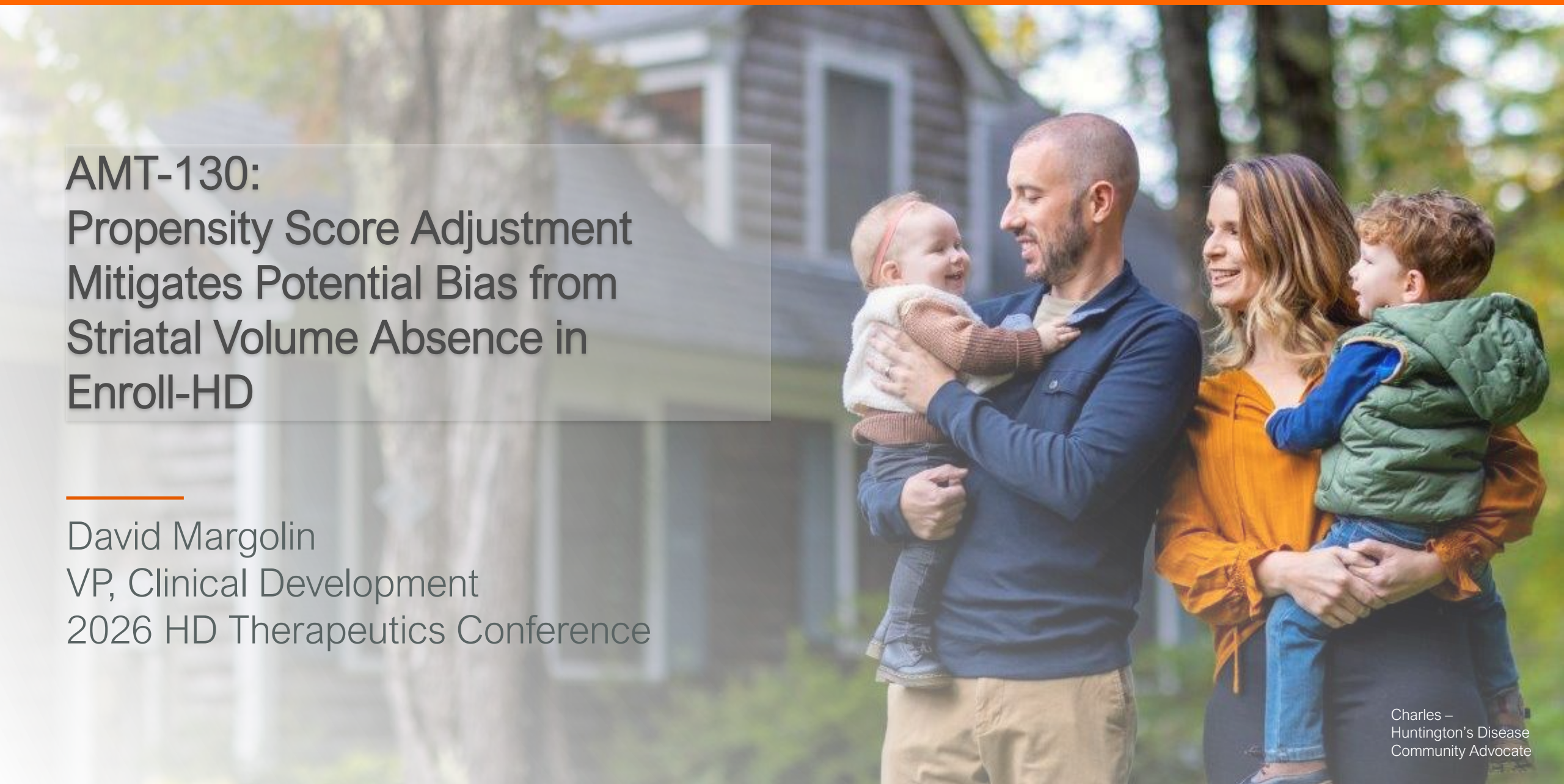
uniQure

AMT-130: Propensity Score Adjustment Mitigates Potential Bias from Striatal Volume Absence in Enroll-HD

David Margolin
VP, Clinical Development
2026 HD Therapeutics Conference



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Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements and, except as required by law, we assume no obligation to update these forward-looking statements to reflect events that occur or circumstances that exist after the date on which they were made.

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Author Disclosures

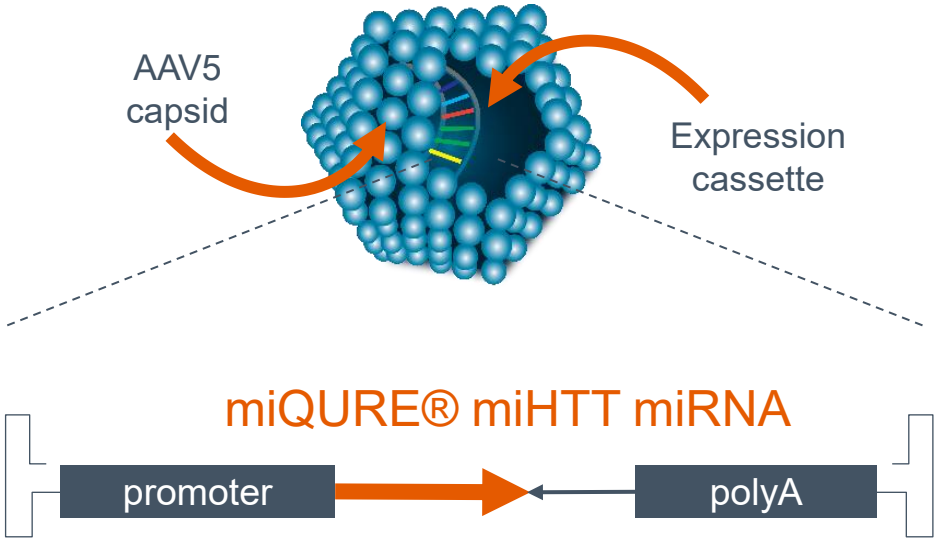
David Margolin, Kenechi Ejebe, Wenfei Zhang, and Na Wang are employees of uniQure, Inc., and own stock or stock options

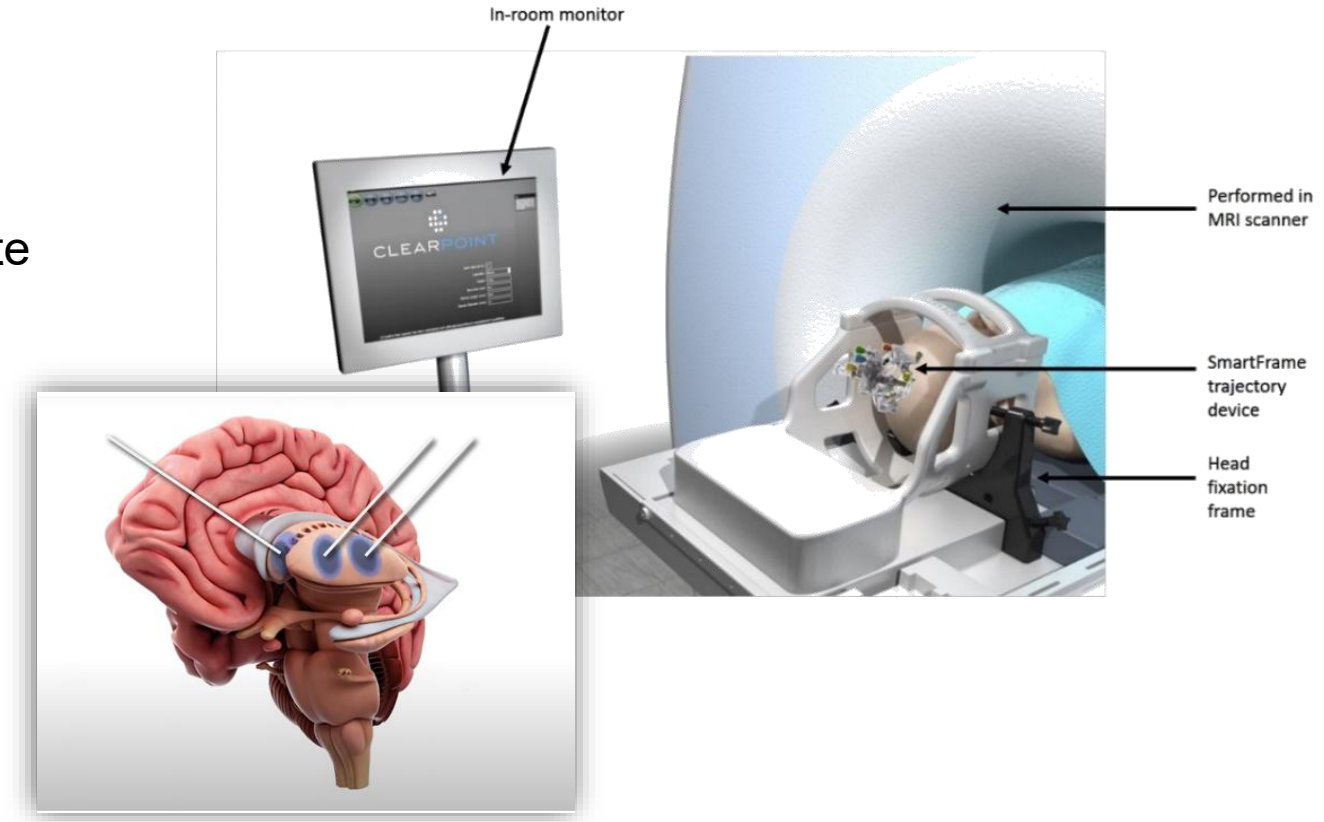
Walid Abi-Saab is an employee of uniQure Switzerland GmbH and owns stock or stock options

AMT-130 is an investigational agent currently being studied in the treatment of Huntington's Disease. Its safety and efficacy have not been established and it has not been approved by the United States Food and Drug Administration (FDA), European Medicines Agency (EMA), or any other regulatory body. There is no guarantee that investigational agents will receive health authority approval or become commercially available.

