect
	$ in thousands			
Unrecognized temporary differences				
Deductible temporary differences	463,274	119,524	105,515	27,223
Net operating loss carryforwards	593,720	153,180	608,584	157,015
Total	1,056,994	272,704	714,099	184,238

Unrecognized deferred temporary differences, net	**1,056,994**	**272,704**	**714,099**	**184,238**
Unrecognized deferred temporary differences to be recorded within equity				
Share issuance costs incurred in relation to public offerings	40,700	9,914	14,548	3,157
Unrecognized deferred temporary differences to be recorded within equity	**40,700**	**9,914**	**14,548**	**3,157**

Netherlands

As of December 31, 2025, the total amount of net operating losses carried forward under the Dutch tax regime was EUR 480.1 million ($564.2 million) (December 31, 2024: EUR 593.6 million ($616.5 million)).

The Company evaluates all positive and negative evidence in assessing whether to recognize deferred temporary differences. Management considers reversing taxable temporary differences, projected future taxable income, and tax-planning strategies in making this assessment. Based on this assessment, the Company concluded that as of December 31, 2025, and December 31, 2024 it is probable that the remaining unrecognized temporary differences will not be realized.

As of December 31, 2025, a portion of the not recognized deferred temporary differences continues to relate to share issuance costs incurred in relation to public offerings in 2019 and 2025. Any subsequently recognized tax benefits will be credited directly to share premium. As of December 31, 2025, that amount was EUR 8.4 million ($9.9 million) (December 31, 2024: EUR 3.0 million ($3.2 million)).

The Dutch corporate tax rate is 25.8% from 2022 onwards.

Net operating loss carryforwards can be carried forward indefinitely subject to a limit of offsetting taxable profit in excess of EUR 1.0 million ($1.1 million) to 50% of the taxable profit.

The fiscal periods from 2021 onwards are still open for inspection by the Dutch tax authorities.

United States of America

The federal corporate tax rate in the U.S. is 21.0%. In addition, the Company is subject to state income taxes resulting in a combined tax rate of 27.3% for its U.S. operation. As of December 31, 2025, an estimated $20.6 million of net operating losses remain to be carried forward (December 31, 2024: $22.9 million). These net operating losses carried forward will expire in 2036 and 2037 except for $0.7 million which may be carried forward indefinitely with a deduction limited to 80% of taxable income in a given year.

The Company's U.S. operations have been generating taxable income since 2018 with the exception of 2022. The Company expects to continue to generate taxable income in the U.S. during the foreseeable future.

Under the provision of the Internal Revenue Code, the U.S. net operating losses carried forward may become subject to an annual limitation in the event of certain cumulative exchange in the ownership interest of significant shareholders over a three-year period in excess of 50 percent, as defined under Section 382 and 383 of the Internal Revenue Code. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation.

The fiscal periods from 2022 are still open for inspection by the IRS. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the IRS or Massachusetts Department of Revenue to the extent utilized in a future period. The Company is currently not under examination by the IRS for any tax years.

France

The French corporate tax rate for fiscal year 2024 was 25.0%. In addition, the Company is subject to a surcharge of 3.3% of the 25.0% standard corporate tax rate resulting in a combined rate of 25.8%.

The Company's French operations have incurred losses since incorporation and are expected to continue incurring tax losses for the foreseeable future. The French operations as of December 31, 2025 have an estimated EUR 59.8 million ($70.3 million) of net operating losses that are available for carry forward indefinitely (December 31, 2024: EUR 48.9 million ($50.8 million). The Company evaluates all positive and negative evidence including future income from reversing taxable temporary differences (particularly from reversing the deferred tax liability related to the acquired IPR&D intangible asset), projected future taxable income subject to the loss limitation rules applicable in France and tax-planning strategies in making this assessment. As such, the Company limited the recognition of the deferred tax asset during such periods to income generated from the reversal of temporary differences, given the uncertainty of other sources of income.

19. Basic and diluted loss per ordinary share

Basic net loss per ordinary share is computed by dividing net loss for the period by the weighted average number of ordinary shares outstanding during the period. Diluted earnings per ordinary share is calculated by adjusting the weighted average number of ordinary shares outstanding, assuming conversion of all potentially dilutive ordinary shares. As the Company incurred a net loss for the year ended December 31, 2025 and December 31, 2024, all potentially dilutive ordinary shares would have an anti-dilutive effect, if converted, and thus have been excluded from the computation of loss per ordinary share. The ordinary shares are presented without giving effect to the application of the treasury method or exercise prices that would be above the price of an ordinary share as of December 31, 2025 and December 31, 2024, respectively.

Loss attributable to ordinary shareholders:

	Years ended December 31,			
	2025		2024	
	Continuing operations	Total	Continuing operations	Total
	$ in thousands			
Loss attributable to ordinary shareholders (basic and diluted)	(159,595)	(159,595)	(223,921)	(223,921)

The weighted-average number of ordinary shares are summarized below:

	Years ended December 31,	
	2025	2024
	ordinary shares	
Weighted-average number of ordinary shares - basic and diluted	57,502,068	48,648,129

The potentially dilutive ordinary shares are summarized below:

	Years ended December 31,	
	2025	2024[1]
	ordinary shares	
Anti-dilutive ordinary shares equivalents		
Stock options under 2014 Plans	4,531,991	4,967,160
Non-vested RSUs and PSUs	2,417,920	1,810,018
Pre-Funded Warrants	526,316	—
ESPP	13,936	—
Total anti-dilutive ordinary share equivalents	**7,490,163**	**6,777,178**

[1] During the year ended December 31, 2025, the Company identified that the anti-dilutive ordinary shares equivalents in Note 22, "*Basic and diluted earnings per ordinary share*" in the 2024 Annual Report contained an omission.

The omission did not affect the basic net loss per ordinary share for the year ended December 31, 2024, nor did it affect the recognition or measurement of assets, liabilities, income, or expenses, and was assessed as not material to the prior year consolidated financial statements within the 2024 Annual Report. The comparative anti-dilutive ordinary shares equivalents disclosure has been updated in the current year consolidated financial statements to reflect the complete information.

20. Leases

The Company's most significant leases relate to office and laboratory space under the following lease agreements:

Lexington, Massachusetts / United States

In February 2022, the Company entered into a lease for an office facility in Lexington, Massachusetts, U.S. of 12,716 square feet. The lease commenced in December 2022 and is set for a non-cancellable period ending March 2030. The lease is renewable for one five-year term.

