



APRIL 2025

Investor Presentation

We have an audacious mission to develop novel therapies for diseases with high unmet needs, with a focus on serious and fatal neurodegenerative diseases and endocrine conditions.



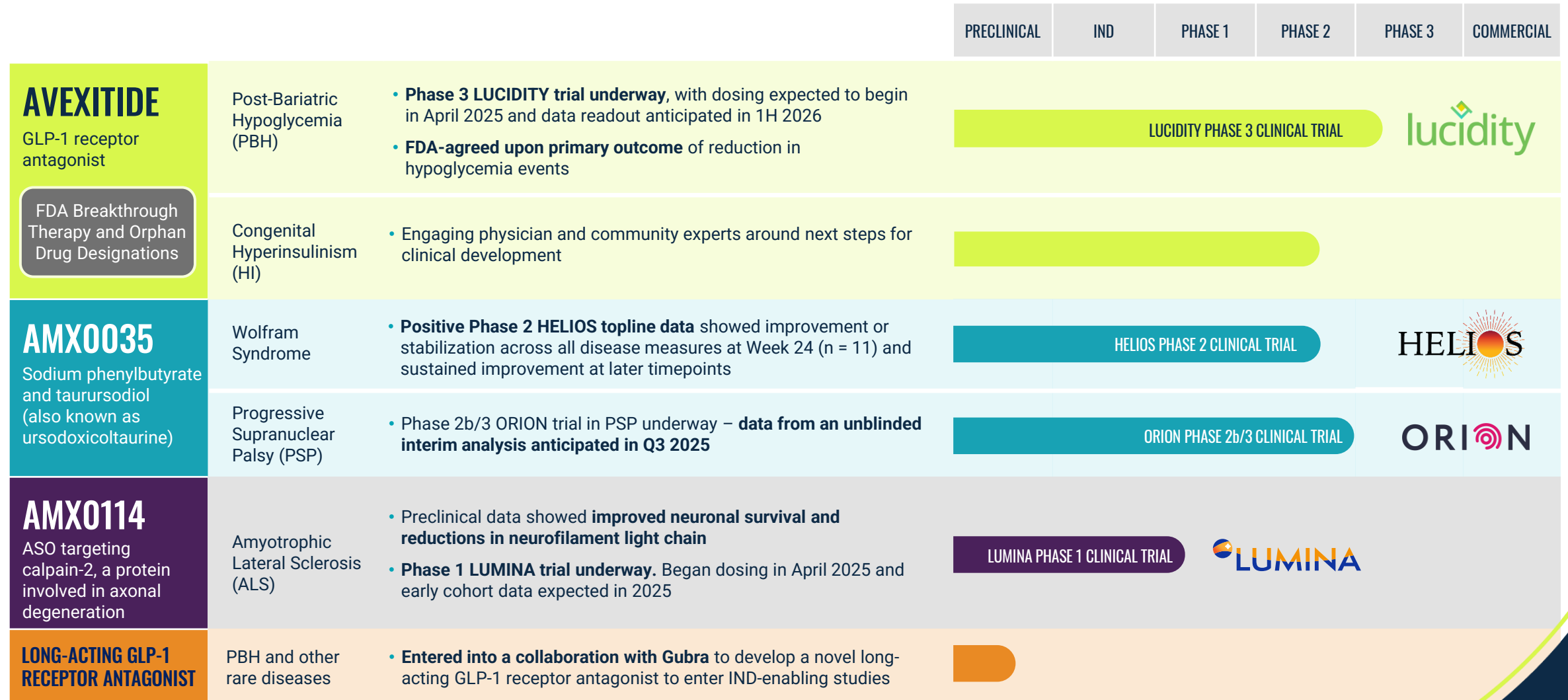
Raquel, living with Wolfram syndrome

DISCLAIMER

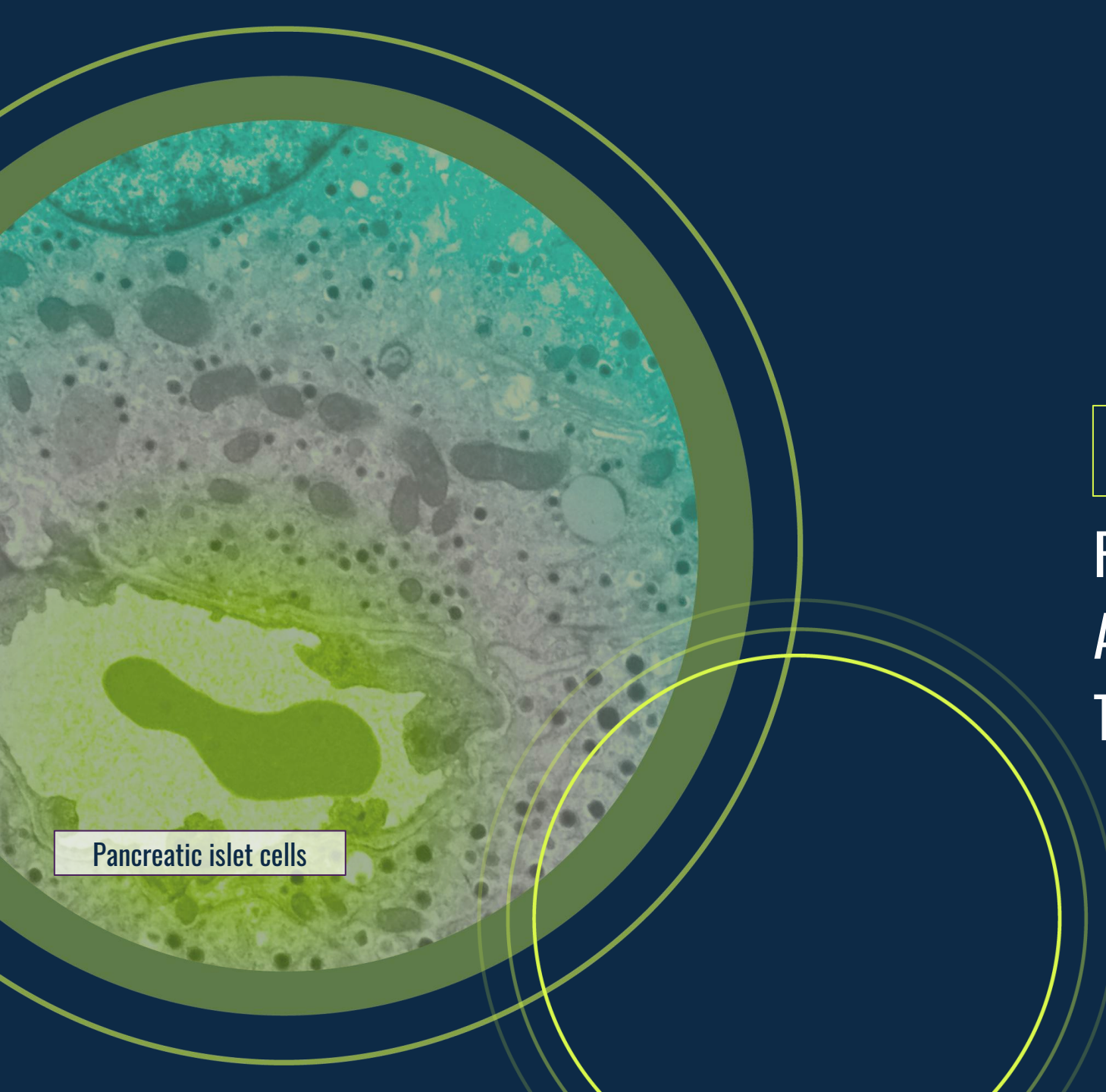
Statements contained in this presentation regarding matters that are not historical facts are “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Such statements include, but are not limited to, the Company’s plans to explore the use of avexitide as a treatment for post-bariatric hypoglycemia (PBH) and congenital hyperinsulinism, AMX0035 for neurodegenerative diseases, including progressive supranuclear palsy (PSP) and Wolfram syndrome (WS), AMX0114 for ALS; statements regarding the timing of clinical trials for PBH, PSP, WS and/or ALS; and expectations regarding our longer-term strategy. Any forward-looking statements in this presentation are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. Risks that contribute to the uncertain nature of the forward-looking statements include: the success, cost, and timing of Amylyx’ program development activities, including ongoing and planned clinical trials, Amylyx’ ability to execute on its development and regulatory strategy, regulatory developments, Amylyx’ cash runway and ability to fund operations, as well as the risks and uncertainties set forth in Amylyx’ United States Securities and Exchange Commission (SEC) filings, including Amylyx’ Annual Report on Form 10-K for the year ended December 31, 2024, and subsequent filings with the SEC. All forward-looking statements contained in this presentation speak only as of the date on which they were made. Amylyx undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

A Growing Pipeline of Therapies to Serve Communities with High Unmet Needs

Led by an experienced team with a proven track record of commercialization in rare diseases



ASO=antisense oligonucleotide; FDA=U.S. Food and Drug Administration; GLP-1=glucagon-like peptide-1; IND=investigational new drug.