AMT-130 Overview

AMT-130 is an investigational gene therapy candidate for the treatment of Huntington's disease[1,2]





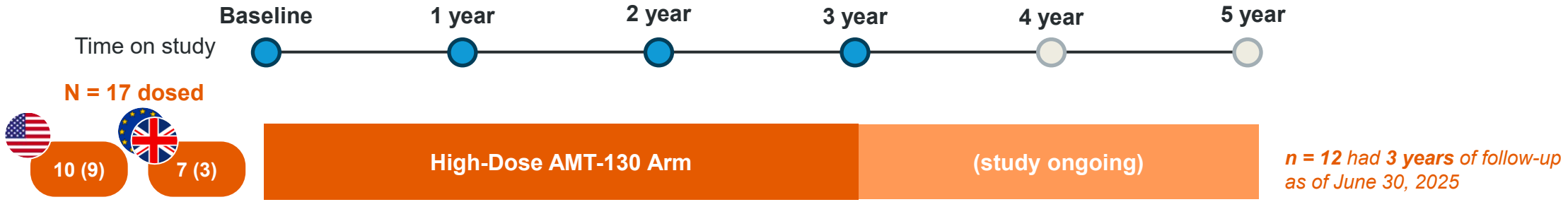
One-time administration via MRI-guided, convection-enhanced stereotactic neurosurgical delivery directly into the striatum (caudate and putamen)[2,3]

- 3 infusions per hemisphere
- AMT-130 is axonally transported to other brain regions affected by HD

Abbreviations: AAV, adeno-associated virus; HD, Huntington's disease; HTT, huntingtin; mi; micro; MRI, magnetic resonance imaging; polyA, polyadenylation.

References: 1. Sogorb-Gonzalez M, et al. *Brain*. 2024;147(12):4043-4055. 2. Cooper DL. Presented at Huntington's Study Group Virtual Meeting; October 20, 2021. 3. Huang S, et al. *Med-X*. 2023;1(1).

Study Design for AMT-130 Pivotal Phase I/II 3-year Analysis: High-Dose Cohort[1]



Key eligibility criteria

- Aged 25-65 years
- Diagnostic confidence level: 3 or 4*
- Total functional capacity: 9-13
- HD medications stable prior 3 months
- Striatal volumes exceed specified minima
  - Putamen: ≥ 2.5 cm^3/side
  - Caudate: ≥ 2.0 cm^3/side

*DCL 4, or DCL 3 if individual also meets definition of multidimensional manifest HD or has cognitive symptoms
Abbreviations: DCL, diagnostic confidence level; HD, Huntington's disease.
Reference: 1. https://clinicaltrials.gov/study/NCT04120493

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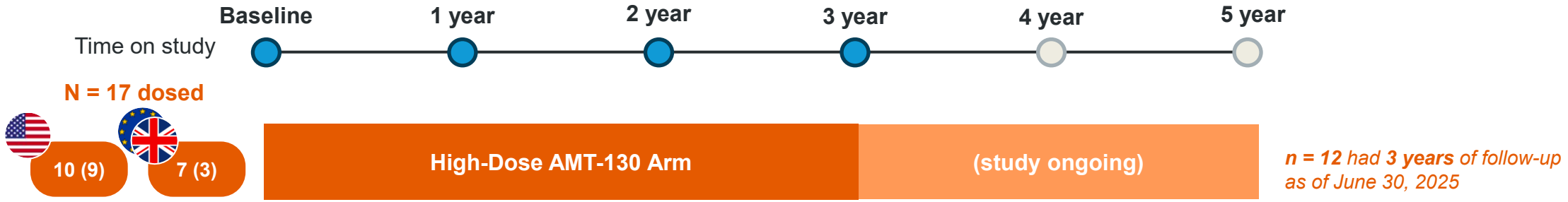

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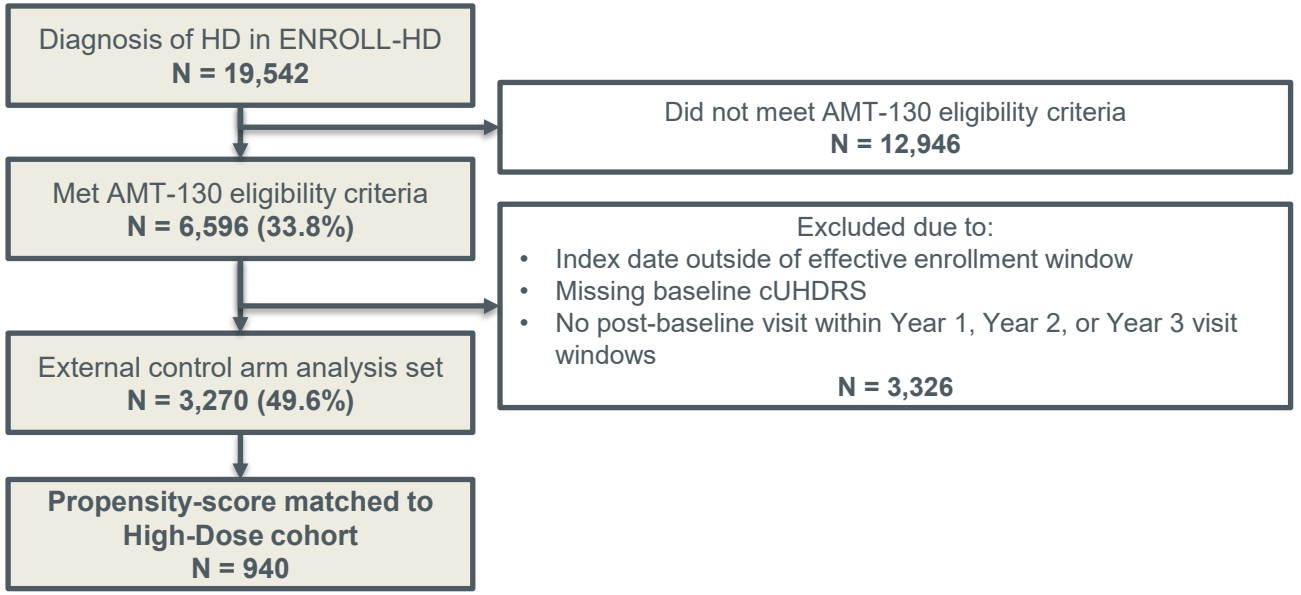
Study Design for AMT-130 Pivotal Phase I/II 3-year Analysis: External Comparator Cohort[1]

ENROLL-HD Matched External Control Arm (N = 940)
Propensity score matched to AMT-130

***n = 583** from Enroll-HD had **3 years** of post-eligibility follow-up*

ENROLL-HD external comparator dataset[2]

- Prospective data collection, largely contemporaneous with AMT-130 studies
- Large size, diverse dataset
- Aligns with clinical measures collected in AMT-130 studies (eg, cUHDRS, TFC, SDMT, SWRT, TMS)
- **MRI data were not collected as part of Enroll-HD protocol**



Abbreviations: BL, baseline; cUHDRS, composite Unified Huntington's Disease Rating Scale; HD, Huntington's disease; MRI, magnetic resonance imaging; SDMT, Symbol Digit Modalities Test; SWRT, Stroop Word Reading Test; TFC, Total Functioning Capacity; TMS, Total Motor Score.

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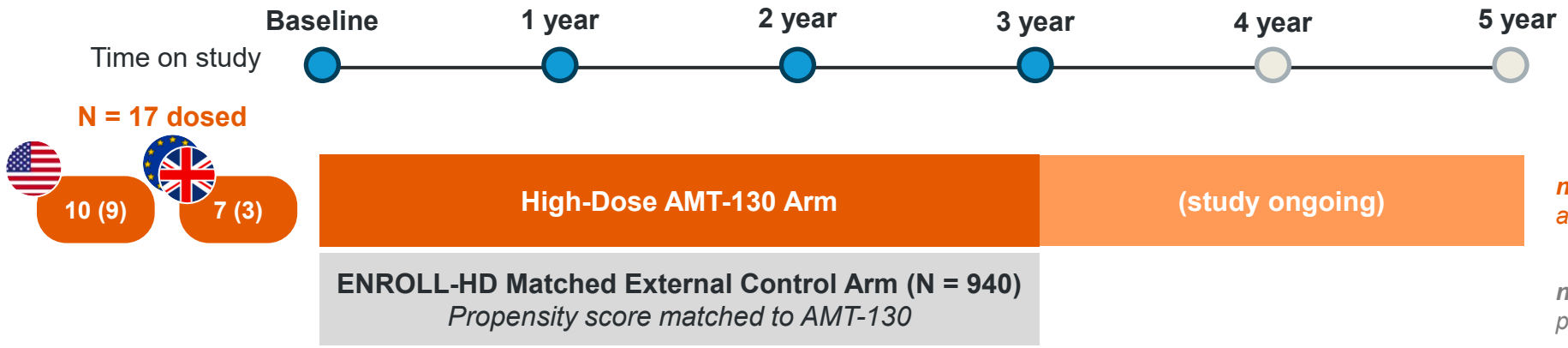

*n = 12 had **3 years** of follow-up as of June 30, 2025*

*n = 583 from Enroll-HD had **3 years** of post-eligibility follow-up*

ENDPOINTS

PRIMARY
Composite Unified Huntington's Disease Rating Scale (cUHDRS)

SECONDARY
- **Total Functional Capacity (TFC)**
- **Symbol Digit Modalities Test (SDMT)**
- **Stroop Word Reading Test (SWRT)**
- **Total Motor Score (TMS)**

SUPPORTIVE
Cerebrospinal fluid (CSF) neurofilament light chain (NfL) change from baseline to 3 years

Change from baseline at 3 years vs Enroll-HD propensity score-matched external control

Abbreviations: BL, baseline; cUHDRS, composite Unified Huntington's Disease Rating Scale; HD, Huntington's disease; MRI, magnetic resonance imaging; SDMT, Symbol Digit Modalities Test; SWRT, Stroop Word Reading Test; TFC, Total Functioning Capacity; TMS, Total Motor Score.

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Propensity Score Matching (PSM)

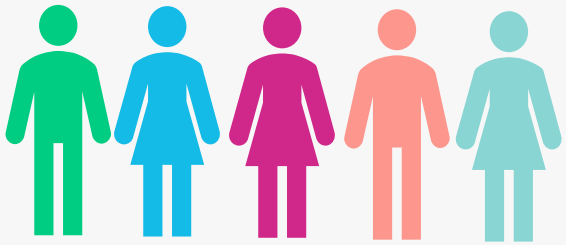
Abbreviations: PSM, propensity score matching

Reference: Liau MYQ, et al. *World J Methodol.* 2024 Mar 20;14(1):90590.

