Credit Suisse 29th Annual Virtual Healthcare Conference

John F. Crowley, Chairman & Chief Executive Officer

November 9, 2020

Forward-Looking Statements

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product. In addition to the impact of the COVID-19 pandemic, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. Statements regarding corporate financial guidance and financial goals and the attainment of such goals. With respect to statements regarding projections of the Company's revenue and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2019, the Quarterly Report filed on Form 10-Q for the quarter ended June 30, 2020, and the Quarterly Report filed on Form 10-Q to be filed today. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

In addition to financial information prepared in accordance with U.S. GAAP, this presentation also contains adjusted financial measures that we believe provide investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. Full reconciliations of GAAP results to the comparable non-GAAP measures for the reported periods appear in the financial tables section of this presentation. When we provide our expectation for non-GAAP operating expenses on a forward-looking basis, a reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure generally is not available without unreasonable effort due to potentially high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure in the relevant future period, such as unusual gains or losses. The variability of the excluded items may have a significant, and potentially unpredictable, impact on our future GAAP results.

A RARE COMPANY

A leading fully integrated, global rare disease biotechnology company

Galafold® (migalastat)

First Oral Precision Medicine for Fabry Disease

Gene Therapy PLATFORM

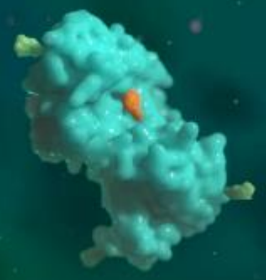
Protein Engineering & Glycobiology

World Class BIOLOGICS Capabilities

EMPLOYEES in 27 Countries

AT-GAA

Phase 3 in Pompe Disease



Robust R&D Engine

Nearly 50+ Lysosomal Disorders and More Prevalent Rare Diseases

$509.1M Cash

as of 9/30/20

Two Clinical-Stage Gene Therapies

GLOBAL COMMERCIAL ORGANIZATION

Key Takeaways

Recent successes across our science, clinical, regulatory and commercial efforts position us for the future

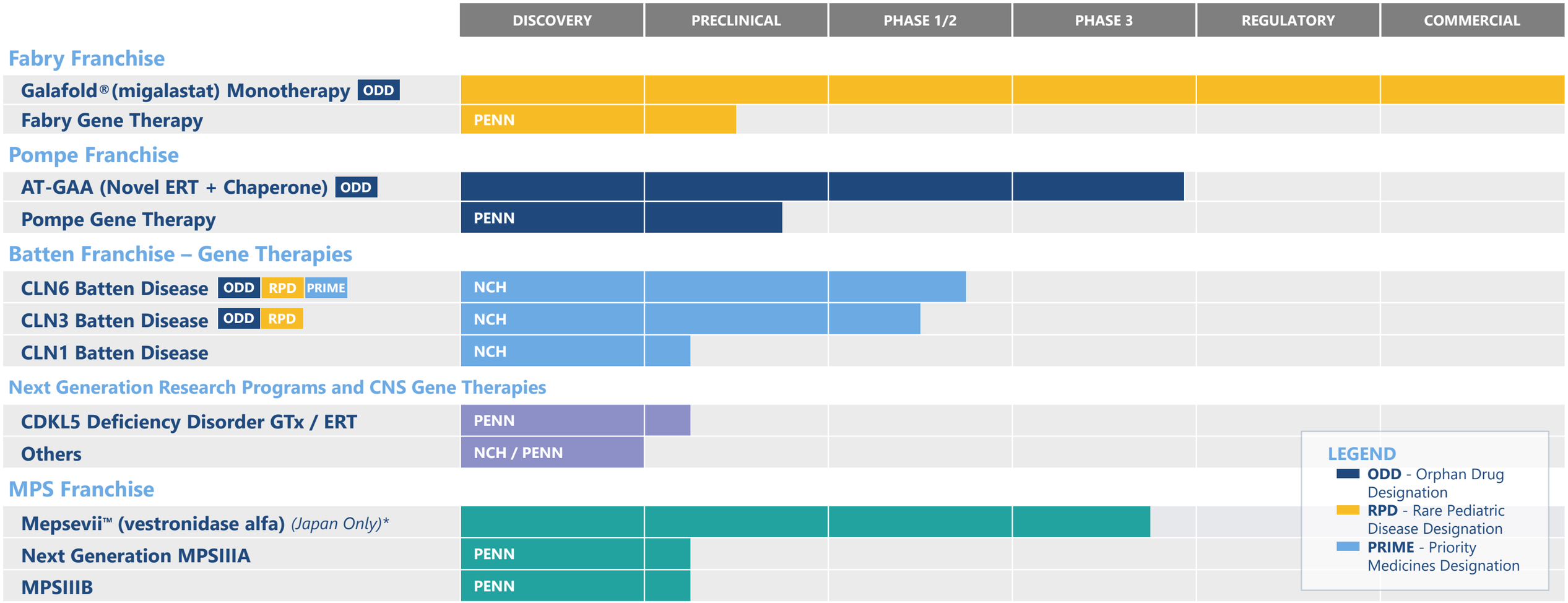
Galafold Continues Strong Launch Performance & Cornerstone of Amicus Success

AT-GAA for Pompe Advances Toward Approval as "Crown Jewel" of Amicus Portfolio

Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future

Cash Position Sufficient to Achieve Self-Sustainability without the Need for Future Dilutive Financing

A RARE PORTFOLIO



*Exclusive license from Ultragenyx for Japanese rights to Mepsevii™, investigator-sponsored trial in Japan underway

Amicus Therapeutics

Galafold® (migalastat) Global Launch…

…taking a leadership role in the treatment of Fabry disease

"We push ideas as far and as fast as possible"
- Amicus Belief Statement

Galafold Snapshot (as of September 30, 2020)

Galafold is an orally delivered small molecule precision medicine with a unique mechanism of action for Fabry patients with amenable variants that replaces the need for intravenously delivered ERT

