

BRIDGEBIO



Corporate Presentation

March 2026



Forward Looking Statements and Disclaimer

The presentation contains forward-looking statements. Statements made or presented may include statements that are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Words such as “believe,” “anticipate,” “plan,” “expect,” “intend,” “will,” “may,” “goal,” “potential,” “should,” “could,” “aim,” “estimate,” “predict,” “continue” and similar expressions or the negative of these terms or other comparable terminology are intended to identify forward-looking statements, though not all forward-looking statements necessarily contain these identifying words. We intend these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Exchange Act. These forward-looking statements, including express and implied statements relating to the commercial success of Attruby/Beyontra (acoramidis), the advantages of our business model and overall strategy, the timing of ongoing clinical trials, including the RECLAIM-HP Phase 3 study of encalret in chronic hypoparathyroidism, the Phase 2/3 registrational study for encalret in pediatric patients with ADH1, the Phase 2 study of infigratinib in hydrochondroplasia, the Phase 1/2 study of BBP-812 in Canavan disease, the Phase 3 FORTIFY study of BBP-418 in LGMD21/R9, BridgeBio Oncology Therapeutics’ and Gondola Bio’s clinical trials and pipelines, the clinical, therapeutic and market potential of our clinical development programs and our pipeline, the anticipated timing of submission of NDAs and BLAs to the FDA and corresponding submissions to foreign regulatory authorities and results of our interactions with the FDA, our speed of creating new and meaningful drugs and related impact on patients, the sustainability and velocity of our engine to deliver medicines, our value creation potential for patients, the potential market sizes and opportunities for the indications we are pursuing, the safety, efficacy, ease of access and mechanisms of our newly FDA-approved Attruby/EMA, PMDA and MHRA-approved Beyontra, the potential of acoramidis to be used to delay the onset of, or prevent ATTR, the potential of our deleter program to reverse ATTR, the potential of our pipeline of molecules, our financial position, including our expectations regarding reaching regulatory and commercial milestones, the potency and safety of our product candidates, the potential benefits of our products and product candidates, the affordability, maximum pricing and availability of insurance coverage of our medications, and the timing and expectations regarding results of our various clinical trials, reflect our current views about our plans, intentions, expectations and strategies, which are based on the information currently available to us and on assumptions we have made.

Such statements reflect the current views of the Company with respect to future events and are subject to known and unknown risks, including business, regulatory, economic and competitive risks, uncertainties, contingencies and assumptions about the Company, including, without limitation, risks inherent in developing therapeutic products, and those risks and uncertainties described under the heading “Risk Factors” in the Company’s most recent Annual Report on Form 10-K filed with the U.S. Securities and Exchange Commission (“SEC”), and in subsequent filings made by the Company with the SEC, which are available on the SEC’s website at www.sec.gov. In light of these risks and uncertainties, many of which are beyond the Company’s control, the events or circumstances referred to in the forward-looking statements, express or implied, may not occur. The actual results may vary from the anticipated results and the variations may be material. You are cautioned not to place undue reliance on these forward-looking statements, which speak to the Company’s current beliefs and expectations only as of the date of the presentation. Except as required by law, the Company disclaims any intention or responsibility for updating or revising any forward-looking statements made or presented at the presentation in the event of new information, future developments or otherwise. No representation is made as to the safety or effectiveness of the product candidates for the therapeutic use for which such product candidates are being studied. Certain information communicated at the presentation may relate to or be based on studies, publications, surveys and other data obtained from third-party sources and the Company’s own internal estimates and research. While the Company believes these third-party sources to be reliable as of the date of the presentation, it has not independently verified, and makes no representation as to the adequacy, fairness, accuracy or completeness of, any information obtained from third-party sources. In addition, certain information to be communicated at the presentation involves a number of assumptions and limitations, and there can be no guarantee as to the accuracy or reliability of such assumptions. Finally, such research has not been verified by any independent source. Such information is provided as of the date of the presentation and is subject to change without notice. The Company has not verified, and will not verify, any part of this presentation, and the Company makes no representation or warranty, express or implied, as to the accuracy or completeness of the information to be communicated at the presentation or as to the existence, substance or materiality of any information omitted from the presentation at the presentation. The Company disclaims any and all liability for any loss or damage (whether foreseeable or not) suffered or incurred by any person or entity as a result of anything contained or omitted from this document or the related presentation and such liability is expressly disclaimed. This Presentation is for informational purposes only. This Presentation shall not constitute an offer to sell or the solicitation of an offer to buy any securities, nor shall there be any sale of any securities in any state or jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such state or jurisdiction.

BridgeBio's objective function

Patient impact...

$$\text{Objective: max } \int_0^t \sum_{\text{Drugs } i=1}^N \frac{\Delta QALY(i)}{\text{patient}} * \text{patients}(i)$$

BridgeBio maximizes **the speed** of creating
as many new and meaningful drugs
that have a **profound impact** on as
many patients as possible

...through sustainable value creation

Each program must be:

- Based on beautiful science with a high probability of technical success (POTS)
- First-in-class or best-in-class
- NPV positive (driven by ROIC, g, WACC)

Inspiration – “Strategy: A History” by Lawrence Freedman

BridgeBio is a new type of biopharmaceutical company

From:

Slow and bureaucratic decision making

Expensive platforms with long lead times before
proof-of-concept data

High fixed costs

Limited sources of capital

Incentives at the portfolio level



To:

Rapid and decentralized decision making

Assets selected to target genetic diseases
at their source

Variablized and flexible costs

Strategic toolkit of financing options at the levels
of the portfolio and affiliate companies

Incentives at the level of each asset to preserve
focus at the level of biology

Inspiration – “Financing drug discovery for orphan diseases” by Andrew Lo

The right approach: decentralized R&D, centralized infrastructure



Build “**minimum viable companies**” to de-risk programs as quickly and efficiently as possible



Leverage **hyper-experienced R&D practitioners** who are focused on the science of each individual program



Provide investors with **increased choice in where to participate** in our ecosystem



Build **central infrastructure** for functions with economies of scale, such as commercial



Leverage **seasoned company builders** and **centralized capital allocators** to take the best possible shots on goal



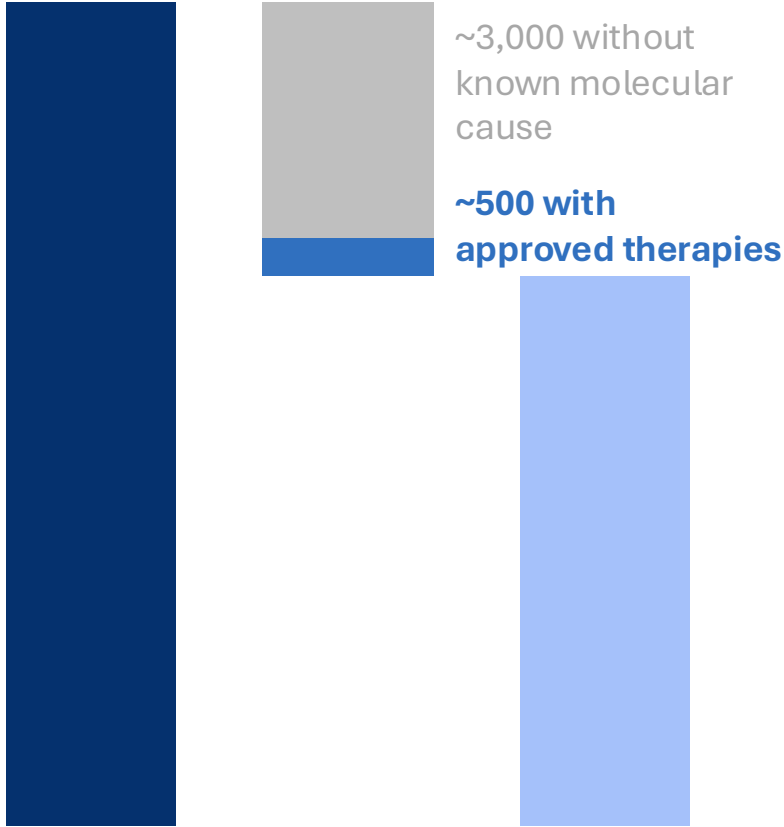
Provide investors with a **de-risked portfolio of assets**; enable access to low-cost debt

Inspiration – “Scale” by Geoffrey West

The right space: capitalizing on a scientific revolution to treat a massive unmet need for genetic diseases

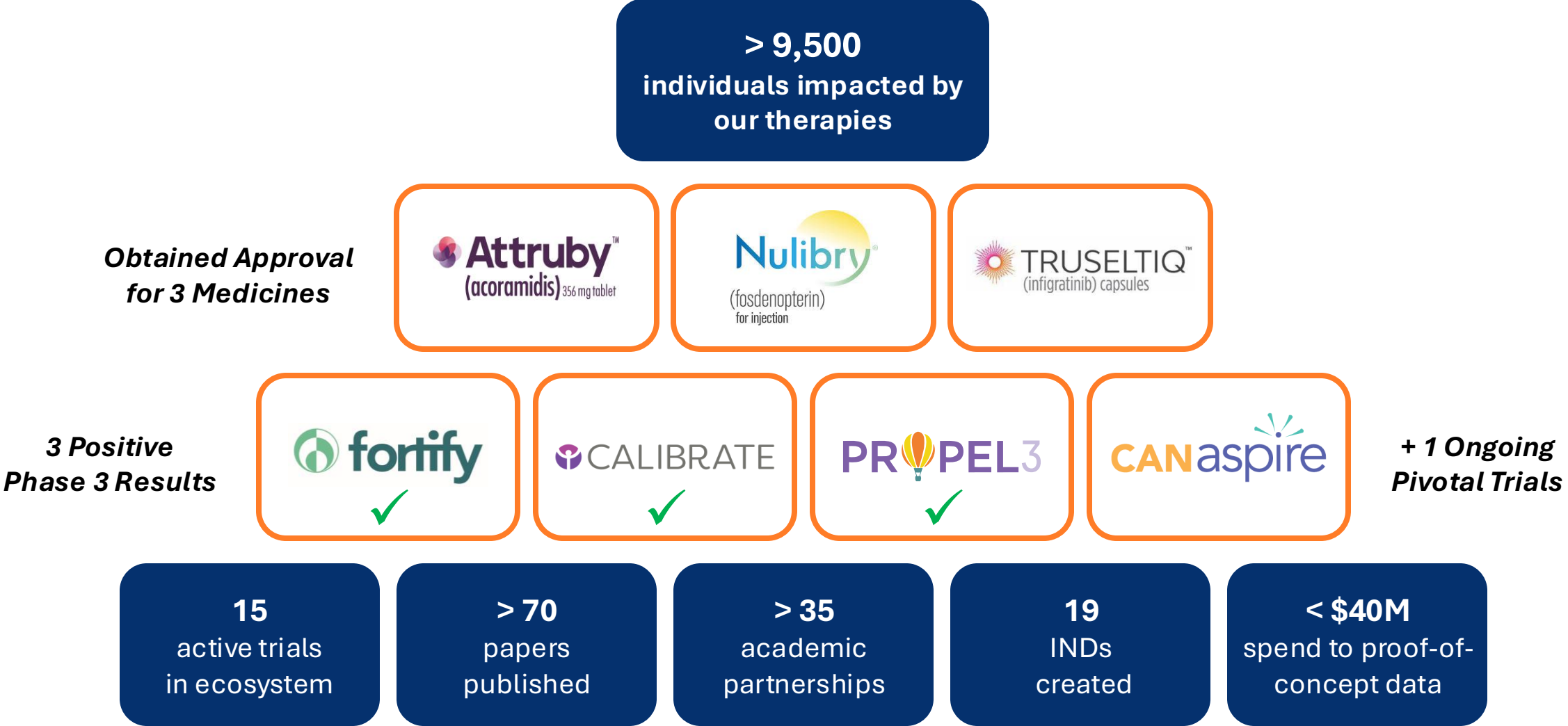
Genetics		Genetic association analysis of 77,539 genomes reveals rare disease etiologies
Macromolecules		Accurate proteome-wide missense variant effect prediction with AlphaMissense
Clinical Diagnosis		The expanding diagnostic toolbox for rare genetic diseases
Therapeutic Modalities		New drug approvals reached an all-time high in 2023, with five gene therapies, the first CRISPR–Cas9-edited therapy and a disease-modifying Alzheimer’s drug.
Regulatory		FDA issues final guidance on rare disease drug development

There are **10,000+** counted rare diseases affecting **450 million+** people globally

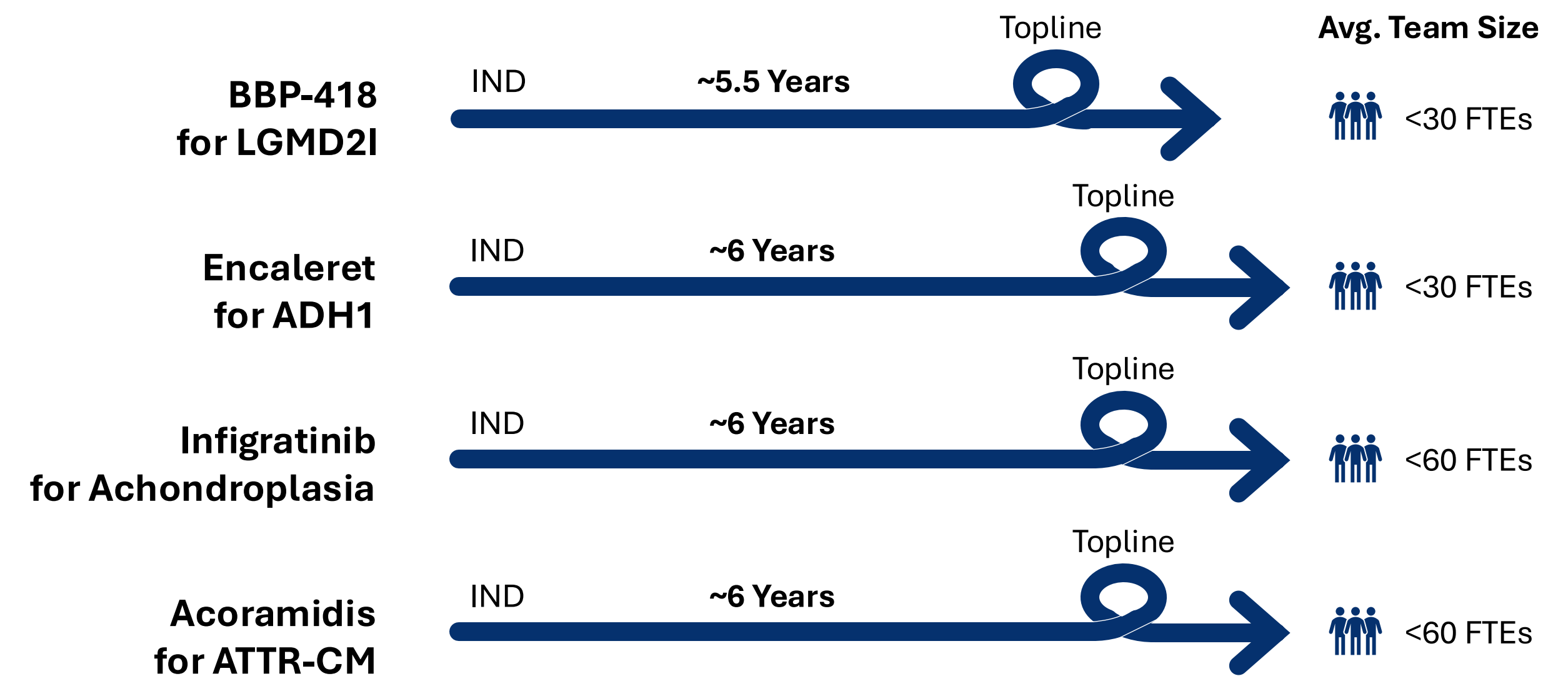


This leaves **hundreds of millions of people** across **6,500 diseases** with known molecular cause who are **anxiously waiting** for therapies

We have built a sustainable, high velocity engine to deliver hope and medicines to the communities that we serve

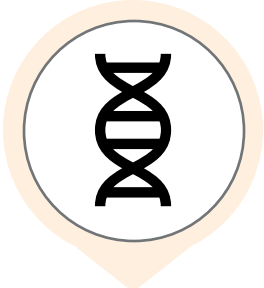


We leverage lean operating teams that advance medicines quickly and efficiently for people living with rare diseases

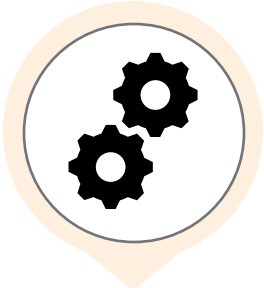


We target well-described diseases at their source, underpinning our industry-leading probability of technical success

Our approach



1. Characterize the genetic source of the disease



2. Define the function of the genetic drivers



3. Drug the target at its source

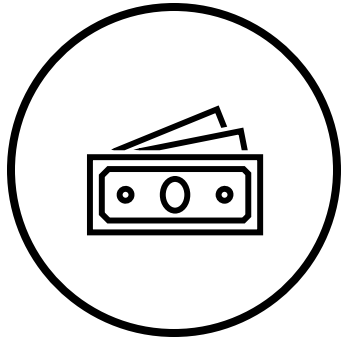
Probability of Technical Success (POTS)

Clinical Dev. Stage	Industry Benchmark ¹	BBIO Historical ²
Phase 1	52%	86%
Phase 2	29%	71%
Phase 3	58%	86%
Cumulative	9%	52%

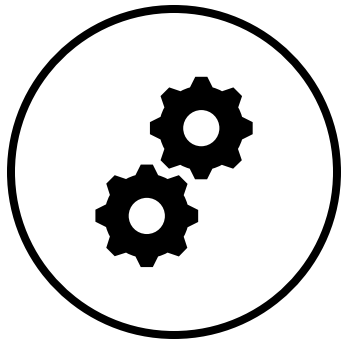
A pipeline of products that sing across the BrigeBio ecosystem

Program	Indication	Pre-Clinical	Phase 1	Phase 2	Phase 3	Approved	Patients (US + EU)	Market Opportunity
Attruby (acoramidis)	Transthyretin Amyloidosis (ATTR-CM)					✓	500,000+	\$20B+
Nulibry (fosdenopterin)	Molybdenum Cofactor Deficiency (MoCD) Type A					✓	<100	Partnered
Truseltiq (infigratinib)	Cholangiocarcinoma					✓	37,000	Partnered
Infigratinib	Achondroplasia (ACH)					Topline Readout	55,000+	\$2B+
	Hypochondroplasia (HCH)					Observational Run-in Fully Enrolled	55,000+	\$2B+
BBP-418	Limb-Girdle Muscular Dystrophy Type 2I/R9 (LGMD2I/R9)					Topline Readout	7,000+	\$1B+
Encaleret	Autosomal Dominant Hypocalcemia Type 1 (ADH1)					Topline Readout	25,000+	\$1B+
	Chronic Hypoparathyroidism (HP)						200,000+	\$1B+
BBP-812	Canavan Disease			Phase 1/2 Pivotal			1,000	TBD
Evanesco (depleter)	Transthyretin Amyloidosis (ATTR-CM)						500,000+	\$20B+
ADPKD (small molecule)	Autosomal Dominant Polycystic Kidney Disease						300,000	TBD
BridgeBio Oncology Therapeutics	3 Oncology Programs (various indications)						Various	Various
GondolaBio	17 Programs (various indications)						Various	Various

We are well-financed to hit a drumbeat of potential milestones in 2026 and beyond



BridgeBio ended 2025 with \$587.5M in cash, cash equivalents, and marketable securities



Cash burn declined in Q4 2025 relative to Q3 2025, driven by rising revenues and improving operating leverage

1H 2026



Infigratinib: ACH Topline



Encaleret: Initiate P2/3 pediatric ADH1



Encaleret: NDA submission



BBP-418: NDA submission

2H 2026



Infigratinib: NDA submission



Infigratinib: HCH P2 data readout



Encaleret: Initiate P3 CHP trial

1H 2027



Encaleret: FDA approval and product launch



BBP-418: FDA approval and product launch

Attruby® Approved for ATTR-CM



Key Attruby highlights



First and only approved product with a label specifying **near-complete stabilization** of TTR



Effect seen as early as 1 month – reduction in cumulative cardiovascular outcomes within the first month of treatment in patients with ATTR-CM



42% reduction in composite of all-cause mortality and recurrent cardiovascular-related hospitalization events at Month 30



50% reduction in the cumulative frequency of cardiovascular-related hospitalization events at Month 30

\$146M

**Q4 2025 Net Product
Revenue**

Continued commercial momentum and patient impact



7,804

Unique U.S. patient
prescriptions for Attruby



1,856

Unique U.S.
prescribing HCPs

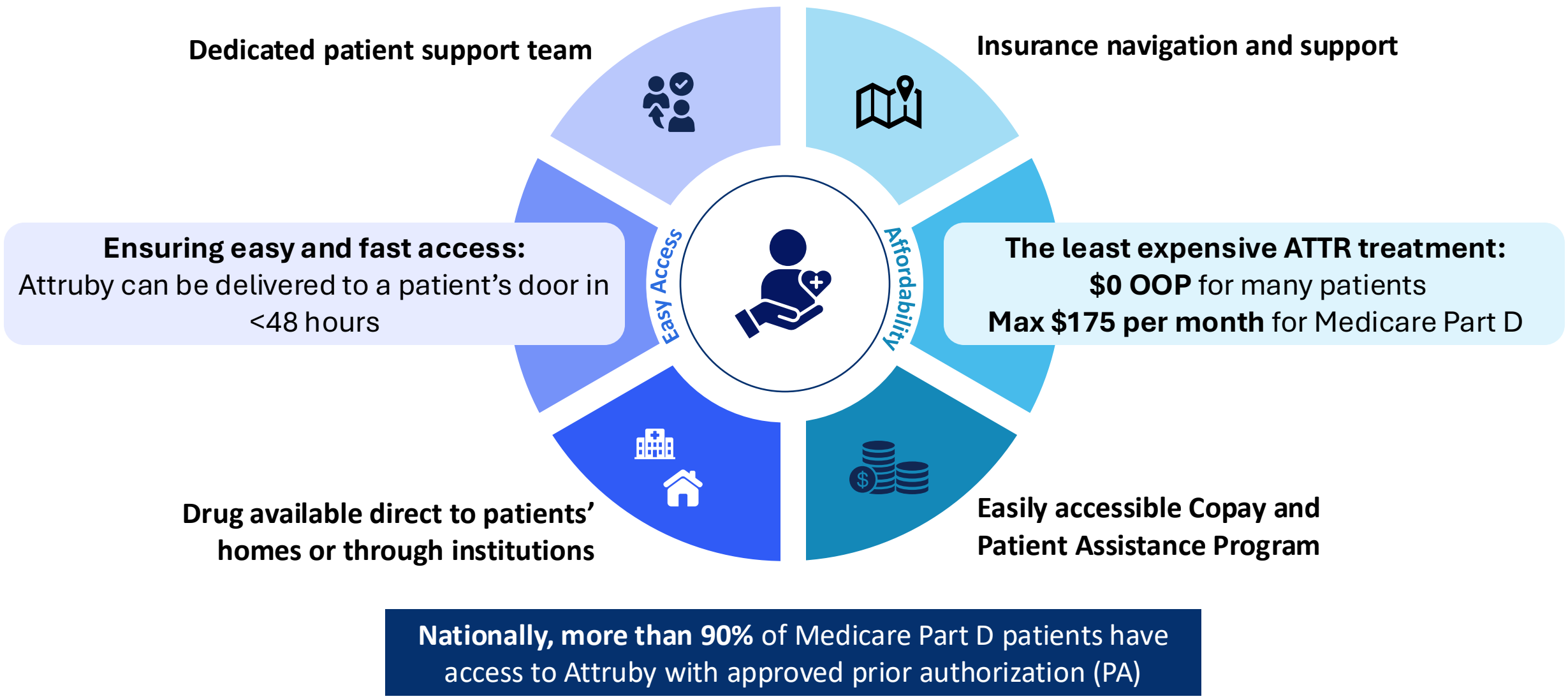


>25%

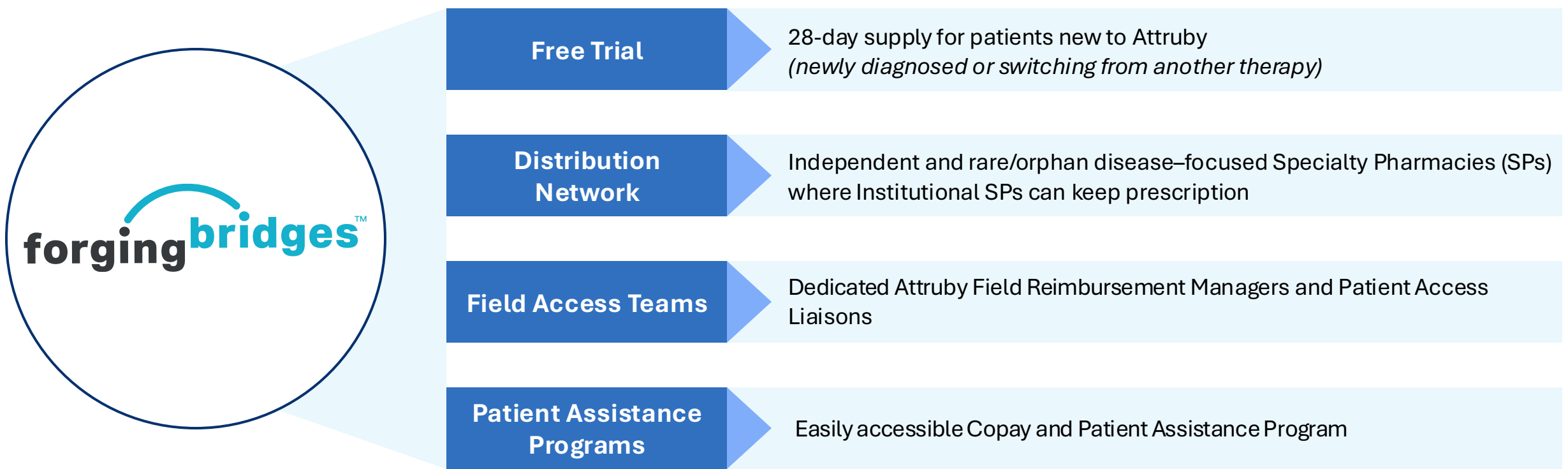
Estimated share of
NBRx for Attruby

As of Feb 20th, 2026

Our responsibility to patients spans beyond R&D – we are committed to ensuring the best access and affordability of any ATTR-CM medicine



We make it easy through simplified, differentiated, and generous access programs



Commitment to clinical trial patients
US patients who participated in the acoramidis clinical trials may receive Attruby at no cost for the duration of their medically indicated treatment

Beyonttra[®] (acoramidis) has seen rapid EU uptake with leading guideline endorsements



Beyonttra (acoramidis) Demonstrates Robust Commercial Uptake in Germany, Exceeding Projections

Global Guidelines Endorse Beyonttra (acoramidis) for Clinical Confidence, Credibility, and Impact

50%
NBRx share

Market Share

98% Volume
Share

Strong Uptake

Achieved by Germany 8 months into launch
(as of November 31, 2025)



Prescriber feedback from several leading German centers indicates >50% acoramidis initiation among newly eligible ATTR-CM patients; a few centers describe near-exclusive use.

Denmark awarded the national tender to Bayer, establishing Beyonttra as the only first-line treatment for ATTR-CM and suggesting that patients switch from tafamidis.

Germany's strong uptake signals powerful Demand ahead for the 15 EU countries with reimbursement and for future launches.

