



37th Annual J.P. Morgan Healthcare Conference

John F. Crowley, Chairman and Chief Executive Officer
January 8, 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, market potential projections, 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, 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 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, 2017 as well as our Quarterly Report on Form 10-Q for the quarter September 30, 2018 filed November 5, 2018 with the Securities and Exchange Commission. 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.

Amicus Highlights

GALAFOLD'S EXTRAORDINARY LAUNCH SUCCESS

- 650+ Patients and ~\$91M Global Sales in FY18
- FY19 Guidance of \$160M-\$180M
- \$500M Potential Sales by 2023
- \$1B+ Addressable Market Opportunity by 2028

AT-GAA IN POMPE: POTENTIAL TO BECOME STANDARD OF CARE

- Continued Strength of Clinical Data
- Multiple Data Expected Throughout 2019
- 100+ Pompe Patients on AT-GAA by YE19
- \$1B-\$2B+ Market Opportunity

LEADING GENE THERAPY PORTFOLIO IN RARE METABOLIC DISEASES

- Pipeline of 14 Gene Therapies
- 2 Clinical Stage Programs
- Amicus as "Consolidator" of Best Minds and Technologies
- \$1B+ Peak Recurring Market Opportunity

FINANCIAL STRENGTH

- \$500M+ Cash at 12/31/18 (runway into mid-2021)
- Growing Contribution from Galafold Revenues

2023 VISION

- 5,000+ Lives Transformed
- \$1B+ in Revenue
- Leading Global Rare Disease Biotech



**WHERE we
came from**

Amicus.

Definition:

\ə'mēkəs (noun) *Latin* Friend



Amicus Founding Beliefs

WE BELIEVE...

We push ideas as far and as fast as possible

WE BELIEVE...

We encourage and embrace constant innovation

WE BELIEVE...

We have a duty to obsolete our own technologies

We are business led and science driven

Our passion for making a difference unites us

- We encourage and embrace constant innovation
- We have a duty to obsolete our own technologies
- We push ideas as far and as fast as possible
- We take smart risks
- We work hard

- We build strategic partnerships

- Our passion for making a difference unites us
- Integrity, competence and thought is essential
- We communicate openly
- Work-life balance keeps us healthy

A background image showing three women in a professional setting, possibly a meeting or collaborative work environment. They are looking down at documents or a device on a table. The image is dimmed to serve as a background for the text.

Build a **great and
enduring company.**



**WHERE we
are today**

A RARE COMPANY.

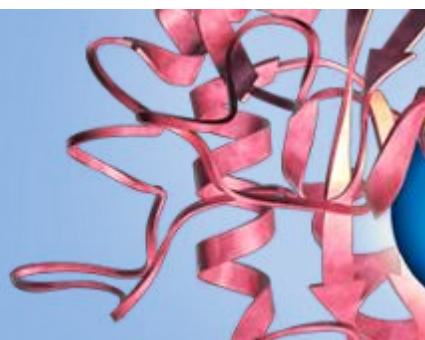
 **Galafold™**
(migalastat)

First Oral Precision
Medicine for Fabry Disease



BIOLOGICS PLATFORM

Protein Engineering
& Glycobiology

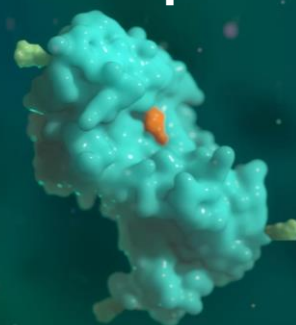


\$500M+
Cash
(12/31/18)

**Gene
Therapy
Platforms**

AT-GAA*

Phase 3
Investigational
Therapy for
Pompe



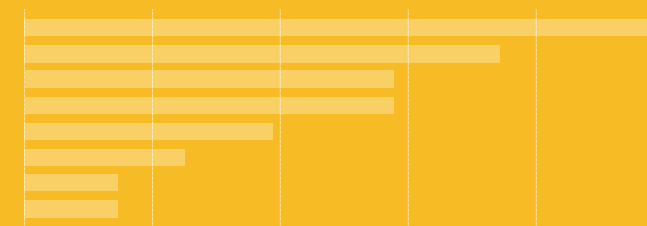
500+
EMPLOYEES
globally



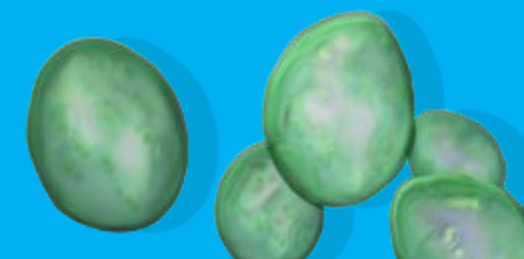
**GLOBAL
FOOTPRINT**
in 27 countries



PORTFOLIO
of 15 programs for rare
metabolic diseases



Leading Expertise in
**Lysosomal
Storage
Disorders**



* AT-GAA, also known as ATB200/AT2221

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					
Rare CNS and Other Gene Therapies						
CDKL5 Deficiency Disorder GTx / ERT	PENN					
Niemann-Pick Type C (NPC)	NCH					
Tay-Sachs Disease	NCH					
Wolman Disease	NCH					
Other	NCH / PENN					

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

A RARE OPPORTUNITY.

Key Drivers of Value

Galafold
*\$1B+
Opportunity*

**Pompe
ERT**
*\$1B-2B+
Opportunity*

**Gene
Therapy
Portfolio**
*\$1B+
Opportunity*

Transform the Lives of Thousands of Patients

2018: A Year in Headlines



Amicus Treatment for Pompe Disease Could be Company's 'Crown Jewel'



AMICUS' GALAFOLD WINS FDA APPROVAL FOR FABRY DISEASE



Amicus Dives into Gene Therapy with Plans to Expand Further



A \$100 Million Biotech Deal is Also a Tale of Two Executives Facing Their Kids' Deadly Diseases



Fabry Disease Treatment Approved in Japan



Amicus Therapeutics, Penn Joins Forces



John Crowley Discusses Amicus' foundations and expanding pipeline

2018 Key Strategic Priorities

- 1  **Galafold (migalastat) revenue of \$80-\$90M** 
- 2  **Secure approvals for migalastat in Japan and the U.S.** 
- 3  **Achieve clinical, manufacturing & regulatory milestones to advance AT-GAA toward global regulatory submissions and approvals** 
- 4  **Develop and expand preclinical pipeline to ensure at least one new clinical program in 2019** 
- 5  **Maintain strong financial position** 



**WHERE we
are going**

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 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**

A RARE VISION. Impacting Lives



>350 Patients* | ~\$36M Global Sales

YE17



>700 Patients* | ~\$91M¹ Global Sales

YE18



5,000 Patients* | \$1B Global Sales

2023

Amicus in 2023

Our Path to Become One of the Leading Global Biotechnology Companies in Rare Diseases



~\$1B / ~5,000 patients in 2023

Approved

**Galafold
~\$500M**

**AT-GAA*
~\$200M**

**Gene Therapies & In-licensed Products*
~\$300M**

Clinical

5+ Programs in Clinic

Preclinical

1+ New IND Every 12-18 Months

*Assumes successful clinical trials and regulatory approvals



Fabry Disease Overview

“We support the disease communities – and their families”

