Amicus Therapeutics®

1Q17 Financial Results & Corporate Updates

Conference Call & Webcast

May 9, 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. 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 news release 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 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

Successful International Launch Underway (as of 4/30/17)

Initial Launch Success Driven by Germany with ERT-Switch & Naïve Patients, Reimbursement Now Available in 12 Countries Including Four of Top EU5*

101 Patients (Switch & Naïve) on reimbursed Galafold (4/30/17)

11 Countries with available reimbursement*

12 Countries with pricing discussions ongoing

27 Countries with Amicus footprint

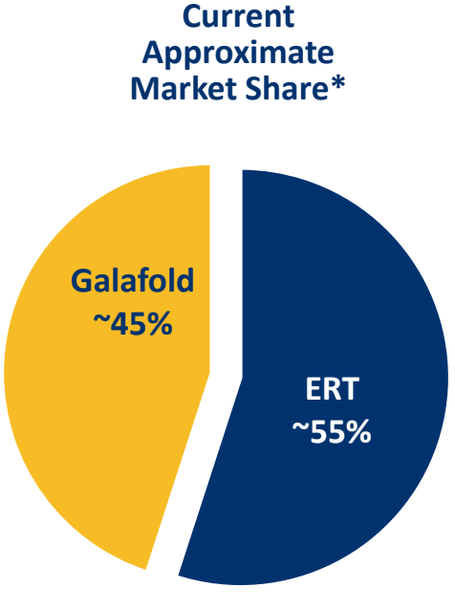
Target Number of Patients on Reimbursed Galafold by YE17

*Commercial and Expanded Access Programs (EAPs)

Amicus Therapeutics

German Launch Update (as of 4/30/17)

Germany is an Important Indicator for EU Launch Success



IMPORTANT EARLY INDICATORS IN GERMANY

- Majority switch patients, but growing naïve segment
- ~45% share of amenable patients (switch and naïve)*
- Switches from both Fabrazyme & Replagal™ commensurate with market share
- Male / female mix
- Most major centers prescribing
- Final price to be effective in 2Q17

*Market share assumptions based on estimated number of ERT-treated patients and naïve patients with amenable mutations in Germany as of April 2017

Amicus Therapeutics

UK Market Dynamics

Galafold Positioned for Success Following Positive Final NICE Publication and more than a Decade of Clinical Experience Among Largest Treatment Centers

MARKET DYNAMICS IN THE UK

- Funding effective May 23, 2017
- Highly concentrated at major centers
- Clinical experience at multiple sites
- ~450 ERT-treated patients
- 50%+ amenability rate projected*

"Migalastat has a lower total cost than ERT, and potentially provides greater health benefits than ERT."

-NICE Highly Specialised Technologies Guidance [HST4]**

* Estimates based on detailed market mapping and physician chart reviews
**Evidence-based recommendations on migalastat (Galafold) for treating Fabry disease in people over 16 - www.nice.org.uk/guidance/hst4

