Amicus Therapeutics®

Barclays Global Healthcare Conference 2018

Bradley Campbell, President and Chief Operating Officer

March 15, 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 filed March 1, 2018. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this presentation to reflect events or circumstances after the date hereof.

Amicus Founding Beliefs

- 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 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

- 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 push ideas as far and as fast as possible

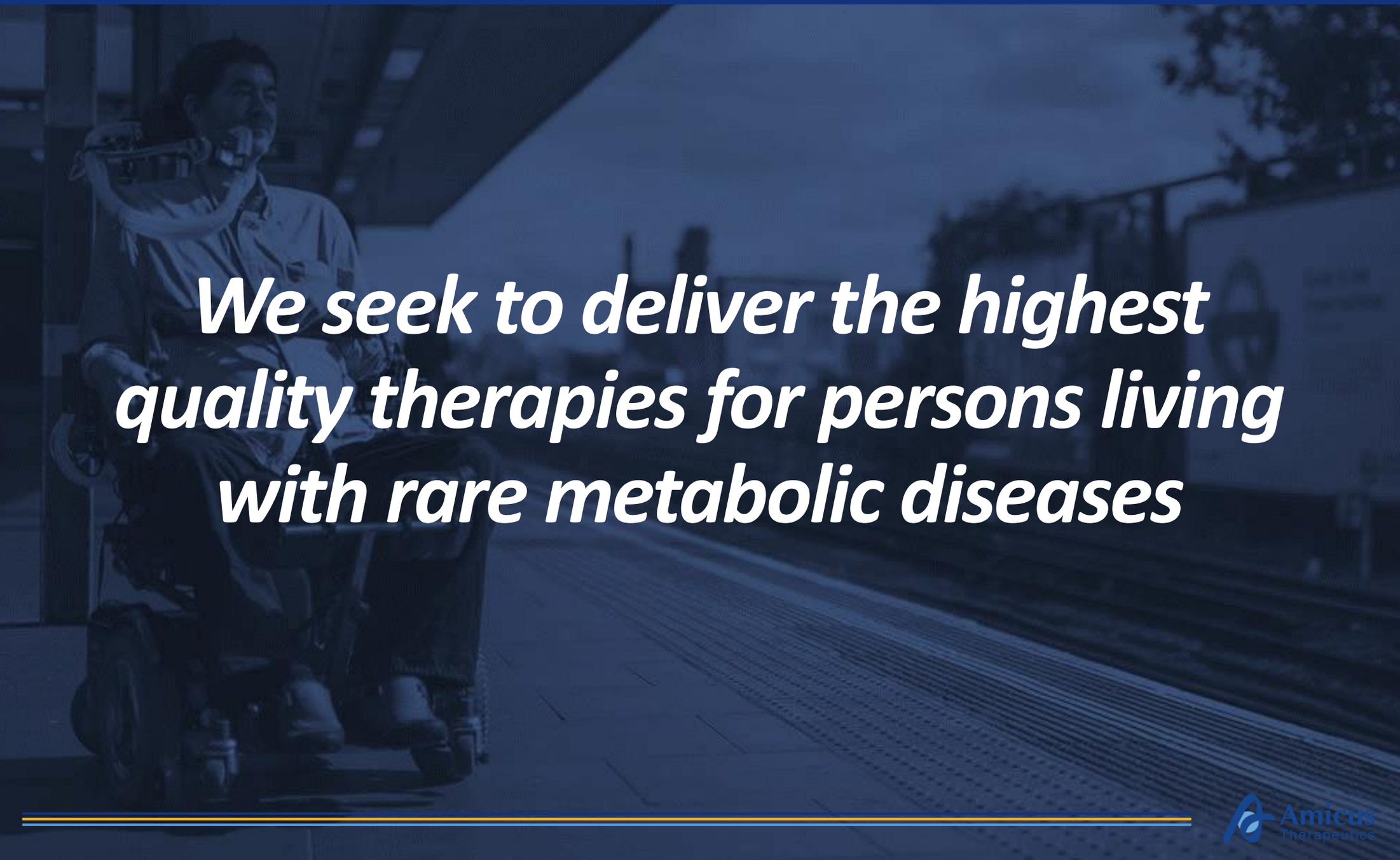
We encourage and embrace constant innovation

We have a duty to obsolete our own technologies

We are business led and science driven

Our passion for making a difference unites us

Amicus Mission



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 Therapeutics

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

Galafold (migalastat)

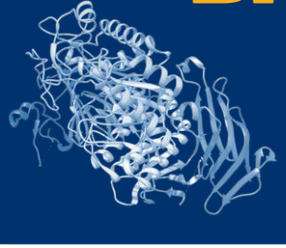
FIRST ORAL PRECISION MEDICINE FOR FABRY DISEASE

ATB200/AT2221

NOVEL TREATMENT PARADIGM for Pompe Completed Phase 1/2

PRECLINICAL PIPELINE

of products for rare metabolic diseases



BIOLOGICS PLATFORM

Protein Engineering & Glycobiology

CHART®

CHAPERONE-ADVANCED REPLACEMENT THERAPY

SMALL MOLECULE

Pharmacological Chaperones

~400 EMPLOYEES globally

~$359M

Cash (12/31/17)

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 ATB200/AT2221** ✓
4. **Maintain financial strength** ✓

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 ATB200/AT2221 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"

