Amicus Therapeutics®

Corporate Overview

January 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 Therapeutics

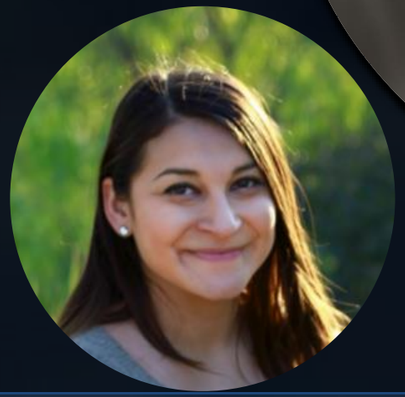
Amicus.

Definition:
\əˈmēkəs (noun) *Latin* Friend










Amicus Founding Beliefs

We push ideas as far and as fast as possible

We encourage and embrace constant innovation

We have a duty to obsolete our own technologies

We are business led and science driven

Our passion for making a difference unites us

Build a great and enduring company.

WHERE we are today

Amicus Therapeutics

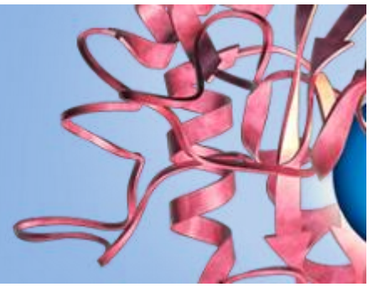
A RARE COMPANY.

Galafold™ (migalastat)

First Oral Precision Medicine for Fabry Disease

BIOLOGICS PLATFORM

Protein Engineering & Glycobiology

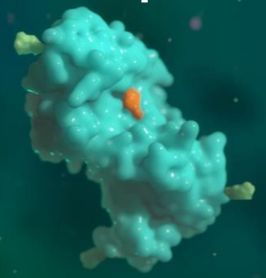


$500M+ Cash (12/31/18)

Gene Therapy Platforms

AT-GAA*

Phase 3 Investigational Therapy for Pompe

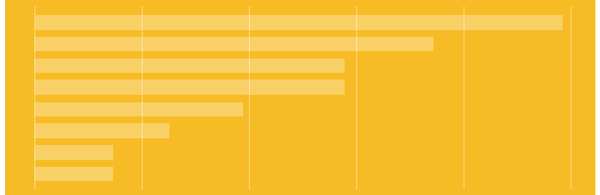


500+ EMPLOYEES globally

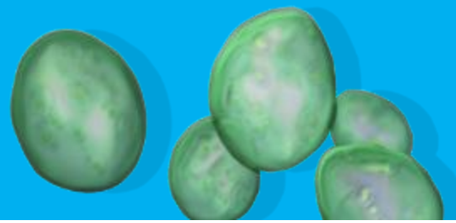
GLOBAL FOOTPRINT in 27 countries

PORTFOLIO

of 15 programs for rare metabolic diseases



Leading Expertise in Lysosomal Storage Disorders



* AT-GAA, also known as ATB200/AT2221

Amicus Therapeutics

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

CNBC

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

REUTERS

AMICUS' GALAFOLD WINS FDA APPROVAL FOR FABRY DISEASE

Bloomberg

Amicus Dives into Gene Therapy with Plans to Expand Further

Forbes

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

RareDR Rare Disease Report

Fabry Disease Treatment Approved in Japan

vantage EP

Amicus Therapeutics, Penn Joins Forces

YAHOO! FINANCE

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

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 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

>700 Patients* | ~$91M[1] Global Sales

5,000 Patients* | $1B Global Sales

YE17 → **YE18** → **2023**

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

Amicus Therapeutics

Amicus in 2023

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

Amicus Therapeutics®

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

*Assumes successful clinical trials and regulatory approvals

Fabry Disease Overview

“We support the disease communities – and their families”