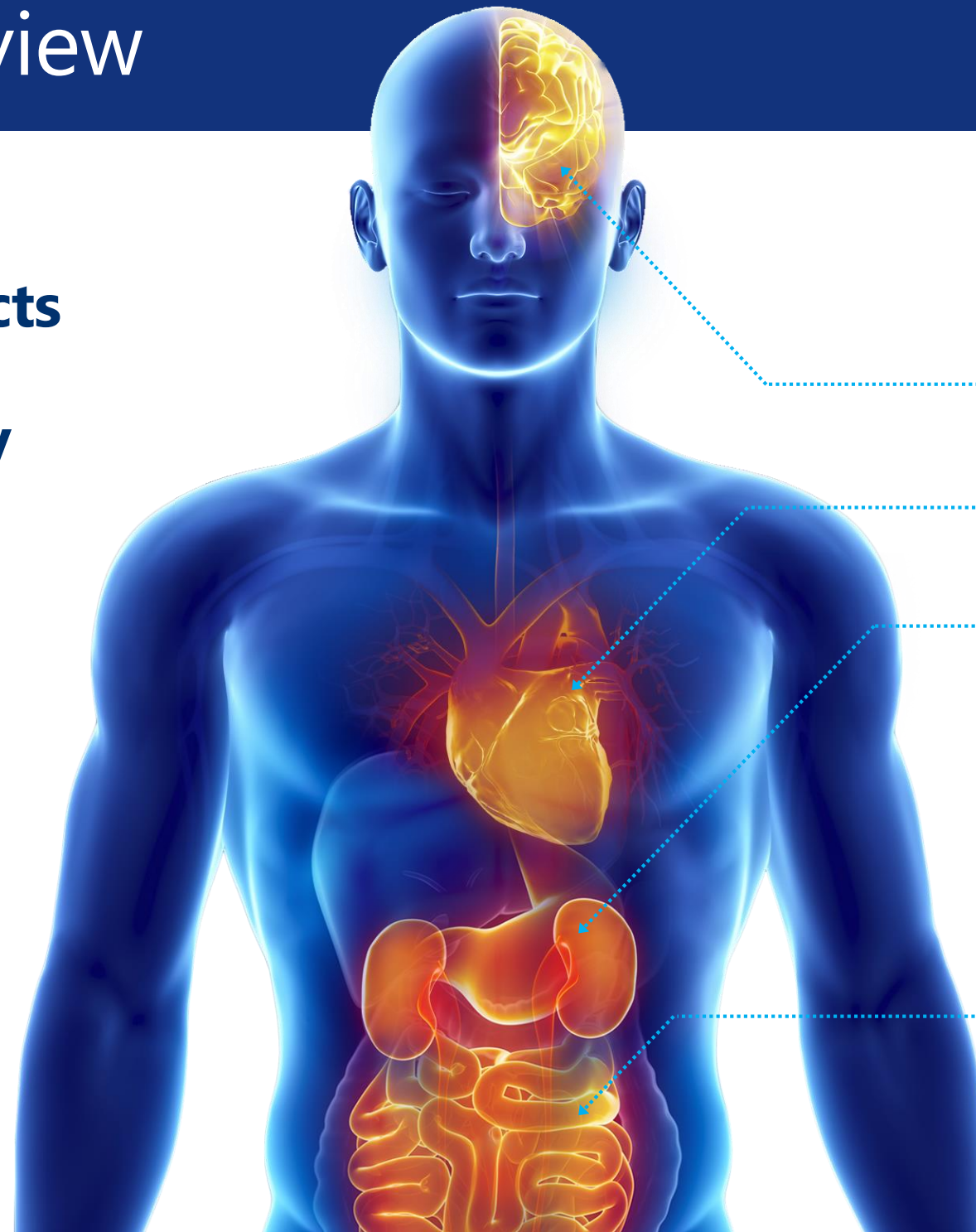
- Amicus Belief Statement

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% male⁴)
- Newborn screening studies suggest prevalence of ~1:1000 to ~1:4000



Leading Causes of Death:

Transient Ischemic Attack (TIA) & Stroke¹

Heart Disease²

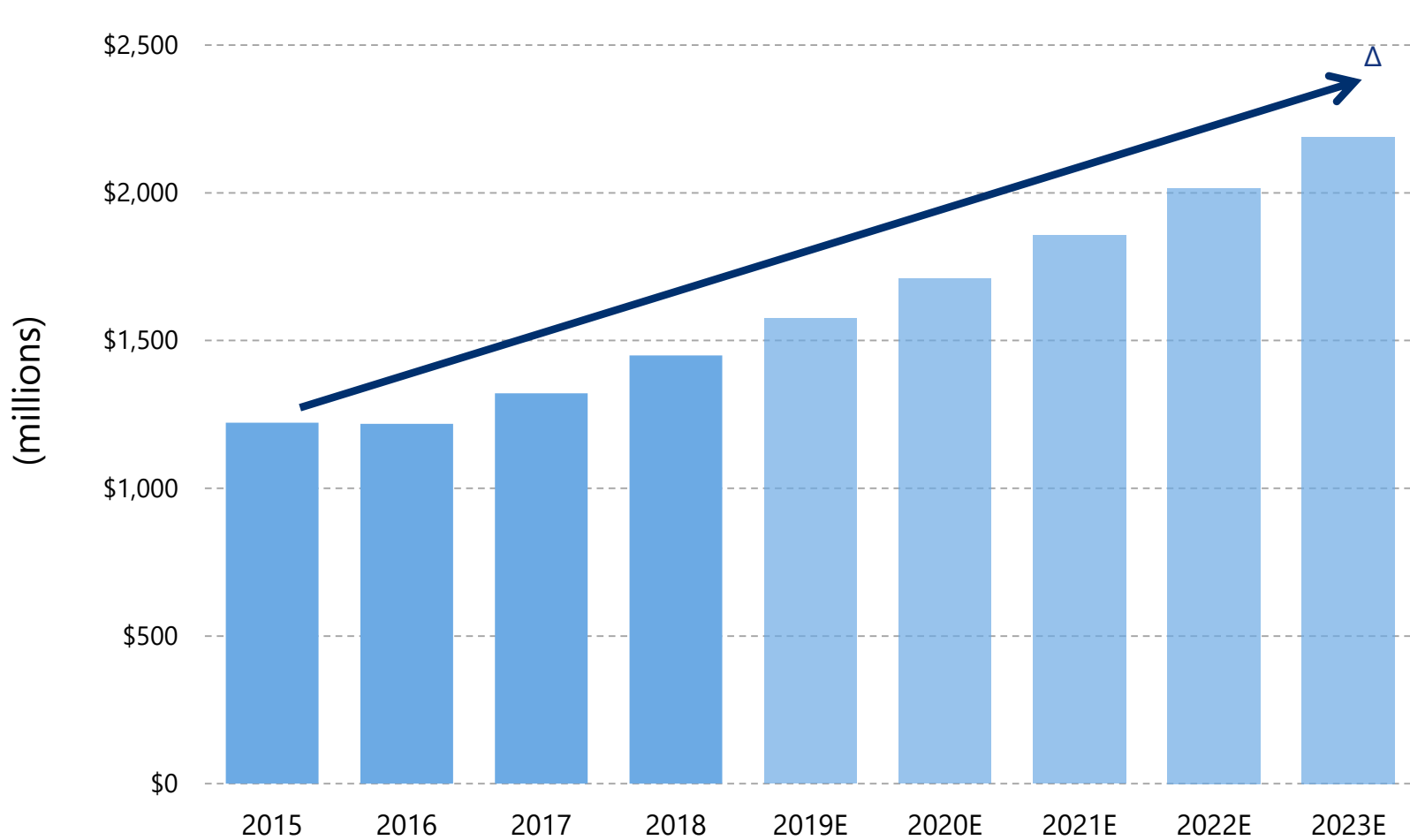
Kidney Disease³

Life-Limiting Symptoms:

Gastrointestinal³

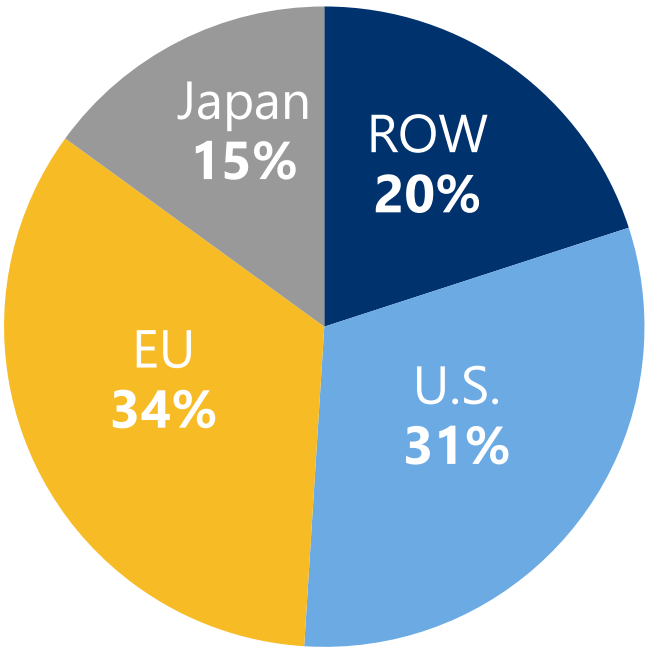
Global Fabry Market Growth Driven by New Patients

Global Fabry Market Exceeded \$1.4B as of 3Q18 and Tracking Toward \$2.2B by 2023 (8.6% 5-Year CAGR)*



Annualized 3Q18 Fabry sales increased

10%



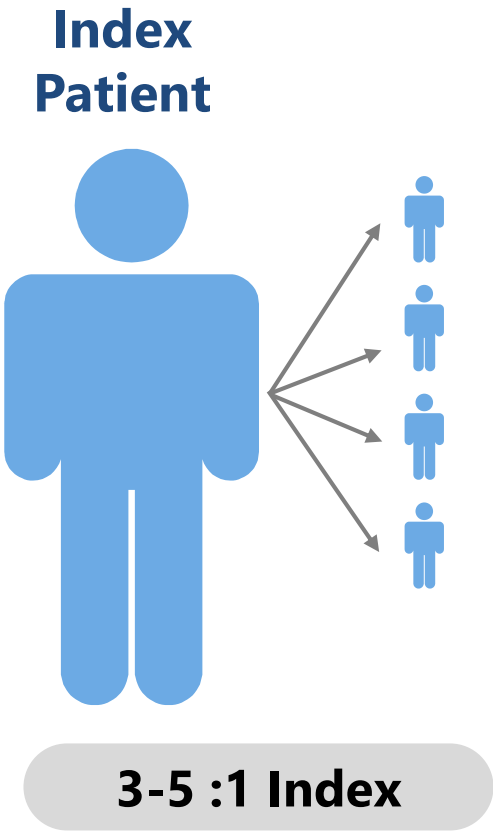
Global Fabry Market and growth measured by reported CER (constant exchange rates) Adjusted Net Sales through 3Q18
Δ 2018 – 2023 are based on estimated 8.6% annual growth rate (5-Year CAGR rate)

Fabry Underdiagnosis

Newborn Screening Studies Suggests Fabry Could Be One of the More Prevalent Human Genetic Diseases

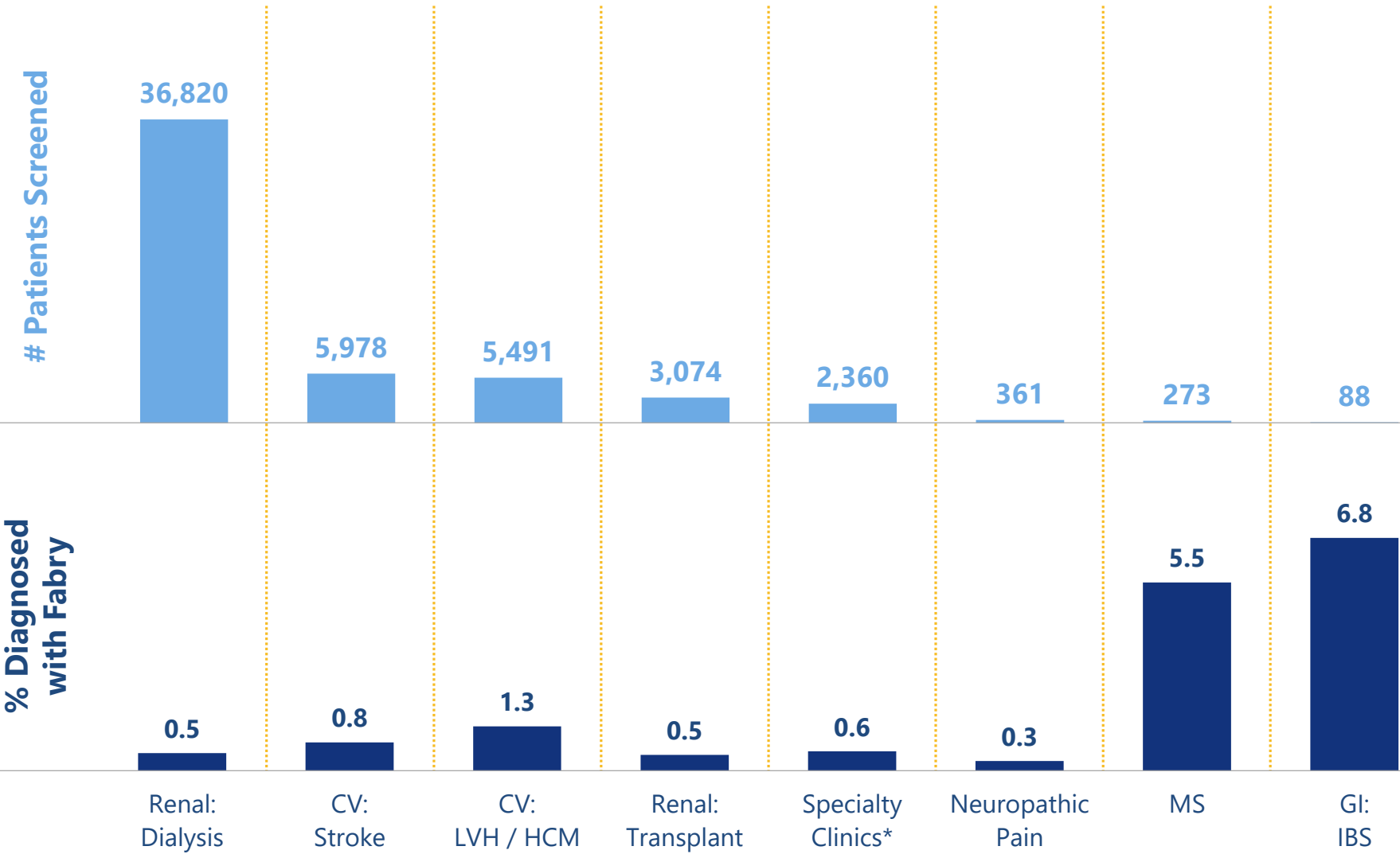
NEWBORN 8454ENING STUDY	NEWBORNS SCREENED	CONFIRMED FABRY MUTATIONS	% AMENABLE
Hopkins, 2018, Missouri, US	43,701	15 [1:~2913]	N/A
Burton, 2017, Illinois, US	219,793	26 [1: ~8454]	N/A
Mechtler, 2011, Austria	34,736	9 [1: ~3800]	100%
Hwu, 2009, Taiwan	171,977	75 [1: ~2300]	75%
Spada, 2006, Italy	37,104	12 [1: ~3100]	86%
Historic published incidence	1:40,000 to 1:60,000		

Majority Diagnosed through Newborn Screening
Have Amenable Mutations



Fabry Misdiagnosis

Recent Studies in Multiple Disease Areas Show Significant Rate of Fabry Disease as Underlying Cause



Source: Summary of N=63 completed high-risk screening associated studies, Huron Analysis *Specialty clinics include neurology, cardiology, nephrology, and pediatrics **1 MS completed study refers to % of FD patients misdiagnosed with MS





Galafold[®] (migalastat) Global Launch...

