Amicus Therapeutics®

Jefferies 2019 Healthcare Conference

Daphne Quimi, Chief Financial Officer

June 7, 2019

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 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 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. 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, 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 news release to reflect events or circumstances after the date hereof.

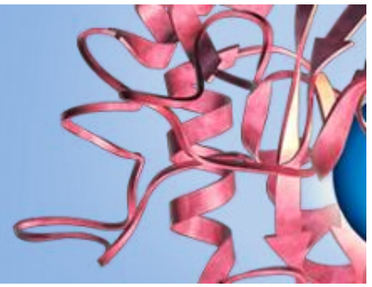
A RARE COMPANY.

Galafold™ (migalastat)

First Oral Precision Medicine for Fabry Disease

Gene Therapy PLATFORM

Protein Engineering & Glycobiology

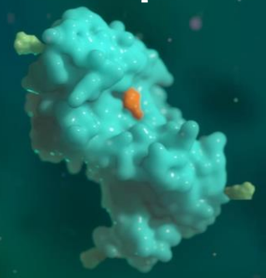


$400M+ Cash (3/31/19)

Global Drug Development

AT-GAA*

Phase 3 Investigational Therapy for Pompe

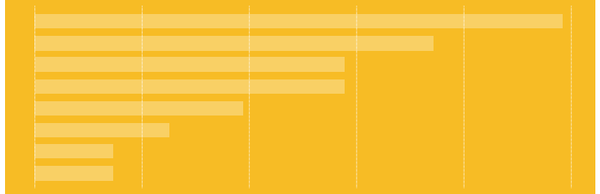


600+ EMPLOYEES in 27 Countries

GLOBAL COMMERCIAL FOOTPRINT

PORTFOLIO

for rare diseases



Robust R&D Engine

Nearly 50+ Lysosomal Disorders and More Prevalent Rare Diseases

* AT-GAA, also known as ATB200/AT2221

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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 1/2 data**
3. **Report additional 2-year clinical results in CLN6-Batten disease and complete enrollment in ongoing CLN3-Batten disease Phase 1/2 study**
4. **Establish preclinical proof of concept for Fabry and Pompe gene therapies**
5. **Maintain strong financial position**

Rare Disease Operational Excellence

Proven Capabilities Position Amicus at the Forefront of Human Genomic Medicine to Develop and Deliver the Highest Quality Therapies and Cures for Persons Living with Rare Diseases

5 Key Pillars of Value

Innovation/ Science

Global Development

Global Commercial

Manufacturing

Partnerships

A RARE PORTFOLIO.

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3	REGULATORY	COMMERCIAL
Fabry Franchise						
Galafold® (migalastat) monotherapy						
Fabry Gene Therapy	PENN					
Pompe Franchise						
AT-GAA (Novel ERT + Chaperone)						
Pompe Gene Therapy	PENN					
Batten Franchise – Gene Therapies						
CLN6 Batten Disease	NCH					
CLN3 Batten Disease	NCH					
CLN8 Batten Disease	NCH					
CLN1 Batten Disease	NCH					
Other Rare CNS Gene Therapies						
CDKL5 Deficiency Disorder GTx / ERT	PENN					
Niemann-Pick Type C (NPC)	NCH / PENN					
Tay-Sachs Disease	NCH					
Other	NCH / PENN					
MPS Franchise						
Next Generation MPSIIIA	PENN					
MPSIIIB	PENN					
Next Generation Research Program						

Advancing one of the **most robust rare disease portfolios** in biotechnology

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 March 31, 2019)

One of the Most Successful Rare Disease Launches

$34.0M
1Q19 Galafold Revenue

$160-180M
FY19 Global Galafold Rev. Guidance

Geographic Expansion in 2019

Galafold™ (migalastat)

24
Countries with Pricing & Reimbursement

8
Regulatory Approvals:
Australia, Canada, EU, Israel, Japan, S. Korea, Switzerland , U.S.

348
Amenable Variants in U.S. Label

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 Global Launch Momentum (as of March 31, 2019)

Q1 was very strong with largest number of net new patient adds (150+) and positive momentum across all key commercial metrics

- **Global**: 150+ new patient adds with continued >90% compliance and adherence. Now estimate ~18% global market share of treated amenable patients*
- **U.S**: 200+ prescription referral forms (PRFs) from 90+ prescribers (as of April 30); shortening time from PRF to shipment
- **International**: strong growth from both switch and previously untreated patients
- **Japan**: Q1 patients ahead of forecast with expanded commercial team
- **Demographics**: balanced mix of males and females, classic and late-onset patients across all markets

*Market share based on reported 2018 global Fabry sales and assumes a 35% amenability rate for Galafold.