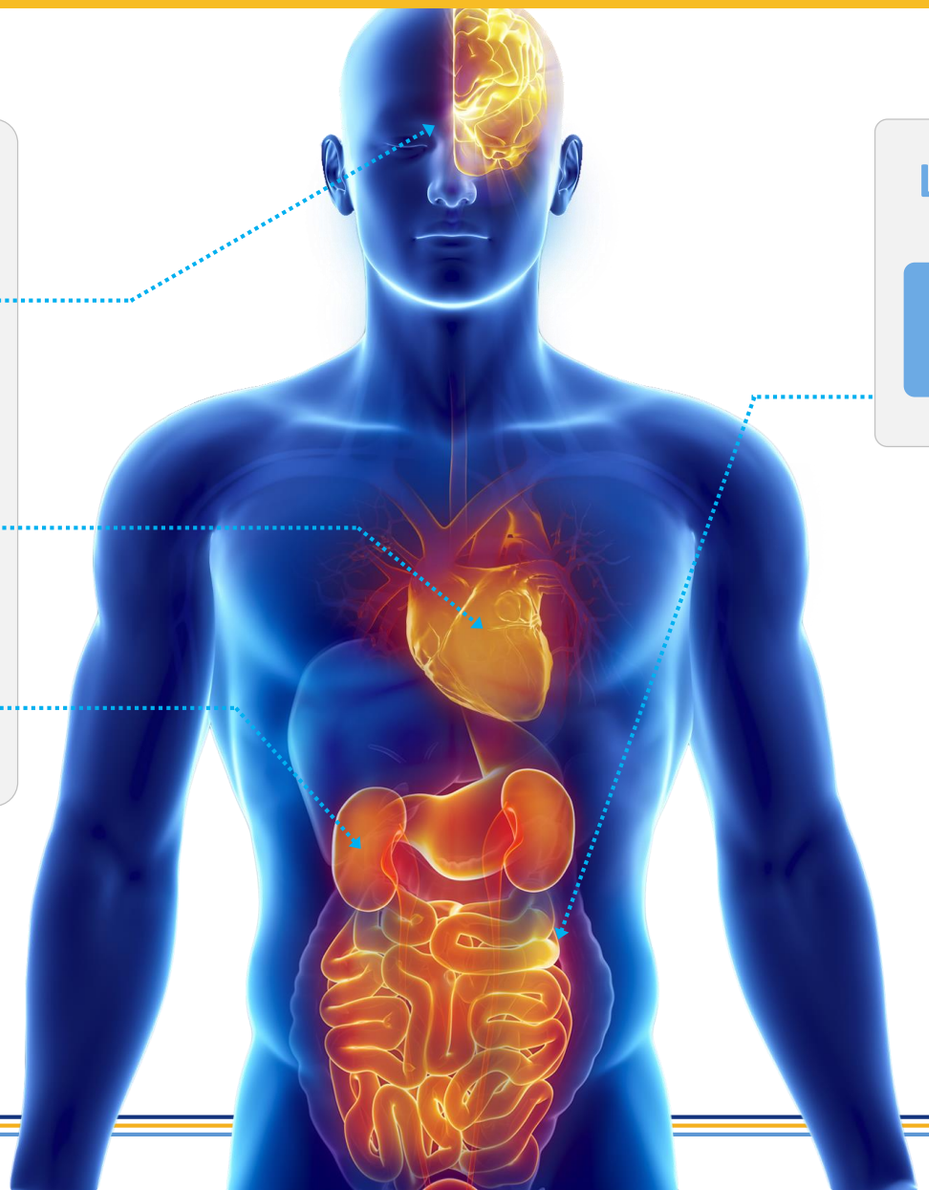
- Amicus Belief Statement

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**[1]
- **HEART DISEASE**[2]
- **KIDNEY DISEASE**[3]



Life-Limiting Symptoms:

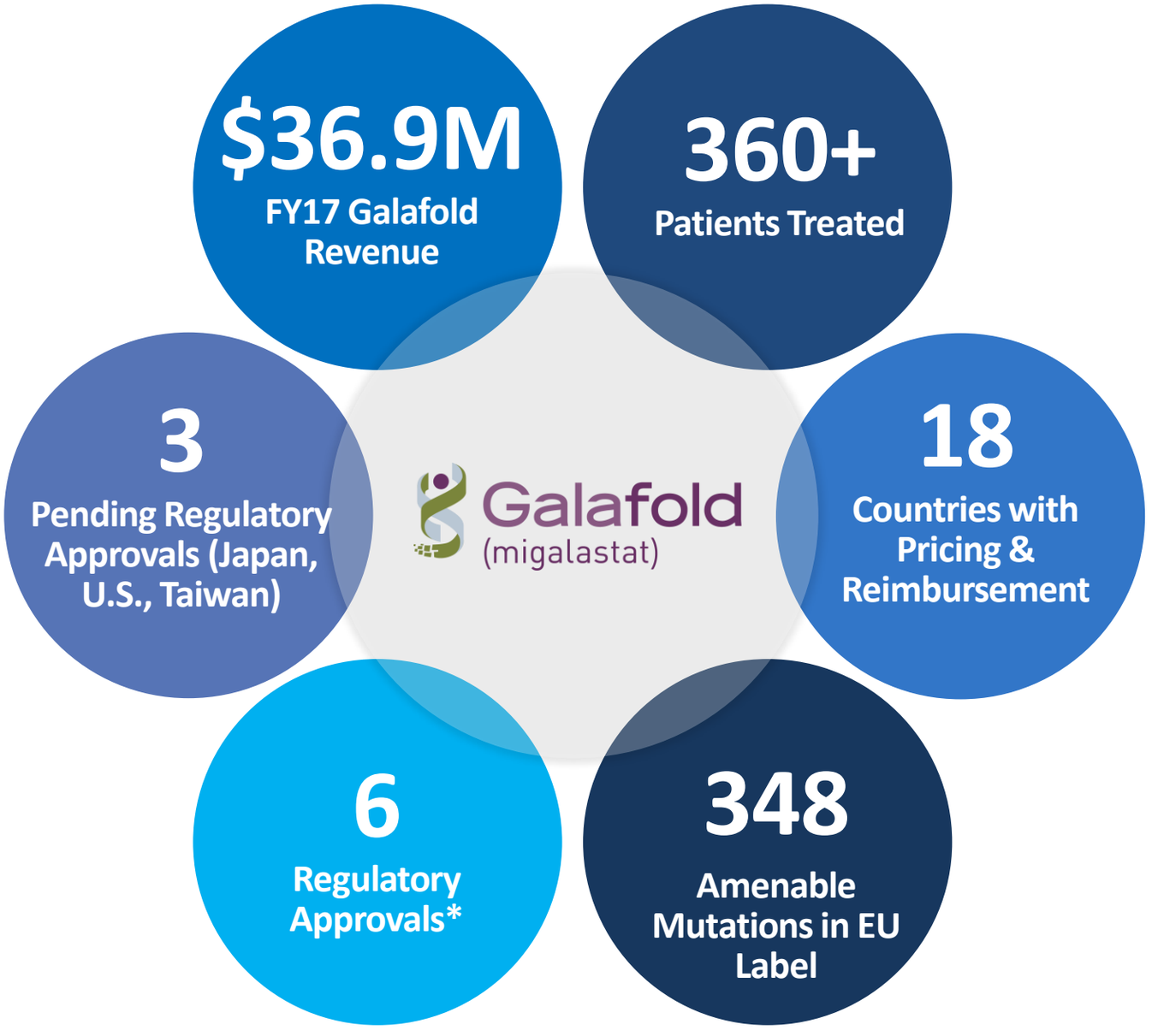
- **GASTROINTESTINAL**[3]

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

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 February 28, 2018)



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, Israel, Switzerland, South Korea
**For important safety information for Galafold visit www.ema.europa.eu.

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Galafold Success and FY18 Galafold Revenue Guidance

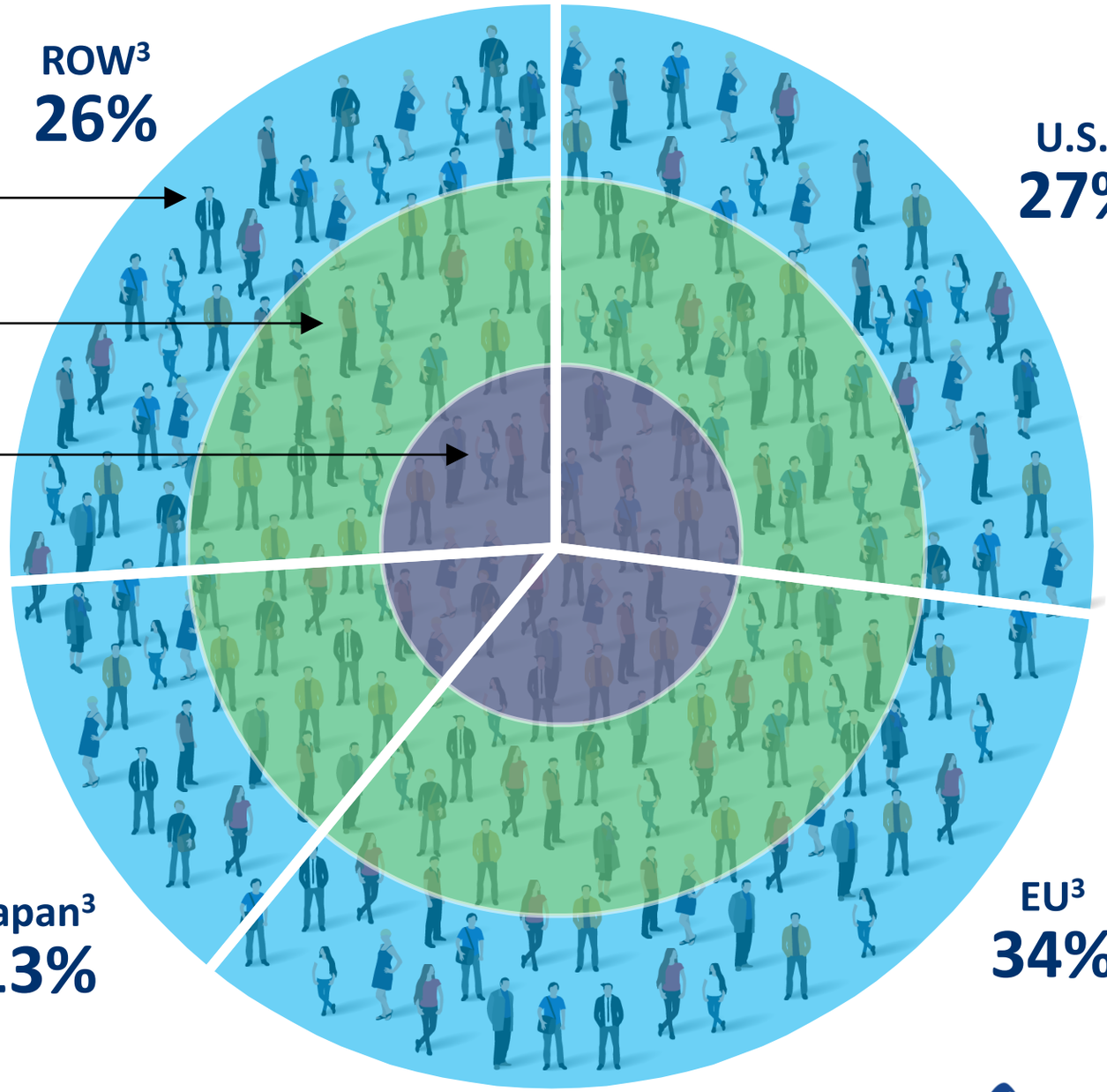
International Launch Success Positions for Significant Growth in 2018 and $500M+ Global Peak Sales Opportunity