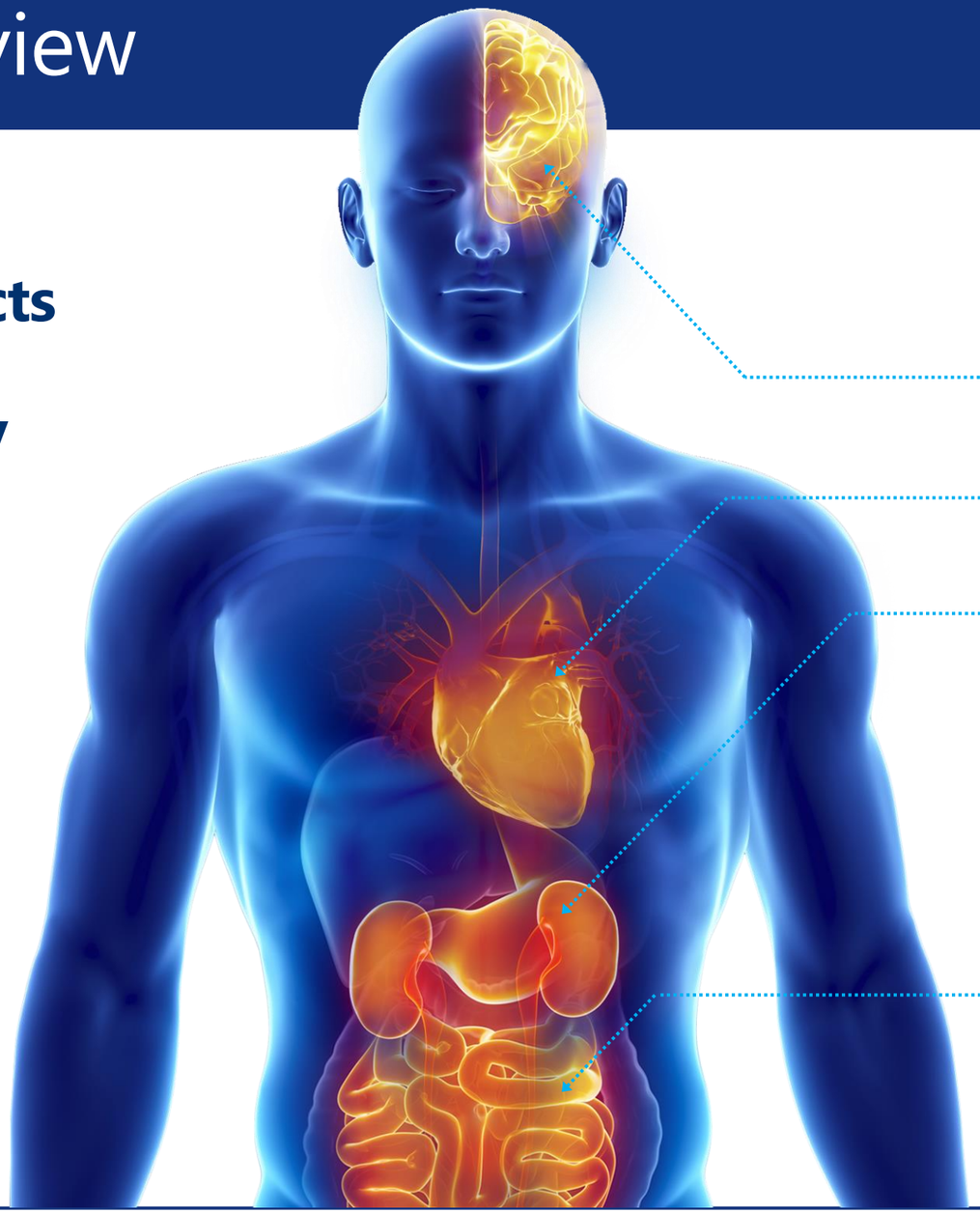
- Amicus Belief Statement

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% male[4])
- Newborn screening studies suggest prevalence of ~1:1000 to ~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

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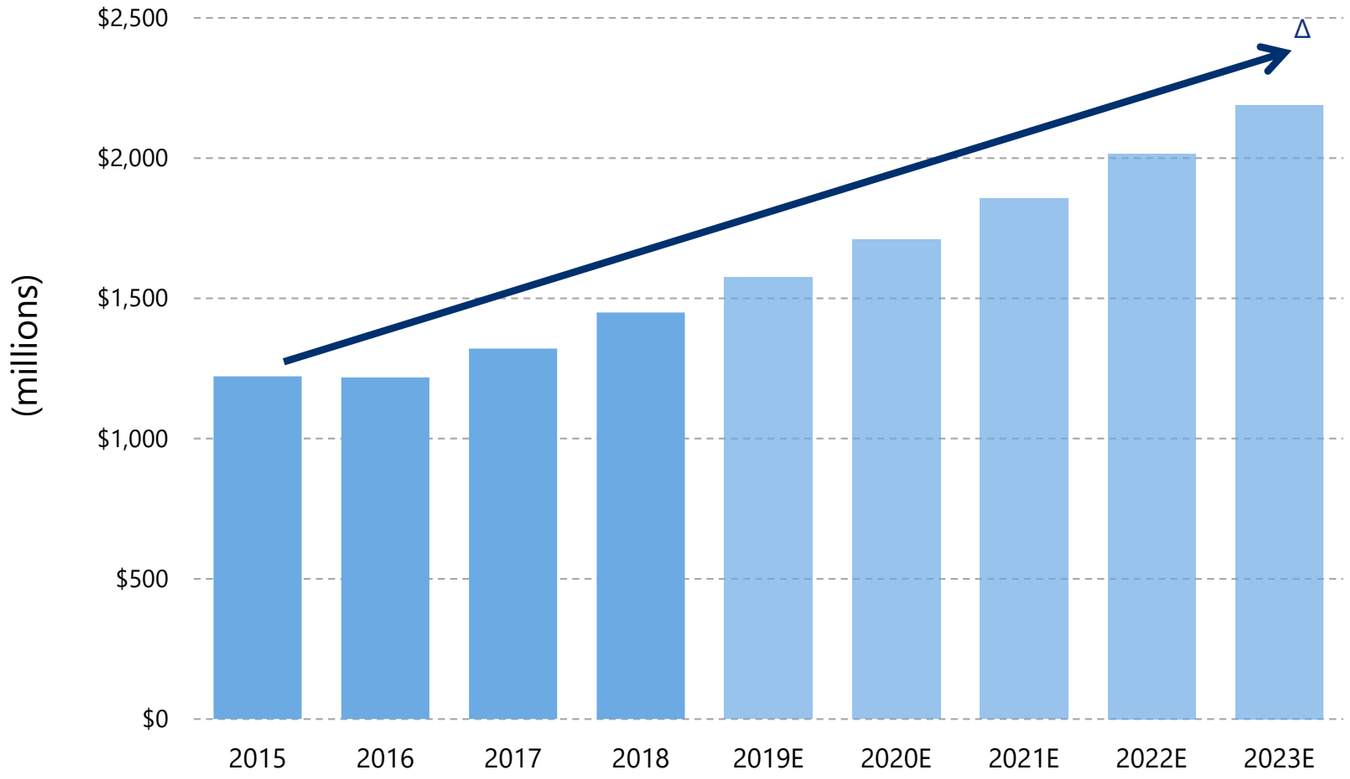
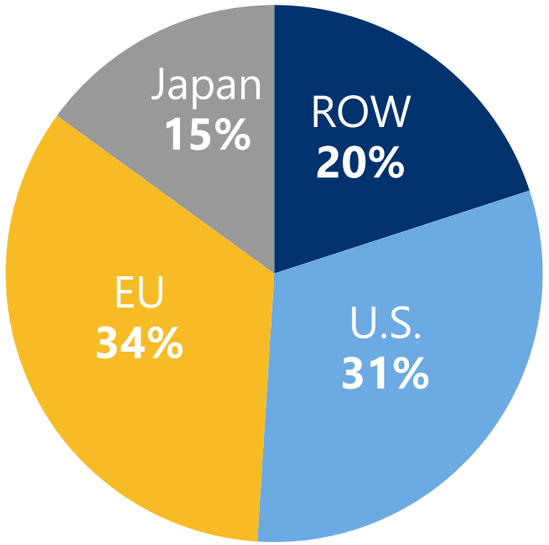
Global Fabry Market Growth Driven by New Patients

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)

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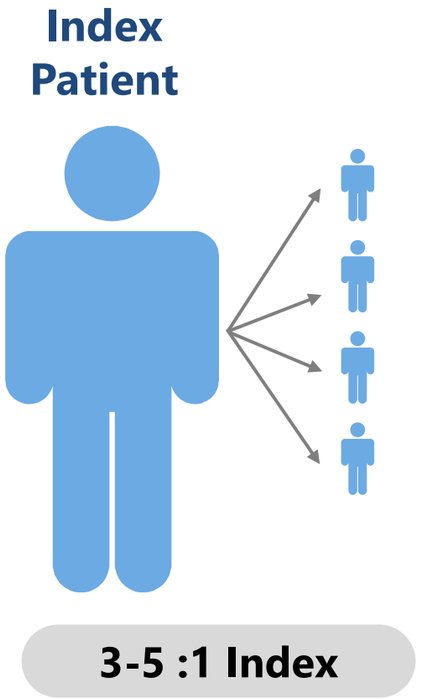
Fabry Underdiagnosis

