38th Annual J.P. Morgan Healthcare Conference

John F. Crowley, Chairman and Chief Executive Officer

January 14, 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, business development plans and the projected revenues, sales, expenses 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 or projections will be achieved. Any or all of the forward-looking statements in this presentation 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, actual results may differ materially from those set forth in this presentation 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. With respect to statements regarding projections of the Company's revenue, sales, expenses and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans and strategies. 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, 2018. 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 presentation to reflect events or circumstances after the date hereof.

Non-GAAP Financial Measures

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. 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

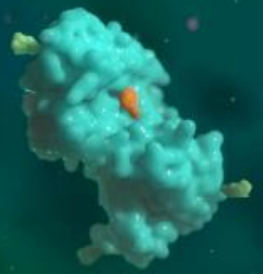
Protein Engineering & Glycobiology

World Class BIOLOGICS Capabilities

EMPLOYEES in 27 Countries

AT-GAA

Phase 3 in Pompe Disease



GLOBAL COMMERCIAL ORGANIZATION

Robust R&D Engine

Nearly 50+ Lysosomal Disorders and More Prevalent Rare Diseases

~$450M+ Cash as of 12/31/19*

Two Clinical-Stage Gene Therapies

*Preliminary and unaudited

A RARE OPPORTUNITY

A broad and patient-focused portfolio to drive value creation

Galafold

$1B+ Opportunity

AT-GAA

Pompe ERT

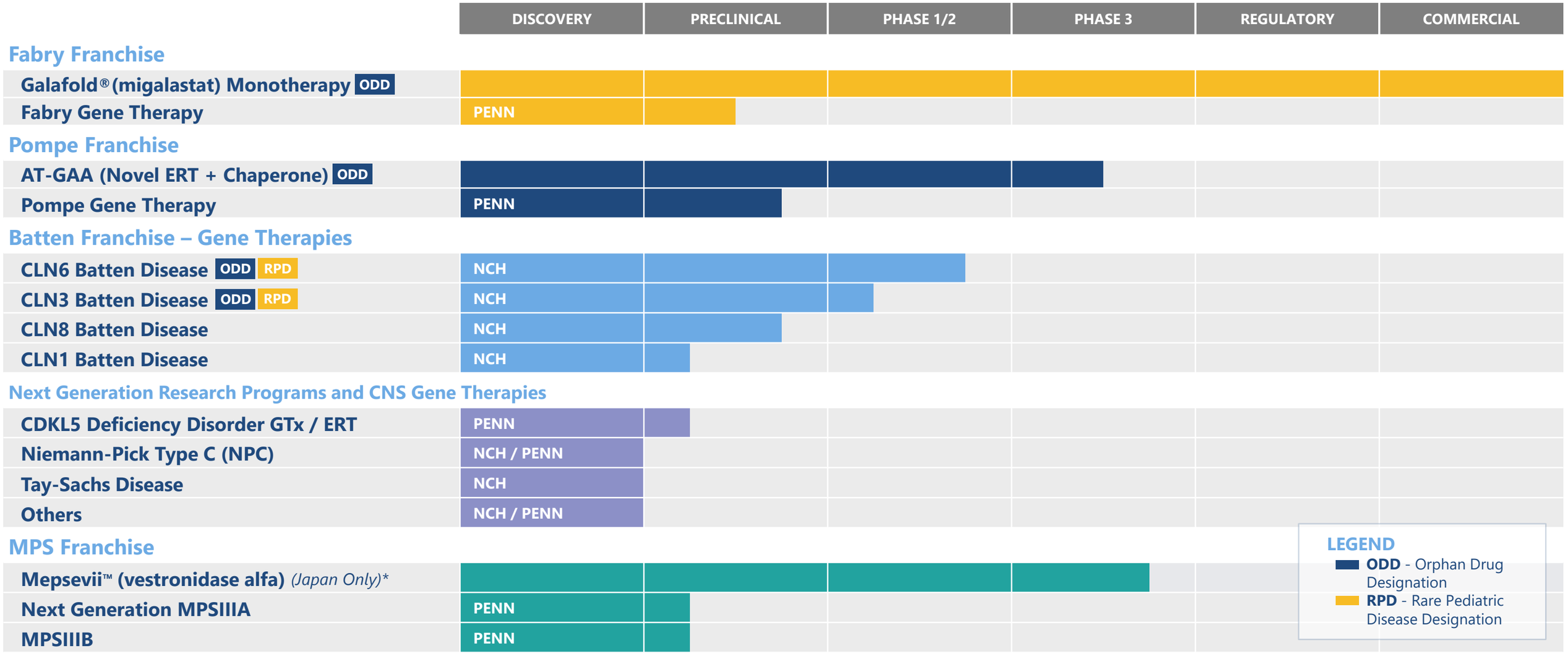
$1B-2B+ Opportunity

Gene Therapy Portfolio

$1B+ Opportunity

To Transform the Lives of Thousands of Patients

A RARE PORTFOLIO



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

Amicus Therapeutics

2019 Key Strategic Priorities

1. **Nearly double annual revenue for Galafold (guidance $160M-$180M)** ✓
2. **Complete enrollment in AT-GAA Pivotal Study (PROPEL) and report additional Phase 2 data** ✓
3. **Report additional 2-year clinical results in CLN6 Batten disease and substantially complete enrollment in ongoing CLN3 Phase 1/2 study** ✓
4. **Establish preclinical proof of concept for Fabry and Pompe gene therapies** ✓
5. **Maintain strong financial position** ✓

2020 Key Strategic Priorities

1. **Achieve global product revenue for Galafold of $250M-$260M**
2. **Complete Pompe Phase 3 PROPEL study, enroll pediatric studies and advance manufacturing to support 2021 BLA and MAA**
3. **Advance clinical development, manufacturing and regulatory discussions for CLN6 and CLN3 Batten programs**
4. **Progress Pompe gene therapy towards IND and disclose up to two additional IND candidates**
5. **Maintain strong financial position**

Our Passion for Making a Difference Unites Us

Amicus is now poised to create significant shareholder value while advancing our mission for patients







Amicus Therapeutics

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

Strong Financial Outlook with Current Cash Well into 2022

Fabry Disease Overview

“We support the disease communities – and their families”