One of the Most Successful Rare Disease Launches

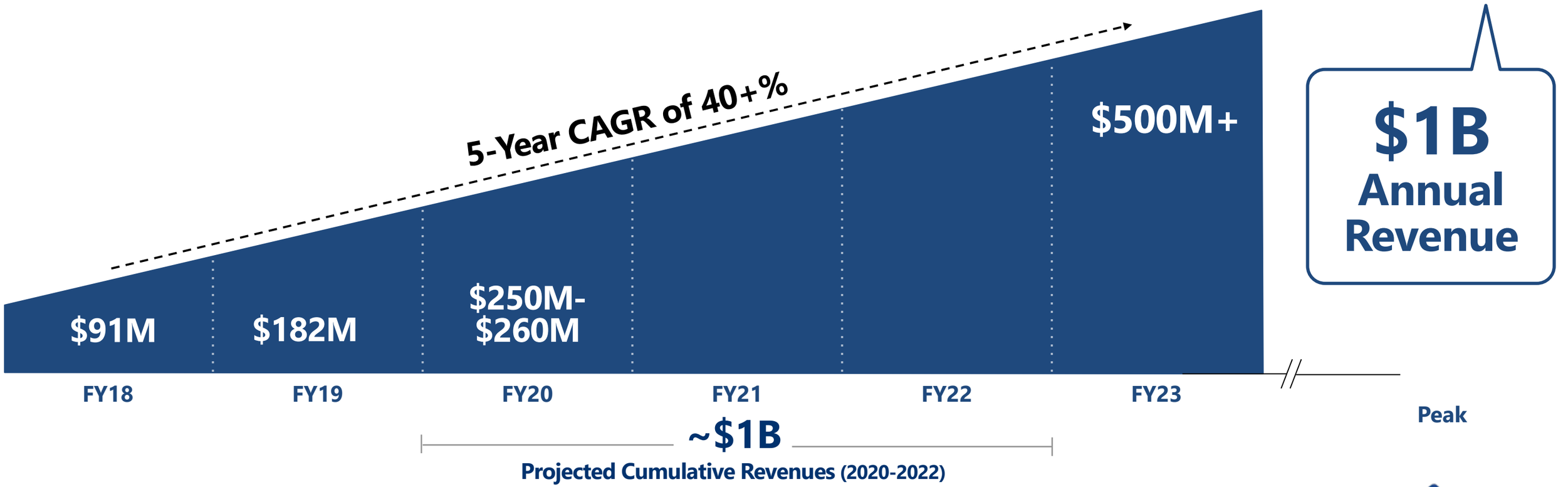
- **$67.4M** 3Q20 Galafold Revenue
- **$250-260M** FY20 Global Galafold Rev. Guidance
- **1,384** Amenable Mutations Included in the EU Label
- **348** Amenable Variants in U.S. Label
- **Continued Expansion in 2020**
- **40+** Countries with Regulatory Approvals: including U.S., EU, Japan, and other Countries

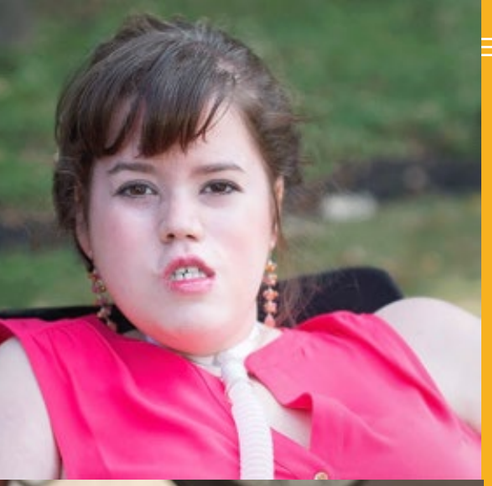
Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit https://www.amicusrx.com/pi/Galafold.pdf. For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at www.ema.europa.eu.

Amicus Therapeutics

Galafold Growth Trajectory

Galafold is on track to generate $1B+ in projected cumulative revenues from 2020-2022 and is on an anticipated path to $500M+ in annual sales in 2023 and $1B+ annual sales at peak





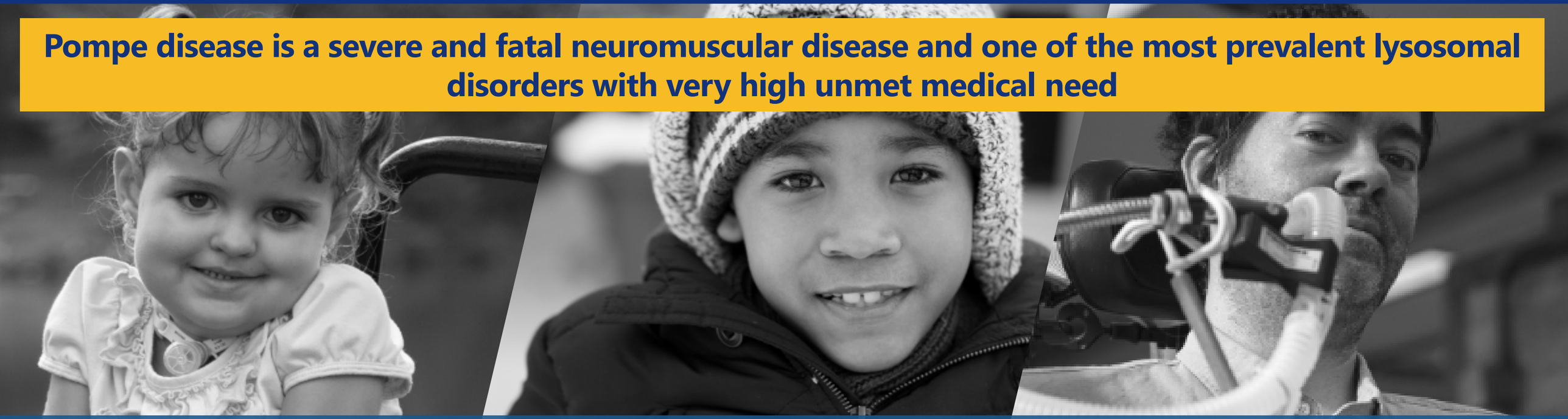
AT-GAA: Next Potential Standard of Care for Pompe Disease

"We encourage and embrace constant innovation"

- Amicus Belief Statement

Pompe Disease Overview

Pompe disease is a severe and fatal neuromuscular disease and one of the most prevalent lysosomal disorders with very high unmet medical need



5,000 – 10,000+ patients diagnosed WW[1]; newborn screening suggests underdiagnosis

