



# Corporate Overview

June 2018

# Safe Harbor

*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 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 ended March 31, 2018 filed May 9, 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 presentation to reflect events or circumstances after the date hereof.*

# Amicus Founding Beliefs

## WE BELIEVE...

**In the Fight to Remain  
at the Forefront of  
Therapies for Rare and  
Orphan Diseases**

- We seek to deliver the highest quality therapies for persons living with these diseases
- We support the disease communities - and their families
- We are passionate about what we do
- 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 keep asking the tough questions
- We will never be constrained by prior thinking
- We learn from our mistakes
- We think differently - very differently

## WE BELIEVE...

**In Our Future to Build  
Long-term Value for  
Our Stakeholders**

- We are all owners of this business
- We are business led and science driven
- Maximizing value for our shareholders is the foundation of our future successes
- Our medicines must be fairly priced and broadly accessible
- We build strategic partnerships
- We will not lie, cheat or steal
- We take full responsibility for our actions

## WE BELIEVE...

**In Each Other to  
Foster Teamwork and  
Respect for Each  
Individual's  
Contribution**

- Our passion for making a difference unites us
- Diversity of experience and thought is essential
- We communicate openly, honestly and respectfully
- Our families are part of the Amicus experience
- Work-life balance keeps us healthy

# Amicus Founding Beliefs

WE BELIEVE...

**We push ideas as far and as fast as possible**

Orphan Diseases

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
- Our diversity of experience and thought is essential
- We communicate openly
- Work-life balance keeps us healthy

## Amicus Mission

*We seek to deliver the highest quality therapies for persons living with rare metabolic diseases*

# Amicus Vision: Delivering for Patients and Shareholders

To build a top-tier, fully integrated, global biotechnology company whose medicines treat 5,000+ patients with \$1B+ in worldwide sales revenue by 2023



>350 Patients\* | \$36.9M Global Sales



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

YE17

2023

\*Clinical & commercial, all figures approximate

# Amicus Strategy

## Strategic Goals:

Create...

Manufacture...

Test...

Deliver...

...Great Medicines

## Critical Initiatives:

Invest in core internal scientific technologies

Actively in-license complementary products and technologies in rare metabolic diseases

Strengthen and expand relationships with WuXi Biologics and other core manufacturing partners

Build internal capabilities and capacity for biologics manufacturing

Complete build-out of global commercial and development footprint with world-class teams

Apply highest levels of business ethics and social responsibility

# Amicus Today



FIRST ORAL PRECISION MEDICINE  
FOR FABRY DISEASE

**ATB200/AT2221**  
NOVEL TREATMENT  
PARADIGM for  
Pompe Completed Phase 1/2

PRECLINICAL  
**PIPELINE**  
of products for rare  
metabolic diseases

**BIOLOGICS**  
PLATFORM

Protein Engineering  
& Glycobiology



CHAPERONE-ADVANCED  
REPLACEMENT THERAPY

**SMALL  
MOLECULE**  
Pharmacological  
Chaperones

**~400**  
**EMPLOYEES**  
**globally**

**~\$600M**  
Cash  
(3/31/18)

**GLOBAL  
FOOTPRINT**  
in 27 countries



# Our Passion for Making a Difference Unites Us



# Excellence in Execution in 2017

**Successful Achievement of FOUR Key Strategic Priorities in 2017 to Build a Top Global Biotechnology Company Focused on Rare Metabolic Diseases**

**1 Advance International Galafold Launch (Target 300 Patients)**



**2 Submit Japanese and U.S. NDAs for Migalastat**



**3 Establish Definitive Proof of Concept for AT-GAA\***



**4 Maintain financial strength**



\*Advanced and Targeted GAA (AT-GAA, also known as ATB200/AT2221)

# 2018 Key Strategic Priorities

Focused on FIVE Key Strategic Priorities in 2018

- 1** Double Galafold (migalastat) revenue to \$75-\$85M
- 2** Secure approvals for migalastat in Japan and the U.S.
- 3** Achieve clinical, manufacturing and 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 financial strength

# Building a World Class Organization

Global Organization of ~400 Employees Dedicated to Create, Manufacture, Test, and Deliver Medicines for Rare Metabolic Diseases





# Galafold™ (Migalastat) Precision Medicine for Fabry Disease

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

- Amicus Belief Statement

# Fabry Disease Overview

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

## Leading Causes of Death:

**TRANSIENT ISCHEMIC ATTACK (TIA) & STROKE<sup>1</sup>**

**HEART DISEASE<sup>2</sup>**

**KIDNEY DISEASE<sup>3</sup>**

## Life-Limiting Symptoms:

**GASTROINTESTINAL<sup>3</sup>**

## Key Facts:

- $\alpha$ -Gal A enzyme deficiency leads to substrate (GL-3) accumulation
- >1,000 known mutations
- ~10K diagnosed WW (51% female/49% male<sup>4</sup>)
- Newborn screening studies suggest prevalence of ~1:1000 to ~1:4000