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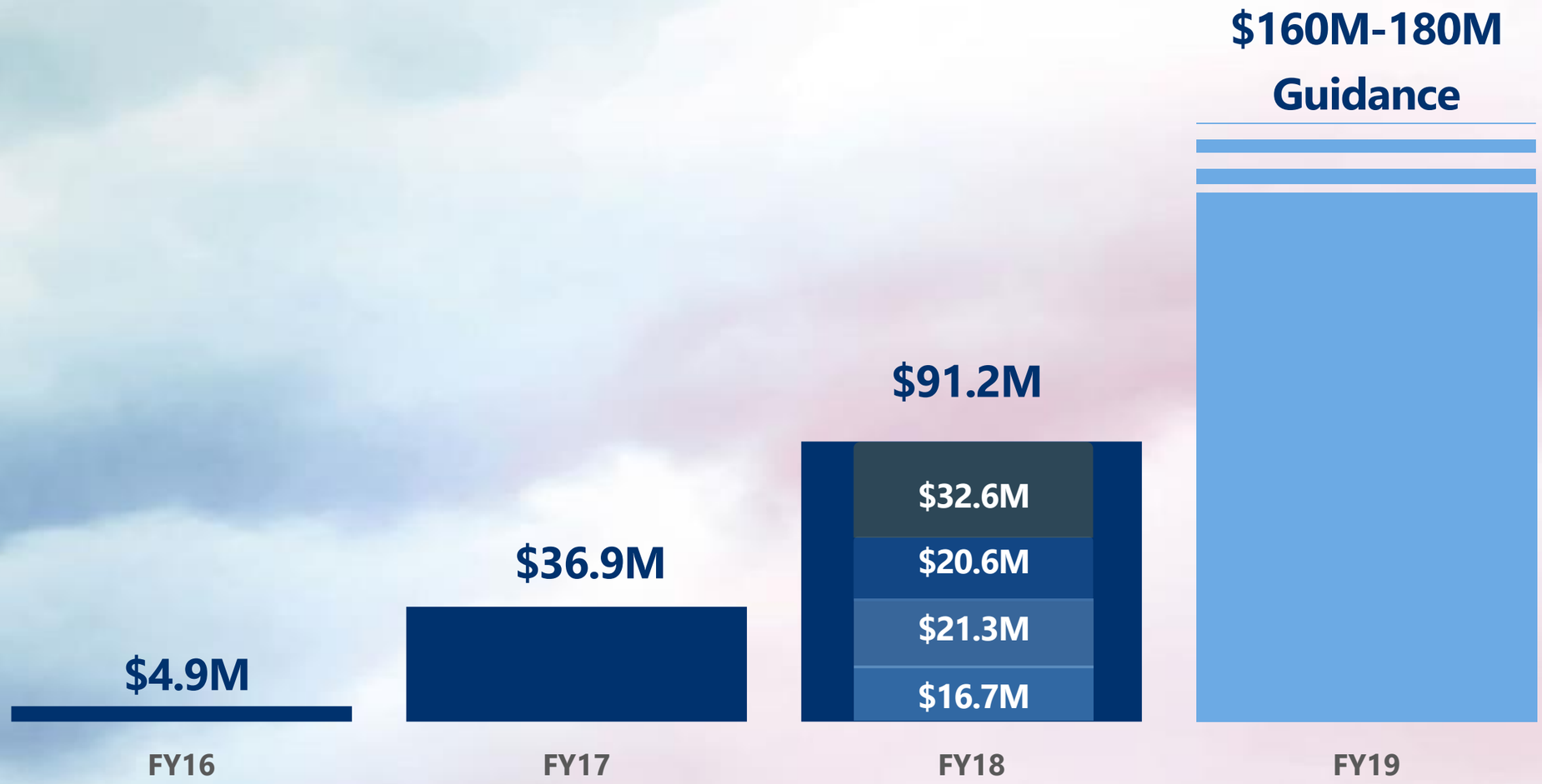
Galafold Success and FY19 Galafold Revenue Guidance

On Track to Nearly DOUBLE Revenue Again and Serve 1,000+ Patients in 2019



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Total Amenable Patient Population ("TAPP")

Estimate based on 35% - 50% amenability

$1B+ Addressable Market Opportunity by 2028

2017

EU & ROW Only

TAPP: 2,000-3,000

2018

Today

WORLDWIDE*
(U.S. & Japan Added)