Age of onset ranges from infancy to adulthood

Patients on current standard of care decline after ~2 years

Respiratory and cardiac failure are leading causes of morbidity and mortality

Deficiency of GAA leading to glycogen accumulation and cellular dysfunction

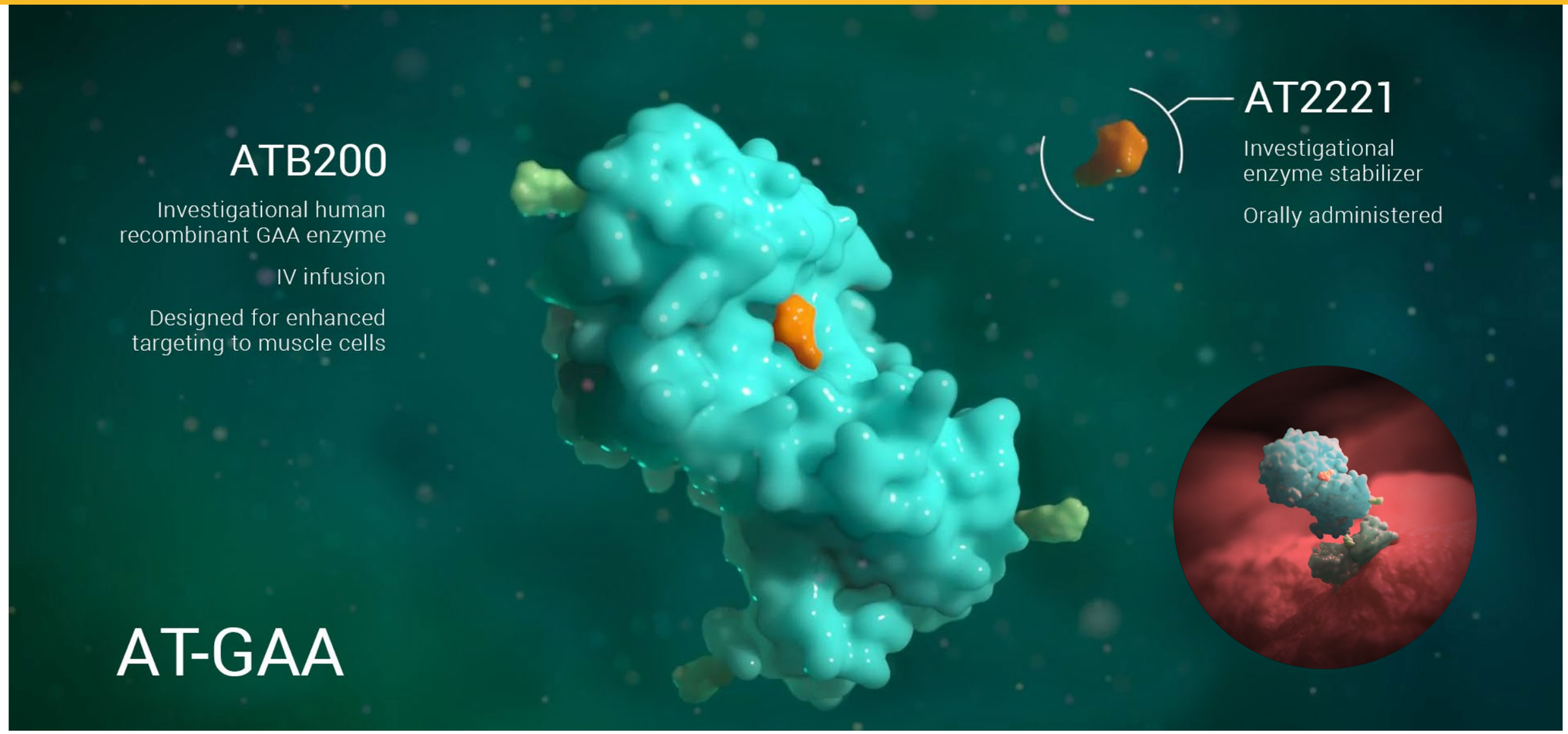
Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

~$1B+ global Pompe ERT sales[2]

1. National Institute of Neurological Disorders and Stroke (NIH). 2. Based on calendar year ending September 30, 2019. Exchange rate as of 1/6/19. Source: Sanofi Press Releases

AT-GAA: Foundation in Protein Engineering

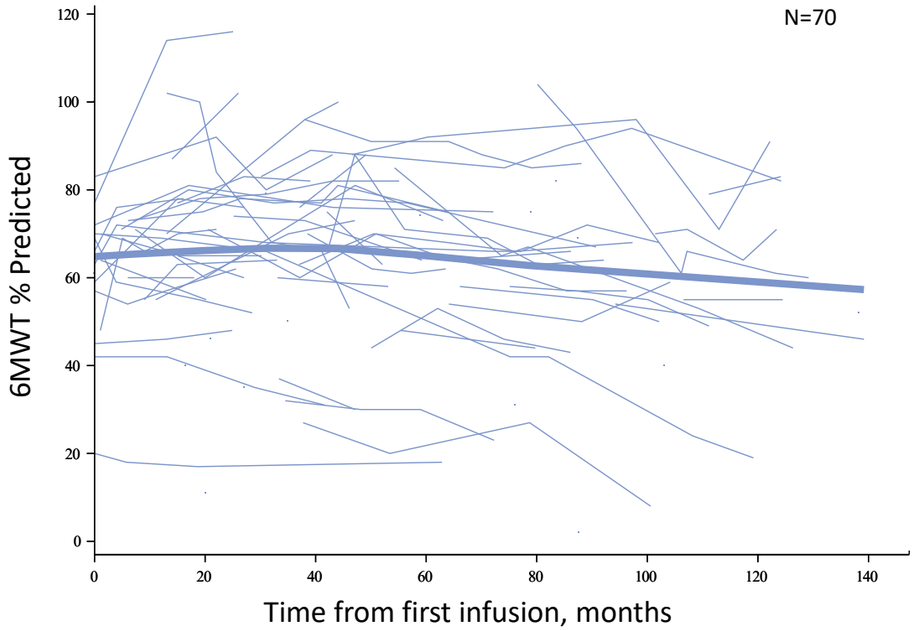
Amicus scientists created a uniquely glycosylated and highly phosphorylated ERT (AT-GAA) that significantly enhances targeting to key muscles affected



Amicus POM-002 Natural History Study

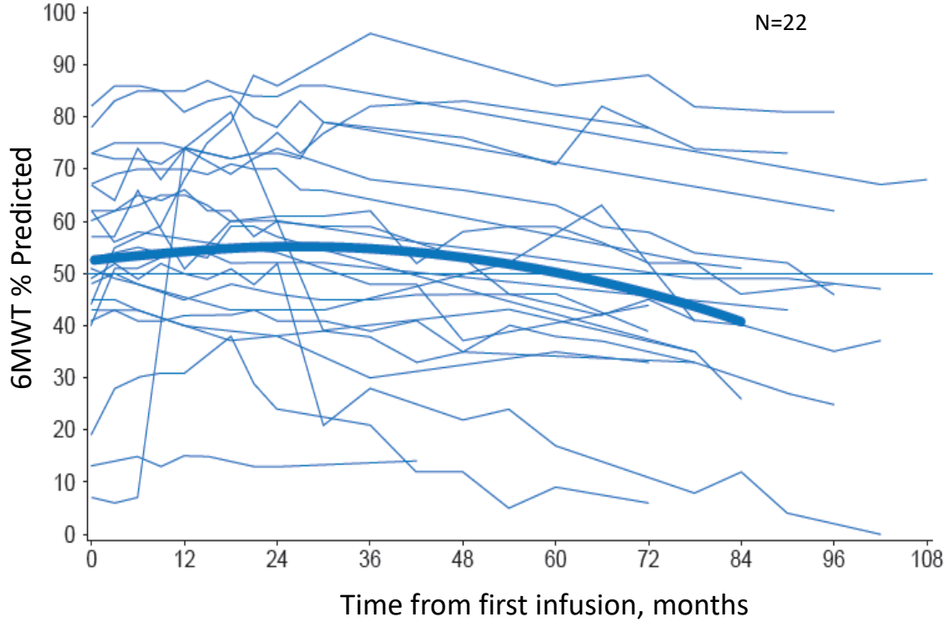
Data from the Amicus POM-002 chart review study in patients treated long-term with alglucosidase alfa is consistent with the medical literature and further supports PROPEL design assumptions