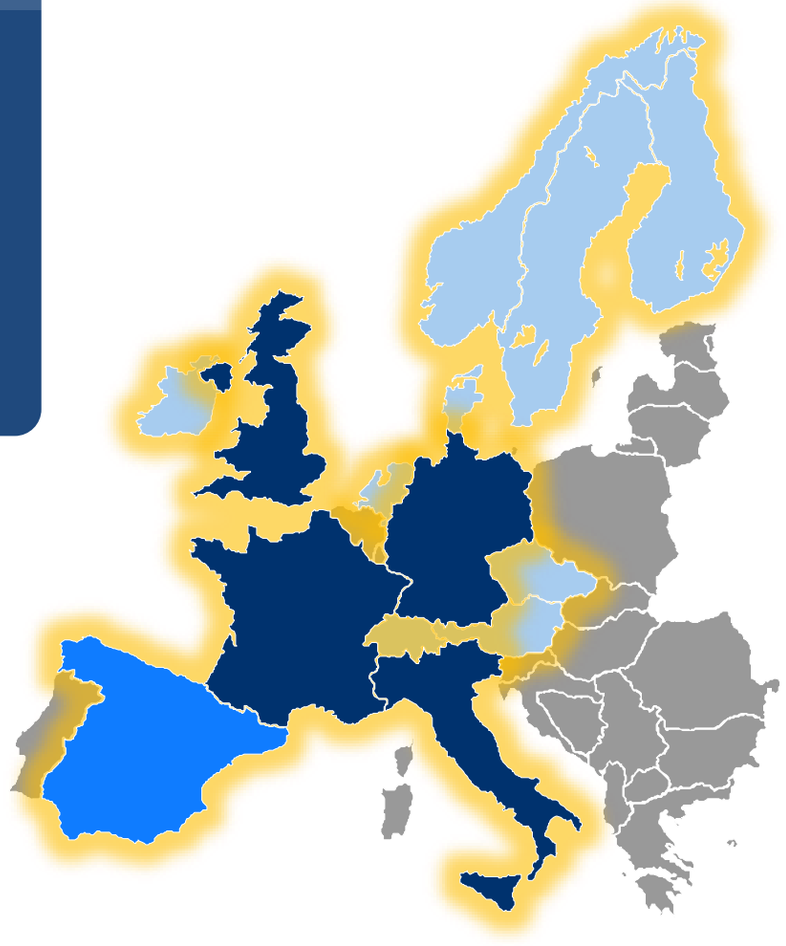
Amicus Therapeutics

EU Launch Strategy

Focus on EU Top 5 Plus Key Mid-Sized EU Markets in 2017

INITIAL FOCUS ON TOP 5 COUNTRIES

- Launched in Germany, UK, Italy and France
- Spain reimbursement discussions underway
- ~2,000 Fabry patients treated
- ~70-75% of EU market value
- ~25% of global Fabry market

