



FY17 Results Conference Call & Webcast



February 28, 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 to be 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.

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



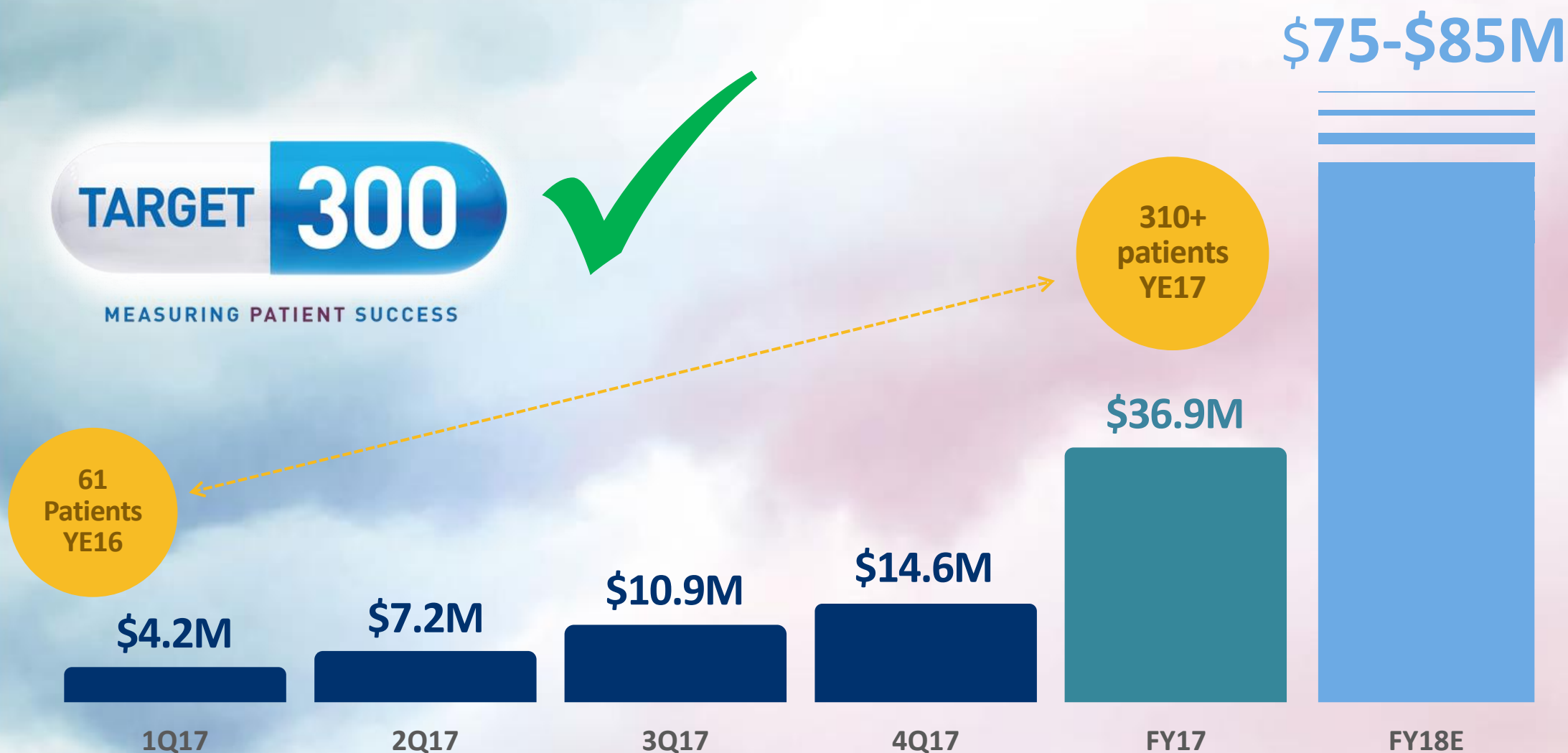
Galafold™ (Migalastat) Precision Medicine for Fabry Disease