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

# Galafold Snapshot (as of March 31, 2018)

**\$16.7**  
1Q18 Galafold  
Revenue

**\$75-  
\$85M**  
FY18 Galafold  
Revenue Guidance

**2**  
Pending Regulatory  
Approvals (U.S.,  
Taiwan)

 **Galafold**  
(migalastat)

**19**  
Countries with  
Pricing &  
Reimbursement

**7**  
Regulatory  
Approvals\*

**348**  
Amenable  
Mutations in EU  
Label

**FIRST Oral  
Precision Medicine  
for Fabry Disease**

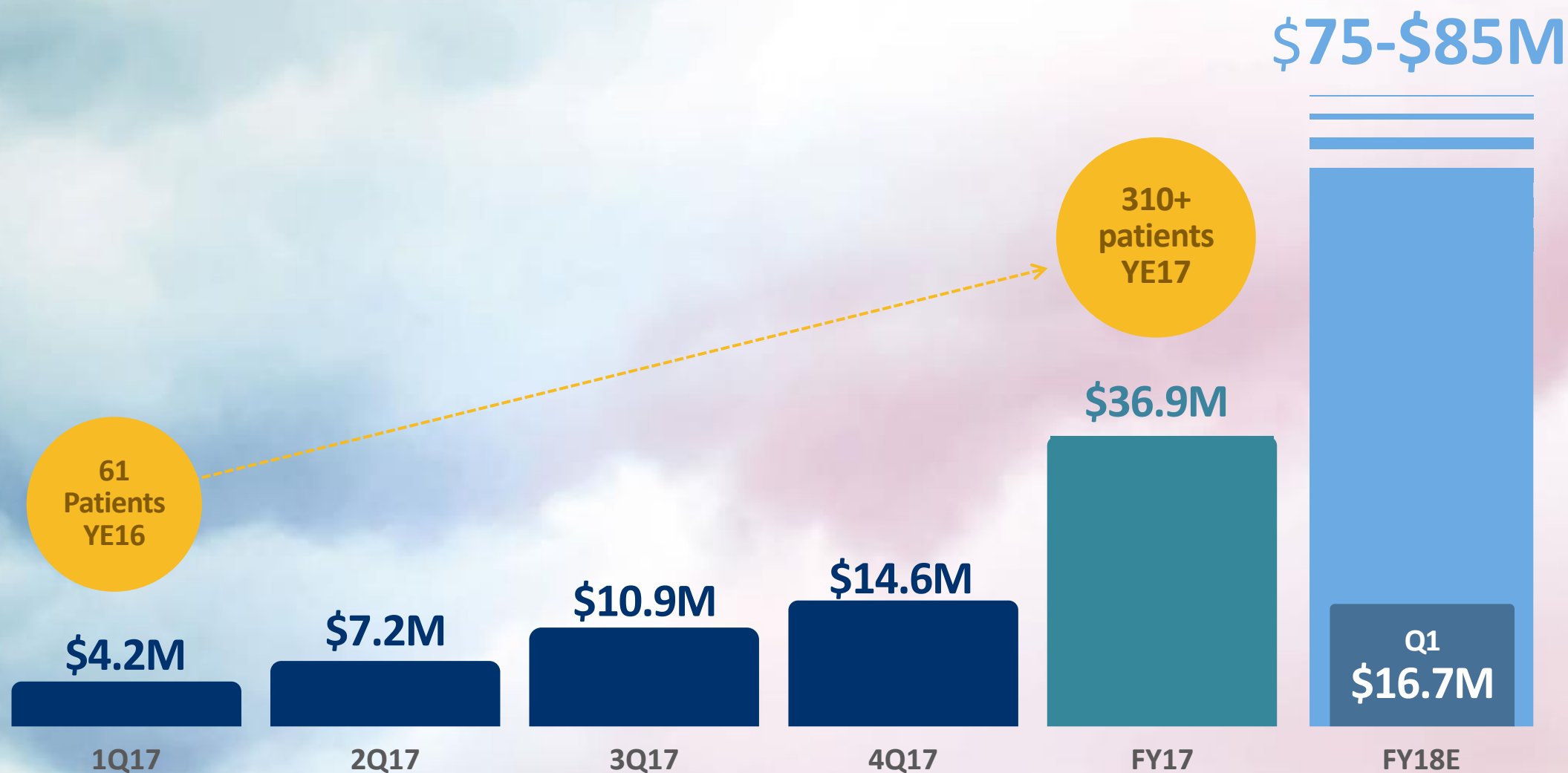
**Galafold Indicated  
for Long-Term  
Treatment of  
Adults and  
Adolescents Aged  
≥ 16 years with a  
Confirmed  
Diagnosis of Fabry  
Disease and Who  
Have an Amenable  
Mutation\*\***

\*EU, Australia, Canada, Japan, Israel, Switzerland, South Korea

\*\*For important safety information for Galafold visit [www.ema.europa.eu](http://www.ema.europa.eu).

