



3Q16 Financial Results and Program Updates

November 7, 2016

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 press release 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. 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 Annual Report on Form 10-K for the year ended December 31, 2015 and Quarterly Report on Form 10-Q for the quarter ended September 30, 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.

2016: Significant Progress with Key Strategic Priorities

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

Galafold International Launch

Migalastat Regulatory Approvals

Pompe & EB Clinical Studies

Balance Sheet Strength

Pipeline



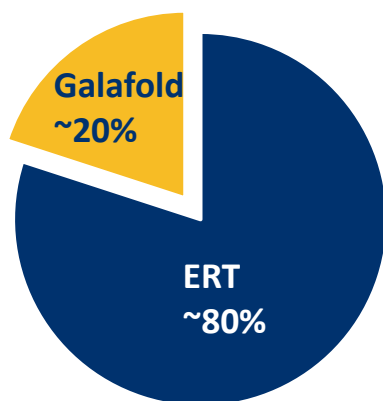
Galafold™ (Migalastat) Precision Medicine for Fabry Disease

International Launch Underway

International Launch Update

Successful First Full Quarter Driven by German Launch with ERT-Switch and Naïve Patients on Galafold

German Launch Update



Eligible ERT-Switch Patients¹

IMPORTANT EARLY INDICATORS IN GERMANY

- Vast majority switch patients
- ~20% of eligible switch patients now on Galafold¹
- All newly experienced patients & physicians



Patients (Switch & Naïve) on Reimbursed Galafold (10/31/16)



Countries with Active Pricing Discussions



Countries with Reimbursement (Commercial and EAP)

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

EU Launch Update

**Galafold Early Launch Strength in EU Market Representing 34% of FY15 ERT Global Sales (\$1.2B) –
Focusing on Patient Access and Reimbursement**

GERMANY

Diagnosed patients : ~1000 (~50% untreated)
Galafold launched – initial patients on treatment