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

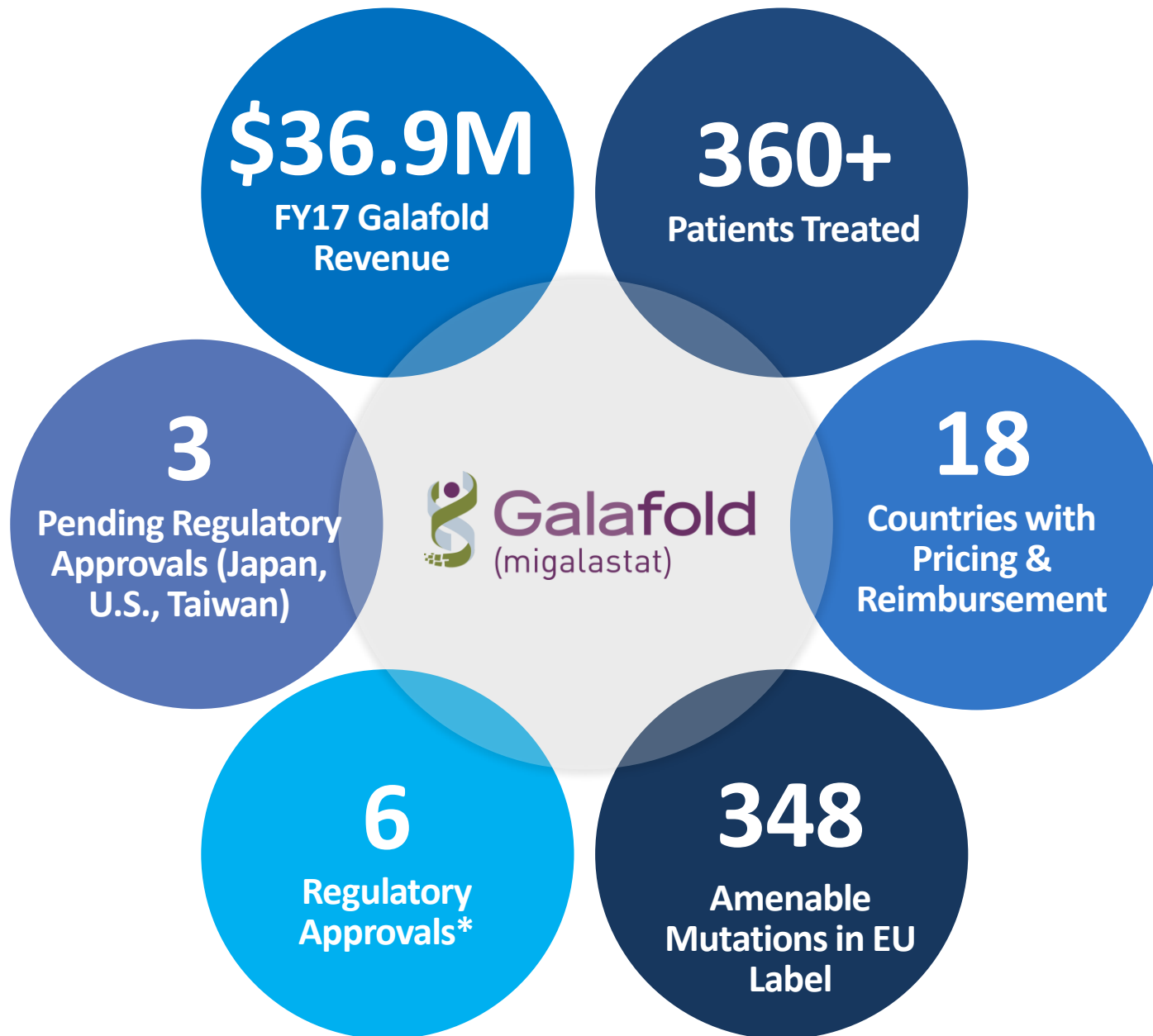
- Amicus Belief Statement

Galafold Success and FY18 Galafold Revenue Guidance

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



Galafold Snapshot (as of February 28, 2018)