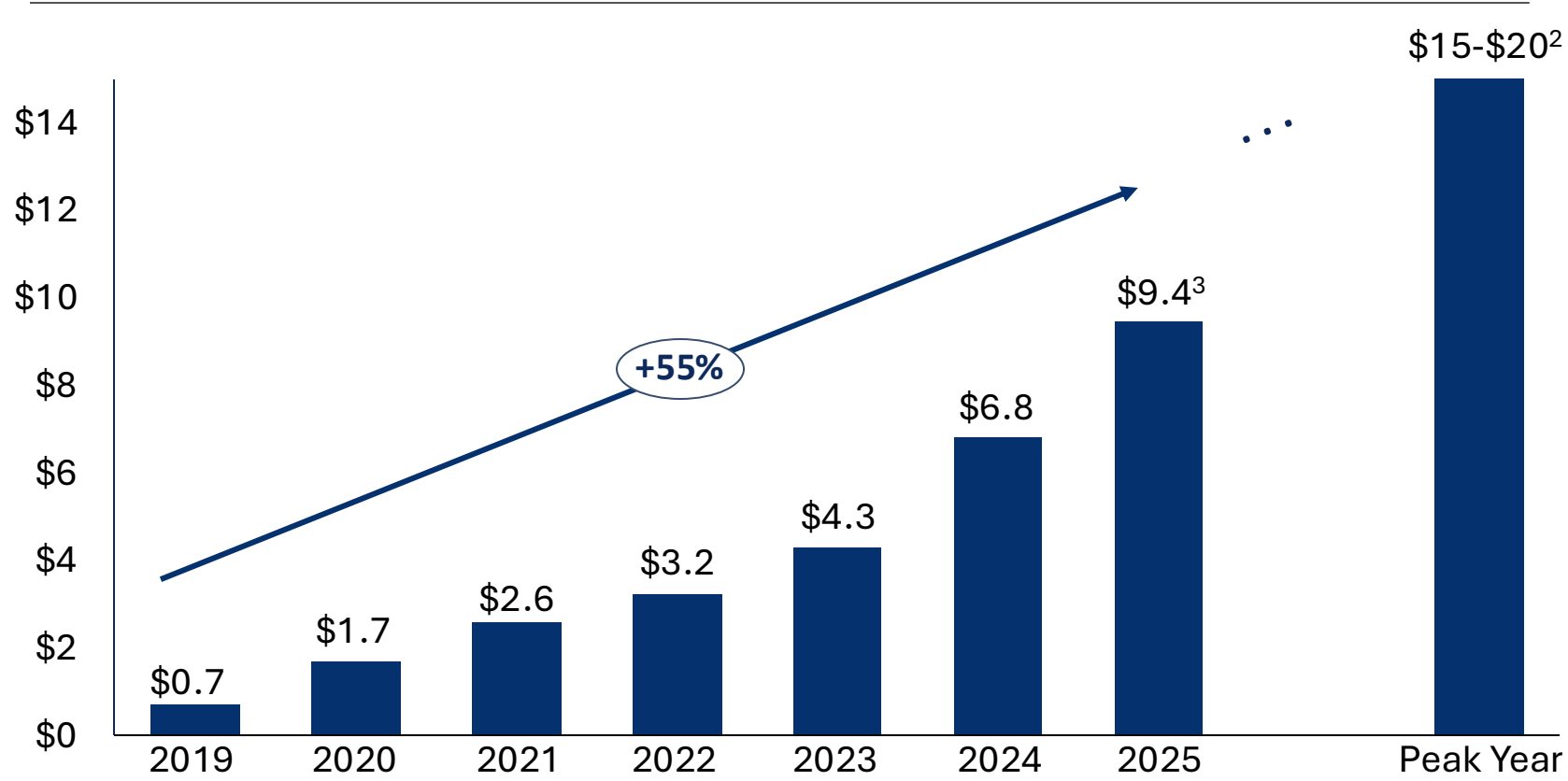


Acoramidis endorsed with highest tier recommendation and highlighted as near-complete stabilizer while tafamidis described as partial stabilizer

Beyonttra is partnered with Bayer in the EU. References: 1. JCS/JHFS 2025 Guideline; J Card Fail. 2025 Mar 27; S1071-9164(25)00100-9; 2. iCARDIO Alliance HF 2025, Chopra V. et al., Heart Lung Circ 34(7):e55–e82. 3. Marques N, Aguiar Rosa S, Cordeiro F, et al. Portuguese recommendations for the management of transthyretin amyloid cardiomyopathy (Part 1 of 2): Screening, diagnosis and treatment. Rev Port Cardiol. 2025;44(1):7-48. doi:10.1016/j.repc.2024.12.002. 4. IQVIA[™] LRx (Germany). NBRx share for acoramidis; period ending August 31 2025, reporting 15 Oct 2025. Definitions: JCS=Japanese Circulation Society; JHFS=Japanese Heart Failure Society; MHLW=Ministry of Health, Labour & Welfare; ROW=Rest of World; NBRx=New to brand prescriptions.

ATTR is a multi-billion-dollar market primed for continued expansion

Global annual ATTR market sales¹
\$B



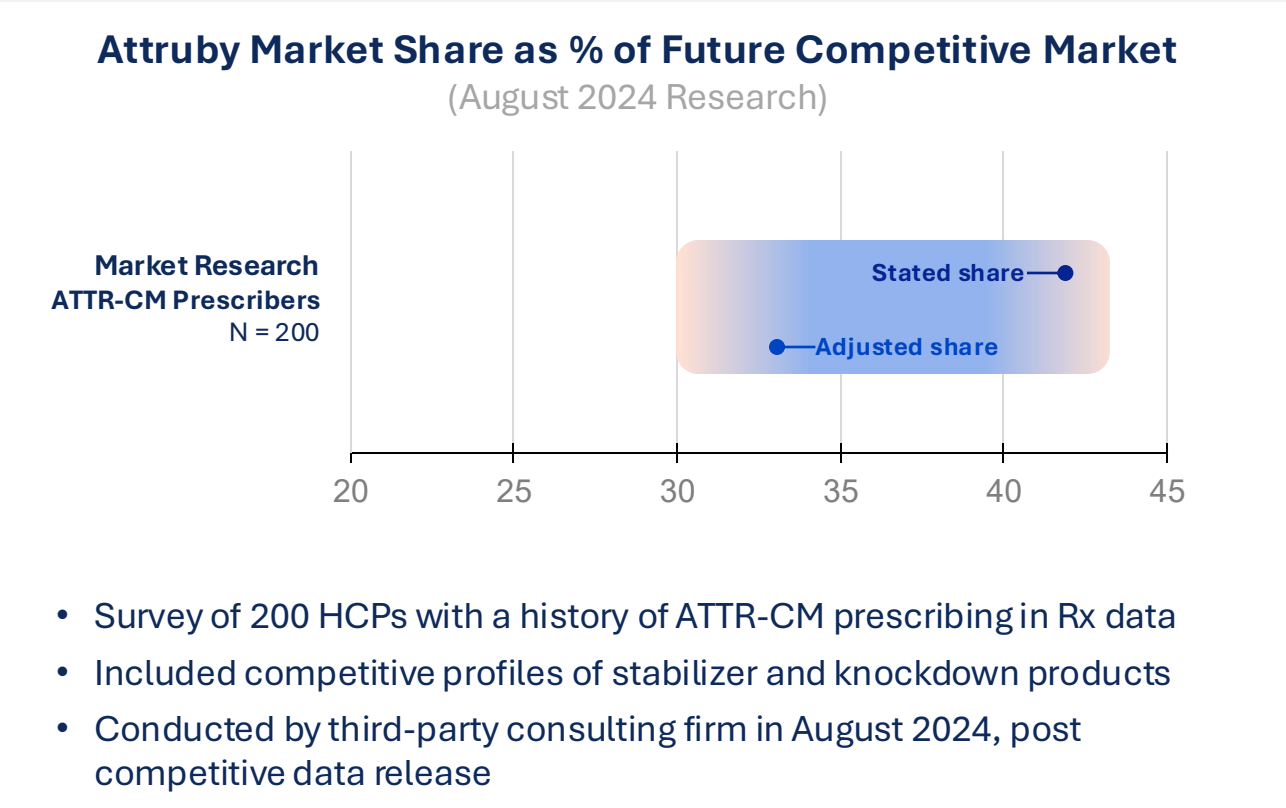
Market growth drivers include:

- With **more sponsors**, there is expanding disease awareness
- Increased global adoption of **non-invasive diagnostic tools**

¹ATTR market includes all approved drugs for ATTR-PN and ATTR-CM.
²Consensus estimates of \$15B-\$20B ATTR market.
³Based on 2025 numbers

Demand study shows significant Attruby market potential vs. partial stabilizer and partial knockdown

Surveyed HCPs expect 30 - 40%+ peak market share for Attruby



HCP sentiment towards Attruby is positive

- “Acoramidis showed dramatic reduction in cardiovascular hospitalizations, and improvement in patient QoL.”
HCP - Northeast
- “There is a mortality benefit and there are also quality of life benefits. It is an oral medication, so that will be well-liked by patients.”
HCP - West
- “Very impressive treatment effect and best data to date on what happens in a contemporary ATTR-CM population.”
HCP - Central

We continue to research the differentiated clinical role of Attruby

11 published manuscripts across 8 journals and 47 abstracts across 9 conferences in 2025

Clinical Outcomes

Effect of Acoramidis on Recurrent and Cumulative Cardiovascular Outcomes in ATTR-CM

Exploratory Analysis From ATTRIBUTE-CM



Ahmad Masri, MD, MS,¹ Daniel P. Judge, MD,² Frederick L. Ruberg, MD,³ Julian D. Gillmore, MD, PhD,⁴ Justin L. Grodin, MD, MPH,⁵ Laura Obici, MD,⁶ Mathew S. Maurer, MD,⁷ Marianna Fontana, MD, PhD,⁸ Steen Hvitfeldt Poulsen, MD,⁹ Peter van der Meer, PhD,¹⁰ Richard K. Cheng, MD, MS,¹¹ Sarah A.M. Cuddy, MD,¹² Amrut V. Ambardekar, MD,¹³ Kuangnan Xiong, PhD,¹⁴ Xiaofan Cao, PhD,¹⁵ Gillian Murtagh, MD,¹⁶ Suresh Siddhanti, PhD,¹⁷ Adam Castaño, MD, MS,¹⁸ Jean-François Tamby, MD,¹⁹ Jonathan C. Fox, MD, PhD,²⁰ Kevin M. Alexander, MD,²¹ Ronald Wittles, MD²²

Efficacy of Acoramidis on All-Cause Mortality and Cardiovascular Hospitalization in Transthyretin Amyloid Cardiomyopathy

Editorial Comment: Transthyretin Amyloid Cardiomyopathy



Authors: Daniel P. Judge, MD, Kevin M. Alexander, MD, Francesco Cappelli, MD, Marianna Fontana, MD, Pablo Garcia-Pavia, MD, Simon D.J. Gibbs, MD, Martha Grogan, MD, Mazen Hanna, MD, Ahmad Masri, MD, Mathew S. Maurer, MD, Laura Obici, MD, Prem Soman, MD, Xiaofan Cao, MD, Ted Lystig, MD, Jean-François Tamby, MD, Suresh Siddhanti, MD, Adam Castaño, MD, Leonid Katz, MD, Jonathan C. Fox, MD, Kenneth W. Mahaffey, MD, and Julian D. Gillmore, MD

Publication: JACC • Volume 85, Number 10

Continuous Acoramidis Treatment Significantly Reduced Risk of All-Cause Mortality and Cardiovascular-Related Hospitalization Through Month 42 in Participants with Wild-Type and Variant Transthyretin Amyloidosis Cardiomyopathy

Martha Grogan,¹ Amrut V. Ambardekar,² Justin L. Grodin,³ Lily K. Stern,⁴ Prem Soman,⁵ Marianna Fontana,⁶ Pablo Garcia-Pavia,⁷ Kuangnan Xiong,⁸ Suresh Siddhanti,⁹ Jean-François Tamby,¹⁰ Jonathan C. Fox,¹¹ Nowell Fine,¹² Mathew Maurer¹³

¹Wayne Clinic, Rochester, MN, USA; ²University of Colorado Anschutz Medical Campus, Aurora, CO, USA; ³University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁴California Heart Center, Cedars-Sinai Medical Center, Beverly Hills, CA, USA; ⁵University of Pittsburgh School of Medicine, UPMC Heart and Vascular Institute, Pittsburgh, PA, USA; ⁶University of Colorado Denver, Reginal Fife Hospital, Denver, CO, USA; ⁷University of Zaragoza, Hospital General de Zaragoza, Zaragoza, Spain; ⁸University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁹University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹⁰University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹¹University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹²University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹³University of Texas Southwestern Medical Center, Dallas, TX, USA

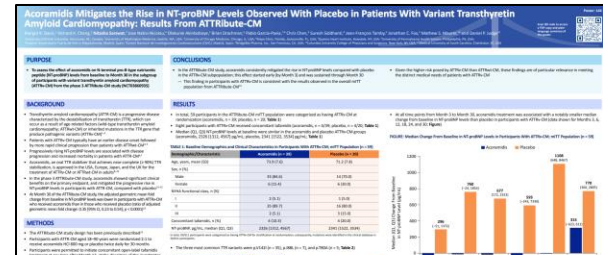
Presenter: Lily K. Stern

Variant

JAMA Cardiology

Efficacy of Acoramidis in Wild-Type and Variant Transthyretin Amyloid Cardiomyopathy: Results From ATTRIBUTE-CM and Its Open-Label Extension

Kevin M. Alexander, MD, Margot K. Davis, MD, Olakunle Alakiboye, MD, MPH, MBA, John Berk, MD, Kunal Bhatt, MD, Francesco Cappelli, MD, PhD, Sarah A.M. Cuddy, MD, Marianna Fontana, MD, PhD, Pablo Garcia-Pavia, MD, PhD, Julian D. Gillmore, MD, PhD, Jan M. Griffin, MD, Justin L. Grodin, MD, MPH, Daniel P. Judge, MD, Michel G. Khouri, MD, Kaitlyn Lam, MBBS, Ahmad Masri, MD, MS, Mathew S. Maurer, MD, Laura Obici, MD, Frederick L. Ruberg, MD, Nitasha Sarawat, MD, Keyur Shah, MD, Prem Soman, MD, PhD, Lily Stern, MD, Richard Wright, MD, Kuangnan Xiong, PhD, Xiaofan Cao, PhD, Ted Lystig, MD, Jean-François Tamby, MD, Adam Castaño, MD, MS, Leonid Katz, MD, Uma Sinha, PhD, Jonathan C. Fox, MD, PhD, Scott D. Solomon, MD, Martha Grogan, MD



Acoramidis Reduces All-Cause Mortality and First Cardiovascular Hospitalization in Participants With Variant Transthyretin Amyloid Cardiomyopathy: Results From the ATTRIBUTE-CM Study

Margot K. Davis,¹ Jan M. Griffin,² Nitasha Sarawat,³ Justin L. Grodin,⁴ Kevin M. Alexander,⁵ Daniel P. Judge,⁶ Julian D. Gillmore,⁷ Francesco Cappelli,⁸ Richard Wright,⁹ Prem Soman,¹⁰ John Berk,¹¹ Xiaofan Cao,¹² Jean-François Tamby,¹³ Adam Castaño,¹⁴ Jonathan C. Fox,¹⁵ Keyur Shah,¹⁶ Martha Grogan¹⁷

¹University of British Columbia, Vancouver, BC, Canada; ²Medical University of South Carolina, Charleston, SC, USA; ³University of Chicago Medicine, Chicago, IL, USA; ⁴University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁵University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁶University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁷University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁸University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁹University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹⁰University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹¹University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹²University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹³University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹⁴University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹⁵University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹⁶University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹⁷University of Texas Southwestern Medical Center, Dallas, TX, USA

Biomarkers

Early Increase in Serum Transthyretin by Acoramidis Independently Predicts Improved Survival in TTR Amyloid Cardiomyopathy



Mathew S. Maurer, MD,¹ Daniel P. Judge, MD,² Julian D. Gillmore, MD, PhD,³ Pablo Garcia-Pavia, MD, PhD,⁴ Ahmad Masri, MD,⁵ Francesco Cappelli, MD, PhD,⁶ Kevin M. Alexander, MD, MS,⁷ Nitasha Sarawat, MD,⁸ Martha Grogan, MD,⁹ Amrut V. Ambardekar, MD,¹⁰ Anique Ducharme, MD,¹¹ Steen H. Poulsen, MD,¹² Kaitlyn Lam, MBBS,¹³ Laura Obici, MD,¹⁴ Prem Soman, MD, PhD,¹⁵ Satish Rao, PhD,¹⁶ Jean-François Tamby, MD,¹⁷ Adam Castaño, MD, MS,¹⁸ Jonathan C. Fox, MD, PhD,¹⁹ Brian Adam, PhD,²⁰ Surendhar Reddy Cheppala, PhD,²¹ Bill Poland, PhD,²² Uma Sinha, PhD,²³ Marianna Fontana, MD, PhD²⁴

SERUM TRANSTHYRETIN LEVELS AT DAY 28 ARE ASSOCIATED WITH CARDIOVASCULAR OUTCOMES: INSIGHTS FROM THE ATTRIBUTE-CM STUDY

Nitasha Sarawat,¹ Amrut V. Ambardekar,² Richard Wright,³ Margot K. Davis,⁴ Julian D. Gillmore,⁵ Justin L. Grodin,⁶ Joshua D. Mitchell,⁷ Deirdre Mooney,⁸ Jose Nativi-Nicolau,⁹ Frederick L. Ruberg,¹⁰ Chris Chen,¹¹ Alan Ji,¹² Jean-François Tamby,¹³ Uma Sinha,¹⁴ Jonathan C. Fox,¹⁵ Mathew S. Maurer,¹⁶ and Richard K. Cheng¹⁷

¹Division of Cardiovascular Medicine, University of Chicago Medicine, Chicago, IL, USA; ²Division of Cardiology, Department of Medicine, University of Colorado, Aurora, CO, USA; ³Mayo Clinic, Rochester, MN, USA; ⁴University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁵University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁶University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁷University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁸University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁹University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹⁰University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹¹University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹²University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹³University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹⁴University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹⁵University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹⁶University of Texas Southwestern Medical Center, Dallas, TX, USA; ¹⁷University of Texas Southwestern Medical Center, Dallas, TX, USA

ACORAMIDIS IMPROVES SERUM TTR LEVELS IN PATIENTS WITH WILD-TYPE OR VARIANT TRANSTHYRETIN AMYLOID CARDIOMYOPATHY – RESULTS FROM ATTRIBUTE-CM

Mathew Maurer,¹ Nitasha Sarawat,² Martha Grogan,³ Amrut V. Ambardekar,⁴ Anique Ducharme,⁵ Steen Hvitfeldt Poulsen,⁶ Justin Grodin,⁷ John Berk,⁸ Jing Du,⁹ Alan X. Ji,¹⁰ Satish Rao,¹¹ Jean-François Tamby,¹² Adam Castaño,¹³ Jonathan C. Fox,¹⁴ and Uma Sinha¹⁵

¹Columbia College of Physicians and Surgeons, New York, NY, USA; ²University of Chicago Medicine, Chicago, IL, USA; ³Mayo Clinic, Rochester, MN, USA; ⁴University of Colorado, Aurora, CO, USA; ⁵Montreal Heart Institute and Université de Montréal, Montréal, QC, Canada; ⁶Aarhus University Hospital, Aarhus, Denmark; ⁷Parkland Health and Hospital System, and University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁸Boston University School of Medicine, Boston, MA, USA; ⁹BridgeBio Pharma, Inc., San Francisco, CA, USA

HEOR/Other

2026 Themes

Variant

Arrhythmias

Early Tx Effect

Biomarkers

Prevention /
ACT-EARLY

And more...

Attruby's advantages stem from the fact that it is the most potent stabilizer

Acoramidis is the only stabilizer with “near complete” stabilization in the label



- ✓ **Sees more target**
(superior free fraction)
- ✓ **Binds more target**
(superior K_d)
- ✓ **Glues the target together stronger**
(enthalpic binding mode)

The multiplicity of advantages to superior stabilization continue to be better understood and elucidated in novel research

RESEARCH ARTICLE | BIOCHEMISTRY

Mass spectrometry footprinting reveals how kinetic stabilizers counteract transthyretin dynamics altered by pathogenic mutations

Francisca Pinheiro , Ravi Kant , Saketh Chemuru, , and Salvador Ventura

Edited by Jeffery Kelly, Scripps Research, La Jolla, CA; received July 25, 2025; accepted November 19, 2025

December 31, 2025 | 123 (1) e2519908122 | <https://doi.org/10.1073/pnas.2519908122>

“Our thermodynamic analysis further supports the notion that binding enthalpy (ΔH), not affinity (K_d or ΔG), better predicts the conformational stabilization imparted by kinetic stabilizers.... These results underscore the need to prioritize enthalpy-driven interactions during stabilizer design.”

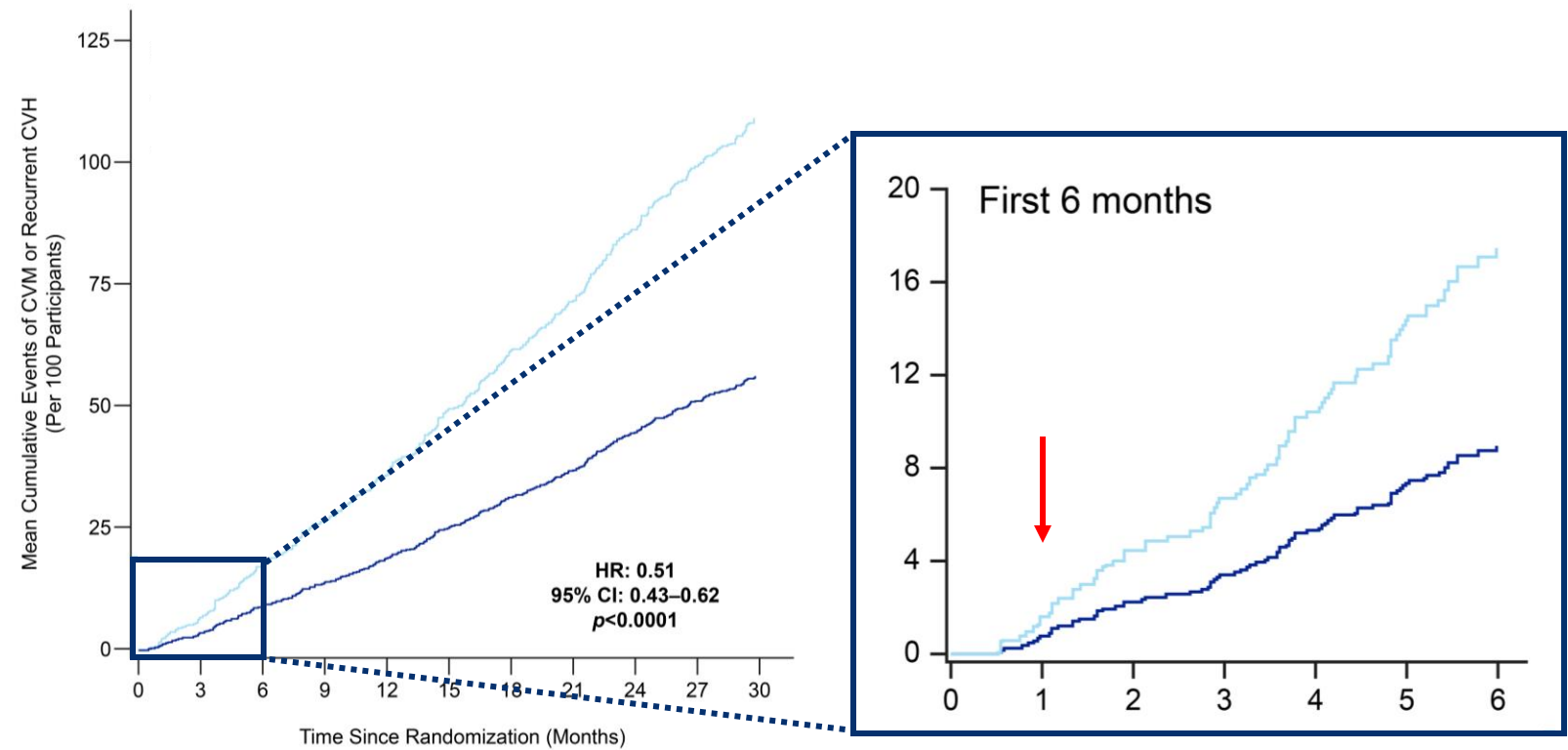
“Given the variability in stoichiometry in the experiments between tafamidis and AG10 and TTR, **the data always tell the same story, that AG10 is better than tafamidis** as would be expected from the determined binding constants.”

– Prof. Jeffery Kelly (inventor of tafamidis) in email correspondence with Dr. Isabella Graef, February 12, 2013. Bold added.