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

Burton 2017 J Pediatr 2017;190:130-5 ; Mechtler *et al.*, **The Lancet**, 2011 Dec. Hwu *et al.*, **Hum Mutation**, 2009 Jun; Spada *et al.*, **Am J Human Genet.**, 2006 Jul

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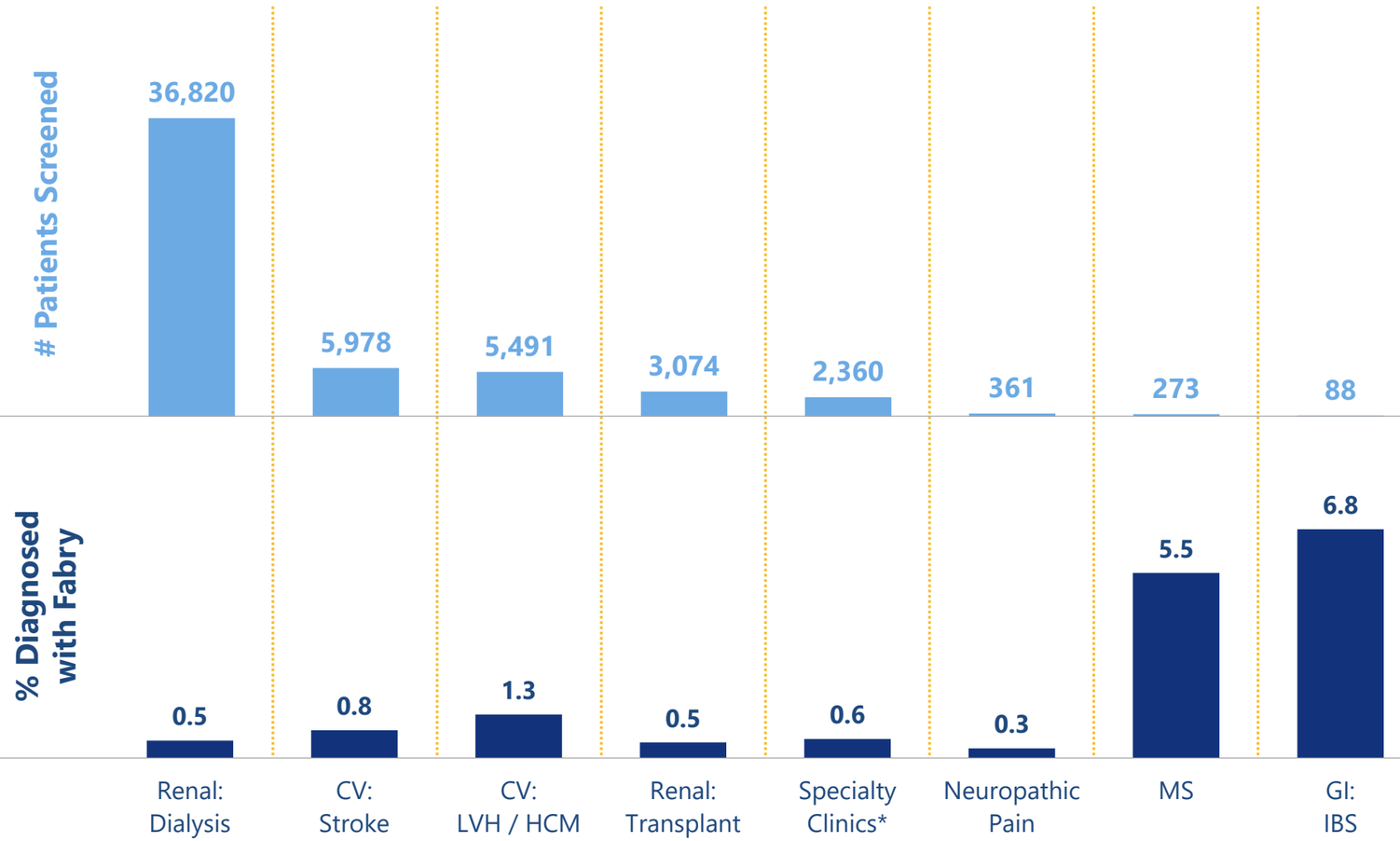
Fabry Misdiagnosis

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

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, 2018)

One of the Most Successful Rare Disease Launches

~$91M*
FY18 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.

