Amicus Therapeutics®

FY16 Financial Results Conference Call & Webcast

March 1, 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. In particular, this presentation relates to the preliminary data from a global Phase 1/2 study (ATB200-02) to investigate ATB200/AT2221. The inclusion of forward-looking statements arising from this preliminary data and study 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. The preliminary data and Phase 1/2 study discussed herein is inherently preliminary and early in the study, derived from a limited patient set, and later trial results with this patient set or others may not be consistent with these preliminary results. With respect to statements regarding projections of our cash position, actual results may differ based on market factors and our ability to execute 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 to be filed later today. 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.

Introduction

Key Accomplishments in 2016

	2016
Fabry Disease (Galafold™)	• EU approval • International launch success • Regulatory progress
Pompe Disease (ATB200/AT2221)	• Positive preliminary data in Phase 1/2 study in Pompe patients
Epidermolysis Bullosa (EB) (SD-101)	• Phase 3 enrollment near complete
Strong Balance Sheet	• $330M in cash (12/31/16)

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 New Drug Application (J-NDA) 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**

Galafold™ (Migalastat) Precision Medicine for Fabry Disease

Continue Launch Execution and Geographic Expansion

Early Success with International Launch (as of 2/28/17)

Initial Launch Success Driven by Germany with ERT-Switch & Naïve Patients, Reimbursement Now Available in 10 Countries*

75 Patients (Switch & Naïve) on reimbursed Galafold (2/28/17)

10 Countries with available reimbursement*

14 Countries with pricing discussions ongoing

27 Countries with Amicus footprint

Target Number of Patients on Reimbursed Galafold by YE17

*Commercial and Expanded Access Programs (EAPs)

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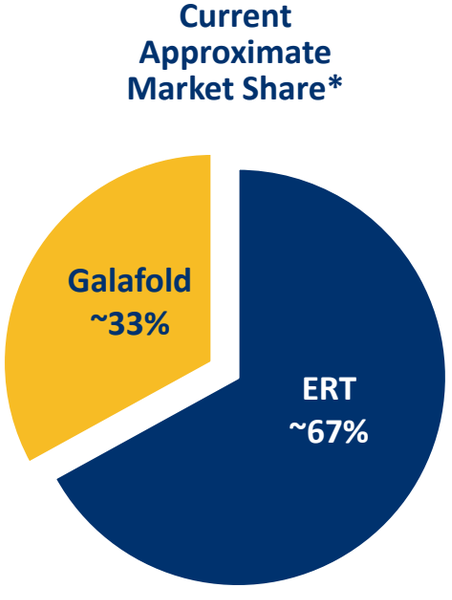
German Launch Update (as of 2/28/17)

Germany is an Important Indicator for EU Launch Success



IMPORTANT EARLY INDICATORS IN GERMANY

- Vast majority switch patients
- ~33% of eligible switch patients now on Galafold*
- All newly experienced patients & physicians
- Majority of switches from Replagal™
- Male / female mix
- 16 unique prescribers

*Market share assumptions based on estimated number of ERT-treated patients with amenable mutations in Germany as of May 2016