Attruby significantly reduced the risk of CVM or recurrent CVH through Month 30 vs. placebo by 49% with curve separation starting by Month 1

Estimated Mean Cumulative Events of CVM or Recurrent CVH Through Month 30

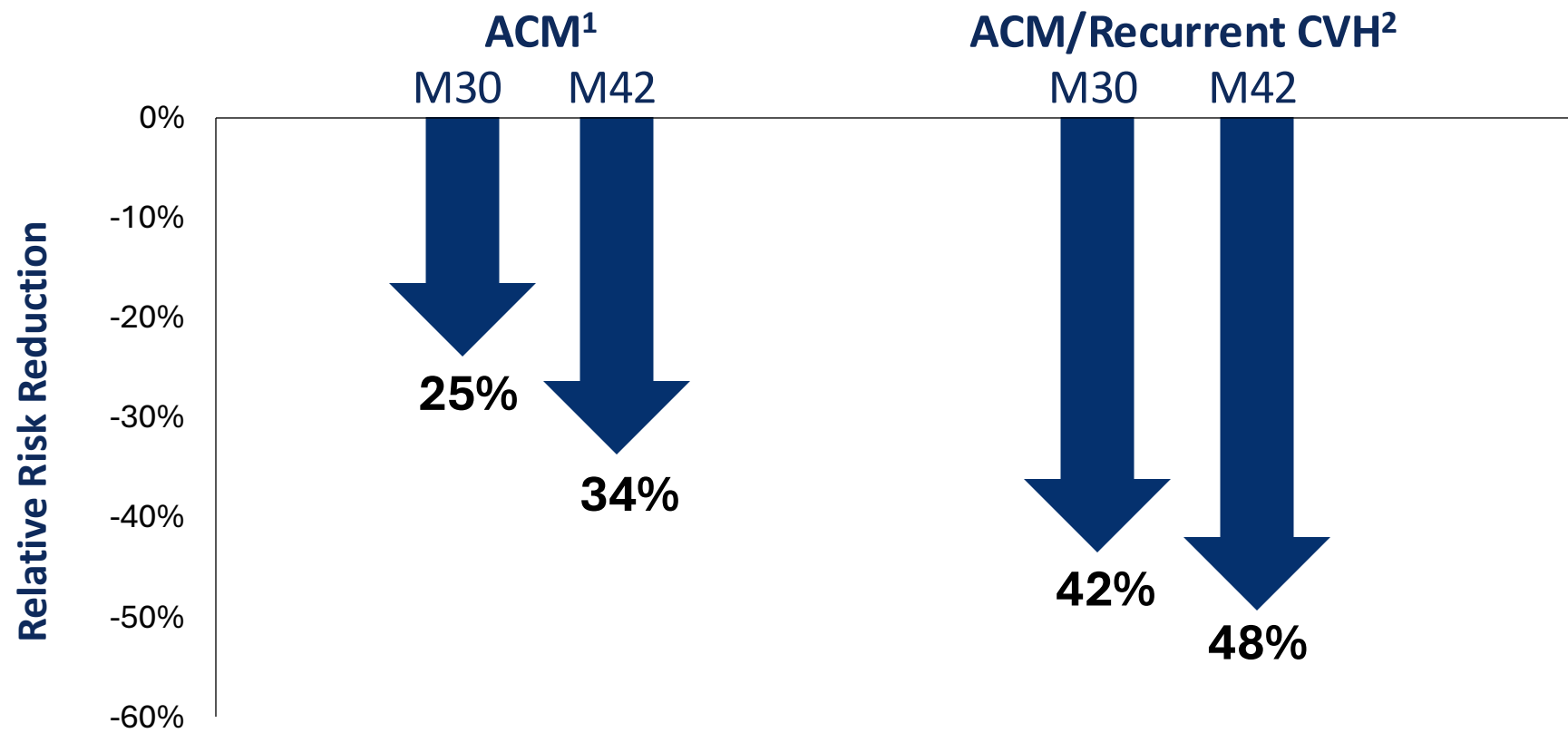
(mITT Population, Acoramidis, n = 409; Placebo, n = 202)



	Acoramidis (n = 409)	Placebo (n = 202)
Participants with CVM or recurrent CVH, n (%)	136 (33.3)	98 (48.5)
Hazard ratio (95% CI) ^a	0.51 (0.43, 0.62)	
p value	< 0.0001	

Source: Masri et al. (2025) Early, Long-Term Reduction in CV-Outcomes With Acoramidis, JACC; HFSA 2025 Presentation (Masri) Acoramidis Reduces Cumulative Cardiovascular Outcomes Within the First Month of Treatment in Transthyretin Amyloid Cardiomyopathy: Results From ATTRIBUTE-CM; Data are for the mITT population in the ATTRIBUTE-CM study, defined as all the participants who had undergone randomization, received at least one dose of acoramidis or placebo, and had at least one efficacy evaluation after baseline; participants with eGFR <30 mL/min/1.73 m² were excluded a Modified Andersen-Gill model with a robust variance estimator, with treatment, age, NYHA class, genotype, eGFR, and log-transformed baseline NT-proBNP as covariates CI, confidence interval; CVH, cardiovascular-related hospitalization; CVM, cardiovascular mortality; eGFR, estimated glomerular filtration rate; mITT, modified intention-to-treat; NT-proBNP; N-terminal pro-B-type natriuretic peptide; NYHA, New York Heart Association

Published data from the OLE further support Attruby's statistically significant benefit on ACM and ACM/Recurrent CVH

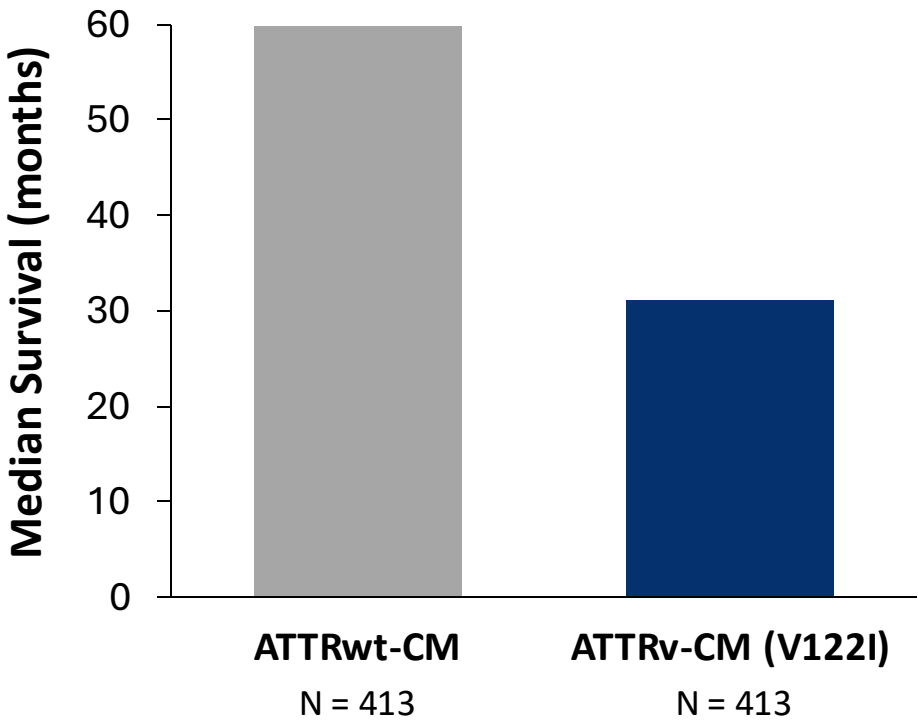


Attruby resulted in a **statistically significant** ACM and ACM/Recurrent CVH relative risk reduction at **both Month 30 and Month 42**

Attruby delivers outstanding results in patients with poor prognosis

Natural History

V122I ATTRv-CM has an aggressive phenotype and poor prognosis¹



ATTRibute-CM mITT Population

Statistically significant benefit on composite ACM or first CVH in ATTRv-CM participants vs. placebo²

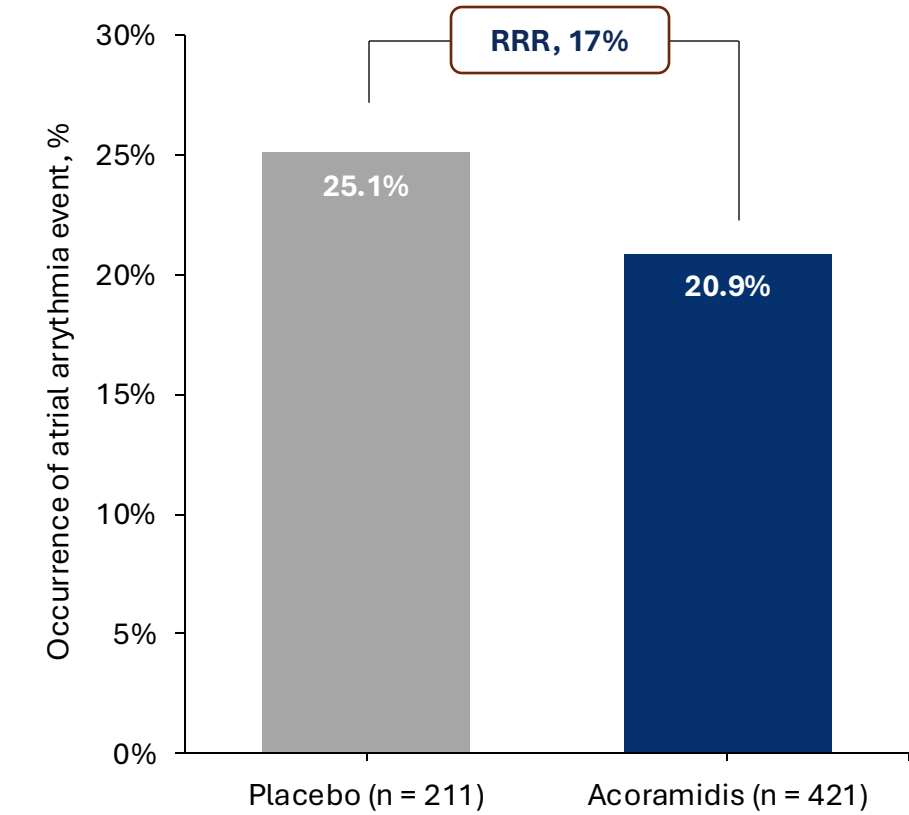
	N (%)	Hazard Ratio (95% CI)	p value
Overall Population	611 (100%)	0.65 (0.50-0.83)	0.0008
ATTRv-CM	59 (9.7%)	0.41 (0.21-0.81)	0.0109
V122I	35 (5.7%)	0.31 (0.12-0.81)	0.016

Unprecedented improvements in QOL and Functional Capacity in Variant ATTR-CM³

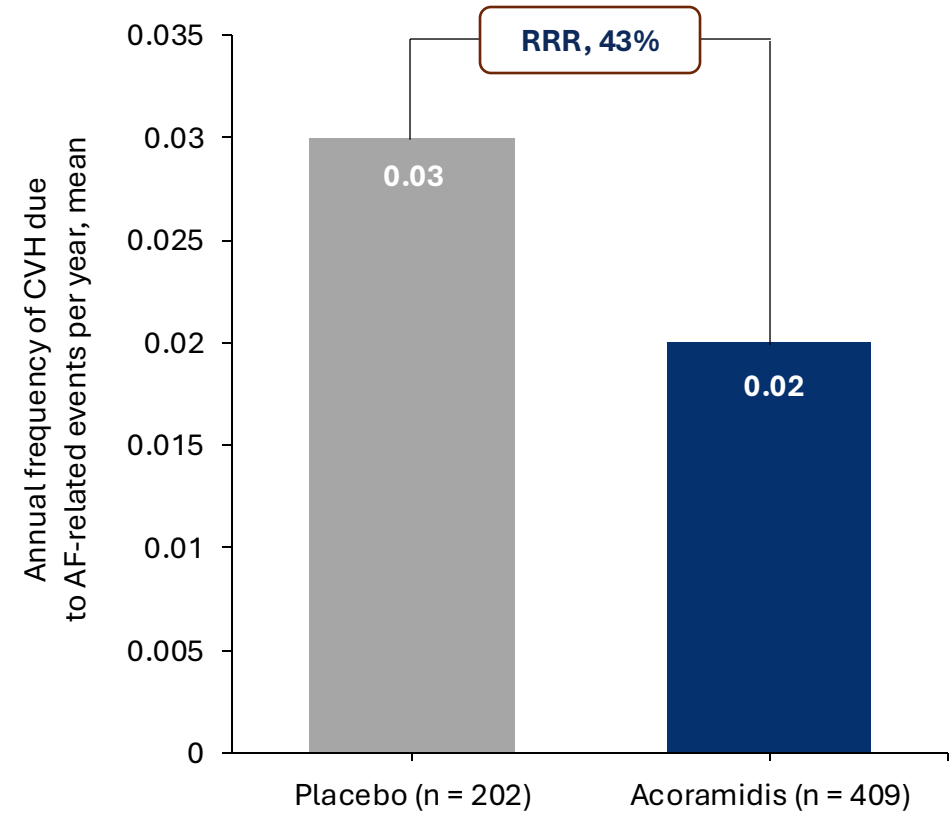
	LS Mean Difference Acor vs. Placebo	p value
6MWD	+86.7 meters	0.0048
KCCQ-OS	+20.3 points	0.0019

Acoramidis reduced the risk of AF adverse events and AF-related cardiovascular hospitalizations

Incidence AF Adverse Event

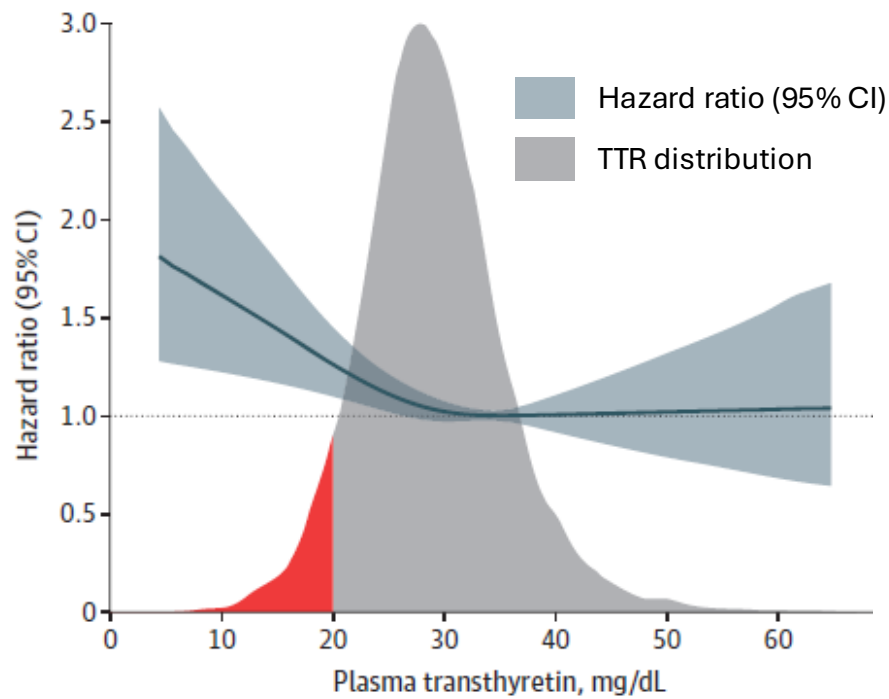


Annual Frequency of CVH Due to AF

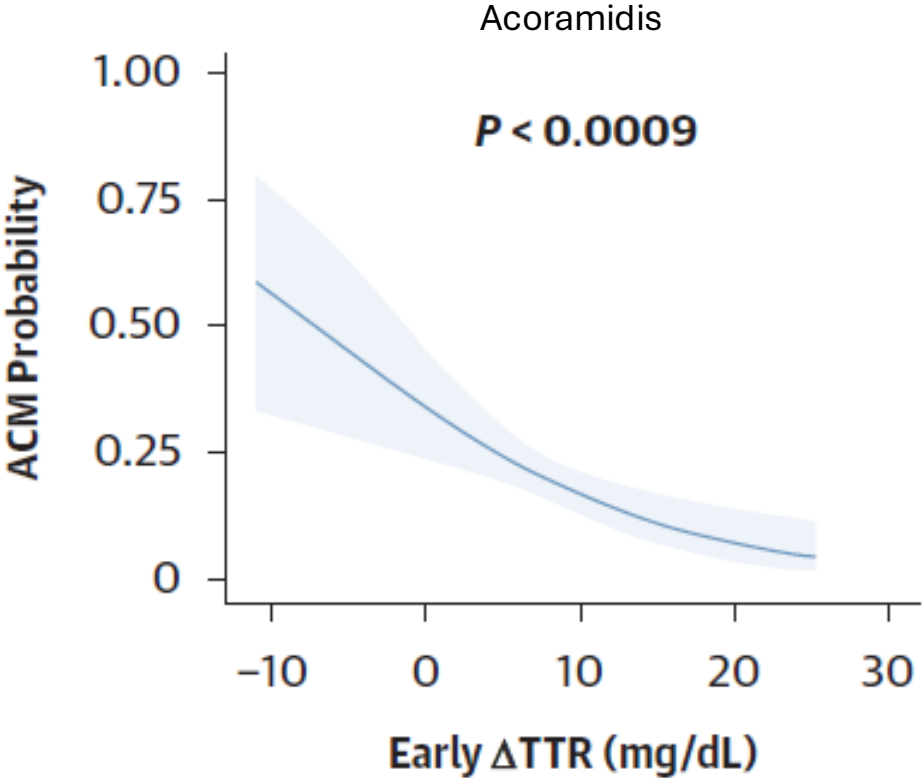


Additional data: Elevated TTR levels are associated with improved survival

Higher TTR concentration associated with greater life expectancy (N≈102K individuals)¹



Each 5 mg/dL rise in serum TTR is associated with ~32% lower odds of death by 30 months



¹Figure adapted from Christoffersen, M. et al., JAMA Cardiology, 2024. Figure 3D: Cardiovascular mortality, adjusted multifactorially.
²Maurer et al. Early Increase in Serum Transthyretin by Acoramidis Independently Predicts Improved Survival in TTR Amyloid Cardiomyopathy (JACC 2025)

Recruitment continues for ACT-EARLY, the first ATTR primary prevention clinical trial, opening a new potential therapeutic paradigm for patients



Current therapeutic paradigm: slow the progression of disease in patients with diagnosed ATTR



ACT-EARLY is exploring whether acoramidis has the potential to be used to delay the onset of, or prevent, ATTR

Deleter for ATTR-CM

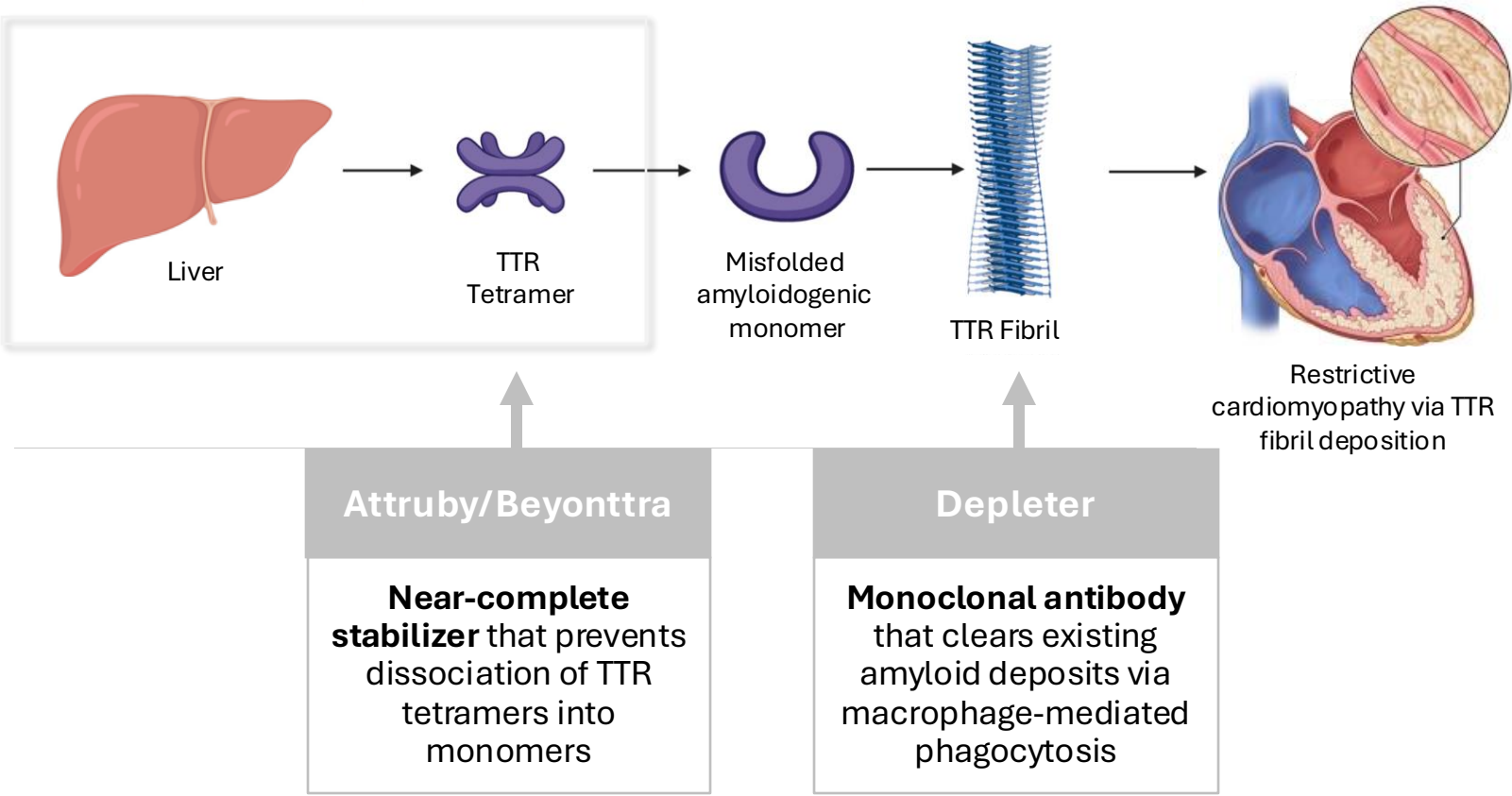


BridgeBio has initiated a depleter program to explore the potential of ATTR-CM disease reversal

ATTR-CM Disease Pathophysiology

Existing TTR therapies target upstream tetramer stabilization and synthesis...

...but unmet need remains for patients with existing amyloid deposits for downstream clearance



Led by Renowned Antibody Expert

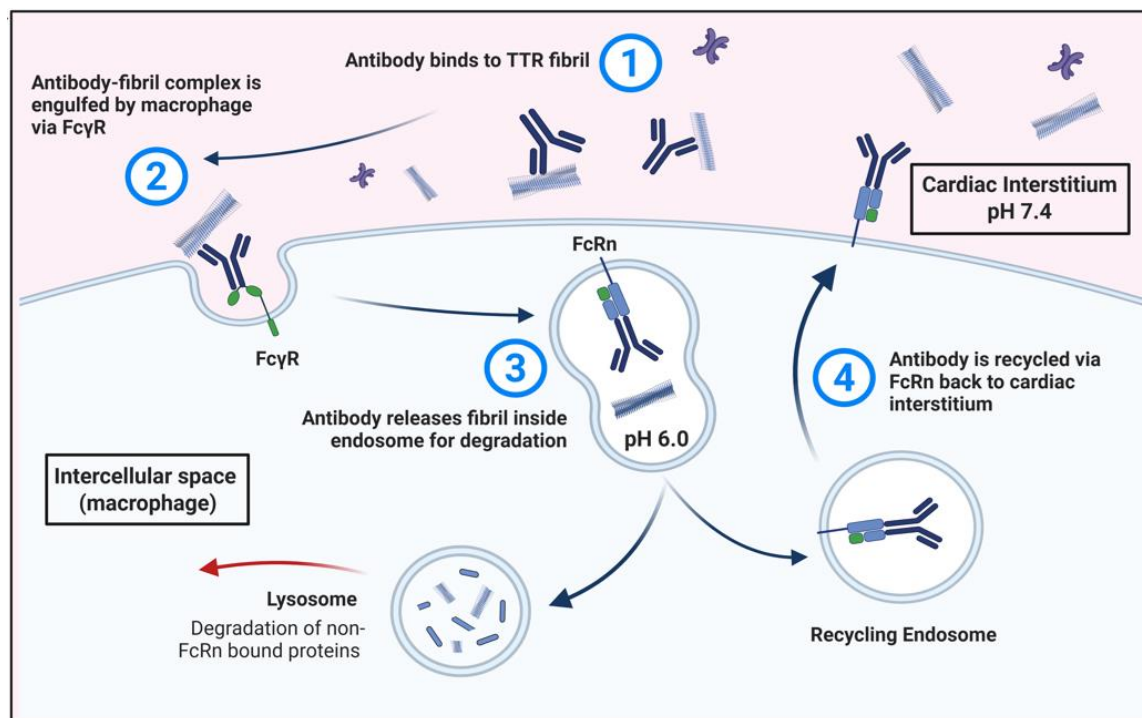


Dr. Richard Scheller

- Former Professor, Stanford
- Former CSO, Genentech
- Former EVP and Executive Committee, Roche Genentech
- Chairman of R&D, BridgeBio

BridgeBio's depleter is engineered across 4 novel properties for potential differentiation on clinical efficacy and dosing convenience

Depleter Mechanism of Action



Keywords



BridgeBio's Differentiated Target Properties

- ① Improved fibril:tetramer binding ratio**
 - >10× preferential binding to misfolded TTR fibrils vs. native TTR tetramers
 - ➔ **Binds more target**
- ② Faster macrophage recruitment**
 - First depleter to activate Fcγ receptors to boost macrophage activity
 - ➔ **Clears more target**
- ③ pH sensitivity**
 - Intentionally designed for pH-dependent antigen release inside macrophages
 - ➔ **Extends antibody half-life**
- ④ Half-life extension**
 - First depleter engineered for enhanced FcRn binding
 - ➔ **Extends antibody half-life**

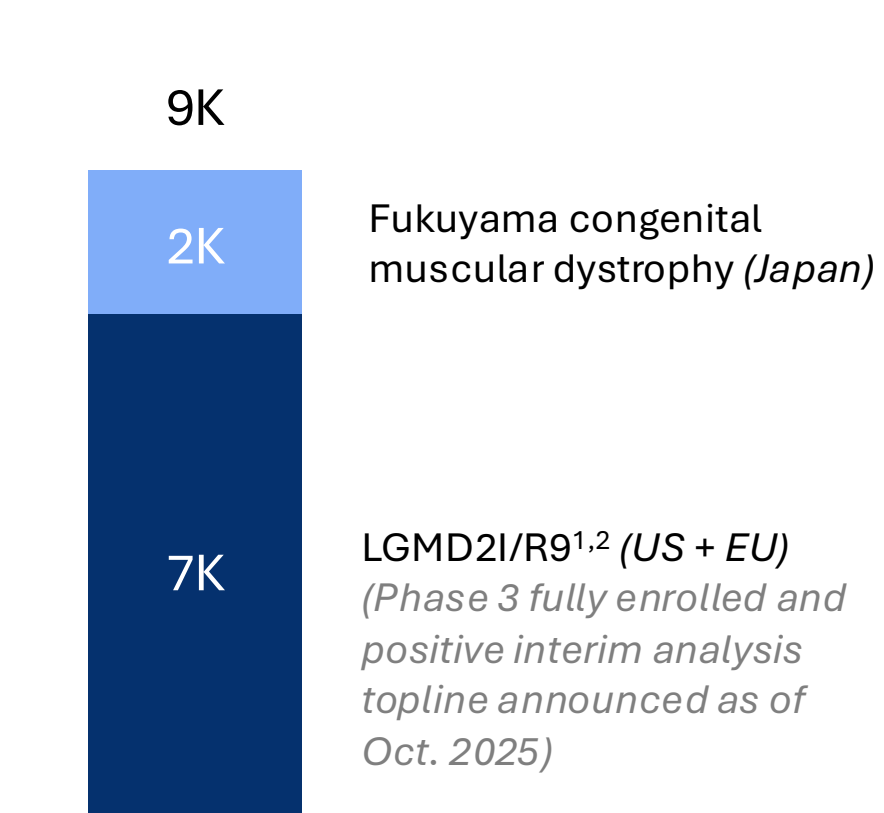
Program expected to advance into the clinic in 2027–2028

BBP-418



LGMD2I/R9 is a progressive neuromuscular disease with high unmet need, representing a >\$1B market opportunity in the US and EU

Addressable patients by indication



Unmet need

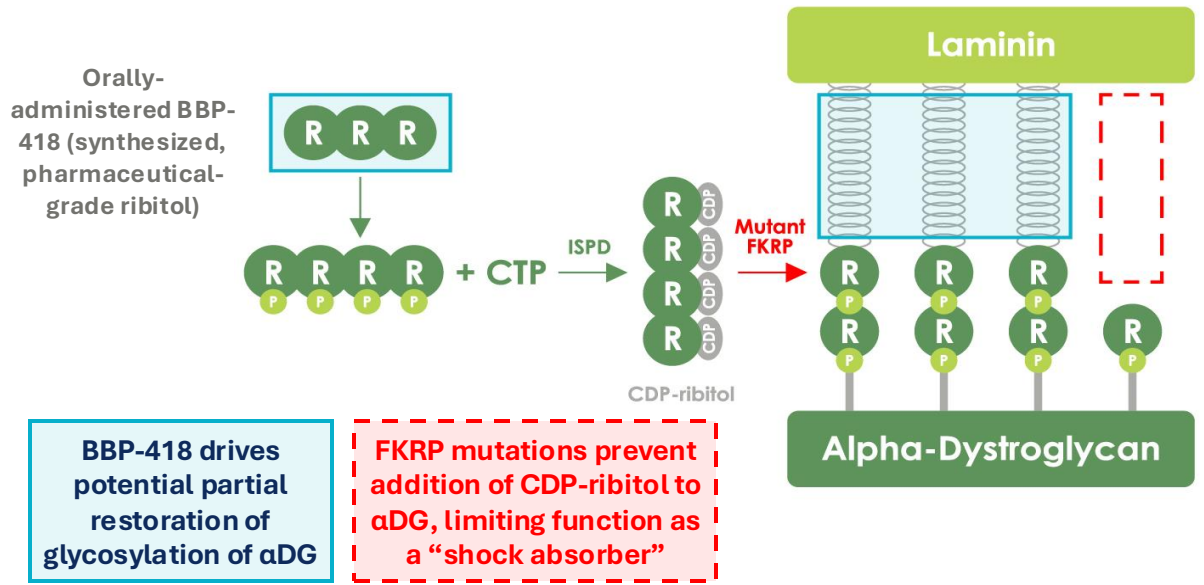
- **LGMD2I/R9 is an inherited neuromuscular disorder** characterized by lower-limb weakness and loss of ambulation as well as respiratory decline and cardiac dysfunction
- **No approved therapies** for LGMD2I/R9
- Current **standard of care is aimed at symptom management** and includes physical therapy, steroids, and pain management
- **Standard of care does not prevent continuous and progressive decline** in LGMD2I/R9 patients