TAPP: 3,800-5,500

2028

Peak Potential

WORLDWIDE

Diagnosis continues at current rate

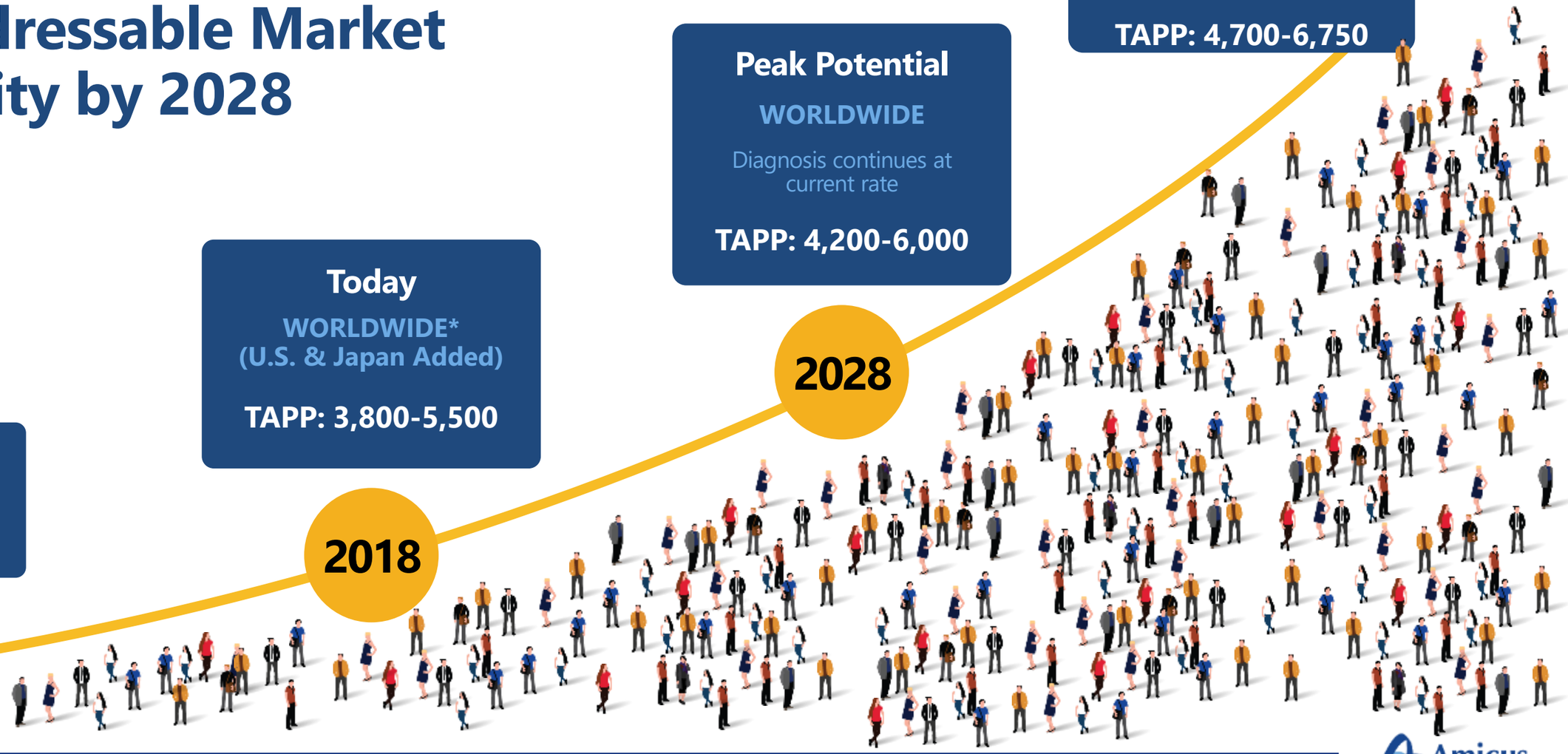
TAPP: 4,200-6,000

Upside Potential

WORLDWIDE

Diagnosis grows due to newborn screening and patient finding initiatives in U.S. & Japan

TAPP: 4,700-6,750



*WORLDWIDE includes total amenable patient population in all Fabry ERT commercial markets today Estimated effect of newborn screening on adult diagnostic rate.

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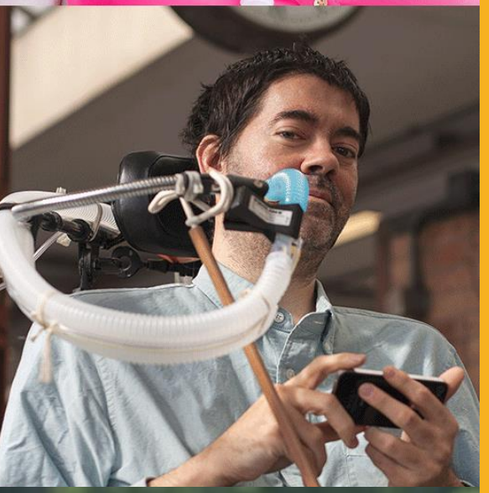


AT-GAA Novel ERT for Pompe Disease

“We encourage and embrace constant innovation”

- Amicus Belief Statement

Pompe Disease Overview

Pompe Disease is a Fatal Neuromuscular and Motorneuron Disorder that Affects a Broad Range of People