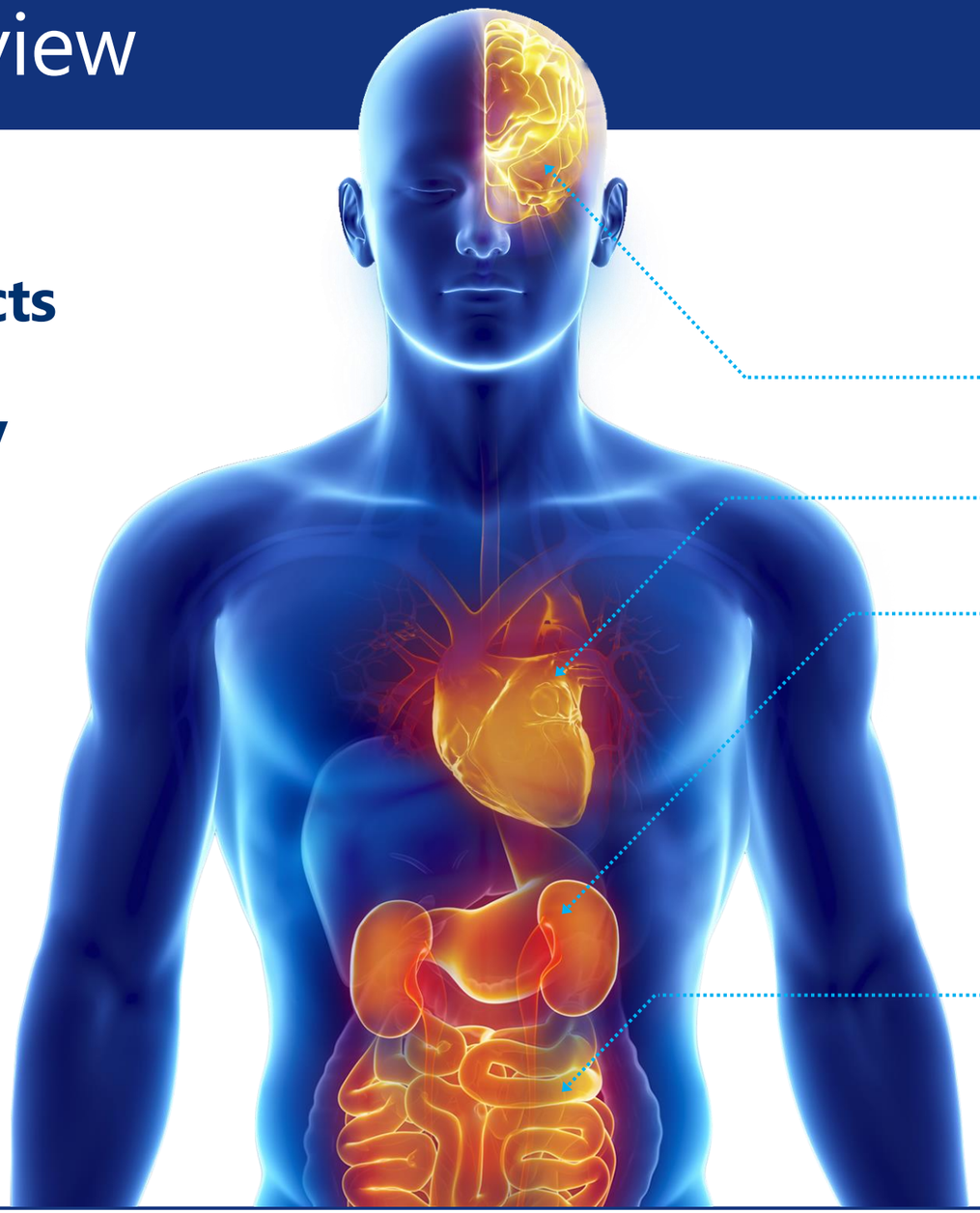
- Amicus Belief Statement

Fabry Disease Overview

Fabry Disease is a Fatal Genetic Disorder that Affects Multiple Organs and is Believed to be Significantly Underdiagnosed

Key Facts:

- α-Gal A enzyme deficiency leads to substrate (GL-3) accumulation
- >1,000 known mutations
- ~10K diagnosed WW (51% female/49% male[4])
- Newborn screening studies suggest 5-10 fold greater incidence (~1:1000 - 1:4000)



Leading Causes of Death:

- Transient Ischemic Attack (TIA) & Stroke[1]
- Heart Disease[2]
- Kidney Disease[3]

Life-Limiting Symptoms:

- Gastrointestinal[3]

1. Desnick R, *et al.* Ann Intern Med. 2003; 2. Yousef Z, *et al.* Eur Heart J. 2013; 3. Germain D. Orphanet J Rare Dis.; 2010 4. Fabry Registry 2011

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 December 31, 2019)

Galafold is the cornerstone of Amicus' success. It 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 enzyme replacement therapy

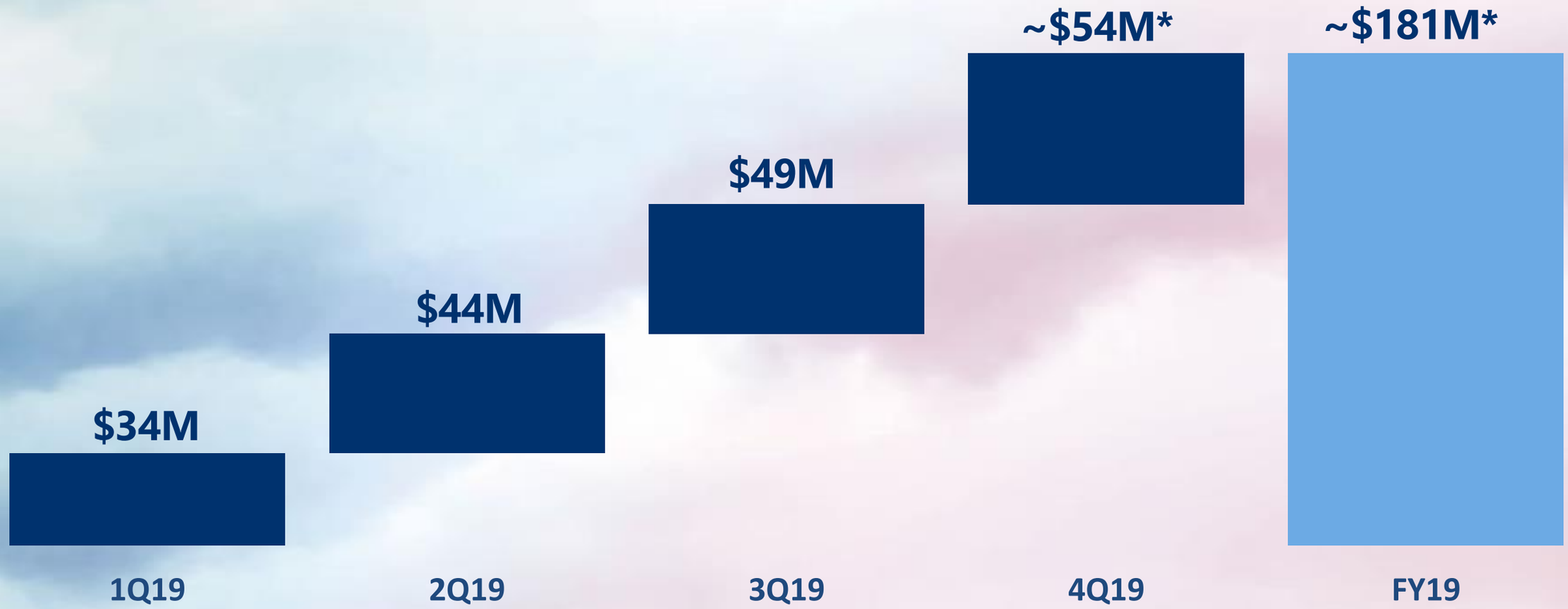
One of the Most Successful Rare Disease Launches

- **~$181M** FY19 Galafold Revenue (preliminary unaudited)
- **$250-260M** FY20 Global Galafold Rev. Guidance
- **Geographic Expansion in 2019** Including Argentina, Brazil, Colombia, Taiwan
- **348** Amenable Variants in U.S. Label
- **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.

Galafold Success and FY19 Galafold Revenue Guidance

Strong full-year performance of ~$181M (preliminary/unaudited) revenue, exceeding guidance of $170-$180M