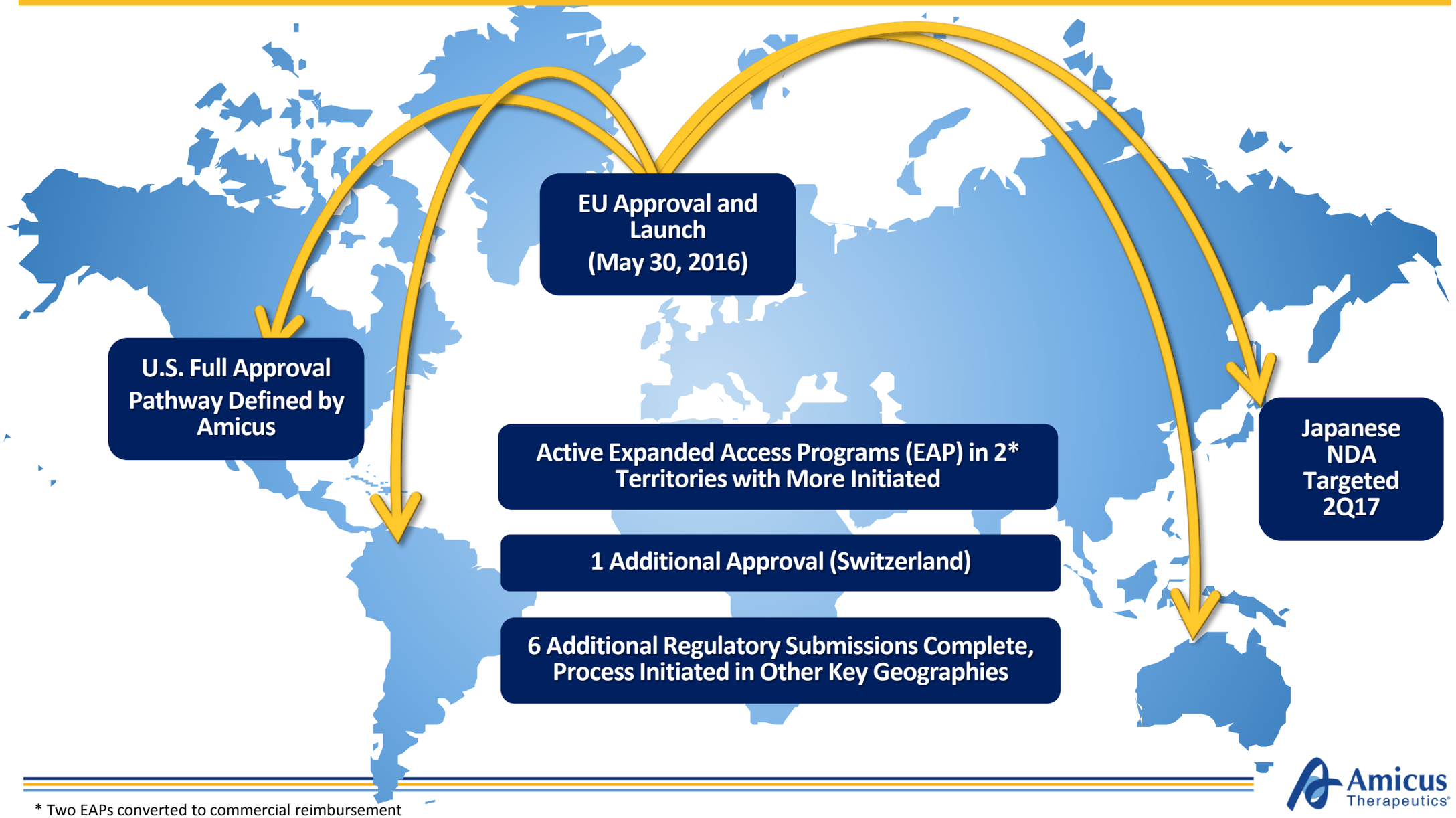


INVEST IN KEY MID-SIZED EU COUNTRIES AND SELECT EAP OPPORTUNITIES

- Austria, Nordics, Netherlands, Belgium, etc.
- ~10% of EU market value
- Selectively invest in key EAP markets

Global Regulatory Strategy to Reach More Patients

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



* Two EAPs converted to commercial reimbursement

Amicus Therapeutics

ATB200 Novel ERT for Pompe Disease

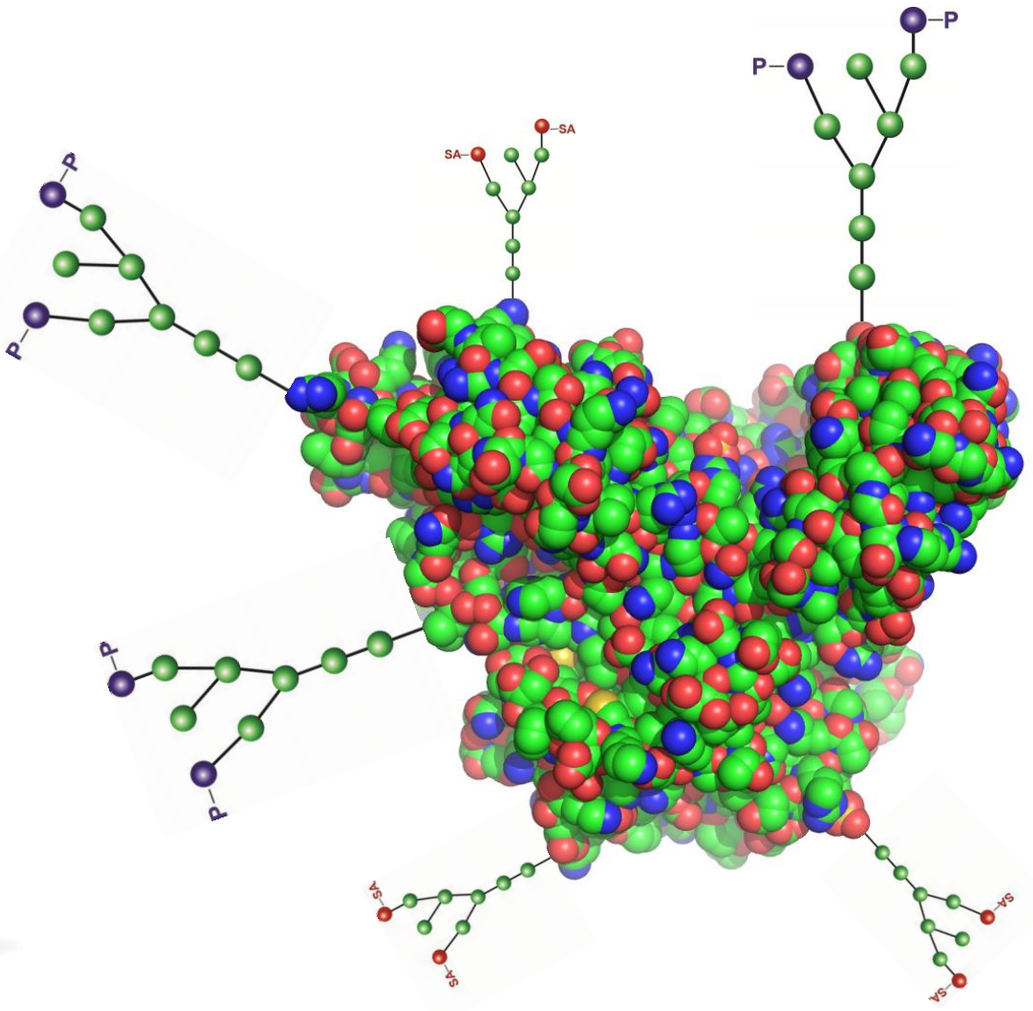
Establishing Human Proof of Concept and Validating Biologics Platform in 2017