Market opportunity \$1B+

BBP-418 is an oral, disease-modifying therapy that targets LGMD2I/R9 at its source by restoring glycosylation of alpha-dystroglycan

Mechanism

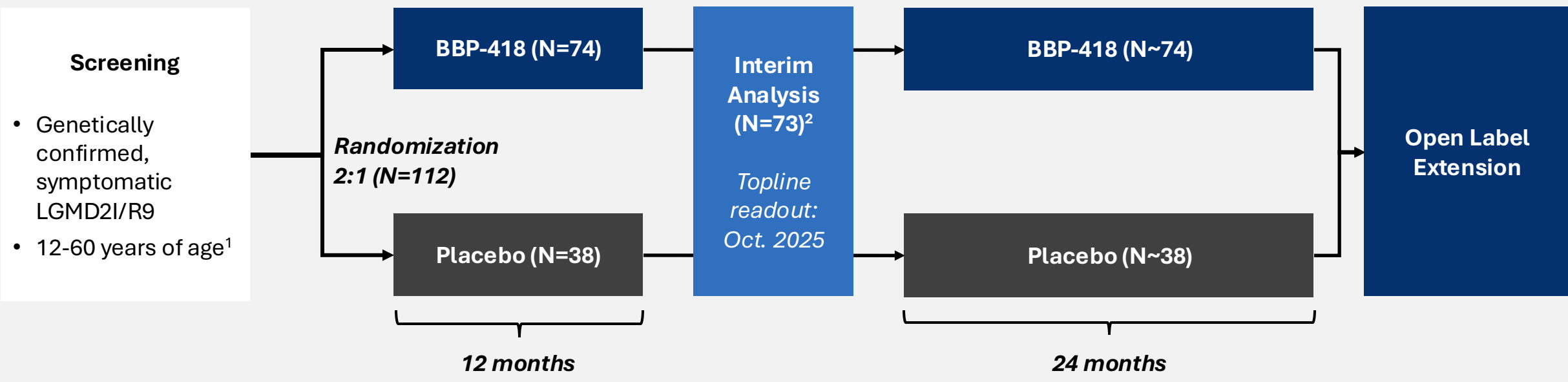
-  FKRPs glycosylates alpha-dystroglycan (αDG), stabilizing muscle cells by binding extracellular ligands to act as a “shock absorber” for muscle fibers
-  Partial loss of function in FKRPs results in dysfunctional, hypo-glycosylated αDG in muscle cells, increasing cell susceptibility to damage
-  Supply supraphysiological levels of synthesized, pharmaceutical grade ribitol upstream aiming to drive residual activity of mutant FKRPs enzyme and increase αDG glycosylation levels



Design Principles

-  **Provide first disease-modifying therapy**
For patients with LGMD2I/R9 and potentially applicable for other α-dystroglycanopathies
-  **Avoid safety concerns with FKRPs modulation**
Avoid off-target effects using a synthesized version of an endogenous compound with an encouraging safety profile
-  **Convenient oral medicine**
To reduce burden for patients and mitigate safety concerns

FORTIFY is an ongoing randomized, placebo-controlled Phase 3 study; we reported topline results from a planned interim analysis in Oct. 2025



Pre-specified Interim Endpoints³:

- Glycosylated αDG (*primary*)
- Serum creatine kinase (CK)
- Ambulatory measure: 100MTT
- Pulmonary function: FVC

Final Analysis Endpoints⁴:

- NSAD (*primary*)
- Ambulatory measures
 - 10MWT
 - 100MTT
- Pulmonary function: FVC
- Upper limb function: PUL 2.0
- QoL measures

Placebo & BBP-418 arms were stratified by age group (adult vs. pediatric), ambulatory status, and genotype (L276I homozygous vs. Other *FKRP* genotype)

Interim analysis topline data reflect highly statistically significant results on all pre-specified endpoints, including biomarker and clinical measures at 12 months

BBP-418 in LGMD2I/R9: FORTIFY Phase 3 interim analysis topline results

Primary endpoint (3 months)	p-value
Change from baseline in glycosylated αDG, % of control	p<0.0001
.....	
Key secondary endpoints (12 months)	p-value
Change from baseline in glycosylated αDG, % of control	p<0.0001
Change from baseline in serum CK, U/L	p<0.0001
Change from baseline in 100MTT, m/s	p<0.0001
Change from baseline in FVC, % predicted	p=0.0071

Unprecedented and consistent improvement across primary and all key secondary efficacy endpoints combined with well-tolerated safety profile

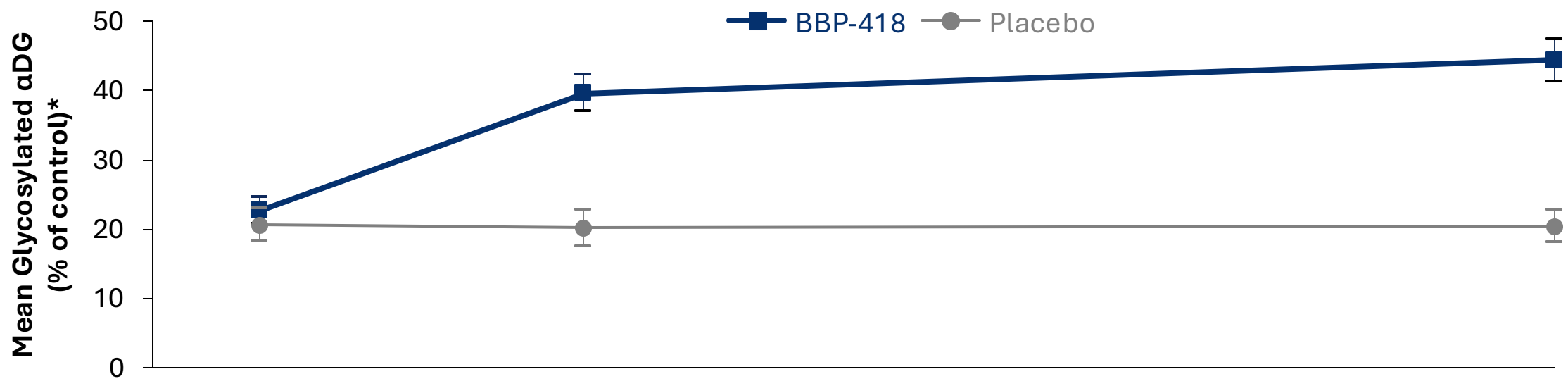


Ph. 3 FORTIFY interim analysis

Type	Endpoint	Upside case target	Outcome observed
Primary (3 months)	Glycosylated αDG	- Statistically significant increase vs. placebo 1.5x CFB in BBP-418 treated vs. approx. no change in placebo	✓ Highly statistically significant increase (1.8x CFB; absolute increase of 17% of control) at 3 months ✓ Increase sustained at 12 months
	Creatine kinase (CK)	Average decline of ≥50% CFB in BBP-418 treated	✓ Highly statistically significant average decline of 82% in BBP-418 treated
Key secondary (12 months)	- Ambulatory measures (100MTT) - Pulmonary (FVC)	Trends in one or more measures favoring BBP-418 treated vs. placebo	✓ Statistically significant and clinically meaningful improvement in BBP-418 treated ✓ 100MTT: Increase in velocity of 0.14 m/s from baseline and 0.27 m/s vs. placebo ✓ FVC: Increase in ventilatory capacity of ~3% predicted volume from baseline and a difference of ~5% predicted volume vs. placebo
Exploratory (12 months)	NSAD	Trend favoring BBP-418 treated vs. placebo	✓ Nominally statistically significant* and clinically meaningful improvement (2.6 point benefit vs. placebo)
Safety		Well-tolerated (consistent with Ph. 2 results)	✓ Well-tolerated; consistent with Ph. 2 results

Interim analysis showed statistically significant increase (+17% of control) from baseline in glycosylated αDG for BBP-418 treated, which was sustained at 12 months

Mean glycosylated αDG (% of control)* (± standard error of the mean, SEM)

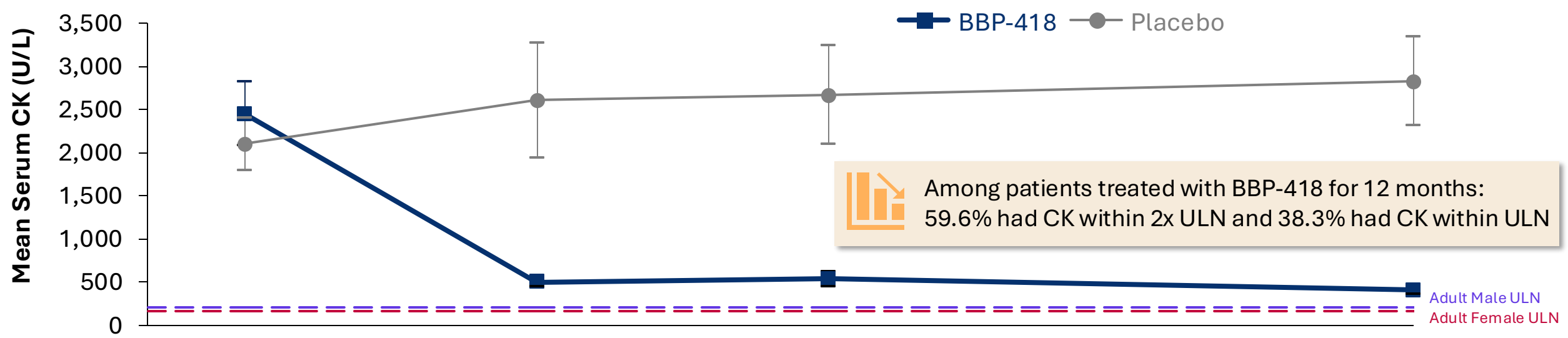


Timepoint	0 months	3 months	12 months
Cohort	Full analysis set [†]	Full analysis set [†]	Full analysis set [†]
BBP-418	22.7% (n=73)	39.6% (n=71)	44.5% (n=49)
Placebo	20.7% (n=38)	20.2% (n=36)	20.4% (n=22)

*% of control describes glycosylated αDG levels relative to a control reference standard from a healthy donor. [†]The Full Analysis Set consists of randomized participants who had at least one efficacy assessment postrandomization. αDG=alpha-dystroglycan

In addition, patients treated with BBP-418 experienced a rapid reduction in serum CK from baseline at 3 months that was sustained at 12 months

Mean CK (U/L) ($\pm$ SEM)*

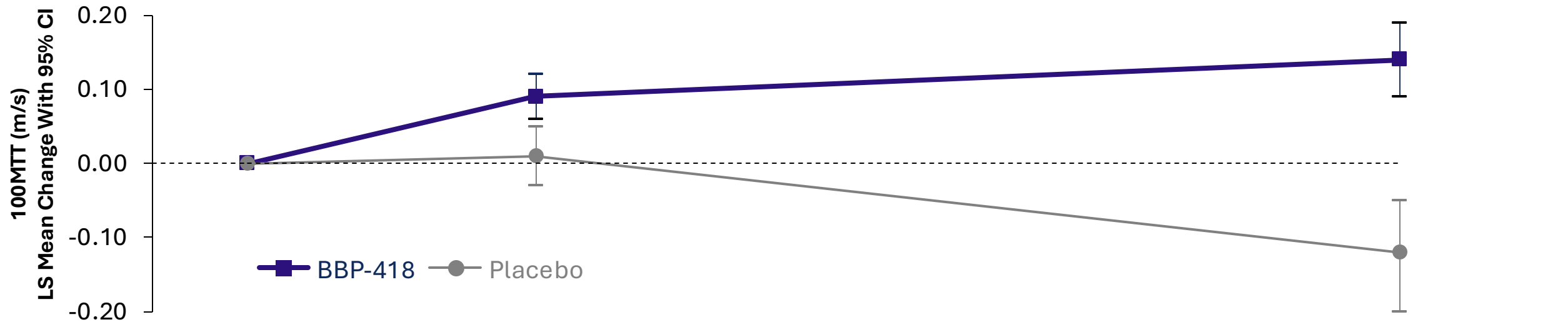


Timepoint	0 months		3 months		6 months		12 months
Cohort	Primary efficacy set [†]		Primary efficacy set [†]		Primary efficacy set [†]		Primary efficacy set [†]
BBP-418	2449.5 (n=67)		500.7 (n=45)		534.1 (n=67)		410.1 (n=47)
Placebo	2099.5 (n=36)		2607.4 (n=23)		2670.9 (n=35)		2828.5 (n=22)

*Mean $\pm$ SEM from Baseline to 12-month timepoint; [†]The Primary Efficacy Set consists of randomized participants who provided a total score of ≥ 3 on the NSAD at baseline. CK=creatine kinase; SEM=standard error of the mean; U/L=units/liter; ULN=upper limit of normal.

Results translated to clinical endpoints with a difference of 0.27 m/s in 100MTT at 12 months and separation from placebo as early as 3 months

LS Mean Change from Baseline in 100MTT ($\pm 95\%$ CI)



Timepoint	3 months		12 months
Cohort	Ambulatory Analysis Set*		Ambulatory Analysis Set*
BBP-418	0.09 (n=61)		0.14 (n=40)
Placebo	0.01 (n=32)		-0.12 (n=20)

*This analysis is based on the secondary efficacy set which consisted of randomized participants with nonzero scores at screening (or if a score was not available at screening, then it was nonzero at baseline) for all of the following: (1) 10MWT (or if 10MWT was not available, then NSAD Item #29a [velocity]), (2) PUL 2.0 (3) FVC (% predicted, sitting) (or if FVC was not available, then the investigator reported that spirometry was performed). LS mean change from baseline at 3- and 12-month timepoints; comparison of BBP-418 change from baseline to Placebo change from baseline is based on a linear mixed model for repeated measures. n = number of participants with observed values. 100MTT=100-meter timed test; CI=confidence interval; FVC=forced vital capacity; LS=least-squares; m/s=meter/second; NSAD=North Star Assessment for Limb Girdle-type Dystrophies; PUL 2.0=Performance of Upper Limb Scale 2.0

A post hoc analysis of time to complete 100MTT was significantly different between the treatment and placebo arms, favoring BBP-418 by 31 seconds

Endpoint	LS mean time to complete 100MTT at 12 months (s)	LS mean (99% CI) change from baseline to 12 months (s)
BBP-418	85.1	-12.4 (-26.9, 2.0)
Placebo	116.1	+18.6 (-1.1, 38.2)
Treatment difference		-31.0 (-55.4, -6.6; p=0.0011)*



A time to complete the 100MTT of <83 seconds (>1.2 m/s) reflects the ability to ambulate in the community, while completion in >125 seconds (<0.8 m/s) reflects ambulation largely limited to household settings^{1,2}

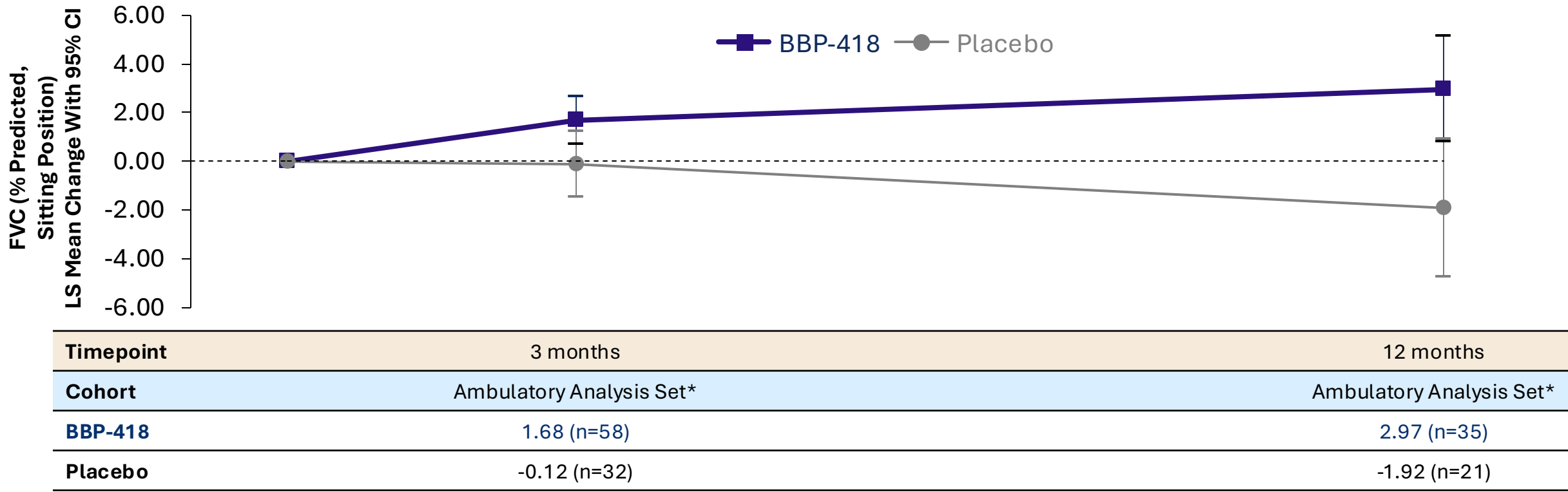


BBP-418-treated patients completed ~31 seconds faster than placebo-treated

*Nominally statistically significant based on exploratory analysis; analysis not part of α -controlled hierarchy at interim analysis. Least-squares mean change from baseline at 12-month timepoint; Comparison of BBP-418 change from baseline to Placebo change from baseline is based on a linear mixed model for repeated measures. Test conducted in secondary efficacy set.
100MTT=100-meter timed test; CI=confidence interval; LS=least-squares; m/s=meter/second; s=second
1. Alfano LN, et al. 2025;12(7):1324-1333. doi: 10.1002/acn3.70064. 2. James MK, et al. *Neurol Genet*. 2025;11:e200323. doi:10.1212/NXG.0000000000200323.

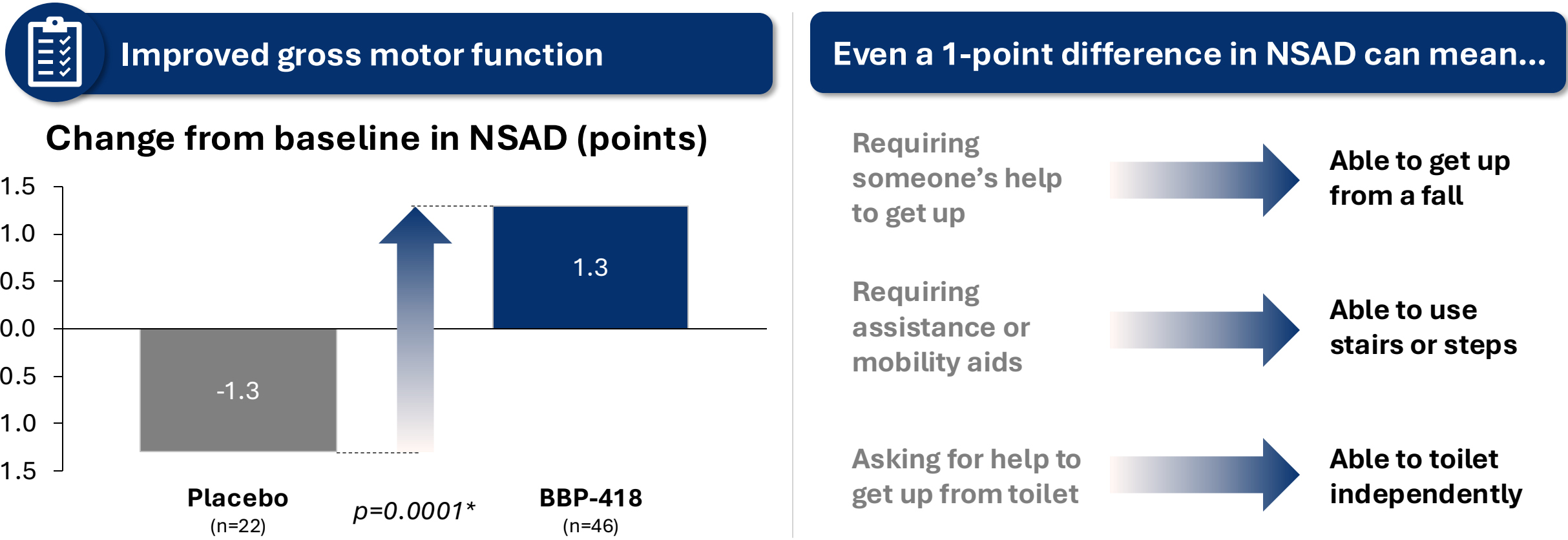
BBP-418 treated patients also registered ~3% increase in predicted volume from baseline FVC, resulting in a difference of ~5% predicted volume versus placebo

LS Mean Change from Baseline in FVC (% predicted) (±95% CI)



*This analysis is based on the secondary efficacy set which consisted of randomized participants with nonzero scores at screening (or if a score was not available at screening, then it was nonzero at baseline) for all of the following: (1) 10MWT (or if 10MWT was not available, then NSAD Item #29a [velocity]), (2) PUL 2.0 (3) FVC (% predicted, sitting) (or if FVC was not available, then the investigator reported that spirometry was performed). LS mean change from baseline at 3- and 12-month timepoints; comparison of BBP-418 change from baseline to Placebo change from baseline is based on a linear mixed model for repeated measures.
n = number of participants with observed values.
10MWT=10-meter walk test; CI=confidence interval; FVC=forced vital capacity; LS=least-squares; NSAD=North Star Assessment for Limb Girdle-type Dystrophies; PUL 2.0=Performance of Upper Limb Scale 2.0

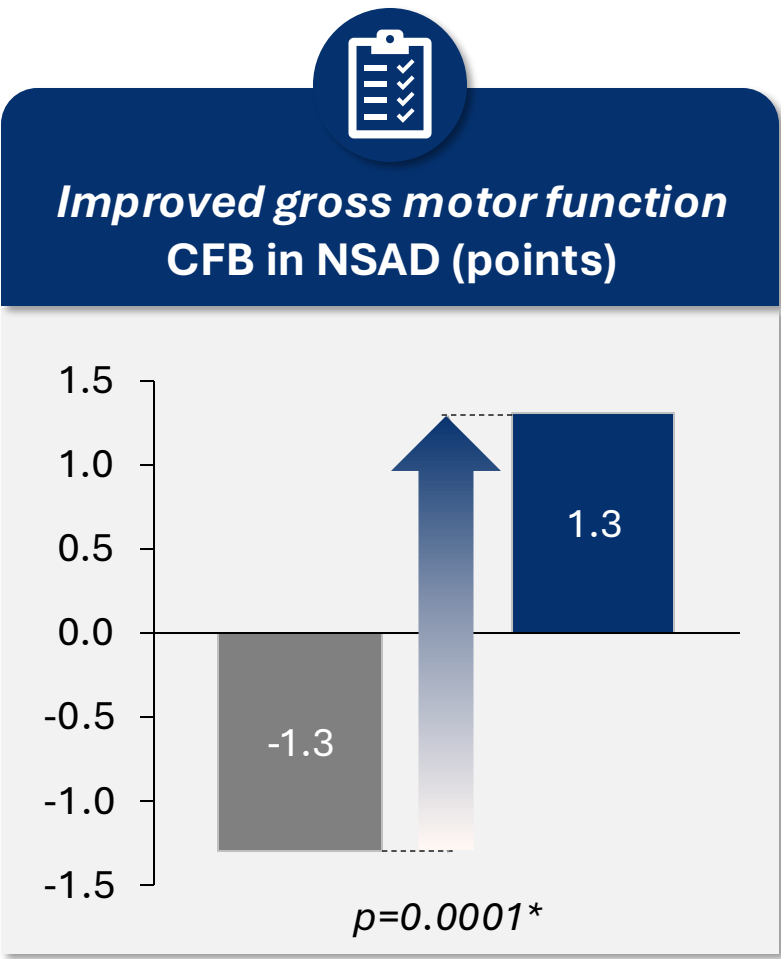
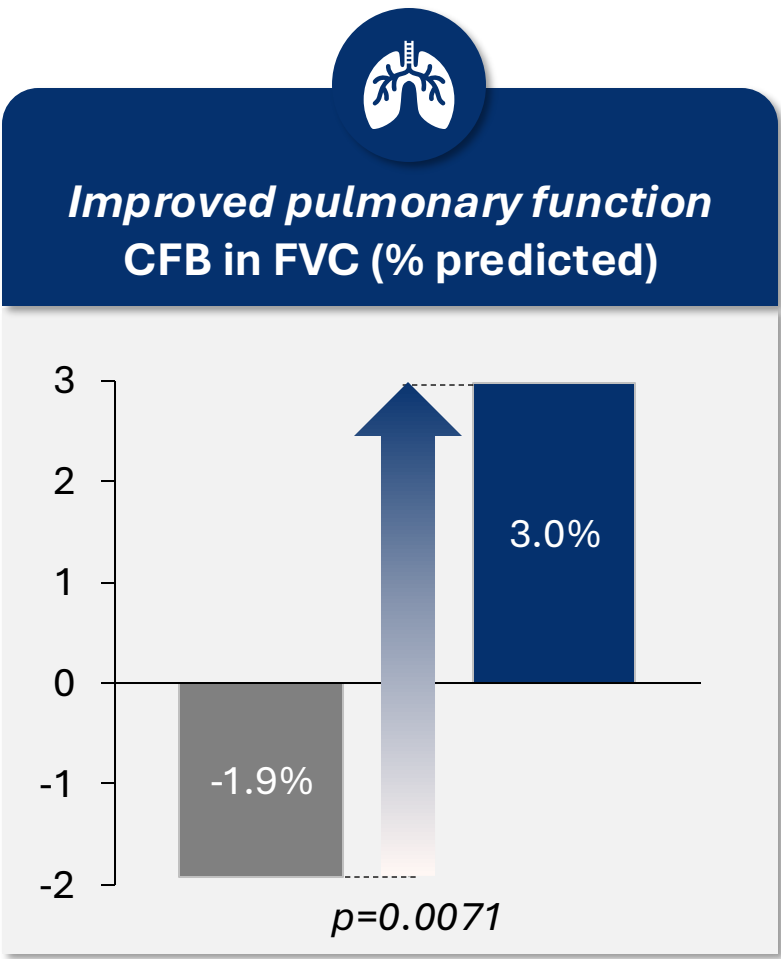
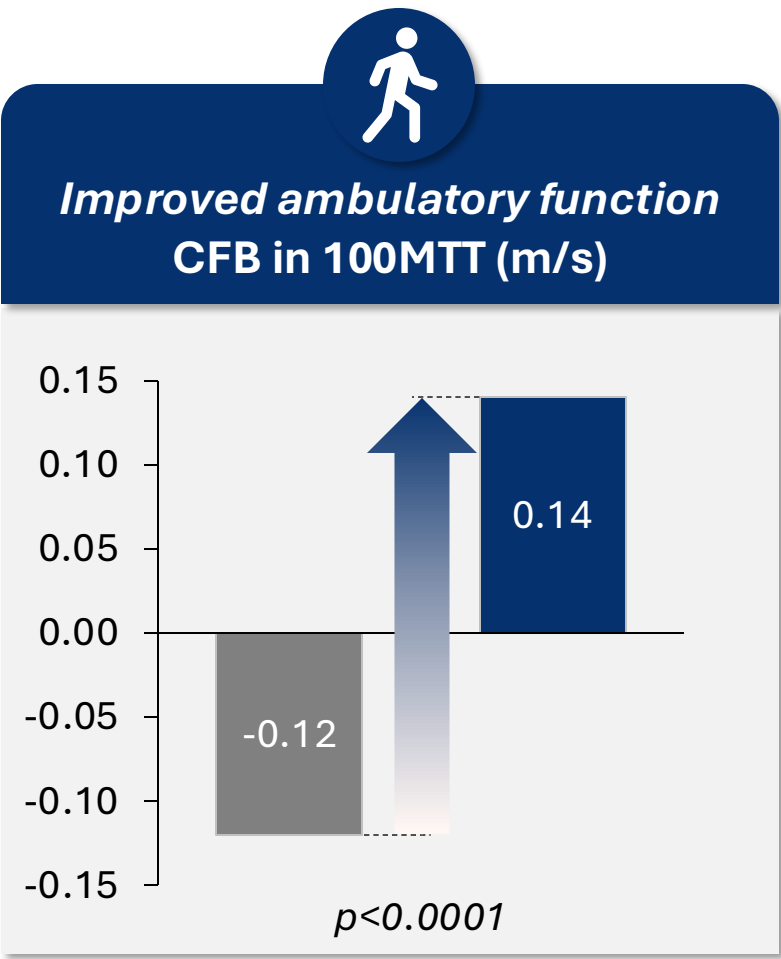
In an exploratory analysis, BBP-418 treated patients experienced highly clinically meaningful 2.6 point benefit on NSAD relative to placebo even at early 12-month timepoint