POM-002: Longitudinal 6MWD



Note: The blue solid curve is the estimated trendline obtained using a Loess regression with the smooth parameter at 0.8. Time (in months) on the X-axis was calculated from the actual assessment dates relative to the first dose of ERT.

van der Ploeg 2017: Longitudinal 6MWD



Note: Data were integrated from 68 patients from two sequential clinical studies (Late-Onset Treatment Study [LOTS; NCT00158600] and LOTS Extension [NCT00455195]2) and the Pompe Registry; Ans T. van der Ploeg et al. Poster presented at the 13th Annual WORLD Symposium™ 2017, February 13–17, 2017, San Diego, CA, USA

Pompe Patient Experience in Phase 1/2 Clinical Study (ATB200-02)

Phase 1/2 results showed strong and durable effects in patients out to two years, leading to dramatic improvements in muscle strength and function, as well as significant improvements in key biomarkers of disease

6-Min Walk Test (m)

Cohort	Baseline (n=10)	Change at Month 6 (n=10[a]) Mean (SD)	Change at Month 12 (n=10[a]) Mean (SD)	Change at Month 24 (n=9[a,b]) Mean (SD)
Cohort 1 ERT-Switch Ambulatory	**397.2** (96.8)	**+23.9** (52.2)	**+42.2** (46.5)	**+36.4** (60.5)
Cohort	**Baseline (n=5)**	**Change at Month 6 (n=5) Mean (SD)**	**Change at Month 12 (n=5) Mean (SD)**	**Change at Month 24 (n=5) Mean (SD)**
Cohort 3 ERT-Naïve	**399.5** (83.5)	**+41.8** (29.4)	**+63.1** (29.1)	**+60.7** (36.5)

FVC (% Predicted)

Cohort	Baseline (n=9[c])	Change at Month 6 (n=9[c]) Mean (SD)	Change at Month 12 (n=9[c]) Mean (SD)	Change at Month 24 (n=8[b,c]) Mean (SD)
Cohort 1 ERT-Switch Ambulatory*	**52.6** (14.7)	**-1.2** (4.0)	**-3.0** (6.0)	**+0.9** (4.9)
Cohort	**Baseline (n=5)**	**Change at Month 6 (n=5) Mean (SD)**	**Change at Month 12 (n=5) Mean (SD)**	**Change at Month 24 (n=5) Mean (SD)**
Cohort 3 ERT-Naïve	**53.4** (20.3)	**+4.4** (5.6)	**+4.6** (8.8)	**+6.8** (6.8)

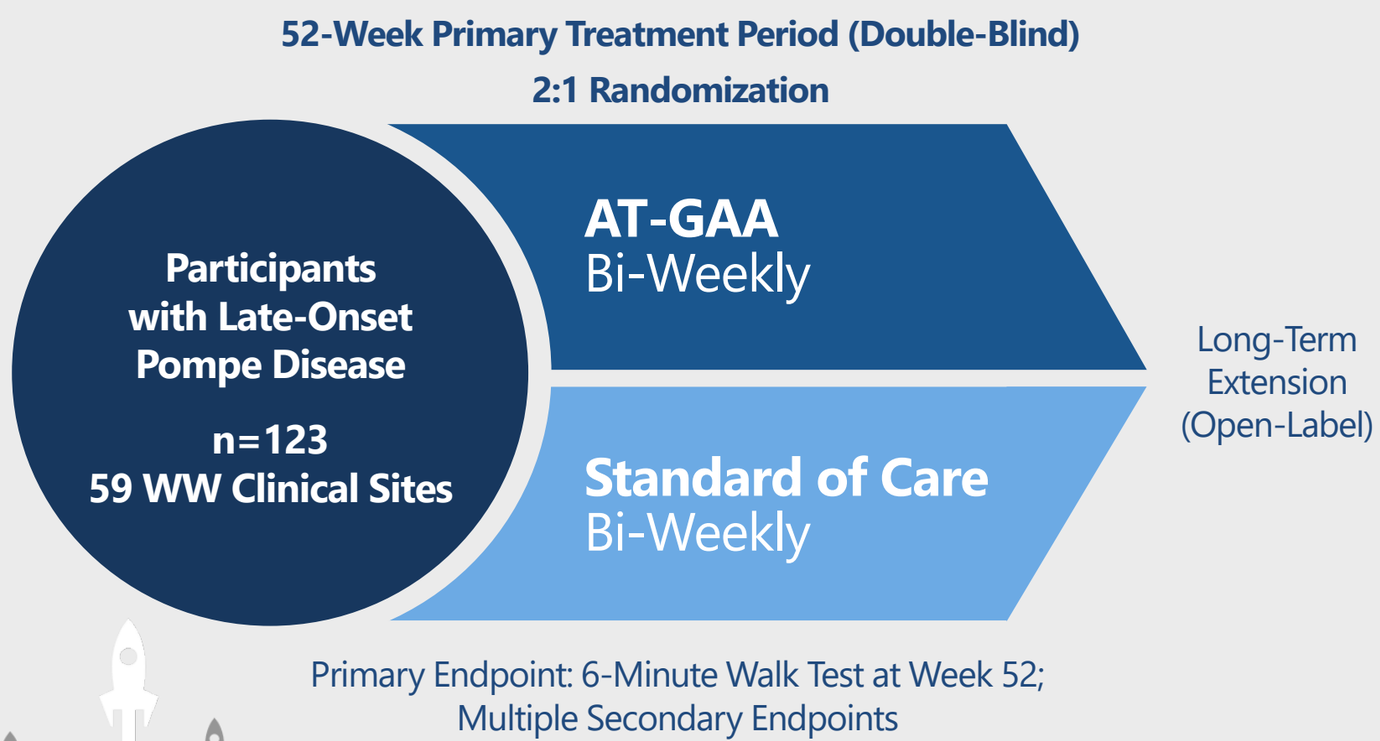
Data from interim analysis 8.