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

Amicus Therapeutics

Phase 1/2 ATB200-02 Study Design

Phase 1/2 Clinical Study to Evaluate Safety, Tolerability, Pharmacokinetics (PK), and Pharmacodynamics (PD) of ATB200 + Chaperone (ATB200/AT2221)

18-Week Primary Treatment Period with Long-Term Extension (n=20)

Cohort 1 (Ambulatory ERT-Switch, n=11)

ATB200
5mg/kg (wk 2)
10mg/kg (wk 4)
20mg/kg (wk 6)

→ **ATB200 20mg/kg + AT2221 (Low Dose)** wks 8,10,12

→ **ATB200 20mg/kg + AT2221 (High Dose)** wk 14+

Cohort 2 (Non-Ambulatory ERT-Switch, n=4) & Cohort 3 (ERT-Naïve, n=5)

ATB200 20mg/kg + AT2221 (High Dose) wk 2+

Assessments:

- Plasma PK
- Safety/Tolerability
- Infusion-Associated Reactions
- Antibody & Cytokine Levels
- Pharmacodynamics
- Efficacy (Long-Term Extension)

Preliminary Clinical Data Summary (as of February 2017)

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

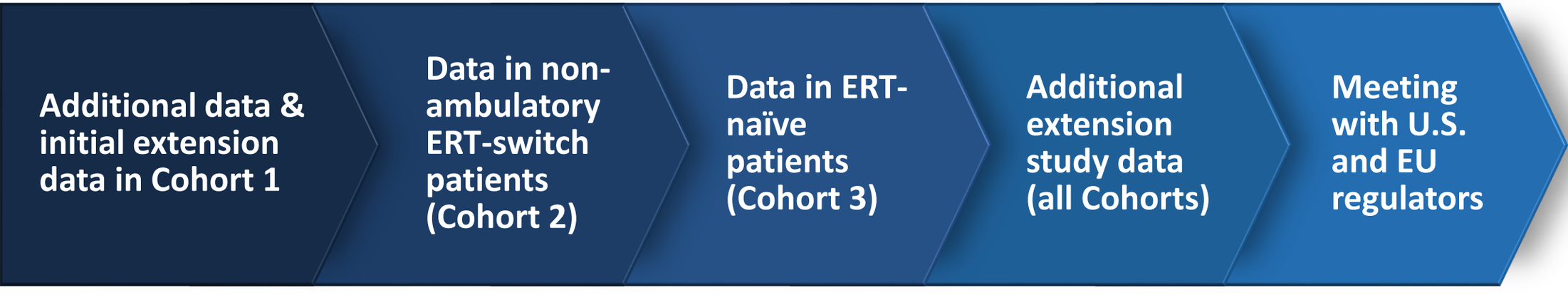
- Safety (N=13)*
  - No serious adverse events (SAEs) related to ATB200/AT2221
  - AEs were generally mild and transient
- Tolerability
  - No infusion-associated reactions following 150+ infusions in all patients enrolled to date
- PK (N=10)**
  - Clinical PK profile as predicted consistent with previously 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