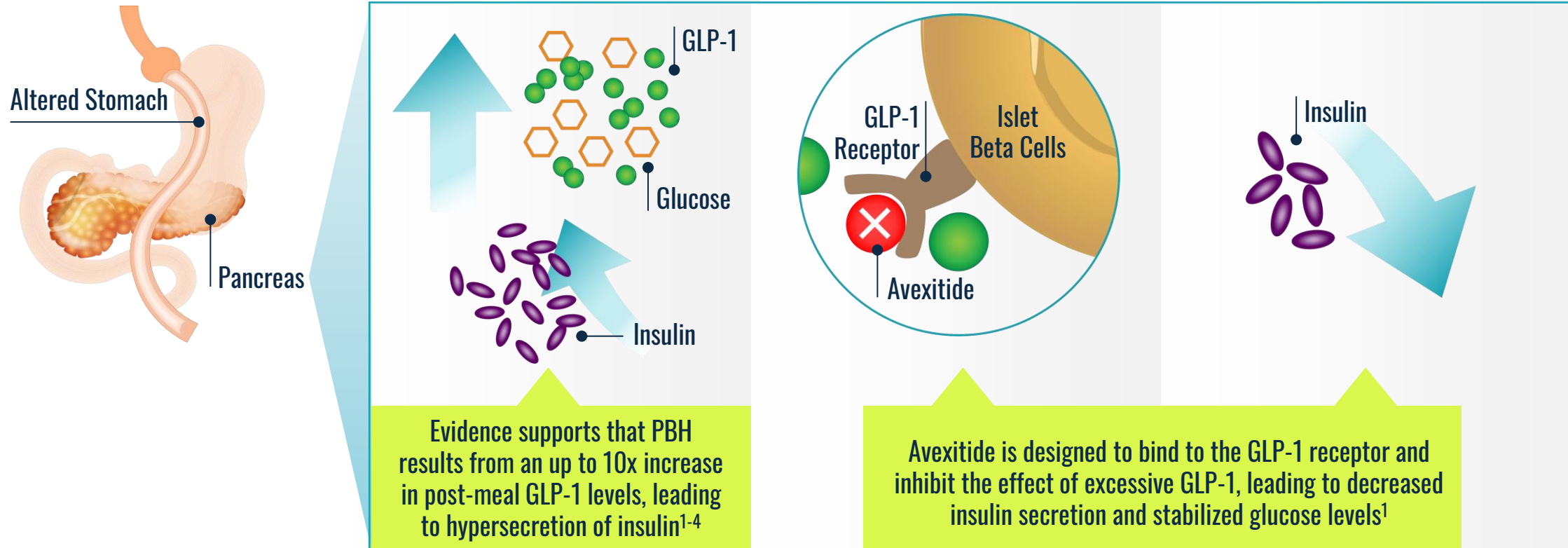
Pancreatic islet cells

AVEXITIDE

First-in-class, Phase 3 GLP-1 Receptor Antagonist with FDA Breakthrough Therapy Designation

The logo for Lucidity, featuring a stylized diamond shape above the word "lucidity" in a lowercase, sans-serif font.

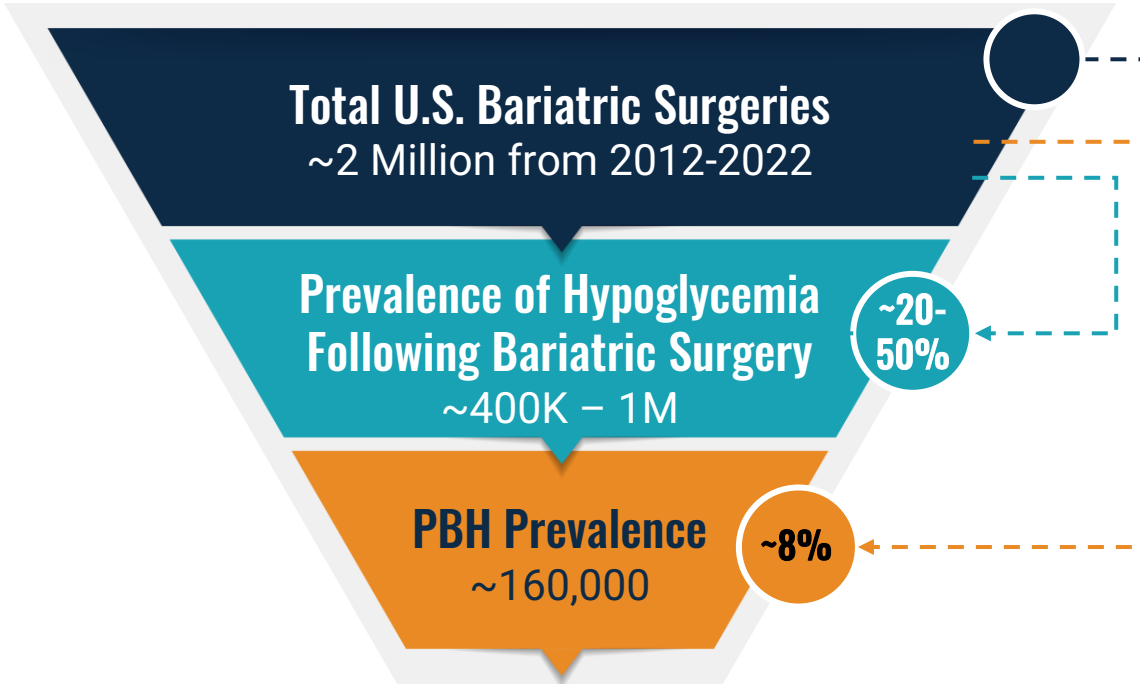
Post-Bariatric Hypoglycemia (PBH) is Believed to be Caused by Excessive GLP-1 Response that Leads to Hyperinsulinemic Hypoglycemia Post-Meal



GLP-1=glucagon-like peptide-1; 1. Craig, C. M. et al. *Diabetes, Obesity & Metabolism*. 2018;20:352–361. doi.org/10.1111/dom.13078. 2. Jalleh, R. J. et al. *Reviews in Endocrine and Metabolic Disorders*. 2023;24:1075-1088. doi.org/10.1007/s11154-023-09823-3. 3. van den Broek, M. et al. *International Journal of Obesity*. 2021;45(3):619-630. doi.org/10.1038/s41366-020-00726-w. 4. Larraufie et al., 2019, *Cell Reports* 26, 1399–1408. doi.org/10.1016/j.celrep.2019.01.047.

~160K People Live With PBH in U.S.

Based on representative sample of available studies



Preliminary data from two industry-leading medical claims providers align with current literature understanding of ~160K prevalent population¹⁰

Source	Finding
ASMBS Registry of Bariatric Surgery^{1a}	2.4M bariatric surgeries of which 1.9M were either sleeve gastrectomy [SG] or Roux-en-Y gastric bypass [RYGB])
8-Study Meta-Analysis (N=280)²	56.1% prevalence of hypoglycemia in studies specifically examining RYGB and 54.3% in those examining SG
Prospective, Longitudinal Cohort (N=1,448)³	43.2% prevalence of post-RYGB hypoglycemia symptoms
Prospective 1-Year Study (N=186)⁴	32.8% of participants had at least one OGTT-related hypoglycemia after laparoscopic sleeve gastrectomy
Prospective 2-Year Study (N=281)⁵	32.6% showed post-challenge hypoglycemia after RYGB; 22.6% after SG
Retrospective Survey (N=341)⁶	29% with new-onset hypoglycemia symptoms post-RYGB or SG
Retrospective Study (N=120)⁷	Of 107 individuals with PBH treated with acarbose, 39% had persistent/unacceptable frequency of hypoglycemic events [Note: Equates to ~8-16% of total bariatric surgery population ^b]
Retrospective Study (N=1,206)⁸	13.1% met criteria for PBH 5 years post-op and 5% of those with PBH had severe symptoms ^c
Prospective, Longitudinal Cohort (N=177)⁹	7.9% met criteria for PBH 5-years post-RYGB
Prospective, Longitudinal Cohort (N=1,448)³	Symptoms of PBH requiring hospitalization or ER visit occurred in 2.6-3.6% of people who underwent RYGB at 5 years of follow-up






^aThe ASMBS total bariatric procedure numbers are based on the best estimation from available data (BOLD,ACS/MBSAQIP, National Inpatient Sample Data and outpatient estimations) ^b Assumes 20-40% post-surgical hypoglycemic symptom prevalence; ^cSevere symptoms defined as glucose <40 mg/dL or emergency room/hospital visit

1. Estimate of Bariatric Surgery Numbers, 2011-2022. American Society for Metabolic and Bariatric Surgery (ASMBS). Accessed July 9, 2024. 2. Lupoli R, et al. *Nutr Metab Cardiovasc Dis.* 2022;32(1):32-39. 3. Fischer LE, et al. *Surg Obes Relat Dis.* 2021;17(10):1787-1798. 4. Belilgoli A, et al. *Obes Surg.* 2017;27:3179-3186 797-802. 5. Brix JM, et al. *Obes Facts.* 2019;12:397-406. 6. Lee CJ, et al. *Surg Obes and Relat Dis.* 2018;14(6):797-802. 7. de Heide LJM, et al. *Diabetes Obes Metab.* 2023;25:735-747. 8. Lee CJ, et al. *Obesity.* 2016.24(6):1342-1348. 9. Raverdy V, et al. *Annals Surg.* 2016.264(5):878-885. 10. Data on file. 11. Hazelhurst J, et al. *Endocr Connect.* 2024;13(5):e230285.

Avexitide Reproducibly Improved Insulin and Glucose Responses During Standardized Meal Tests in People with PBH