Galafold $500M+ Global Peak Revenue Opportunity

Undiagnosed (Est. 100,000[1])

Diagnosed Untreated (~5,000[2])

ERT-Treated (~5,000[2])



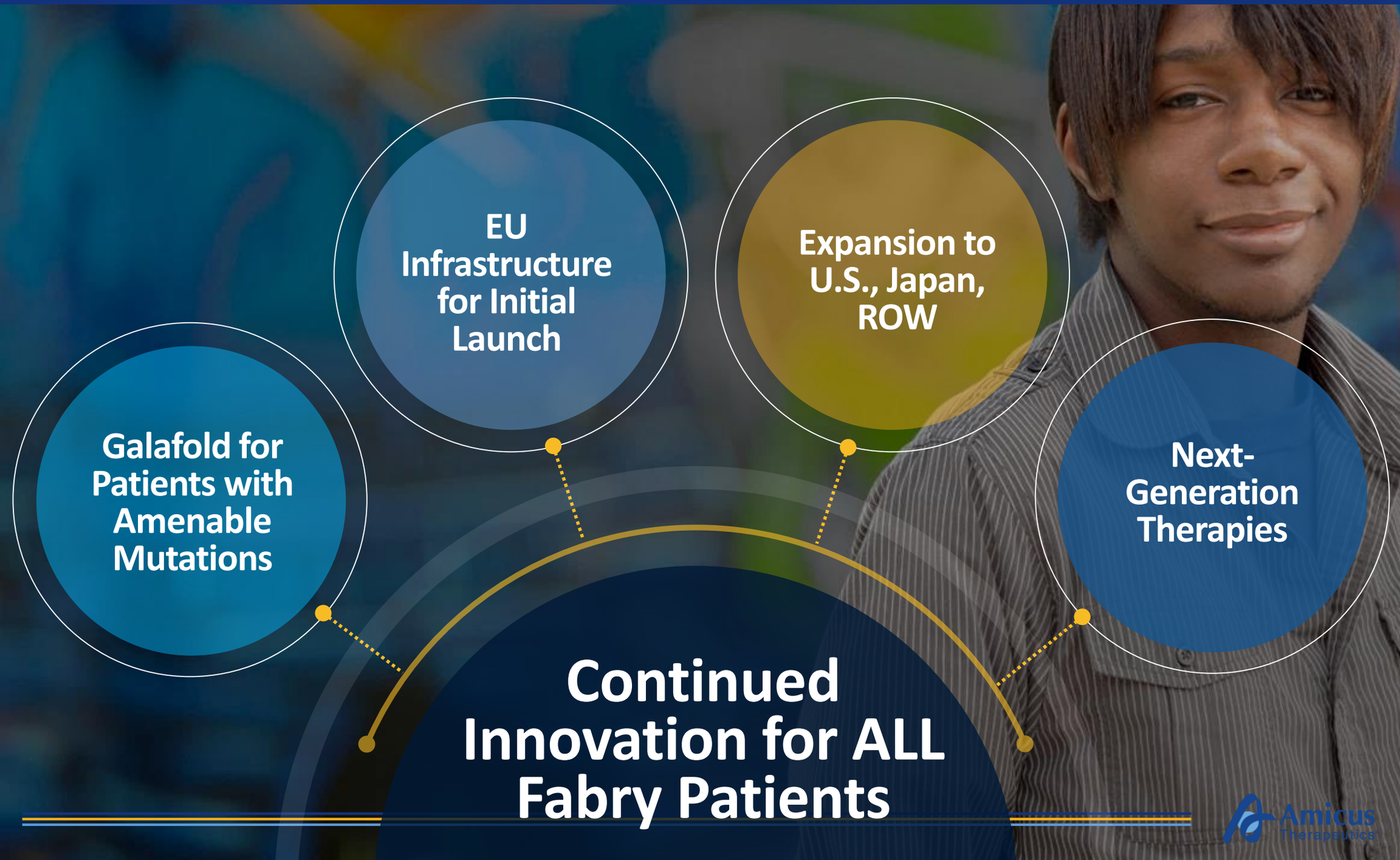
Fabry Market is $1.3B Today and Growing

Galafold Targets 35%-50% of Population with Amenable Mutations

1. Bokhari SRA, Bhimji SS. Fabry Disease. December 2017 2. Sanofi presentation February 2017 3. Fabry registries, Sanofi investor reports, Shire investor reports