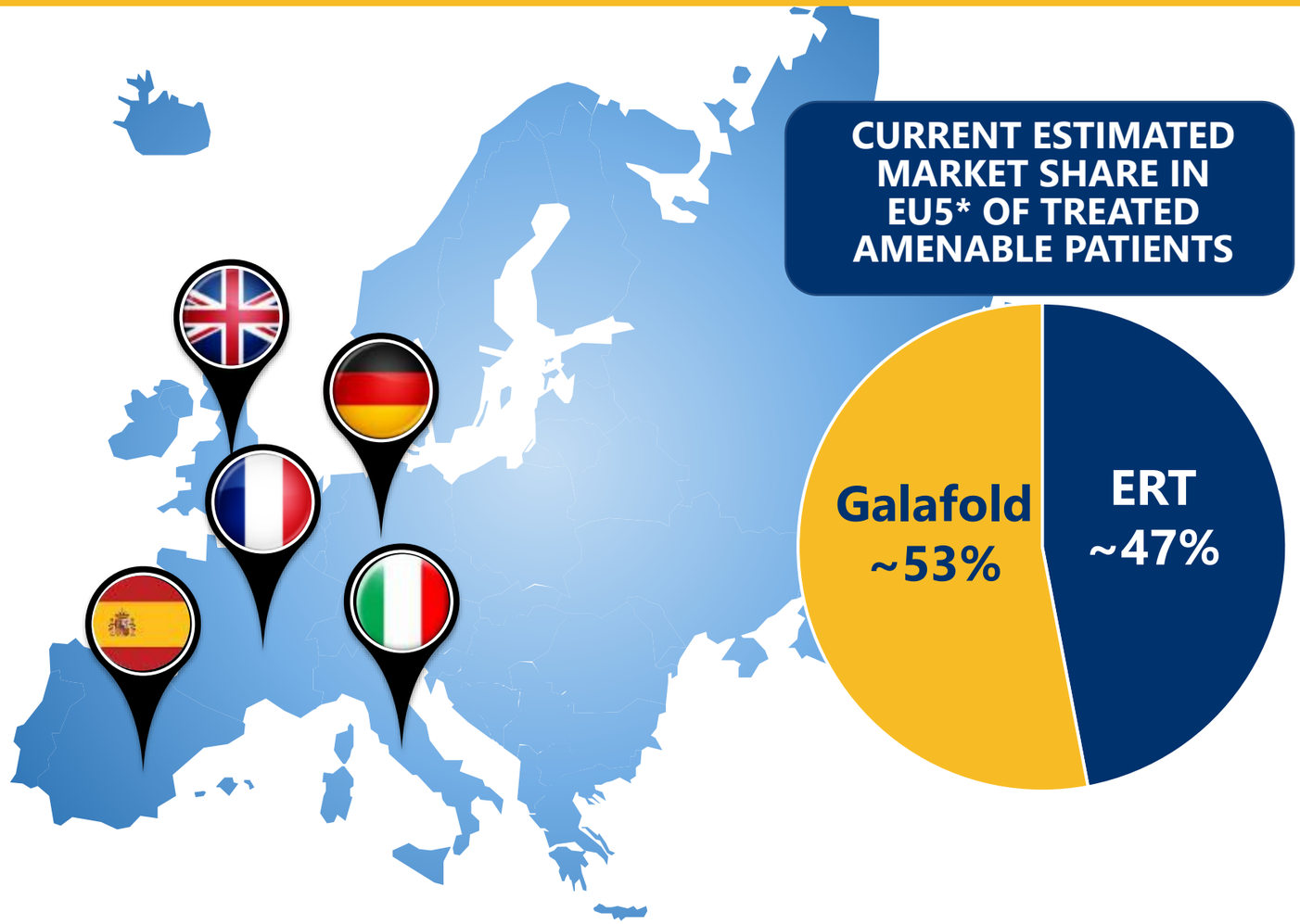
348
Amenable Variants in U.S. Label

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.

*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

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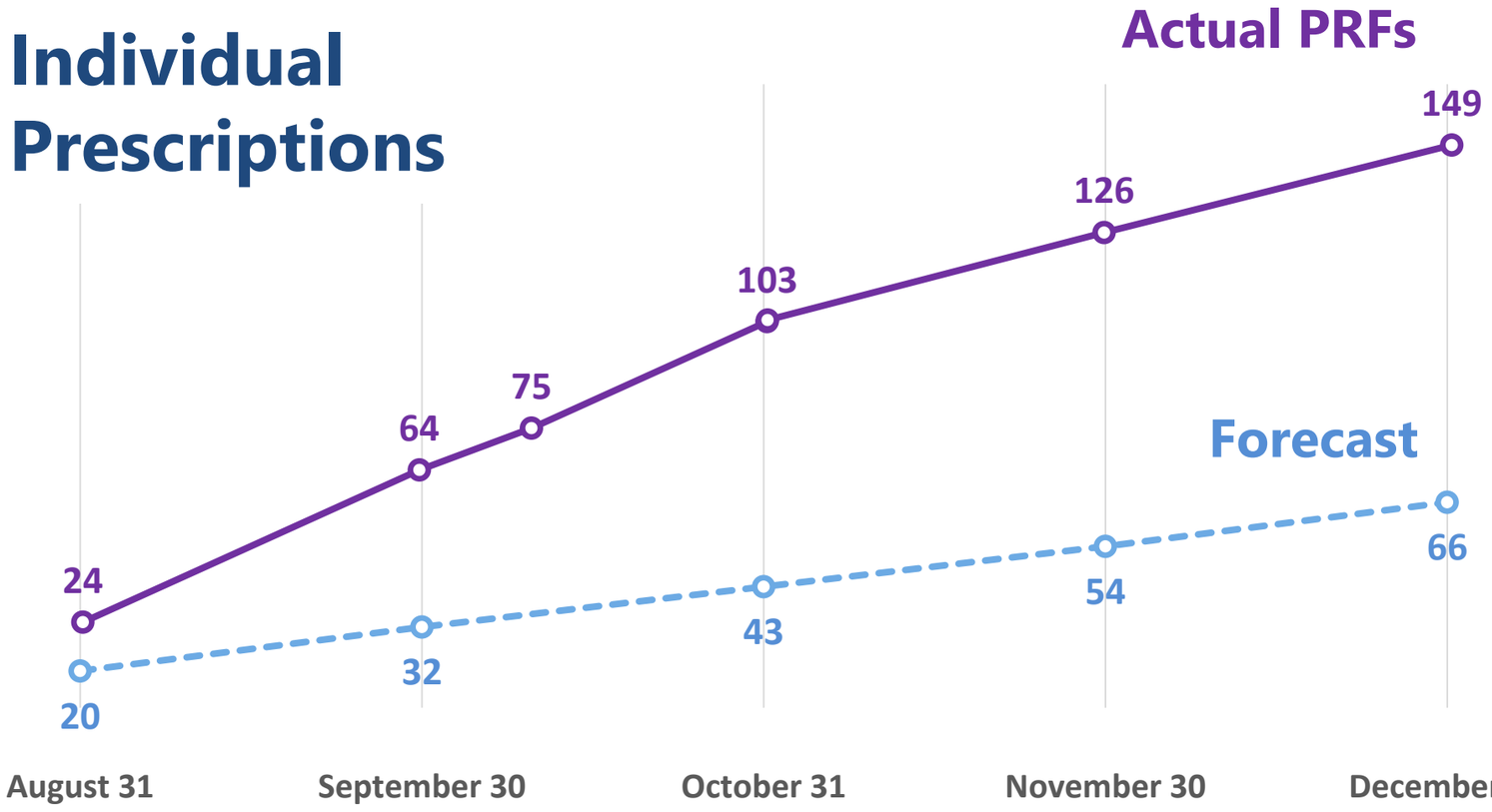
Key U.S. Launch Metric – Individual Prescriptions (Patient Referral Forms)