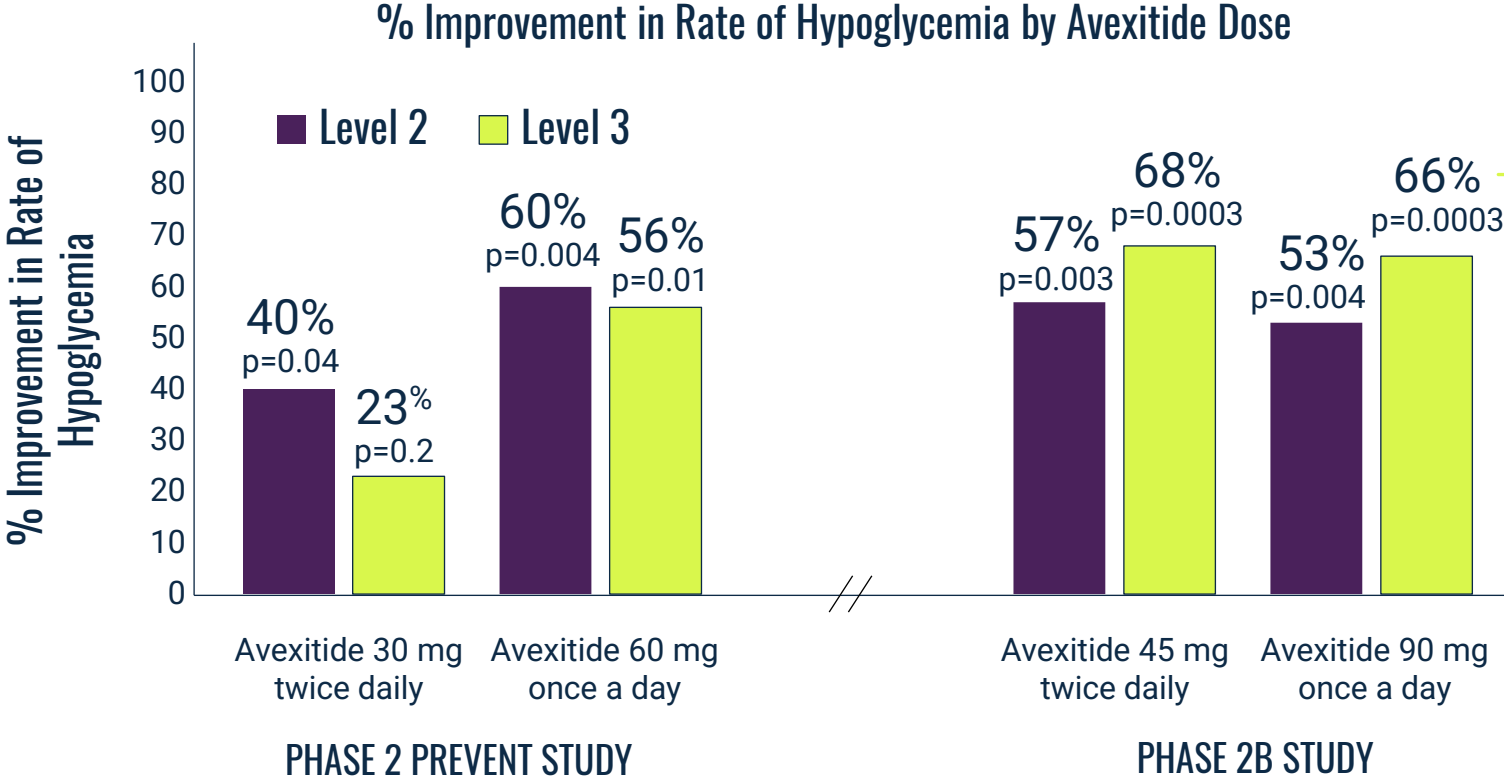
Phase 1 ¹	SAD ²	MAD ³	Phase 2 PREVENT ⁴	
Avexitide IV infusion (N=10)	Avexitide SC injection (N=8)	Avexitide 30 mg BID SC injection (n=5)	Avexitide 30 mg BID SC injection (N=17) ⁵	Avexitide 60 mg QD SC injection (N=17) ⁵

Improvement vs. Placebo

Postprandial Glucose Nadir	 Increase (p<0.001)	 Increase (p<0.001)	 Increase (p<0.05)	 Increase (p=0.001)	 Increase (p=0.0002)
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BID=twice daily; IV=intravenous; MAD=multiple ascending dose; MMTT=mixed meal tolerance testing; PBH=post-bariatric hypoglycemia; QD=once daily; SAD=single ascending dose; SC=subcutaneous; **1.** Craig, C. M. et al. *Diabetologia*. 2017;60(3):531-540. doi:10.1007/s00125-016-4179-x; **2.** Craig, C. M. et al. *Diabetes, Obesity & Metabolism*. 2018;20:352–361. doi.org/10.1111/dom.13078; **3.** Tan, M. et al. *Diabetes, Obesity & Metabolism*. 2020;22(8):1406-1416. doi:10.1111/dom.14048; **4.** Craig, C. M. et al. *The Journal of Clinical Endocrinology & Metabolism*. 2021;106(8):e3235-e3248. doi:10.1210/clinem/dgab103; **5.** 18 participants were randomized and completed the trial with 17 included in the efficacy analysis due to a major protocol deviation (glycemic rescue was not administered as indicated per protocol during the Period 1 placebo MMTT).

Avexitide Significantly Reduced Rates of Hypoglycemia in Two Phase 2 Clinical Trials in PBH



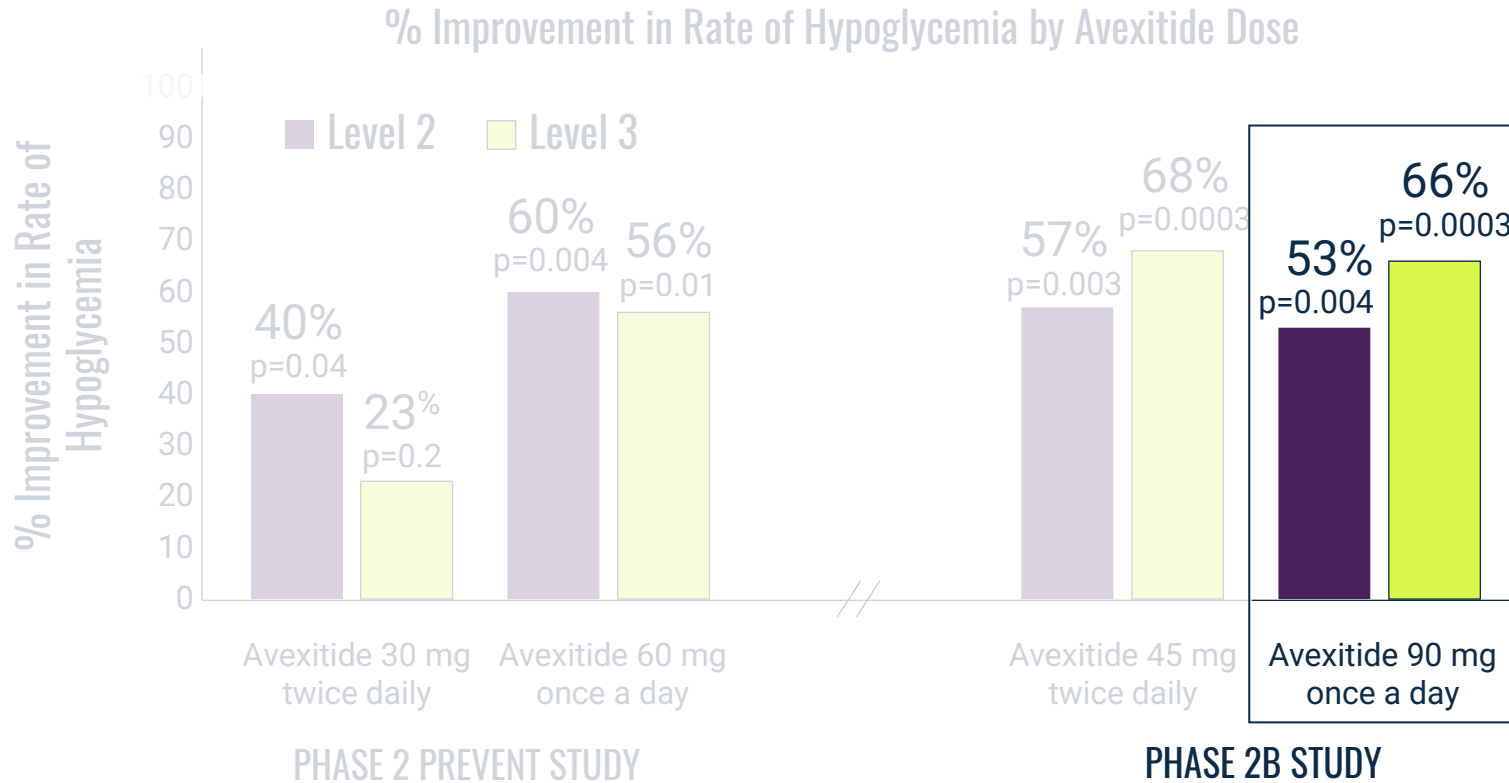
Avexitide cut rates of hypoglycemia events by **>50%**

Treatment effect supported by consistent, dose-dependent effects across Phase 1, SAD, and MAD trials in PBH

Avexitide 90 mg QD demonstrated a half-life of ~3 hours, a Tmax ranging from 6-9 hours, and therapeutic exposure through 24 hours.

MAD=multiple ascending dose; PBH=post-bariatric hypoglycemia; QD=once daily; SAD=single ascending dose; Tmax=time to peak drug concentration; 1. Craig, C. M. et al. *The Journal of Clinical Endocrinology & Metabolism*. 2021;106(8):e3235-e3248. doi.org/10.1210/jeandso/bvac150.725. 2. Tan, M. (2022). *Efficacy and Safety of Avexitide for Treatment of Hypoglycemia after Gastrointestinal Surgery: Assessment of Novel Dosing Regimens in an Expanded Indication* [Conference presentation]. ENDO Annual Symposium.

Phase 3 Endpoint Met in Phase 2 and Phase 2b



Phase 3 program will evaluate 90 mg QD in people living with PBH

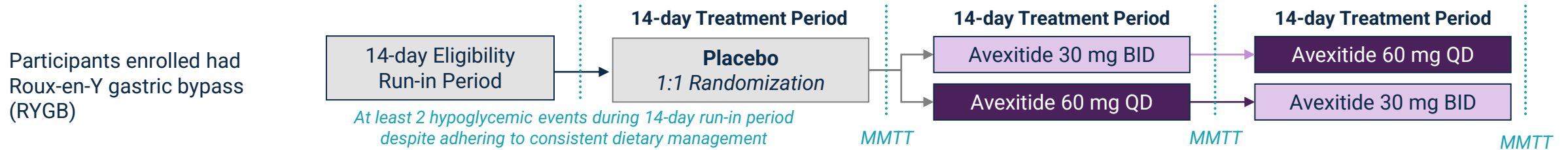
FDA-agreed upon primary endpoint: Composite of Level 2 and Level 3 Hypoglycemia events

FDA Breakthrough Therapy Designation

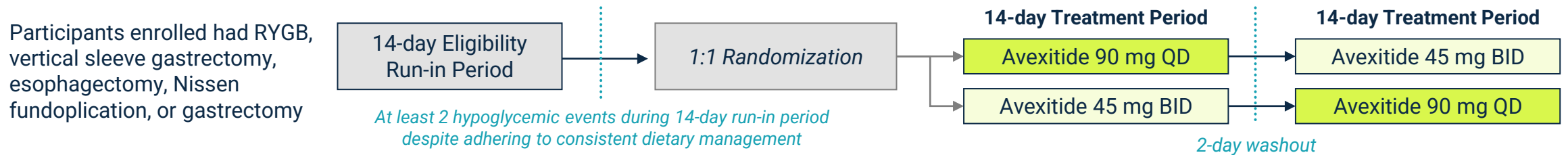
FDA=U.S. Food and Drug Administration; PBH=post-bariatric hypoglycemia; QD=once daily; 1. Craig, C. M. et al. *The Journal of Clinical Endocrinology & Metabolism*. 2021;106(8):e3235-e3248. doi.org/10.1210/jendso/bvac150.725. 2. Tan, M. (2022). *Efficacy and Safety of Avexitide for Treatment of Hypoglycemia after Gastrointestinal Surgery: Assessment of Novel Dosing Regimens in an Expanded Indication* [Conference presentation]. ENDO Annual Symposium.