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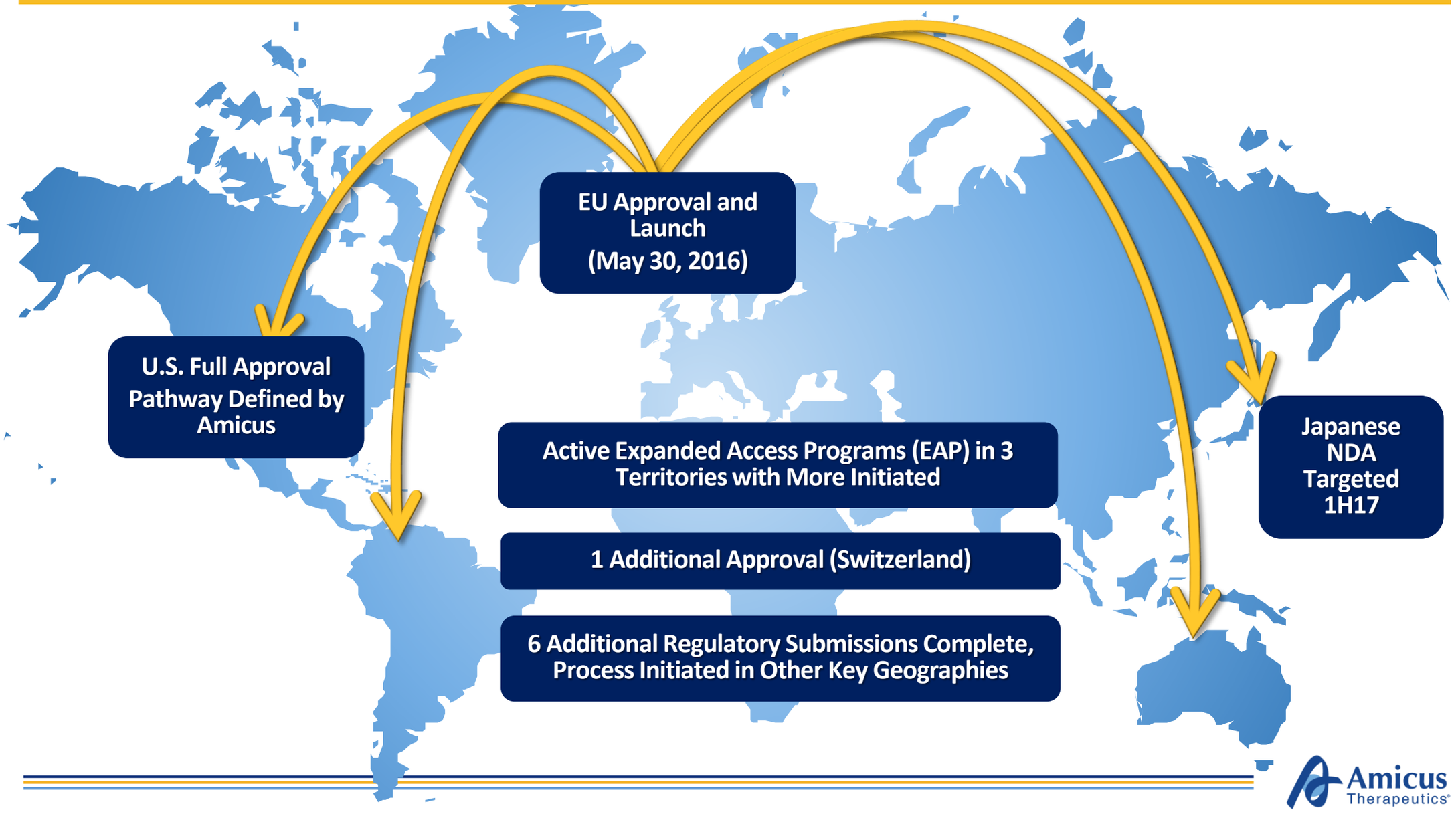
Global Regulatory Strategy to Reach More Patients