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

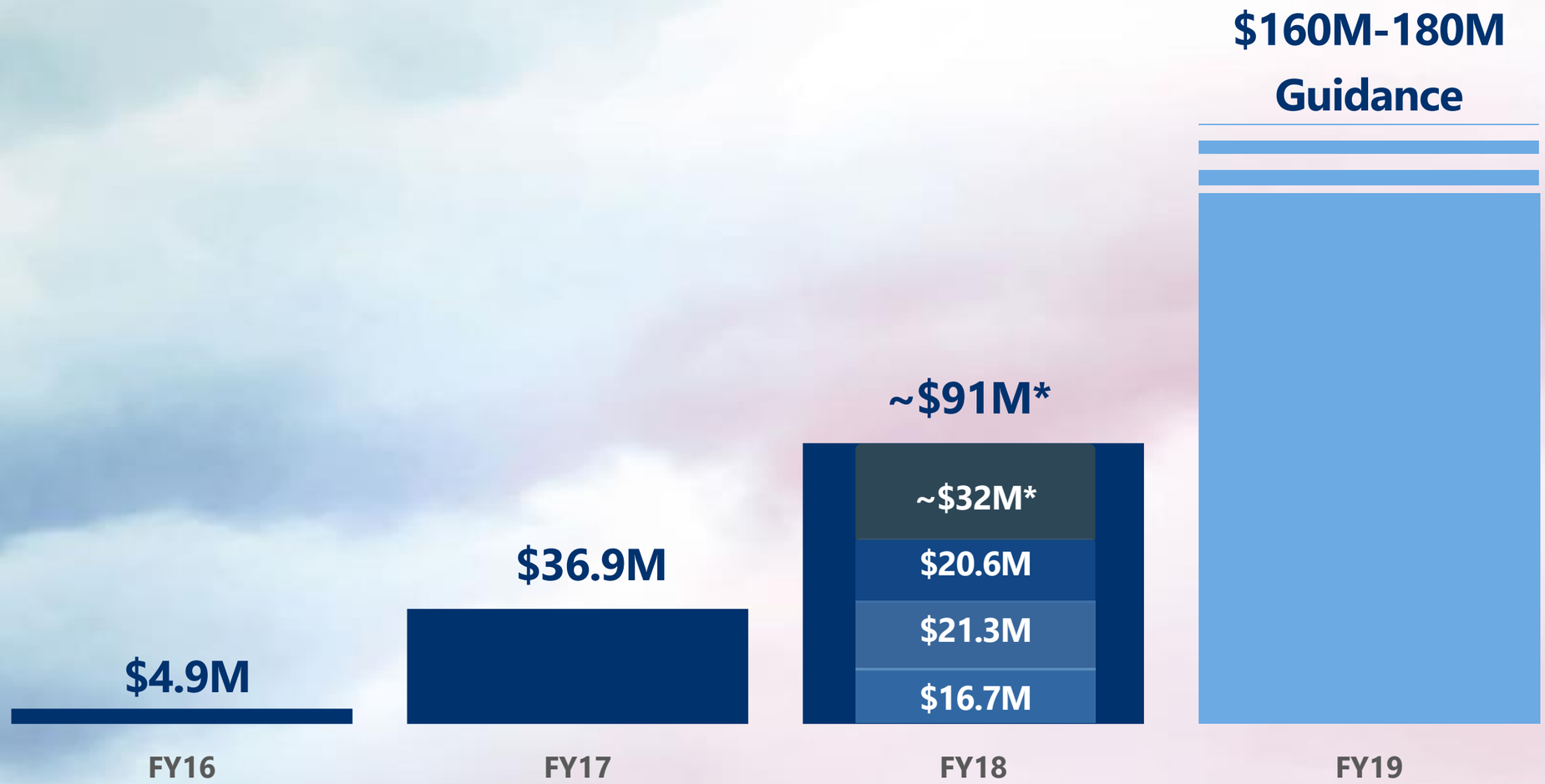


Market Dynamics

- 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

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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