In December 2021, the Company entered into a lease for a research and development facility in Lexington, Massachusetts, United States of 13,501 square feet of space. The lease commenced in May 2022 and is set for a non-cancellable period ending March 2029. Commencing August 2024, the Company subleased this facility for the residual term.

The fixed lease payments to be received during the remaining term under the agreement to sub-lease amount to $2.5 million as of December 31, 2025.

Amsterdam / The Netherlands

In March 2016, the Company entered into a 16-year lease for a facility in Amsterdam, the Netherlands and amended this agreement in June 2016. The lease for the facility terminates in February 2032, with an option to extend in increments of five-year periods. The lease contract includes variable lease payments related to annual increases in payments based on a consumer price index. In May 2021, the Company leased an additional approximately 1,080 square meters of office space. The lease expires in October 2028.

As of December 31, 2025 the Company sub-leases two of the seven floors of its Amsterdam facility for a ten-year term ending on December 31, 2027.

The fixed lease payments to be received during the remaining term under the agreement to sub-lease amount to EUR 1.9 million ($2.2 million) as of December 31, 2025.

Other information related to leases is included below for the year ended December 31, 2025:

	Year ended December 31,			
	2025			
	Lexington	Amsterdam	Other	Total
	$ in thousands			
Depreciation expense for right-of-use assets	1,056	1,275	407	2,738
Interest expense on lease liabilities	485	1,410	70	1,965
Variable lease payments not included in the measurement of lease liabilities	871	—	—	871
Total cash outflows	1,394	2,642	484	4,520
Carrying amount of right-of-use assets as of December 31, 2025	4,056	7,647	522	12,225

Other information related to leases is included below for the year ended December 31, 2024:

	Year ended December 31, 2024			
	Lexington	Amsterdam	Other	Total
	$ in thousands			
Depreciation expense for right-of-use assets	2,087	1,088	379	3,554
Interest expense on lease liabilities	1,677	1,361	105	3,143
Variable lease payments not included in the measurement of lease liabilities	631	—	—	631
Total cash outflows	3,678	2,371	459	6,508
Derecognition of right-of-use-assets (see Note 3, "*Divestiture of commercial manufacturing activities*")	(8,540)	—	—	(8,540)
Carrying amount of right-of-use assets as of December 31, 2024	5,112	7,057	811	12,980

Sublease income for the year ended December 31, 2025 was $2.1 million (December 31, 2024: $1.2 million).

As of December 31, 2025, the lease liability maturity analysis of contractual undiscounted cash flows is as follows:

	Year ended December 31, 2025			
	Lexington	Amsterdam	Other	Total
	$ in thousands			
Not later than 1 year	1,431	2,824	349	4,604
Later than 1 year and not later than 5 years	3,870	10,812	88	14,770
Later than 5 years	—	2,400	—	2,400
Total undiscounted lease liabilities at December 31, 2025	**5,301**	**16,036**	**437**	**21,774**
Lease liabilities included in the Consolidated Statement of Financial Position as of December 31, 2025	**4,433**	**11,377**	**425**	**16,235**
Current	1,431	2,825	348	4,604
Non-current	3,002	8,552	77	11,631

As of December 31, 2024, the lease liability maturity analysis of contractual undiscounted cash flows was as follows:

	Year ended December 31, 2024			
	Lexington	Amsterdam	Other	Total
	$ in thousands			
Not later than 1 year	1,394	2,318	445	4,157
Later than 1 year and not later than 5 years	5,172	9,027	384	14,583
Later than 5 years	130	4,094	—	4,224
Total undiscounted lease liabilities at December 31, 2024	**6,696**	**15,439**	**829**	**22,964**
Lease liabilities included in the Consolidated Statement of Financial Position as of December 31, 2024	**5,342**	**10,391**	**741**	**16,474**
Current	1,394	2,318	444	4,156
Non-current	3,948	8,073	297	12,318

21. Commitments and contingencies

In the course of its business, the Company enters as a licensee into contracts with other parties regarding the development and marketing of its pipeline products. Among other payment obligations, the Company is obligated to pay royalties to the licensors based on future sales levels and milestone payments whenever specified development, regulatory and commercial milestones are met. As both future sales levels and the timing and achievement of milestones are uncertain, the financial effect of these agreements cannot be estimated reliably. The Company also has obligations to make future payments that become due and payable upon the collection of milestone payments from CSL Behring. The achievement and timing of these milestones is not fixed and determinable. Relevant commitments and contingencies are further discussed in other sections of this Annual Report, such as Note 3, "*Divestiture of commercial manufacturing activities*" and Note 14, "*Collaboration arrangements and concentration of credit risk*", amongst others.

Legal Proceedings

On February 10, 2026, a class action complaint captioned Christopher Scocco v. uniQure N.V., et al., Case No. 1:26-cv-01124, was filed against the Company, certain of the Company's executive officers and another party in the United States District Court for the Southern District of New York. The complaint purports to assert claims pursuant to Sections 10(b) and 20(a) of the Securities Exchange Act of 1934, as amended, and Rule 10b-5 promulgated thereunder, on behalf of a putative class of investors who purchased or otherwise acquired our ordinary shares between September 24, 2025 and October 31, 2025. Plaintiff seeks to recover damages allegedly caused by purported false and misleading misstatements and omissions with respect to the Company's Phase I/II study of AMT-130 and the timing of the potential BLA filing for AMT-130. The Company intends to vigorously defend against the claims in this action.

At each reporting date, the Company evaluates whether or not a potential loss amount or a potential range of loss is probable and reasonably estimable under the provisions of the authoritative guidance that addresses accounting for contingencies. At this time, the Company is unable to predict the outcome of the class action litigation or reasonably estimate a range of possible losses.

22. Related party transactions

In the years ended December 31, 2025, and 2024, executive directors received regular salaries, post-employment benefits and share-based payments. Additionally, non-executive directors received compensation for their services in the form of cash compensation and equity grants.

23. Key management compensation

On June 11, 2025, the Company's shareholders reappointed Matthew Kapusta as executive director and Robert Gut and Jeremy P. Springhorn as non-executive directors of the Board at the Company's 2025 annual general meeting.