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

Respiratory and cardiac failure are leading causes of morbidity and mortality

Age of onset ranges from infancy to adulthood

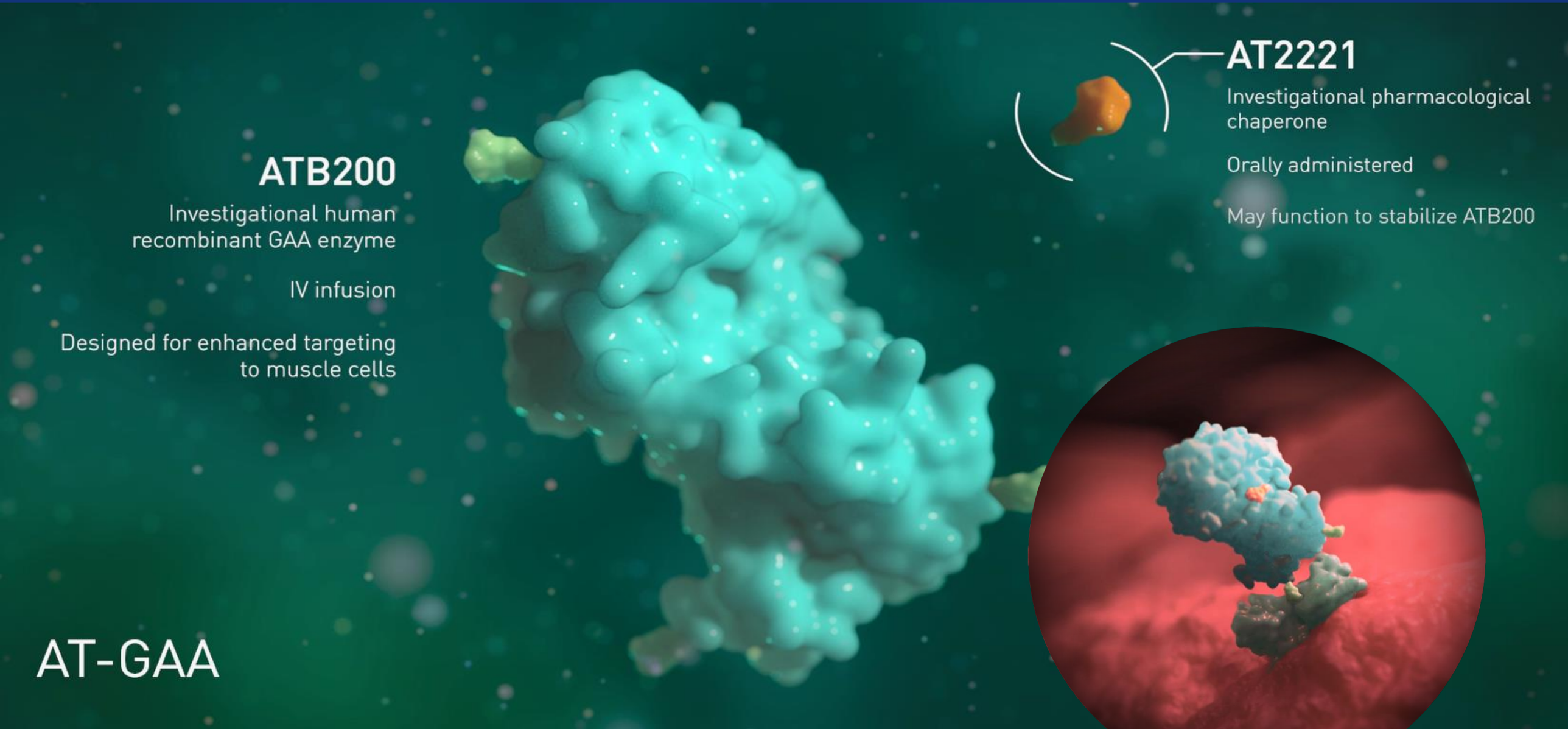
Deficiency of GAA leading to glycogen accumulation and cellular dysfunction

Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

~$900M+ Global Pompe ERT sales in FY18[2]

1. National Institute of Neurological Disorders and Stroke (NIH). 2. Sanofi Press Release & 10-K

AT-GAA (ATB200 + Chaperone): A Differentiated Treatment Paradigm



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

Consistent and Durable Responses Across Key Measures of Safety, Functional Outcomes and Biomarkers in both ERT-Switch and ERT-Naïve Pompe Patients out to Month 24

6-Minute Walk Test (m)

Cohort	Baseline (n=10)	Change at Month 24[a,b] (n=8) Mean (SD)
Cohort 1 ERT-Switch Ambulatory	**397.2** (96.8)	**+53.6** (36.4)
Cohort	**Baseline (n=5)**	**Change at Month 21 (n=5) Mean (SD)**
Cohort 3 ERT-Naïve	**399.5** (83.5)	**+54.8** (34.7)