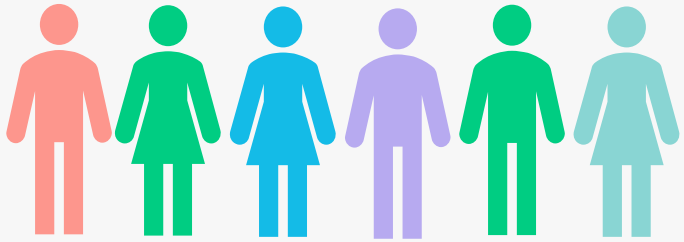
Propensity Score Matching (PSM)

Original, **unadjusted** population

Clinical trial participants



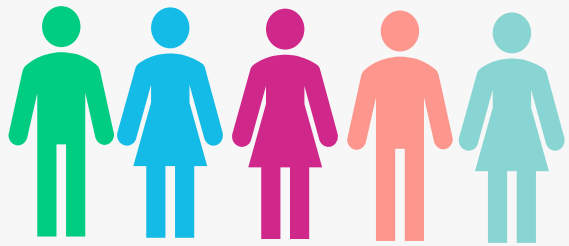
Real-world participants



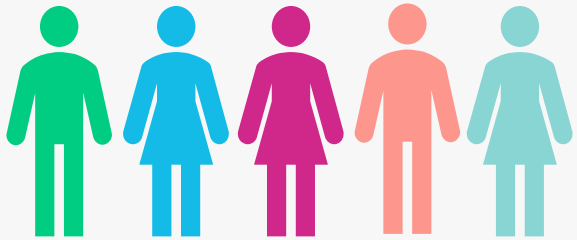
PSM Modeling

PSM-**adjusted** population

Clinical trial participants



Real-world participants

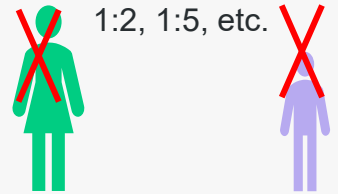


Adjustment summary

Trial participants are matched to people with the most similar propensity score.

Individuals with dissimilar propensity scores are excluded.

Matching can be 1:1 or (for more statistical power) 1:2, 1:5, etc.



Abbreviations: PSM, propensity score matching

Reference: Liau MYQ, et al. *World J Methodol.* 2024 Mar 20;14(1):90590.

Baseline Demographics and Disease Characteristics of High-Dose Participants and Propensity-Matched Controls

Demographics and Disease Characteristics Mean	**AMT-130 High-Dose (N = 17)**	**PSM External Control (Enroll-HD) (N = 940)**
Sex, Males (%)	47.1	55.6
Age, years	45.8	45.2
CAG repeats	42.4	42.8
CAP100 score	86.2	86.8
DCL = 3, 4 (%)	35.3, 64.7	30.5, 69.5
PIN Score	0.77	0.81
cUHDRS	14.9	14.7
TFC	12.2	12.1
SDMT	46.1	45.3
SWRT	89.9	87.6
TMS	12.1	11.6
HD-ISS Stage 2, 3 (%)	47.1, 52.9	51.6, 48.4
Region; No. US, Other (%)	58.8, 41.2	28.9, 71.1

Notes: Primary analysis used restricted full matching with matching weights for Enroll-HD data.
Propensity score covariates are shown in **orange bold** font

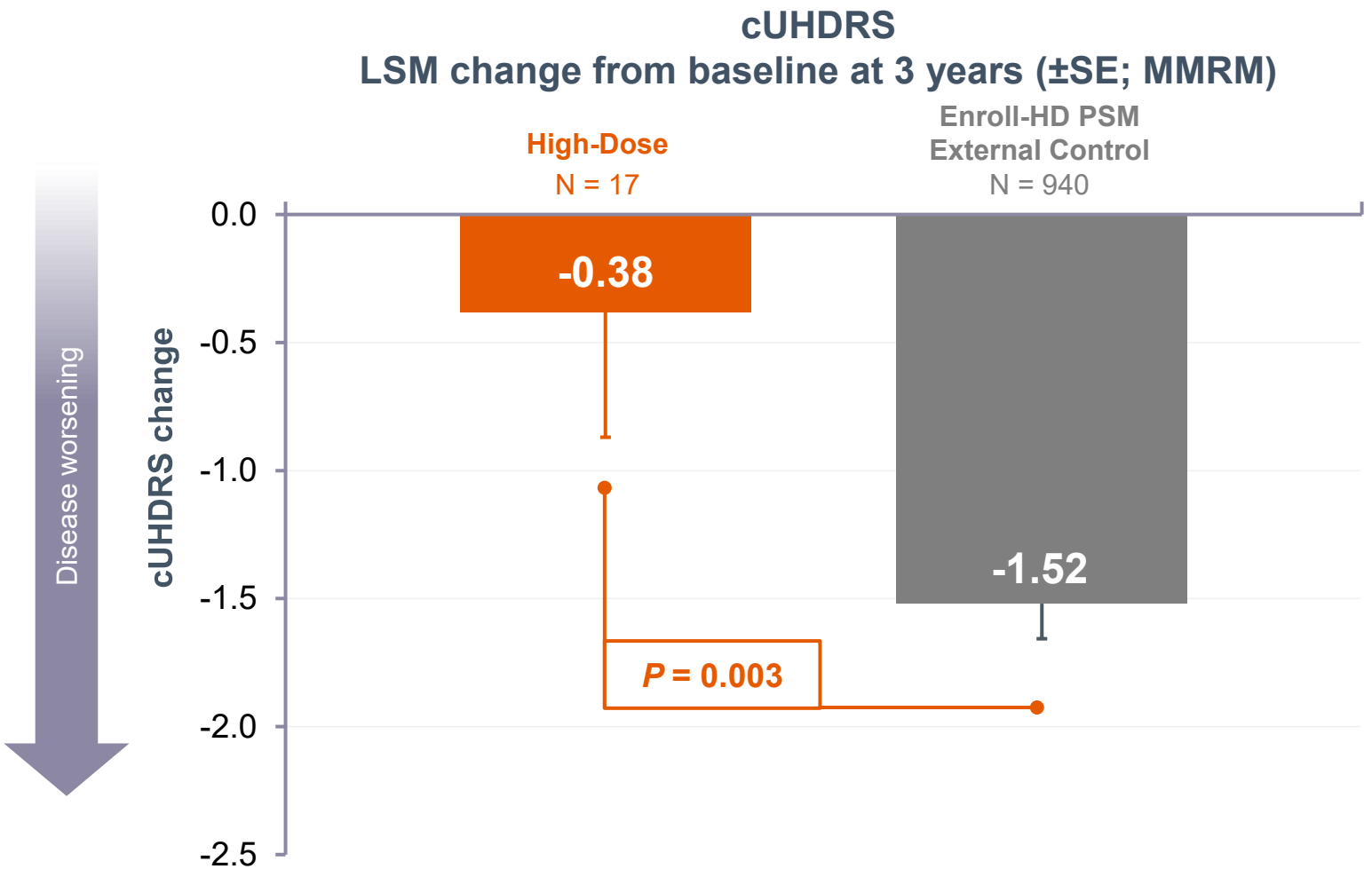
Abbreviations: CAG: Cytosine-adenine-guanine CAP, CAG-Age-Product; cUHDRS, composite Unified Huntington's Disease Rating Scale; DCL, diagnostic confidence level; HD-ISS, HD Integrated Staging System; PIN, Prognostic Index; PSM, propensity score matching; SDMT, Symbol Digit Modalities Test; SMD, standardized mean difference; SWRT, Stroop Word Reading Test; TFC, Total Functioning Capacity; TMS, Total Motor Score.

Reference: Sung V, et al. AMT-130 Huntington's disease gene therapy: Clinical study outcomes at 3 years. Huntington's Study Group, Nashville TN, Oct 10-13 2025.

Primary Endpoint: cUHDRS at 3 Years

At 3 years:
High-dose AMT-130 **significantly reduced disease progression by 75% based on cUHDRS** compared with propensity score-matched external control (P=0.003)

Participants	Baseline	3 years
AMT-130 High-Dose	17	12
PSM External Control	940	568