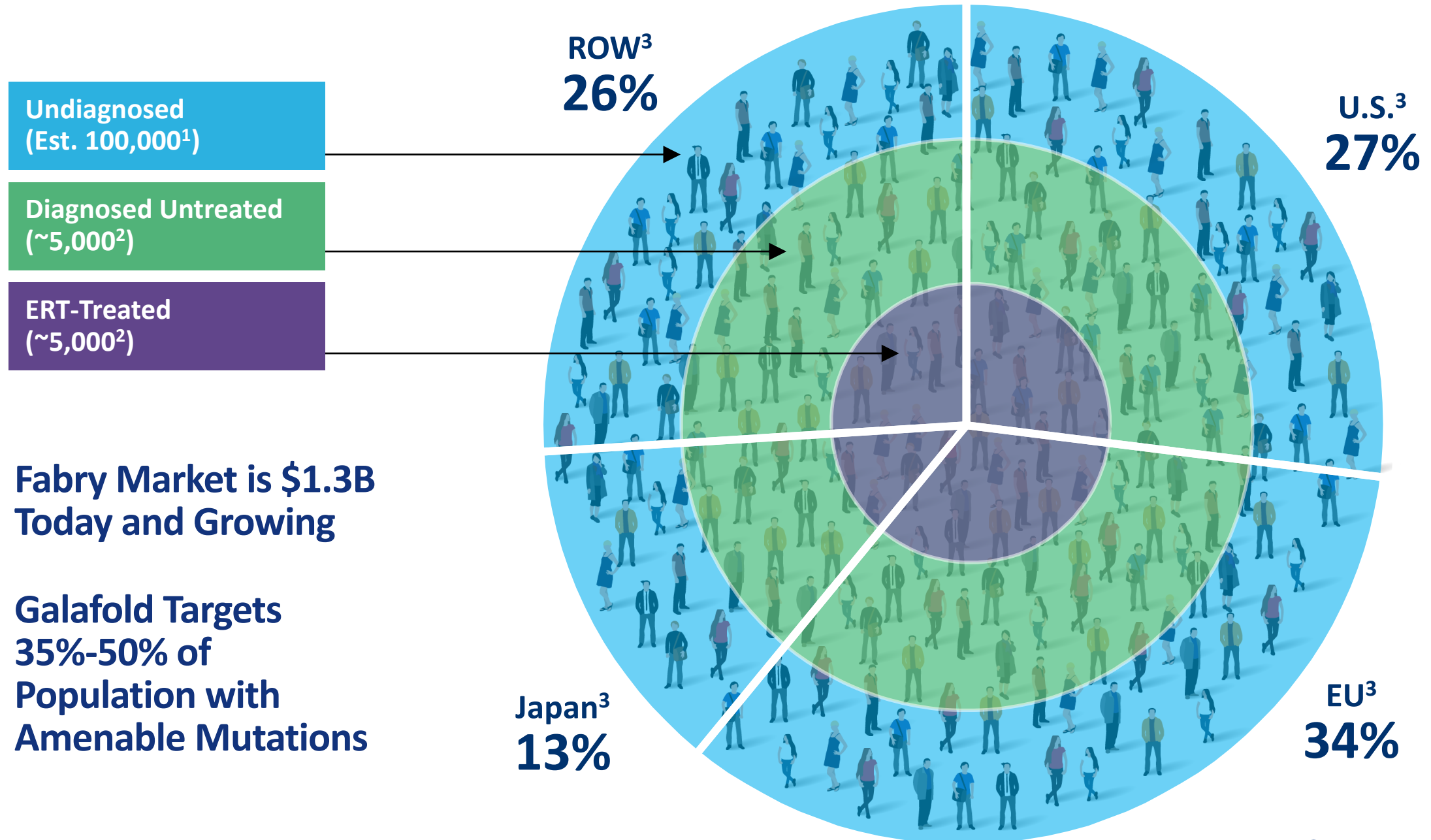
# Galafold Success and FY18 Galafold Revenue Guidance

International Launch Success Positions for Significant Growth in 2018  
and \$500M+ Global Peak Sales Opportunity





# Galafold \$500M+ Global Peak Revenue Opportunity



# Fabry Franchise Strategy

**Galafold for Patients with Amenable Mutations**

**EU Infrastructure for Initial Launch**

**Expansion to U.S., Japan, ROW**

**Next-Generation Therapies**

**Continued Innovation for ALL Fabry Patients**

# Fabry Precision Medicine Driven by a Patient's Genotype

**Amicus Therapeutics  
is Committed to  
Delivering the  
Highest Quality  
Therapies**

**Migalastat**  
Oral Precision Medicine



**~\$1.3B**  
Global Fabry  
Market Today



Growing to  
**~\$2B**  
Global Fabry Market



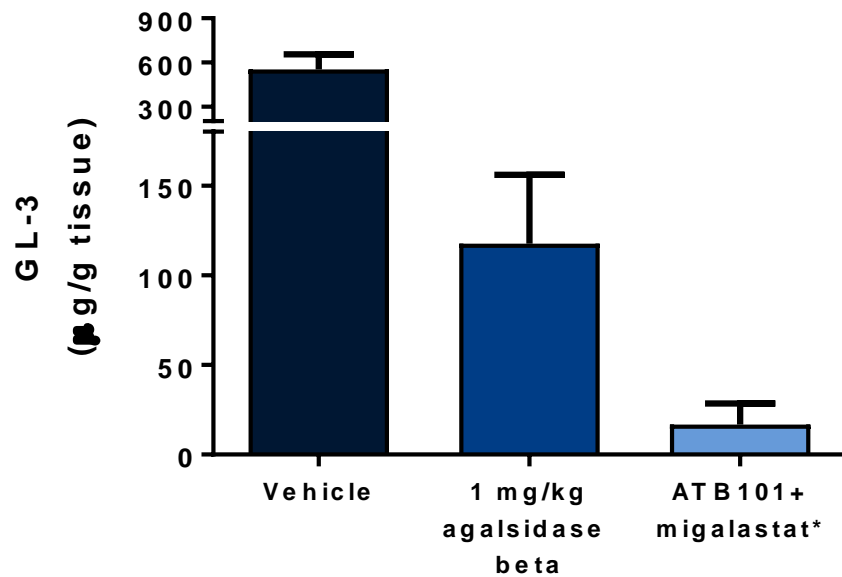
**Novel ERT Co-Formulated  
with Migalastat**