[a] One patient in Cohort 1 discontinued after 18 weeks due to burden of travel; baseline value is not shown for this patient. [b]One patient in Cohort 1 discontinued from study before Month 24. [c]Baseline FVC not available for 1 patient in Cohort 1

Amicus Therapeutics

PROPEL (ATB200-03) Study Design

Phase 3 exceeded enrollment with data expected in 1Q2021. The study is highly powered for success and supports a broad label, with FDA and EMA agreement on study design and primary endpoint (6MWT)



- **PROPEL pivotal study over-enrolled with data expected in 1Q2021**
- **Study includes ERT-Switch and ERT-Naïve Patients**
- **FDA and EMA agreed upon primary endpoint of 6MWD, an integrated measure of disease progression that evaluates both cardiopulmonary and musculoskeletal systems**

AT-GAA: Key Takeaways

AT-GAA for Pompe Advances Toward Approval as "Crown Jewel" of Amicus Portfolio

- PROPEL study timelines are on track with data expected 1Q2021
  - To date, **97%+** of the 3,100+ planned infusions and assessments for the ongoing PROPEL study have been completed on schedule
- Breakthrough Therapy Designation and the Promising Innovative Medicine designation highlight unmet need in Pompe disease
- U.S. FDA grants rolling BLA submission and on-track to initiate in 4Q2020
- Expanded Access Program for infantile-onset Pompe patients underway
- Process performance qualification (PPQ) runs with our partners at WuXi have been successfully completed for the drug substance and drug product
- Peak revenue potential of $1B-$2B, with exclusivity well into 2030s

Next Generation Gene Therapy Platform

“We have a duty to obsolete our own technologies”

- Amicus Belief Statement

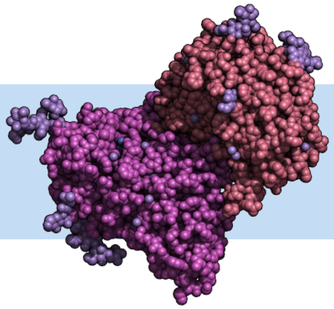
A Natural Evolution: Chaperones to Optimized ERT to Gene Therapy

Amicus' expansion into gene therapy is built upon years of experience in developing genetic medicines designed to deliver deficient proteins to target cells and organelles

Pharmacological Chaperones

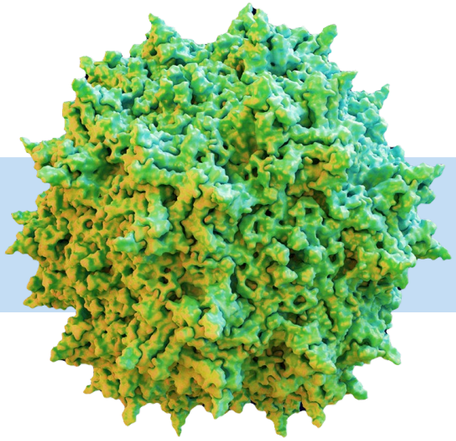
Stabilize
"naturally produced" enzymes

Next-Generation ERTs



Stabilize and target
"externally produced" enzymes

Gene Therapies



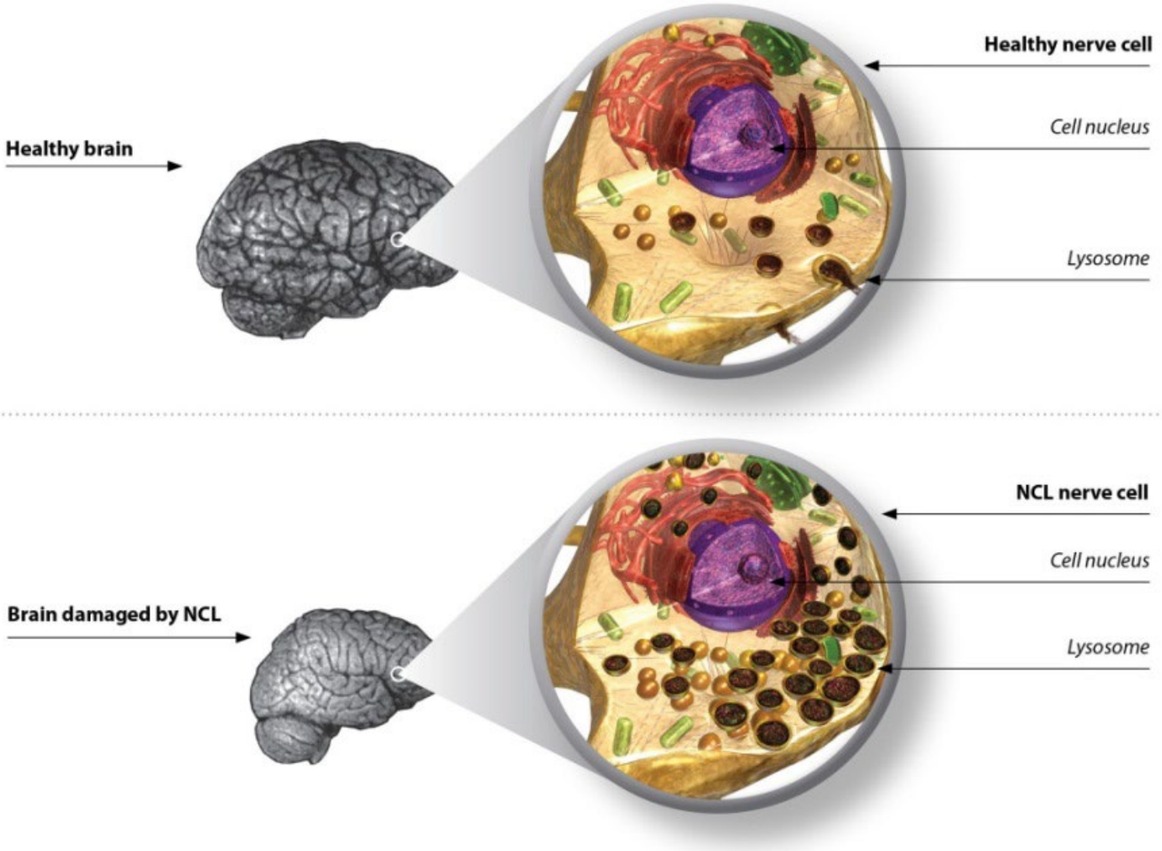
Stabilize and target
"internally produced" enzymes

Batten Disease Overview

Batten disease is a devastating early childhood disease that is 100% fatal in children

Disease Overview

- A group of disorders known as neuronal ceroid lipofuscinoses (NCLs), collectively referred to as Batten disease
- Mutation in one of 13 different CLN genes leads to lysosomal dysfunction
- Signs and symptoms include loss of speech, ambulation, vision and cognition

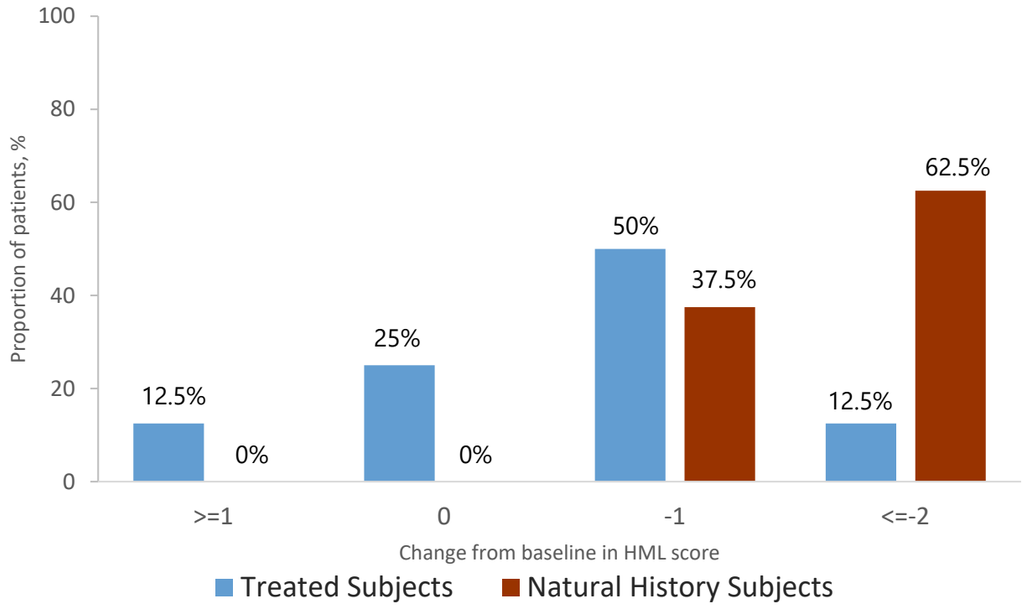


Source: Batten Disease Fact Sheet, NINDS, Publication date June 2018.