NSAD (primary endpoint at 36 months) benefit is highly clinically meaningful even at 12 months

** Nominally statistically significant based on exploratory analysis; analysis not part of alpha-controlled hierarchy at interim analysis*

More than stabilization: BBP-418 treated individuals experienced meaningful improvements across clinical endpoints at 12 months

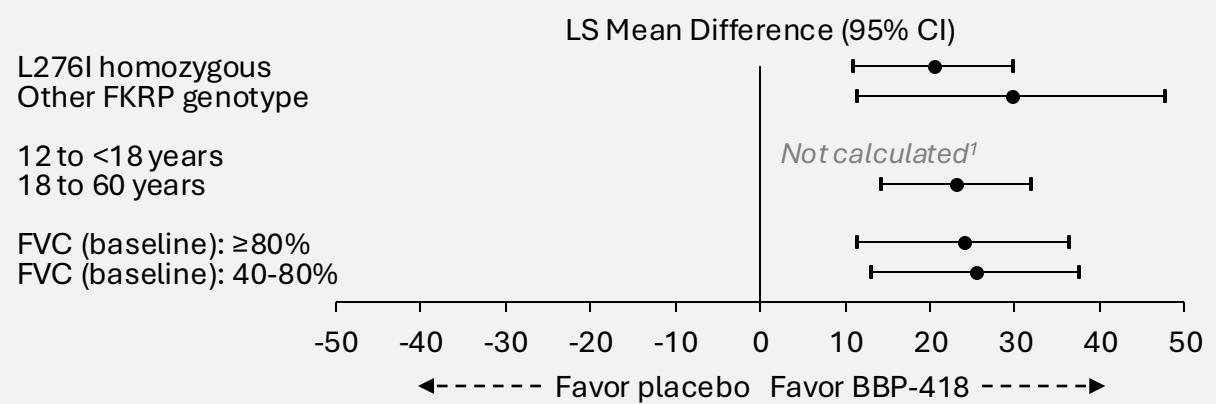


Key: Placebo BBP-418

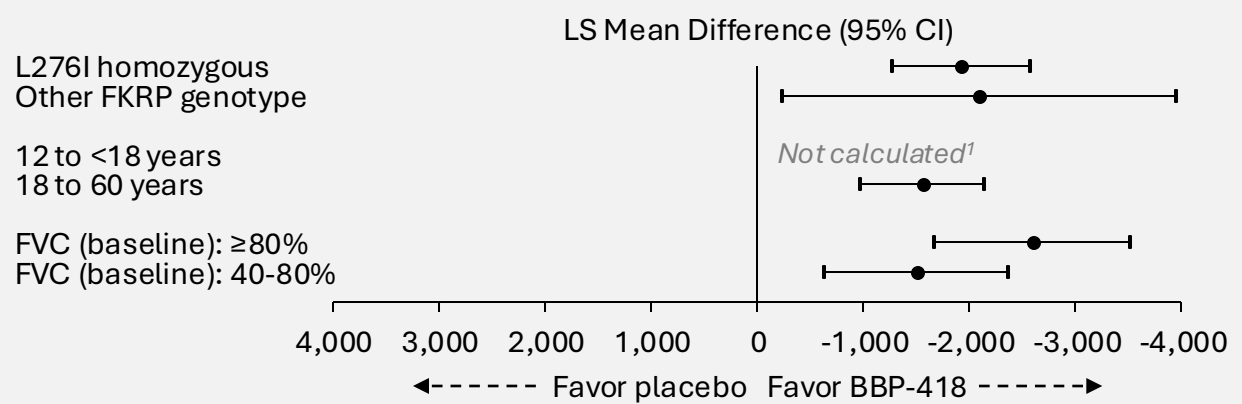
Planned subgroup analyses show consistent benefit of BBP-418 vs. placebo in all subgroups across α-controlled endpoints at 12 months

Biomarker

Glycosylated αDG (% of control)

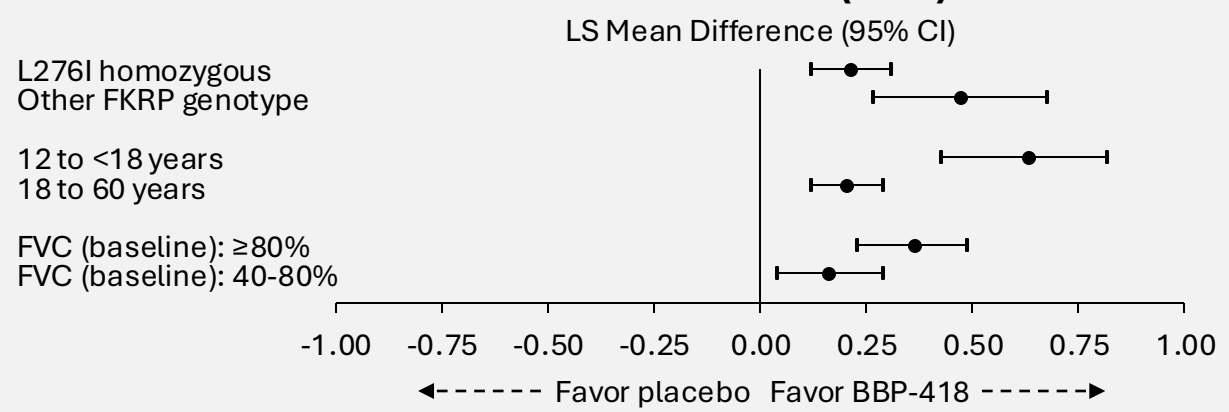


Serum creatine kinase (U/L)

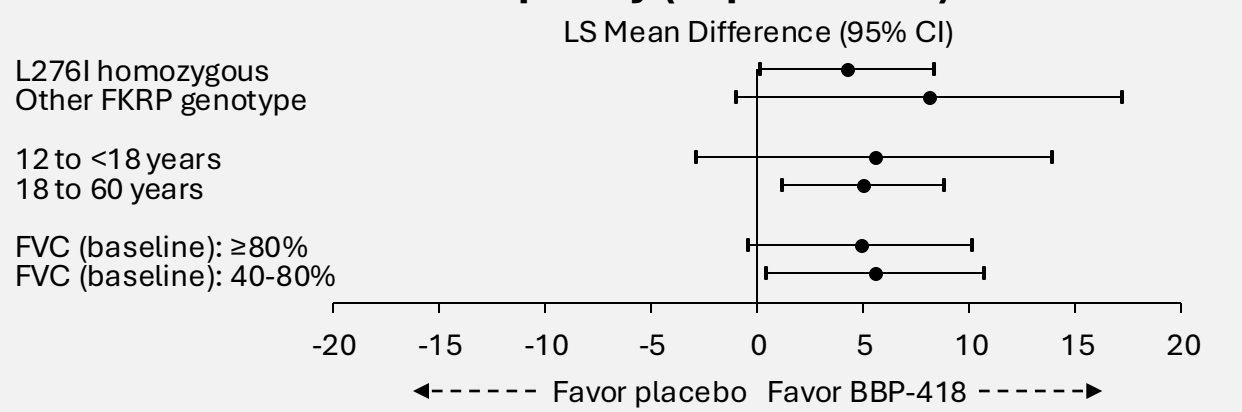


Functional

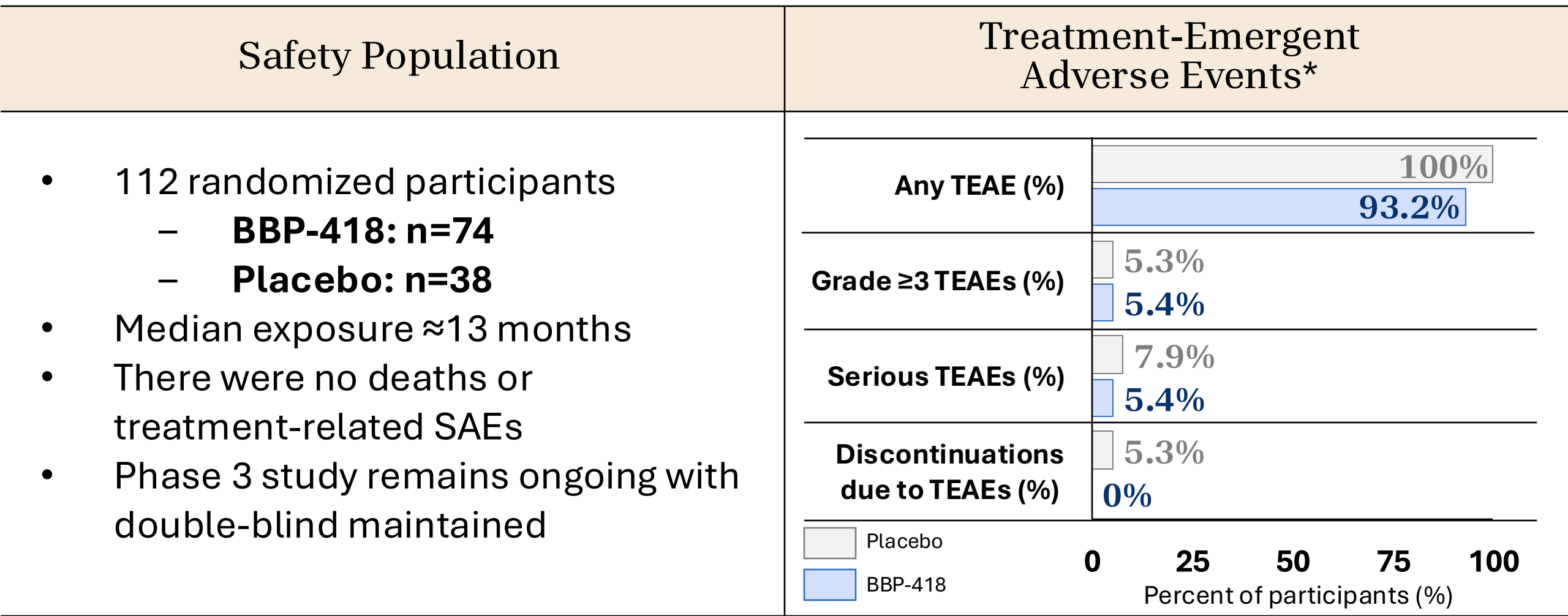
100-meter timed test (m/s)



Forced vital capacity (% predicted)

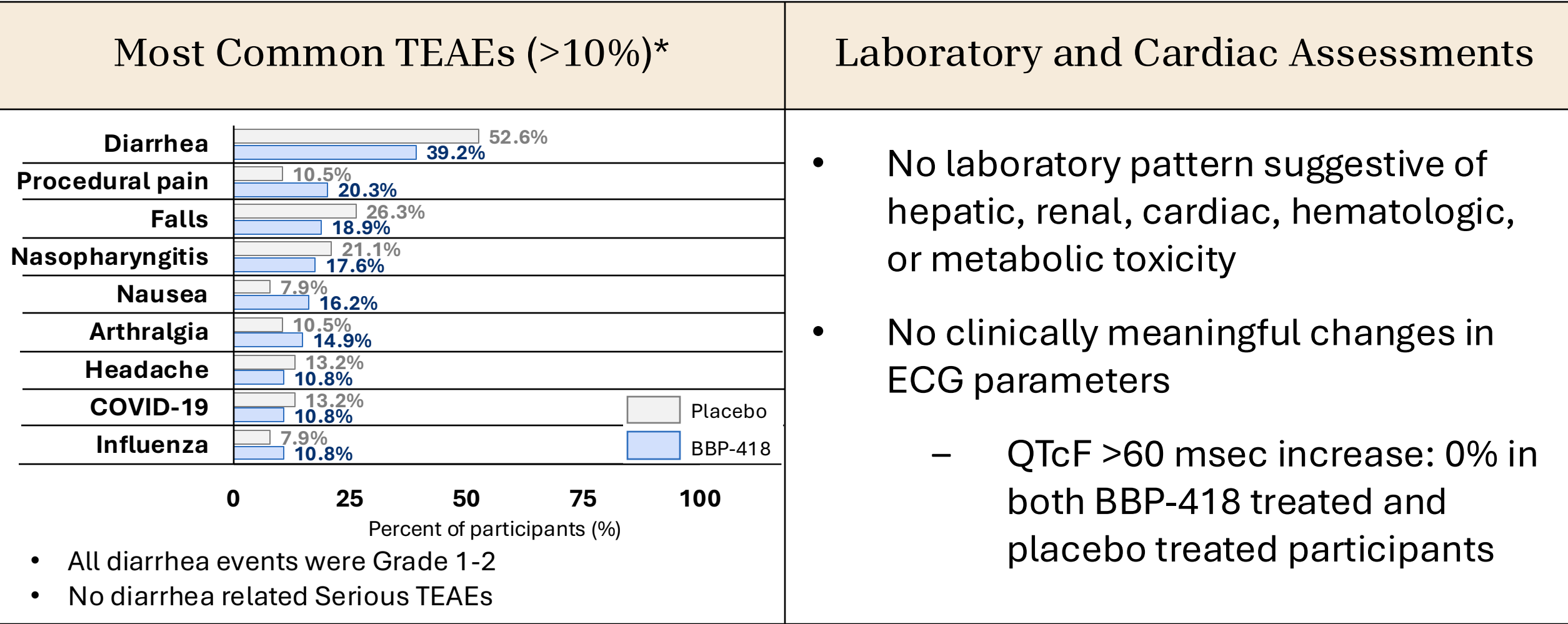


Safety data support a tolerable and well-characterized safety profile for BBP-418 in LGMD2I/R9, reinforcing favorable risk-benefit profile



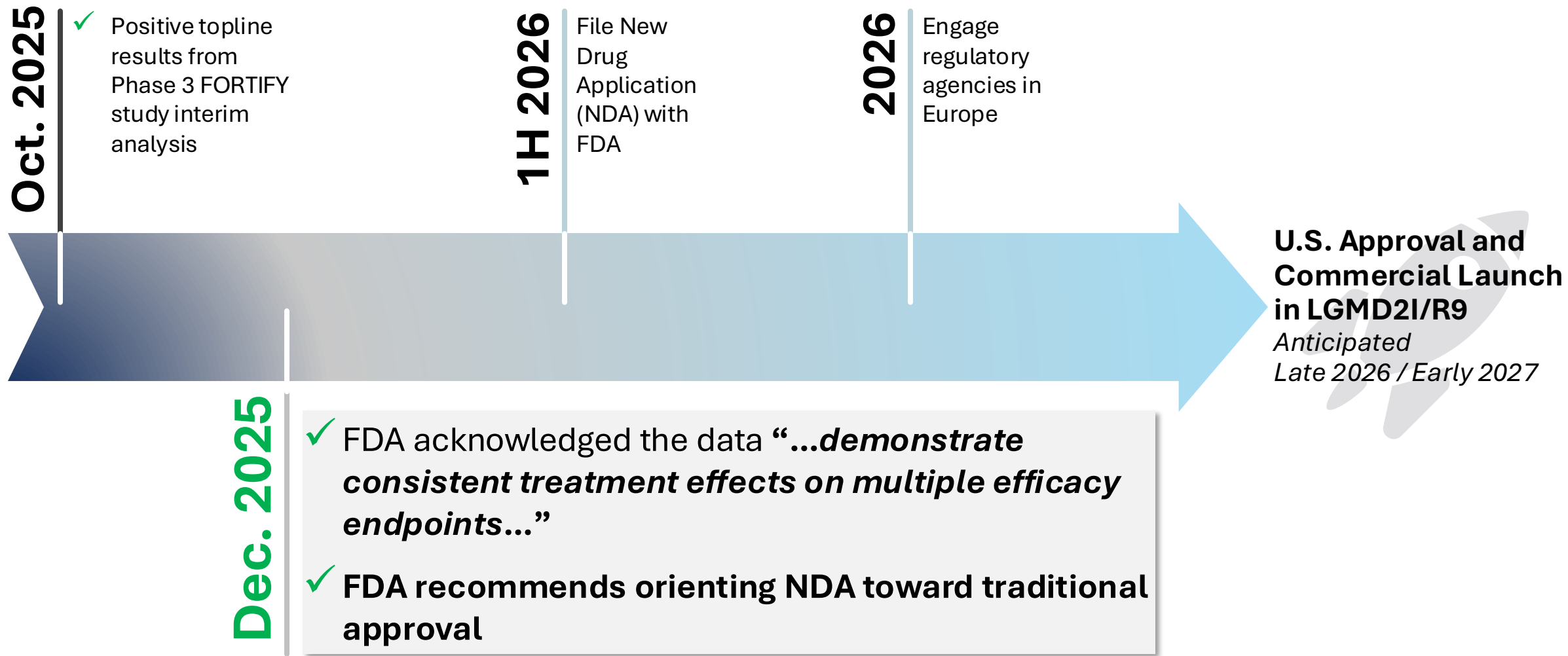
*Percentages reflect participant-level incidence percentages.
LGMD2I/R9=limb-girdle muscular dystrophy type 2I/R9; SAE=serious adverse event; TEAE=treatment-emergent adverse event.

Most common TEAE associated with BBP-418 was diarrhea; no new or unexpected safety findings were observed



*Percentages reflect participant-level incidence percentages
ECG=electrocardiogram; msec=millisecond; QTcF=QT interval corrected for heart rate using Fridericia's formula; TEAE=treatment-emergent adverse event

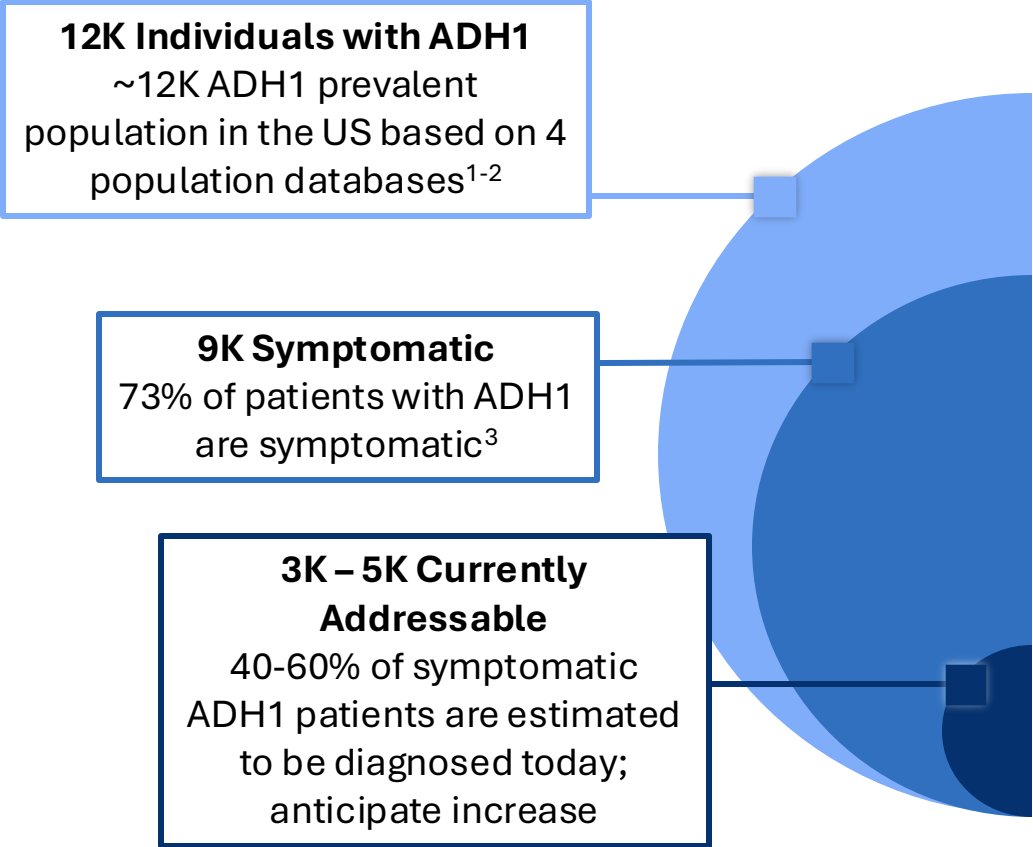
We completed a successful meeting with FDA post interim analysis, and they recommend orienting our NDA toward traditional approval



Encalaret



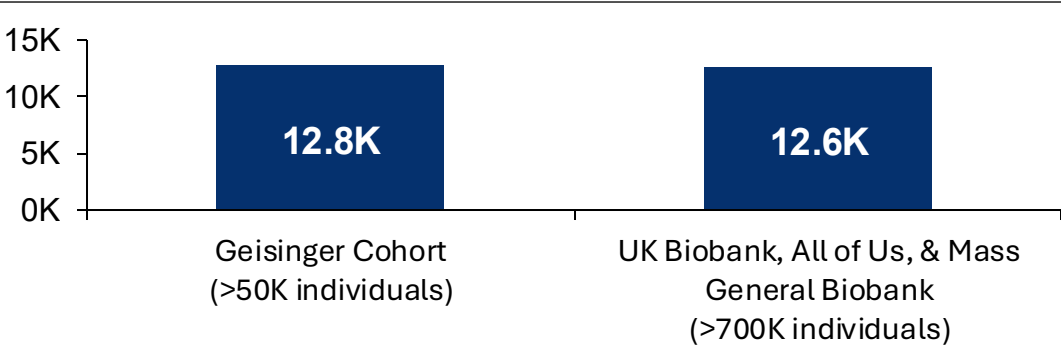
There are no therapies currently indicated to treat ADH1, a serious and rare genetic condition



An analogous ADH1 market is XLH

	XLH	ADH1
Prevalence (US)	12K ⁴	12K
Disease burden	Hypophosphatemia	Acute - hypocalcemia Chronic - hypercalciuria
Standard of care	Vitamin D, daily phosphate	Vitamin D, daily calcium
Registrational endpoint	Serum phosphate	Serum and urine calcium
Projected peak year sales	\$2B+ ⁵	\$1B+

ADH1 variant frequency estimates in literature¹⁻²



Encaleret is an investigational, potential first-in-class therapy that targets the underlying disease mechanism of ADH1

Design Principles

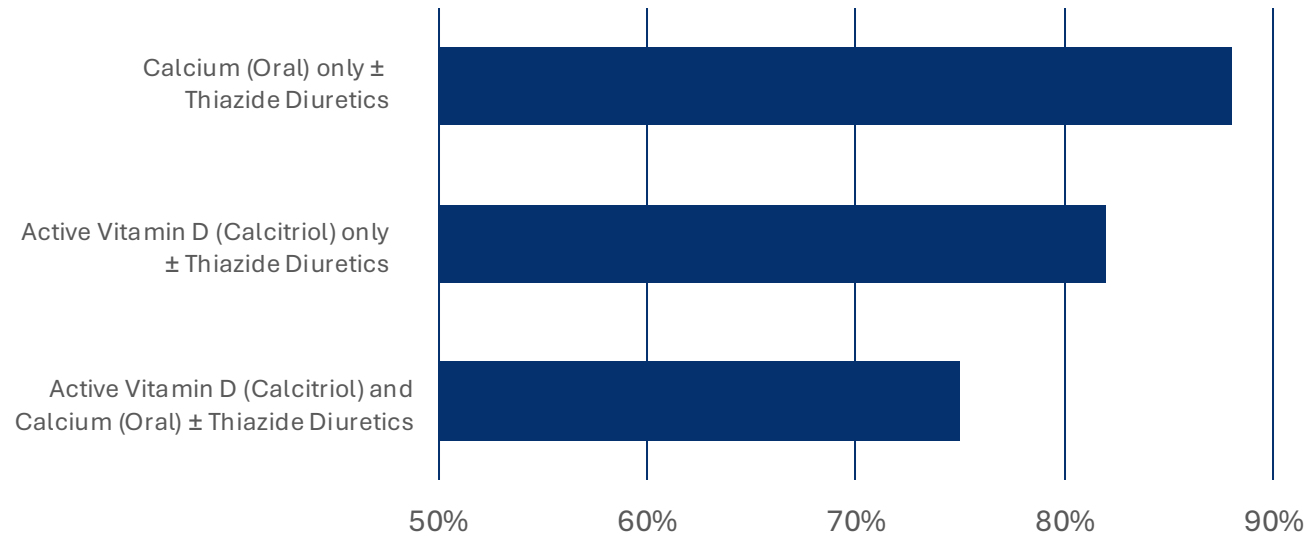
-  **Only investigational treatment directly targeting ADH1 at its source**
Potential to restore physiologic mineral homeostasis that is disrupted by CaSR oversensitivity
-  **Address common symptomatology**
Designed to normalize PTH, serum Ca, and urine Ca levels, potentially correcting the root cause of neuromuscular and renal consequences
-  **Convenient oral dosing**
First potential targeted treatment for ADH1 in a convenient form for patients and providers

Endocrinologists & nephrologists report low satisfaction with conventional therapy, leading to strong interest in novel investigational agents like encaleret

HCPs are not satisfied with conventional therapy

Dissatisfaction with Currently Available Treatment Options

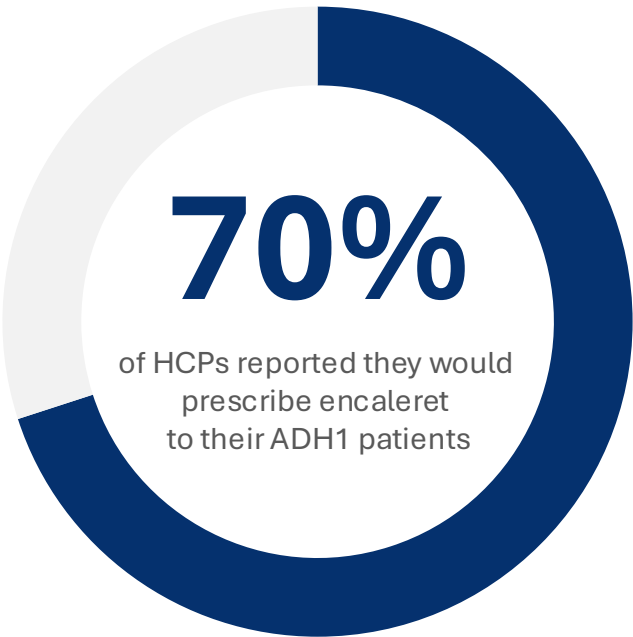
(N=44, % of HCPs indicating not highly satisfied with treatment option)



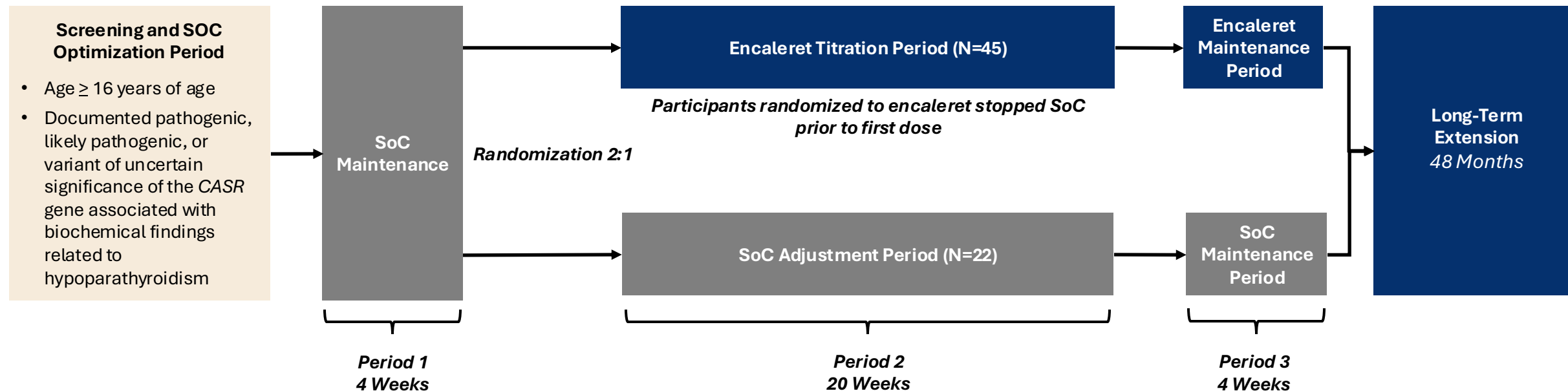
Survey of N=44 HCPs managing nonsurgical hypoparathyroidism and ADH1 patients