**FIRST Oral
Precision Medicine
for Fabry Disease**

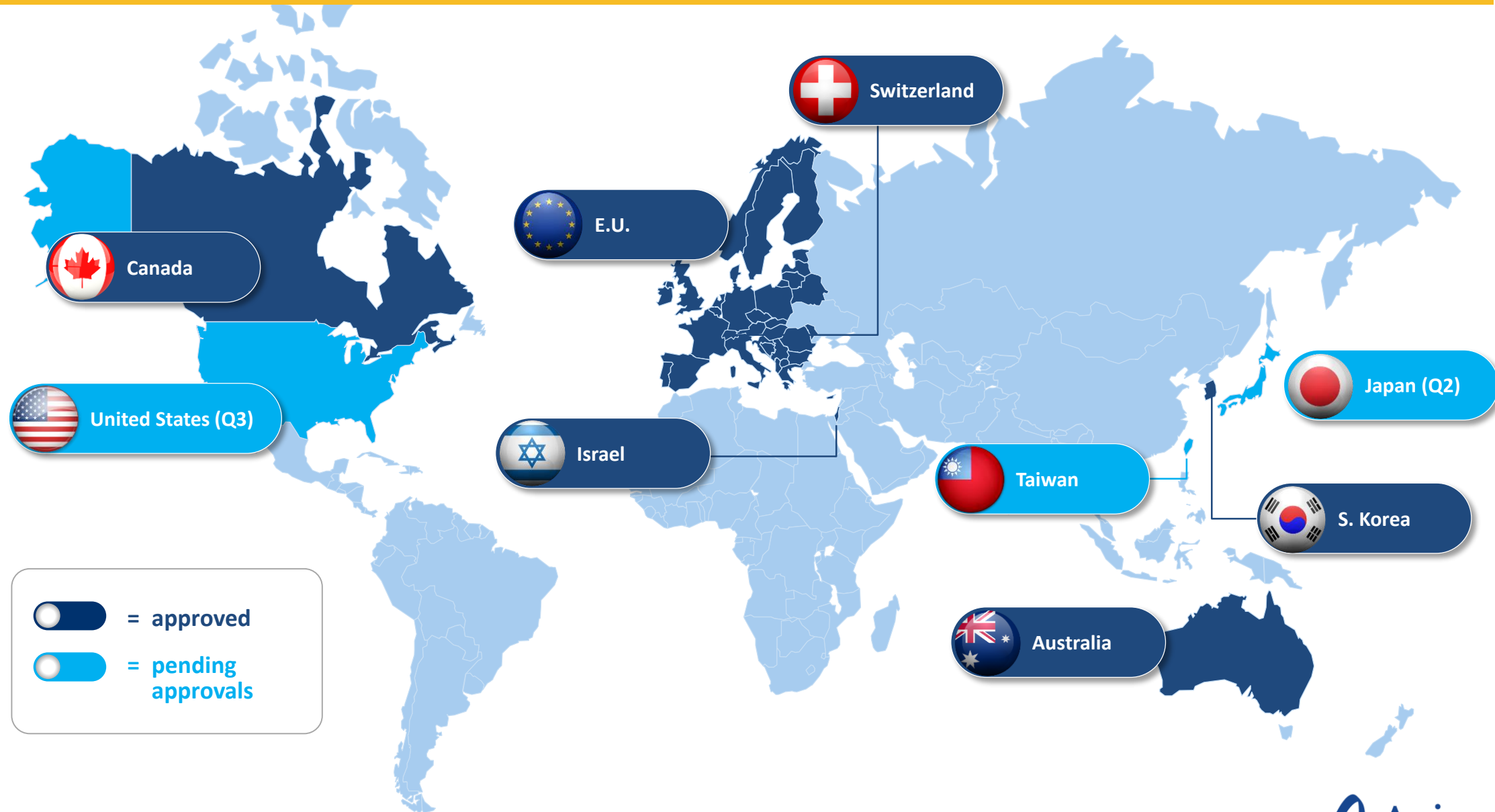
**Galafold Indicated
for Long-Term
Treatment of
Adults and
Adolescents Aged
≥ 16 years with a
Confirmed
Diagnosis of Fabry
Disease and Who
Have an Amenable
Mutation****

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

**For important safety information for Galafold visit www.ema.europa.eu.

Migalastat Launch Progress (2/28/18)

360+ Patients Currently Treated with Approvals in 6 Target Markets





ATB200 Novel ERT for Pompe Disease

“We encourage and embrace constant innovation”

- Amicus Belief Statement

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



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 2Q18



Financial Summary and Upcoming Milestones

FY17 Select Financial Results

FY17 Revenue of \$36.9M from Sales of Galafold

(\$000s) except per share data	December 31, 2017	December 31, 2016
Product revenue	\$36,930	\$4,958
Cost of Goods Sold	\$6,236	\$833
R&D Expense	\$149,310	\$104,793
SG&A Expense	\$88,671	\$71,151
Changes in fair value of contingent consideration	(\$234,322)	\$6,760
Loss on impairment of assets	\$465,427	-
Loss from operations	(\$441,985)	(\$181,890)
Income tax benefit	\$165,119	\$3,739
Net Loss	(\$284,002)	(\$200,042)
Net Loss Per Share	(\$1.85)	(\$1.49)

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
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FINANCIAL GUIDANCE

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

¹Includes shares from the February 2018 equity offering

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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



Healing Beyond Disease™

Healing Beyond Disease: Our Broader Corporate Responsibility



HEALING BEYOND DISEASE
A UNIQUELY AMICUS INITIATIVE

1
Recognize and build upon the previous achievements and efforts of all Amicus employees

BUILDING ON CORPORATE CULTURE MOMENTUM AS WE GROW:
Impact as Many People Living with Rare Diseases as Possible

2
Drive, define, and integrate patient centricity into the long term fabric and culture of Amicus

3
Provide additional structure to allow all Amicus employees to go above and beyond in supporting the rare disease community

Healing Beyond Disease: Our Broader Corporate Responsibility

HEALING BEYOND DISEASE PILLARS

Healing Beyond Disease is inspired by and adaptive to rare disease communities and reflects the existing generosity of our corporate culture.

time

Evolve volunteerism companywide to further our commitment to the rare disease patient community with information and incentives for employees

talent

Leverage the expertise within Amicus to empower organizations and individuals impacted by rare diseases to accomplish their mission

treasure

Advance philanthropy for rare diseases by providing a broader opportunity for financial support and contributions

pledge

Designate a portion from any Amicus marketed drug sales to reinvest in that specific disease until that disease has a cure

bridges

Build rare bridges across the globe to provide access to our medicines in the near and long-term in the developed and developing world

2017 • • • • • 2018 • • • • • 2019 • • • • • 2020

Thank You

“Our passion for making a difference unites us”

-Amicus Belief Statement