Amicus Therapeutics

Fabry Franchise Strategy



Fabry Precision Medicine Driven by a Patient's Genotype

Amicus Therapeutics is Committed to Delivering the Highest Quality Therapies

~$1.3B
Global Fabry Market Today

Growing to ~$2B Global Fabry Market

Migalastat

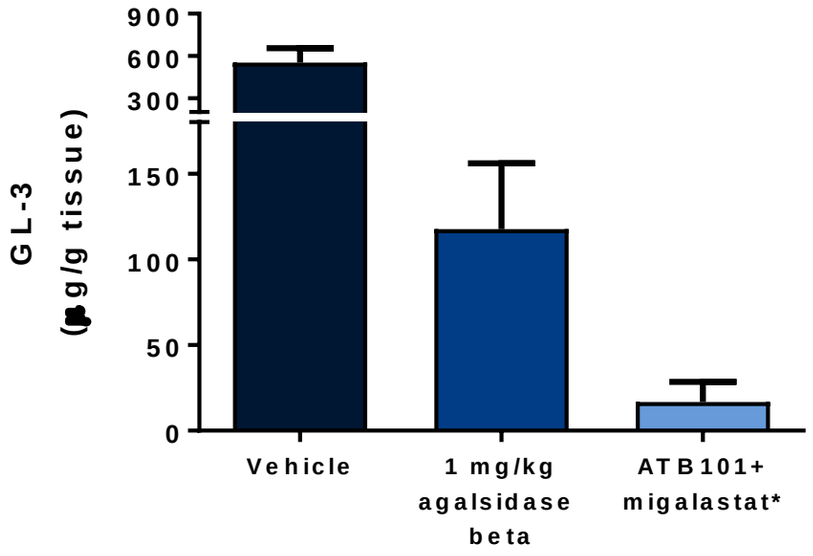
Oral Precision Medicine

Novel ERT Co-Formulated with Migalastat

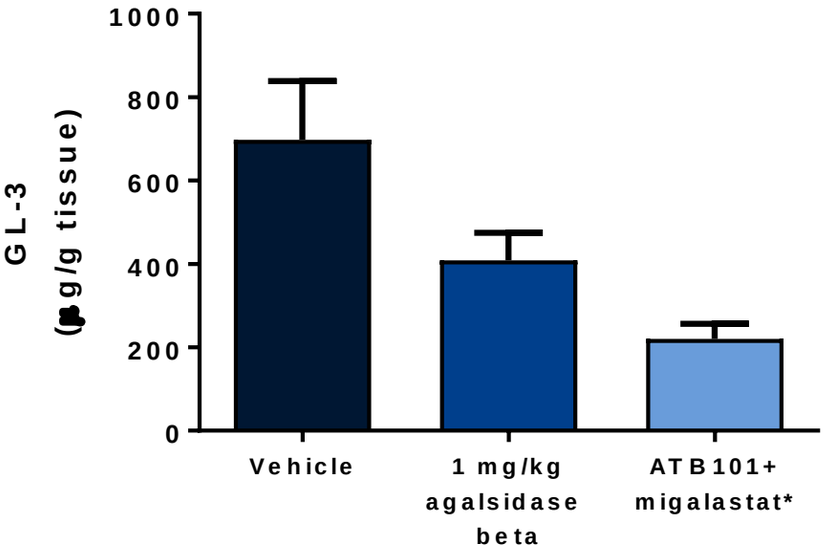
Amicus Proprietary ERT Preclinical Proof of Concept

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

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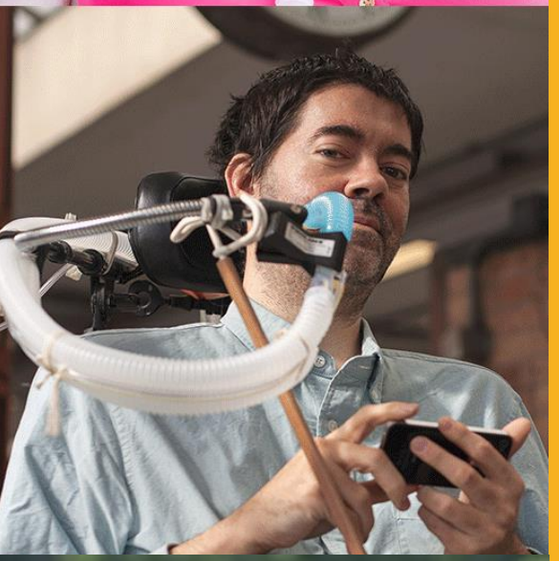


ATB200 Novel ERT for Pompe Disease

"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[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]