EU Approval is Gateway to ~75% of Global ERT Market



ATB200 Novel ERT for Pompe Disease

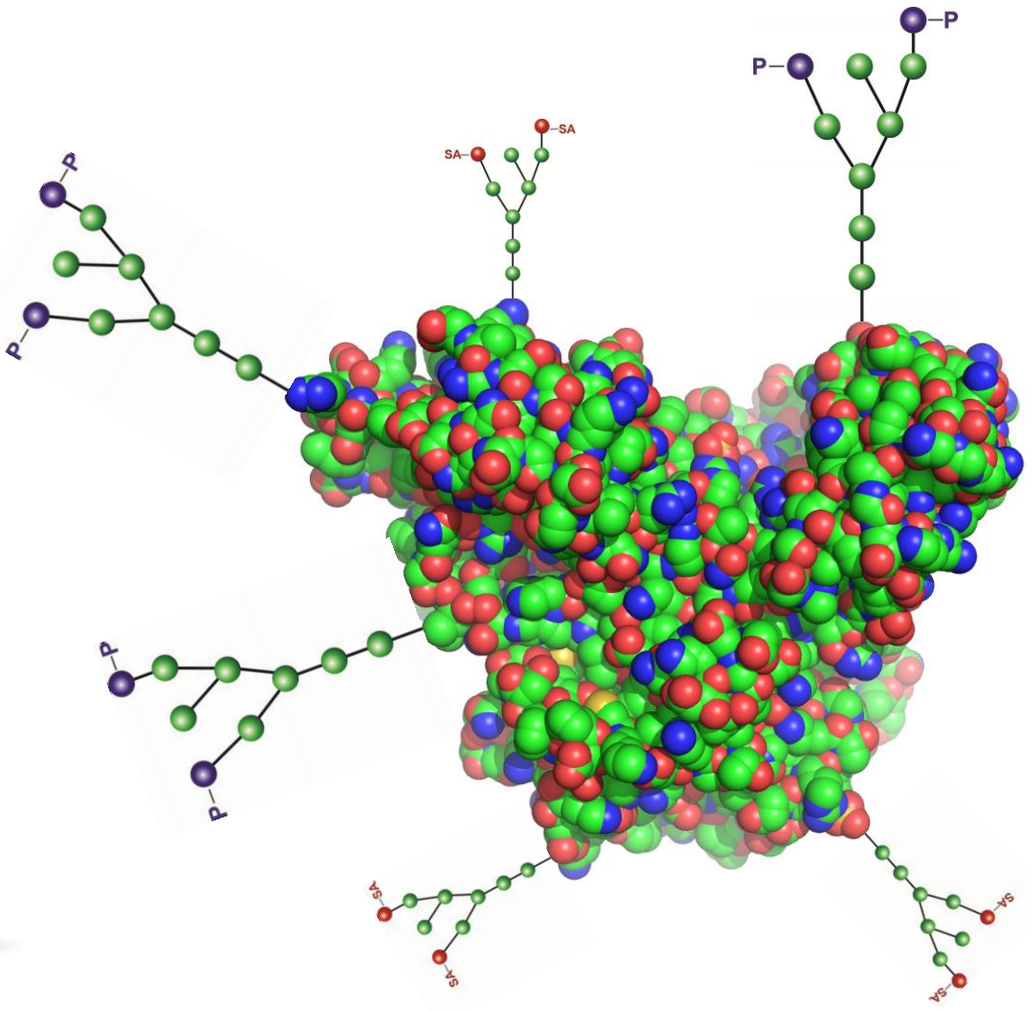
Establishing Human Proof of Concept and Validating Biologics Platform in 2017

ATB200 + Chaperone: A Highly Differentiated Approach

Novel Pompe Treatment Paradigm with Three Key Differentiators

ATB200 (Novel ERT)