FVC (% Predicted)

Cohort	Baseline (n=9*)	Change at Month 24[a,b,c] (n=7) Mean (SD)
Cohort 1 ERT-Switch Ambulatory*	**52.6** (14.7)	**-0.6** (2.8)
Cohort	**Baseline (n=5)**	**Change at Month 21 (n=5) Mean (SD)**
Cohort 3 ERT-Naïve	**53.4** (20.3)	**+6.1** (9.7)

[a]One patient in Cohort 1 discontinued from study (withdrew consent) before Month 24. [b]At the time of this interim analysis, 1 patient in Cohort 1 had not reached Month 24. [c]Baseline FVC missing for 1 patient in Cohort 1

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AT-GAA: Breakthrough Therapy Designation

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

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

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 managers
- All Fast Track program features
- Potential Rolling BLA
- Potential for Priority Review

PROPEL (ATB200-03) Study Design

Participants with Late-Onset Pompe Disease

~100 Patients
90 Clinical Sites Worldwide

ERT-Switch
ERT-Naïve

52-Week Primary Treatment Period (Double-Blind)

AT-GAA
Bi-Weekly

Standard of Care
Bi-Weekly

Long-Term Extension (Open-Label)

AT-GAA
Bi-weekly

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

Pompe Biologics Manufacturing

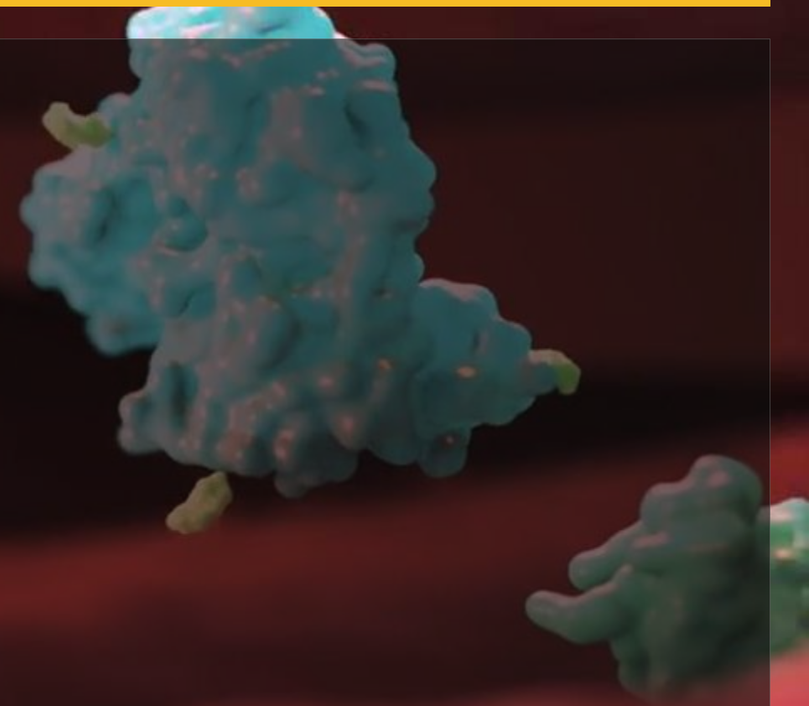
Successful Scale Up to 1000L GMP Clinical and Commercial Scale to Fully Supply Global Pompe Population

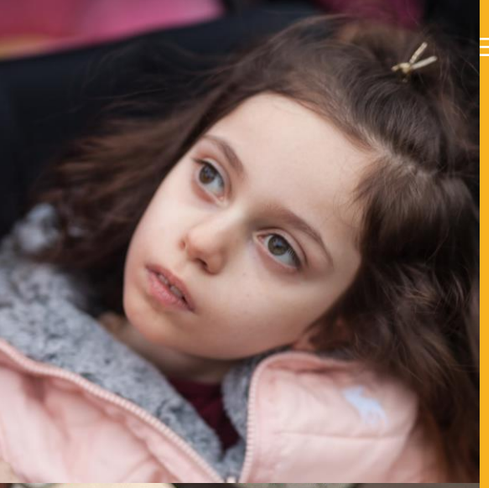
- Key quality attributes maintained from 5L to 250L to 1000L
- Agreements on biocomparability with key regulators (FDA, BfARM)
- PROPEL participants now treated with drug manufactured at 1000L
- Current bioreactor capacity to supply global population
- WuXi partnership strengthened with 5-year supply agreement
- PPQ process underway
- Building commercial supply