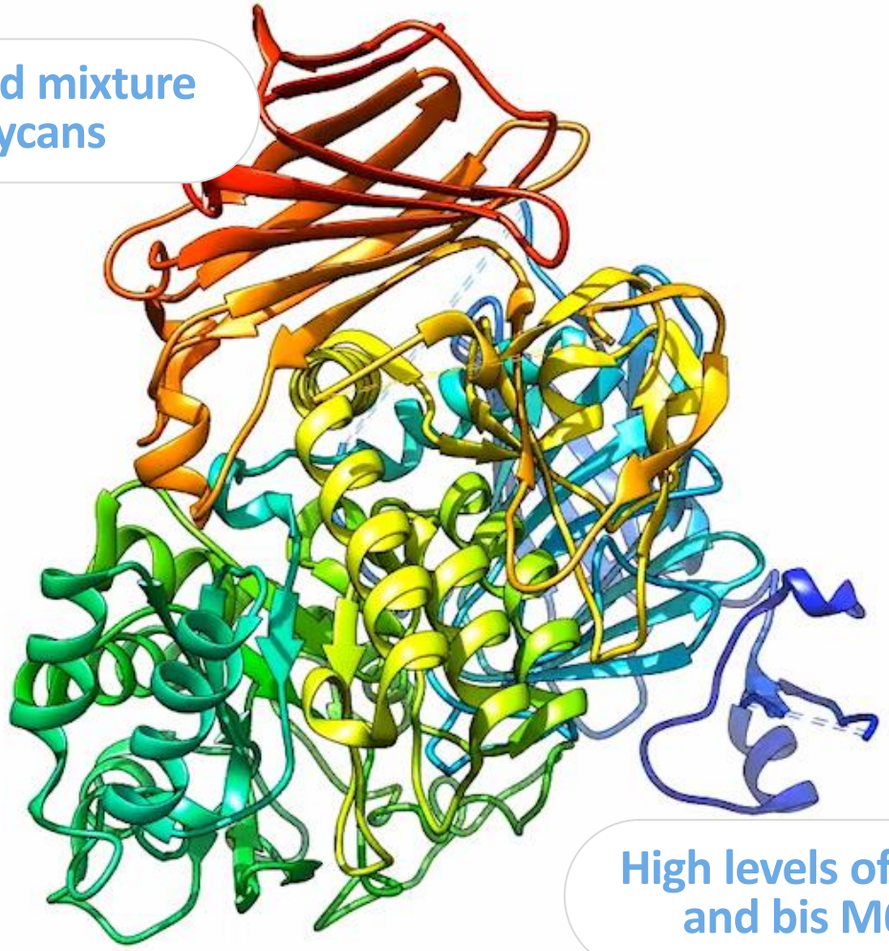
1. National Institute of Neurological Disorders and Stroke (NIH). 2. Sanofi Press Release & 10-K

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

CHART®

CHAPERONE-ADVANCED REPLACEMENT THERAPY

Chaperone addition

*Artist rendering, not actual product image

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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	**397.2** (96.8)	+23.9 (52.2)	+24.5 (40.8)	+57.4 (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	**399.5** (83.5)	+41.8 (29.4)	+63.5 (23.1)	+86.8 (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*	**52.6** (14.7)	**-1.3** (4.1)	**-1.7** (3.9)	**-3.1** (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	**53.4** (20.3)	+4.2 (5.6)	+6.2 (5.3)	+6.0 (7.1)

*FVC not available for one subject

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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
Mean (SD)	**397.2** (96.8)	**+23.9** (52.2)	**+24.5** (40.8)	**+57.4** (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-Naive 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
Mean (SD)	399.5 (83.5)	+41.8 (29.4)	+63.5 (23.1)	+86.8 (11.1)

- 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

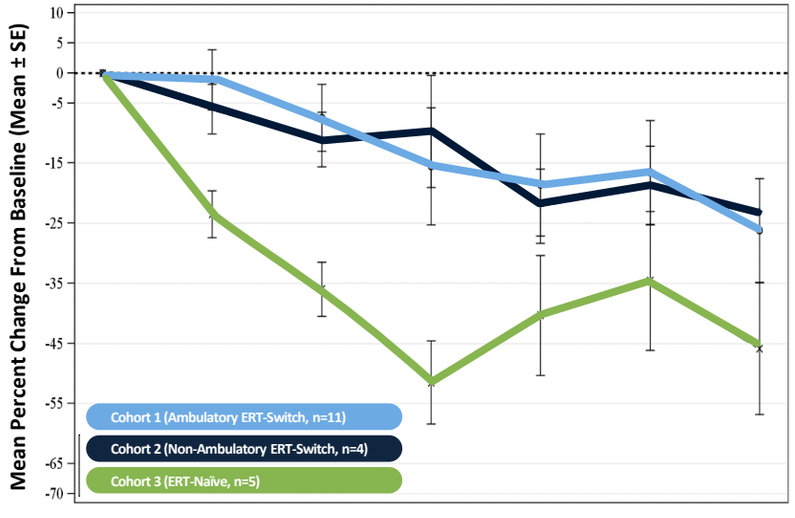
Assessment	Muscle Group Tested	Baseline (n=4)	Change to Month 6 (n=4)	Change to Month 9 (n=4)
QMT- Quantitative Muscle Testing - Dynamometer (pounds force)	Shoulder Adduction *	**5.7** (8.8)	**+8.1** (12.8)	**+9.6** (12.3)
	Shoulder Abduction	**16.7** (18.1)	**+1.0** (6.6)	**+0.5** (9.3)
	Elbow Flex	**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)

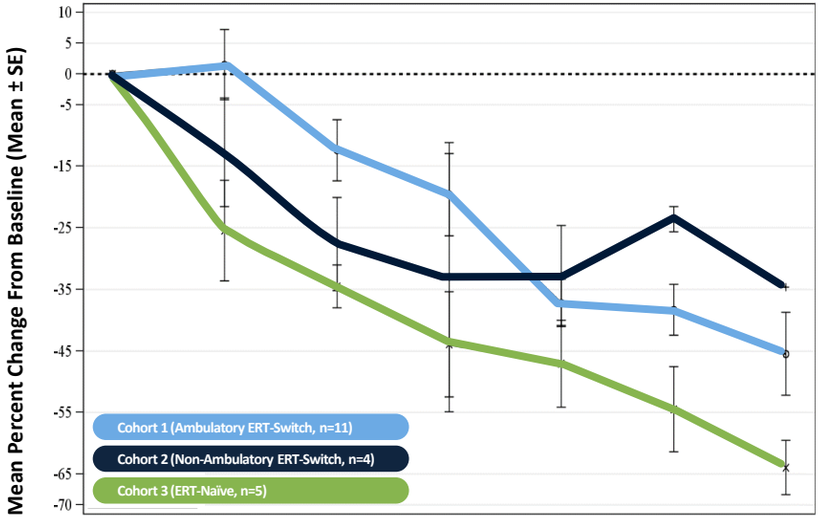
All Cohorts Demonstrated Persistent Improvement in Biomarkers of Muscle Damage (CK) and Disease Substrate (Hex4) For Up To 12 Months