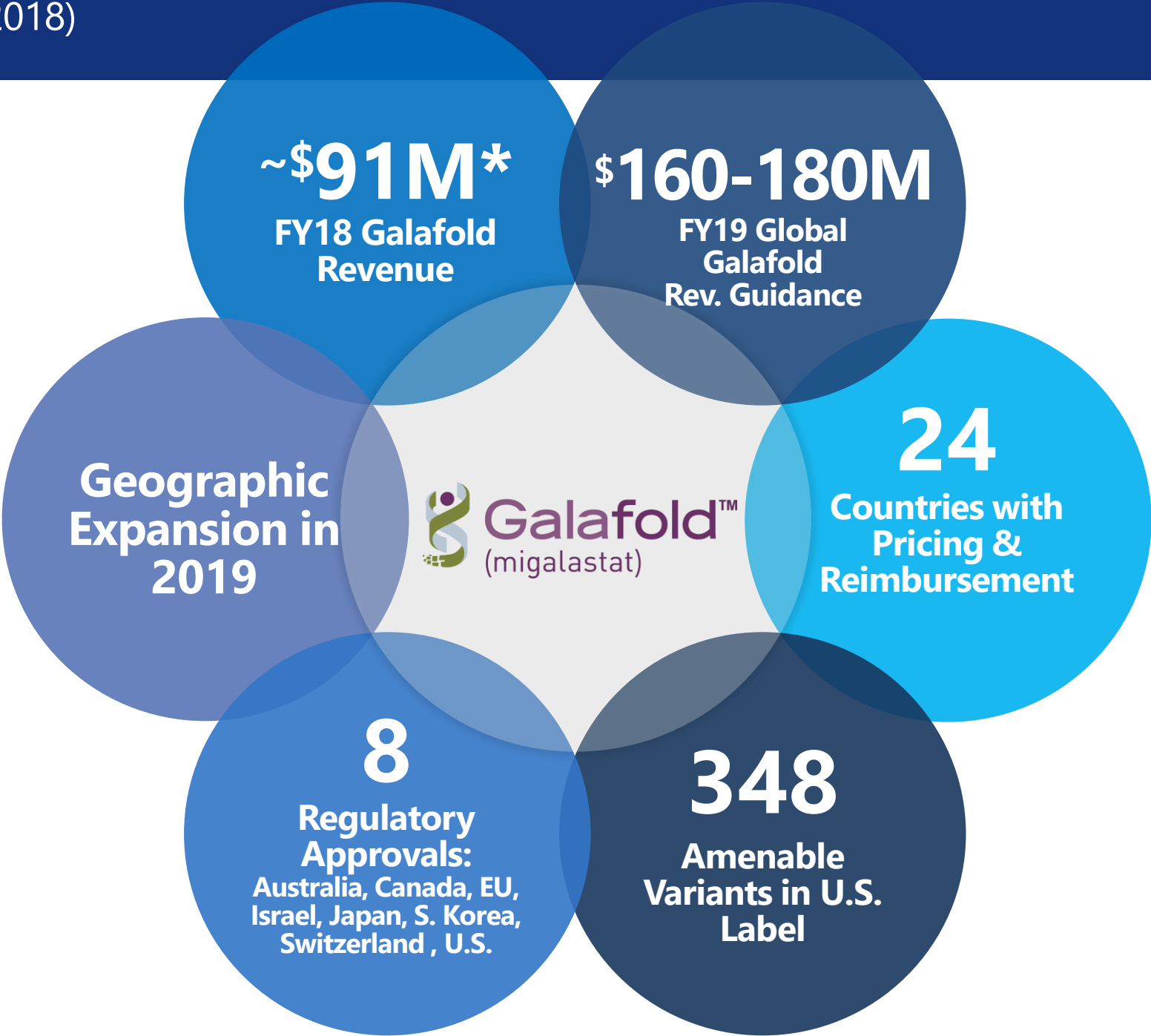
...taking a leadership role in the
treatment of Fabry disease

“We push ideas as far and as fast as possible”

- Amicus Belief Statement

Galafold Snapshot (as of December 31, 2018)

One of the Most Successful Rare Disease Launches

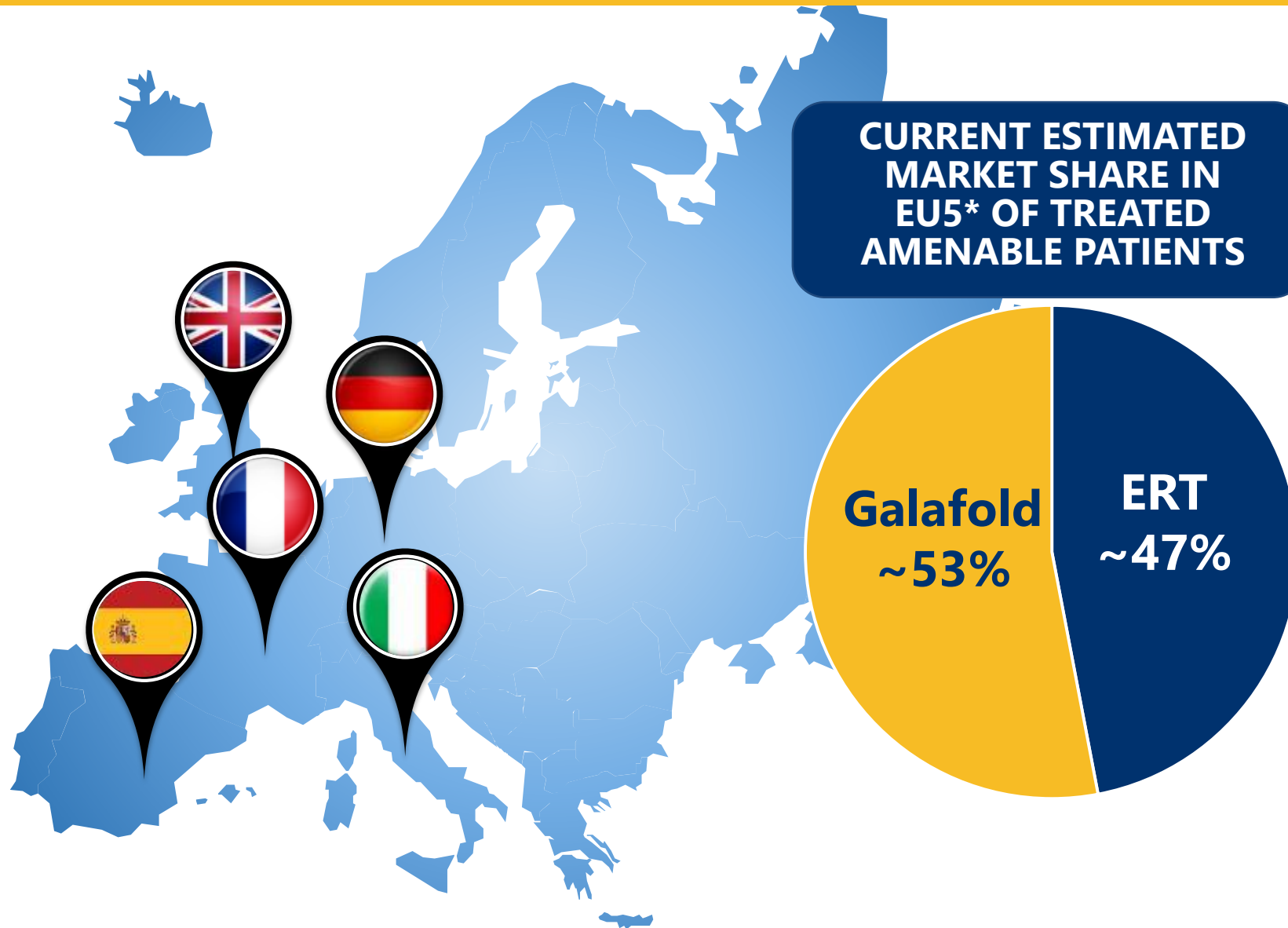


Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including 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.