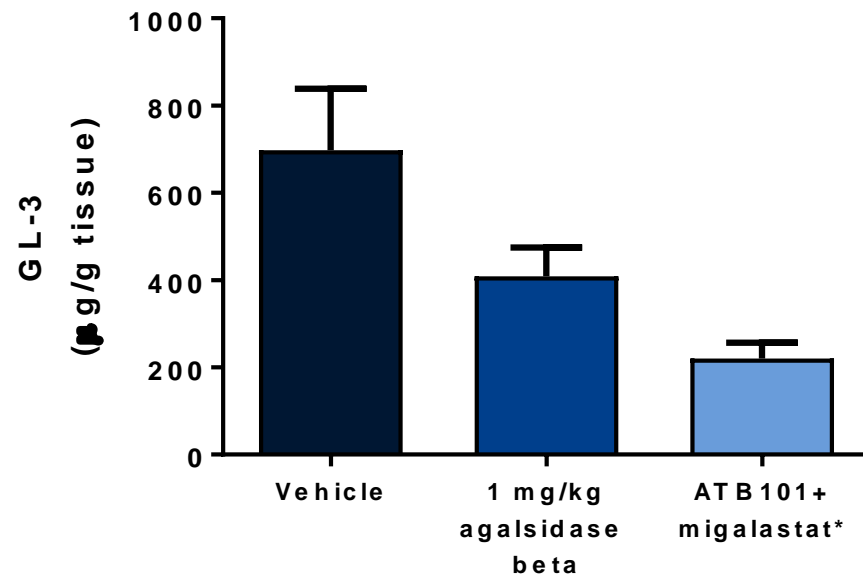
# Amicus Proprietary ERT Preclinical Proof of Concept

## ATB101 Co-formulated with Migalastat Results in Significantly Greater Substrate Reduction In Fabry KO Model

### Heart



### Kidney



Notes: \*3 mg/kg ATB101 + 10 mg/kg AT1001; Data from Gla KO mice administered two bi-weekly doses; p<0.05



# ATB200 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<sup>1</sup>

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

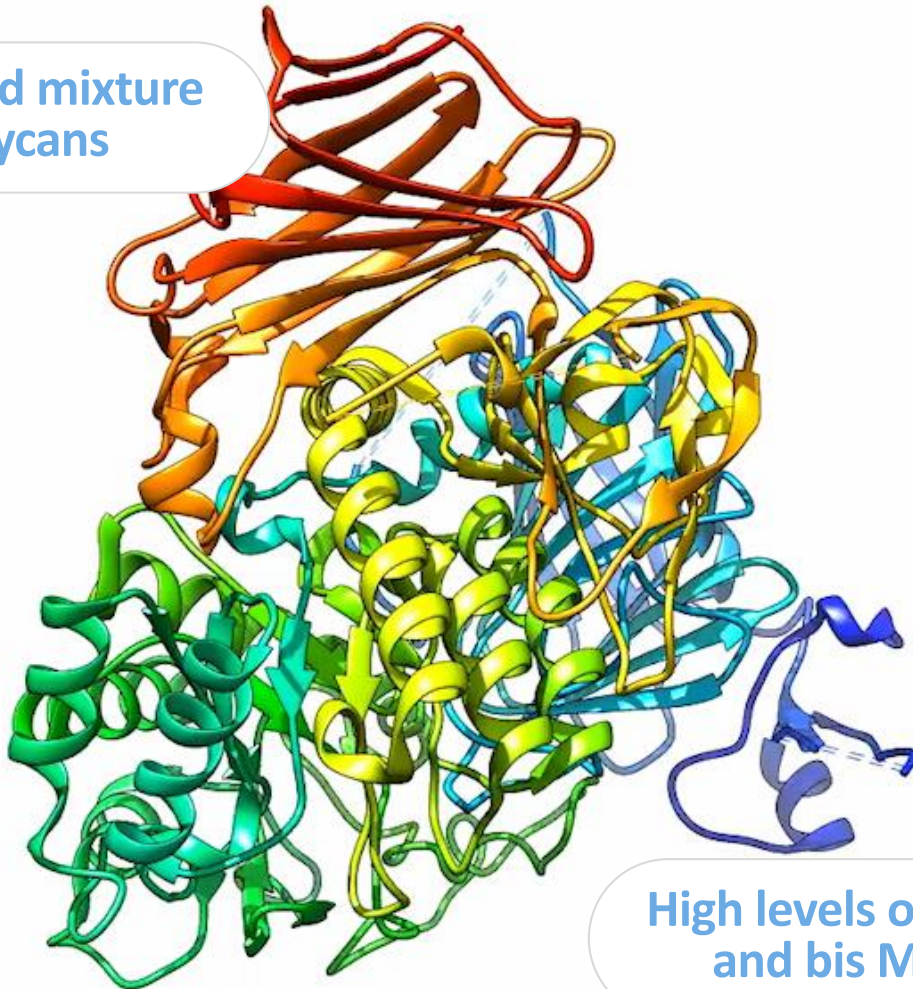
~\$900M+ Global Pompe ERT sales in FY17<sup>2</sup>