Phase 3 LUCIDITY Trial Designed to be Consistent with Phase 2 PREVENT and Phase 2b Trials Evaluating Avexitide for the Treatment of PBH

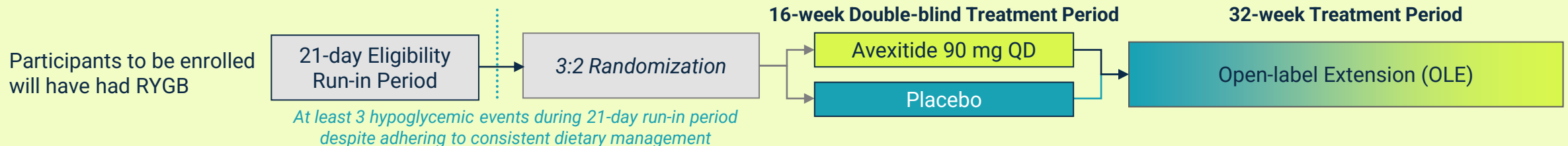
PHASE 2 PREVENT TRIAL DESIGN - 28-day, randomized, placebo-controlled crossover trial (N = 18)



PHASE 2B TRIAL DESIGN - 28-day, open-label, investigator-initiated, crossover trial (N = 16)



PHASE 3 LUCIDITY TRIAL DESIGN - Multicenter, randomized, double-blind, placebo-controlled trial (N = ~75)



BID=twice daily; MMTT=mixed meal tolerance testing; PBH=post-bariatric hypoglycemia; QD=once daily.

Avexitide was Generally Well-Tolerated with a Favorable Safety Profile Across Both Phase 2 Trials

Phase 2 PREVENT Study ¹	Phase 2b Study ²
AEs generally mild to moderate and transient	AEs generally mild to moderate and transient
No treatment-related serious AEs <ul style="list-style-type: none"> • 1 serious adverse event (presyncope during avexitide 60 mg once daily) occurred; reported as unrelated to study drug and self-limited 	No serious AEs
Most common AEs were injection* site bruising, headache, and nausea	Most common AEs were diarrhea, headache, bloating, and injection* site reaction/bruising
No participant discontinuations	No participant discontinuations

No clinically meaningful increases were observed in fasting or peak postprandial plasma glucose levels (i.e., no hyperglycemia observed)

*Injection site reactions generally mild and transient with no grade 3 events or resulting discontinuations

AE=adverse event; 1. Craig, C. M. et al. *The Journal of Clinical Endocrinology & Metabolism*. 2021;106(8):e3235-e3248. doi.org/10.1210/jendso/bvac150.725. 2. Tan, M. (2022). *Efficacy and Safety of Avexitide for Treatment of Hypoglycemia after Gastrointestinal Surgery: Assessment of Novel Dosing Regimens in an Expanded Indication* [Conference presentation]. ENDO Annual Symposium.

Phase 3 LUCIDITY Trial Underway, Readout in First Half of 2026

April 2025

Recruitment underway; Expect to begin dosing in April 2025

2025

Expected Phase 3 Recruitment Completion 2025

1H 2026

Planned Pivotal Study Readout First Half of 2026

2027

Anticipated Commercial Launch 2027

Strong IP position with **patent rights through 2037**, potential for additional term through Patent Term Extensions

Granted **Orphan Drug Designation** and positioned for **NCE exclusivity**

IP=intellectual property; NCE=new chemical entities.

A decorative graphic on the left side of the slide consisting of several overlapping circles and arcs in shades of teal and light blue. One large teal circle is prominent in the lower-left quadrant, with other lighter circles and arcs overlapping it and extending towards the top-left.

AMX0035

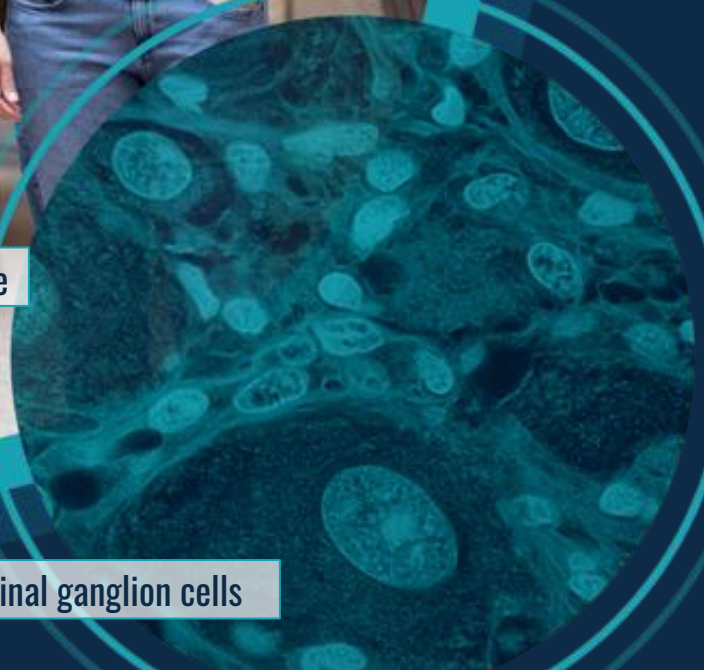
Fixed-dose combination of sodium phenylbutyrate and taurursodiol designed to slow or mitigate neurodegeneration



Wolfram Syndrome Program



Raquel, living with Wolfram Syndrome



Retinal ganglion cells

Wolfram Syndrome is a Rare, Fatal, Monogenic, Progressive Disorder¹⁻⁵



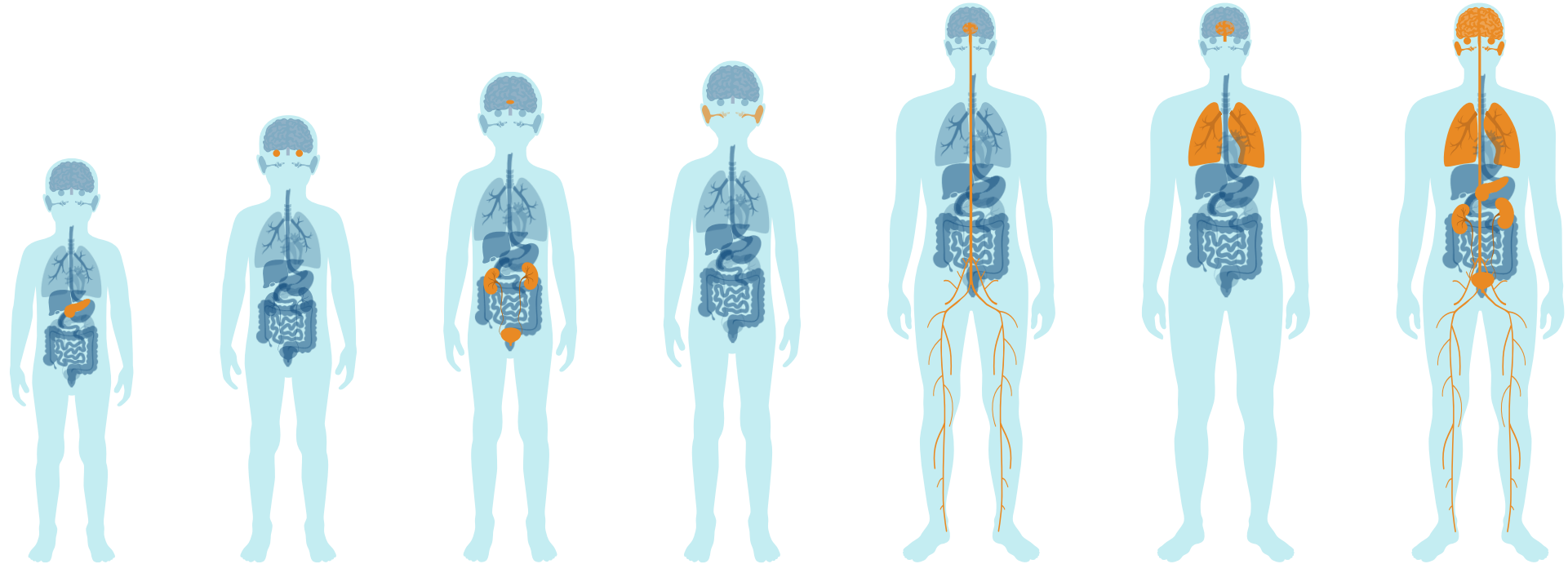
WFS1 GENE MUTATION

PROGRESSIVELY IMPACTS MULTIPLE ORGANS AND SYSTEMS¹⁻⁵

~3,000 people

Living with Wolfram Syndrome in the U.S.^{1,2}

No approved therapies for Wolfram syndrome⁶



Childhood-onset Diabetes Mellitus
Elevated blood sugar levels from insulin-producing beta cell death