Chaperone addition



Optimized mixture of glycans

High levels of M6P and bis M6P

*Artist rendering, not actual product image

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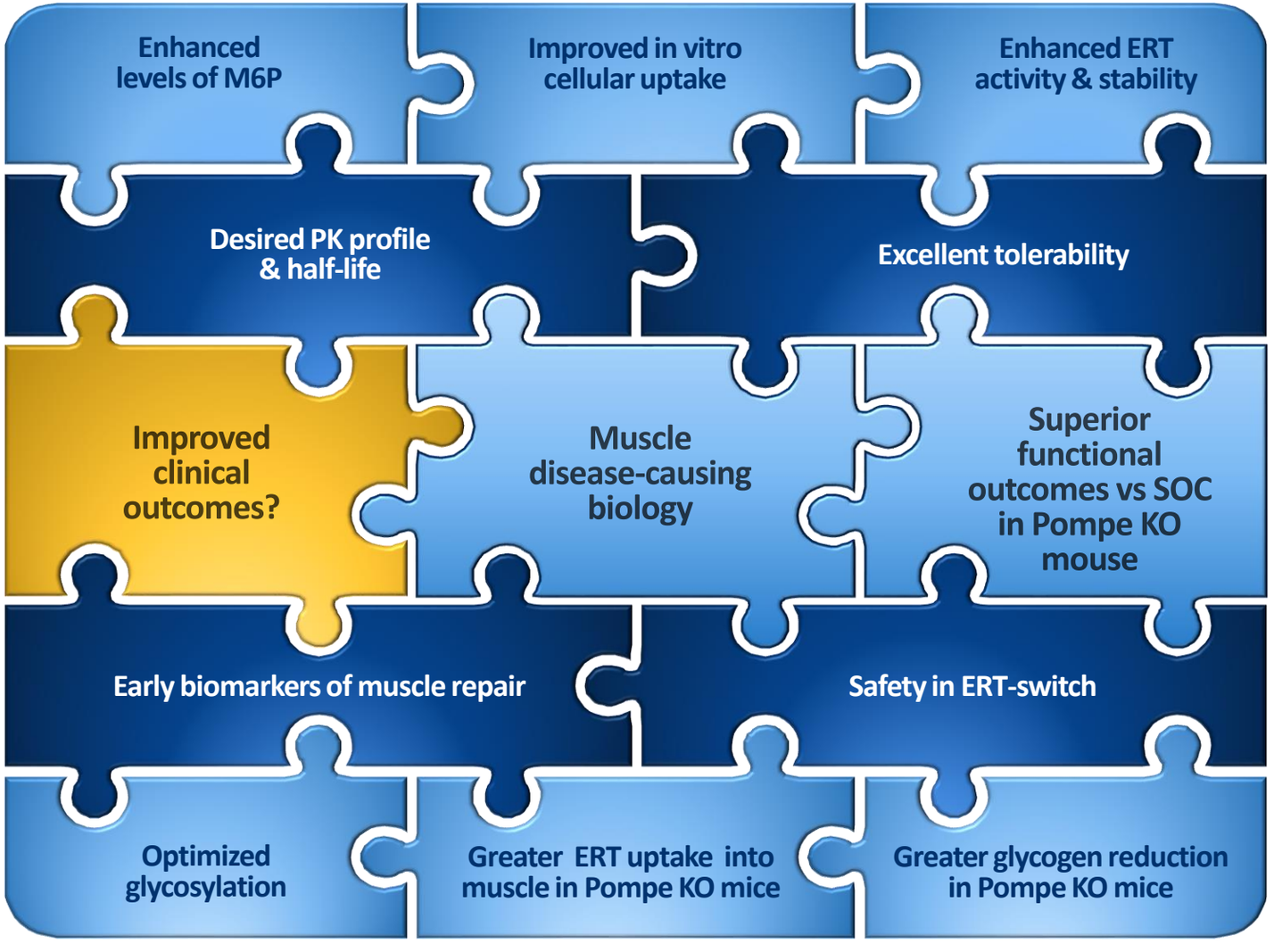
Pompe Disease: A Complex Disease with Significant Unmet Needs

We've Made Great Strides and Expect to Address Key Remaining Questions in 2017



preclinical | clinical | key question

"The scientific findings and preclinical data are profound and shed new light on questions about the underlying cause of muscle damage and weakness in Pompe patients. Furthermore, these results provide a window into a potential underlying link among key muscular dystrophies, such as Pompe, Limb Girdle, and Duchenne. Amicus has been a pioneer in advancing the scientific understanding of Pompe disease and in developing next-generation therapies for patients."

Grace K. Pavlath, Ph.D., Senior Vice President, Scientific Program Director of Muscular Dystrophy Association

Preliminary Clinical Data Summary

ATB200/AT2221 Demonstrates Promising Preliminary Results in Initial ERT-Switch and Naïve Patients at the Targeted Therapeutics Dose

- Safety (N=13)*
  - No serious adverse events (SAEs)
  - AEs were generally mild and transient
- Tolerability
  - No infusion-associated reactions following 150+ infusions in all patients enrolled to date
- PK (N=10)**
  - Clinical PK profile as predicted consistent with previously reported preclinical data
- Biomarkers of muscle damage (CK, AST, ALT) and substrate (urine Hex4) (N=10)**
  - Decrease or normalization of muscle injury biomarkers in a majority of patients
  - Decreases in urine Hex4 in all patients
  - Improvement in all biomarkers suggests positive effect of ATB200/AT2221 on muscle cells