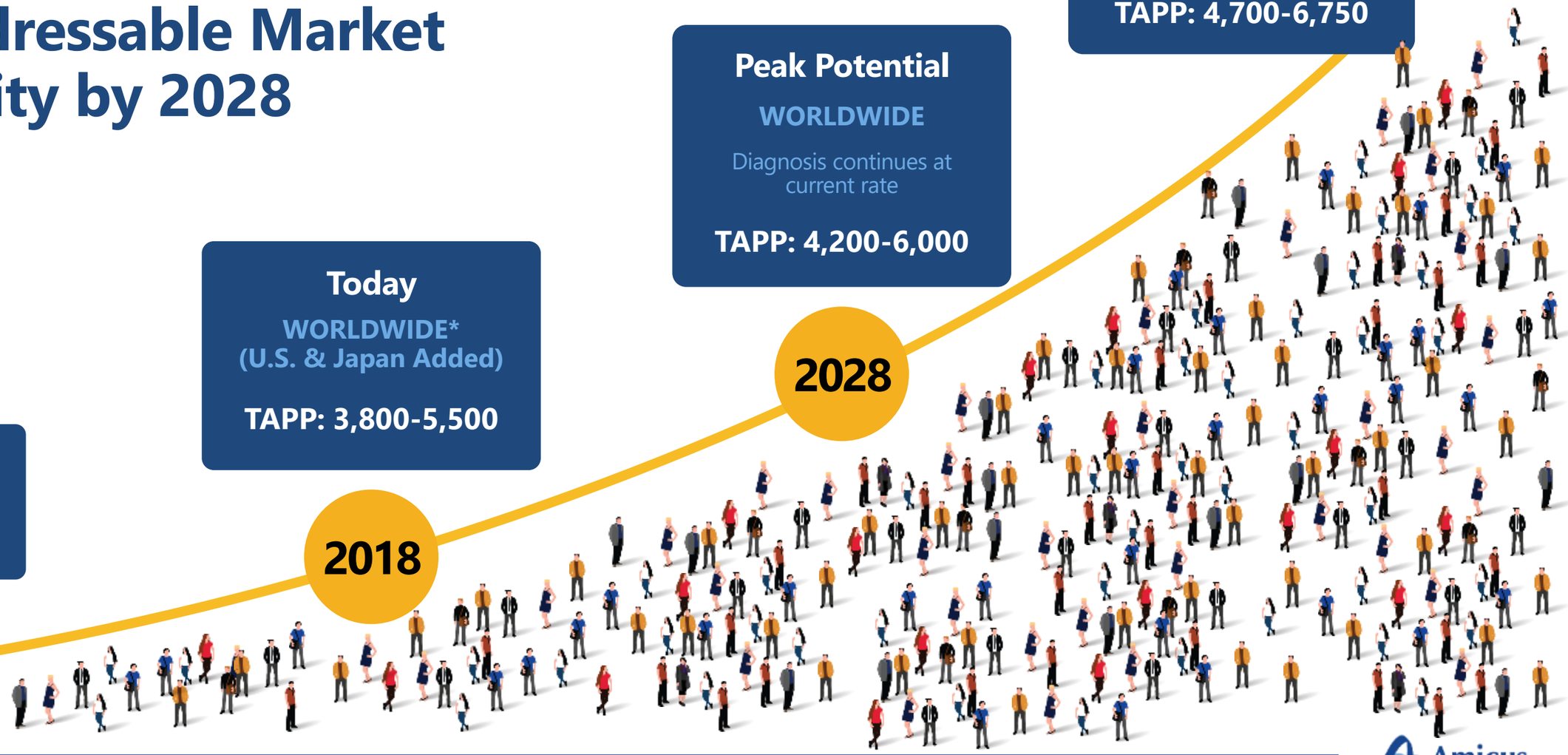
TAPP: 4,200-6,000

Upside Potential

WORLDWIDE

Diagnosis grows due to newborn screening 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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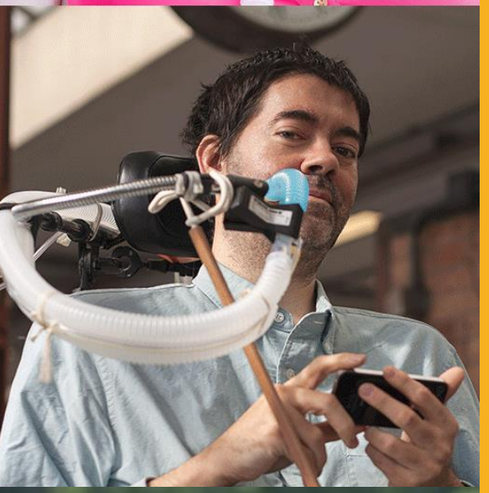


AT-GAA Novel ERT for Pompe Disease

“We encourage and embrace constant innovation”

- Amicus Belief Statement

Pompe Disease Overview

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

5,000 – 10,000 patients diagnosed WW[1]

Respiratory and cardiac failure are leading causes of morbidity and mortality

Age of onset ranges from infancy to adulthood

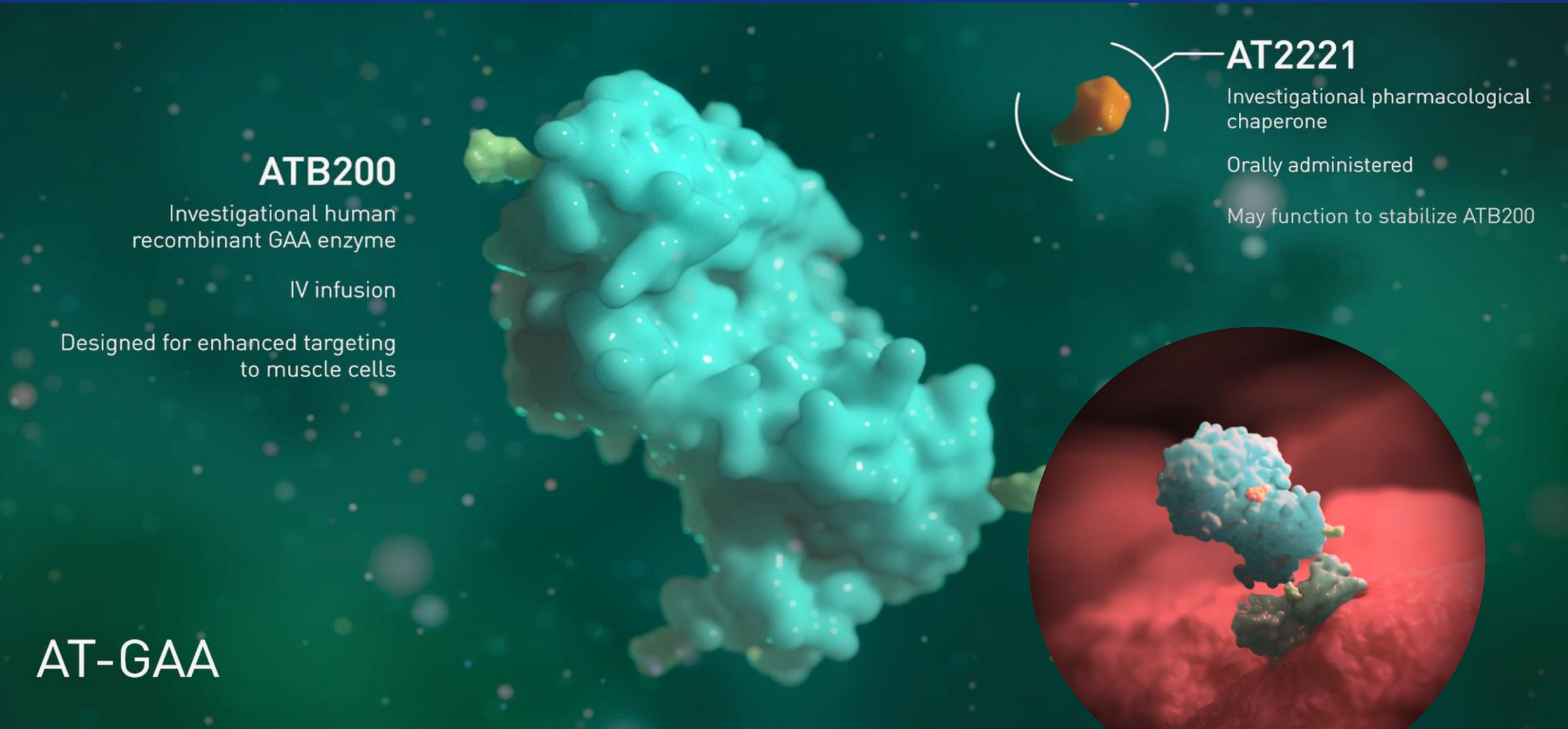
Deficiency of GAA leading to glycogen accumulation

Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

~$900M+ Global Pompe ERT sales in FY17[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 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)

Amicus Therapeutics

PROPEL (ATB200-03) Study Design

52-Week Primary Treatment Period (Double-Blind)

Long-Term Extension (Open-Label)

Participants with Late-Onset Pompe Disease

~100 Patients
90 Clinical Sites Worldwide

ERT-Switch
ERT-Naïve

AT-GAA
Bi-Weekly

Standard of Care
Bi-Weekly

AT-GAA
Bi-weekly

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

Pompe Biologics Manufacturing

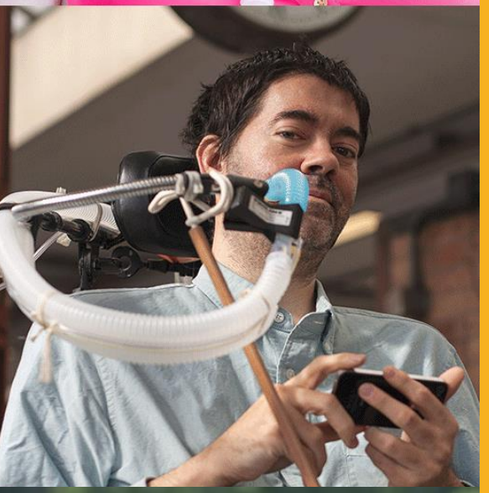
Successful Scale Up to 1000L GMP Clinical and Commercial Scale to Fully Supply Global Pompe Population