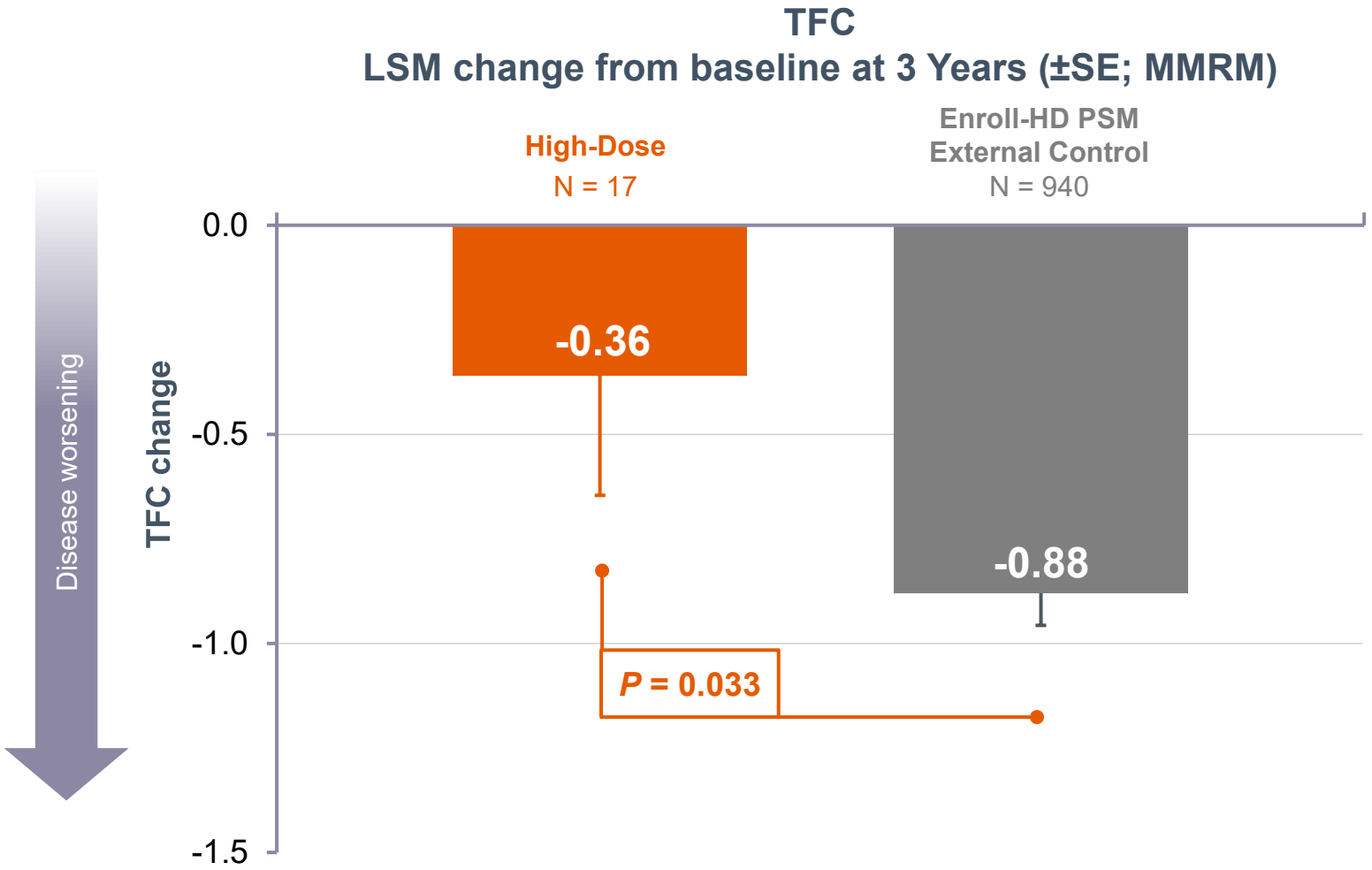


Abbreviations: cUHDRS, composite Unified Huntington's Disease Rating Scale; HD, Huntington's disease; LSM, least squares mean; MMRM, mixed models for repeated measures; PSM, propensity score-matched; SE, standard error.
Reference: Sung V, et al. AMT-130 Huntington's disease gene therapy: Clinical study outcomes at 3 years. Huntington's Study Group, Nashville TN, Oct 10-13 2025.

Key Secondary Endpoint: TFC at 3 Years

At 3 years:
High-dose AMT-130 **significantly reduced disease progression by 60% based on TFC** compared with propensity score-matched external control (P=0.033)

Participants	Baseline	3 years
AMT-130 High-Dose	17	12
PSM External Control	940	583



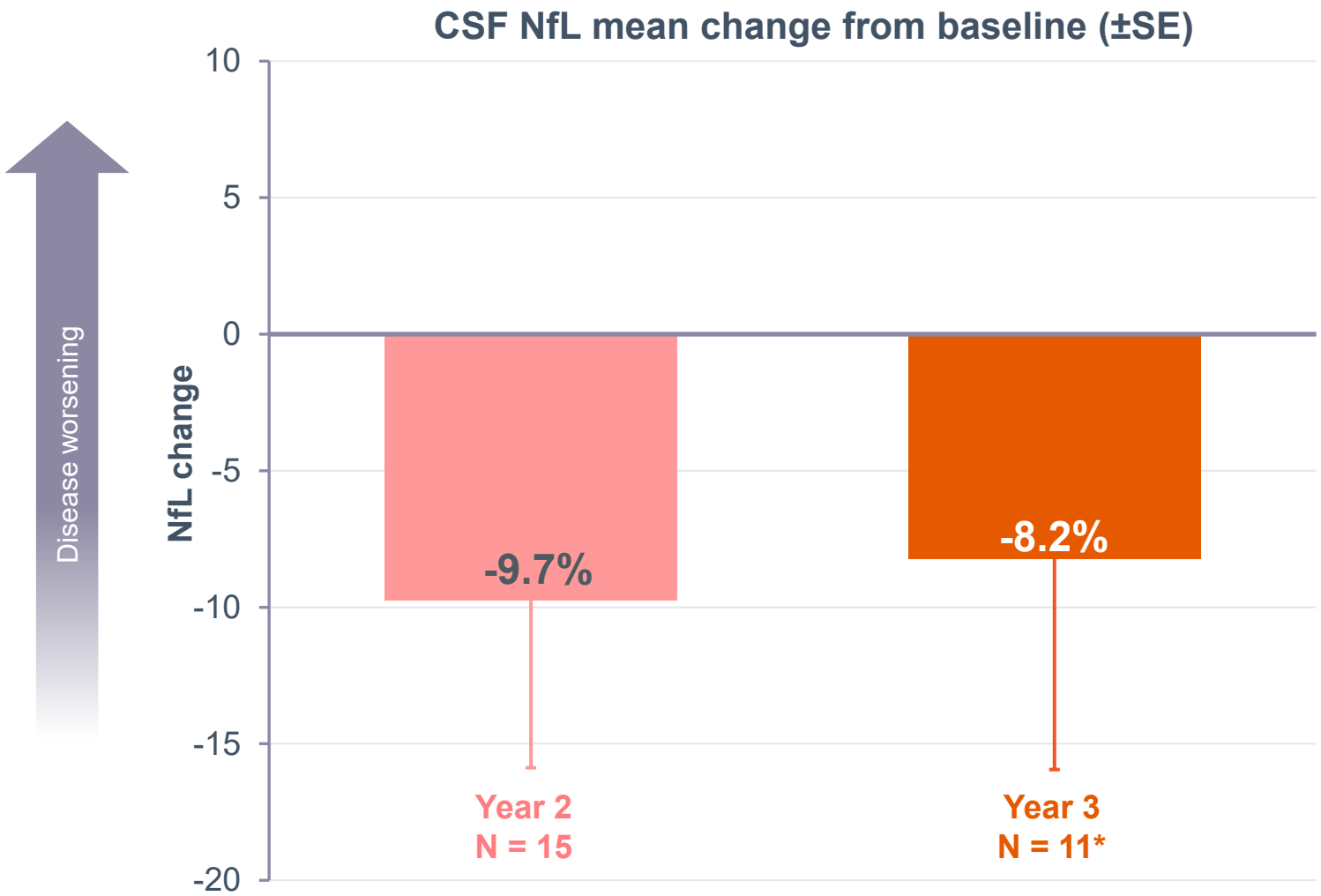
Above graph represents weighted observed data

Abbreviations: HD, Huntington's disease; LSM, least squares mean; MMRM, mixed models for repeated measures; PSM, propensity score-matched; SE, standard error; TFC, Total Functional Capacity
Reference: Sung V, et al. AMT-130 Huntington's disease gene therapy: Clinical study outcomes at 3 years. Huntington's Study Group, Nashville TN, Oct 10-13 2025.

Supportive Endpoint: Change in CSF NfL

Mean CSF NfL were below baseline at 2 and 3 years following administration of high-dose AMT-130

In natural history studies, NfL increases 10-15% per year[1,2]



*1 of 12 participants did not receive lumbar puncture

Abbreviations: CSF, cerebrospinal fluid; NfL, neurofilament light chain; SE, standard error

References: 1. Rodrigues FB, et al. *Sci Transl Med*. 2020;12(574):eabc2888. 2. Scahill RI, et al. *Nat Med.* 2025;31(3):807-818.

Overview of Adverse Events as of June 30, 2025 Data Cutoff

	Sham Surgery (N = 10)		Low-dose AMT-130 (Cohort 1) (N = 13[a])		High-dose AMT-130 (Cohort 2) (N = 20[a])		Dose-Blinded (Cohort 3) (N = 12)		All AMT-130 (Cohorts 1, 2 and 3) (N = 45[a])	
	N	%	N	%	N	%	N	%	N	%
Any TEAEs[b]	10	100.0	12	92.3	20	100.0	12	100.0	44	97.8
Any drug-related TEAE	0	0.0	0	0.0	6	30.0	3	25.0	9	20.0
Any SAEs	1	10.0	3	23.1	9	45.0	3	25.0	15	33.3
Any SAEs (peri-operative)[c]	1	10.0	2	15.4	6	30.0	0	0.0	8	17.8
Any drug-related SAE	0	0.0	0	0.0	4[d]	20.0	0	0.0	4[d]	8.8
Most Common TEAEs (≥30% in ≥1 group)										
Headache	3	30.0	3	23.1	9	45.0	6	50.0	18	40.0
Procedural headache	5	50.0	4	30.8	10	50.0	2	16.7	16	35.6
Procedural pain	6	60.0	2	15.4	7	35.0	2	16.7	11	24.4
Post lumbar puncture syndrome	6	60.0	2	15.4	5	25.0	2	16.7	10	22.2
Procedural complication	4	40.0	4	30.8	5	25.0	0	0.0	9	20.0
Anxiety	0	0.0	0	0.0	4	20.0	4	33.3	8	17.8
Constipation	0	0.0	0	0.0	2	10.0	6	50.0	8	17.8
Insomnia	0	0.0	1	7.7	1	5.0	6	50.0	8	17.8
Back pain	1	10.0	0	0.0	0	0.0	5	41.7	5	11.1

Safety data as of June 30, 2025.
[a]One low-dose and 3 high-dose cross-over patients included. [b]TEAEs are defined as AEs after Day 0. [c]Perioperative AEs had onset from Day 0 to 13. [d]One SAE reported as "tension headache" was retrospectively considered by uniQure as a case of CNS inflammation.
Abbreviations: AE, adverse event; N, number of patients; TEAE, treatment-emergent adverse event; SAE, serious adverse event.
Reference: Sung V, et al. AMT-130 Huntington's disease gene therapy: Clinical study outcomes at 3 years. Huntington's Study Group, Nashville TN, Oct 10-13 2025.

Pivotal Phase I/II Study Summary

- **High-dose AMT-130 met primary and key secondary endpoints at 3 years**

1. Statistically significant **75%** slowing of disease progression based on **cUHDRS**
2. Statistically significant **60%** slowing of disease progression based on **TFC**
3. Reduction in CSF **NfL below baseline**
4. Continued to be **generally well tolerated** with a manageable safety profile; **no new treatment-related SAEs**
   Most common AEs (≥20%): Headache, procedural pain, post-lumbar puncture syndrome

Abbreviations: AE, adverse event; cUHDRS, composite Unified Huntington's Disease Rating Scale; MRI, magnetic resonance imaging; NfL, neurofilament light chain; SAE, severe adverse event; TFC, Total Functional Capacity.

