



# 2Q18 Financial Results & Corporate Highlights

August 7, 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 June 30, 2018 to be filed August 7, 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

*As of January 2018*

## Focused on FIVE Key Strategic Priorities in 2018

- 1 Double Galafold (migalastat) revenue to \$75-\$85M (Now \$80-\$90M)**
- 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**

\* AT-GAA, also known as ATB200/AT2221



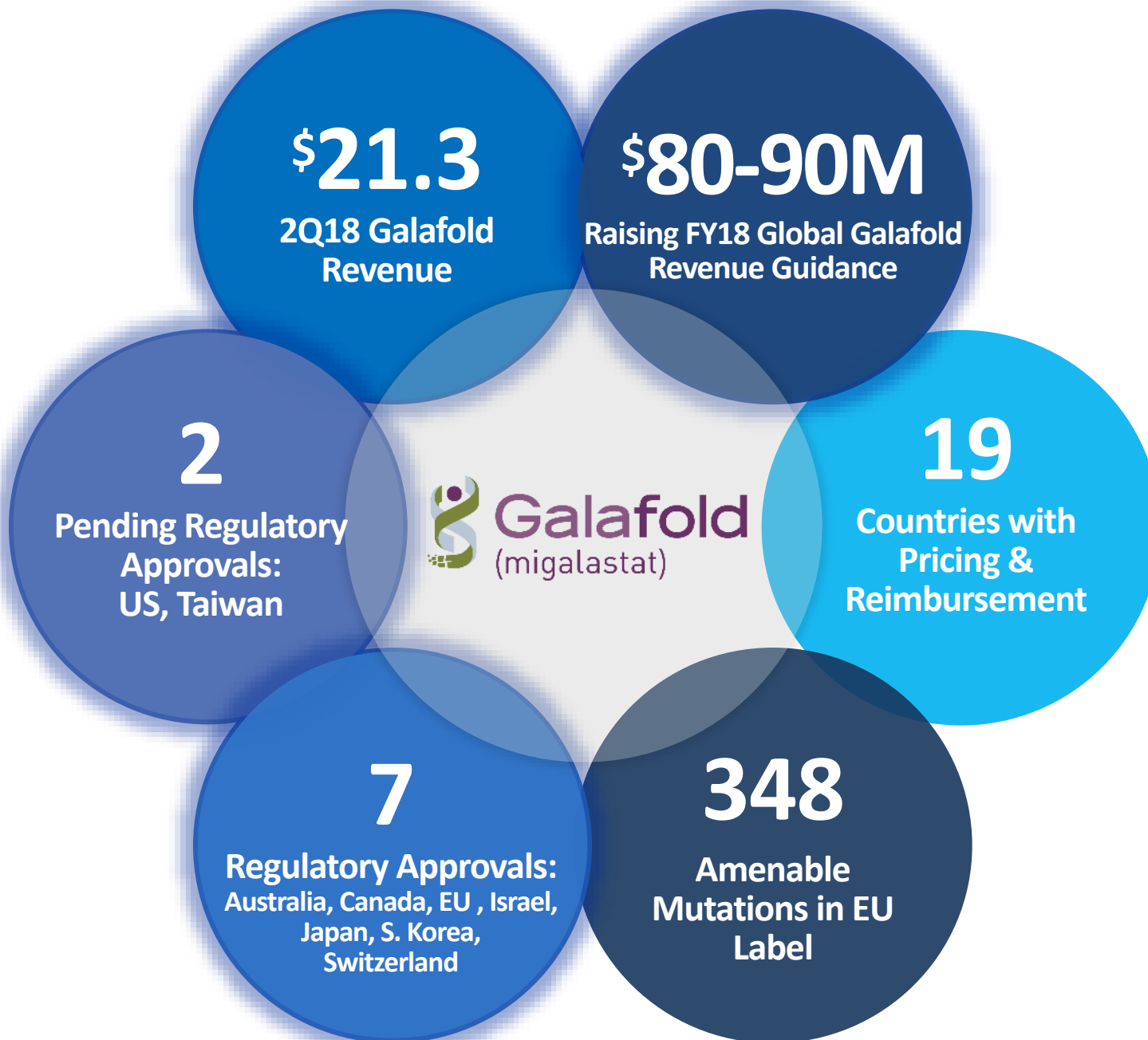


# Galafold™ (Migalastat) Precision Medicine for Fabry Disease

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

- Amicus Belief Statement

# Galafold Snapshot (as of June 30, 2018)



**FIRST Oral Precision Medicine for Fabry Disease**

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

# Japan Launch Update

## Japan Launch of Galafold Underway

**Full commercial team hired and trained**

**First commercial patients commenced treatment in late 2Q18**

**Successful launch symposium attended by 100+ HCPs**

**>850 patients diagnosed (>700 treated with an ERT)\***

**No ERT home infusion currently available**

**Physicians tend to initiate treatment early**



\*Clinical & commercial, all figures approximate



# U.S. Demographics for Galafold



## Prepared for Launch Pending FDA Decision

**NDA Filed (Priority Review)**

**August 13, 2018 PDUFA**

**Full launch team hired and trained**

**Appropriate stakeholder engagement ongoing**

**>3,000 diagnosed (~1,500 treated), 35-50% amenable\***

**Orphan Drug and Fast Track designations**

\*Clinical & commercial, all figures approximate



# Financial Summary



# 2Q18 Select Financial Results

## 2Q18 Revenue of \$21.3M from Sales of Galafold

*(in thousands, except per share data)*

	June 30, 2018	June 30, 2017
Product revenue	21,309	7,158
Cost of goods sold	3,135	1,061
R&D expense	34,660	31,985
SG&A expense	29,172	19,311
Changes in fair value of contingent consideration	300	1,050
Loss from operations	(46,931)	(47,061)
Change in fair value of derivatives	(7,600)	-
Income tax benefit (expense)	(339)	(49)
Net loss	(61,833)	(48,136)
Net loss per share	(0.33)	(0.34)

# Financial Summary & Guidance

**Strong Balance Sheet with \$553M Cash at 6/30/18- Cash Runway into at Least 2021**

FINANCIAL POSITION		June 30, 2018
Cash		\$553M
Debt		\$250M
Cash Runway <sup>1</sup>		Into at least 2021