- **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**

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

Nationwide Children's

Gene Therapy Program

Penn

Amicus Therapeutics

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		

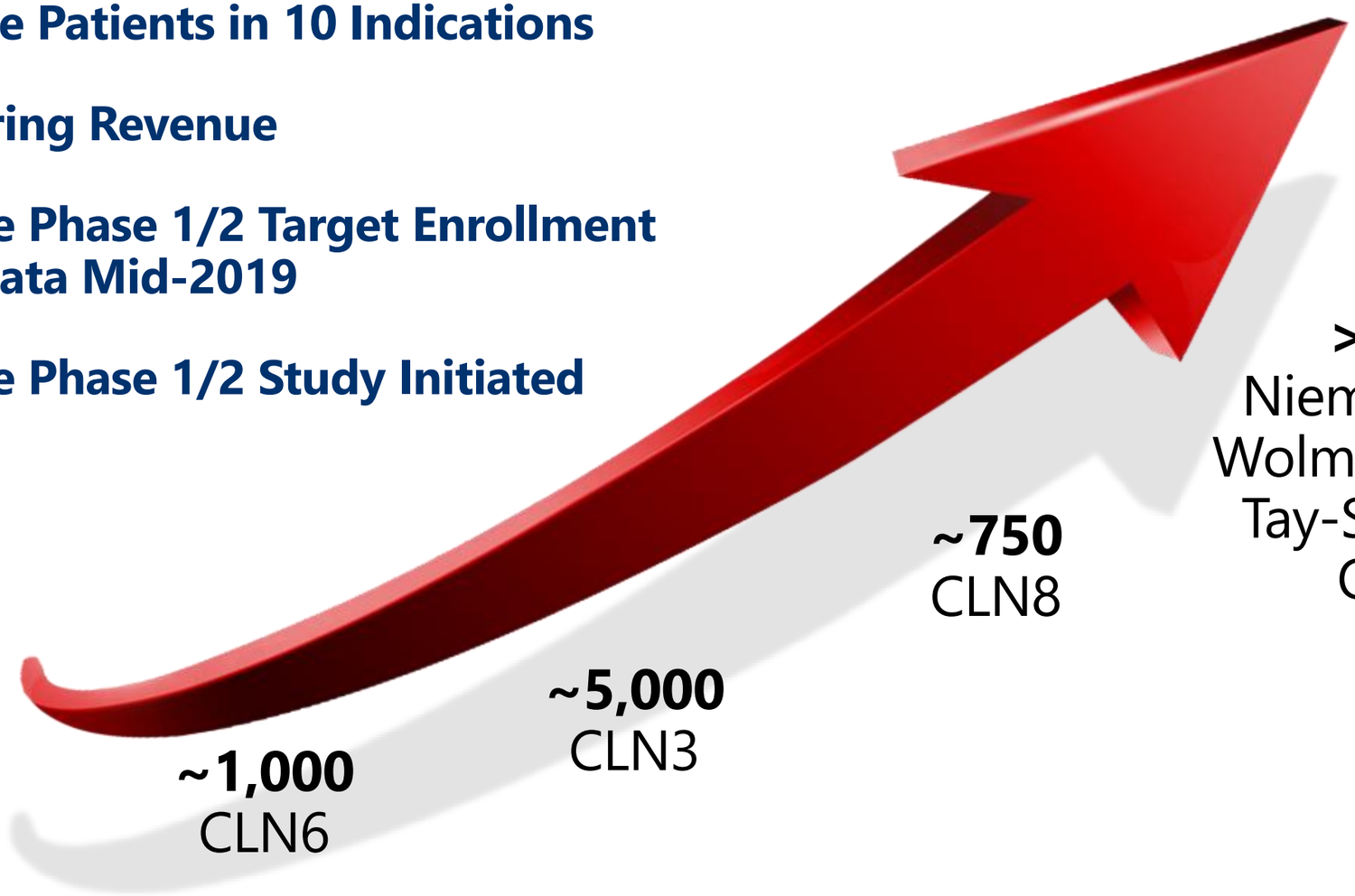
Addressable Patient Populations in Neurologic LSDs*

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



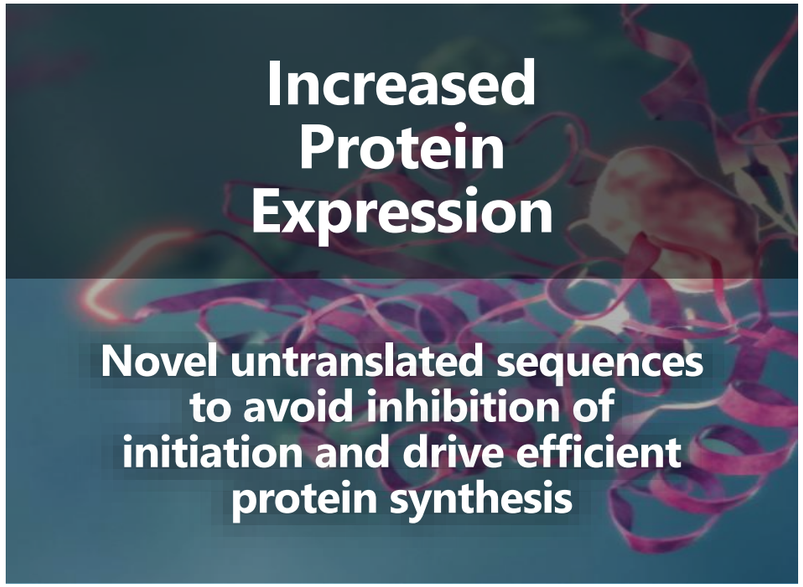
Nationwide Children's

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

Amicus Therapeutics

Amicus Protein Engineering Expertise & Technologies for Gene Therapy

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



GENE THERAPY PROGRAM

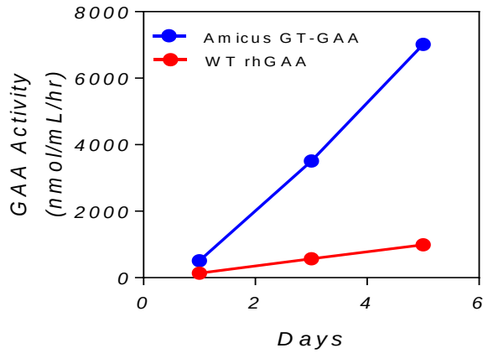
Penn

Early Proof of Principle for Optimized Gene Therapy

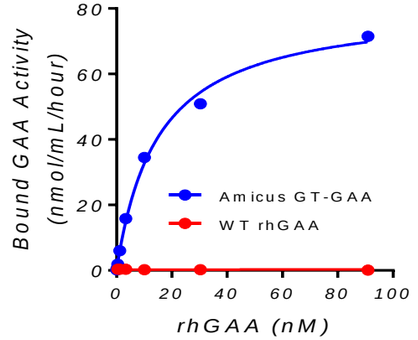
Amicus DNA Constructs Enable Optimized Gene Therapy in Pompe and Fabry