Muscle Damage Biomarkers (% Change from Baseline for CK)



Visit	BL	WK 2	WK 4	M3	M6	M9	M12
N	11,4,5	11,4,5	10,4,4	8,3,5	10,4,5	10,4,5	8,1,2

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



Visit	BL	WK 2	WK 4	M3	M6	M9	M12
N	11,4,5	11,4,5	11,4,5	11,4,5	10,4,5	9,4,5	7,1,2

CK=creatine 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

Pompe Development Pathways

Our Goal: To Work with Global Regulators to Ensure That as Many People Living with Pompe Have Access to This Novel Treatment Paradigm as Quickly as Possible

Potential Pathways Include:*

*Subject to ongoing discussions with regulatory authorities, update anticipated 1H18

Amicus Therapeutics

Biologics Manufacturing Capabilities

Scaling up Manufacturing to Meet Needs of the Pompe Community

1000L

(Registration & Commercial)

First GMP Manufacturing Campaign of Drug Substance at 1000L Scale Successfully Completed

Analytical and *in vivo* comparability studies completed between 250L and 1000L engineering batches

FDA agreement reached on comparability between 250L GMP scale and 1000L engineering batches

FDA agreement reached on testing strategy for demonstrating comparability between 250L scale and 1000L GMP batches

Key Clinical & Manufacturing Activities 2018

Significant 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
- Additional 4-6 patients added to Phase 1/2 study
- Retrospective natural history of ERT-treated patients
- Prospective data collection on current ERT-treated patients
- Initiation of larger registration-directed study

MANUFACTURING

- Final regulatory agreement on comparability between 1,000L and 250L GMP scale
- Completion and release for clinic of 1,000L GMP commercial scale material
- Continued capacity to ensure sufficient medicines to supply patient population
- Announce plan for long term commercial manufacture and capacity

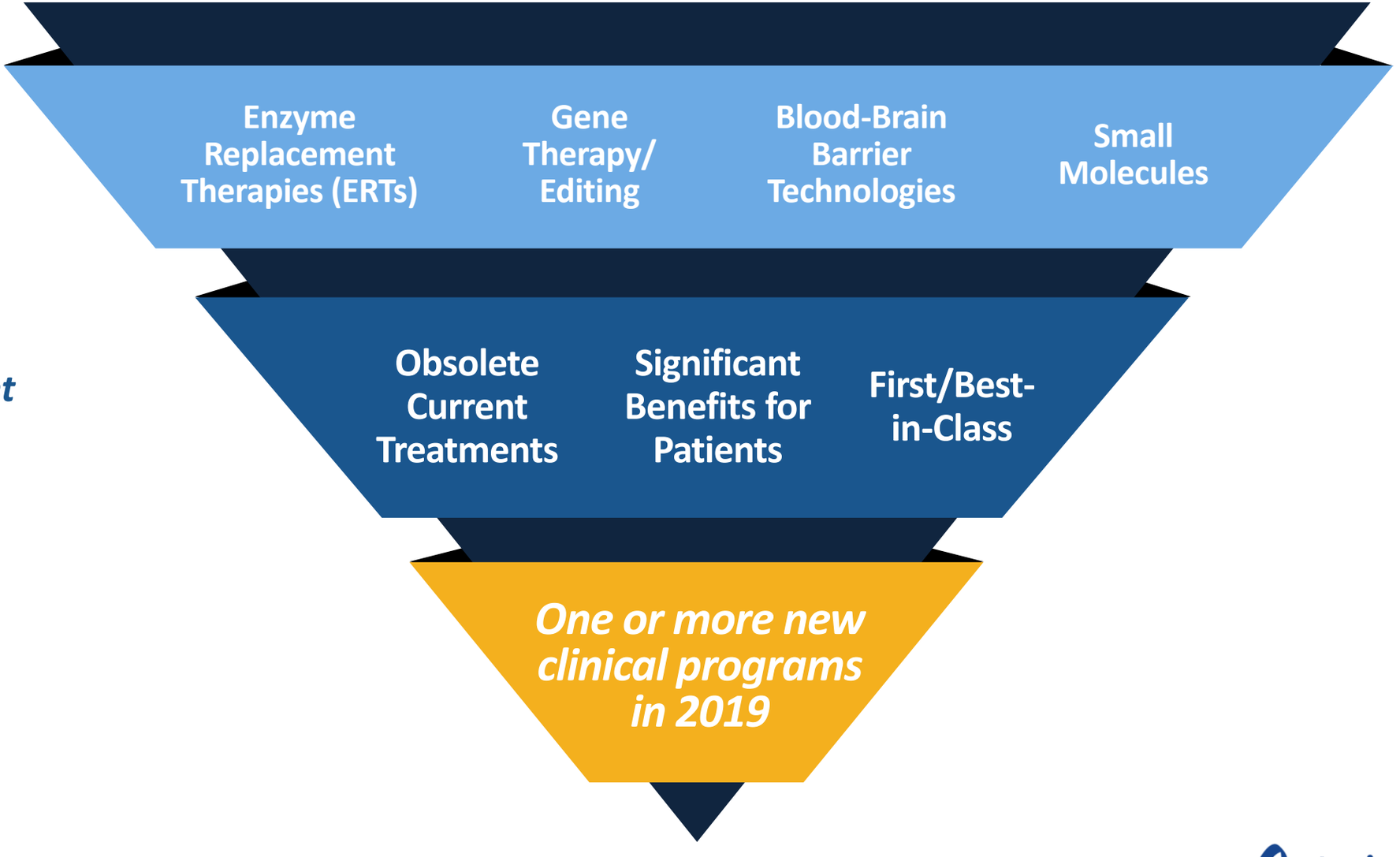
Pipeline Strategy

"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



Financial Summary & Key Milestones

"We are business led and science driven"