*Preliminary and unaudited

International Update (as of December 31, 2018)

Strong Continued Growth with High Compliance and Adherence



MARKET DYNAMICS

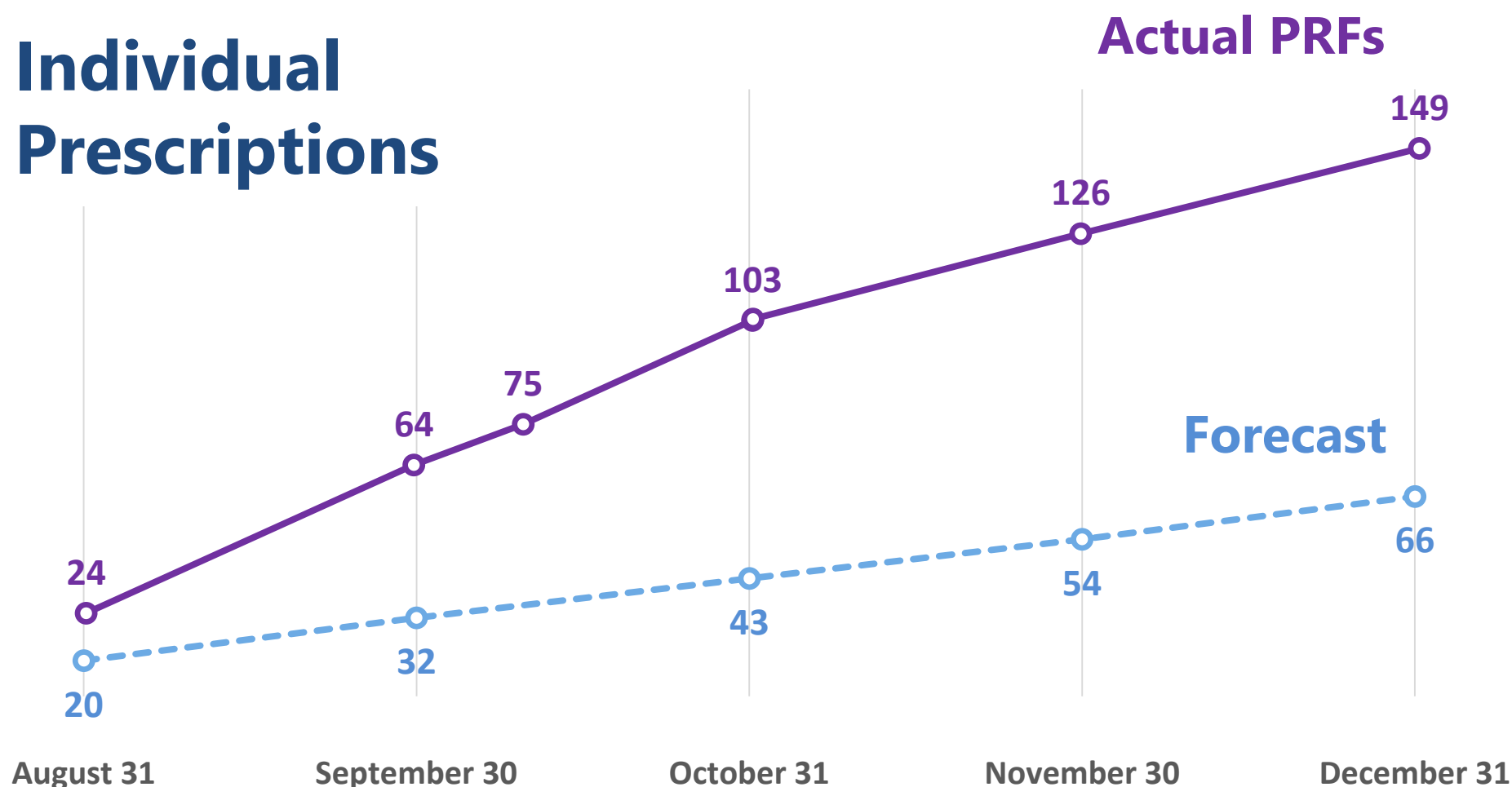
- Continued strong uptake in ERT-switch patients
- Increasing adoption by diagnosed untreated patients
- Very high rates of adherence and compliance (>90%)
- Balanced mix of males and females, classic and late-onset patients
- Robust interest from physician community

*Market share assumptions based on estimated number of treated amenable patients in EU5 as of October 2018

Key U.S. Launch Metric – Individual Prescriptions (Patient Referral Forms)