Board of Directors

The aggregate remuneration of the Board of Directors amounted to $5.8 million for the year ended December 31, 2025 (December 31, 2024: $6.9 million). Details by director are as follows:

		Year ended December 31, 2025					
		Short-term employee benefits	Share-based payments [1]	Post-employment benefits	Board fee	Termination benefits	Total
		$ in thousands					
Matthew Kapusta[3]	Executive	1,218	2,908	2	—	—	4,128
Total executive director		**1,218**	**2,908**	**2**	**—**	**—**	**4,128**
David Meek[2]	Non-Executive, Chairman	—	160	—	98	—	258
Madhavan Balachandran	Non-Executive	—	160	—	65	—	225
Robert Gut[3]	Non-Executive	—	160	—	53	—	213
Rachelle Jacques[2]	Non-Executive	—	160	—	70	—	230
Jack Kaye	Non-Executive	—	160	—	73	—	233
Leonard Post	Non-Executive	—	160	—	60	—	220
Jeremy P. Springhorn[3]	Non-Executive	—	160	—	78	—	238
Total non-executive directors		**—**	**1,120**	**—**	**497**	**—**	**1,617**

		Year ended December 31, 2024					
		Short-term employee benefits	Share-based payments [1]	Post-employment benefits	Board fee	Termination benefits	Total
		$ in thousands					
Matthew Kapusta [3]	Executive	1,162	3,518	1	—	—	4,681
Total executive director		**1,162**	**3,518**	**1**	**—**	**—**	**4,681**
David Meek[2]	Non-Executive, Chairman	—	220	—	93	—	313
Madhavan Balachandran	Non-Executive	—	220	—	60	—	280
Robert Gut [3]	Non-Executive	—	222	—	53	—	275
Rachelle Jacques[2]	Non-Executive	—	231	—	55	—	286
Jack Kaye	Non-Executive	—	220	—	73	—	293
Leonard Post	Non-Executive	—	220	—	60	—	280
Paula Soteropoulos[4]	Non-Executive	—	187	—	27	—	214
Jeremy P. Springhorn[3]	Non-Executive	—	220	—	73	—	293
Total non-executive directors		**—**	**1,740**	**—**	**494**	**—**	**2,234**

[1] The share-based payment reflects the value of equity settled share options, RSUs and PSUs expensed during the year, as required by IFRS 2, *Share-based Payment*.
[2] Reappointed on June 18, 2024.
[3] Reappointed on June 11, 2025.
[4] On April 9, 2024, Ms. Soteropoulos notified the Company that she will not stand for re-election to the Board when her term expires, at the 2024 Annual Meeting.

Management team

The compensation costs of the Management Team (excluding Mr. Kapusta) for the years ended December 31, 2025, and 2024 were as follows:

	Short-term employee benefits	Share-based payments	Post-employment benefits	Termination benefits	Total
			$ in thousands		
Year ended December 31, 2025	5,105	6,064	317	—	11,486
Year ended December 31, 2024	5,601	4,463	368	938	11,370

Refer to Note 12, "*Share-based compensation*" for further information regarding share-based payment awarded to key management personnel and directors. Expenses resulting from the acceleration of performance share units for executives leaving the Company are presented within share-based payments.

24. Events after the reporting date

Amyotrophic Lateral Sclerosis ("AMT-162") program discontinuation

In April 2026, the Company determined that further development of the AMT-162 program will be discontinued. This is considered to be a non-adjusting event after the December 31, 2025 reporting date, and accordingly, no adjustment has been made to the consolidated financial statements. As at December 31, 2025, the carrying amount of the AMT-162 program-related intangible asset within the Company's Consolidated Statement of Financial Position was $6.5 million. The Company expects that this decision may substantially reduce the recoverable amount of the related intangible asset and could result in an impairment loss. The Company is also assessing the accounting treatment of contractual commitments related to the long-term patient follow-up and the in-licensed nature of the AMT-162 program. The resulting financial effects of the decision cannot yet be reliably estimated.

Termination agreements with CSL Behring and Genezen

Since the Company's July 2024 divestiture of its commercial manufacturing activities to Genezen, the Company had been sourcing HEMGENIX® under the Genezen CSA. The Company entered into the Genezen CSA to enable it to meet its obligations to supply CSL Behring with HEMGENIX® in accordance with the CSL Behring Agreement (refer to Note 3, "*Divestiture of Commercial Manufacturing*") until such time that the HEMGENIX® manufacturing technology had been transferred to CSL Behring or a contract manufacturing organization designated by CSL Behring. The Company, since July 2024, had served as an agent in the sale of HEMGENIX® to CSL Behring as title to HEMGENIX® drug product supply had been directly passing from Genezen to CSL Behring (refer to Note 2.22(b), "*Revenue Recognition*"). In April 2026, we entered into agreements with CSL Behring and Genezen that provide for i) the termination of our obligation to supply HEMGENIX® and any minimum purchase commitments under the Genezen CSA once the contractually specified batches have been supplied to CSL Behring, which we expect to occur in mid-2026, and ii) the designation of Genezen as CSL Behring's contract manufacturing organization. In addition, the CSL Behring Agreement was amended to terminate certain manufacturing-related terms associated with both the CSL Behring CSA and the Genezen CSA, as well as our development support that CSL Behring could request from time to time with respect to HEMGENIX®. All other terms of the CSL Behring Agreement remain in full force and effect.

This is considered a non-adjusting event after the December 31, 2025 reporting date and, accordingly, no adjustment has been made to the consolidated financial statements as of December 31, 2025. The Company expects that by the time the contractually specified batches have been supplied to CSL Behring, it will i) have fully amortized the carrying amount of the intangible asset recorded with respect to the favorable supply terms under the Genezen CSA of $8.7 million as of December 31, 2025, and ii) have fully released the onerous contract provision with a December 31, 2025 carrying amount of $6.0 million (refer to Note 3, "*Divestiture of commercial manufacturing activities*").

C Company-Only Financial Statements uniQure N.V. for the year ended December 31, 2025

uniQure N.V.