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MRI data were not collected as part of ENROLL-HD protocol

AMT-130 had striatal volume minimum as inclusion criteria

Does baseline striatal volume predict progression, independent of other known covariates?

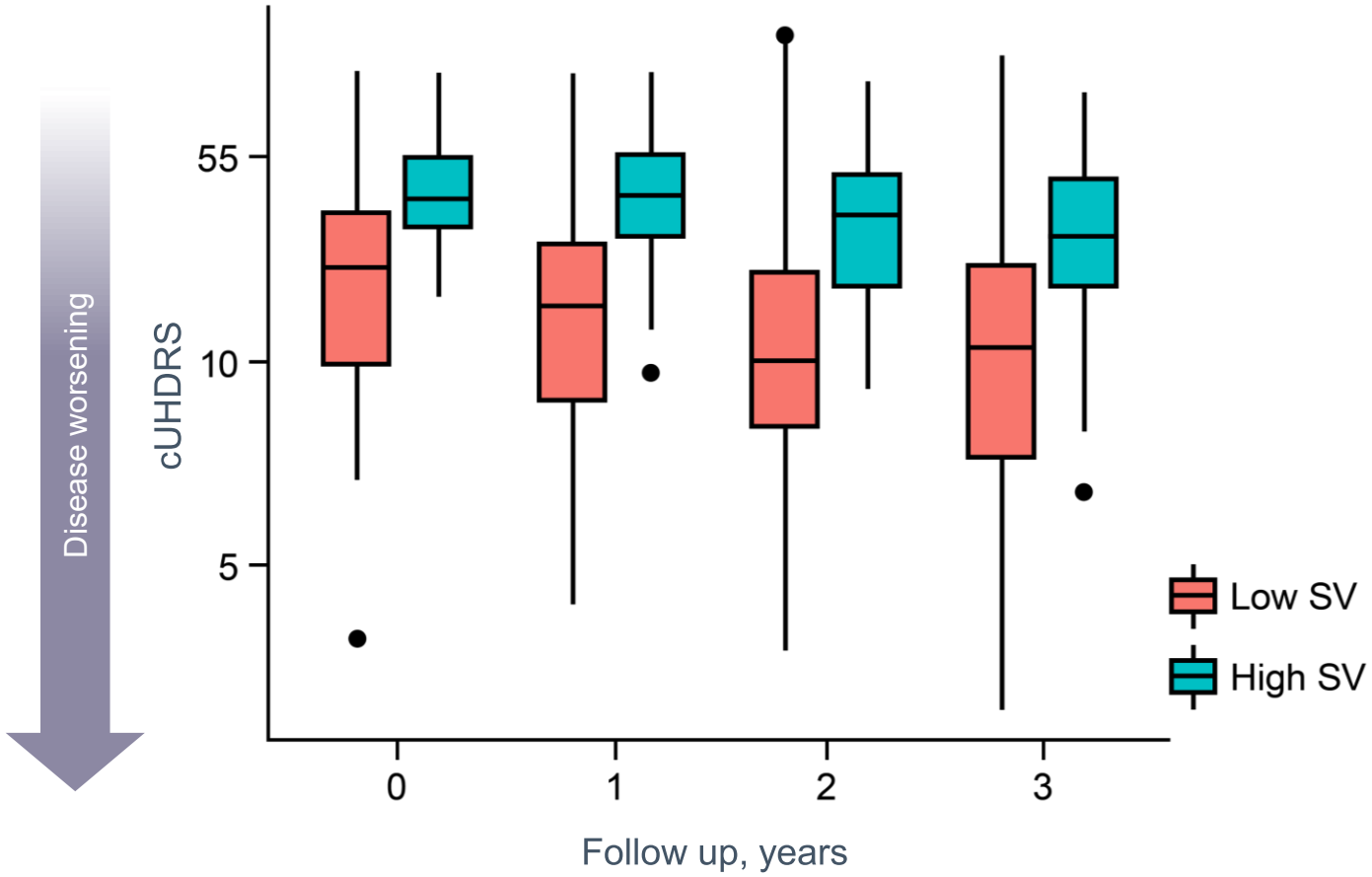
Abbreviations: AE, adverse event; cUHDRS, composite Unified Huntington's Disease Rating Scale; MRI, magnetic resonance imaging; NfL, neurofilament light chain; SAE, severe adverse event; TFC, Total Functional Capacity.

Baseline Striatal Volume is a Predictor of Clinical Progression in TRACK-HD

105 participants from TRACK-HD with appropriate clinical criteria (DCL=4; TFC 9-13; ≥1 evaluable follow-up MRI scan) with 1–5 years of follow-up, stratified by baseline imaging

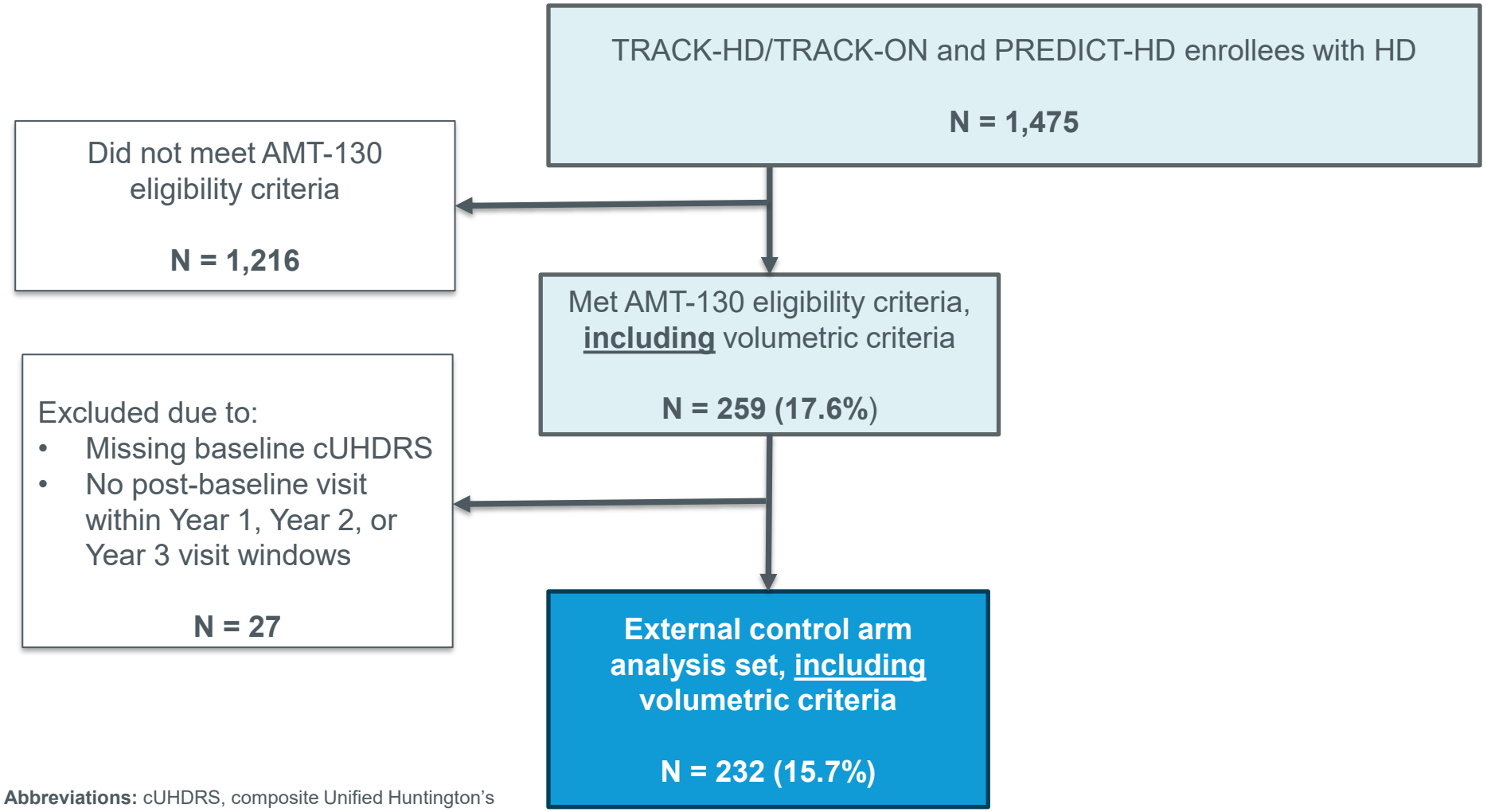
- **Low striatal volume**, n = 74
  (<2.5 cm^3 putamen, <2.0 cm^3 caudate – per side)
- **High striatal volume**, n = 31
  (≥2.5 cm^3 putamen, ≥2.0 cm^3 caudate – per side)

Without correction for other baseline characteristics, participants with **high striatal volume** tended to have **slower clinical and MRI progression**



Abbreviations: cUHDRS, composite Unified Huntington's Disease Rating Scale; DCL, diagnostic confidence level; MRI, magnetic resonance imaging; SV, striatal volume; TFC, Total Functional Capacity.
Reference: Tabrizi SJ, et al. Radiographic and clinical progression after motor diagnosis in HD is a function of initial striatal volume and functional scores: a re-analysis of TRACK-HD/TRACK-ON HD MRI images and data. Poster presented at the 17th annual HD Therapeutics Conference, Palm Springs, CA; Feb 28-Mar 3 2022.