*Preliminary and unaudited

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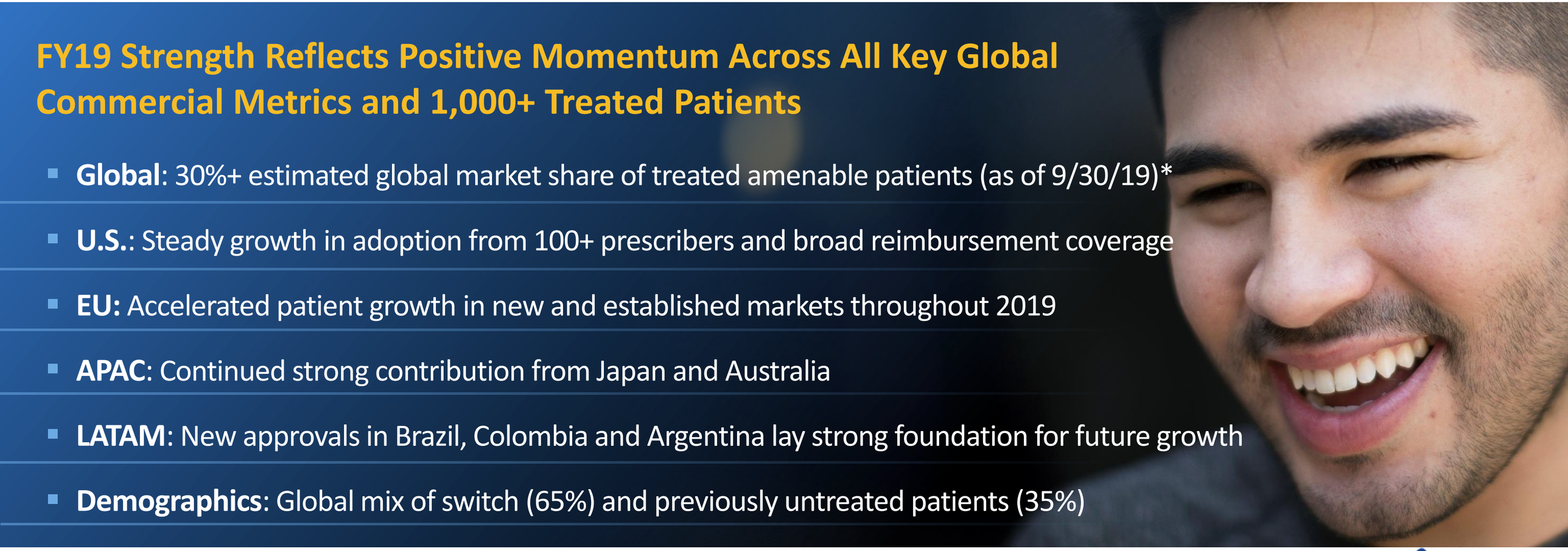
Galafold Global Launch Momentum (as of December 31, 2019)

Global commercial metrics continue to be very strong with >90% compliance and adherence, 30% global market share of treated amenable patients and continued broad market access

FY19 Strength Reflects Positive Momentum Across All Key Global Commercial Metrics and 1,000+ Treated Patients

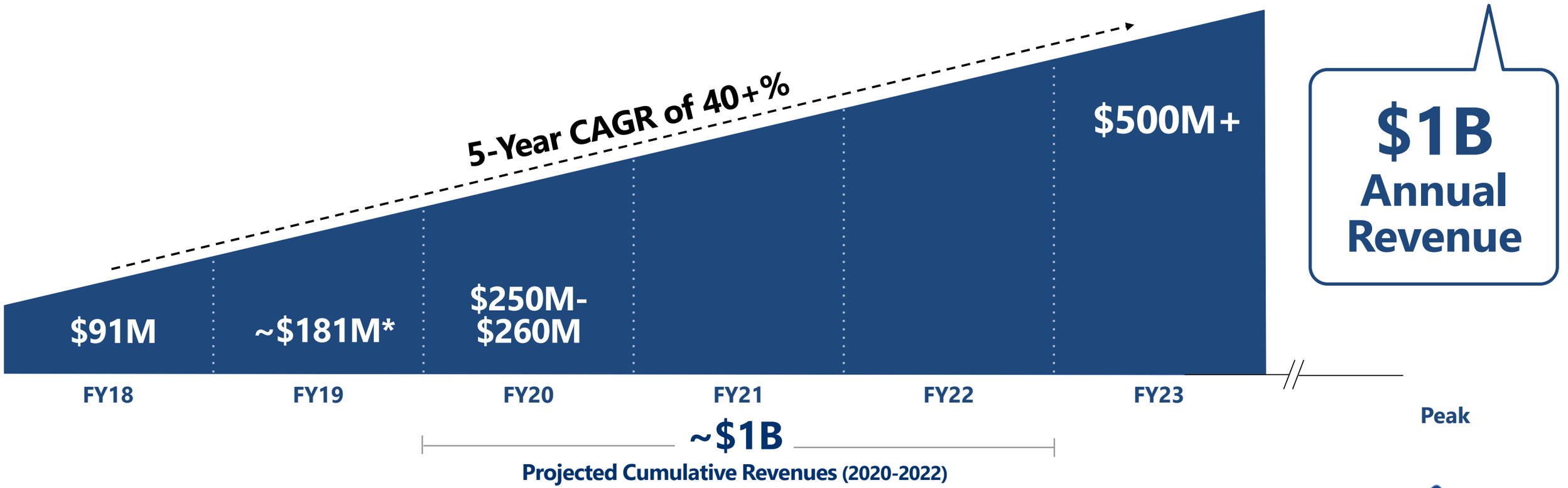
- **Global**: 30%+ estimated global market share of treated amenable patients (as of 9/30/19)*
- **U.S.**: Steady growth in adoption from 100+ prescribers and broad reimbursement coverage
- **EU:** Accelerated patient growth in new and established markets throughout 2019
- **APAC**: Continued strong contribution from Japan and Australia
- **LATAM**: New approvals in Brazil, Colombia and Argentina lay strong foundation for future growth
- **Demographics**: Global mix of switch (65%) and previously untreated patients (35%)



*Market share based on reported global Fabry sales for the calendar year ending 3Q19 and assumes a 35% amenability rate.

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

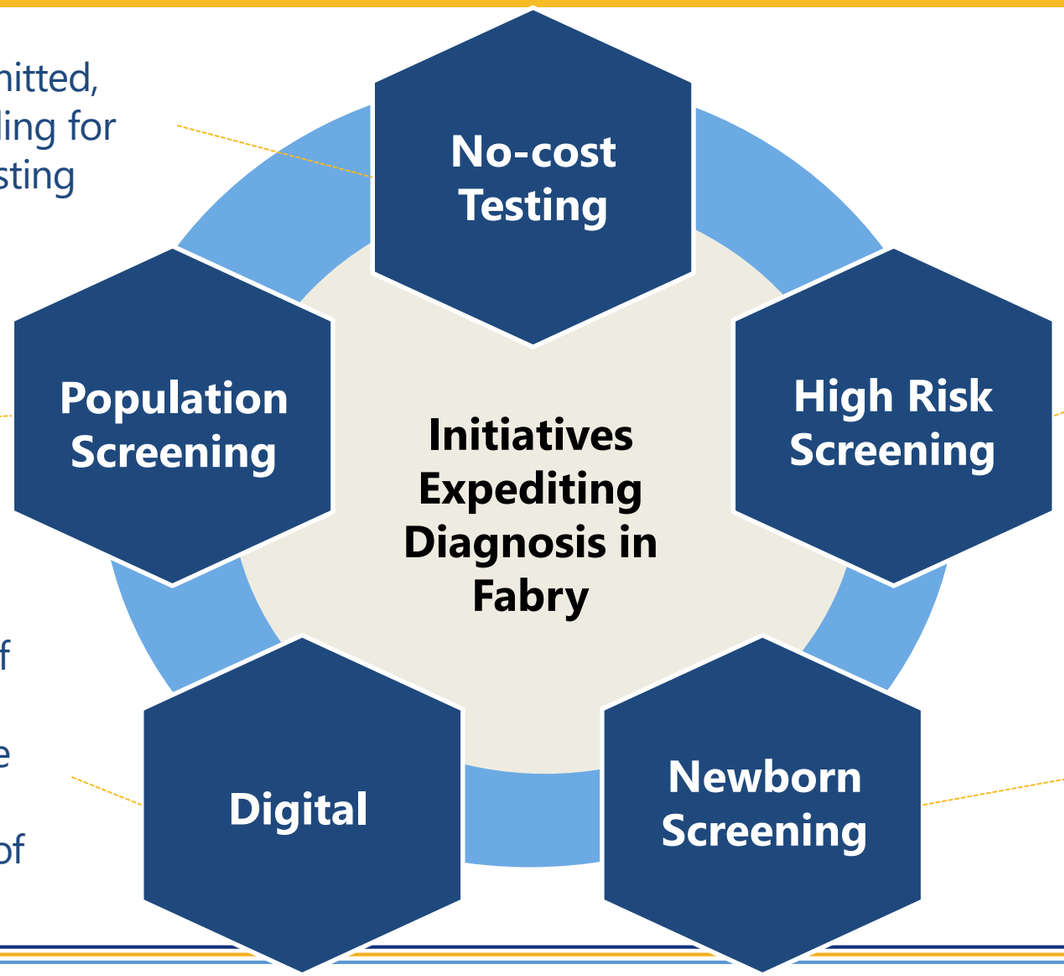


*Preliminary and unaudited

Amicus Therapeutics

Fabry Disease Diagnostic and Growth Drivers

Fabry disease is both underdiagnosed and misdiagnosed. Expanded screening initiatives have the potential to drive a shorter pathway to correct diagnosis for individuals living with Fabry disease