Gradual Loss of Vision Leading to Blindness
Optic nerve cell death

Diabetes Insipidus
Kidneys produce too much urine from a faulty pituitary gland

Hearing Loss
From cranial nerve damage

Balance and Coordination Difficulty
Ataxia from cerebellum damage

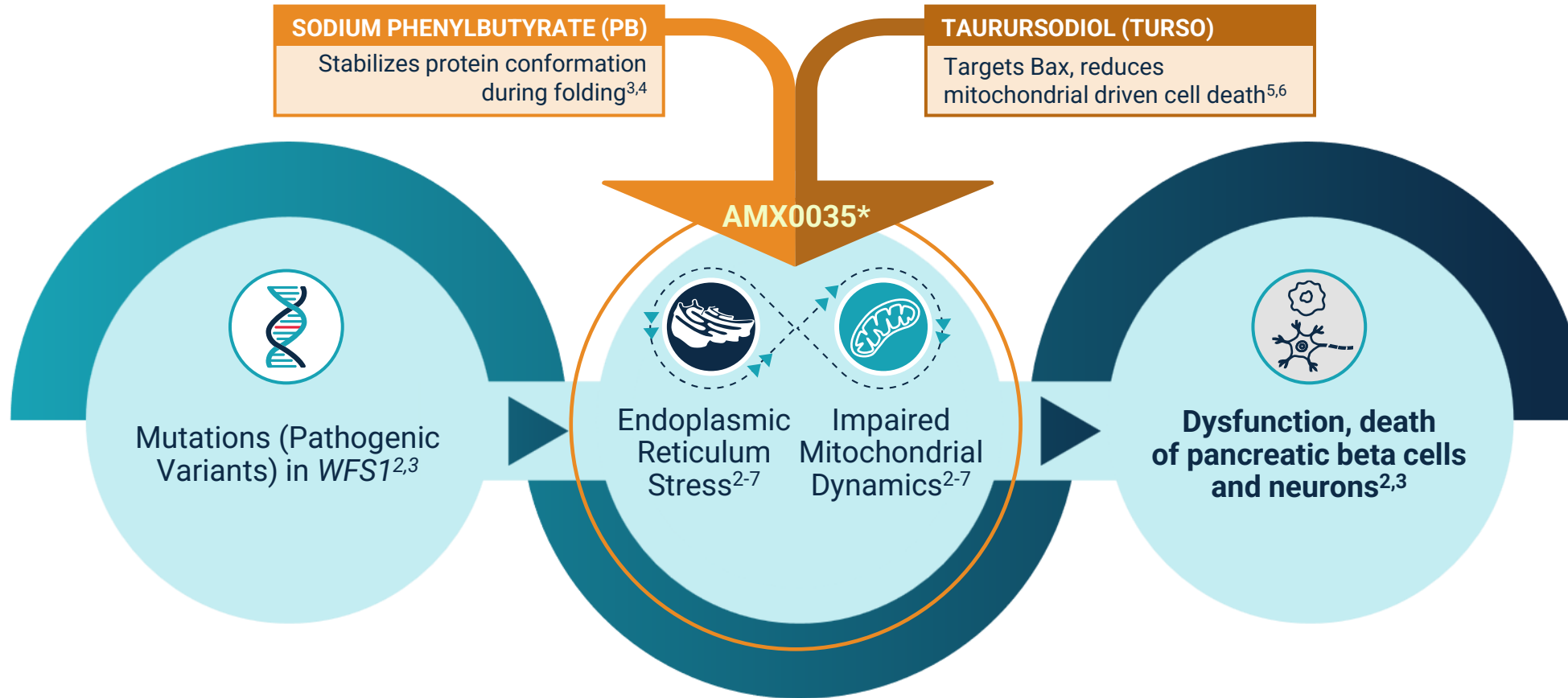
Difficulty Breathing
From brain stem damage

Death occurs at a median age of 30 years (range 25-49 years), mainly from respiratory failure

WFS1=Wolfram syndrome type 1 gene; 1. Urano, F. *Diabetes*. 2014;63(3):844-846. 2. Pallotta MT, et al. *J Transl Med*. 2019;17:238. 3. Lee, E., et al. *Front Genet*. 2023;14:1198171. 4. Leslie, M. *Science*. 2021;371(6530):663-665. 5. Matsunaga et al. *Plos One*. 2014;9(9):106906. 6. Urano, F. *Curr Diab Rep*. 2016;16(1):6.

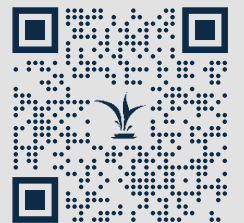
Wolfram Syndrome is a Prototypical Endoplasmic Reticulum Stress Disorder¹

AMX0035 targets endoplasmic reticulum stress and related mitochondrial dysfunction pathways



JCI insight

AMX0035 has been extensively studied in Wolfram models including patient-derived cells and mouse model

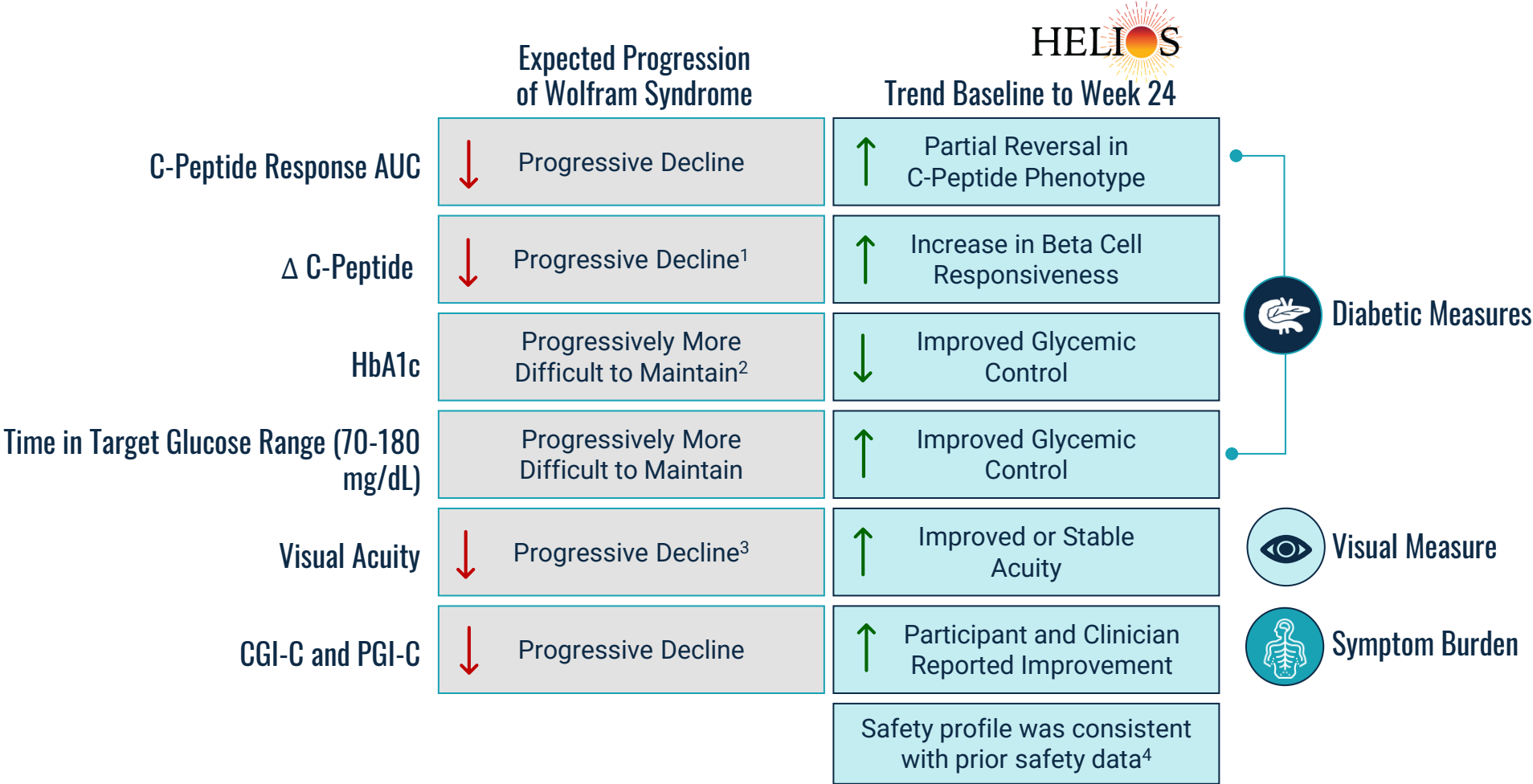


Clear Link of Mechanism of Disease and Mechanism of AMX0035

WFS1=Wolfram syndrome type 1 gene; * Results for AMX0035 are synergistic relative to PB or TURSO alone. Supported by data on file with Amylyx & Cohen. J., et al. Clinical trial design for a phase II, randomized, placebo-controlled trial of AMX0035 in amyotrophic lateral sclerosis (CENTAUR). Poster presented at: 28th International Symposium for ALS/MND; December 4–10, 2017; Boston, MA 1. Urano, F. *Diabetes*. 2014;63(3):844-846. 2. Sarmara A, et al. *Orphanet J Rare Dis*. 2019; 14(1):279. 3. Pallotta MT, et al. *J Transl Med*. 2019;7(1):238-249. 4. Shang L, et al. *Diabetes*. 2014;63(3):923-933. 5. Zhou W. *J Biol Chem*. 2011;286(17):14941-14951. 6. Rodrigues CM, Steer CJ. *Expert Opin Investig Drugs*. 2001;10(7):1243-1253. 7. Mishra R, et al. *Ther Adv Rare Dis*. 2021;2:26330040211039518.

Topline Data Suggest Potential Benefit of AMX0035 in Wolfram Syndrome

Improvements across disease measures observed



AUC=area under the curve; CGI-C=clinician-reported global impression of change ; HbA1c=glycated hemoglobin A1c; PGI-C=patient-reported global impression of change; Data on File. Amylyx Pharmaceuticals Inc. 2024. 1. Recent natural history study demonstrated C-peptide levels progressively decline in people with Wolfram syndrome. 2. Recent natural history study demonstrated that average HbA1c increases and time in target glucose range declines in people with Wolfram syndrome. 3. Recent natural history study demonstrated visual acuity progressively worsens in people with Wolfram syndrome. 4. AMX0035 was generally well-tolerated. All adverse events (AEs) were mild or moderate, and there were no serious AEs related to AMX0035 treatment.