TRACK-HD and PREDICT-HD Participants Meeting Pivotal Phase I/II AMT-130 Clinical Eligibility Criteria, with/without Volumetric Criteria



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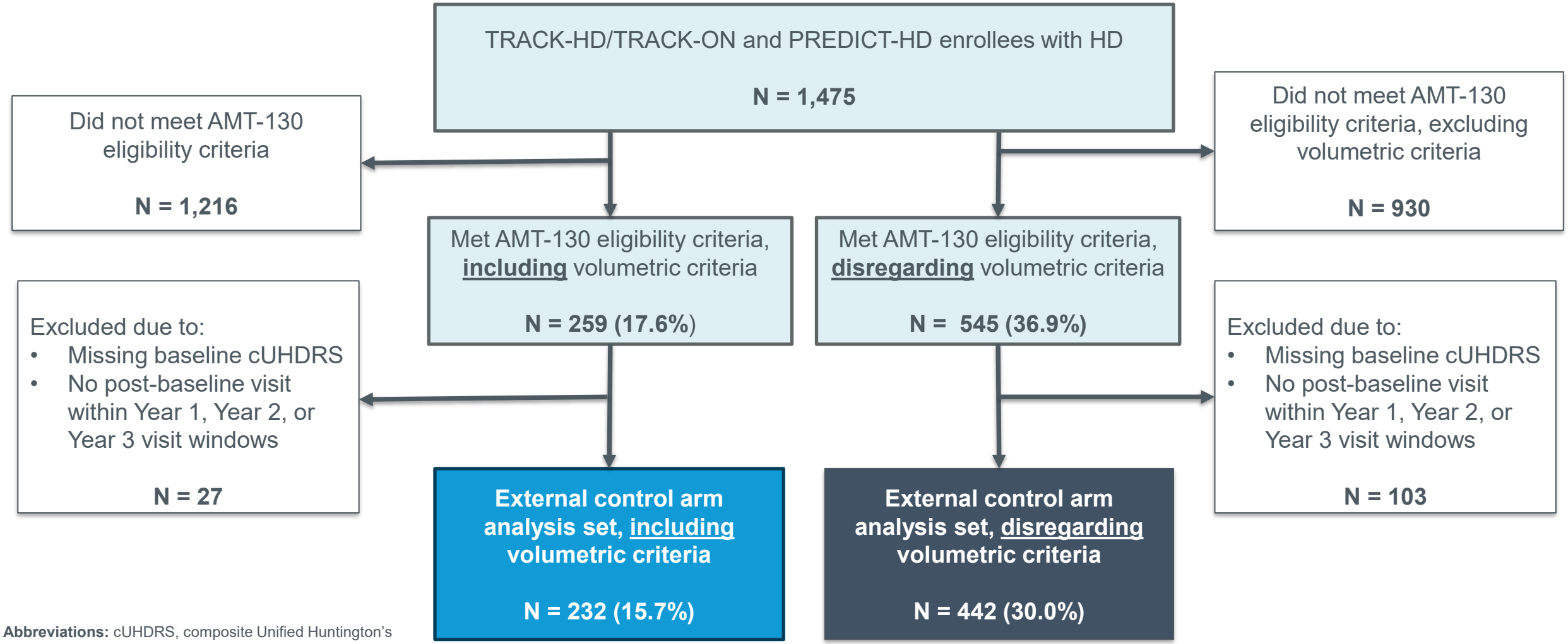
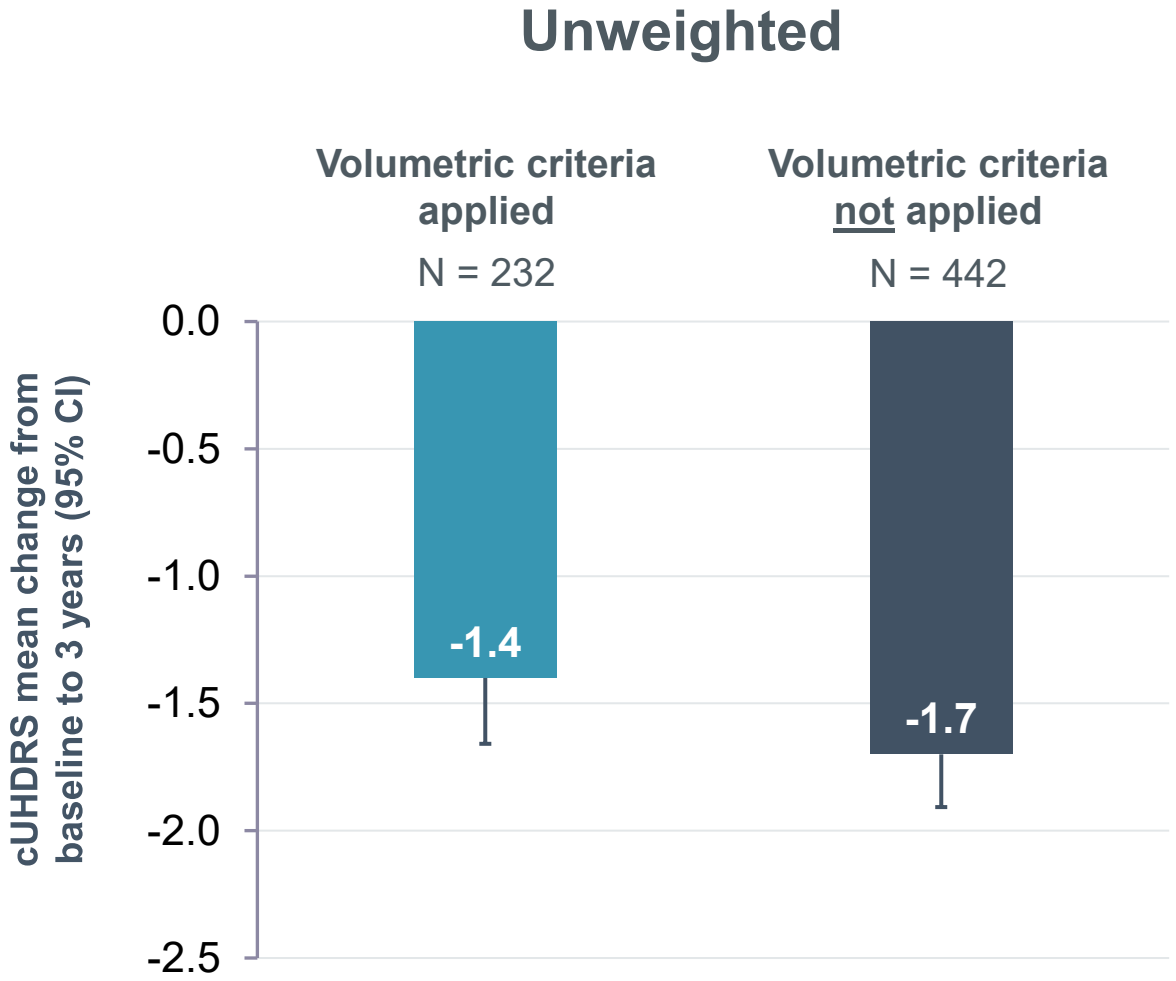


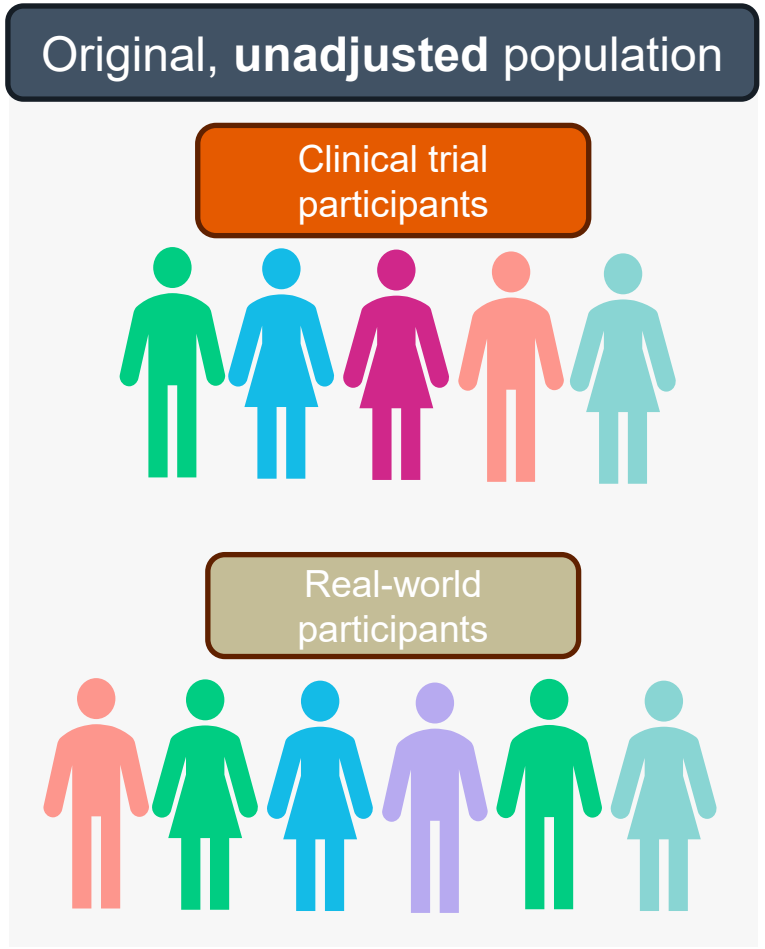
Abbreviations: cUHDRS, composite Unified Huntington's Disease Rating Scale; HD, Huntington's disease.