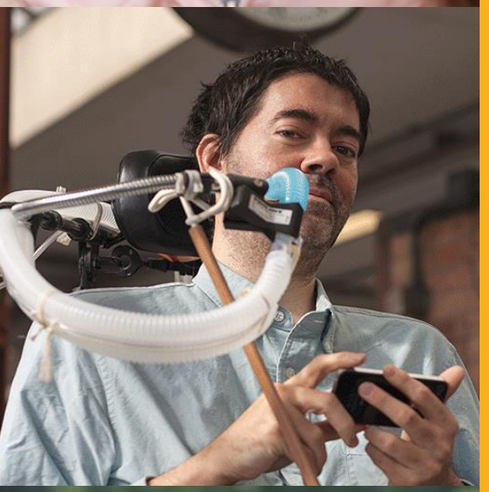
AT-GAA: 2019 Objectives

Advance AT-GAA for as Many Patients Worldwide as Quickly as Possible

- ✓ Additional Phase 1/2 Data (up to 24 Months)
- ✓ Breakthrough Therapy Designation
- ✓ Full enrollment of Phase 1/2 Study (Cohorts 1-4)
- Full enrollment of PROPEL study (n=100)
- Present additional Phase 1/2 data (Cohort 4)
- Report natural history study data
- Initiate supportive pediatric study
- Advance agreed upon CMC requirements to support BLA







Gene Therapy Pipeline

“We have a duty to obsolete our own technologies”

- Amicus Belief Statement

Leading Lysosomal Disorder Gene Therapy Portfolio

Amicus at the Forefront of Human Genomic Medicine with Multiple Platforms and Robust R&D Engine for Future Growth

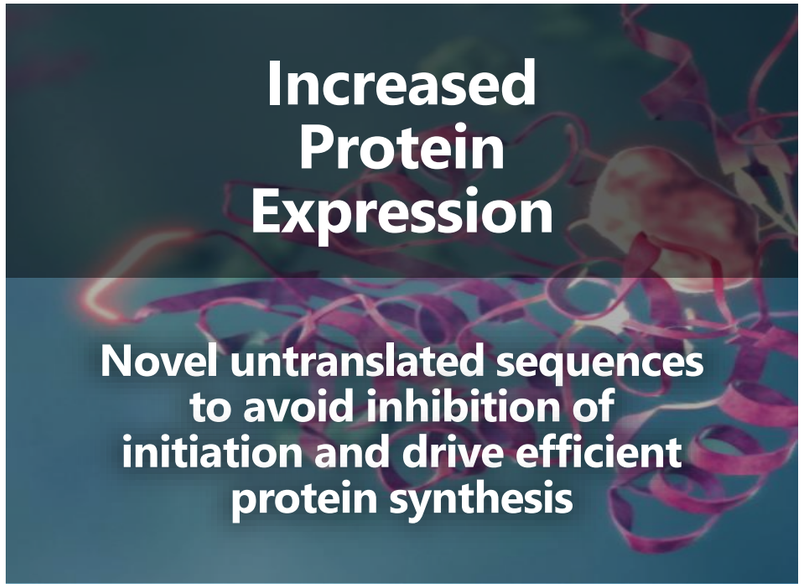
GENE THERAPY PROGRAM
Penn
Amicus Therapeutics
Nationwide Children's

Gene Therapy Program Updates

- **Positive initial preclinical data for Pompe gene therapy**
- **CLN3 Batten Disease Phase 1/2 Study Enrolling; Low Dose Cohort Complete (n=3)**
- **Additional 2-Year CLN6 Batten Disease Phase 1/2 Data on Track for 3Q19**
- **Additional preclinical studies in progress including CLN8, CLN1, Pompe and Fabry**
- **Expanded Penn Collaboration Creates R&D Engine for Future Growth**

Amicus Protein Engineering Expertise & Technologies for Gene Therapy

Collaboration to Enable Greater Protein Expression and Delivery at Lower Gene Therapy Doses



GENE THERAPY PROGRAM

Penn

Expanded Amicus-Penn Collaboration Terms

World Class Industry-Academia Partnership in Rare Diseases

- Exclusive Disease-Specific Worldwide Rights to Penn's Next Generation Gene Therapy Technologies from the Wilson Lab for the Majority of Lysosomal Disorders
- Current Collaboration Extended to Include Three New Indications: Niemann-Pick Type C (NPC) Mucopolysaccharidosis Type IIIA (MPS IIIA), and Mucopolysaccharidosis Type IIIB (MPS IIIB)
- New Research Programs also Encompass 12 Additional Rare Diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy and Select Other Muscular Dystrophies
- Robust R&D Engine Created through Combination of Amicus Expertise in Protein Engineering with Penn's Vector Technology, Translational Science, Manufacturing and Immunology Capabilities
- Amicus to Invest $10M / Year for 5 Years for Research to Improve Safety, Efficacy and Manufacturability of Next Generation Vectors with Option to Extend
- Amicus to Contribute Protein Engineering Platform Technology and Capabilities to Collaboration in Lieu of Any Additional Upfront Payments to Penn
- Amicus to Assume Development Costs for NPC, Next Generation MPS IIIA, and MPS IIIB, to begin immediately*