AMX0035 Safety and Tolerability in HELIOS

- AMX0035 was **generally well tolerated**
 - > Diarrhea was the most common TEAE (50.0%); all cases were of mild severity
 - > All TEAEs were graded mild or moderate
- **No new safety signals** were identified
- Nearly all participants reported ≥ 1 TEAE during the trial
 - > Most did not lead to modification or interruption of AMX0035 dosing and **none led to drug discontinuation**

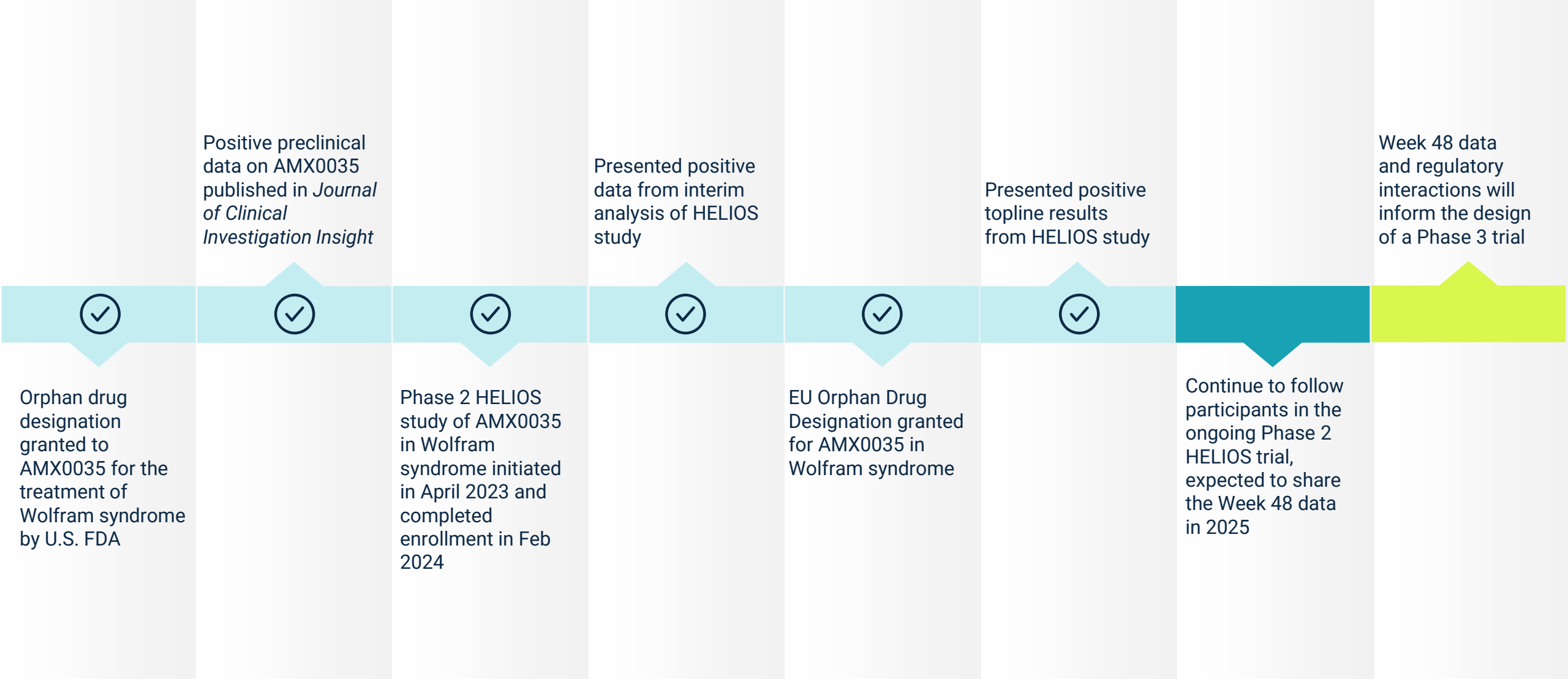
Summary of Treatment Emergent Adverse Events (TEAEs)

	AMX0035 (N=12)* n (%)
Participants with ≥ 1 TEAE	11 (91.7)
TEAE related to study drug**	9 (75.0)
Serious adverse events	0 (0)
Drug interrupted owing to TEAE	3 (25.0)
Dose reduced owing to TEAE	3 (25.0)
Drug discontinued owing to TEAE	0 (0)

*All available safety data as of July 31, 2024 included

**Includes those with TEAEs considered possibly related to treatment; none considered "probably related" or "definitely related"

AMX0035 Wolfram Syndrome Program Next Steps



EU=European Union; FDA=U.S. Food and Drug Administration.

Progressive Supranuclear Palsy (PSP) Program

ORION

Purkinje nerve cell in the cerebellum

PSP is a Rare, Progressive, and Fatal Tauopathy

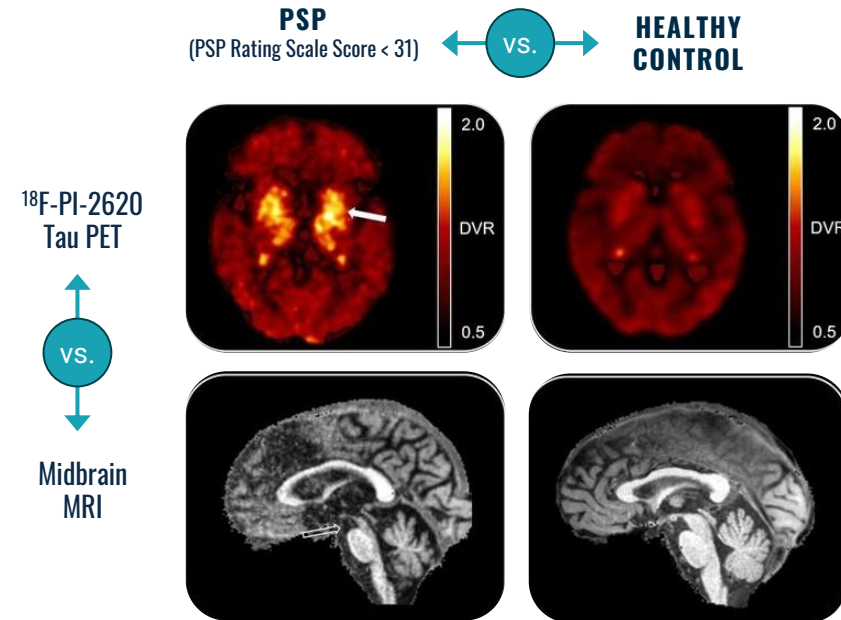
- PSP affects body movements, including balance and eye movements
- No disease-modifying therapies approved
- PSP is considered a tauopathy based on the strong genetic link between tau variants and disease development and the presence of abnormal tau protein deposits in the brain
- Biomarker data from Phase 2 trial of AMX0035 in Alzheimer's disease demonstrated a significant reduction in tau

PSP
is typically fatal

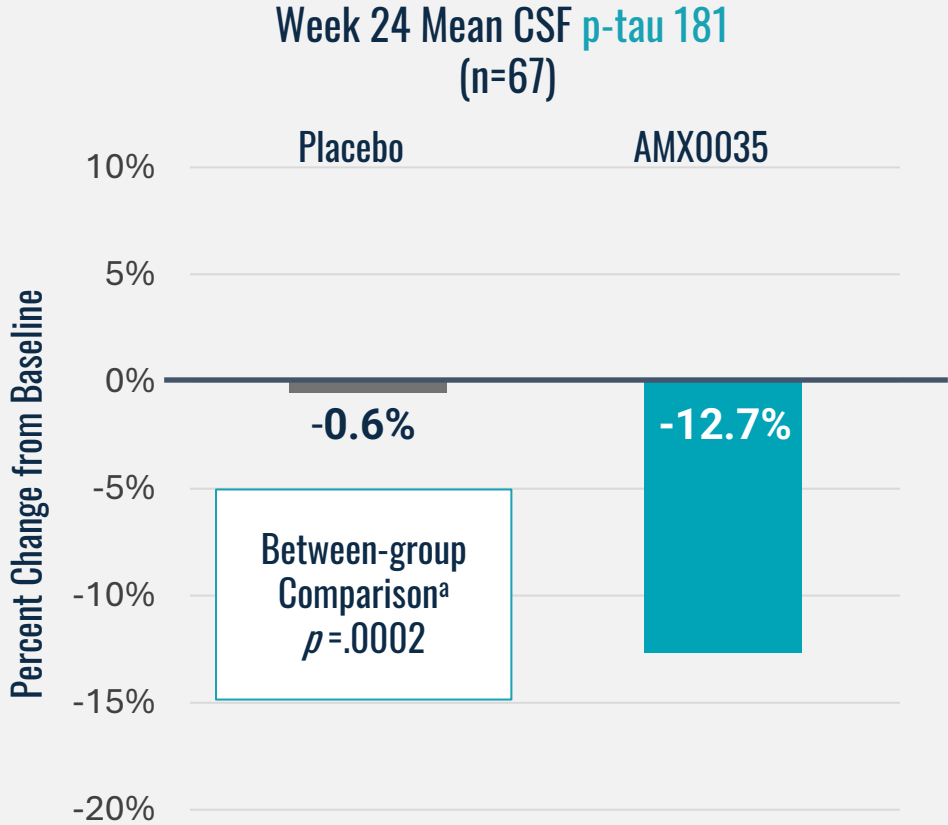
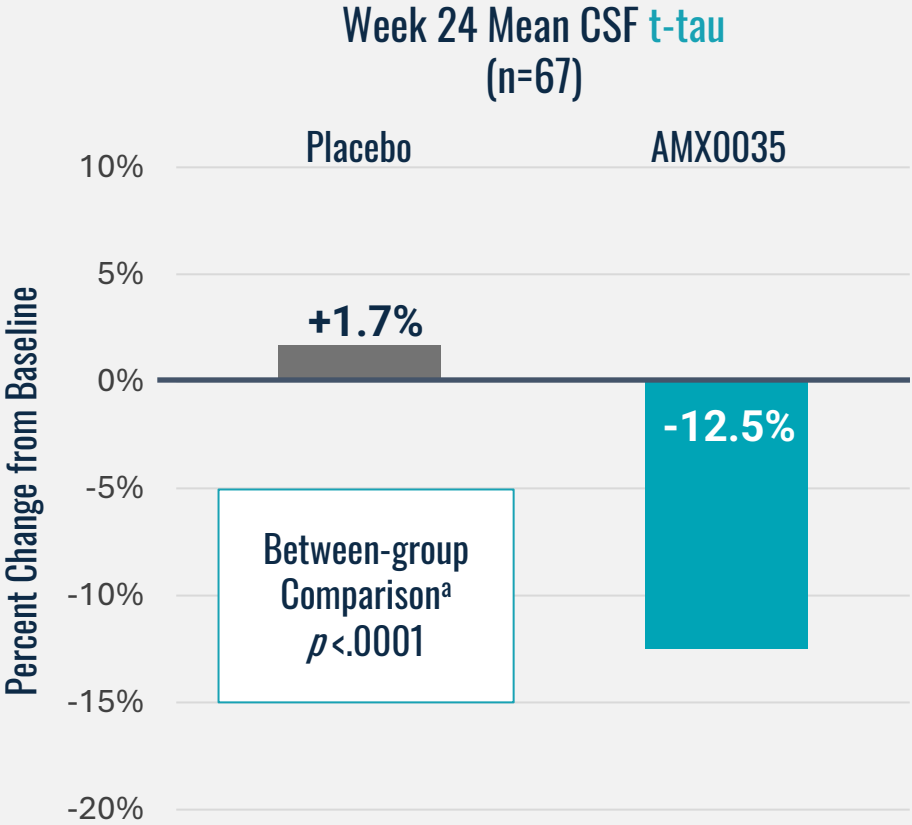
Within 6-8 years from symptom onset³⁻⁶