80% of HCPs indicated a product's ability to normalize urine calcium levels is the top attribute influencing treatment decisions for ADH1 patients

High likelihood HCPs would prescribe encaleret



Encaleret phase 3 registrational study design



Primary Composite Endpoint:

Proportion of participants achieving:

- Corrected Ca¹ within the target range of 8.3-10.7 mg/dL

AND

- 24-hour urine Ca within the reference range (<300 mg/day for men & <250 mg/day for women)

Key Secondary Endpoint:

Proportion of participants achieving iPTH above the lower limit of the reference range

Select Secondary Endpoints:

- 1,25-(OH)₂ Vitamin D, magnesium, and phosphate
- Bone turnover markers
- Renal ultrasound and renal function

>90% of CALIBRATE participants administered encaleret demonstrated a pharmacologic response

Primary Analysis – Within Group	Week 4 SoC (N=45)	Week 24 Encaleret (N=45)	p-value ³
Number of Participants Meeting The Primary Endpoint (Responder status) ^{1,2}	2	34	
Proportion, %	4%	76%	
Difference in Proportion of Responders (95% CI)	71% (58%, 84%)		<0.0001

Key Secondary Analysis – Within Group	Week 4 SoC (N=45)	Week 24 Encaleret (N=45)	p-value ³
Number of Participants With <u>iPTH</u> ≥ LL Reference Range	3	41	
Proportion, %	7%	91%	
Difference in Proportion of Responders (95% CI)	84% (74%, 95%)		<0.0001

¹The primary endpoint assessed responder status of participants who achieved both corrected serum calcium and 24-hour urine calcium in the target range at the completion of the maintenance periods.
²Participants randomized to receive encaleret who required doses of elemental calcium >600 mg/day for >7 days during Period 3 were evaluated as non-responders. ³Analyzed by McNemar’s test. CI = Confidence Interval; iPTH = Intact Parathyroid Hormone; LL = Lower Limit

Encaleret was well-tolerated with no TEAEs resulting in encaleret or study discontinuation

	Period 1	Periods 2 and 3	
	SoC N=67	SoC N=22	Encaleret N=45
Participants experiencing any Serious TEAE	2 (3%)	3 (14%)	4 (9%)
Serious Related TEAE	1 (2%)	0 (0%)	1 (2%)
Participants experiencing any TEAE	30 (45%)	14 (64%)	40 (89%)
Mild	23 (34%)	6 (27%)	21 (47%)
Moderate	4 (6%)	6 (27%)	16 (36%)
Severe	3 (5%)	2 (9%)	3 (7%)
Related TEAE	4 (6%)	0 (0%)	16 (36%)
TEAE of Hypocalcemia	4 (6%)	3 (14%)	3 (7%)
TEAE of Hypercalcemia	3 (5%)	0 (0%)	10 (22%)
TEAE Leading to Study Discontinuation	0 (0%)	0 (0%)	0 (0%)

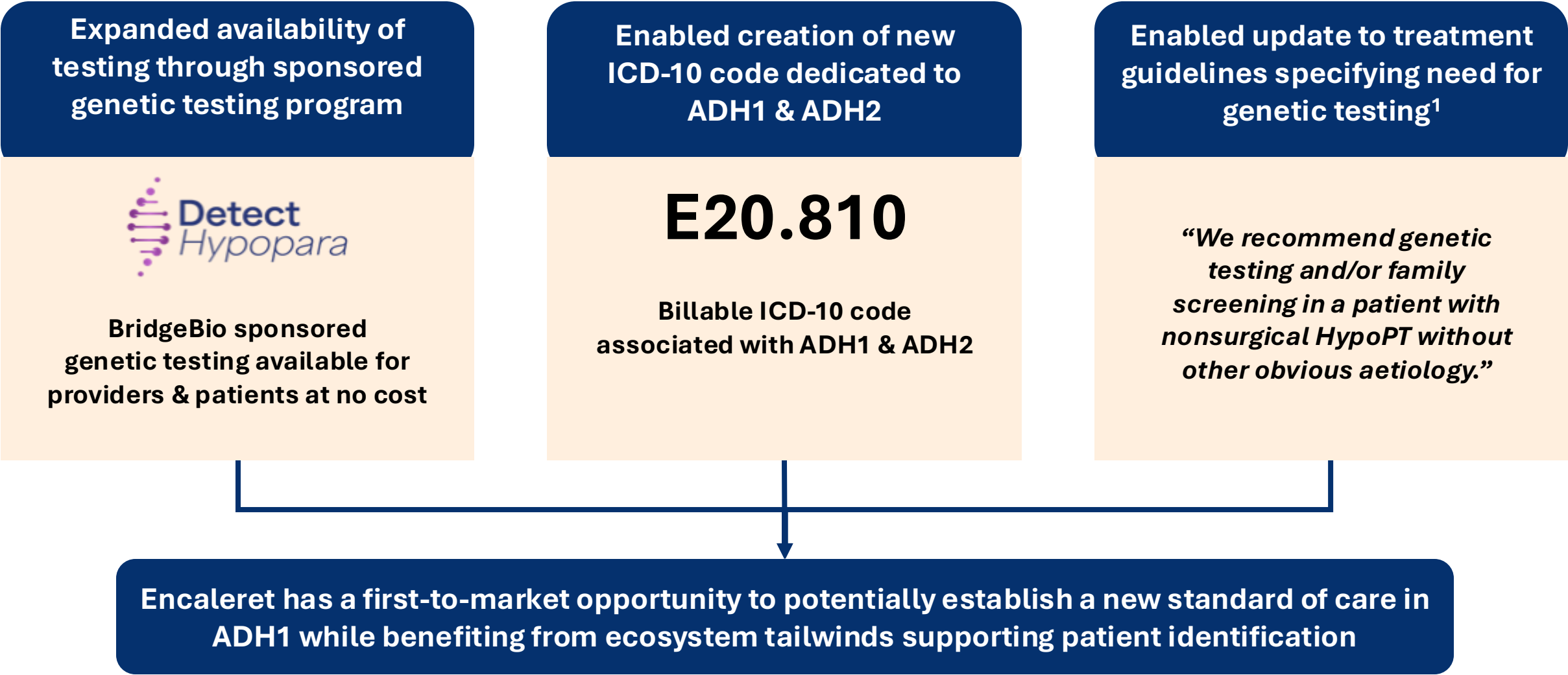
Encaleret was found to restore physiologic mineral homeostasis through its action on the CaSR

- 76% of participants randomized to encaleret achieved serum and urine calcium in the target range compared to the same individuals on conventional therapy (difference 71%, $p < 0.0001$)^{1,2}
 - Among encaleret responders at Week 24, none required conventional therapy during Period 3³
- Clinically meaningful restoration of intact PTH in participants administered encaleret compared to the same individuals on conventional therapy (difference 84%, $p < 0.0001$)¹
- Clinically meaningful increase in corrected serum calcium ($p < 0.0001$) and decrease in 24-hour urine calcium excretion ($p < 0.0001$) at Week 24
- Similar changes for the above parameters were also demonstrated between treatment arms
- Encaleret was well tolerated, with no discontinuations related to study drug

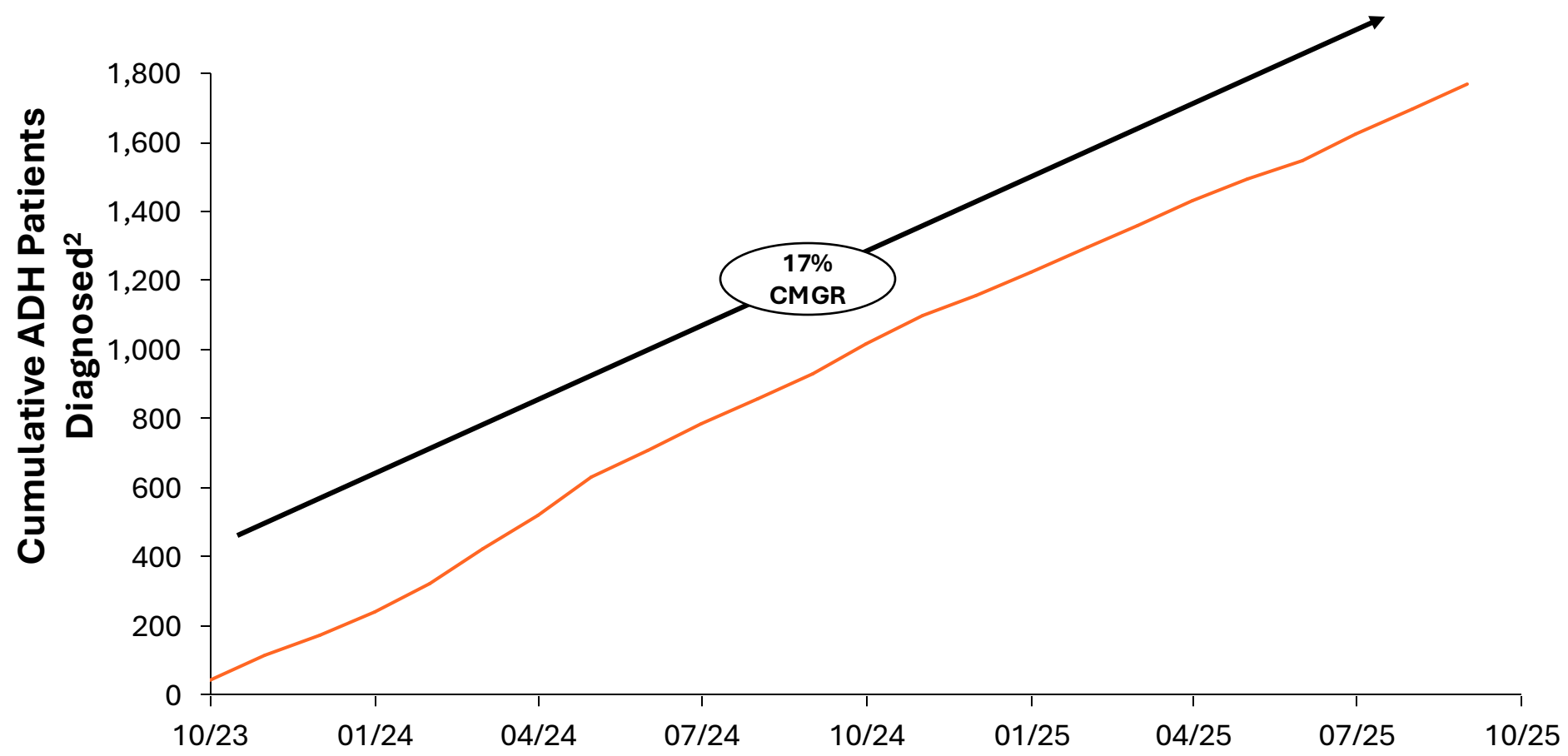
CALIBRATE achieved & exceeded all criteria set forth as an upside target, with a 76% responder rate following 24 weeks of encaleret treatment

Upside Target Clinical Profile		Outcome Observed	
	Statistically significant primary analysis result compared to conventional therapy	Primary endpoint met (p <0.0001) demonstrating superiority to conventional therapy	
	At Week 24, ≥50% of study participants achieve target serum and urine Ca on encaleret	76% (34 out of 45) achieved target serum and urine Ca on encaleret vs. 4% on conventional therapy	
	Majority of participants randomized to encaleret able to remain independent from conventional therapy ¹	Among encaleret responders at Week 24, none required conventional therapy during Period 3¹	
	At Week 24, mean iPTH within normal range on encaleret	>90% of participants administered encaleret achieved iPTH above the lower limit of the reference range	
	Comparable safety and tolerability profile to conventional therapy	Encaleret was well-tolerated; no discontinuations related to study drug	

BridgeBio is pioneering efforts to enable the successful launch of encaleret, potentially the first calcilytic molecule to be approved for any condition

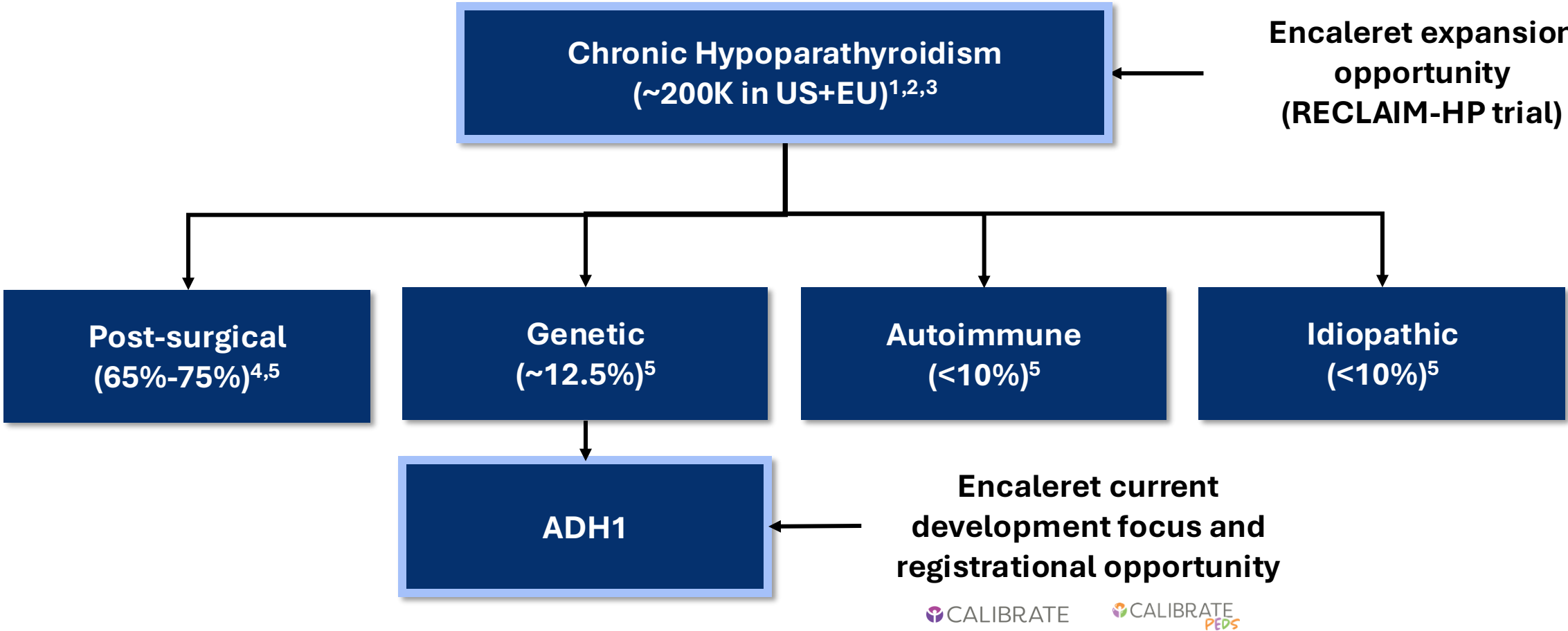


Over a 24-month period, >1,700 unique patients were diagnosed with ADH in the US



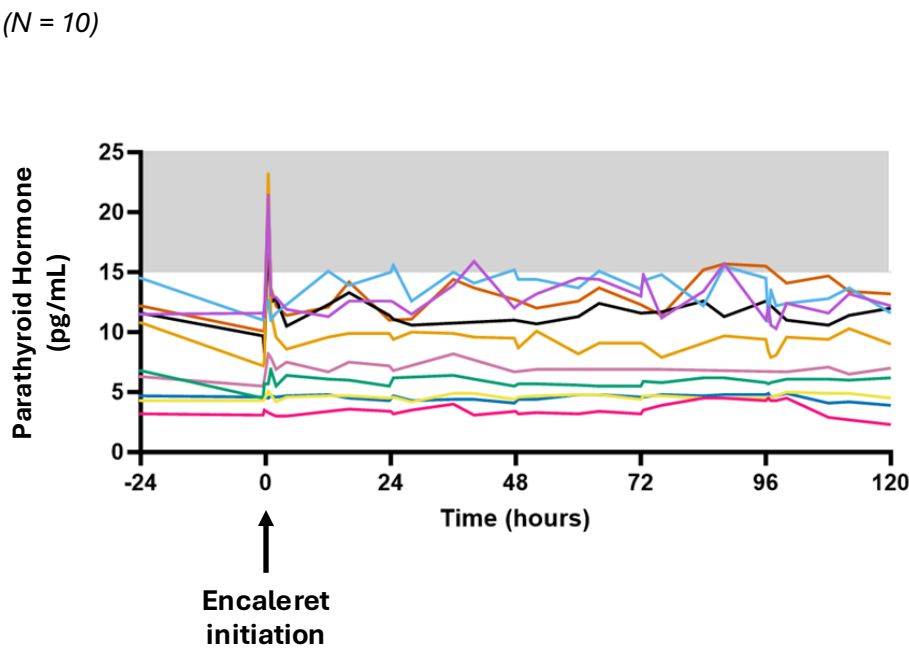
~50% of HCPs diagnosing ADH patients manage ≥5 non-surgical hypoparathyroidism patients³

Encaleret also has potential as an oral medication for other etiologies of chronic hypoparathyroidism (HP)



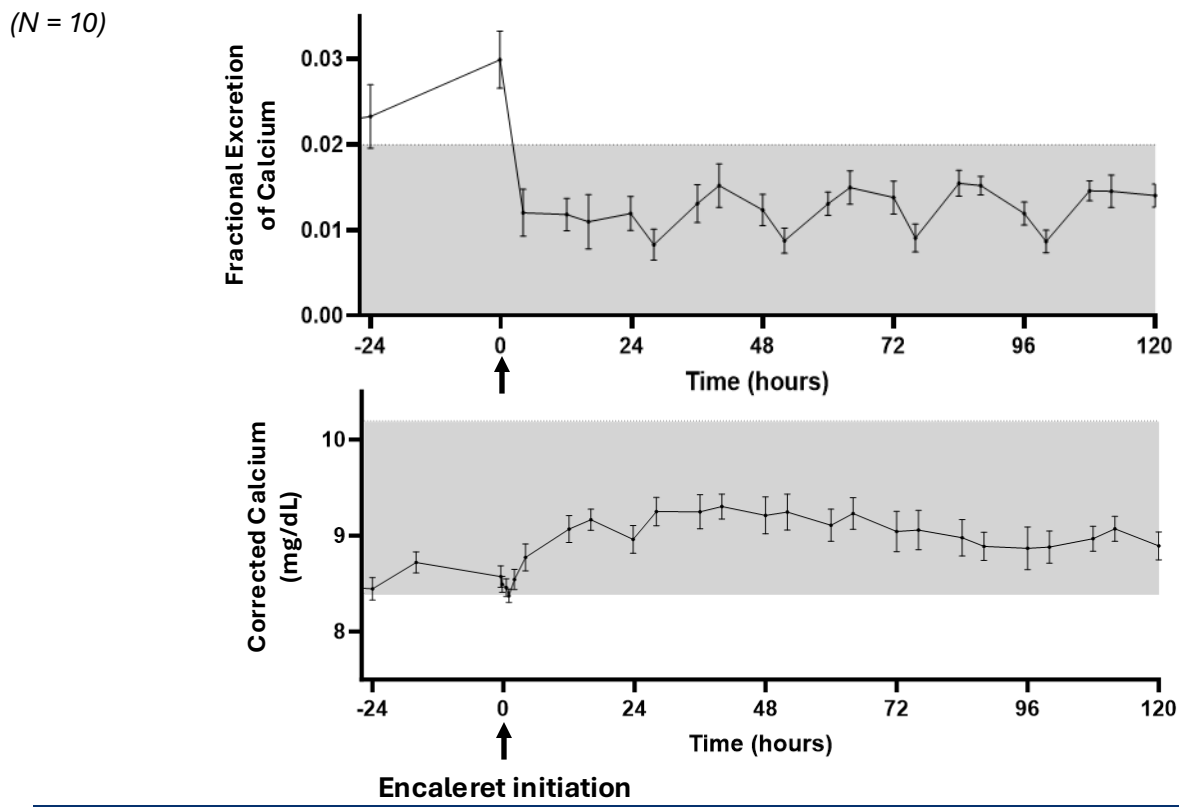
Normalization of calcium homeostasis in post-surgical hypoparathyroidism (PSH) patients is indicative of encaleret's broader potential

Effect on PTH in PSH patients^{1,2}



Encaleret did not restore PTH levels in PSH patients

Effect on calcium homeostasis in PSH patients^{1,2}



Encaleret normalized blood and urine calcium through PTH independent pathways, demonstrating its potential to modulate urine and serum calcium levels through renal CaSRs for a broader set of chronic HPT patients

Encaleret has the potential to be an orally administered option for patients with chronic hypoparathyroidism (CHP)

Encaleret has the potential to normalize blood and urine calcium in CHP patients

- CHP patients present similarly as ADH1 patients (i.e., hypocalcemia and hypercalciuria)
- Current guidelines specify normalization of blood and urine calcium as therapeutic goals^{1,2}
- In a Phase 2 study (N=10) presented at the ASBMR 2025 meeting, encaleret demonstrated a PTH-independent effect to normalize blood and 24-hour urine calcium in 80% of study participants within 5 days³

Announcing the RECLAIM-HP Phase 3 Study of Encaleret in CHP *Initiating in summer 2026*



Completed successful End of Phase 2 interaction with the FDA



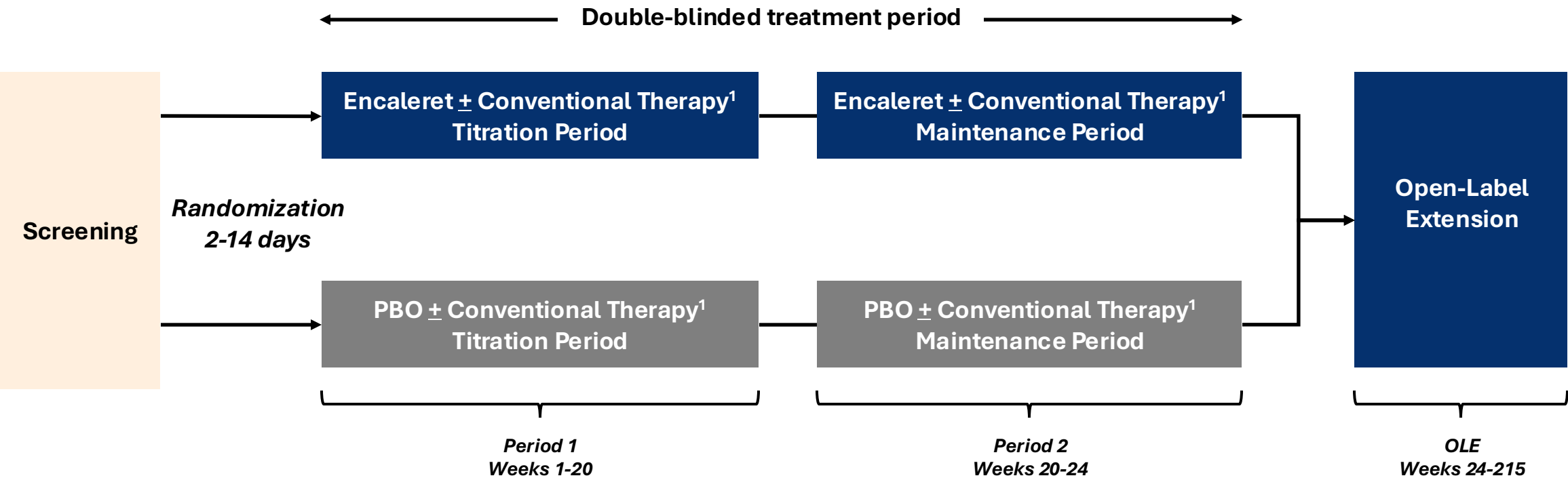
Phase 3 registrational trial to evaluate encaleret in CHP



Primary endpoint will assess achievement of target blood and urine calcium

RECLAIM-HP Phase 3 study of encaleret in chronic hypoparathyroidism to initiate in summer 2026

RECLAIM-HP: Global, multi-center, randomized, double-blind, placebo-controlled study

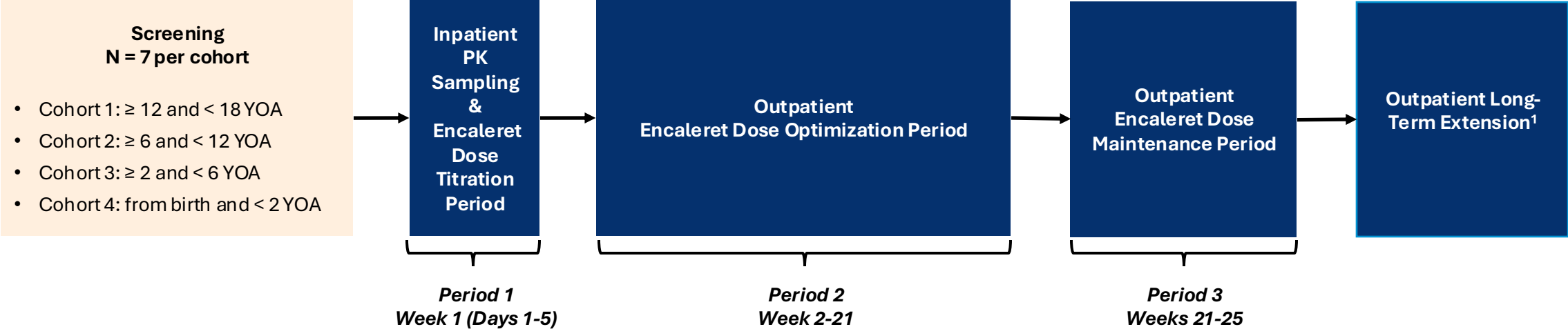


Primary Endpoint

Proportion of participants achieving albumin-corrected blood and urine calcium within target range

CALIBRATE-PEDs Phase 2/3 registrational trial has initiated to study encaleret in pediatric ADH1 patients