# ATB200 + Chaperone: A Differentiated Treatment Paradigm

## Application of Platform Technologies for Potential New Treatment Paradigm

### ATB200 (Novel ERT)

Optimized mixture  
of glycans



High levels of M6P  
and bis M6P



CHAPERONE-ADVANCED  
REPLACEMENT THERAPY

Chaperone  
addition



# 6-Minute Walk Test (6MWT) and Forced Vital Capacity (FVC) (as of 2/7/18)

Improvements in Key Functional Measure in both ERT-Naïve and ERT-Switch at Months Six and Nine with Continued Benefit Out to Month 12

## 6-Minute Walk Test (m)

Cohort	Baseline (n=10)	Change at Month 6 (n=10) Mean (SD)	Change at Month 9 (n=10) Mean (SD)	Change at Month 12 (n=8) Mean (SD)
Cohort 1 ERT-Switch Ambulatory	<b>397.2</b> (96.8)	<b>+23.9</b> (52.2)	<b>+24.5</b> (40.8)	<b>+57.4</b> (34.4)
Cohort	Baseline (n=5)	Change at Month 6 (n=5) Mean (SD)	Change at Month 9 (n=5) Mean (SD)	Change at Month 12 (n=2) Mean (SD)
Cohort 3 ERT-Naïve	<b>399.5</b> (83.5)	<b>+41.8</b> (29.4)	<b>+63.5</b> (23.1)	<b>+86.8</b> (11.1)

## FVC (% Predicted)

Cohort	Baseline (n=9)	Change at Month 6 (n=9) Mean (SD)	Change at Month 9 (n=9) Mean (SD)	Change at Month 12 (n=7) Mean (SD)
Cohort 1 ERT-Switch Ambulatory*	<b>52.6</b> (14.7)	<b>-1.3</b> (4.1)	<b>-1.7</b> (3.9)	<b>-3.1</b> (4.8)
Cohort	Baseline (n=5)	Change at Month 6 (n=5) Mean (SD)	Change at Month 9 (n=5) Mean (SD)	Change at Month 12 (n=2) Mean (SD)
Cohort 3 ERT-Naïve	<b>53.4</b> (20.3)	<b>+4.2</b> (5.6)	<b>+6.2</b> (5.3)	<b>+6.0</b> (7.1)

\*FVC not available for one subject



## 6-Minute Walk Test Patient-Level Data – Cohort 1 ERT-Switch (n=10) (as of 2/7/18)

6MWT Improved for ERT-Switch Patients at Months 6 and 9 With Continued Benefit Observed Out to Month 12

### 6-Minute Walk Test (m)

ID	Baseline	Change From Baseline		
		Month 6	Month 9	Month 12
1052	544	+51	+56	+112
1252	379	+125	+110	+103
1251	339	+21	+45	+73
1751	332	+8	+26	+45
1201	456	-5	+8	+41
1451	500	+55	+20	+33
1051	220	+29	+21	+30
1053	410	+38	+11	+22
1701	464	-4	-9	N/A
1601	328	-78	-43	N/A
<b>Mean (SD)</b>	<b>397.2</b> (96.8)	<b>+23.9</b> (52.2)	<b>+24.5</b> (40.8)	<b>+57.4</b> (34.4)

➤ 6MWT increased in 7/10, 8/10, and 8/8 ERT-switch patients at Months 6, 9 and 12 respectively

N/A = data not available (patients have not reached 12 month time point)

## 6-Minute Walk Test Patient Level Data – Cohort 3 ERT-Naïve (n=5) (as of 2/7/18)

All Five ERT-Naïve Patients Showed Increases in 6MWT Distance Out to Month 12

### 6-Minute Walk Test (m)

ID	Baseline	Change From Baseline		
		Month 6	Month 9	Month 12
3551	480	+41	+72	+95
3552	384	+62	+78	+79
3051	460	+79	+89	N/A
3554	406	+14	+44	N/A
3553	267	+13	+35	N/A
<b>Mean (SD)</b>	<b>399.5 (83.5)</b>	<b>+41.8 (29.4)</b>	<b>+63.5 (23.1)</b>	<b>+86.8 (11.1)</b>

➤ 6MWT increased in 5/5, 5/5, and 2/2 ERT-naïve patients at Months 6, 9 and 12 respectively

N/A = data not available (patients have not reached 12 month time point)

# Muscle Strength Testing : Cohort 2 Non-Ambulatory ERT-Switch (n=4) (as of 2/7/18)

## Substantial Increases Observed in Upper Extremity Strength in Non-Ambulatory ERT-Switch Patients at Month 6 and Month 9