ESTIMATED PREVALENCE
7 in **100,000**
Worldwide^{1,2}

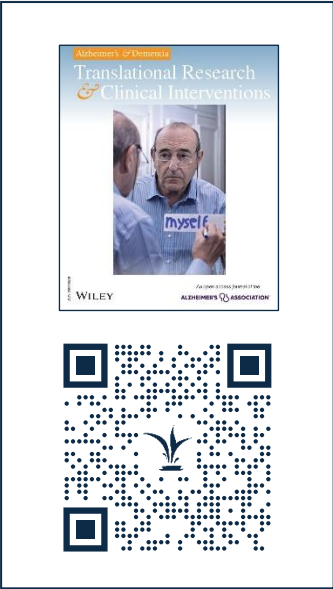
U.S. PREVALENCE
23,000
Approximately



AMX0035 Significantly Lowered CSF Tau and p-Tau in Randomized Placebo Controlled Phase 2 PEGASUS Trial in People with Alzheimer's Disease



t-Tau = total tau
p-Tau181 = phosphorylated tau at site threonine 181



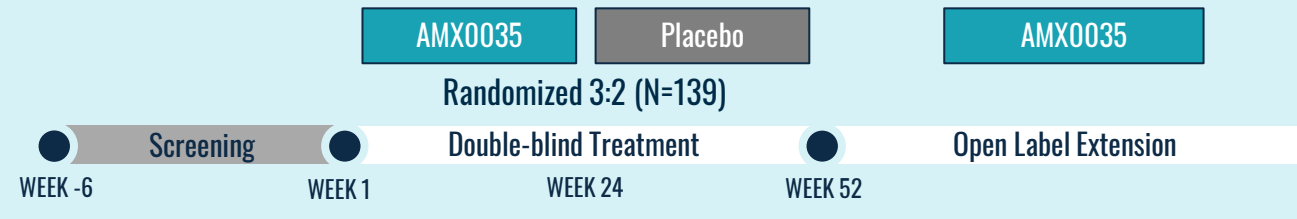
ORION: Operationally Seamless Phase 2b/3 Clinical Trial Underway

★ INTERIM ANALYSIS EXPECTED IN Q3 2025



PRIMARY OBJECTIVE: To assess the impact of AMX0035 compared to placebo on disease progression rate as measured by PSPRS

PHASE 2B STUDY PORTION DESIGN



Interim Analysis expected in Q3 2025

Proceed to Phase 3 if Data are Strong

PHASE 3 STUDY PORTION DESIGN



Key Eligibility Criteria

- Adults 40-80 years old
- Possible or Probable PSP (Steele-Richardson-Olszewski Syndrome) according to MDS 2017 criteria^{1,2}
- Presence of PSP symptoms < 5 years
- Able to walk independently or with minimal assistance³
- PSPRS total score < 40
- MMSE score ≥ 24
- Study partner required
- No feeding tube use

Primary Endpoint

- PSPRS score*

Secondary Endpoints

- PSPRS score*
- MDS-UPDRS Part II score

Additional Endpoints

- Brain volume (MRI)
- Participant QoL and caregiver burden
- CSF and plasma biomarkers of neuronal injury and neuro-inflammation
- Overall survival

*Given regional evidentiary requirements, the 10-item PSPRS is the primary endpoint in the U.S. and the 28-item PSPRS is the primary endpoint outside of the U.S.; for each region, the other form of the PSPRS is considered a secondary endpoint. MDS, Movement Disorders Society; MMSE, mini-mental status exam; PSPRS, Progressive Supranuclear Palsy Rating Scale; MRI, magnetic resonance imaging; QoL, quality of life; CSF, cerebrospinal fluid 1. Gradually progressive disorder, with age at disease onset ≥ 40 years 2. Either or both of the following two items are met: i. Vertical supranuclear gaze palsy OR slow velocity of vertical saccades AND postural instability with repeated unprovoked falls within 3 years OR tendency to fall on the pull-test within 3 years ii. Slow velocity of vertical saccades AND postural instability with more than two steps backward on the pull-test within 3 years. 1,2. Höglinger et al. Movement Disorders 2017. 3. Ability to walk 5 steps with minimal assistance (stabilization of one arm).



In memory of Mick, a husband and father, who was a gifted tattoo artist and musician.



Nerve cells in the brain

AMX0114 PROGRAM

Potent antisense oligonucleotide (ASO) targeting calpain-2



Calpain-2 Plays a Critical Role in Axonal Degeneration, a Key Mechanism Underlying ALS Pathophysiology

Evidence for Targeting Calpain-2 in ALS¹⁻⁴



Calpain-2 levels are elevated in people with ALS



Inhibition of calpain-2 has shown benefit in ALS mouse model



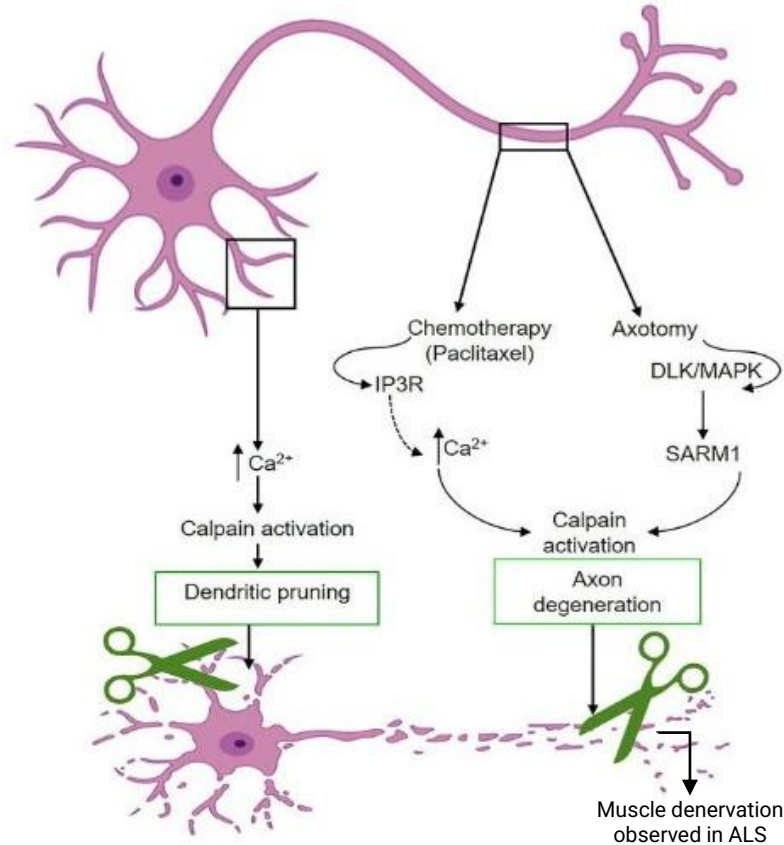
Calpain-2 substrates include neurofilament and TDP-43



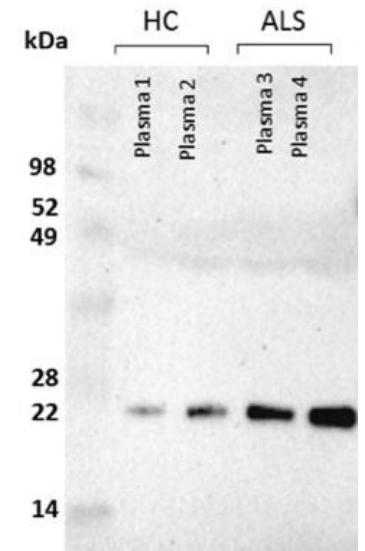
AMX0114 has shown efficacy in pre-clinical ALS models

Multiple injury paradigms and hypotheses of axonal degeneration converge on calpain-2

Mechanisms of Axonal Degeneration⁵

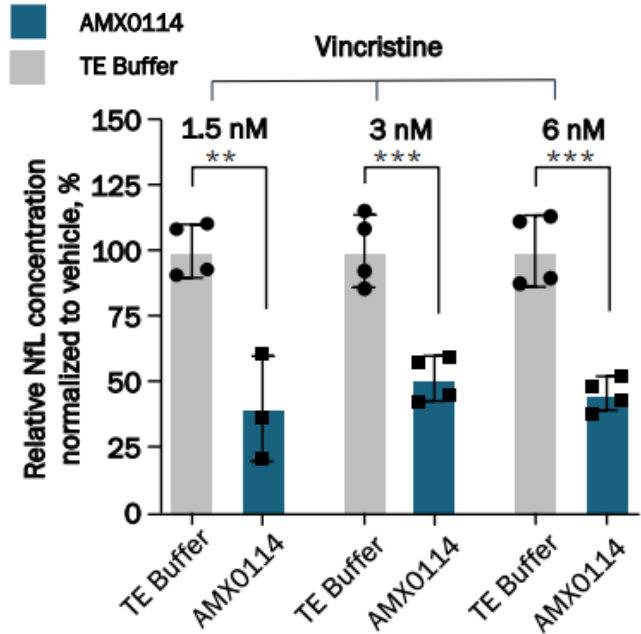


Full Length Neurofilament (68 kDa) is not observed in ALS or Healthy Control

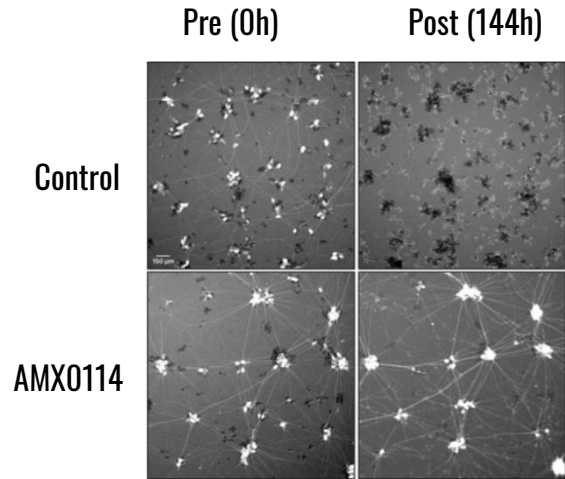


ALS=amyotrophic lateral sclerosis; 1. Ueyama H et al. J Neurol Sci. 1998;155(2):163-169. 2. Yamashita T et al. Nat Commun. 2012;3:1307. 3. Rao MV, et al. J Neurochem. 2016;137(2):253-65. 4. Ma M, et al. Neurobiol Dis. 2013;56:34-46. 5. Asakawa, K., Handa, H., Kawakami, K. Multi-phaseted problems of TDP-43 in selective neuronal vulnerability in ALS. Cell Mol Life Sci. 2021;78(10):4453-4465. doi:10.1007/s00018-021-03792-z