149 Individual Prescriptions (12/31/18) Significantly Exceeds Internal Forecast and Provides Strong Foundation for 2019

Individual Prescriptions

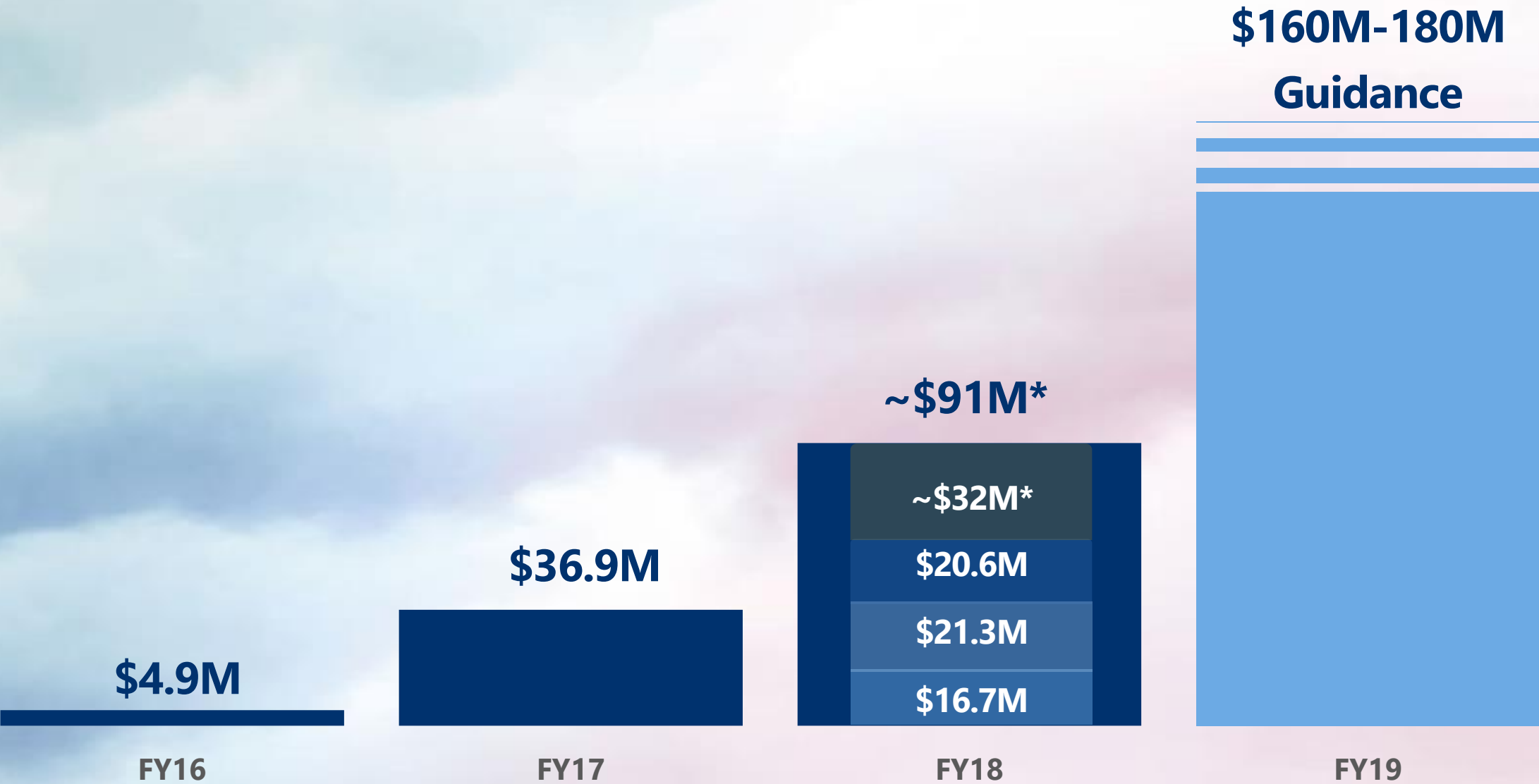


Market Dynamics

- 100+ U.S. patients now on Galafold
- Strong patient and physician demand
- Growing prescriber base of 60+ physicians
- <60 day average PRF to shipment
- Patient demographics in line with launch strategy
- Broad reimbursement coverage

Galafold Success and FY18 Galafold Revenue Guidance

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



*Preliminary and unaudited

Total Amenable Patient Population ("TAPP")

Estimate based on 35% - 50% amenability

\$1B+ Addressable Market Opportunity by 2028

Upside Potential

WORLDWIDE

Diagnosis grows due to newborn screening in U.S. & Japan

TAPP: 4,700-6,750

Peak Potential

WORLDWIDE

Diagnosis continues at current rate

TAPP: 4,200-6,000

Today

WORLDWIDE*
(U.S. & Japan Added)

TAPP: 3,800-5,500

2018

2028

EU & ROW Only

TAPP: 2,000-3,000

2017

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



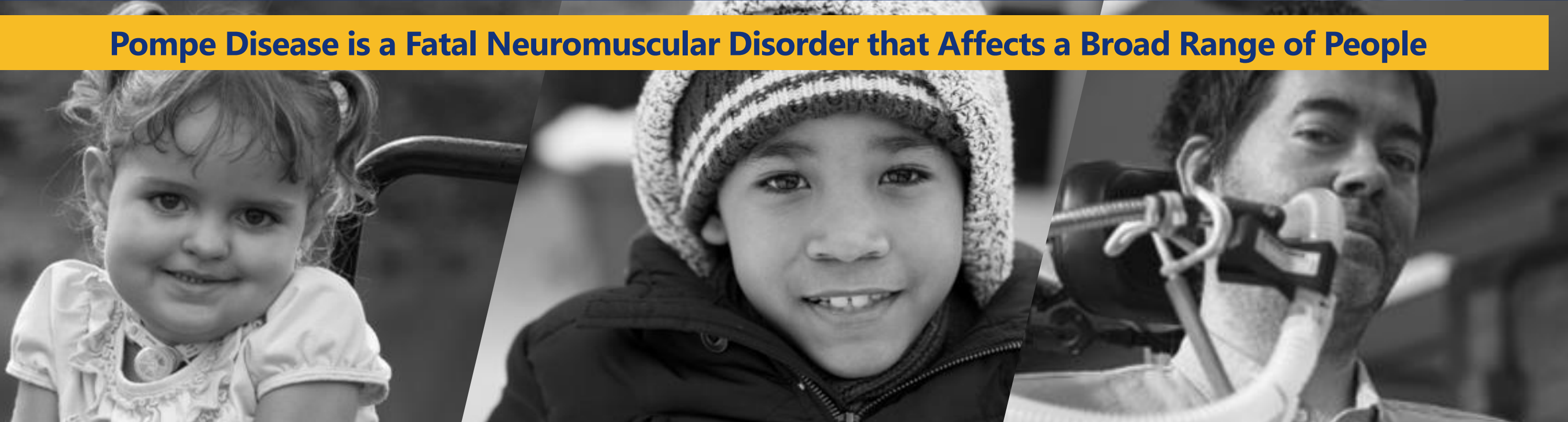
AT-GAA Novel ERT for Pompe Disease

“We encourage and embrace constant innovation”