*Sufficient cash into 2021 including additional spend required under the Amicus-Penn collaboration

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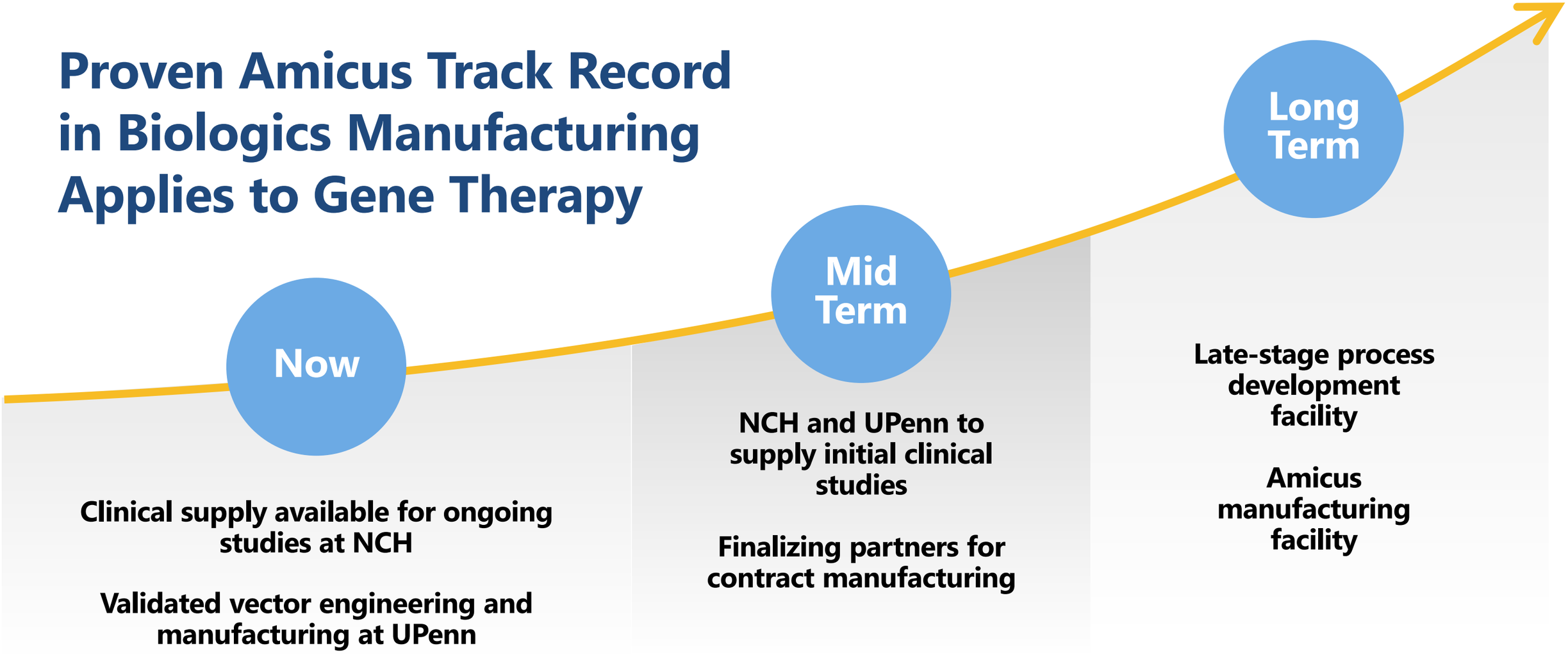
Manufacturing: Three-Pronged Approach

Proven Amicus Track Record in Biologics Manufacturing Applies to Gene Therapy



Financial Summary and Guidance

Strong Balance Sheet with $438M+ Cash at 3/31/19 - Cash Runway into 2021

FINANCIAL POSITION	March 31, 2019
Cash	~$438M
Cash Runway[1]	Into 2021
Debt[2]	$153.7M
CAPITALIZATION	
Shares Outstanding[3]	~253M
FINANCIAL GUIDANCE	
FY19 Galafold Revenue Guidance	$160M-$180M

[1]Based on existing operating plan including proceeds from June 2019 equity offering to invest in manufacturing
[2]Includes $3.7 million of convertible debt and $150 million of straight debt
[3]Includes shares from June 2019 equity offering

Anticipated Milestones: 2019

Well-Positioned to Create Significant Value for Patients and Shareholders in 2019

Galafold: Fabry Disease

- FY19 revenue guidance $160M-$180M
- Growth in existing markets
- Expansion into new markets
- Diagnostic initiatives