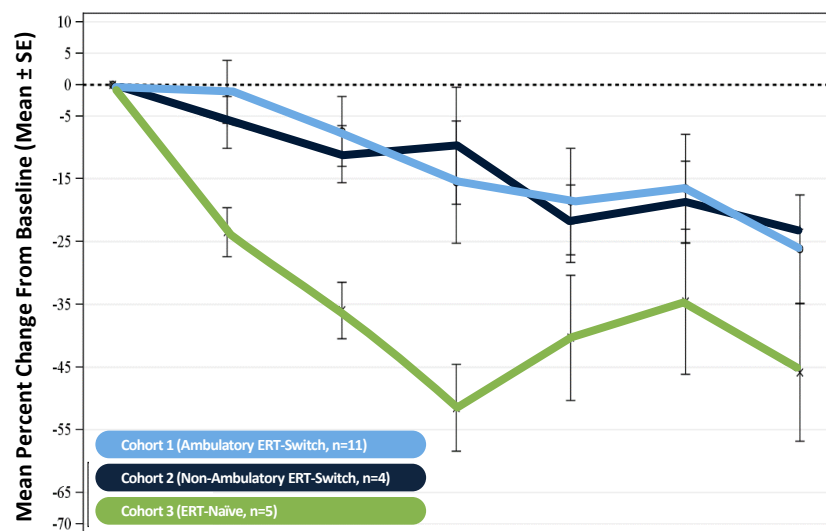
Assessment	Muscle Group Tested	Baseline (n=4)	Change to Month 6 (n=4)	Change to Month 9 (n=4)
QMT- Quantitative Muscle Testing - Dynamometer (pounds force)	Shoulder Adduction *	5.7 (8.8)	+8.1 (12.8)	+9.6 (12.3)
	Shoulder Abduction	16.7 (18.1)	+1.0 (6.6)	+0.5 (9.3)
	Elbow Flex	12.7 (13.7)	+2.4 (15.9)	+6.0 (19.3)
	Elbow Extension	12.3 (13.9)	+5.5 (4.7)	+7.5 (8.2)
Assessment	Muscle Group Tested	Baseline ** (n=3)	Change to Month 6 (n=3)	Change to Month 9 (n=3)
MMT - Manual Muscle Testing (manual score)	Shoulder Adduction	2.3 (2.1)	+1.3 (2.3)	0.0 (4.0)
	Shoulder Abduction	2.7 (2.3)	+0.5 (0.7)	-1.0 (2.7)
	Elbow Flex	4.3 (4.5)	+1.7 (1.5)	+1.7 (1.5)
	Elbow Extension	4.0 (4.0)	+1.7 (1.5)	+1.7 (1.5)

\* Shoulder adduction not available for one subject; \*\* Total Score MMT = 10 (R+L) N=3 due to assessment not being performed at some visits for some patients

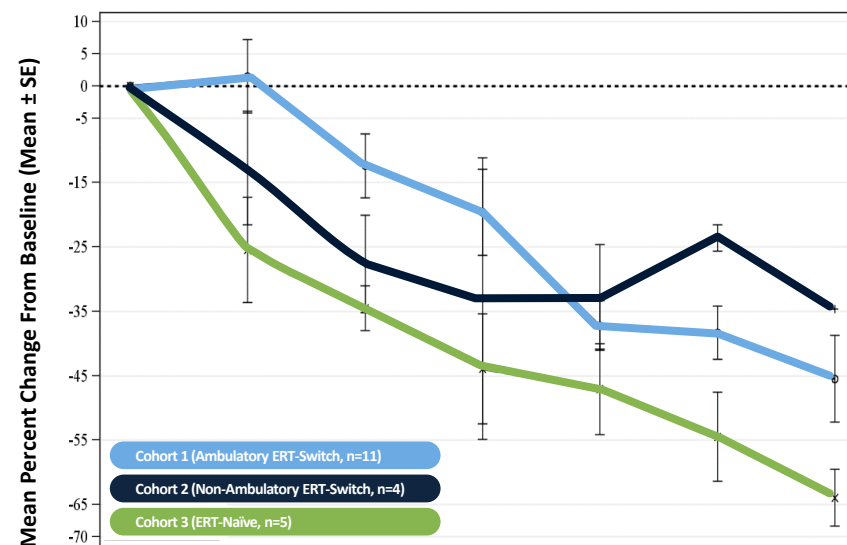
# Biomarkers (n=20) (as of 2/7/18)

All Cohorts Demonstrated Persistent Improvement in Biomarkers of Muscle Damage (CK) and Disease Substrate (Hex4) For Up To 12 Months

## Muscle Damage Biomarkers (% Change from Baseline for CK)



## Disease Substrate Biomarker (% Change from Baseline for Hex 4)



CK=creatinine kinase; Hex4=urine hexose tetrasaccharide.  
Missing values either unable to be analyzed or not yet analyzed.

# Safety Summary (n=20)\* (as of 2/7/18)

## AEs Have Been Generally Mild and Transient with Very Low Rate of Infusion-Associated Reactions (< 1%) After 550+ Total Infusions Across All Cohorts

- AEs were generally mild and transient
  - Most common treatment emergent AEs (TEAEs) were abdominal pain\*\* (8/20), diarrhea (8/20), nasopharyngitis (6/20), nausea (5/20), headache (5/20), upper respiratory tract infection (5/20).
- Three incidents of infusion-associated reactions in 550+ infusions which were controlled by standard premedication
  - One IAR event in one non-ambulatory ERT-switch patient (skin discoloration)
  - Two IAR events in one ERT-naïve patient (localized pruritus, erythema and burning sensation)
- Longest duration of treatment is 20+ months

AE, adverse events; IAR, infusion-association reaction.