Where permitted, provide funding for genetic testing

Exploring partnerships with genetic testing companies

Algorithm analysis of claims/EHR data underway in multiple geographies across common symptoms of Fabry

Efforts underway in areas where patients may be misdiagnosed or do not know the cause of their symptoms (e.g. pain and MS clinics)

Amicus held NBS Summit with world experts to discuss strategies to expand new born screening

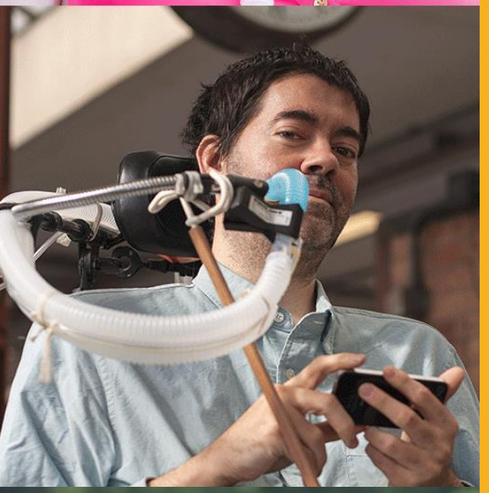
Amicus Therapeutics

Galafold Opportunity

With inherent Fabry market growth and our work to improve diagnosis and screening, Galafold has the potential to drive $1B+ annual revenue at peak.

*Preliminary and unaudited

Amicus Therapeutics



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 muscular dystrophy 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

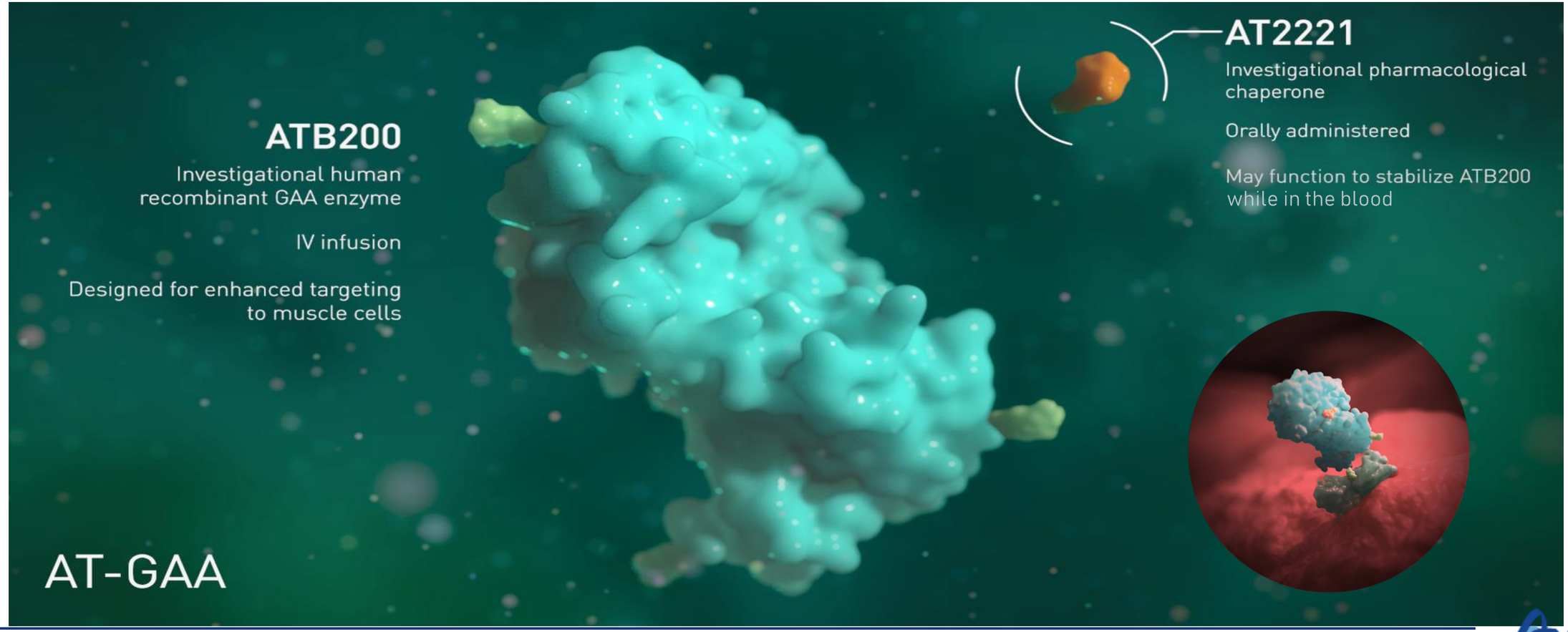
Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

~$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 specializing in protein engineering and glycobiology created a uniquely glycosylated and highly phosphorylated ERT (AT-GAA) that significantly enhances targeting to key muscles affected in patients



U.S. FDA Granted BTD to AT-GAA in Late-Onset Pompe Disease (LOPD)

AT-GAA is the first ever second-generation product for <u>any</u> lysosomal disorder to earn FDA Breakthrough Therapy Designation (BTD)

Plans to apply for and initiate a rolling BLA submission for AT-GAA in LOPD in 2020

AT-GAA BTD Based on Ph 1/2 Clinical Efficacy

- Improvements in 6-minute walk distance
- Comparison to natural history of treated patients

BTD Features

- Intensive guidance on an efficient drug development program
- Organizational commitment involving senior agency staff
- All Fast Track program features including rolling submission