Interim Clinical Data for CLN6 Batten Disease Gene Therapy

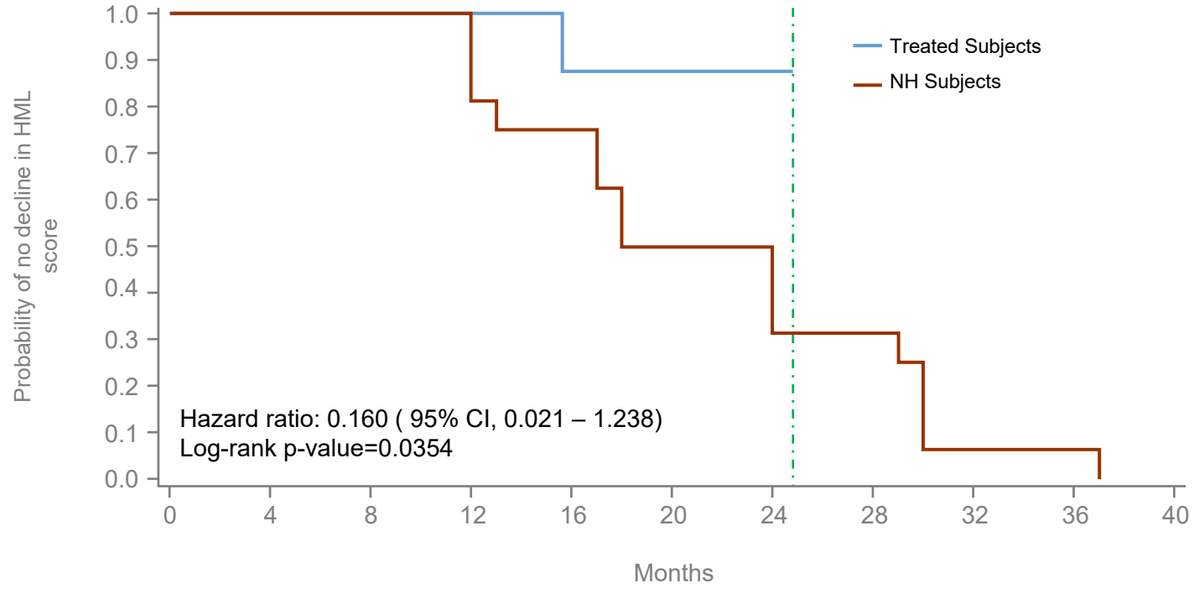
The Hamburg Motor & Language (HML) Score, an assessment of ambulation and speech, shows a meaningful effect in slowing disease progression at 24 months

Proportion of Patients with a Change in the HML Aggregate Score at Month 24[a,b]



Difference in risk of unreversed 2-point decline:
−50% (95% CI, −77.5%, −3.6%)
Exact p-value=0.0335

Time to Unreversed 2-point Decline in the HML Aggregate Score



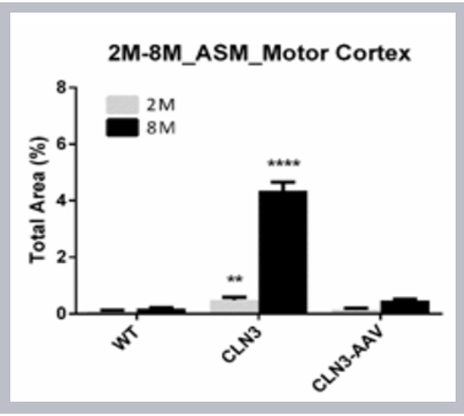
Treated Subjects	8						7					7		
NH Subjects	16			13	12		10	8			5		4	1			0

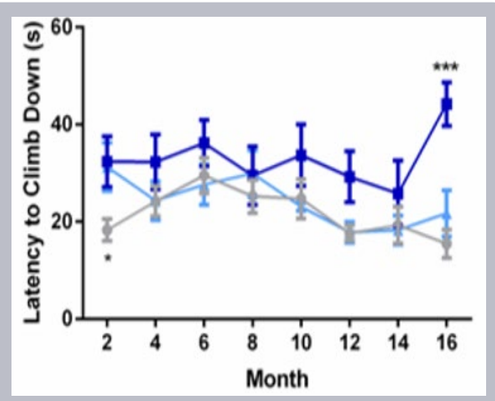
M+L, motor and language. NH, natural history.
[a]The efficacy analysis included all patients with 24-month HML data (8 of 13 treated patients in the study). [b]24-month HML data are available for 16 of 17 patients in the natural history cohort derived from a retrospective CLN6 natural history study conducted by Emily de los Reyes, MD (ClinicalTrials.gov Identifier: NCT03285425). Data cutoff March 13, 2020.

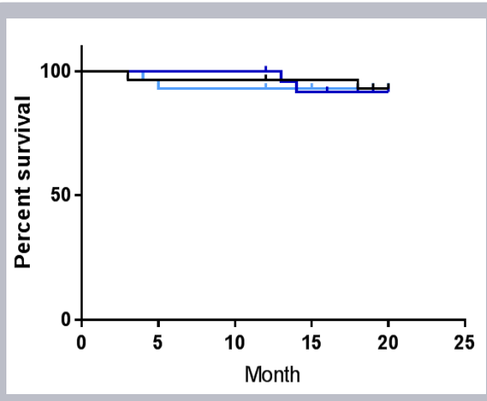
CLN3 Batten Disease: Preclinical and Clinical Summary

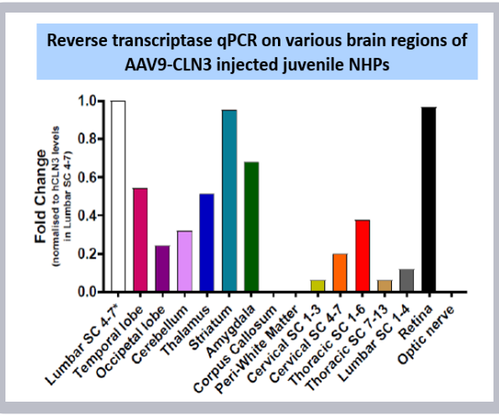
Amicus' second clinical stage gene therapy in CLN3 Batten disease has successfully completed dosing in four children with initial data expected 1Q21