\*Reported through interim data analysis (maximum 20+ months)

\*\*Includes upper and lower abdominal pain

# Key Clinical & Manufacturing Activities 2018

**Significant Progress Against Clinical and GMP Manufacturing Activities Ongoing in 2018 to Lay Foundation for Most Successful and Fastest Approval Pathways**

## CLINICAL

- ✓ Additional Phase 1/2 extension data presented at *WORLDSymposium*
- ✓ Additional patients in Phase 1/2 study (ongoing)
- ✓ Retrospective natural history of ERT-treated patients (ongoing)
- ✓ Prospective data collection on current ERT-treated patients (ongoing)
- Initiation of larger registration-directed study planned in 2018



## MANUFACTURING

- ✓ Additional 1000L GMP campaigns completed
- ✓ Added capacity to ensure sufficient medicines to supply patient population
- Final regulatory agreement on comparability between 1,000L and 250L GMP scale
- Release for clinic of 1,000L GMP commercial scale material
- Announce plan for long term commercial manufacture and capacity



# Pompe Regulatory Strategy

**A series of regulatory updates regarding a registration-directed study for full approval, manufacturing activities and the best and fastest path forward for AT-GAA**

- Significant progress in collaborative discussions with EMA and FDA since 4Q17
- Ongoing interactions include formal meetings scheduled with both agencies
  - EMA - scientific advice meeting and EU update anticipated in 2Q18
  - U.S. FDA – type C meeting and US update anticipated in 3Q18
- Goals of interactions and formal meetings
  - Alignment on design of pivotal and supportive studies for full approval
  - Discussion of potential conditional (EU) and accelerated (US) approval pathways
- A series of further iterative discussions with regulators and additional updates are expected as program advances, new data is collected, and registration-directed study initiated
- Goal continues to remain to determine the best and fastest pathway to approval

# Biologics Manufacturing Progress & Upcoming Milestones

## Maintaining Identical Key Quality Attributes Throughout Scale Up to 1000L

- ✓ Analytical and *in vivo* comparability studies completed between 250L and 1000L engineering batches
- ✓ FDA agreement on comparability between 250L GMP scale and 1000L engineering batches; and testing strategy for demonstrating comparability between 250L scale and 1000L GMP batches
- ✓ GMP Manufacturing Campaigns of Drug Substance and Drug Product at 1000L GMP Scale Successfully Completed
- Release of 1,000L GMP material for initiation of registration-directed study
- Final regulatory agreement on comparability between 1,000L and 250L GMP scale





# Long-Term Biologics Manufacturing Strategy

## Strategy for Added Capacity and Long-Term Commercial Manufacture

- ✓ Additional capacity at WuXi (China & Ireland) to ensure sufficient medicine to supply patient population
- ✓ Diligence ongoing for long-term U.S. commercial manufacture and capacity
- Project update anticipated 2H18



# Pipeline Strategy

*“We have a duty to obsolete our own technologies”*  
- Amicus Belief Statement

# Pipeline Strategy

**Sharply Focused on Developing Therapies for People Living with Rare Metabolic Diseases**

***Technology Landscape***

Enzyme Replacement Therapies (ERTs)

Gene Therapy/Editing

Blood-Brain Barrier Technologies

Small Molecules

***Development Criteria***

Obsolete Current Treatments

Significant Benefits for Patients

First/Best-in-Class

***Pipeline Expansion***

*One or more new clinical programs in 2019*



# Financial Summary & Key Milestones

*"We are business led and science driven"*

- Amicus Belief Statement

# Financial Summary & Guidance

**Strong Balance Sheet with \$605M Cash at 3/31/18 - Cash Runway into at Least 2021**

## FINANCIAL POSITION

March 31, 2018

Cash	\$605M
Debt	\$250M
Cash Runway <sup>1</sup>	Into at least 2021

## CAPITALIZATION

Shares Outstanding <sup>2</sup>	187,972,218
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## FINANCIAL GUIDANCE

FY18 Net Cash Spend Guidance	\$230-\$260M
Galafold Revenue Guidance	\$75-\$85M

<sup>1</sup>Based on existing operating plan for Fabry and Pompe programs. <sup>2</sup>Includes shares from the February 2018 equity offering

# 2018 Key Strategic Priorities

Focused on FIVE Key Strategic Priorities in 2018

- 1 Double Galafold (migalastat) revenue to \$75-\$85M**
- 2 Secure approvals for migalastat in Japan and the U.S.**
- 3 Achieve clinical, manufacturing and 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 financial strength**

# Amicus Vision: Delivering for Patients and Shareholders

To build a top-tier, fully integrated, global biotechnology company whose medicines treat 5,000+ patients with \$1B+ in worldwide sales revenue by 2023