CALIBRATE-PEDS: Global, multi-center, open-label, single-arm study



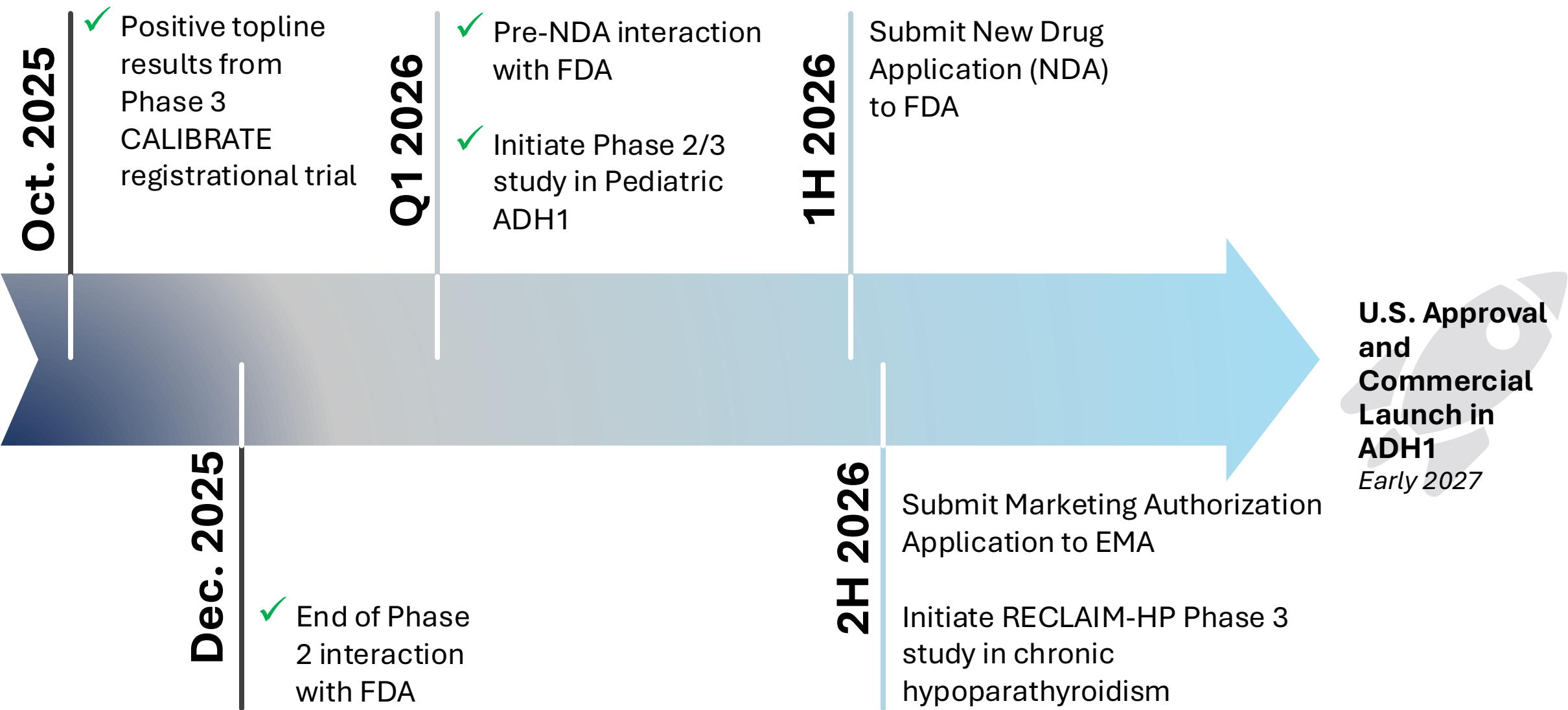
Primary Endpoint:

- PK parameters following encaleret administration in Period 1
- Proportion of participants achieving at Week 25:
 - Blood cCa within the target range **AND**
 - 24-hour urine Ca/Spot urine Ca/Cr within the reference range

Select Secondary and Exploratory Endpoints:

- Blood iPTH, 1,25-(OH)₂ Vitamin D, magnesium, and phosphate
- Safety assessment
- Bone turnover markers and bone mineral density
- Renal ultrasound and renal function
- Quality of life (SF-10) in children ≥ 6 YOA

NDA submission for ADH1 planned in the first half of 2026 with two additional registrational studies to initiate this year



Infigratinib





Infigratinib for Achondroplasia

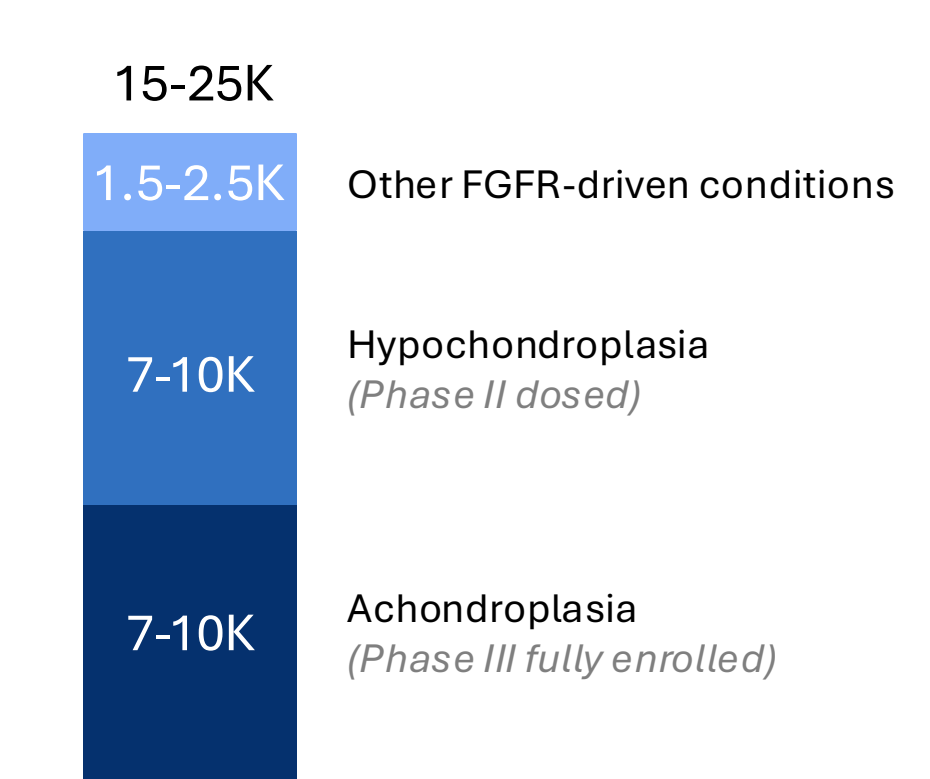
**THANK YOU to the study
participants, their families,
investigators, clinical research
staff, advocates and collaborating
research partners**

“Taken together, these best-in-class results highlight the transformative potential for infigratinib to address aspects of achondroplasia beyond linear height, and with a product administered orally.”

Ravi Savarirayan, M.D., Ph.D. of Murdoch
Children’s Research Institute in
Melbourne, Australia

There remains a significant unmet need for many children with skeletal dysplasias; this represents a large and rapidly growing market

Addressable people by indication in US/EU¹
(current population eligible for treatment)



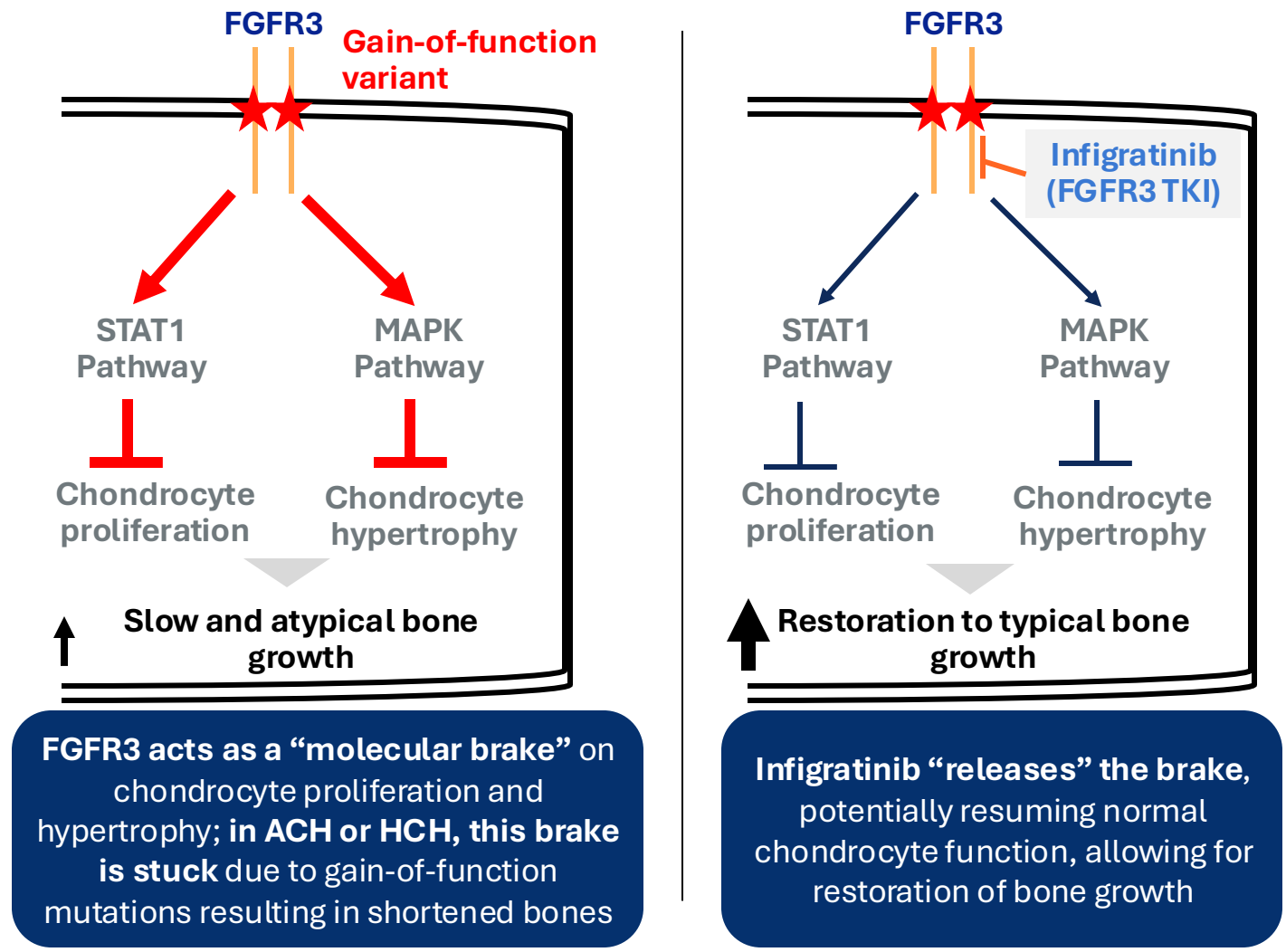
55,000
individuals with
achondroplasia
globally

*Represents
diagnosed and
addressable ACH
population with
open growth plates*

\$5B+ potential
global market

Infigratinib is a potentially best-in-class FGFR3 inhibitor that targets achondroplasia and hypochondroplasia at their source

Mechanism



Design Principles

- **Maximize efficacy by targeting condition at the source**
For all the manifestations of ACH and HCH, not just height, which matter for families and physicians
- **Demonstrate safety with low dosing**
Avoiding hypotension & injection site reactions with no hyperphosphatemia, ocular effects or VEGFR3 off-target effects
- **Avoid injections and provide an oral option**
For children and families, to reduce burden and pain of treatment

Infigratinib is a sprinkle capsule that can be taken intact whole, or sprinkled

Capsules
(17 mm long*)



Granules
(2 mm long)



- Infigratinib is being studied in children over 3 years of age with achondroplasia (0.25 kg/mg/day) as a sprinkle capsule
- Capsules can be swallowed whole or content (granules) sprinkled on soft food
- The dosage strength of each capsule depends on how many granules are inside
- Each child's dose is based on their weight

Infigratinib exceeded the bar for a potential best-in-class therapeutic option, in the broadest age range studied across any achondroplasia trial

Target Clinical Profile for Commercial Success

CFBL in AHV: More than +1.5 cm/yr against placebo at Week 52

CFBL in height Z-score (ACH charts): More than +0.3 SD on treatment arm at Week 52

Proportionality: More than 0.05 decrease in upper to lower body ratio on treatment arm

Safety: No symptomatic hypotension. Less than 10% low-grade hyperphosphatemia rate.

Outcomes

Met the primary endpoint with mean difference against placebo of **+2.10 cm/yr** ($p<0.0001$), and a LS mean difference against placebo of **+1.74 cm/yr** ($p<0.0001$), the largest change observed in a randomized trial for ACH.

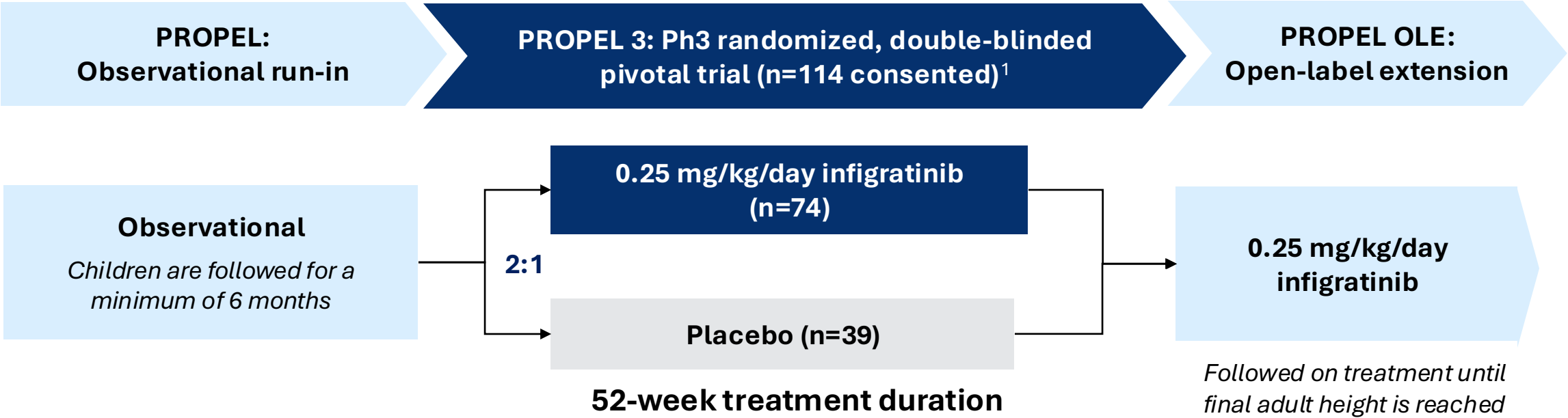
The LS mean improvement on the tx arm was +0.41 SD, the largest improvement observed in a RCT in ACH. LS mean difference against placebo was +0.32 SD ($p<0.0001$), the largest difference observed in a RCT in ACH.

In a pre-specified exploratory analysis of children <8yrs of age (>50% of trial), there was a statistically significant LS mean change from baseline against placebo of -0.05 ($p<0.05$).

In the overall population, infigratinib achieved a LS mean decrease of -0.05 on the tx arm, with a favorable LS treatment difference of -0.02 versus placebo at Week 52 ($p=0.1849$)

- Well-tolerated safety profile, **consistent with no inhibition of FGFR1 and FGFR2.**
- No discontinuations related to study drug
 - No serious adverse events related to study drug
 - 3 cases of hyperphosphatemia (4%), all mild, asymptomatic, transient and did not require dose reduction or discontinuation
 - No FGFR1 or FGFR2 associated AEs (E.g., retinal or corneal)
 - No AEs associated with CNP analogues: symptomatic hypotension, ISRs, or hypertrichosis

PROPEL 3 - Phase 3 registrational study design



Key inclusion criteria	Primary endpoint:	Key secondary endpoints:
Children 3 – <18 years old with open epiphyses	Change from baseline in annualized height velocity (AHV) at week 52 compared to placebo	- Change from baseline in height z-score (in relation to ACH tables) - Change from baseline in upper body : lower body segment ratio
		Other secondary endpoints: Change in physical functioning; HRQoL, cognitive function, participant and caregiver evaluation of treatment benefit (qualitative interview)

Demographic and baseline characteristics

Baseline demographics	Infigratinib (n=74)	Placebo (n=39)	All Participants (n=113)
Age (yrs), mean (SD) (min, max)	7.92 (2.7) (3.7 – 14.4)	7.74 (2.5) (3.9 – 14.9)	7.86 (2.7) (3.7 – 14.9)
Age group, n (%)			
3 – <5	10 (13.5)	4 (10.3)	14 (12.4)
5 – <11	50 (67.6)	31 (79.5)	81 (71.7)
11 – <18	14 (18.9)	4 (10.3)	18 (15.9)
Sex			
Male, n (%)	42 (56.8)	23 (59.0)	65 (57.5)
Female, n (%)	32 (43.2)	16 (41.0)	48 (42.5)
Baseline AHV (cm/year), mean (SD)	4.28 (1.39)	4.57 (1.45)	4.38 (1.41)
Height z-score (ACH reference), mean (SD)	0.09 (0.87)	-0.00 (1.00)	0.05 (0.91)

The study population was adequate to evaluate the study objectives,
with well balanced treatment arms

Primary endpoint: Change from baseline to Week 52 in AHV

	Infigratinib (n=74)	Placebo (n=39)
Baseline AHV (cm/yr), Mean (SE)	4.28 (0.16)	4.57 (0.23)
Week 52 AHV (cm/yr), Mean (SE)	5.75 (0.15)	3.95 (0.14)
CFBL AHV at Week 52, Mean (SE)	1.48 (0.22)	-0.62 (0.29)
Infigratinib vs. placebo difference, Mean (95% CI) P-value¹	2.10 (1.38, 2.81) p < 0.0001	
CFBL AHV at Week 52, LS Mean (SE)	1.58 (0.19)	-0.16 (0.23)
Primary Endpoint: Infigratinib vs. placebo difference, LS Mean (95% CI) P-value²	1.74 (1.31, 2.17) p < 0.0001	

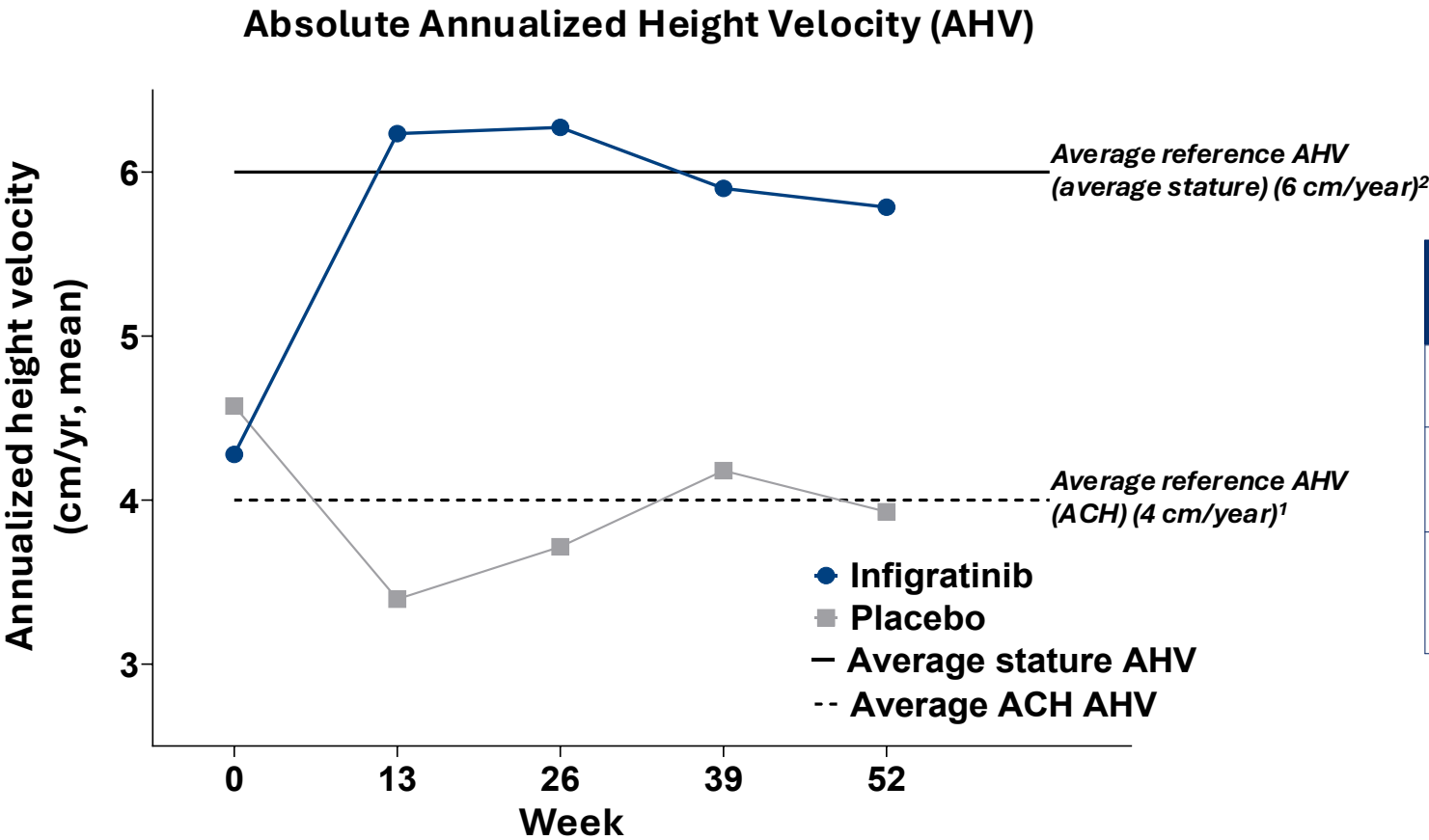
Infigratinib has demonstrated the largest CFBL in AHV at Week 52 in a randomized trial

Subgroup analyses: Change from baseline to Week 52 in AHV by age group

Age subgroups	3 to < 5 years		5 to < 11 years		11 to < 18 years	
	Infigratinib (n=10)	Placebo (n=4)	Infigratinib (n=50)	Placebo (n=31)	Infigratinib (n=14)	Placebo (n=4)
Infigratinib vs. placebo difference, Mean	2.30		1.93		2.78	
Infigratinib vs. placebo difference, LS Mean	1.47		1.84		1.40	

Across all of the above age subgroups, infigratinib demonstrated the largest CFBL in AHV at Week 52 in a randomized trial for ACH

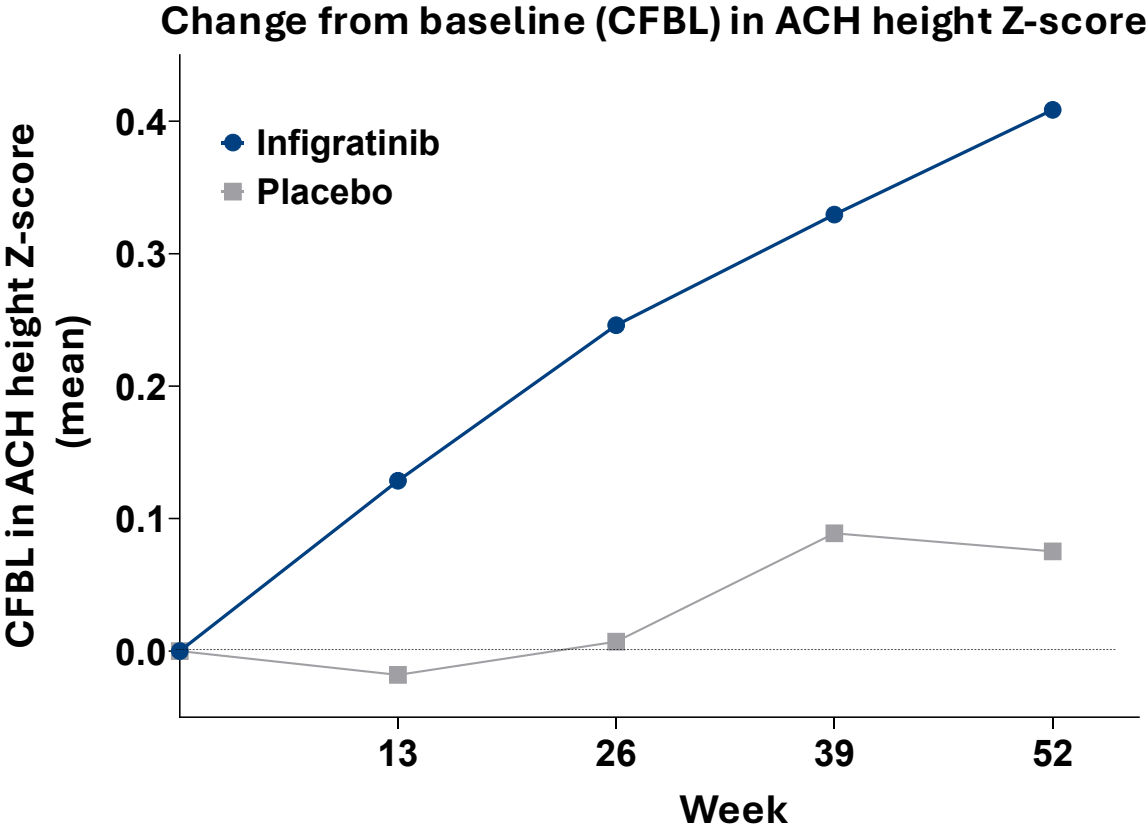
Secondary endpoint: Absolute AHV at Week 52



Absolute AHV at Week 52	Infigratinib (n=74)	Placebo (n=39)
LS Mean	5.96	4.22
Infigratinib vs. placebo difference, LS mean	1.74	
P-value	p<0.0001	

Infigratinib has demonstrated the largest absolute AHV (LS mean) at Week 52 in any randomized trial

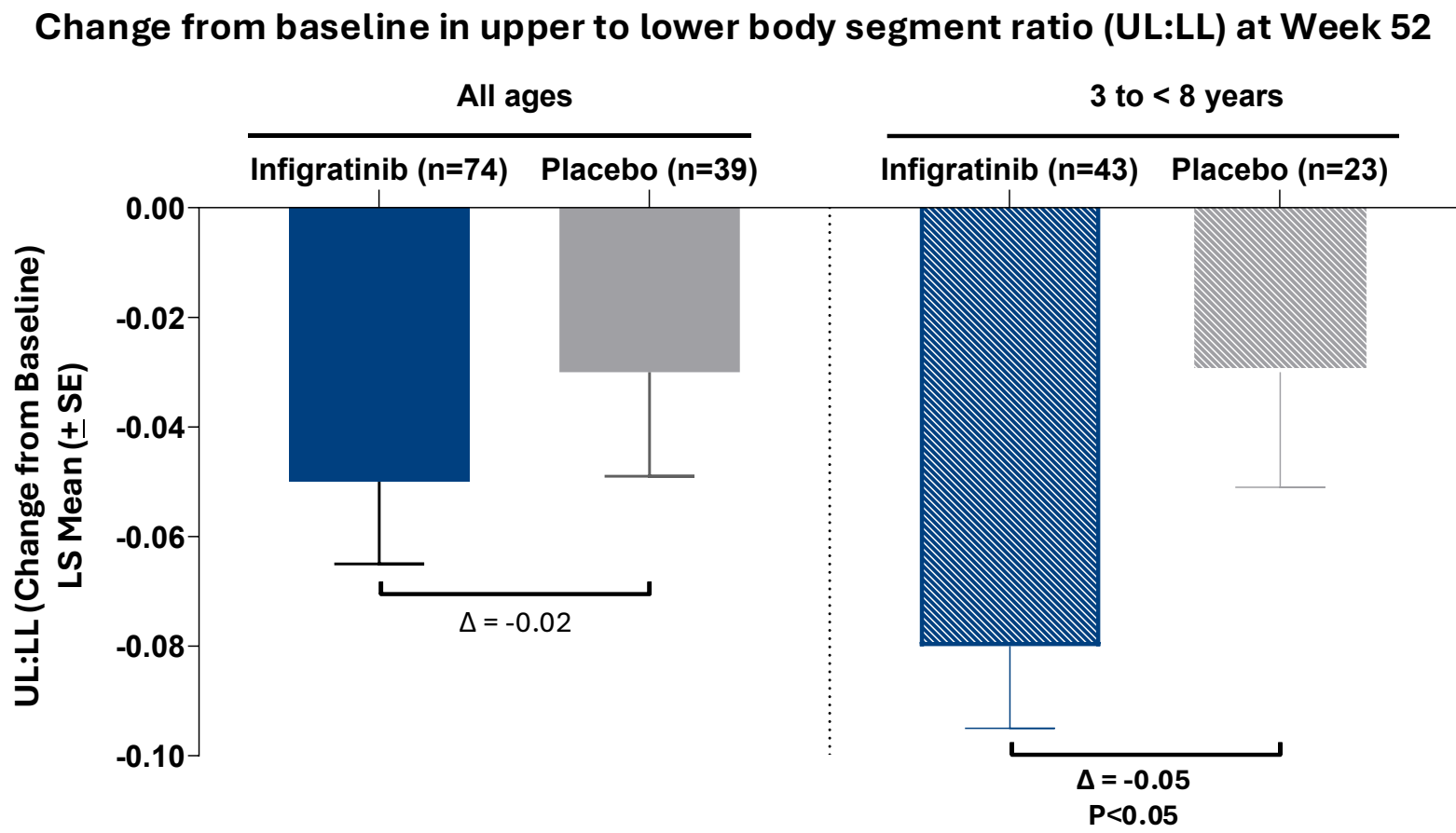
Key secondary endpoint: Change from baseline in height Z-score in relation to an achondroplasia population over 52 weeks



