



2Q17 Financial Results Conference Call & Webcast

August 7, 2017

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, financing plans, and the projected 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 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; and the potential that we will need additional funding to complete all of our studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results for any of our product candidates. With respect to statements regarding projections of the Company's 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 previous filings with the SEC and in our Annual Report on Form 10-K for the year ended December 31, 2016 and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2017. 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.

2017 Key Strategic Priorities

We Remain Sharply Focused on FIVE Key Strategic Priorities as We Continue to Build a Top Global Biotechnology Company Focused on Rare Devastating Diseases

Advance International Galafold Launch

Submit Japanese and U.S. New Drug Applications for Migalastat

Establish Definitive Proof of Concept for ATB200/AT2221 with Clear Path to Registration for Pompe Disease

Successfully Complete Phase 3 EB Study

Maintain Financial Strength

Key Anticipated Milestones in 2017

2017

Fabry Disease (Galafold)

- Japan NDA submission in 2Q17
- U.S. NDA submission in 4Q17
- 300 patients on reimbursed Galafold by YE17*

Pompe Disease (ATB200/AT2221)

- Phase 1/2 data cascade in 2Q and 3Q
- Meetings with U.S. and EU regulators

Epidermolysis Bullosa (EB) (SD-101)

- Phase 3 top-line data 3Q17

Strong Balance Sheet

- Significant revenue contribution
- Cash runway into 2H19

*Commercial and Expanded Access Programs (EAPs)



Galafold™ (Migalastat) Precision Medicine for Fabry Disease

Continue Successful Launch Execution and Geographic Expansion

Successful International Launch Underway (as of 7/31/17)

**Driven by Top EU5 Countries, Galafold is Quickly Reaching ERT-Switch & Naïve Patients,
Reimbursement Now Available in 12 Countries***

179

Patients (Switch & Naïve) on
reimbursed Galafold (7/31/17)

12

Countries with available reimbursement*

13

Countries with pricing discussions ongoing

27

Countries with Amicus footprint

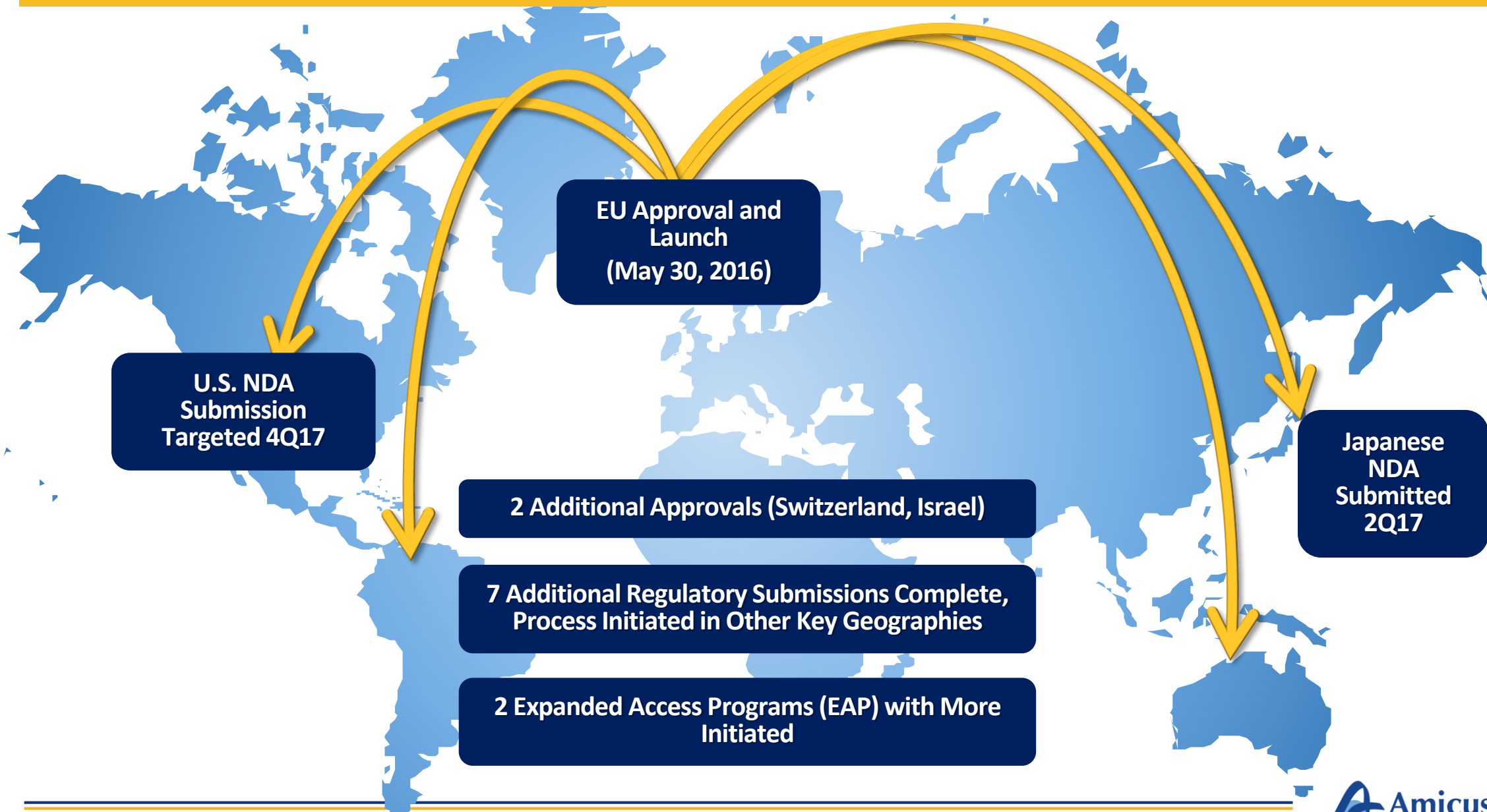
300

**Target Number of
Patients on Reimbursed
Galafold by YE17**

*Commercial and Expanded Access Programs (EAPs)

Global Regulatory Strategy to Reach More Patients

EU Approval and Planned U.S. NDA Submission Provide Pathway to Reach Global Fabry Market



* Two EAPs converted to commercial reimbursement



Financial Summary

2Q17 Select Financial Results

2Q17 Revenue of \$7.2M from Sales of Galafold

(\$000s) except per share data	June 30, 2017	June 30, 2016
Product revenue	\$7,158	-
R&D Expense	\$31,985	\$18,281
SG&A Expense	\$19,311	\$19,300
Net Loss	\$(48,136)	\$(51,050)
Net Loss Per Share	\$(0.34)	\$(0.40)

Financial Summary & Guidance

Strong Balance Sheet with \$227M Cash at 6/30/17 and Cash Runway Into 2H19

Financial Position	June 30, 2017
Cash	\$227M
Debt	\$250M
FY17 Net Operating Cash Spend Guidance	\$175-\$200M
FY17 Net Cash Spend Guidance*	\$200-\$225M
Cash Runway	2H19
Capitalization	July 25, 2017
Shares Outstanding	164,566,069

*Includes third party milestone payments and capital expenditures



Closing Remarks

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Strong Balance Sheet

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*Commercial and Expanded Access Programs (EAPs)

Our Vision – Maximizing Impact on Patients to Drive Shareholder Value

**The Ultimate Measure of Our Success
Will be the Number of Patients with
Devastating Rare Diseases Treated
with an Amicus Medicine**



= 20 patients

~37 Patients

~90 Patients

~250 Patients*

~800 Patients*

~5,000 Patients*

2010

2014

YE2016

2018

2023

Thank You

