



1Q18 Financial Results & Corporate Highlights

May 8, 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 to be 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.

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**

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

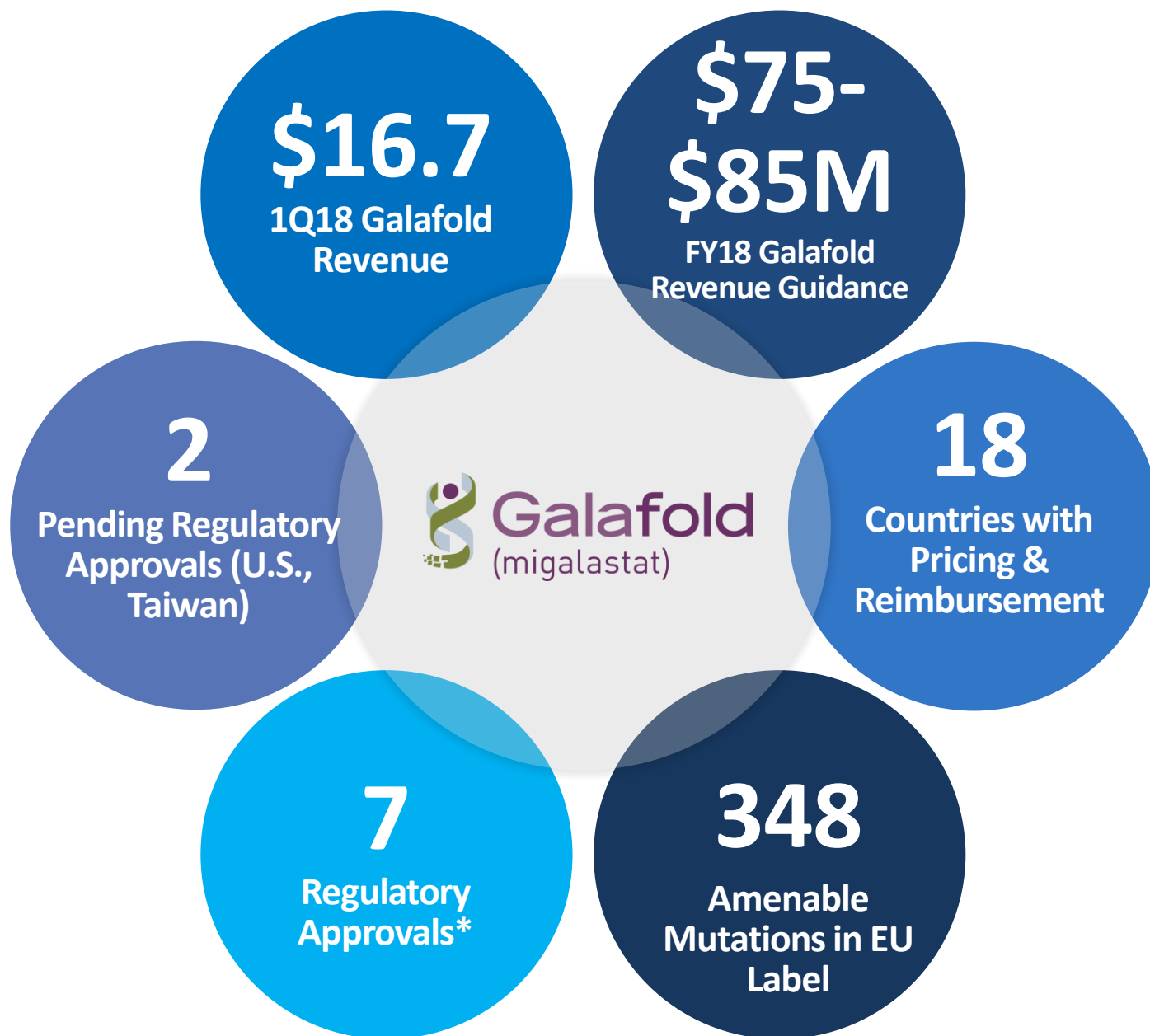


Galafold™ (Migalastat) Precision Medicine for Fabry Disease

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

- Amicus Belief Statement

Galafold Snapshot (as of March 31, 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, Japan, Israel, Switzerland, South Korea

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

Migalastat Launch Progress (3/31/18)

Approved in Japan in 2Q18 – Now Approved in 7 Target Markets



Additional Galafold Launch Dynamics

Key Success Factors Driving Strong Launch Across Geographies and Support \$500M+ Global Peak Sales Potential

- Robust clinical data set^{1,2,3}
- Broad labels for amenable mutations (35%-50% of Fabry patients)
- Growth in patient and physician adoption from existing markets
- Expansion into new countries
- Very high rate of compliance and adherence
- Innovative precision medicine
- Compelling value proposition leading to rapid reimbursement
- Focus on hiring the best and brightest from a range of leading rare disease companies

U.S. and Japan Demographics for Galafold

U.S. NDA Filed Under Priority Review



US Overview

NDA Filed (Priority Review)
August 13, 2018 PDUFA
Leadership and majority of field team in place
Appropriate stakeholder engagement underway
~3,000 diagnosed (1,500 treated), 35-50% amenable
Orphan Drug and Fast Track designations

Japan Launch Anticipated in 2Q18



Japan Overview

J-NDA Approved March 2018
Launch team hired and trained
Launch anticipated 2Q18
~850 patients diagnosed (>700 treated)
No ERT home infusion currently available
Physicians tend to initiate treatment early



AT-GAA Novel ERT for Pompe Disease

“We encourage and embrace constant innovation”

- Amicus Belief Statement

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

Key Clinical Activities 2018

Significant Progress In Clinical Activities in 2018 to Support Fastest and Best Approval Pathways

- ✓ Additional Phase 1/2 ATB200-02 extension data presented at *WORLDSymposium*
- ✓ Additional patients in Phase 1/2 ATB200-02 clinical 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 (2H18)
- ❑ 18-month data from ATB200-02 clinical study (4Q18)



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 and Upcoming Milestones

1Q18 Select Financial Results

1Q18 Revenue of \$16.7M from Sales of Galafold

(\$000s) except per share data	March 31, 2018	March 31, 2017
Product revenue	16,696	4,169
Cost of Goods Sold	2,615	775
R&D Expense	40,798	30,876
SG&A Expense	27,396	19,132
Changes in fair value of contingent consideration	1,100	4,578
Loss from operations	56,182	52,015
Change in fair value of derivatives	4,861	-
Income tax benefit (expense)	1,392	(56)
Net Loss	49,916	54,992
Net Loss Per Share	0.28	0.39

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 Runway¹	Into at least 2021

CAPITALIZATION

Shares Outstanding²	187,972,218
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FINANCIAL GUIDANCE

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

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

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Thank You

“Our passion for making a difference unites us”

-Amicus Belief Statement