ACH Height Z-score at Week 52	Infigratinib (n=74)	Placebo (n=39)
LS Mean	0.41	0.09
Infigratinib vs. placebo difference, LS mean	0.32	
P-value	p<0.0001	

Infigratinib significantly increased the height Z-score in relation to an ACH population over 52 weeks by 0.32SDs (p<0.0001)

Key secondary endpoint: Change from baseline to Week 52 in upper-to-lower body segment ratio



Infigratinib is the first therapeutic option to demonstrate a statistically significant result on proportionality against placebo in a RCT for ACH

Infigratinib at 0.25 mg/kg was well-tolerated, with no safety signal indicating inhibition of FGFR1 or 2

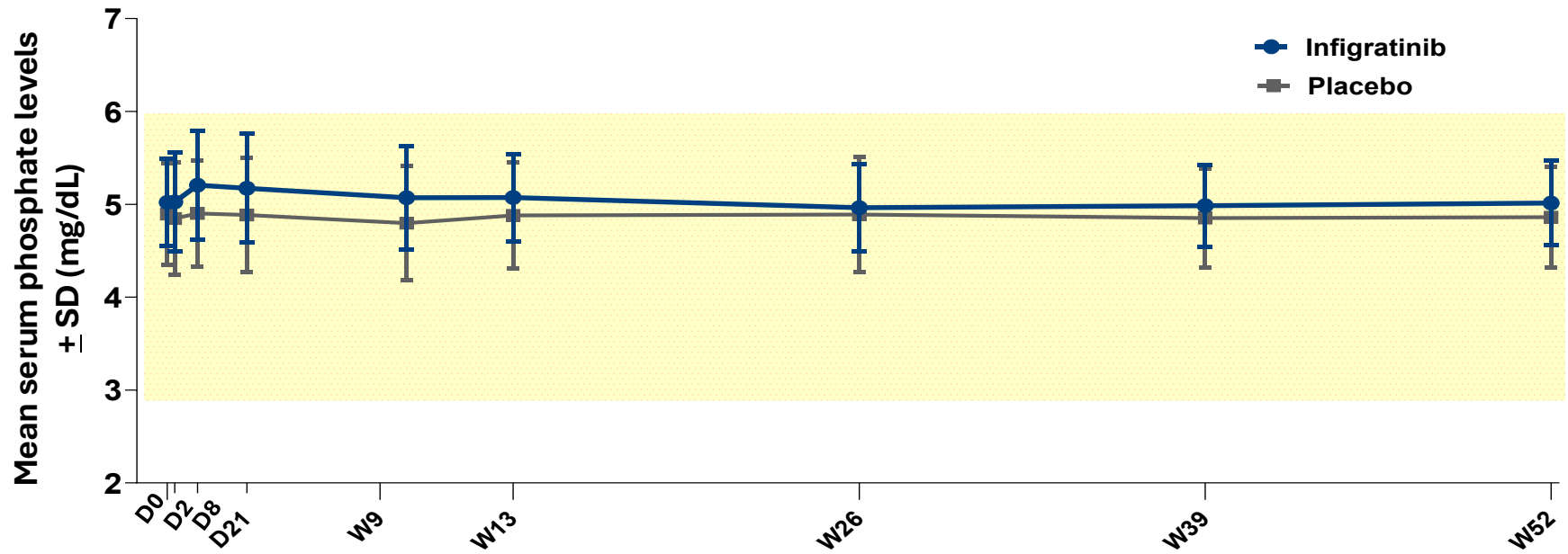
- The majority of treatment-emergent adverse events (TEAEs) were grade 1 or 2 in severity and typical for children of these ages
- TEAEs were balanced between the treatment and placebo arms
- No serious adverse events related to study drug
- No discontinuations due to AEs related to study drug

Areas of interest

- 3 cases of hyperphosphatemia, all mild, asymptomatic, and transient; no cases required a dose reduction or discontinuation
- No AEs associated with the inhibition of FGFR1 or FGFR2 (e.g. retinal or corneal)

Mean serum phosphate levels were comparable across ifigratinib and placebo at all measured time points

Mean Serum Phosphate Levels



For all participants, the maximal change in phosphate levels was within the typical intra-subject variability in the pediatric population¹

Mean phosphate levels (mg/dL)	Baseline	Day 2	Day 8	Day 21	Week 9	Week 13	Week 26	Week 39	Week 52
Infigratinib	5.0	5.0	5.2	5.2	5.1	5.1	5.0	5.0	5.0
Placebo	4.9	4.9	4.9	4.9	4.8	4.9	4.9	4.9	4.9

Mean phosphate levels at all measured time points were similar and within normal ranges

NDA and MAA submissions expected in 2H 2026

Advance infigratinib towards registration in achondroplasia



Submit New Drug Application to FDA
2H 2026



Submit Marketing Authorization Application to EMA
2H 2026

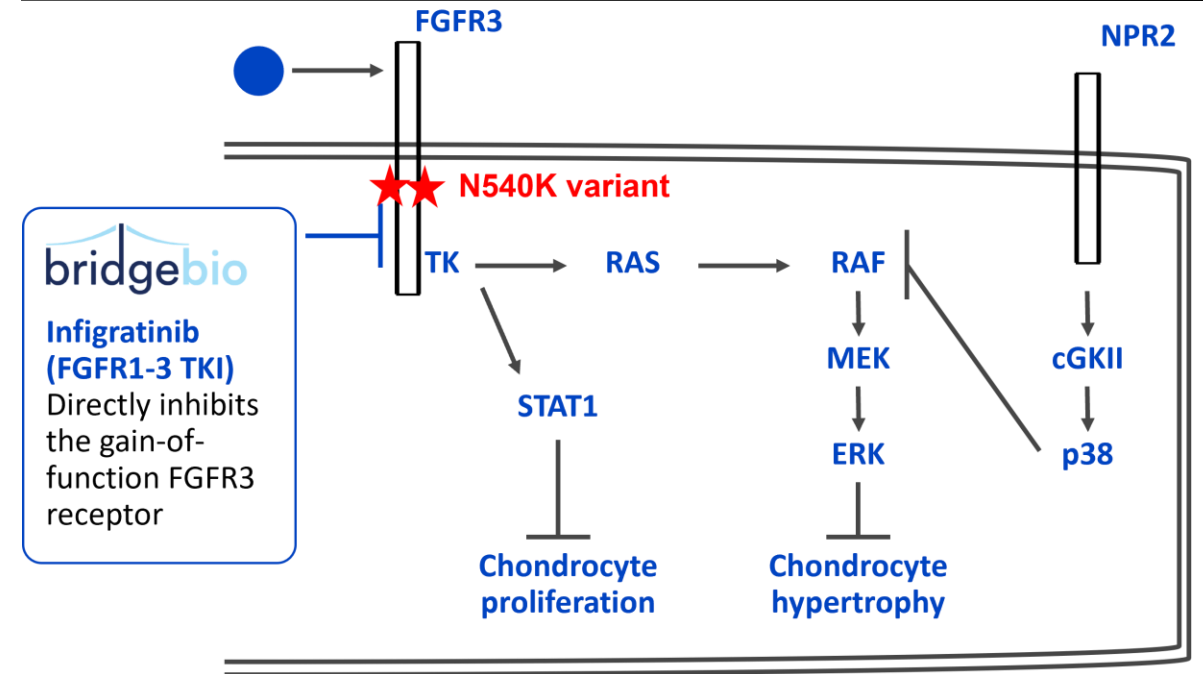


Detailed PROPEL 3 results planned for presentation at scientific and advocacy conferences
2026

Hypochondroplasia is a FGFR3-related skeletal dysplasia

- Autosomal dominant condition
- Similar incidence to achondroplasia
- Greater genetic heterogeneity in *FGFR3* pathogenic variants (e.g. N540K in addition to others)
- Clinical features:
 - Moderate disproportionate short stature
 - Head circumference larger than average
 - Tibial bowing
- Medical complications are milder and less frequent than in achondroplasia
 - Motor milestones less delayed
 - Reports of epilepsy, temporal lobe abnormalities & other cognitive functions^{2,3}

Infigratinib directly targets FGFR3 signaling



Infigratinib directly targets the underlying cause of hypochondroplasia, FGFR3 overactivity

Hypochondroplasia is a large, mechanistically de-risked expansion opportunity; First participant dosed in Phase 2 study

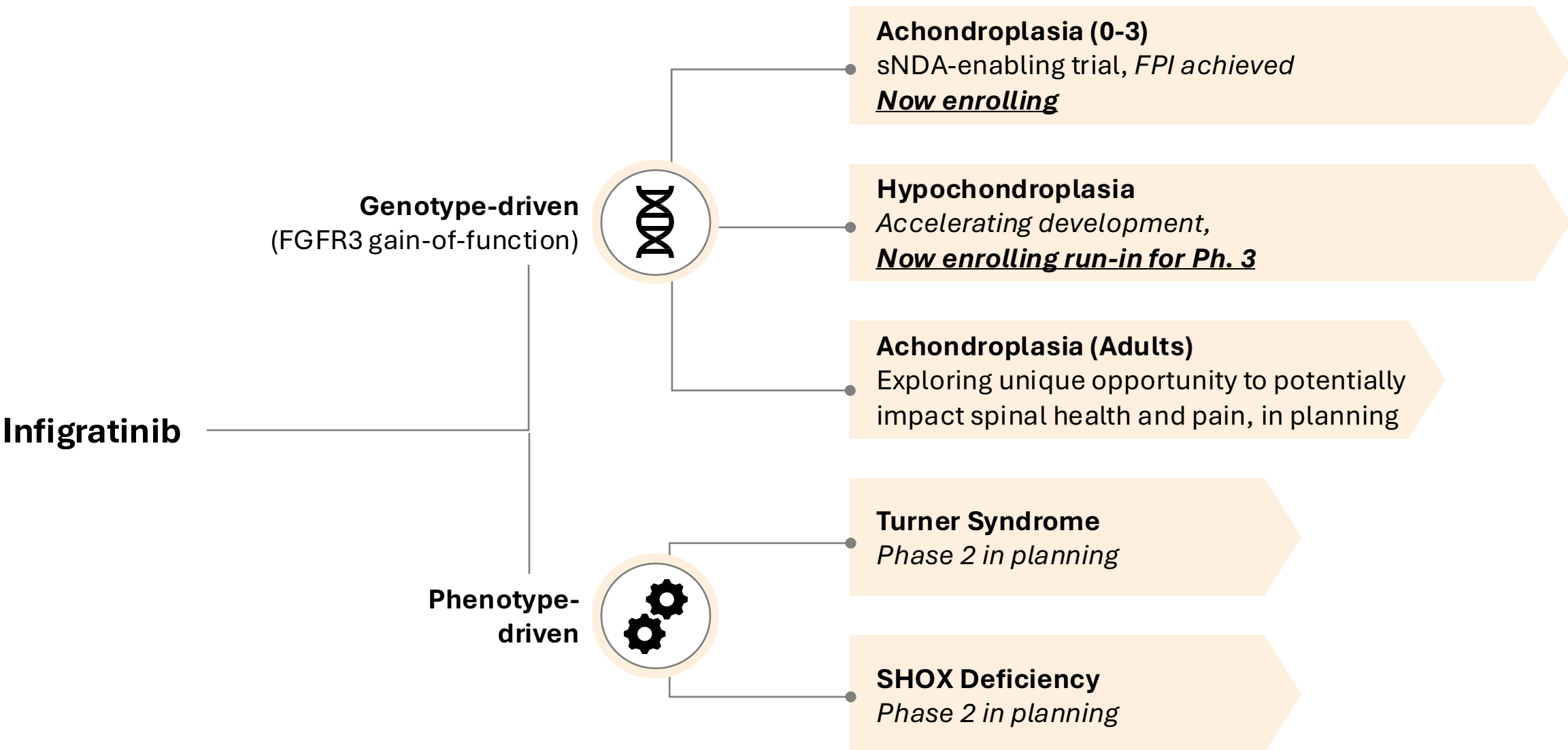
Rationale for infigratinib

- **Directly targets the underlying cause of hypochondroplasia, FGFR3 overactivity^{1,2}**
- **Single-digit nM potency against multiple FGFR3 variants associated with hypochondroplasia, including in vivo improvement of bone growth in an HCH mouse model^{1,2}**
- **Ability for infigratinib to cross the blood-brain barrier³ to potentially address any neurological or cognitive manifestations^{4,5}**

Recent progress and upcoming milestones

- **The observational run-in study for the Phase 3 is now enrolling**
- **Based on totality of data in ACH, we are now accelerating our HCH program**
- **Phase 2 results expected in 2H 2026**

We are on Day 1 of the opportunity for infigratinib



BBP-812



Canavan disease is a severe, fatal, and ultra-rare neurodegenerative pediatric disease with no approved therapies

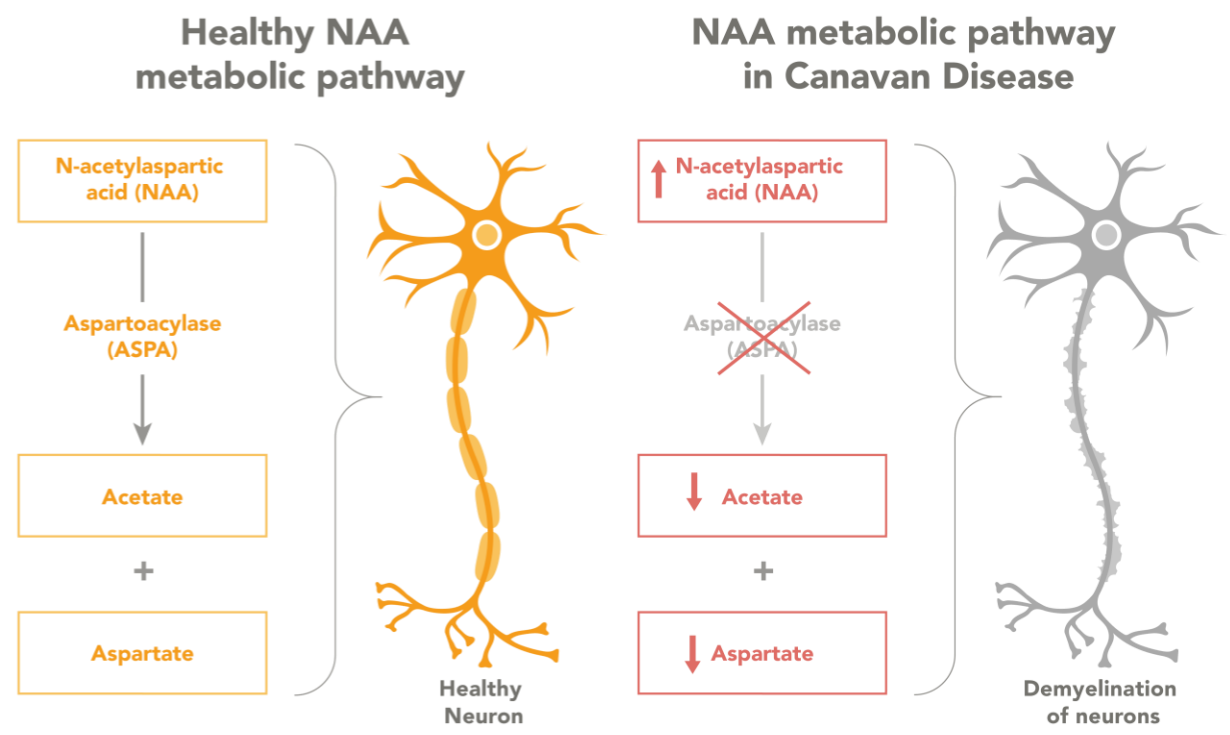
Unmet need

- Canavan disease (CD) is an ultra-rare neurodegenerative disease with ~1,000 patients across the US and EU
- CD is usually fatal within the first two decades of life, and >25% of patients die by the age of 10 years
- Children with CD exhibit global and severe cognitive, motor, and language impairment, missing or regressing on most developmental milestones
- Children with CD require around the clock care – they cannot hold their heads up, sit, crawl, walk, are generally unable to speak, and suffer from seizures and spasticity
- There are no therapies available for Canavan disease



BBP-812 is a potentially first-in-class, disease-modifying therapy that targets Canavan disease directly at its source

Mechanism



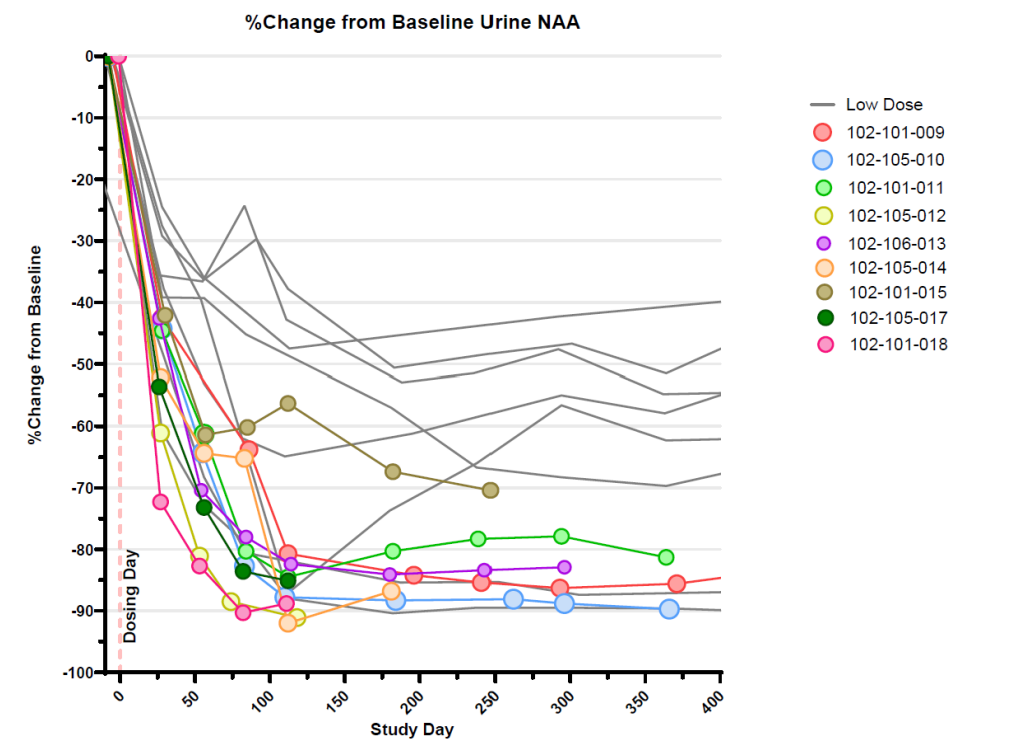
BBP-812 is an AAV9 gene therapy which directly replaces the mutated ASPA gene that causes Canavan disease

Design Principles

- Provide first disease-modifying therapy**
Target the condition directly at the source, utilize single registrational study & biomarker for accelerated approval
- Provide therapy based on known safety profile**
Leverage safety profile from approved IV AAV9 gene therapy
- Avoid invasive neurosurgery**
Provide a less invasive IV treatment option to minimize burden for patients and their caregivers

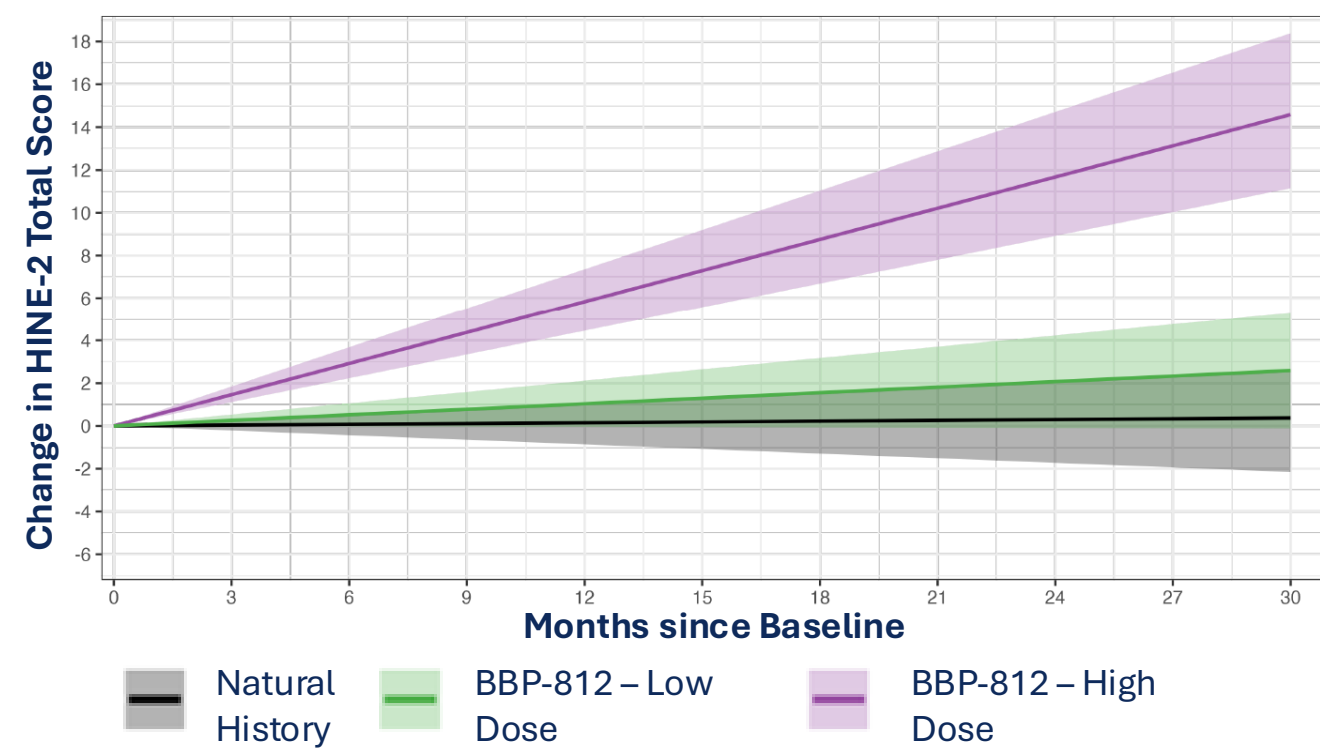
Current path to a potential BLA filing in 2027 based on reductions in urine NAA (surrogate endpoint) supported by motor function improvements

Urine N-acetylaspartic acid (NAA) levels



- **BBP-812 dose-dependently reduces urine NAA** to levels associated with only mild disease
- **FDA is open to the use of urine NAA as a surrogate endpoint** to support accelerated approval of BBP-812

Hammersmith Infant Neurological Examination (HINE-2) Trajectory



- Trajectory analysis shows **clear, dose-dependent separation in HINE-2 total score** with BBP-812 vs. natural history study
- Children are also showing **improvement on key motor metrics such as sitting, head control, and reaching / grasping**

BBP-812 is changing the lives of children with Canavan and the lives of their families

Study Investigator Quotes “

Patient is showing “lots of improvement at 3-month visit, **emotional presence, visual tracking, reaching for things**”

Dr. Alexander Fay, UCSF Benioff Children's Hospital

“One patient in particular is **sitting independently and taking steps and walking**, and that is certainly something I’ve never seen with Canavan disease.”

Dr. Florian Eichler, Mass General Hospital for Children

"It is **unheard of for a child with typical Canavan disease to be able to walk independently**. None of the 23 individuals with Canavan disease described in the natural history by Bley et al (2021) were able to walk independently. **In our experience, this is a remarkable finding.**"

Dr. Amanda Nagy, Mass General Hospital for Children

Post-Treatment Patient Progression

+ gene therapy (22 months old)

12 months old (pre-treatment)



24 months old



30 months old



48 months old



BridgeBio Ecosystem Highlights



BBOT has progressed a pipeline of molecules into the clinic that are poised to close key activity gaps in the RAS-focused therapeutic space

BBO-8520

First direct inhibitor
of KRAS^{G12C} ON/OFF with
effector blockade function

- ✓ Promising monotherapy data with **65% ORR, 66% 6-mo. PFS, and 83% of patients remaining on treatment** for ≥ 6 mo follow-up; **differentiated tolerability**
- ✓ **Differentiated pembro combination safety** observed at **optimally active dose level** with **favorable liver safety profile compared to pembro alone**
- ✓ Promising early efficacy signals in combination with pembro
- ✓ Encouraging early efficacy signals in patients **with STK11/KEAP1 co-mutations**

BBO-11818

Potent, direct inhibitor
of panKRAS ON/OFF

- ✓ **Partial response (PR) observed in PDAC**
- ✓ Anti-tumor activity across dose levels and tumor types with tumor reductions at higher dose levels
- ✓ **Differentiated safety profile** observed in dose escalation
- ✓ **PK exposure approximately dose proportional**

BBO-10203

Novel RAS:PI3K α Breaker
specifically blocks RAS activation
of PI3K α

- ✓ Potentially **differentiated safety** profile
- ✓ **No observed events of hyperglycemia with no enrollment restrictions on HbA1c and glucose levels**
- ✓ Achieved **target systemic exposure and rapid full target engagement**
- ✓ Recommended dose for expansion has been determined **and combo cohorts are open**

The GondolaBio pipeline features a diverse set of programs across therapeutic areas and modalities

Indication	Patient Population (US+EU)	Discovery	Lead Op	IND Enabling	Phase 1	Phase 2
Erythropoietic Protoporphyria (EPP)	25k					
Autosomal Dominant Polycystic Kidney Disease (ADPKD)	300k					
Alpha-1 Antitrypsin Deficiency (AATD)	200k					
Charcot-Marie-Tooth 1A (CMT1A)	130k					
Neurofibromatosis Type 1 (NF1)	200k					
Hereditary Pancreatitis	30k					
Fibrous Dysplasia	50k					
Tuberous Sclerosis Complex 1/2 (TSC)	65k					
Genetic Epilepsy Driven by SynGAP1 Mutations	15k					
Dup15q Developmental Epileptic Encephalopathy	20k					
Recurrent Oxalate Kidney Stones	300k					
Best vitelliform macular dystrophy	15k					
Early onset preeclampsia	40k					
+4 discovery programs						

About Attruby® and BridgeBio

About Attruby® (acoramidis)

Attruby is the only near-complete ($\geq 90\%$) stabilizer of Transthyretin (TTR) approved in the U.S. for the treatment of adult patients with ATTR-CM to reduce cardiovascular death and cardiovascular-related hospitalization. Attruby was generally well-tolerated. The most common side effects were mild and included diarrhea and abdominal pain that were resolved without drug discontinuation. BridgeBio offers an extensive suite of programs to help patients access our medicines. Visit [Attruby.com](https://attruby.com) for more information, including full Prescribing Information.

About BridgeBio Pharma, Inc.

BridgeBio Pharma, Inc. (BridgeBio) is a new type of biopharmaceutical company founded to discover, create, test, and deliver transformative medicines to treat patients who suffer from genetic diseases. BridgeBio's pipeline of development programs ranges from early science to advanced clinical trials. BridgeBio was founded in 2015 and its team of experienced drug discoverers, developers and innovators are committed to applying advances in genetic medicine to help patients as quickly as possible. For more information visit bridgebio.com.