Company-Only Statement of Financial Position

	Note	December 31, 2025	December 31, 2024
		$ in thousands	
Non-current assets			
Intangible fixed assets	3	24,836	21,955
Other investments	4	30,346	26,160
Financial fixed assets	5	99,235	48,724
Total fixed assets		**154,417**	**96,839**
Current assets			
Receivables from affiliated entities	7	88,961	678
Other current assets		4,457	275
Cash and cash equivalents		35,148	3,818
Total current assets		**128,566**	**4,771**
Total assets		**282,983**	**101,610**
Shareholders' equity			
Share capital		5,264	3,716
Share premium		1,210,178	818,554
Legal reserves		(61,529)	(53,775)
Other reserves		235,606	219,599
Accumulated deficit		(985,608)	(761,687)
Net result for the year		(159,595)	(223,921)
Total shareholders' equity	8	**244,315**	**2,486**
Provisions			
Provision for participating interest	5	—	21,879
Total provisions		**—**	**21,879**
Non-current liabilities			
Non-current liabilities	6	19,374	11,230
Total non-current liabilities		**19,374**	**11,230**
Current liabilities			
Payable to affiliated entity	7	1,758	65,574
Liability related to pre-funded warrants		12,595	—
Accrued expenses and other current liabilities		4,941	441
Total current liabilities		**19,294**	**66,015**
Total liabilities and shareholders' equity		**282,983**	**101,610**

Before appropriation of the result for the year.

The accompanying notes are an integral part of these Company-only financial statements.

uniQure N.V.

Company-Only Statement of Profit or Loss

	Years ended December 31,	
	2025	2024
	$ in thousands	
Share in results from participating interests	(134,692)	(204,292)
Other income and expenses	(24,903)	(19,629)
Net loss	**(159,595)**	**(223,921)**

The accompanying notes are an integral part of these Company-only financial statements.

Notes to the Company-only Financial Statements

1. General

uniQure N.V. ("uniQure" or the "Company") was incorporated on January 10, 2012.

The Company-only financial statements are part of the 2025 financial statements of uniQure N.V. On February 10, 2014, the Company converted from a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*) incorporated under the laws of the Netherlands into a public company with limited liability (*naamloze vennootschap*), and changed its legal name from uniQure B.V. to uniQure N.V.

The Company is the parent company of the uniQure group and is listed on Nasdaq. The Company provides intercompany funding to its operational subsidiaries in the form of loans and equity financing. The Company conducts its business through its Dutch subsidiary uniQure biopharma B.V., its French subsidiary uniQure France SAS and its Swiss subsidiary uniQure Switzerland GmbH. uniQure biopharma B.V. is the parent of the U.S. operating entity uniQure Inc. The Company issued a joint and several liability statements per Section 2:403 of the Dutch Civil Code, for the benefit of its Dutch subsidiaries, thereby establishing a contingent liability. The Company has no employees.

uniQure N.V. forms a fiscal unity with its Dutch subsidiaries for income tax purposes. In accordance with the standard conditions, a company and its subsidiaries that form the fiscal unity are jointly and severally liable for tax payable by the fiscal unity.

2. Basis of preparation

These Company-only financial statements have been prepared in accordance with Part 9 of Book 2 of the Dutch Civil Code. With respect to the setting of principles for the recognition and measurement of assets and liabilities and determination of the result for its Company-only financial statements, uniQure makes use of the option provided in Section 2:362 (8) of the Dutch Civil Code. This means that the principles for the recognition and measurement of assets and liabilities and determination of the result are the same as those applied in the consolidated financial statements and are based on IFRS Accounting Standards as endorsed by the European Union for the financial year ended December 31, 2025.

Please see the notes to the consolidated financial statements for a description of these recognition and measurement principles, including those for foreign currency transactions and intra-group transactions. For an appropriate interpretation of these separate financial statements, the separate financial statements should be read in conjunction with the consolidated financial statements. These financial statements are presented in U.S. dollars as the Company is listed on Nasdaq and its reporting currency is the U.S. dollar.

With reference to the Company-only income statement of uniQure, use has been made of the exemption pursuant to Section 2:402 of the Dutch Civil Code.

In the Company-only financial statements, participating interests in group companies and long-term loan receivables are presented at their net asset value, being the equity of the respective participating interest in group companies. If the net asset value of a participating interest in group companies is negative, then the carrying amount of the long-term loan receivable from that participating interest in group companies is reduced with the negative equity amount. The Company adopted a policy whereby a reduction of negative equity will first be recorded as a reversal of a reduction of a long-term loan receivable's carrying amount before reversing reductions of the carrying amount of participating interests in group companies.

The Company-only financial statements have been prepared on a going concern basis based on the Company's cash and cash equivalents as of December 31, 2025, and the Company's budgeted cash flows for the twelve months following the issuance date.

3. Intangible fixed assets

	Goodwill $ in thousands
Cost	
Balance at January 1, 2024	25,891
Disposals (see Note 3, "*Divestiture of commercial manufacturing activities*" in the consolidated financial statements)	(2,500)
Effect of movement in exchange rates	(1,436)
Balance at December 31, 2024	**21,955**
Balance at January 1, 2025	21,955
Effect of movement in exchange rates	2,881
Balance at December 31, 2025	**24,836**
Accumulated amortization and impairment losses	
Balance at January 1, 2024	—
Balance at December 31, 2024	**—**
Balance at January 1, 2025	—
Balance at December 31, 2025	—
Carrying amounts	
Balance at January 1, 2024	25,891
Balance at December 31, 2024	21,955
Balance at December 31, 2025	24,836

Further disclosures relating to goodwill can be found in Note 6, "*Intangible Assets*" to the consolidated financial statements.

4. Other investments

The Company holds an investment in Series C preferred stock in Genezen Holdings Inc. with a fair value of $14.4 million at December 31, 2025 (December 31, 2024: $12.5 million).

The Company holds an investment in a convertible promissory note with a fair value of $16.0 million at December 31, 2025 (December 31, 2024: $13.7 million).

Further disclosures relating to Other investments can be found in Note 4, "*Financial Risk Management*" to the consolidated financial statements.

5. Financial fixed assets

uniQure N.V. holds participating interests in the following group companies:

Name	Percentage of shares	Statutory seat
uniQure biopharma B.V.	100%	Amsterdam, Netherlands
uniQure IP B.V.	100%	Amsterdam, Netherlands
uniQure France SAS	100%	Illkirch-Graffenstaden, France
uniQure Switzerland GmbH	100%	Basel, Switzerland

	Investment in participating interests
	$ in thousands
Cost [1]	751,476
Accumulated share in results from participating interests, including currency translation effects [1]	(520,681)
Share in results from participating interests for the period, including currency translation effects [1]	(182,071)
Carrying amount January 1, 2025	**48,724**
Investments	221,164
Share in results from participating interests for period [2]	(134,692)
Currency translation effects	(35,962)
Carrying amount December 31, 2025	**99,235**
Cost [1]	1,071,757
Accumulated share in results from participating interests, including currency translation effects [1]	(801,869)
Share in results from participating interests for the period, including currency translation effects [1]	(170,653)
Carrying amount December 31, 2025	**99,235**

[1] Translated into the presentation currency at the December 31, 2025 and December 31, 2024 exchange rates, respectively.