- Amicus Belief Statement

Financial Summary & Guidance

Strong Balance Sheet with $359M Cash at 12/31/17 and $300M in Gross Proceeds from February 2018 Following On Offering - Cash Runway into at Least 2021

FINANCIAL POSITION	December 31, 2017
Cash	$359M
Debt	$250M
Cash Runway	Into at least 2021
CAPITALIZATION	
Shares Outstanding[1]	186.9M
FINANCIAL GUIDANCE	
FY18 Net Cash Spend Guidance	$230-$260M
Galafold Revenue Guidance	$75-$85M

[1]Includes shares from the February 2018 equity offering

Amicus Therapeutics

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 ATB200/AT2221 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

Amicus Therapeutics

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement

Amicus Therapeutics®

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

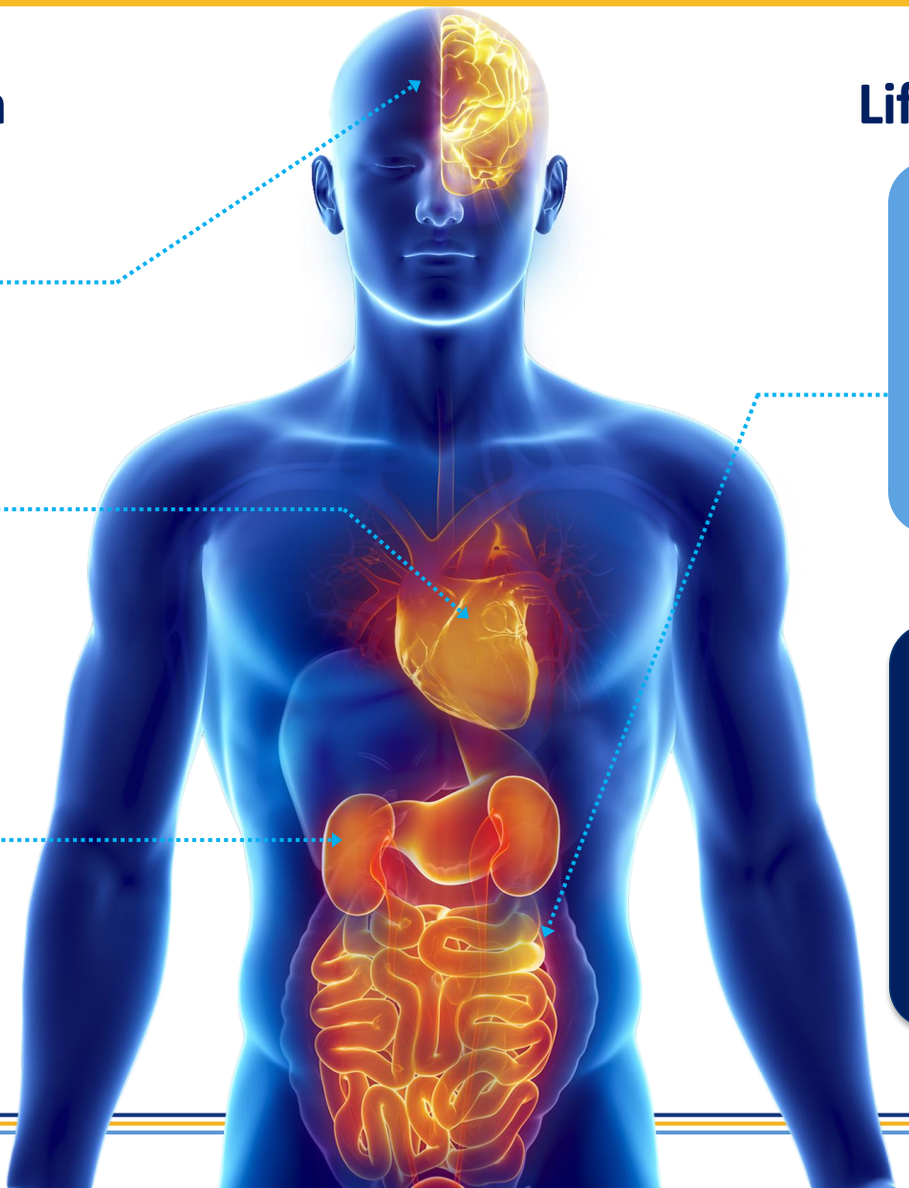
TRANSIENT ISCHEMIC ATTACK (TIA) & STROKE[1]

HEART DISEASE[2]

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

KIDNEY DISEASE[3]

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



Life-Limiting Symptoms

GASTROINTESTINAL[3]

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

Key Facts

- Deficiency of α-Gal A enzyme leading to GL-3 accumulation
- >900 known mutations
- ~10K diagnosed WW (51% female/49% male[4])
- 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

*New England Journal of Medicine, Journal of Medical Genetics

Amicus Therapeutics