Pompe

Secreted GAA in Media

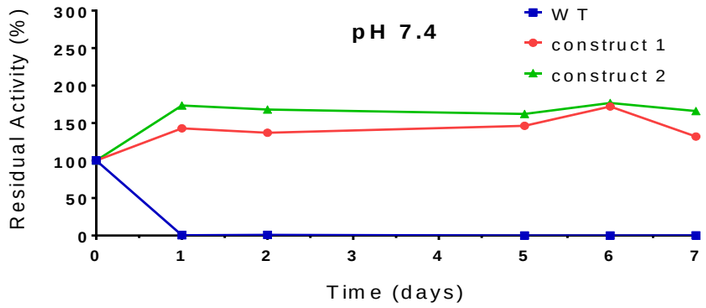


GAA Binding to Intended Receptor

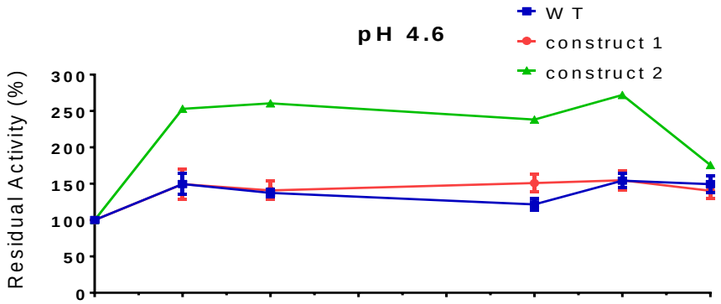


Fabry

Alpha-Gal Activity: pH 7.4

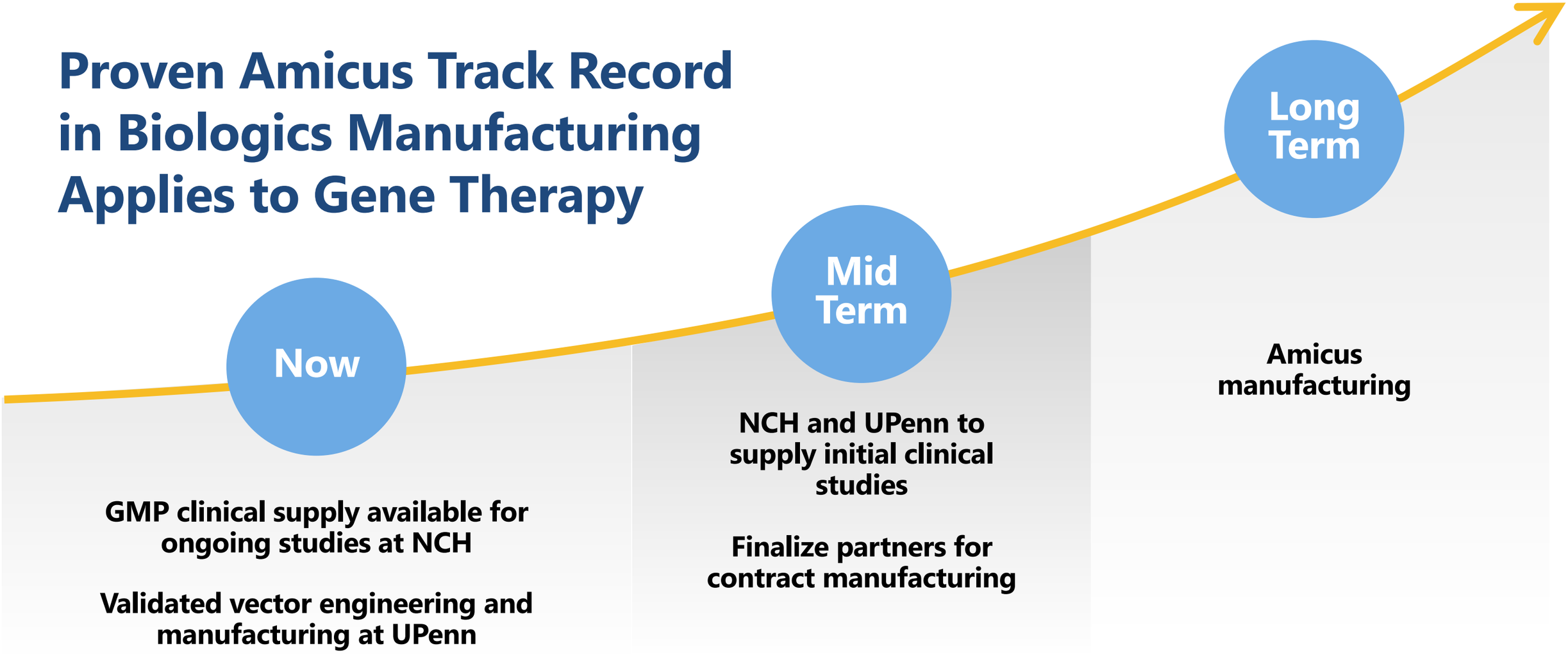


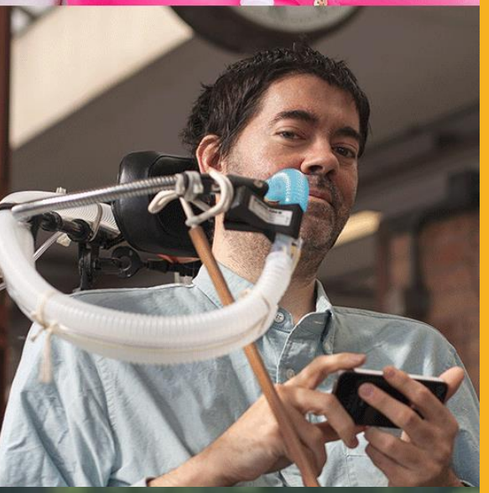
Alpha-Gal Activity: pH 4.6



Manufacturing: Three-Pronged Approach

Proven Amicus Track Record in Biologics Manufacturing Applies to Gene Therapy





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
Cash[1]	~$505M
Cash Runway	Into at least mid-2021
CAPITALIZATION	
Shares Outstanding[1]	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

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

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement

Amicus Therapeutics®