[2] Translated into the presentation currency at monthly average exchange rates for the year ended December 31, 2025.

Services provided by employees of uniQure N.V. group are partially compensated through the issuance of ordinary shares of uniQure N.V. The Company records any share-based compensation incurred by its participating interests as an investment into the respective participating interest together with a corresponding increase of its share premium.

During the year ended December 31, 2025, the Company, and uniQure biopharma B.V. established a share premium contribution agreement in which the Company made, and uniQure biopharma B.V. accepted, a share premium contribution on all shares in uniQure biopharma B.V.'s capital held by the Company. In accordance with the agreement, a share premium contribution was paid by the Company with respect to the shares held in uniQure biopharma B.V. for a cash amount equal to EUR 170.0 million ($199.8 million), without any obligation for uniQure biopharma B.V. to pay consideration or to issue any shares in its capital in return. The capital contribution was accounted for as an increase in the carrying amount of the investment in uniQure biopharma B.V.

During the year ended December 31, 2025, a loan from the Company to uniQure France SAS was converted into an equity investment, resulting in a EUR 8.0 million ($9.4 million) increase in the Company's participating interest.

As of December 31, 2024, one of the participating interests, uniQure biopharma B.V., had a negative net asset value and was valued at nil. A provision of $21.9 million for this negative equity was recognized as of December 31, 2024, consistent with the Company's article (403) declaration. As of December 31, 2025, the net asset value of uniQure biopharma B.V. returned to positive and the $21.9 million provision was released.

6. Non-current liabilities

	As of December 31,	
	2025	2024
	$ in thousands	
Contingent consideration	18,736	10,860
Other non-current liabilities	637	370
Total non-current liabilities	**19,374**	**11,230**

Contingent consideration was recognized in connection with the uniQure France Acquisition which took place in 2021. None of the Company's total contingent consideration of $18.7 million and $10.9 million as of December 31, 2025 and 2024, respectively, was classified as current.

Further disclosures relating to the contingent consideration can be found in Note 4, "*Financial Risk Management*" to the consolidated financial statements.

7. Receivables from and payables to affiliated entities

The receivables from and payables to affiliated entities as of December 31, 2025 and 2024 were as follows:

	As of December 31,	
	2025	2024
	$ in thousands	
Receivables from affiliated entities		
uniQure biopharma B.V.	78,656	—
uniQure Inc.	10,146	678
uniQure France SAS	159	—
Total	**88,961**	**678**

	As of December 31,	
	2025	2024
	$ in thousands	
Payables to affiliated entities		
uniQure biopharma B.V.	—	65,551
uniQure France SAS	—	5
uniQure Switzerland GmbH	1,758	18
Total	**1,758**	**65,574**

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8. Shareholders' equity

During the period covered by these Company-only financial statements uniQure had a single class of shares which are denominated as ordinary shares.

	Attributable to equity holders of the Company						
	Share Capital		Legal Reserves				
	No. of shares	Amount	Share Premium	Currency translation differences	Other Reserves	Accumulated Deficit	Total Equity
	$ in thousands (except number of shares)						
Balance at January 1, 2024	**47,833,830**	**3,067**	**816,493**	**(53,610)**	**206,816**	**(761,687)**	**211,079**
Net loss	—	—	—	—	—	(223,921)	(223,921)
Other comprehensive loss	—	—	—	(165)	(877)	—	(1,042)
Total comprehensive loss	—	—	—	(165)	(877)	(223,921)	(224,963)
Appropriation of result						—	—
Share capital translation result	—	587	—	—	(587)	—	—
Exercise of share options	169,898	9	2,065	—	—	—	2,074
Restricted and performance share units distributed during the period	974,209	52	(52)	—	—	—	—
Share-based compensation expense	—	—	—	—	14,247	—	14,247
Issuance of ordinary shares relating to employee stock purchase plan	10,150	1	48	—	—	—	49
Balance at December 31, 2024	**48,988,087**	**3,716**	**818,554**	**(53,775)**	**219,599**	**(985,608)**	**2,486**
Balance at January 1, 2025	**48,988,087**	**3,716**	**818,554**	**(53,775)**	**219,599**	**(985,608)**	**2,486**
Net loss	—	—	—	—	—	(159,595)	(159,595)
Other comprehensive income	—	—	—	(7,754)	771	—	(6,983)
Total comprehensive loss	—	—	—	(7,754)	771	(159,595)	(166,578)
Share capital translation result	—	804	—	—	(804)	—	—
Tax benefit past share issuance cost			468				468
Follow-on public offerings	11,810,370	655	380,082	—		—	380,737
Exercises of share options	694,879	42	11,120	—	—	—	11,162
Restricted and performance share units distributed during the period	843,381	46	(46)	—	—	—	0
Share-based compensation expense	—	—	—	—	16,040	—	16,040
Balance at December 31, 2025	**62,336,717**	**5,263**	**1,210,178**	**(61,529)**	**235,606**	**(1,145,203)**	**244,315**

Further disclosures relating to the capital contributions and share-based payment expenses can be found in Notes 11, "*Shareholder's equity*" and 12, "*Share-based compensation*" to the consolidated financial statements.

Further information relating to differences between Group and Company equity can be found in Note 5, "*Financial fixed assets*".

As of December 31, 2025, a total of 62,336,717 ordinary shares were issued and paid up in full at a nominal value of €0.05 per share (December 31, 2024: 48,988,087 ordinary shares). Of these, 13,348,630 ordinary shares were issued during the year (December 31, 2024: 1,154,257 ordinary shares).

The total proceeds for issuance of ordinary shares, net of issuance costs during the year ended December 31, 2025 amount to $391.9 million (December 31, 2024: $2.1 million).

The Company proposes to the General Meeting of Shareholders to allocate the net loss after tax for the twelve-month period ended December 31, 2025, of $159.6 million to the accumulated deficit.