AT-GAA: Pompe Disease

- ✓ Additional Phase 1/2 data (21 and 24 months)
- ✓ Breakthrough therapy designation (BTD) in LOPD
- ✓ Phase 1/2 study fully enrolled (Cohorts 1-4)
- PROPEL pivotal study enrollment (n=100)
- Additional Phase 1/2 data (Cohort 4)
- Natural history study data
- Additional supportive studies
- Advance CMC requirements to support BLA

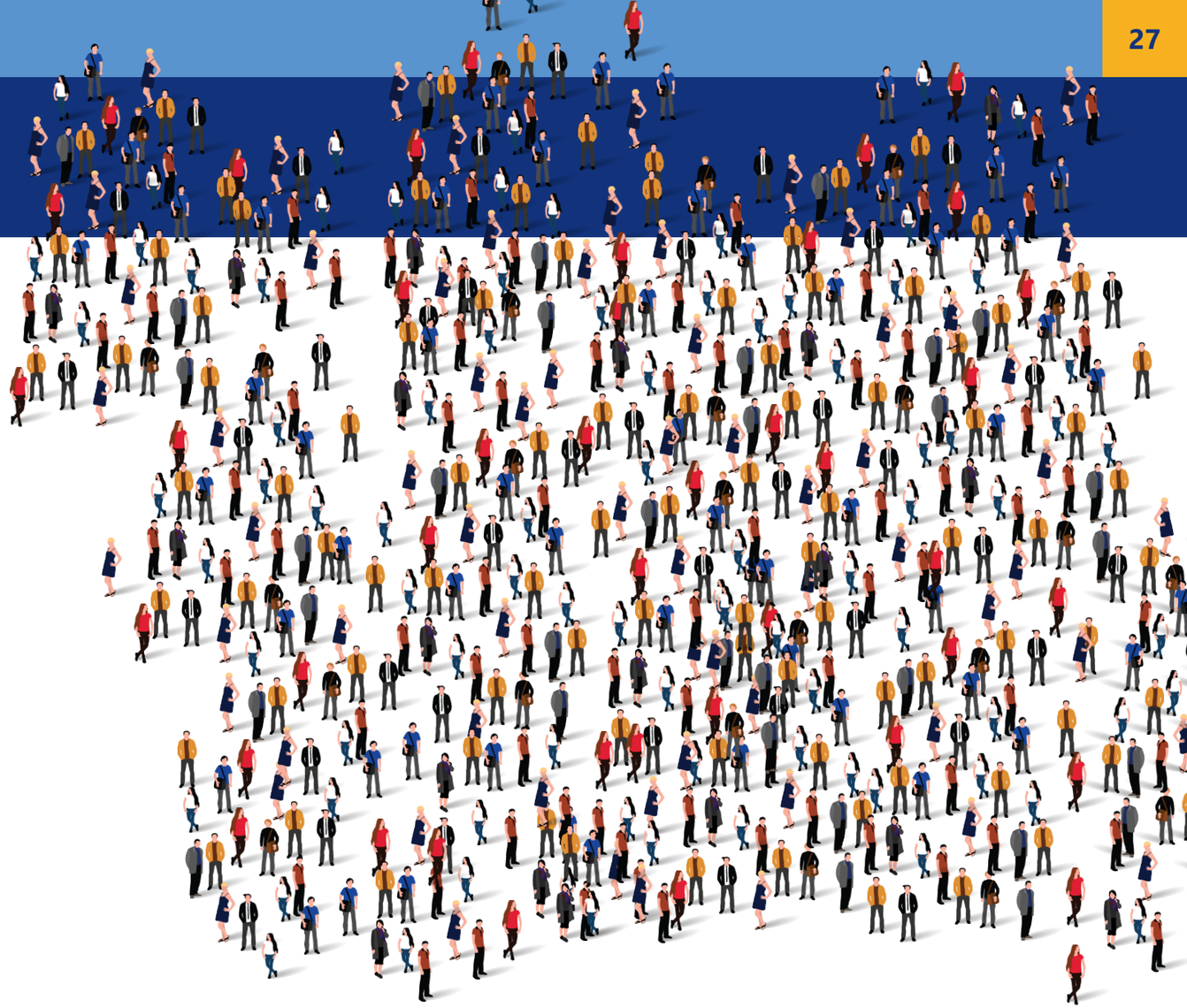
Gene Therapy Programs

- Ongoing CLN3 Batten disease Phase 1/2 study enrollment
- Additional 2-year data from CLN6 Batten disease Phase 1/2 study
- Additional preclinical data including next-generation gene therapies for Fabry and Pompe
- Selection of Pompe AAV gene therapy clinical candidate to move into IND-enabling studies

A RARE VISION.

A Leader in the Human Genome Medicine Revolution

5,000 Patients* $1B Global Sales



MORE Patients More Indications

2023

LONGER-TERM VISION

*Clinical & commercial, all figures approximate [1]Preliminary unaudited

Amicus Therapeutics

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement

Amicus Therapeutics®