>350 Patients\* | \$36.9M Global Sales



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

YE17

2023

\*Clinical & commercial, all figures approximate

# Thank You

*“Our passion for making a difference unites us”*

*-Amicus Belief Statement*







# Appendix

# Fabry Disease Overview

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

## Leading Causes of Death

### TRANSIENT ISCHEMIC ATTACK (TIA) & STROKE<sup>1</sup>

### HEART DISEASE<sup>2</sup>

- Irregular heartbeat (fast or slow)
- Heart attack or heart failure
- Enlarged heart

### KIDNEY DISEASE<sup>3</sup>

- Protein in the urine
- Decreased kidney function
- Kidney failure

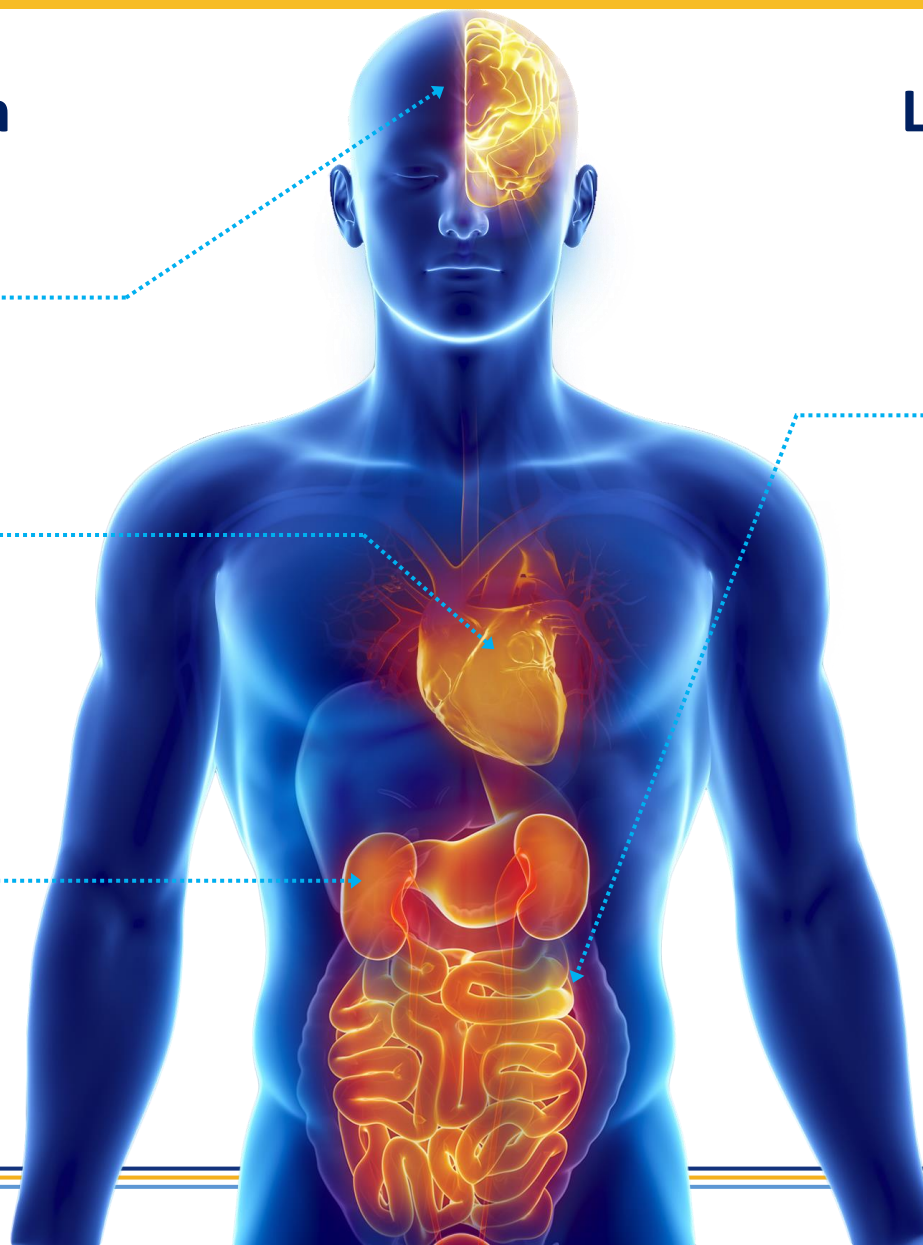
## Life-Limiting Symptoms

### GASTROINTESTINAL<sup>3</sup>

- Nausea, vomiting, cramping, and diarrhea
- Pain/bloating after eating, feeling full
- Constipation
- Difficulty managing weight

## Key Facts

- Deficiency of  $\alpha$ -Gal A enzyme leading to GL-3 accumulation
- >900 known mutations
- ~10K diagnosed WW (51% female/49% male<sup>4</sup>)
- Newborn screening studies suggest prevalence of ~1:1000 to ~1:4000



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

# Fabry Global Operations Excellence

## FABRY Initial Launch Success

### People

Deep experience in rare disease space

Hire “best and brightest” from range of leading biotech companies

Culture of strong patient focus

### Product

Differentiated safety and efficacy published in seminal journals\*

First-in-class oral therapy for Fabry

Precision medicine based on genotype

### Access

Compelling value proposition led to rapid reimbursement

Specialty distributor with high touch services

Commitment to patient access and support services

### Execution

Clear focus at launch on priority patient segments

Efficient outreach to key Fabry centers

Strong education efforts on importance of genotype