The financial statements for the reporting year 2024 were adopted by the General Meeting of Shareholders on June 11, 2025. The General Meeting adopted the appropriation of the loss after tax for the reporting year 2024 to the accumulated deficit as proposed by the Board.

9. Compensation of the Board of Directors

The executive director of uniQure N.V. is employed by a subsidiary of the Company. As of December 31, 2025, the Company recorded an amount of nil (December 31, 2024: nil) for social security and payroll tax obligations, in relation to the Board of Directors. Personal loans or guarantees have not been provided by any member of the uniQure group to any member(s) of the Board of Directors.

Refer to Note 23, "*Key management compensation*" of the consolidated financial statements.

10. Audit fees

The following table sets forth the final fees, for each of the years indicated, when the work was performed by the Company's independent auditors and the percentage of each of the fees out of the total fees when the work was performed by the independent auditors.

	Year ended December 31, 2025			
	KPMG Accountants N.V. $ in thousands	%	Other KPMG network $ in thousands	%
Audit of the financial statements	1,665	88%	—	0%
Other audit services	235	12%	—	0%
Total	**1,900**	**100%**	**—**	**0%**

	Year ended December 31, 2024			
	KPMG Accountants N.V. $ in thousands	%	Other KPMG network $ in thousands	%
Audit of the financial statements	1,501	88%	82	100%
Other audit services	211	12%	—	0%
Total	**1,712**	**100%**	**82**	**100%**

The fees listed above relate to the procedures applied to the Company and its consolidated group entities by its independent auditor as referred to in Section 1, subsection 1 of the Dutch Accounting Firms Oversight Act (Dutch acronym: Wta), as well as by Dutch and foreign-based accounting firms, including their tax services and advisory groups.

11. Events after the reporting date

Certain non-adjusting subsequent events occurred at the level of the Company's subsidiaries. These events do not have a direct impact on the Company's standalone financial position or profit or loss as at December 31, 2025. Further details of these events are included in Note 24, "*Events after the reporting date*" within the consolidated financial statements.

Signing of the Financial Statements

Amsterdam, April 24, 2026

Executive Director

/s/ Matthew Kapusta
Matthew Kapusta, Chief Executive Officer

Non-Executive Directors

/s/ David Meek
David Meek, Chairman

/s/ Madhavan Balachandran
Madhavan Balachandran, Member

/s/ Robert Gut
Robert Gut, Member

/s/ Rachelle Jacques
Rachelle Jacques, Member

/s/ Jack Kaye
Jack Kaye, Member

/s/ Leonard Post
Leonard Post, Member

/s/ Jeremy P. Springhorn
Jeremy P. Springhorn, Member

D Other Information

Statutory Arrangement Concerning the Appropriation of Profit

The statutory arrangements regarding the appropriation of profit are described in article 10.1 of the articles of association:

10.1. **Profit and loss. Distributions on Shares.**

10.1.1. The Board will keep a share premium reserve and profit reserve for the Shares.

10.1.2. The Company may make distributions on Shares only to the extent that its shareholders' equity exceeds the sum of the paid-up and called-up part of the capital and the reserves which must be maintained by law.

10.1.3. Distributions of profit, meaning the net earnings after taxes shown by the adopted Annual Accounts, shall be made after the adoption of the Annual Accounts from which it appears that they are permitted, without prejudice to any of the other provisions of these articles of association.

10.1.4. The Board may determine that any amount out of the profit shall be added to the reserves.

10.1.5. The profit remaining after application of article 10.1.4 shall be at the disposal of the General Meeting, which may resolve to carry it to the reserves or to distribute it among the Shareholders.

10.1.6. On a proposal of the Board the General Meeting may resolve to distribute to the Shareholders a dividend in the form of Shares in the share capital of the Company.

10.1.7. Subject to the other provisions of this article 10.1 the General Meeting may, on a proposal made by the Board resolve to make distributions to the Shareholders to the debit of one (1) or several reserves which the Company is not prohibited from distributing by virtue of the law.

10.1.8. No dividends shall be paid on Shares held by the Company in its own share capital, unless such Shares are encumbered with a right of use and enjoyment (*vruchtgebruik*) or pledge.

Independent auditor's report

To: the General Meeting of Shareholders and the Board of Directors of uniQure N.V.

Report on the audit of the financial statements 2025 included in the annual report

Our opinion

In our opinion:

- the accompanying consolidated financial statements give a true and fair view of the financial position of uniQure N.V. as at 31 December 2025 and of its result and its cash flows for the year then ended, in accordance with IFRS Accounting Standards as endorsed by the European Union (EU-IFRS) and with Part 9 of Book 2 of the Dutch Civil Code;
- the accompanying company financial statements give a true and fair view of the financial position of uniQure N.V. as at 31 December 2025 and of its result for the year then ended, in accordance with Part 9 of Book 2 of the Dutch Civil Code.

What we have audited

We have audited the financial statements 2025 of uniQure N.V. (the 'Company') based in Amsterdam, the Netherlands. The financial statements include the consolidated financial statements and the company-only financial statements.

The consolidated financial statements comprise:

1 the Consolidated Statement of Financial Position as of December 31, 2025;

2 the following Consolidated Statements for 2025: Profit or Loss and Other Comprehensive Income or Loss, Changes in Equity, and Cash Flows; and

3 the Notes to the Consolidated Financial Statements, comprising material accounting policy information and other explanatory information.

The company-only financial statements comprise:

1 the Company-Only Statement of Financial Position as of December 31, 2025;

2 the Company-Only Statement of Profit or Loss for the year ended December 31, 2025; and

3 the Notes to the Company-Only Financial Statements, comprising a summary of the accounting policies and other explanatory information.

Basis for our opinion

We conducted our audit in accordance with Dutch law, including the Dutch Standards on Auditing. Our responsibilities under those standards are further described in the 'Our responsibilities for the audit of the financial statements' section of our report.

We are independent of uniQure N.V. in accordance with the 'Verordening inzake de onafhankelijkheid van accountants bij assurance-opdrachten' (ViO, Code of Ethics for Professional Accountants, a regulation with respect to independence) and other relevant independence regulations in the Netherlands. Furthermore, we have complied with the 'Verordening gedrags- en beroepsregels accountants' (VGBA, Dutch Code of Ethics).