- Amicus Belief Statement

Pompe Disease Overview

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



5,000 – 10,000 patients
diagnosed WW¹

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

Symptoms include muscle weakness,
respiratory failure, and cardiomyopathy

~\$900M+ Global Pompe
ERT sales in FY17²

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

ATB200

Investigational human recombinant GAA enzyme

IV infusion

Designed for enhanced targeting to muscle cells

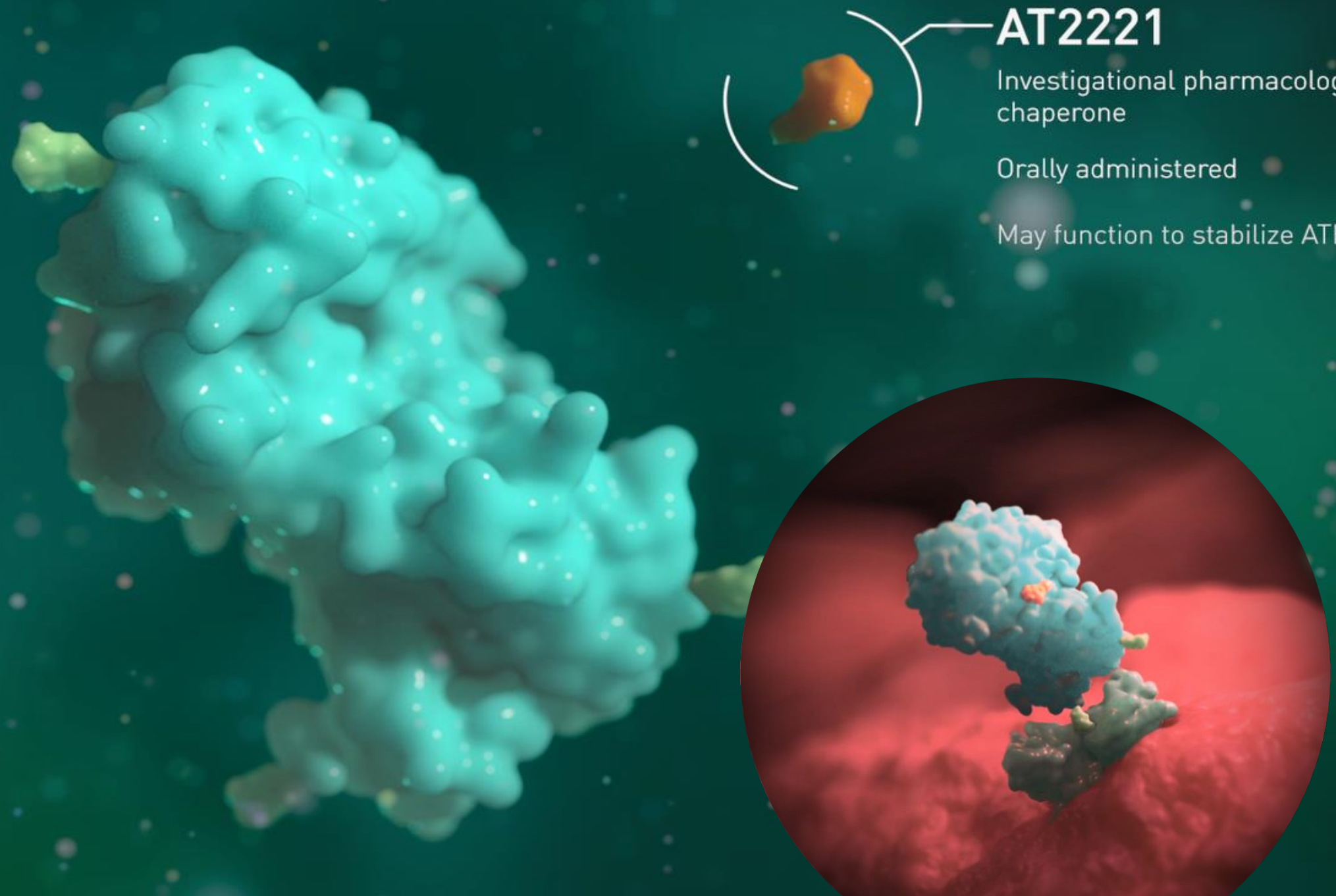
AT2221

Investigational pharmacological chaperone

Orally administered

May function to stabilize ATB200

AT-GAA



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 18

6-Minute Walk Test (m)

Cohort	Baseline (n=10)	Change at Month 18 (n=9) Mean (SD)
Cohort 1 ERT-Switch Ambulatory	397.2 (96.8)	+51.7 (45.9)

Cohort	Baseline (n=5)	Change at Month 18 (n=5) Mean (SD)
Cohort 3 ERT-Naïve	399.5 (83.5)	+49.0 (28.3)

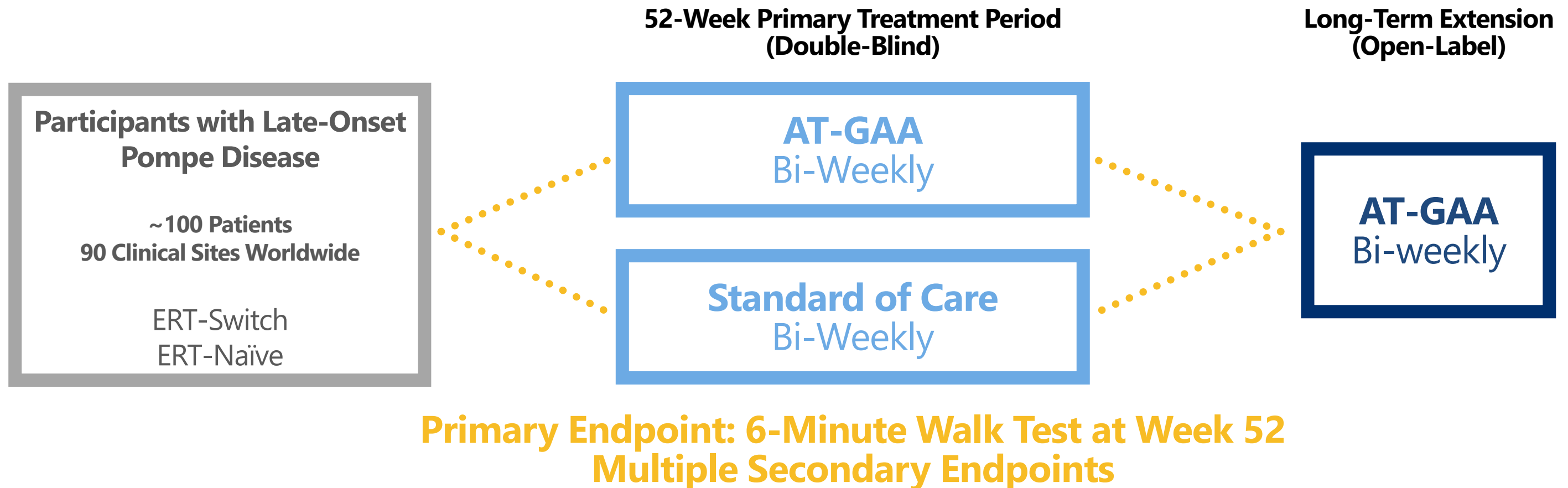
FVC (% Predicted)

Cohort	Baseline (n=9*)	Change at Month 18 (n=8) Mean (SD)
Cohort 1 ERT-Switch Ambulatory*	52.6 (14.7)	-3.7 (7.0)

Cohort	Baseline (n=5)	Change at Month 18 (n=5) Mean (SD)
Cohort 3 ERT-Naïve	53.4 (20.3)	+5.0 (2.9)



PROPEL (ATB200-03) Study Design



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**

AT-GAA: 2019 Objectives

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

- **Enroll PROPEL study (n=100)**
- **Present additional Phase 1/2 data**
- **Report natural history study data**
- **Initiate supportive studies**
- **Advance agreed upon CMC requirements to support BLA**



Gene Therapy Pipeline

“We have a duty to obsolete our own technologies”

- Amicus Belief Statement

Leading LSD Gene Therapy Portfolio

Amicus is the Consolidator of the Most Promising Gene Therapy Programs in LSDs



Amicus Gene Therapy Portfolio

	DISCOVERY	PRECLINICAL	Clinical
CLN6 Batten Disease	NCH		
CLN3 Batten Disease	NCH		
CLN8 Batten Disease	NCH		
CLN1 Batten Disease	NCH		
Fabry Gene Therapy	PENN		
Pompe Gene Therapy	PENN		
CDKL5 Gene Therapy / ERT	PENN		
Niemann-Pick Type C (NPC)	NCH		
Wolman Disease	NCH		
Tay-Sachs	NCH		
Other	NCH/ PENN		

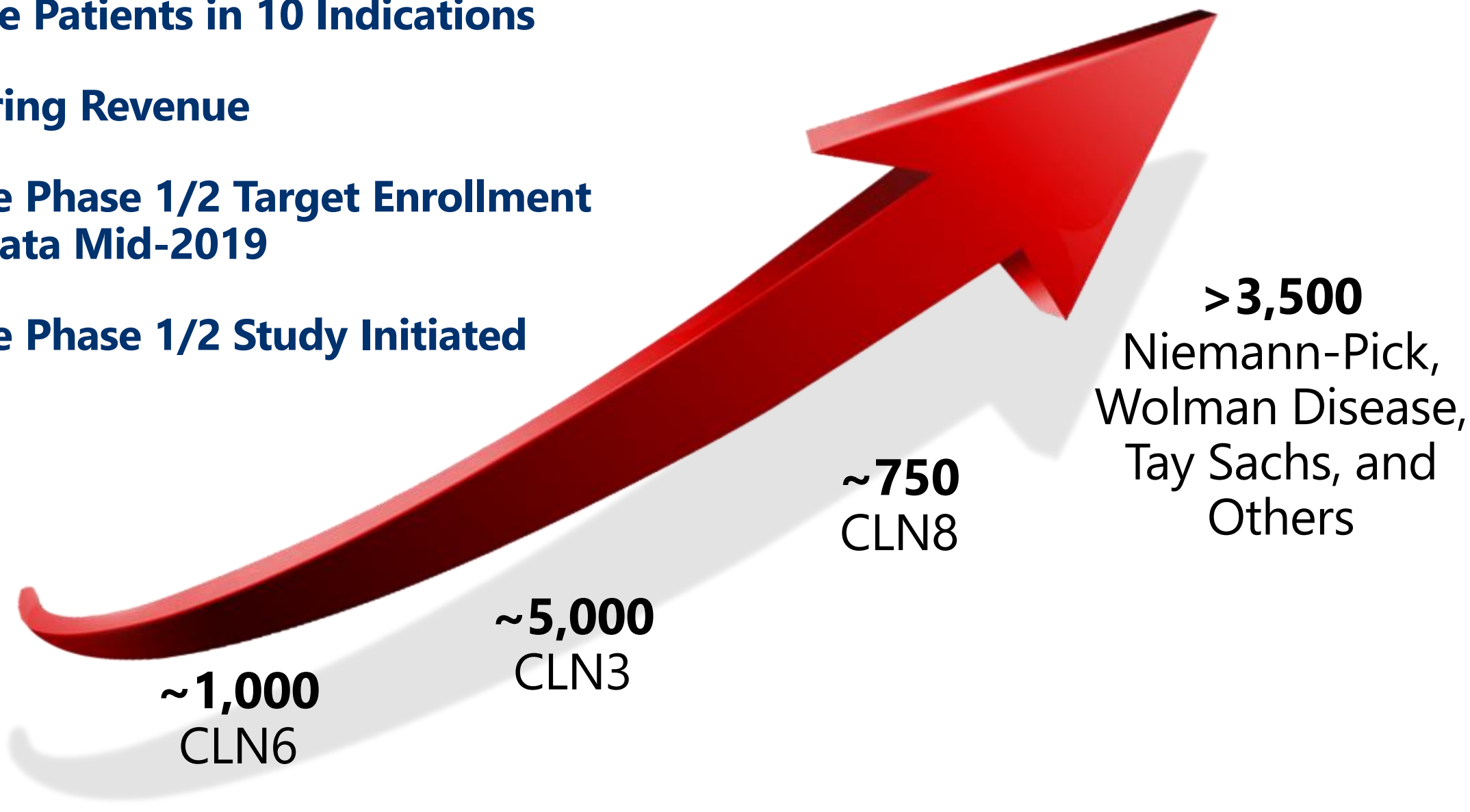
Addressable Patient Populations in Neurologic LSDs*