Preclinical Data in KO Mice









Reduction of storage material in mouse model	Improvement of motor function and cognitive behavior in mouse model	Comparable survival in mouse model	Widespread gene expression in brain of NHPs

Source: Data on file

Combines Amicus and Penn Expertise Across Lysosomal and Rare Diseases

An R&D platform with rights to 50+ diseases

Amicus Therapeutics®

Protein Engineering & Glycobiology Expertise

Clinical and Regulatory Expertise

Global Commercial Infrastructure

Next-Generation Gene Therapy Platform

Team of 200+ scientists bringing expertise and experience in:

Vectors, Tropisms, Capsids

Safety

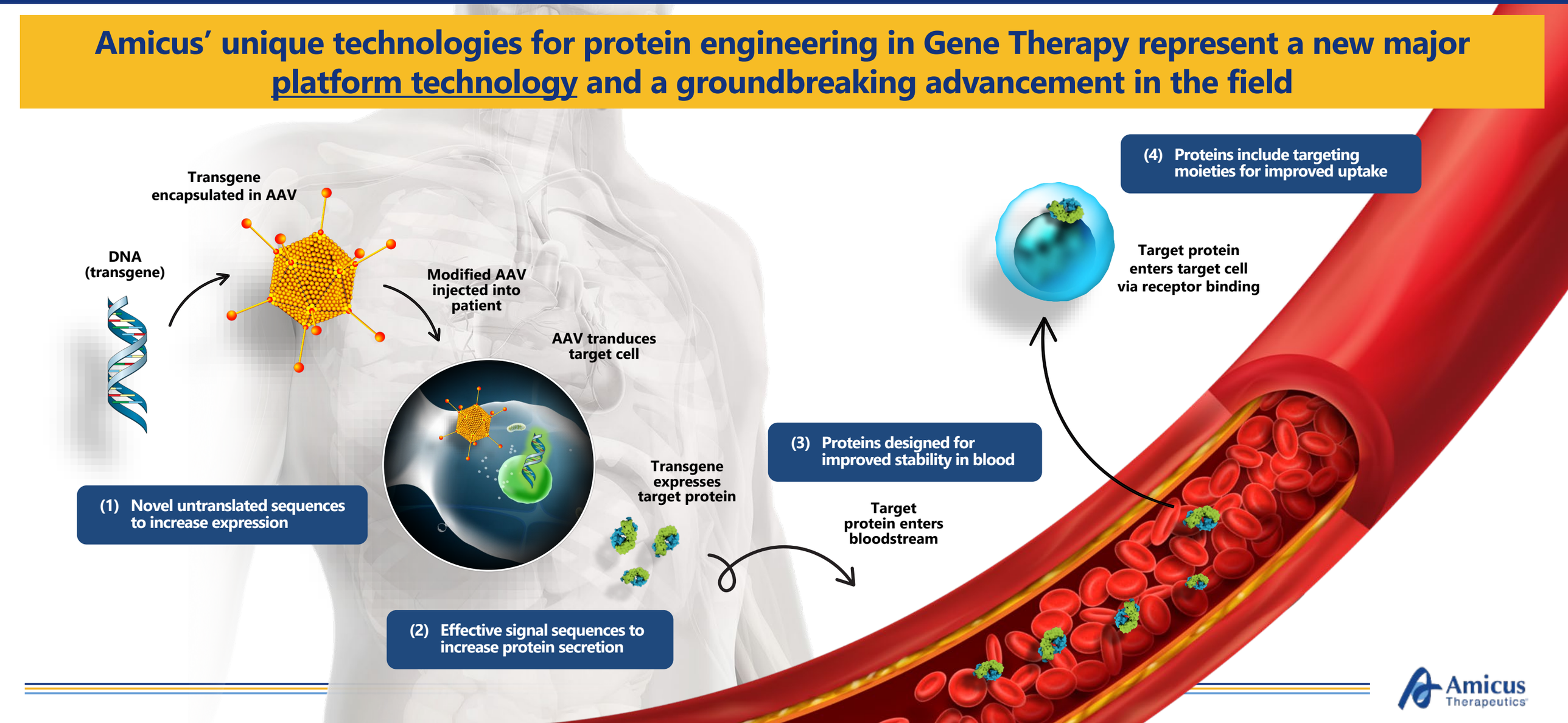
Dosing, Immunology

Manufacturing, Scalability

Penn
University of Pennsylvania

Amicus Approach: Engineered Transgenes for Optimal Cross-Correction

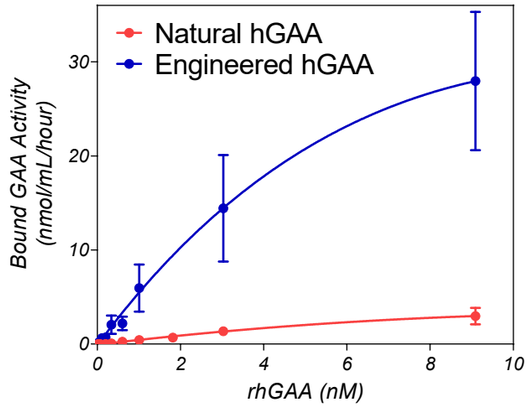
Amicus' unique technologies for protein engineering in Gene Therapy represent a new major platform technology and a groundbreaking advancement in the field



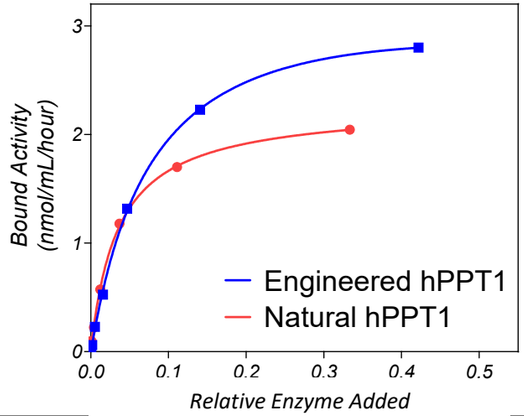
Protein Engineering Platform Has Potential To Be Broadly Applicable to Gene Therapies For Majority of LSDs

Amicus has repeatedly validated the protein engineering platform approach in multiple indications to design transgenes with improved cellular uptake