Baseline Volumetric Criteria Predicts cUHDRS Decline in the Absence of Other Covariates



Abbreviations: CI, confidence interval; cUHDRS, composite Unified Huntington's Disease Rating Scale

Propensity Score Weighting (PSW)



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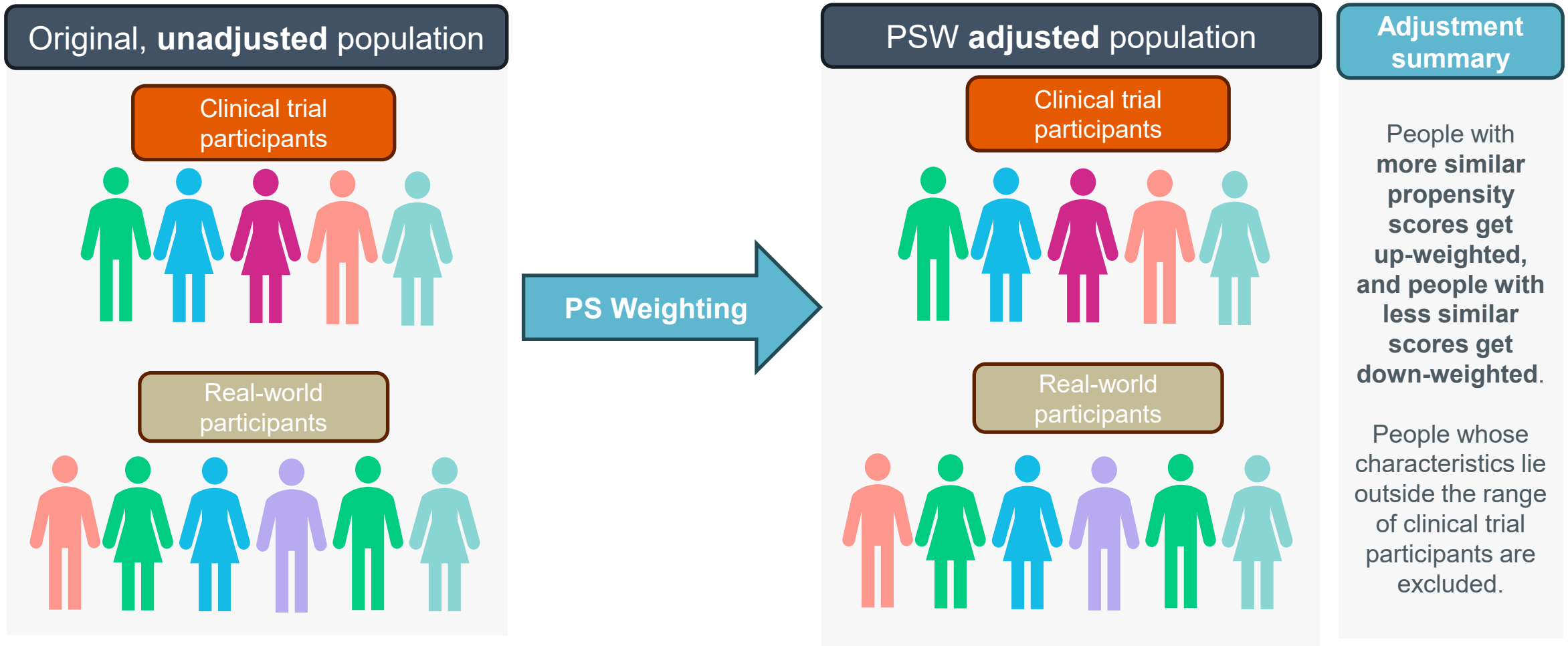

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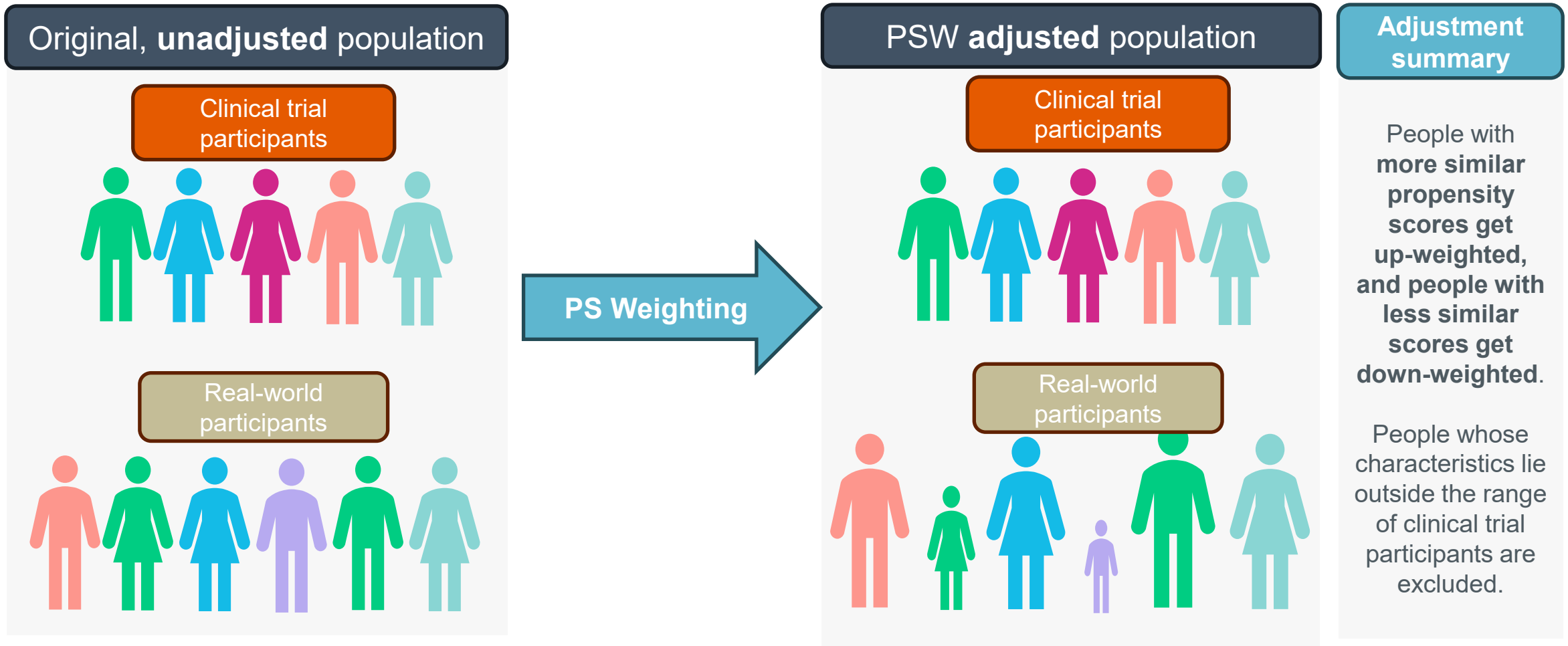


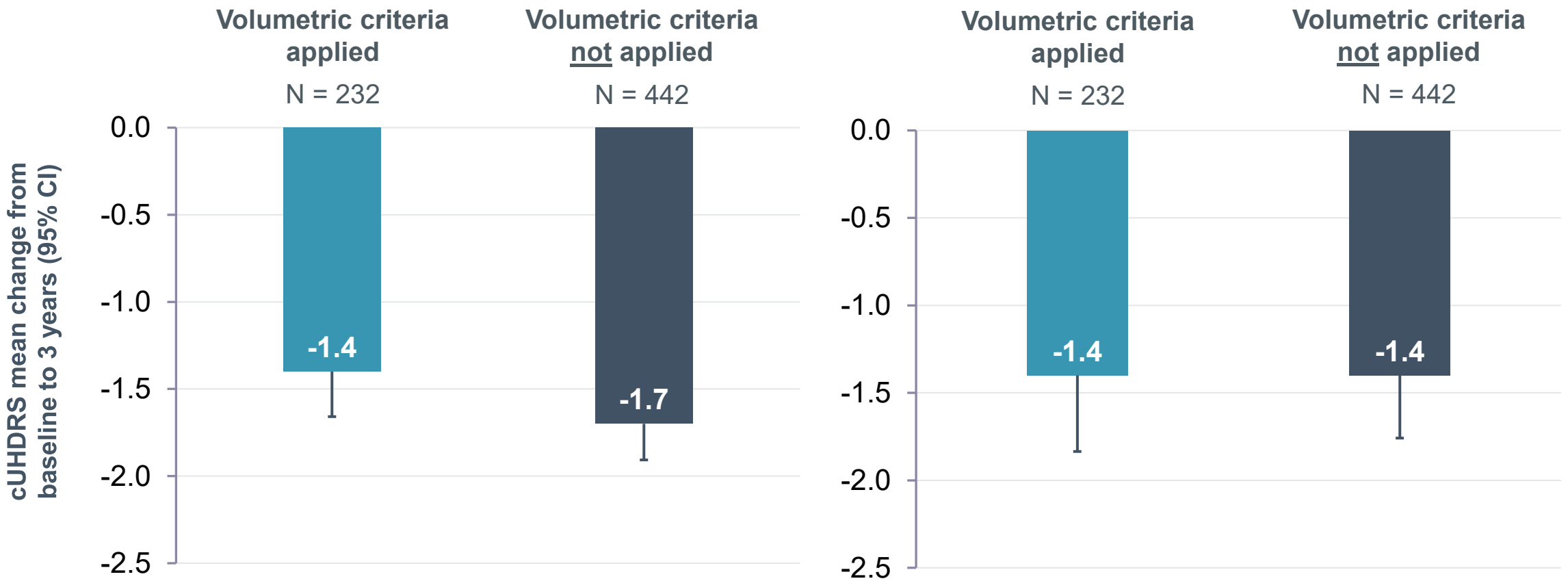
Abbreviations: PS, propensity score; PSW, propensity score weighting.

Baseline Volumetric Criteria Are No Longer Predictive of cUHDRS Decline When Propensity Score Adjustments Are Applied

Unweighted

PS Weighted

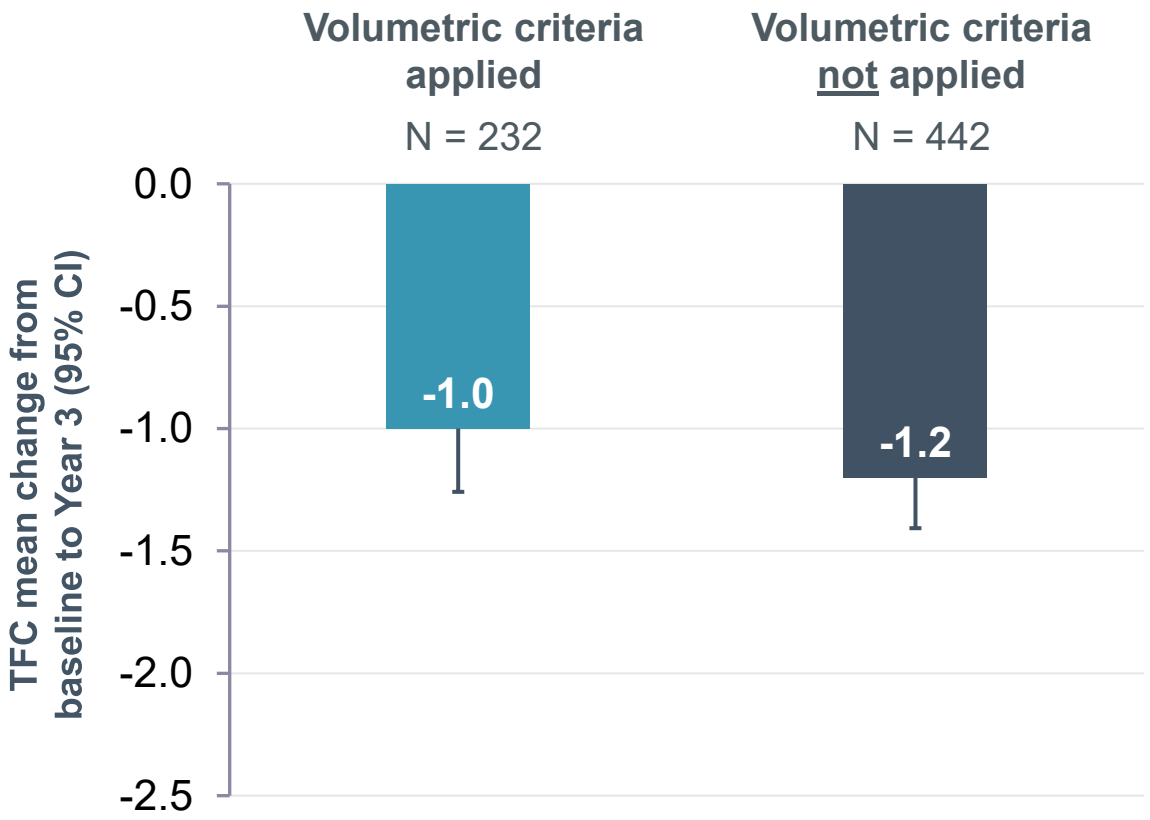
(**Covariates**: baseline age, sex, CAP100, DCL, TFC, TMS, SDMT, SWR)