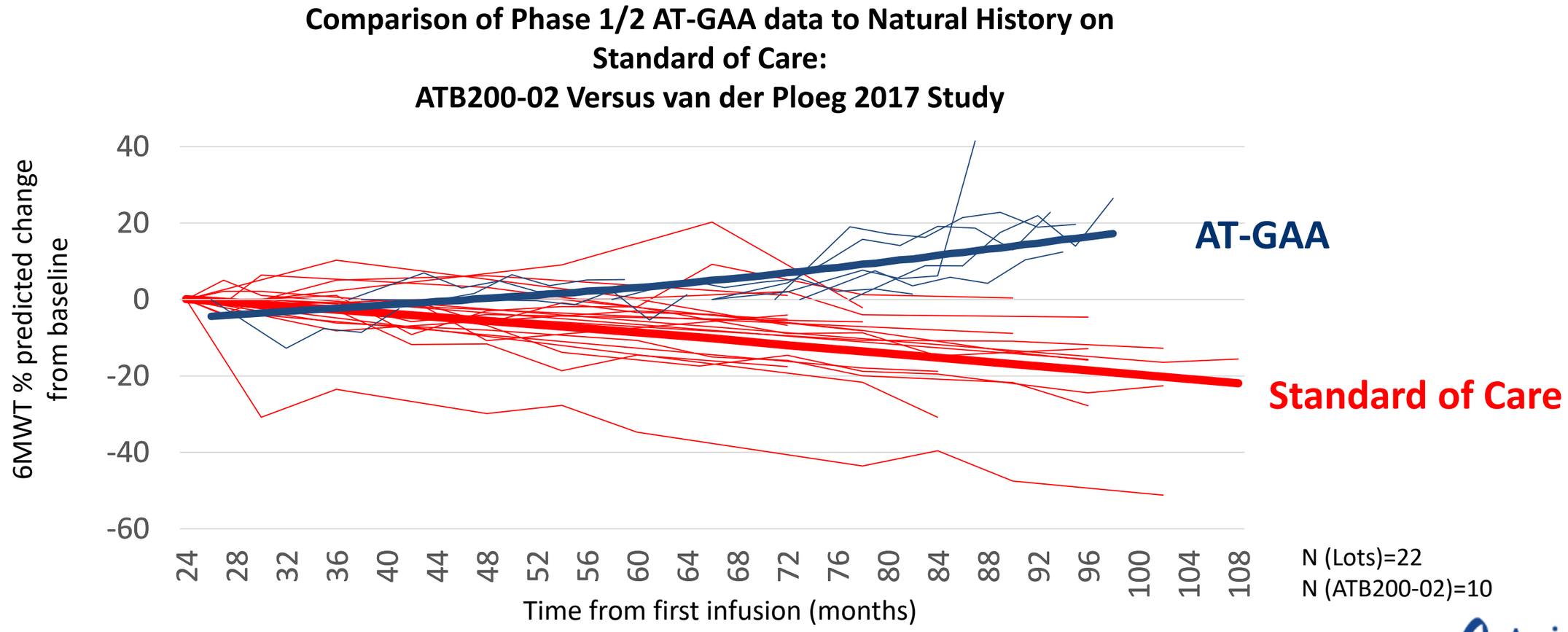
BTD Criteria

- Intended to treat a serious or life-threatening disease or condition
- Preliminary clinical evidence indicates drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints

6MWT Natural History: Phase 1/2 AT-GAA Data vs. Medical Literature

van der Ploeg 2017

Improvement in percentage predicted 6MWD seen in all patients who switched from alglucosidase alfa to AT-GAA



1. Data for AT-GAA represent time from first infusion of SOC ERT and change from baseline at the time of switching from SoC to AT-GAA
2. Source: ATB200-02 IA#7; Ans T. van der Ploeg et al. Poster presented at the 13th Annual WORLD Symposium™ 2017, February 13–17, 2017, San Diego, CA, USA

Amicus Therapeutics

PROPEL (ATB200-03) Study Design

Phase 3 exceeded enrollment with data expected in 1H2021. The study is highly powered for success and supports a broad label, with FDA and EMA agreement on study design and primary endpoint (6MWT)

52-Week Primary Treatment Period (Double-Blind)

2:1 Randomization

Participants with Late-Onset Pompe Disease

n=123
59 WW Clinical Sites

AT-GAA
Bi-Weekly

Standard of Care
Bi-Weekly

Long-Term Extension (Open-Label)

Primary Endpoint: 6-Minute Walk Test at Week 52; Multiple Secondary Endpoints

- **PROPEL pivotal study over-enrolled with data expected in 1H2021**
- **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**

Pompe Biologics Manufacturing

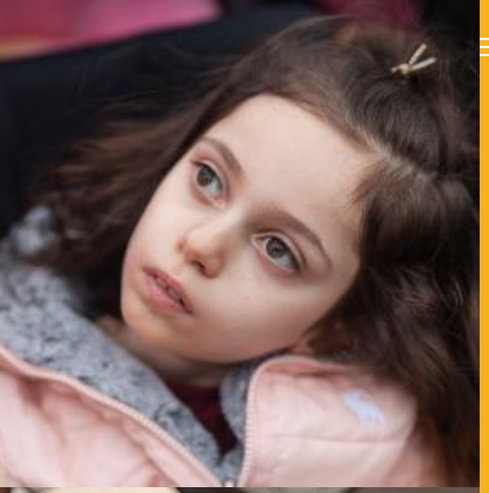
Amicus and partner WuXi Biologics have successfully produced AT-GAA at 1,000L commercial scale, demonstrating unique capabilities in Amicus biologics process science, manufacturing and quality control

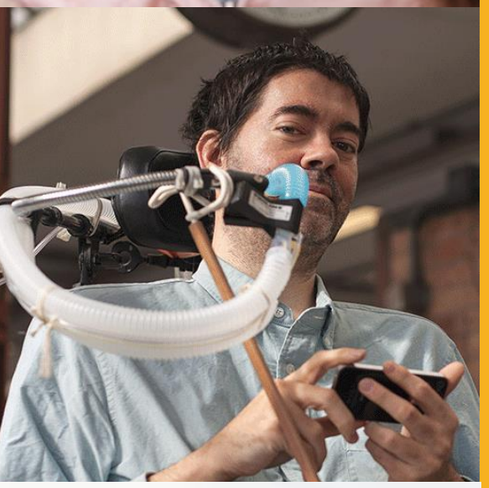
- **Manufacturing PPQ runs at WuXi biologics are underway**
- **Agreements on biocomparability between 250L and 1,000L scale with key regulators (FDA, BfARM)**
- **All PROPEL participants treated with drug manufactured at 1,000L**
- **Current bioreactor capacity to supply global population**

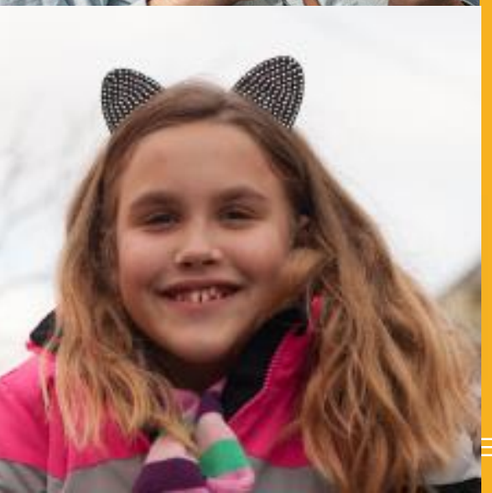
AT-GAA: Key Takeaways

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

- PROPEL pivotal study exceeded enrollment with data expected 1H2021
- Breakthrough Therapy Designation and the Promising Innovative Medicine designation highlight unmet need in Pompe disease today
- Plan to submit and initiate rolling submission of Biologics License Application in 2020
- Manufacturing PPQ runs at WuXi biologics on track
- 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

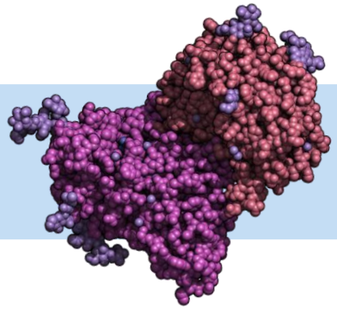
A Natural Evolution: Chaperones to Optimized ERT to Gene Therapy