*N=10 from Cohort 1 (Ambulatory ERT-Switch); N=1 from Cohort 2 (Non-Ambulatory ERT-Switch); N=2 from Cohort 3 (Naïve)
**N=8 from Cohort 1 & N=2 from Cohort 3

Pompe Clinical Study ATB200-02 Data Cascade

Target Enrollment Achieved with a Cascade of Additional Data Points During 2Q17 and 3Q17 to Demonstrate Proof of Concept

Pompe Milestones in 2017

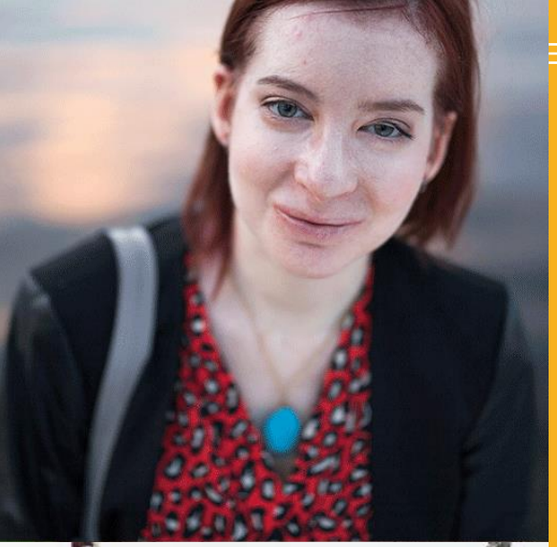
- Additional data & initial extension data in Cohort 1
- Data in non-ambulatory ERT-switch patients (Cohort 2)
- Data in ERT-naïve patients (Cohort 3)
- Additional extension study data (all Cohorts)
- Meeting with U.S. and EU regulators

18-WEEK DATA

- Safety / tolerability
- Pharmacokinetics (PK)
- Biomarkers
- Immunogenicity

EXTENSION DATA

- Motor/pulmonary function



SD-101 for Epidermolysis Bullosa

Potential First-in-Class Treatment with Phase 3 Data Anticipated Mid-2017

Phase 3 Study - Delivering on Our EB Vision

Phase 3 Study Optimized for Success with Top-Line Data On Track for Mid-2017

SD-005 Study Design Optimized

- Sample size of up to 150 patients
- Larger baseline target wound size
- Time to wound closure endpoint elevated

Status

- 95%+ participation in extension study
- Enrollment near complete
- Top-line data anticipated mid-2017

Financial Summary

FY16 Select Financial Results

First-Ever Year to Report Product Revenue of $5M from Sales of Galafold

	December 31, 2016	December 31, 2015
Product revenue	$5.0M	-
R&D Expense	$104.8M	$76.9M
G&A Expense	$71.2M	$47.3M
Net Loss	($200.0M)	($132.1M)
Net Loss Per Share	(1.49)	(1.20)

Financial Summary & Guidance

Balance Sheet Strengthened with $330M Cash at 12/31/16 and Cash Runway Into 2H18

Financial Position	December 31, 2016
Cash	$330M
Debt	$250M
FY17 Net Operating Cash Flow Guidance	$175-$200M
FY17 Net Cash Spend Guidance*	$200-$225M
Cash Runway	2H18
Capitalization	**December 31, 2016**
Shares Outstanding	142,691,986

*Includes third party milestone payments and capital expenditures

Closing Remarks

Key Anticipated Milestones in 2017

2017

Fabry Disease (Galafold)	• 300 patients on reimbursed Galafold by YE17* • Japan NDA submission in 1H17 • Fabry GI study
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 mid-2017
Strong Balance Sheet	• Significant revenue contribution • Cash runway into 2H18

*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 Product

= 20 patients

~37 Patients

2010

~90 Patients

2014

~250 Patients*

YE16

~800 Patients*

2018

~5,000 Patients*

2023

*Clinical & Commercial

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Thank You

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