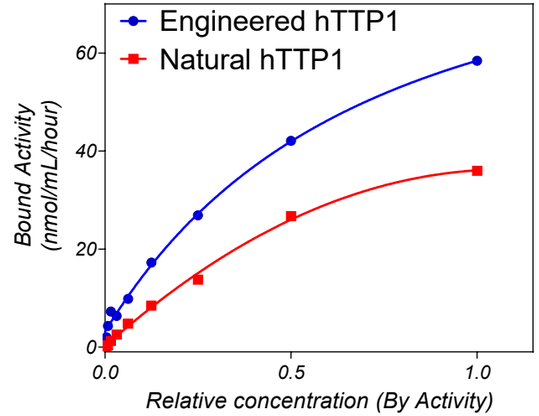
Pompe - GAA



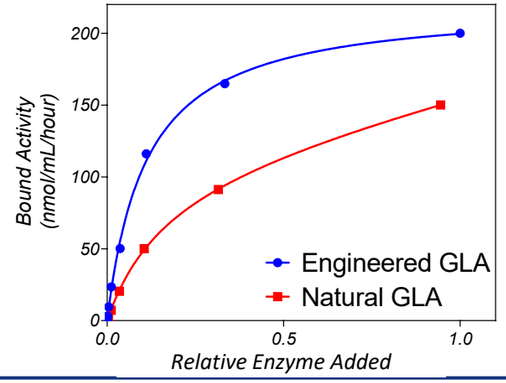
CLN1 – PPT1



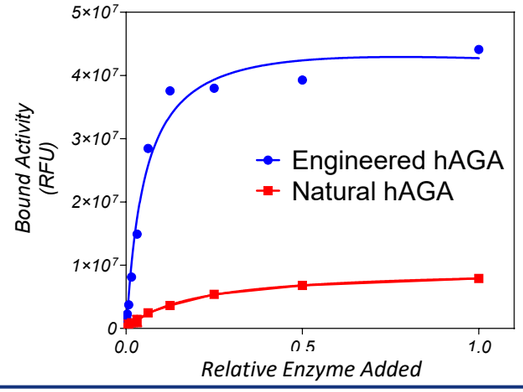
CLN2 – TTP1



Fabry – GLA



AGU – AGA



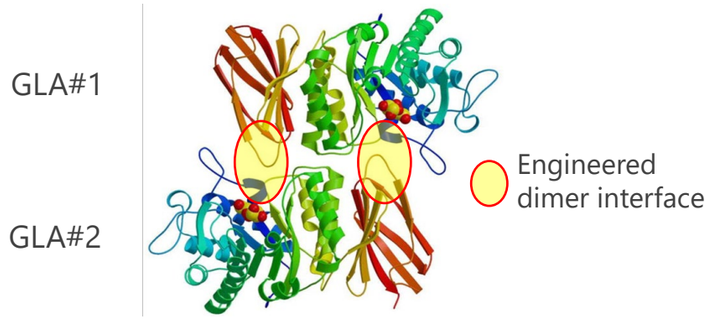
Source: Data on file

Amicus Therapeutics

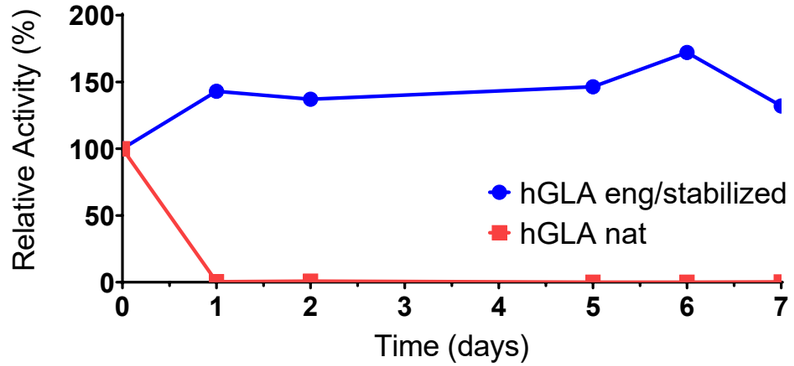
Fabry Gene Therapy IND Candidate

AAV with engineered GLA transgene demonstrated significantly better GL-3 reduction than AAV with wild-type GLA. Full set of preclinical data to be presented at a conference in early 2021

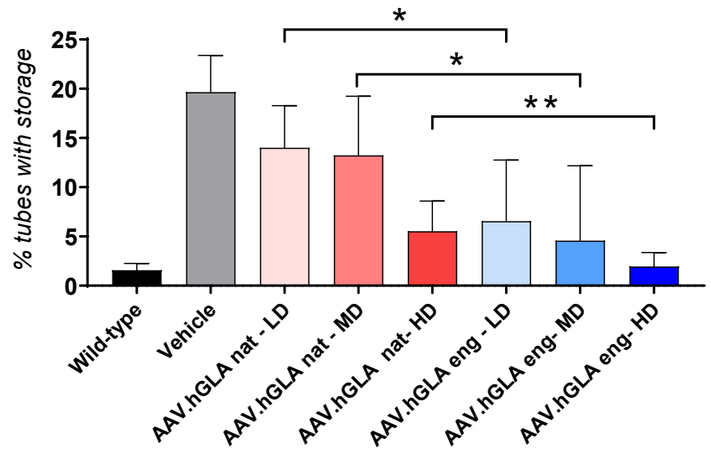
GLA Engineered for Stability



Activity at Neutral pH of Circulation (pH 7.4)



KO Mouse GTx: Kidney GL-3 (% Tubules)



IND Candidate: Approach

- Engineered transgene for improved stability
- Proprietary AAV capsid
- Ubiquitous promoter

Amicus Therapeutics

Gene Therapy: Updates & Key Takeaways

Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future

- CLN6 Phase 1/2 interim data show positive impact with potential to become first-ever approved gene therapy for fatal brain disease in children
- Manufacturing on track to initiate next clinical studies in CLN6 and CLN3 in 2021 using material from planned commercial process
- Orphan drug designations granted in U.S. and EU for intrathecal AAV gene therapies for CLN6 and CLN3 Batten disease; CLN6 granted PRIME designation by EMA; CLN3 granted Fast Track designation by U.S. FDA
- Preclinical studies ongoing for gene therapies in Pompe, Fabry, CDD, MPS IIIA, MPS IIIB and CLN1
- Penn collaboration is R&D engine, with rights to 50+ diseases

Financial & Operational Strategy

“We are business led and science driven”

- Amicus Belief Statement

Financial Outlook: Key Takeaways

- Proceeds from July debt facility places Amicus firmly on a path to self-sustainability
  - Achieved through continued careful expense management, prioritization of early-stage research programs, and measured capital expenditures
- Current cash position of $509.1M as of September 30th
- Company fully funded through major milestones in portfolio and continued global growth
- Cumulative Galafold projected revenue of $1B+ in 2020-2022 offsets significant majority of company spend/investments
- Reaffirming full-year Galafold revenue guidance of $250M to $260M and non-GAAP operating expense guidance of $410M to $420M

At Major Inflection Point: Path to Self-Sustainability

Clear strategy to build our business, advance our portfolio and achieve profitability with the following key priorities:

- Grow Galafold
- Advance AT-GAA to pivotal data, global approvals and launch
- Progress CLN6, CLN3, Pompe and Fabry gene therapies into and through the clinic
- Discover and develop next generation protein engineering and gene therapy technologies with Penn

Cash position sufficient to achieve self-sustainability without the need for any future dilutive financings

Thank You

Amicus Therapeutics®