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

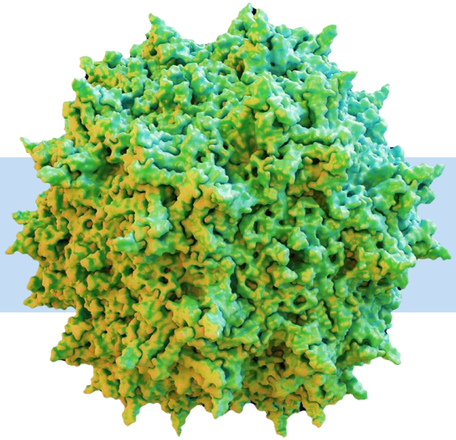
Stabilize
"naturally produced" enzymes

Next-Generation ERTs



Stabilize and target
"externally produced" enzymes

Gene Therapies



Stabilize and target
"internally produced" enzymes

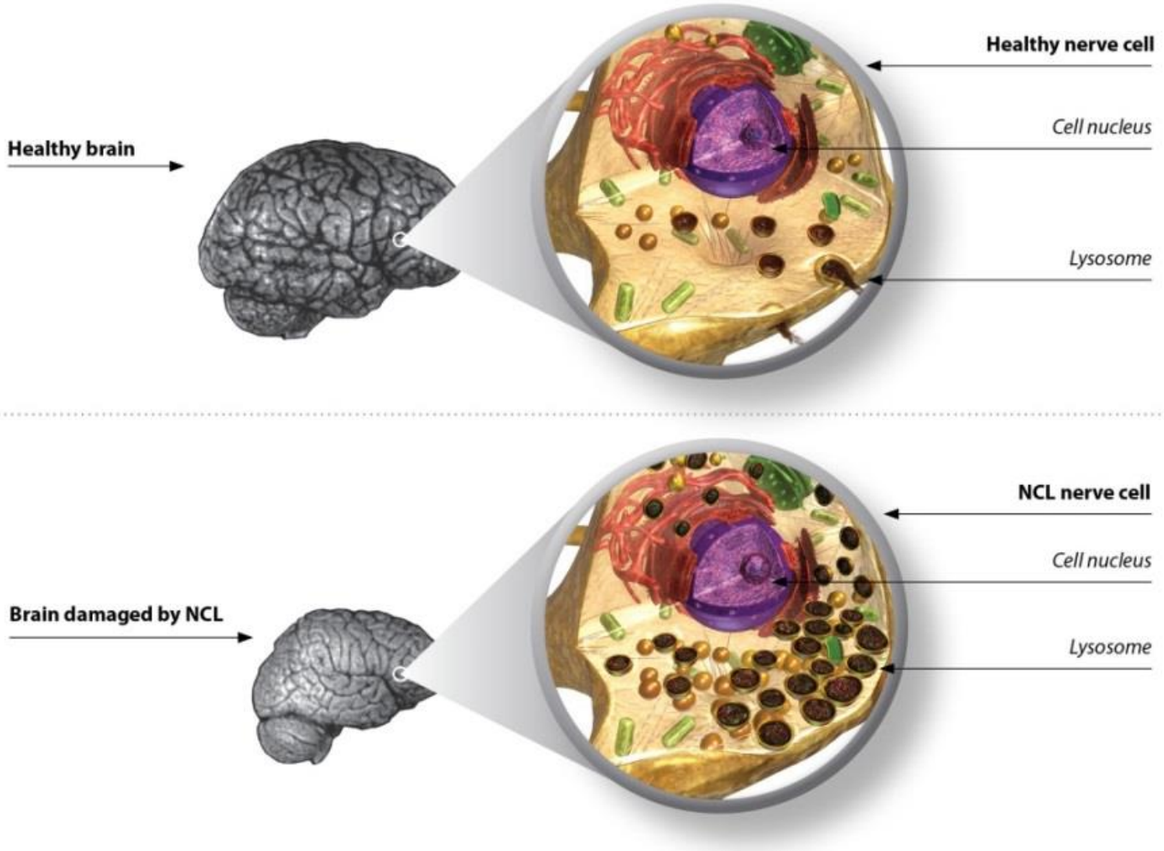
Amicus Therapeutics

Batten Disease Overview

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

Disease Overview

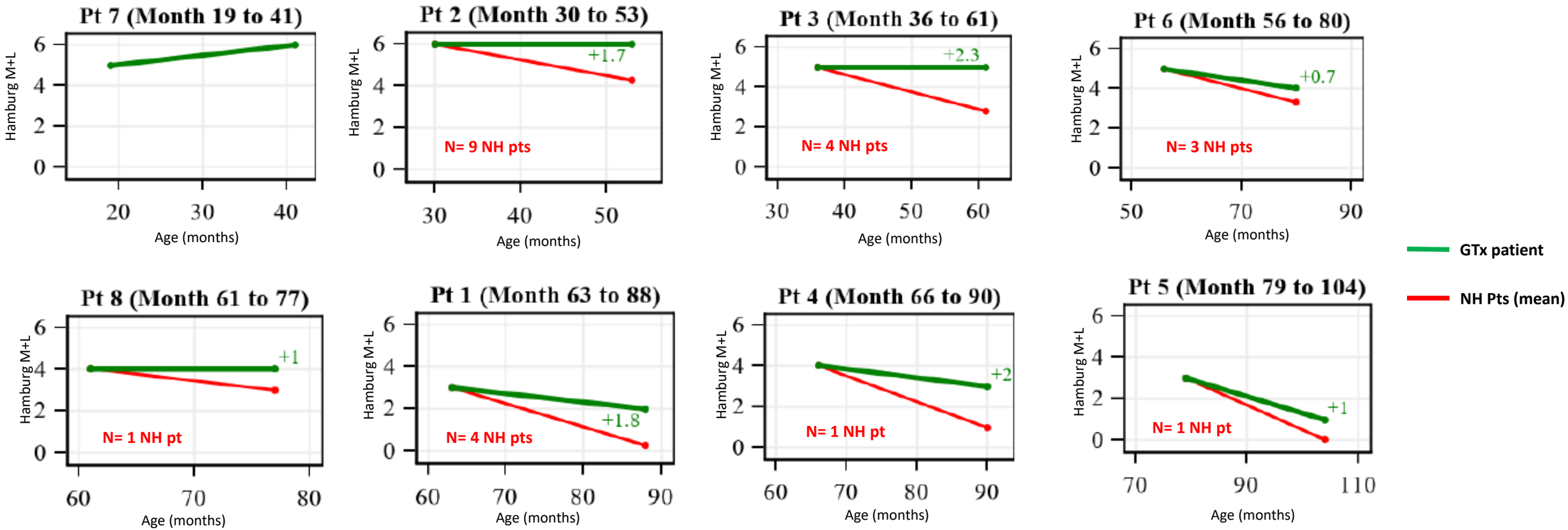
- A group of disorders known as neuronal ceroid lipofuscinoses (NCLs), collectively referred to as Batten disease
- Mutation in one of 13 different CLN genes leads to lysosomal dysfunction
- Signs and symptoms include loss of speech, ambulation, vision and cognition



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

CLN6 Clinical Efficacy Data: Natural History Matched Comparisons

Analysis of treated patients demonstrates improvement compared to natural history patients matched for age and baseline Hamburg M+L score*