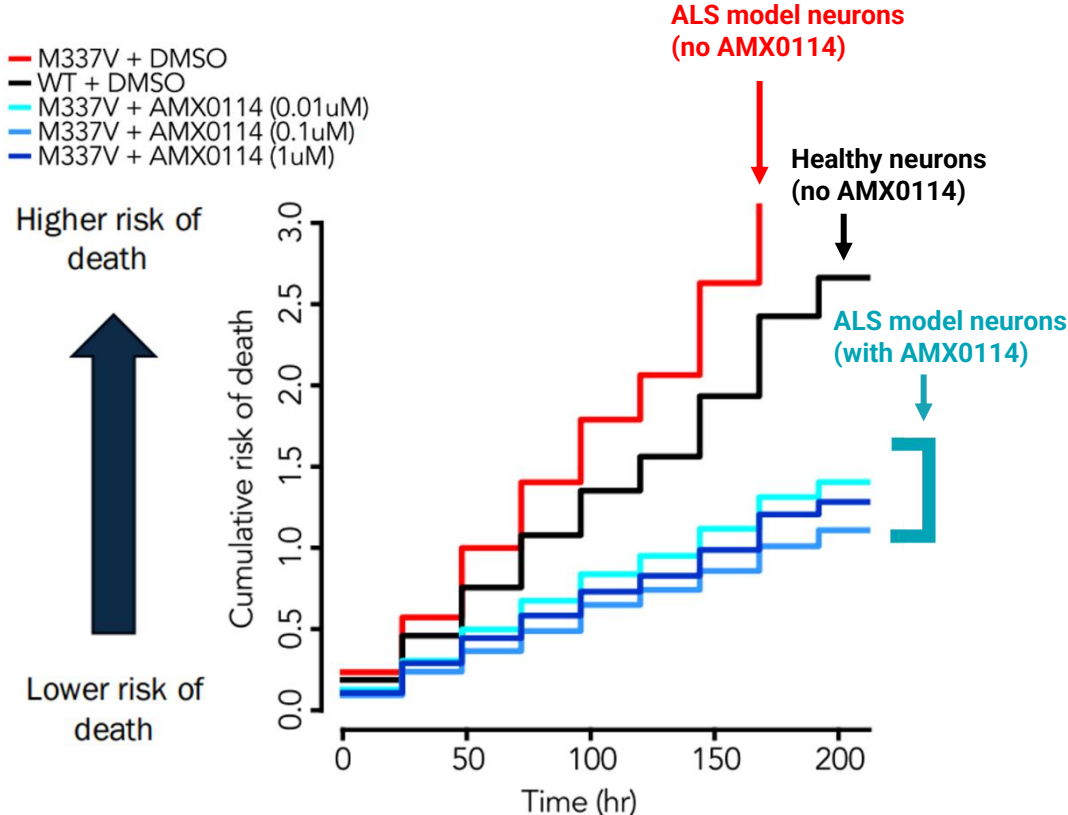
AMX0114 Reduces Extracellular NfL Levels in Multiple Models of Trigger-Induced Neuronal Injury and Improves Survival in Relevant Models



Representative Images of Motor Neurons Pre- and Post-Exposure to H₂O₂



TDP-43 ALS Model



Similar NfL Reduction in Rotenone and Colchicine models

NS = $P > .05$.
 * = $P < .05$.
 ** = $P < .01$.
 *** = $P < .001$.
 **** = $P < .0001$.
 NfL, neurofilament light chain; NS, not significant; TE, tris ethylenediaminetetraacetic acid.

Presented at

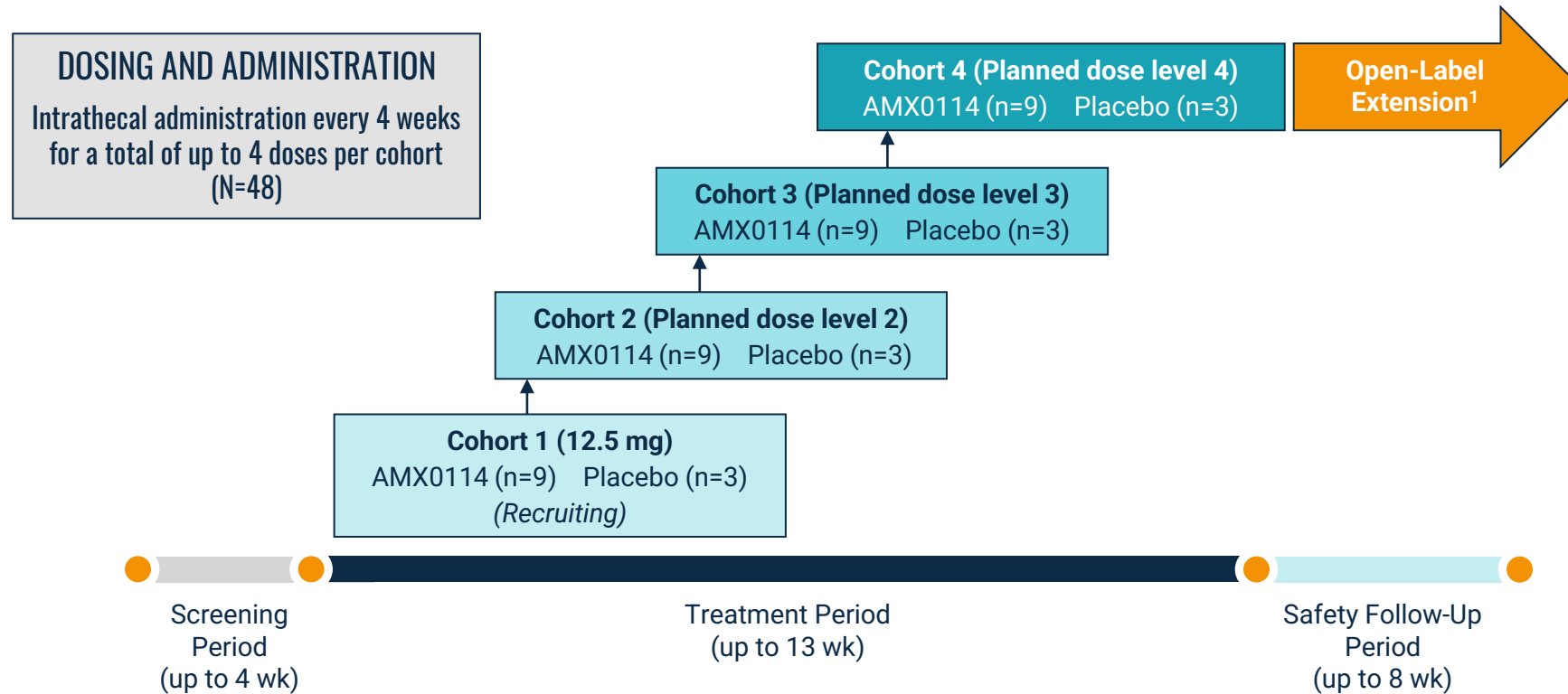
ALS=amyotrophic lateral sclerosis; NfL=neurofilament light chain; Data on File. Amylyx Pharmaceuticals Inc. 2024; Survival analyses performed in the lab of Dr. Sami Barmada at the University of Michigan Medical School by Dr. Michael Bekier.

LUMINA: Phase 1 Clinical Trial of AMX0114 in ALS



PRIMARY OBJECTIVE: To assess the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of AMX0114 in people living with ALS

- Will assess ALS biomarkers, including change from baseline in neurofilament light (NfL) levels



EARLY COHORT DATA EXPECTED IN 2025

ALS=amyotrophic lateral sclerosis; SBDP-145=spectrin breakdown product-145.

1. The open-label extension may be implemented if safety and efficacy data support a positive benefit-risk profile.



Key Corporate Highlights

EXPECTED CASH THROUGH THE END OF 2026¹

As of Dec. 31, 2024

\$176.5M in cash, cash equivalents,
and short-term investments

Public Offering

Closed on January 13, 2025

SHARES ISSUED

19.7MM

NET PROCEEDS

\$65.5MM

1. Cash guidance based on current operating plan including proceeds from Jan. 13, 2025 offering.

Advancing Three Therapies Across Four Indications

Focus on diseases with well-defined mechanistic rationale, clear clinical outcomes and biomarkers, and rigorous preclinical data

AVEXITIDE

First-in-class, GLP-1 receptor antagonist with Breakthrough Therapy and Orphan Drug designations

- Began the pivotal Phase 3 LUCIDITY trial in PBH; dosing expected to begin in April 2025, with **data readout anticipated in first half of 2026**

AMX0035

Oral, fixed-dose combination of two small molecules, sodium PB and taurursodiol

- Plans to share **Week 48 data from the ongoing Phase 2 HELIOS trial in Wolfram syndrome in 2025**; data and regulatory interactions will inform the design of a Phase 3 trial
- Phase 2b/3 ORION trial in PSP underway, **unblinded interim analysis of the Phase 2b portion of ORION in Q3 2025**

AMX0114

Antisense oligonucleotide designed to target calpain-2, a protein involved in axonal degeneration & neurofilament biology

- Began the Phase 1 **LUMINA** trial in ALS; began dosing in April 2025, with **early cohort data expected in 2025**

Ushering in a new era for treating
diseases with high unmet needs