We designed our audit procedures in the context of our audit of the financial statements as a whole and in forming our opinion thereon. The information in respect of going concern, fraud and non-compliance with laws and regulations, was addressed in this context, and we do not provide a separate opinion or conclusion on these matters.

We believe the audit evidence we have obtained is sufficient and appropriate to provide a basis for our opinion.

Information in support of our opinion

Summary

Materiality
• Materiality of USD 6.5 million • 4.07% of loss before tax

Group audit
• Performed substantive procedures for 87% of total assets • Performed substantive procedures for 83% of total expenses

Risk of material misstatements related to Fraud, NOCLAR, and Going concern related risks
• Fraud risks: presumed risk of management override of controls identified and further described in the section 'Audit response to the risk of fraud and non-compliance with laws and regulations' • Non-compliance with laws and regulations (NOCLAR) risks: no reportable risk of material misstatements related to NOCLAR risks identified • Going concern risks: no going concern risks identified

Key audit matters
None identified

Materiality

Based on our professional judgement we determined the materiality for the financial statements as a whole at USD 6.5 million (2024: USD 6.5 million). The materiality is determined with reference to the relevant benchmark loss before tax (4.07%). We consider loss before tax as the most appropriate benchmark based on our analysis of the common information needs of users of the financial statements and stakeholders of the Company. We have also taken into account misstatements and/or possible misstatements that in our opinion are material for the users of the financial statements for qualitative reasons.

We agreed with the Board of Directors that misstatements identified during our audit in excess of USD 325 thousand would be reported to them, as well as smaller misstatements that in our view must be reported on qualitative grounds.

Scope of the group audit

uniQure N.V. is at the head of a group of components (hereafter "Group"). The financial information of this group is included in the financial statements of uniQure N.V.

We performed risk assessment procedures throughout our audit to determine which of the Group's components are likely to include risks of material misstatement to the Group financial statements. To appropriately respond to those assessed risks, we planned and performed further audit procedures, either at component level or centrally. We identified three components associated with a risk of material misstatement. We, as group auditor, audited all components and risks of material misstatement. We set component performance materiality levels considering the component's size and risk profile.

We have performed substantive procedures for 87% of Group total assets (2024: 81%) and 83% of Group total expenses (2024: 73%). At group level, we assessed the aggregation risk in the remaining financial information and concluded that there is less than reasonable possibility of a material misstatement.

We consider that the scope of our group audit forms an appropriate basis for our audit opinion. Through performing the procedures mentioned above we obtained sufficient and appropriate audit evidence about the Group's financial information to provide an opinion on the financial statements as a whole.

Audit response to the risk of fraud and non-compliance with laws and regulations

In chapter 3, Risk management of the Report of the Board of Directors, the Board of Directors describes its procedures in respect of the risk of fraud and non-compliance with laws and regulations.

As part of our audit, we have gained insights into the Company and its business environment and the Company's risk management in relation to fraud and non-compliance.

Our procedures included, among other things, assessing the Company's code of conduct, whistleblowing procedures, incidents register and its procedures to investigate indications of possible fraud and non-compliance. Furthermore, we performed relevant inquiries with the Board of Directors and other relevant functions, such as Chief Executive Officer, Chief Financial Officer, Chief Legal Officer, and the Vice President Global Controller. We have involved forensic specialists in our audit procedures. And, we have also incorporated elements of unpredictability in our audit, such as performing substantive procedures on selected account balances and assertions not otherwise tested due to their materiality or risk.

As a result from our risk assessment, we identified the following laws and regulations as those most likely to have a material effect on the financial statements in case of non-compliance:

- pharmaceutical and intellectual property laws and regulations (reflecting the Company's involvement in the development and manufacturing of gene therapies); and
- anti-bribery and corruption laws and regulations (reflecting the Company's activities in high-risk jurisdictions including a US footprint).

Our procedures did not result in the identification of a reportable risk of material misstatement in respect of non-compliance with laws and regulations.

Further, we assessed the presumed fraud risk on revenue recognition as not significant, because there is limited perceived incentive, pressure and opportunity to fraudulently recognize revenue. The Company recognizes limited license and collaboration revenue.

Based on the above and on the auditing standards, we identified the following fraud risks that are relevant to our audit, including the relevant presumed risks laid down in the auditing standards, and responded as follows:

- **Management override of controls (a presumed risk)**

Risk:

- Management is in a unique position to manipulate accounting records and prepare fraudulent financial statements by overriding controls that otherwise appear to be operating effectively.

Responses:

- We evaluated the design and the implementation and, where considered appropriate, tested the operating effectiveness of internal controls that mitigate fraud risks, such as processes related to journal entries and estimates.
- As part of the fraud risk assessment, we performed a data analysis of the journal entries population to determine if high-risk criteria for testing applies and evaluated relevant estimates and judgments for bias by the Company's management. Where we identified instances of

unexpected journal entries or other risks through our data analysis, we performed additional audit procedures to address each identified risk, including testing of transactions back to source information.
- We identified and selected journal entries and other adjustments made at the end of the reporting period for testing.

Our evaluation of procedures performed related to fraud did not result in a key audit matter.

We communicated our risk assessment, audit responses and results to management and the Audit Committee of the Board of Directors.

Our audit procedures did not reveal indications and/or reasonable suspicion of fraud and non-compliance that are considered material for our audit.

Audit response to going concern

The Board of Directors has performed its going concern assessment and has not identified any going concern risk. To evaluate the Board of Directors' assessment, we performed, inter alia, the following procedures:

- we considered whether the Board of Directors' assessment of the going concern risks includes all relevant information of which we are aware as a result of our audit;
- we analyzed the financial position of the Company as at year-end and compared it to the previous financial year in terms of indicators that could identify going concern risks; and
- we inquired with the Chief Financial Officer on the key assumptions and principles underlying the Board of Directors' assessment of the going concern risks.

The outcome of our risk assessment procedures on the going concern assessment, including our consideration of findings from our audit procedures on other areas, did not give reason to perform additional audit procedures on the Board of Directors' going concern assessment.

Our key audit matters

Key audit matters are those matters that, in our professional judgement, were of most significance in our audit of the financial statements. Key audit matters are not a comprehensive reflection of all matters discussed. We have communicated to the Board of Directors, that we determined that there were no key audit matters.