A Cascade of Additional Data Points on Track for 2Q17 and 3Q17 to Demonstrate Proof of Concept

Pompe Milestones in 2017

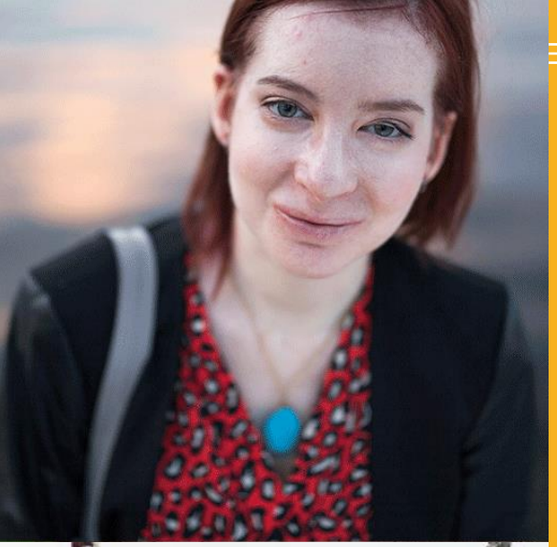


18-WEEK DATA

- 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 3Q17

Phase 3 ESSENCE Study - Delivering on Our EB Vision

Phase 3 Study Overenrolled (>160 Patients) with Top-Line Data On Track for 3Q17

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
- Study overenrolled (>160 patients)
- Top-line data anticipated 3Q17

Financial Summary

1Q17 Select Financial Results

Continue to Focus on Revenue of $4.2M from Sales of Galafold

	March 31, 2017	March 31, 2016
Product revenue	$4.2m	-
R&D Expense	$30.9m	$23.4m
SG&A Expense	$19.1m	$15.7m
Net Loss	($55.0)	($43.7)
Net Loss Per Share	($0.39)	($0.35)

Financial Summary & Guidance

Strong Balance Sheet with $279.8M Cash at 3/31/17 and Cash Runway Into 2H18

Financial Position	March 31, 2017
Cash	$279.8M
Debt	$250M
FY17 Net Operating Cash Flow Guidance	$175-$200M
FY17 Net Cash Spend Guidance*	$200-$225M
Cash Runway	2H18
Capitalization	**March 31, 2017**
Shares Outstanding	142,829,530

*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 2Q17
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 2H18

*Commercial and Expanded Access Programs (EAPs)

Building a Top Global Biotech in Devastating Rare Diseases

Galafold (migalastat)

FIRST ORAL PRECISION MEDICINE FOR FABRY DISEASE

3 PROGRAMS
IN CLINIC IN 3 RARE DISEASES

1 BREAKTHROUGH
THERAPY DESIGNATION

WORLD CLASS SCIENCE & DRUG DEVELOPMENT

ATB200/AT2221
NOVEL TREATMENT PARADIGM FOR POMPE IN PHASE 1/2

TREATING PATIENTS IN 24 COUNTRIES

Two Phase 3 PROGRAMS
(FABRY & EB)

$3B+ MARKET OPPORTUNITY FOR CURRENT PIPELINE

PROTEIN ENGINEERING & GLYCOBIOLOGY

~$280M CASH BALANCE

Thank You

Amicus Therapeutics®