CAPITALIZATION		
Shares Outstanding <sup>2</sup>		189,053,214
FINANCIAL GUIDANCE		
FY18 Net Cash Spend Guidance		\$220-\$250M
Galafold Revenue Guidance		\$80-\$90M

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



# AT-GAA Novel ERT for Pompe Disease

***“We encourage and embrace constant innovation”***

**- Amicus Belief Statement**



# Key Activities in 2018

## Significant Progress toward Clinical, Regulatory, and GMP Manufacturing Activities in 2018 to Lay Foundation for Best and Fastest Approval Pathways

### Year to Date Progress

#### CLINICAL

- ✓ Additional Phase 1/2 ATB200-02 extension data presented at *WORLD Symposium*
- ✓ Additional patients in Phase 1/2 ATB200-02 clinical study
- ✓ Initiation of retrospective natural history of ERT-treated patients
- ✓ Prospective data collection on current ERT-treated patients

#### REGULATORY

- ✓ EMA: Received Scientific Advice Working Party Guidance

#### MANUFACTURING

- ✓ Agreement with German regulatory authorities (BfArM) on strategy to demonstrate comparability of ATB200 between the 1,000 liter scale and the 250 liter scale
- ✓ Release for clinic of 1,000L GMP commercial scale material

### Upcoming Milestones

#### CLINICAL

- ☐ 18-month data from ATB200-02 clinical study (4Q18)
- ☐ Completion of a retrospective natural history study (2H18)
- ☐ Initiation of larger registration-directed study (2H18)

#### REGULATORY

- ☐ U.S. FDA type C meeting and US update (3Q18)

#### MANUFACTURING

- ☐ Announce plan for long term commercial manufacturing



# Pipeline Strategy and Upcoming Milestones

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

# Pipeline Strategy

**Developing Therapies for People Living with Rare Metabolic Diseases with a New Focus on Gene Therapy**

*Technology  
Focus*

**Gene Therapies**

*Development  
Criteria*

**Obsolete  
Current  
Treatments**

**Significant  
Benefits for  
Patients**

**First/Best-  
in-Class**

*Pipeline  
Expansion*

***One or more new  
clinical programs  
in 2019***



# 2018 Key Strategic Priorities

*As of August 2018*

**Focused on FIVE Key Strategic Priorities in 2018**

**1 Double Galafold (migalastat) revenue to \$80-\$90M**

**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**

\* AT-GAA, also known as ATB200/AT2221

# Thank You

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

*-Amicus Belief Statement*