Compared to last year the key audit matter with respect to *the valuation of the intangible asset at initial recognition arising from favorable terms in the Commercial Supply Agreement* is not included, as this specifically relates to the financial year 2024.

Compliance with Regulatory Technical Standard of SBR, including XBRL tagging, not audited

The statutory audit includes verifying that the prepared financial statements comply with the legal requirements under Title 9 of Book 2 of the Dutch Civil Code. Our audit opinion has been issued on the prepared financial statements and will be attached to the digitally filed annual report. This means that compliance with all requirements of the Regulatory Technical Standard within the SBR domain for the Trade Register (including the applied eXtensible Business Reporting Language (XBRL) tags) was not part of the statutory audit.

Report on the other information included in the annual report

In addition to the financial statements and our auditor's report thereon, the annual report contains other information.

Based on the following procedures performed, we conclude that the other information:

- is consistent with the financial statements and does not contain material misstatements; and
- contains the information as required by Part 9 of Book 2 of the Dutch Civil Code for the management report and other information.

We have read the other information. Based on our knowledge and understanding obtained through our audit of the financial statements or otherwise, we have considered whether the other information contains material misstatements.

By performing these procedures, we comply with the requirements of Part 9 of Book 2 of the Dutch Civil Code and the Dutch Standard 720. The scope of the procedures performed is less than the scope of those performed in our audit of the financial statements.

The Board of Directors of uniQure N.V. is responsible for the preparation of the other information, including the information as required by Part 9 of Book 2 of the Dutch Civil Code.

Report on other legal and regulatory requirements

Engagement

We were initially appointed by the General Meeting of Shareholders as auditor of uniQure N.V. on 10 May 2019 as of the audit for the year 2019 and have operated as statutory auditor ever since that financial year.

Description of responsibilities regarding the financial statements

Responsibilities of the Board of Directors of uniQure N.V. for the financial statements

The Board of Directors of uniQure N.V. is responsible for the preparation and fair presentation of the financial statements in accordance with EU-IFRS and Part 9 of Book 2 of the Dutch Civil Code. Furthermore, the Board of Directors of uniQure N.V. is responsible for such internal control as management determines is necessary to enable the preparation of the financial statements that are free from material misstatement, whether due to fraud or error. In that respect the Board of Directors of uniQure N.V., is responsible for the prevention and detection of fraud and non-compliance with laws and regulations, including determining measures to resolve the consequences of it and to prevent recurrence.

As part of the preparation of the financial statements, the Board of Directors of uniQure N.V. is responsible for assessing the Company's ability to continue as a going concern. Based on the financial reporting frameworks mentioned, the Board of Directors of uniQure N.V. should prepare the financial statements using the going concern basis of accounting unless the Board of Directors of uniQure N.V. either intends to liquidate the Company or to cease operations, or has no realistic alternative but to do so. The Board of Directors of uniQure N.V. should disclose events and circumstances that may cast significant doubt on the company's ability to continue as a going concern in the financial statements.

The Audit Committee of the Board of Directors is responsible for overseeing the Company's financial reporting process.

Our responsibilities for the audit of the financial statements

Our objective is to plan and perform the audit engagement in a manner that allows us to obtain sufficient and appropriate audit evidence for our opinion.

Our audit has been performed with a high, but not absolute, level of assurance, which means we may not detect all material errors and fraud during our audit.

Misstatements can arise from fraud or error and are considered material if, individually or in the aggregate, they could reasonably be expected to influence the economic decisions of users taken on the basis of these financial statements. The materiality affects the nature, timing and extent of our audit procedures and the evaluation of the effect of identified misstatements on our opinion.

A further description of our responsibilities for the audit of the financial statements is included in the appendix of this auditor's report. This description forms part of our auditor's report.

Amstelveen, 24 April 2026

KPMG Accountants N.V.

J.R.M. Geerts RA

Appendix:
Description of our responsibilities for the audit of the financial statements

Appendix

Description of our responsibilities for the audit of the financial statements

We have exercised professional judgement and have maintained professional scepticism throughout the audit, in accordance with Dutch Standards on Auditing, ethical requirements and independence requirements. Our audit included among others:

- identifying and assessing the risks of material misstatement of the financial statements, whether due to fraud or error, designing and performing audit procedures responsive to those risks, and obtaining audit evidence that is sufficient and appropriate to provide a basis for our opinion. The risk of not detecting a material misstatement resulting from fraud is higher than the risk resulting from error, as fraud may involve collusion, forgery, intentional omissions, misrepresentations, or the override of internal control;
- obtaining an understanding of internal control relevant to the audit in order to design audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control;
- evaluating the appropriateness of accounting policies used and the reasonableness of accounting estimates and related disclosures made by the Board of Directors of uniQure N.V.;
- concluding on the appropriateness of the Board of Directors of uniQure N.V.'s use of the going concern basis of accounting, and based on the audit evidence obtained, whether a material uncertainty exists related to events or conditions that may cast significant doubt on the Company's ability to continue as a going concern. If we conclude that a material uncertainty exists, we are required to draw attention in our auditor's report to the related disclosures in the financial statements or, if such disclosures are inadequate, to modify our opinion. Our conclusions are based on the audit evidence obtained up to the date of our auditor's report. However, future events or conditions may cause the Company to cease to continue as a going concern;
- evaluating the overall presentation, structure and content of the financial statements, including the disclosures; and
- evaluating whether the financial statements represent the underlying transactions and events in a manner that achieves fair presentation.

We are responsible for planning and performing the group audit to obtain sufficient appropriate audit evidence regarding the financial information of the entities or business units within the group as a basis for forming an opinion on the financial statements. We are also responsible for the direction, supervision and review of the audit work performed for purposes of the group audit. We bear the full responsibility for the auditor's report.

We communicate with the Audit Committee of the Board of Directors regarding, among other matters, the planned scope and timing of the audit and significant audit findings, including any significant findings in internal control that we identify during our audit.

We provide the Audit Committee of the Board of Directors with a statement that we have complied with relevant ethical requirements regarding independence, and to communicate with them all relationships and other matters that may reasonably be thought to bear on our independence, and where applicable, related safeguards.

From the matters communicated with the Audit Committee of the Board of Directors, we determine the key audit matters: those matters that were of most significance in the audit of the financial statements. We describe these matters in our auditor's report unless law or regulation precludes public disclosure about the matter or when, in extremely rare circumstances, not communicating the matter is in the public interest.