10,000+ Addressable Patients in 10 Indications

\$1B+ Annual Recurring Revenue

CLN6 Batten Disease Phase 1/2 Target Enrollment Achieved – 2-Year Data Mid-2019

CLN3 Batten Disease Phase 1/2 Study Initiated



Amicus Protein Engineering Expertise & Technologies for Gene Therapy

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

Increased Protein Expression

Novel untranslated sequences to avoid inhibition of initiation and drive efficient protein synthesis

Increased Protein Secretion

Effective signal sequences to increase protein expression & secretion

Improved Protein Targeting & Stabilization

Targeting moieties
Protein design

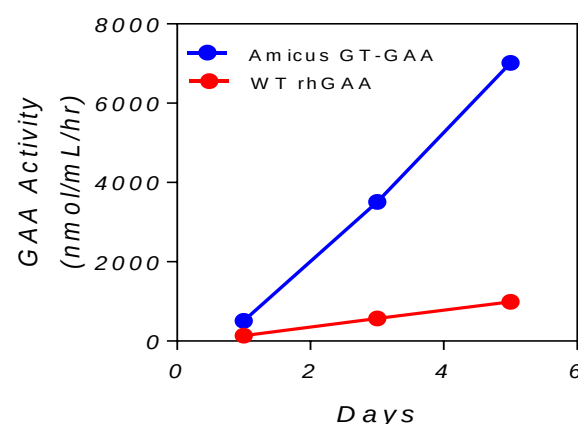


Early Proof of Principle for Optimized Gene Therapy

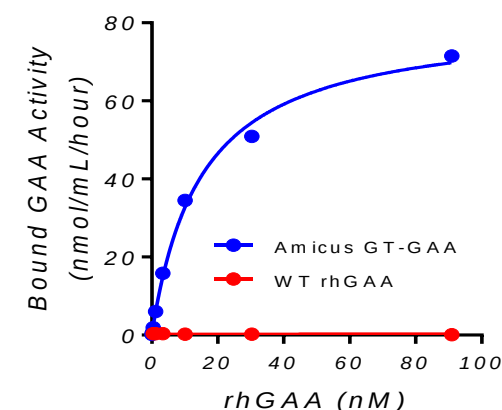
Amicus DNA Constructs Enable Optimized Gene Therapy in Pompe and Fabry

Pompe

Secreted GAA in Media

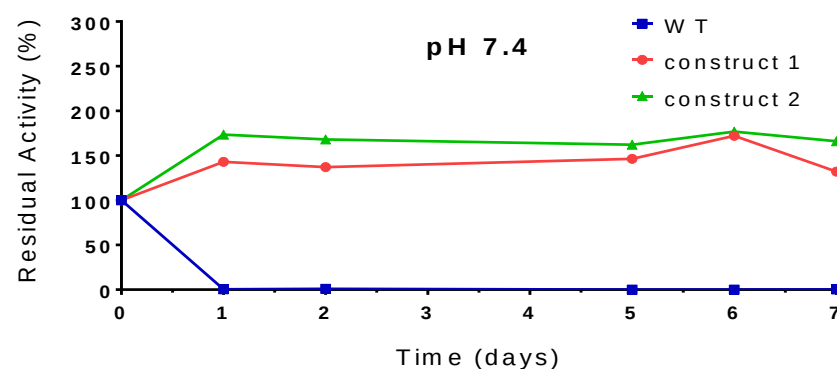


GAA Binding to Intended Receptor

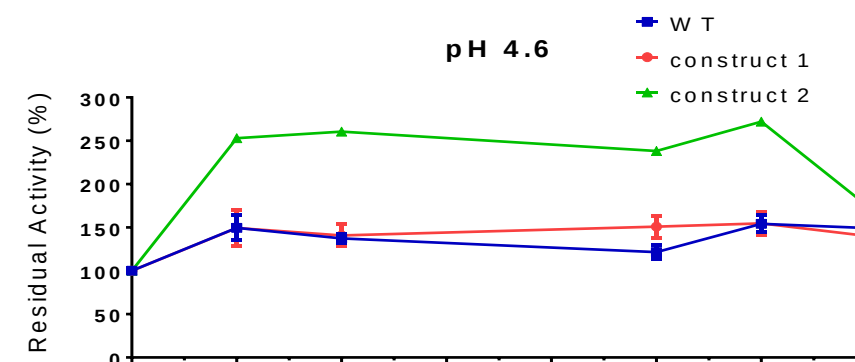


Fabry

Alpha-Gal Activity: pH 7.4



Alpha-Gal Activity: pH 4.6



Manufacturing: Three-Pronged Approach

Proven Amicus Track Record in Biologics Manufacturing Applies to Gene Therapy

Now

GMP clinical supply available for ongoing studies at NCH

Validated vector engineering and manufacturing at UPenn

Mid Term

NCH and UPenn to supply initial clinical studies

Finalize partners for contract manufacturing

Long Term

Amicus manufacturing



Financial Summary & Milestones

“We are business led and science driven”

- Amicus Belief Statement

Financial Summary & Guidance

Strong Balance Sheet with \$500M+ Cash at 12/31/18 - Cash Runway into 2021

FINANCIAL POSITION

December 31, 2018

Cash¹ ~\$505M

Cash Runway Into at least mid-2021

CAPITALIZATION

Shares Outstanding¹ 189,383,924

FINANCIAL GUIDANCE

Projected YE 2019 Cash Balance ~\$300M

Galafold Revenue Guidance \$160M-\$180M

¹ Preliminary unaudited

Anticipated Milestones: 2019

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

Galafold: Fabry Disease

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

AT-GAA: Pompe Disease

- PROPEL pivotal study enrollment (n=100)
- Additional Phase 1/2 data
- 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
- Preclinical proof of concept for Fabry, Pompe and CDD
- Preclinical work across additional neurologic LSDs

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2023 VISION

- 5,000+ Lives Transformed
- \$1B+ in Revenue
- Leading Global Rare Disease Biotech

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement