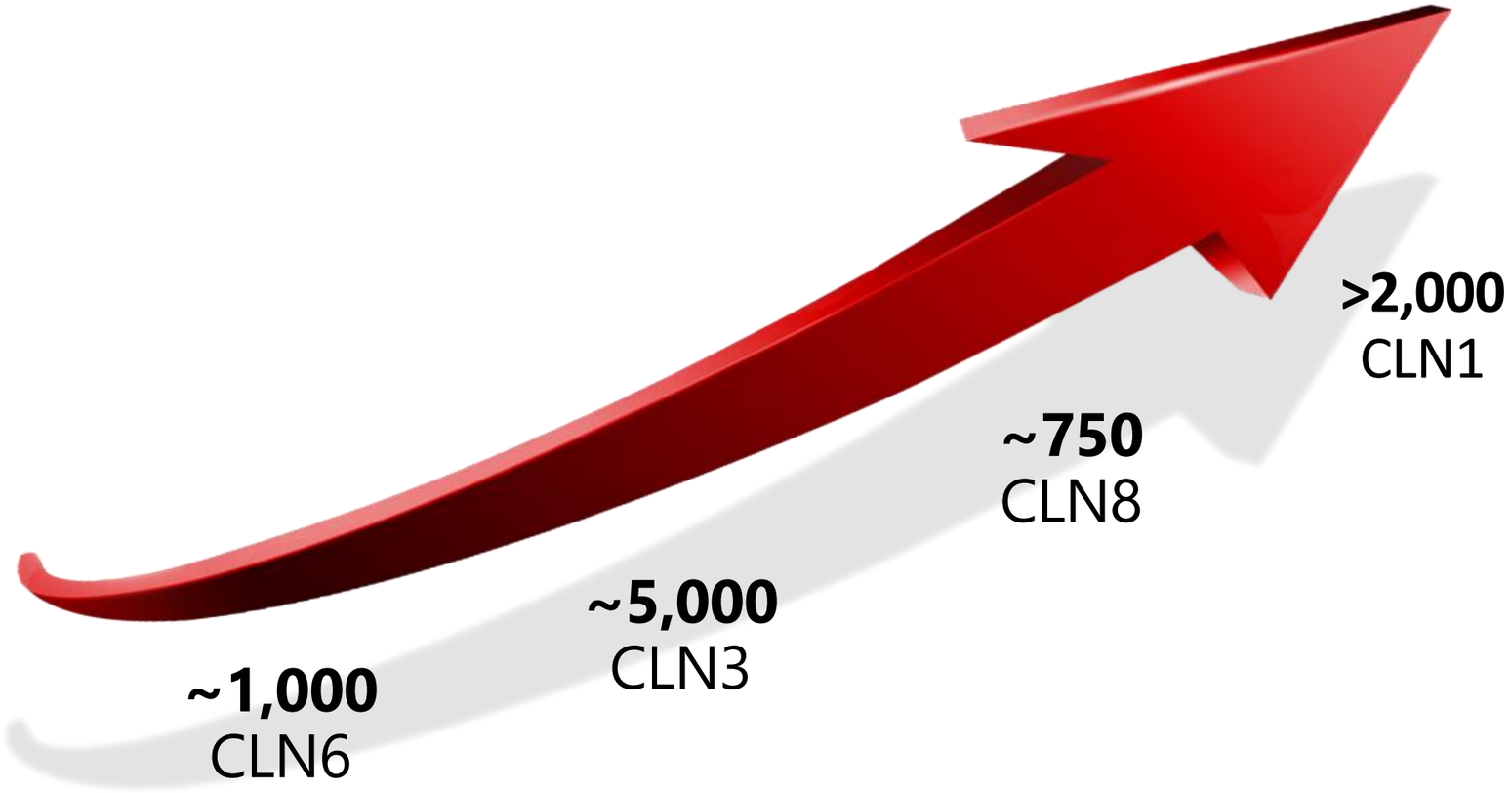


*Matched for age and exact baseline Hamburg score. No current match (for age and exact M+L score at baseline) for youngest patient (pt. 7)

Source: Data on file

Batten Disease Franchise

CLN6 results validate the broad potential of the intrathecal AAV platform to build a valuable and significant franchise to save thousands of children suffering from multiple types of Batten diseases with potential for $1B+ in recurring peak revenue



*Estimated addressable U.S., EU, Japan, and other major, reimbursable markets based on published incidence and prevalence

Combines Amicus and Penn Expertise Across Lysosomal and Rare Diseases

Combines Amicus expertise in protein engineering with Penn expertise in AAV vectors, manufacturing and immunology to improve safety, efficacy and speed development

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

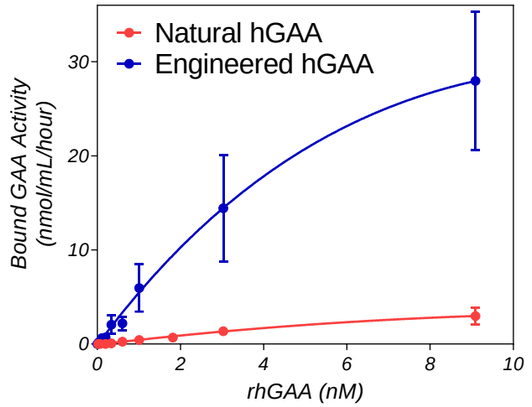
Penn
University of Pennsylvania

Driving 1-2 new INDs every year starting in 2020

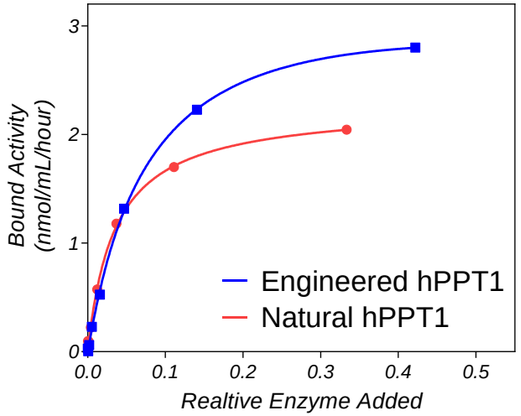
Protein Engineering Platform Has Potential To Be Broadly Applicable to Gene Therapies For Majority of LDs