Abbreviations: CAP, CAG-Age-Product; CI, confidence interval; cUHDRS, composite Unified Huntington's Disease Rating Scale; DCL, diagnostic confidence level; PS, propensity score; SDMT, Symbol Digit Modalities Test; SWR, Stroop Word Reading; TFC, Total Functional Capacity; TMS, Total Motor Score.

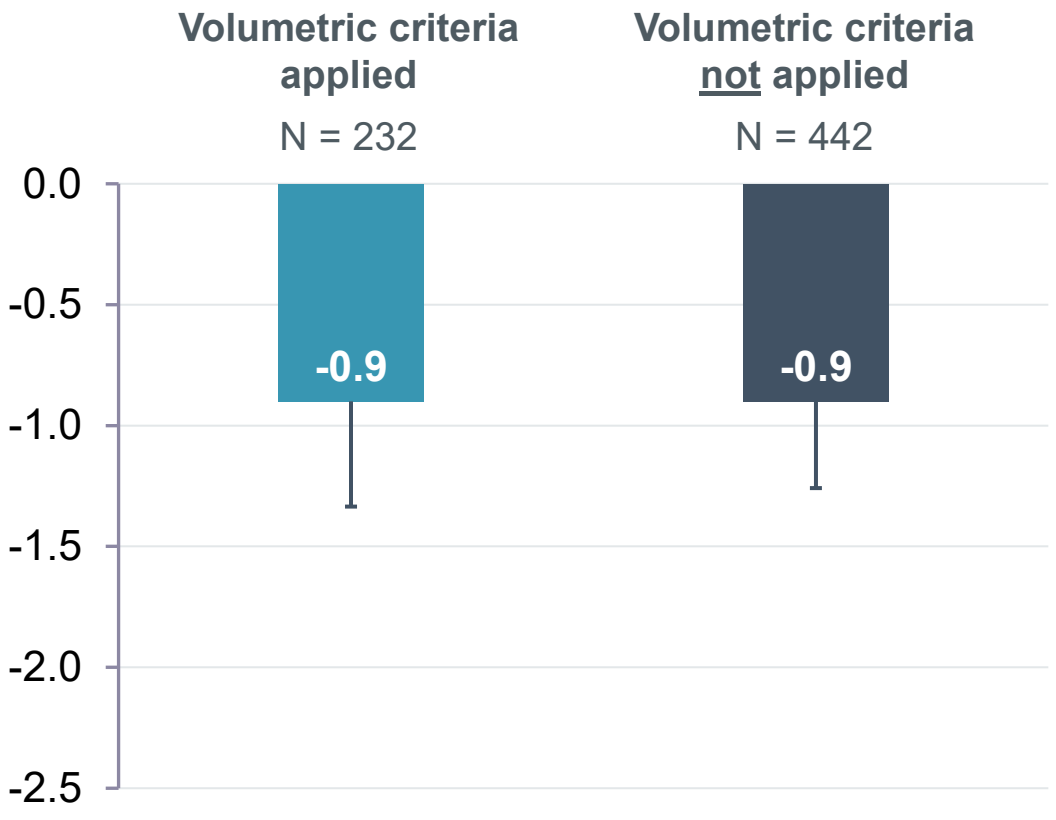
Baseline Volumetric Criteria Are No Longer Predictive of TFC Decline When Propensity Score Adjustments are Applied

Unweighted



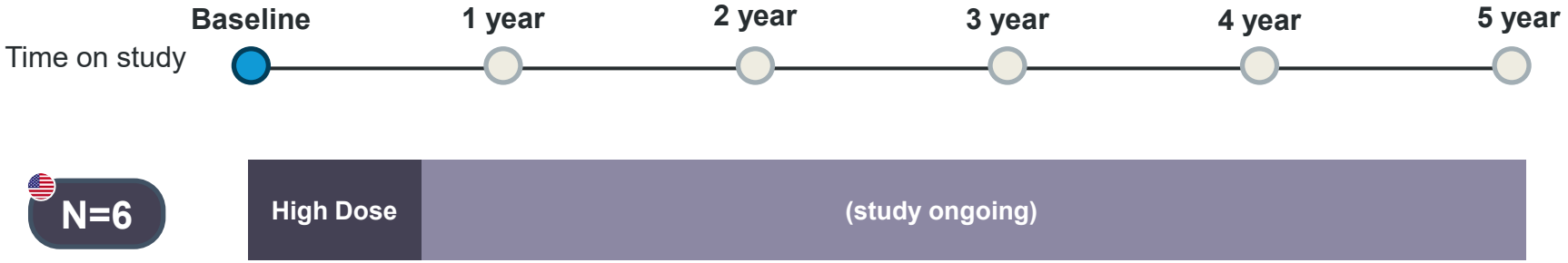
PS Weighted

(**Covariates**: baseline age, sex, CAP100, DCL, TFC, TMS, SDMT, SWR)



Abbreviations: CAG, cytosine-adenine-guanine; CAP, CAG-Age-Product; CI, confidence interval; DCL, diagnostic confidence level; PS, propensity score; SDMT, Symbol Digit Modalities Test; SWR, Stroop Word Reading; TFC, Total Functional Capacity; TMS, Total Motor Score.

Study Design for AMT-130 Phase I/II
Cohort 4: Lower Striatal Volume as Eligibility Criteria



Key eligibility criteria

- Aged 25-65 years
- Diagnostic confidence level: 3 or 4*
- Total functional capacity: 9-13
- HD medications stable prior 3 months
- Surgical approach safely feasible
- Striatal volumes: ≥ 1 volume below previously specified minima
  - Putamen: < 2.5 cm^3/side
  - Caudate: < 2.0 cm^3/side

*DCL 4, or DCL 3 if individual meets definition of multidimensional manifest HD or has cognitive symptoms

Abbreviations: DCL, diagnostic confidence level; HD, Huntington's disease.

Reference: 1. https://clinicaltrials.gov/study/NCT04120493

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Baseline Demographics and Disease Characteristics Relevant to Eligibility: Cohort 4

Demographics and Disease Characteristics Mean or %	**AMT-130 High-Dose Cohort 4 (n=6)**
Sex, Males (%)	16.7
Age	43.7
CAG repeats	44.5
DCL = 3, 4 (%)	16.7, 83.3
TFC	12.0

Abbreviations: CAG, cytosine-adenine-guanine; DCL, diagnostic confidence level; TFC, Total Functional Capacity.

Striatal Volume Summary Points

- Previous studies have indicated that striatal volume is predictive of decline, this was replicated in the TRACK-HD/TRACK-ON/PREDICT-HD combined cohort

- However, propensity score analysis supported that other covariates can be substituted to adjust for the effect of striatal volume on cUHDRS & TFC outcomes
  - The same covariates were used in propensity score matching vs Enroll-HD, effectively mitigating potential bias arising from the absence of striatal volume data in Enroll-HD

- Cohort 4 enrollment is complete and the study is ongoing

Abbreviations: cUHDRS, composite Unified Huntington's Disease Rating Scale; HD, Huntington's disease; TFC, Total Functional Capacity.

Clinical Trial Sites

HD·GeneTRX1

CT-AMT-130-01 (US)

University of Alabama
Principal Investigator: Victor Sung, MD

University of California, San Francisco
Principal Investigator: Michael Geschwind, MD, PhD

Rush University Medical Center
Principal Investigator: Deborah Hall, MD, PhD

Johns Hopkins University
Principal Investigator: Jee Bang, MD, PhD

University of Michigan
Principal Investigator: Praveen Dayalu, MD

Ohio State University
Principal Investigator: Sandra Kostyk, MD, PhD

Vanderbilt University Medical Center
Principal Investigator: Amy Brown, MD

The University of Texas, Houston
Principal Investigator: Erin Furr-Stimming, MD

Virginia Commonwealth University
Principal Investigator: Stephanie Bissonnette, DO

University of Washington Medical Center
Principal Investigator: Ali Samii, MD

CenExel Rocky Mountain Clinical Research, CO
Principal Investigator: Meagan Salinas, MD

University of Arizona – Dept. of Neurology
Principal Investigator: Paul Larson, MD

HD·GeneTRX2

CT-AMT-130-02 (UK, Poland)

Activated Sites:

Cardiff University
Principal Investigator: Anne Rosser, MD, PhD
UK National Coordinating Investigator: Liam Gray, MD

National Hospital for Neurology and Neurosurgery
Principal Investigator: Edward Wild, MD

Wojskowy Instytut Medycyny Lotnicze – Klinika Neurologii
Principal Investigator: Grzegorz Witkowski, MD, PhD

International Neuro Center
Principal Investigator: Miroslaw Zabek, MD

Acknowledgements

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DSMB members

uniQure and MedPace Clinical Studies Teams

HD Community

CHDI, ENROLL-HD, HSG, HDSA/HD COPE, EHDN

Many other collaborators….

And to all the patients and their families…

THANK YOU!

uniQure