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

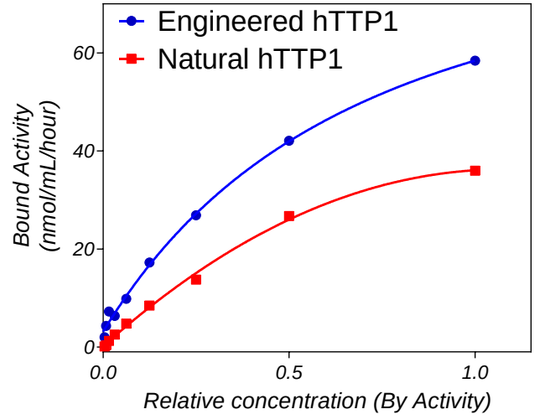
Pompe - GAA



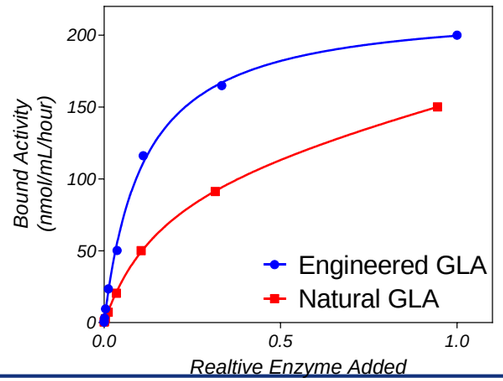
CLN1 – PPT1



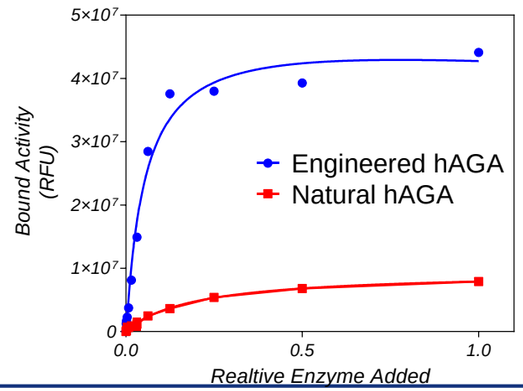
CLN2 – TTP1



Fabry – GLA



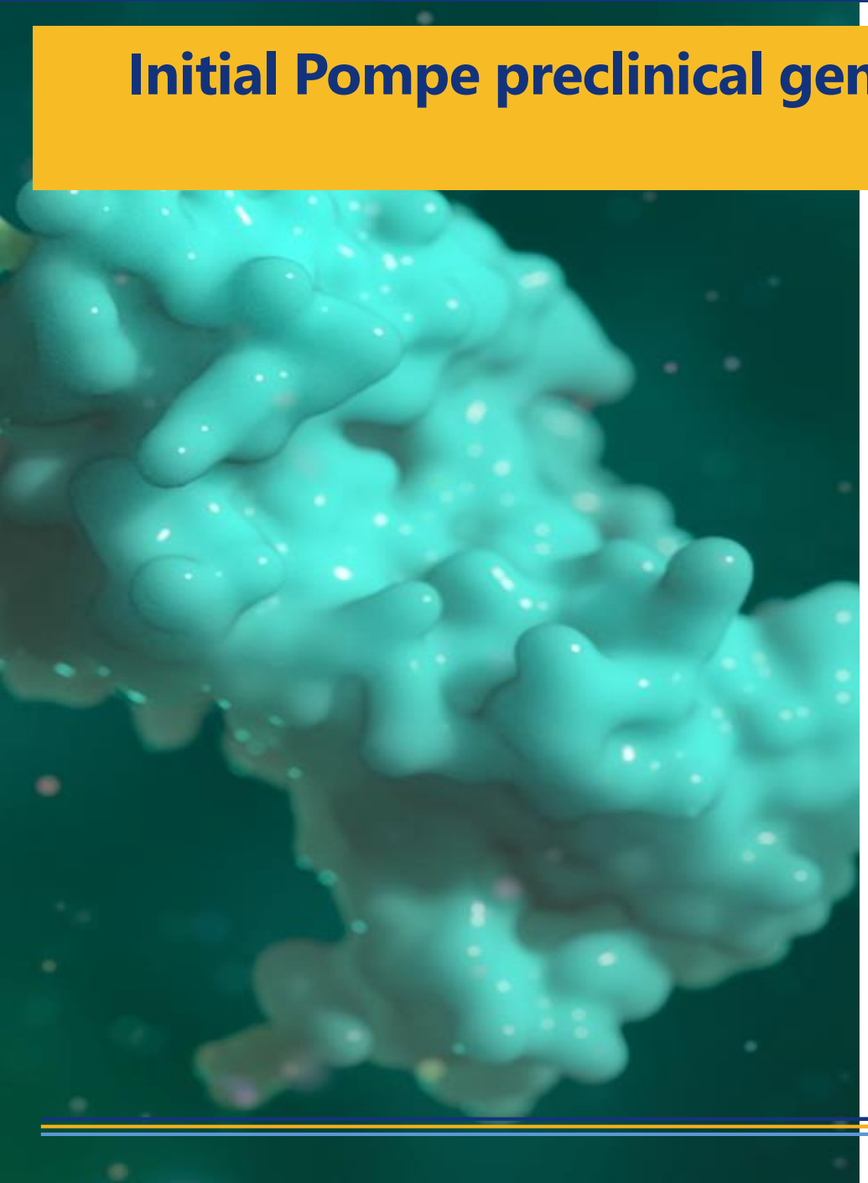
AGU – AGA



Source: Data on file

Pompe Gene Therapy Summary and Next Steps

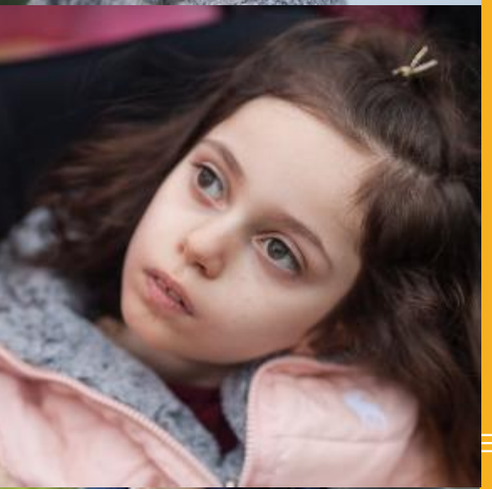
Initial Pompe preclinical gene therapy data demonstrate differentiated profile and clear pathway toward the clinic



- Clinical candidate selected
- Toxicology batch manufacturing and GMP tech transfer to Paragon underway
- IND enabling toxicology work in progress
- Potential to enter clinic in 1H2021

Gene Therapy: Key Takeaways

- CLN6 data show profound impact as compared to natural history matched for age and baseline. Potential to become first ever approved gene therapy for fatal brain disease in children
- AAV intrathecal platform increasingly gives confidence in CLN3 program (largest cause of childhood neurodegeneration, 5,000+ children)
- Penn Collaboration R&D engine, with rights to 50+ diseases and access to world class gene therapy technology and expertise
- 2 clinical and 8 preclinical gene therapies in development and one clinical candidate now generated (Pompe)



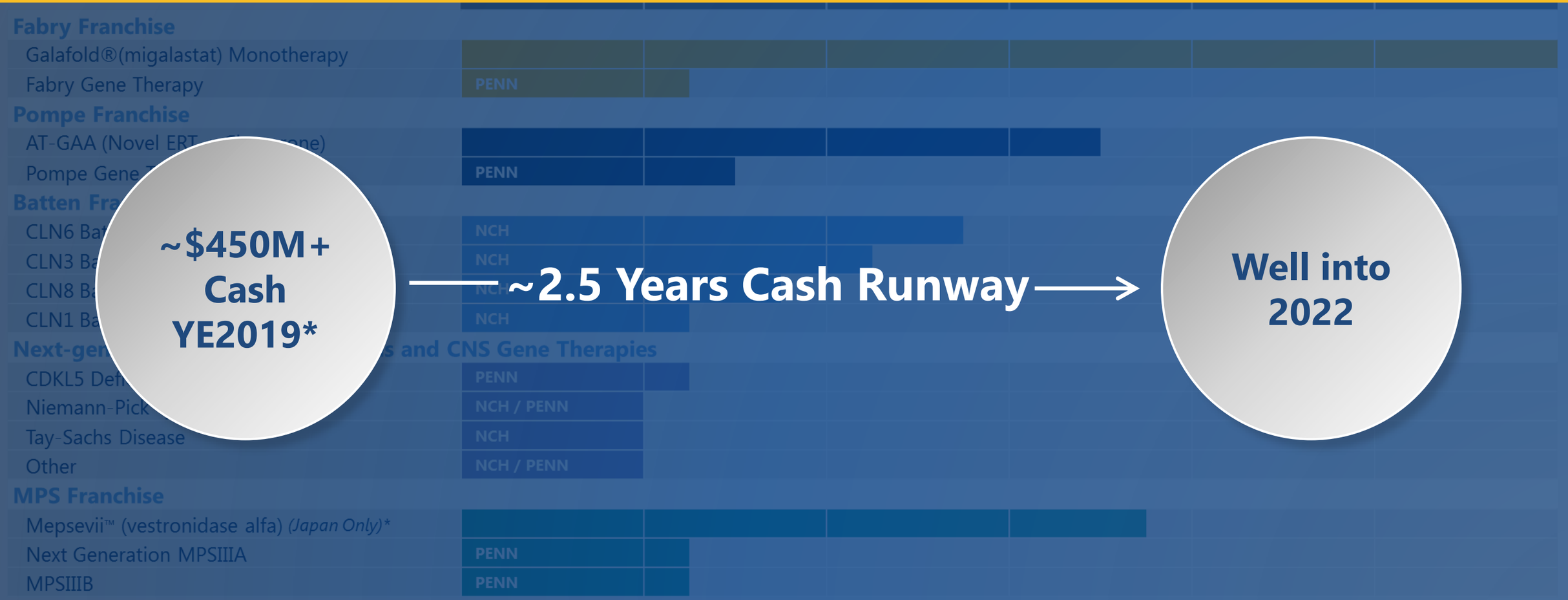
Financial Summary & Milestones

“We are business led and science driven”

- Amicus Belief Statement

Cash Runway Now to Well into 2022 (~2.5 years)

Fully funded through major milestones in portfolio and continued global growth



*Preliminary and unaudited

Financial Outlook: Key Takeaways

- Company now 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
- Extended cash flow runway through OpEx savings, CapEx phasing, program prioritization and increased Galafold revenue projections
- No material business development planned or needed in next several years
- Only modest additional capital required in the outer years to extend runway into profitability with multiple non-equity sources available as/when needed

At Major Inflection Point: Path to Profitability

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 and Pompe gene therapies into and through the clinic
- Generate 1-2 gene therapy INDs every year starting in 2021
- Discover and develop next generation protein engineering and gene therapy technologies with Penn

Only modest additional capital required in outer years to extend runway into profitability with multiple non-equity sources available as/when needed

Thank You

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Appendix

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Financial Summary & Guidance

Strong Balance Sheet with ~$450M+ Cash – Cash Runway Well into 2022

FINANCIAL POSITION	
Cash[1]	~$450M+
Cash Runway[2]	Well Into 2022
Debt[1,3]	$152.8M
CAPITALIZATION	
Shares Outstanding (as of 12/31/2019)	255,417,869
FINANCIAL GUIDANCE	
FY20 Galafold Revenue Guidance	$250M-$260M
FY20 Non-GAAP Operating Expense Guidance	$410M-$420M

[1] Preliminary and unaudited [2]Based on existing operating plan [3]Includes $2.8 million of convertible debt and $150 million of straight debt

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