FRANCE

ERT-treated patients : ~375 patients
Multiple patients treated under ATU

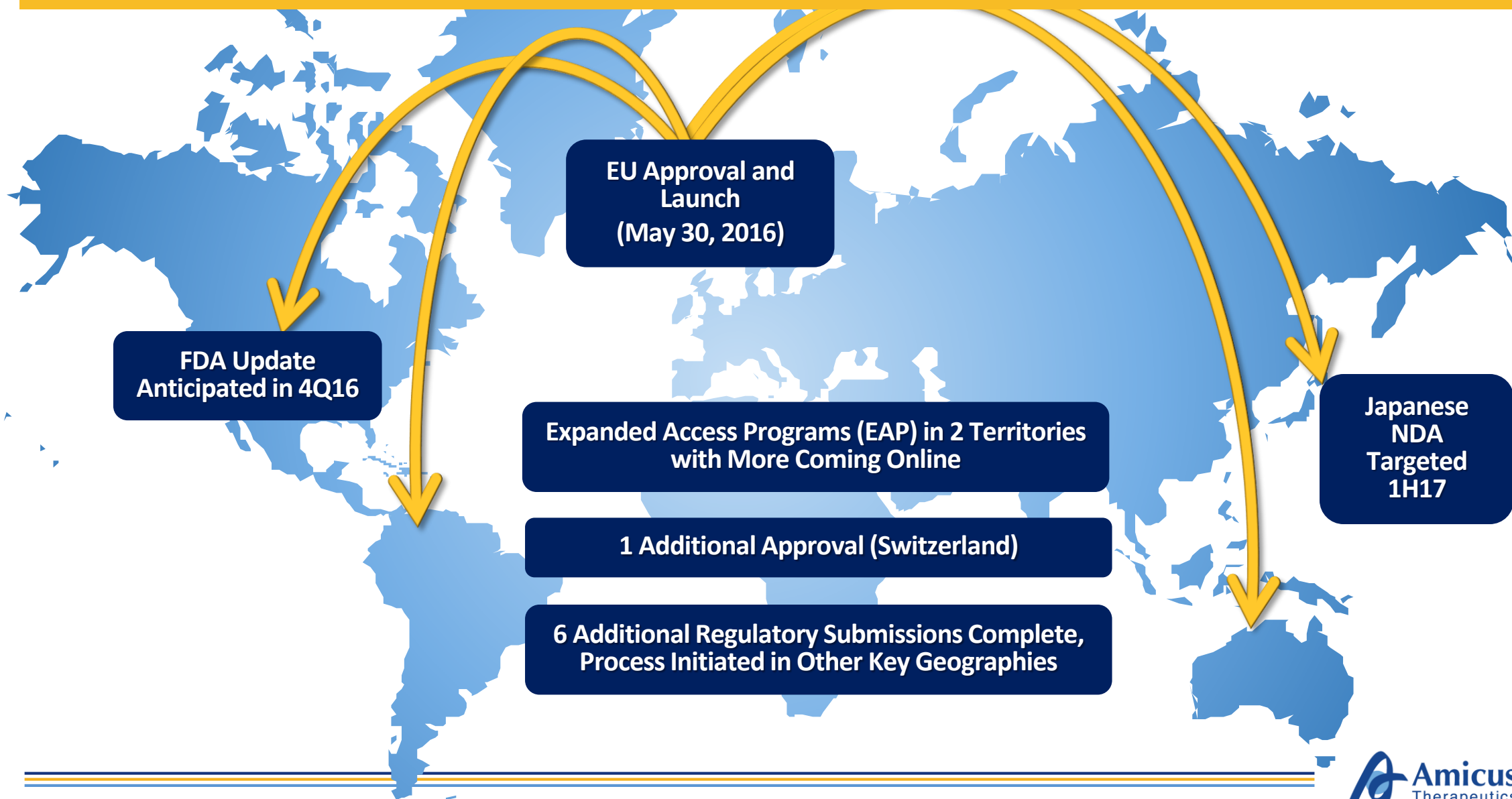
UNITED KINGDOM

ERT-treated patients: ~450
Highly Specialised Technology (HST)



Global Regulatory Strategy

Prioritizing Global Regulatory Submissions in Key Markets (US and Japan) with Additional Submissions Completed or Planned Based on EU Approval (MAA)





ATB200 Novel ERT for Pompe Disease

A Proprietary, Clinical-Stage Biologics Program

Pompe ERT - 3 Challenges

Amicus Technology Platforms with Potential to Address Challenges with Existing Pompe ERT

Activity/ Stability

Rapid denaturation
of ERT in pH of
blood¹

Protein
Aggregation



Tolerability / Immunogenicity

Infusion-associated
reactions in >50%
of late-onset patients³

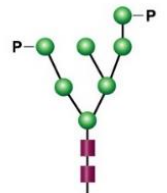
Antibody titers shown
to affect treatment
outcomes^{4,5}



Uptake/ Targeting

Low M6P receptor
uptake into
skeletal muscle²

Vast majority of
rhGAA not delivered
to lysosomes²



Uniquely Engineered
rhGAA Optimized M6P
& Carbohydrates

1Khanna et al., PLoS ONE, 2012; 2Zhu et al., Amer. Soc. Gene Therapy, 2009 June; 3Banati et al., Muscle Nerve, 2011 Dec.; 4Banugaria et al., Gen. Med., 2011 Aug.; 5de Vries et al., Mol Genet Metab., 2010 Dec.

Pompe Clinical Study ATB200-02 Data Cascade

A Cascade of Data Points from 4Q16 through 2017 Offer Clear Parameters to Define Success and Differentiate ATB200/AT2221

Pompe Data Cascade 4Q16 Through 2017

Data in initial ambulatory ERT-switch patients (Cohort 1, n=4)

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)

18-WEEK DATA

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

EXTENSION DATA

- Motor/pulmonary function

Pompe Clinical Study ATB200-02 Parameters for Success

Key Questions to Determine Potential for ATB200/AT2221 to Address 3 Major ERT Challenges in Initial 18-Week Treatment Period

CHALLENGES :

KEY QUESTIONS :

Safety



Do patients safely switch from standard of care to ATB200/AT2221?



Do patients tolerate ATB200/AT2221 with limited infusion-associated reactions?

Exposure, Targeting & Uptake



Is PK profile of ATB200/AT2221 differentiated and in optimal range consistent with preclinical studies?

Tolerability & Immunogenicity



Do antibodies in switch patients remain the same on ATB200/AT2221?



SD-101 for Epidermolysis Bullosa

EB Program Update - Phase 3 ESSENCE Study (SD-005)

Significant Momentum for Ongoing Study with Data on Track for 1H17



PHASE 3 ESSENCE STUDY STATUS

- 28 sites activated as of October 31, 2016
- 100% conversion to extension study (SD-006)
- SAP submitted to FDA for finalization
- Top-line Phase 3 data anticipated 1H17



Financial Summary

3Q16 Select Financial Results

First-Ever Quarter to Report Product Revenue of \$2.1M from Sales of Galafold

(\$000s) except per share data	September 30, 2016	September 30, 2015
Product revenue	2,127	-
R&D Expense	32,457	20,971
SG&A Expense	17,469	15,372
Net Loss	(46,654)	(37,800)
Net Loss Per Share	(0.33)	(0.32)

Strong Balance Sheet

**Balance Sheet Strengthened with ~\$39M in Equity
Since June 30 with Cash Runway Through Late 2017**

Financial Position	September 30, 2016
Cash:	\$212.4M
Debt	\$80.0M (\$66.0M net of discount for warrants issued)
FY16 Net Cash Spend Guidance:	\$135-\$155M (maintained)
Cash Runway	Late 2017
Full Allotment Raised in ATM (average price per share: \$6.67)	\$100M (\$61.7M in 2Q; \$39.3M in 3Q)
Capitalization	September 30, 2016
Shares Outstanding	142,273,085



Closing Remarks

Key Drivers of Value

3 Novel Product Candidates Each with \$500M to \$1B+ Market Potential

Fabry

- Galafold Precision Medicine (Small Molecule)
- EU Full Approval
- Launched in Germany (May 30, 2016)
- U.S. regulatory update on track for 4Q16

Epidermolysis Bullosa (EB)

- Phase 3 Novel Topical Medicine (SD-101)
- U.S. Breakthrough Therapy Designation
- Rolling NDA
- Phase 3 Data Targeted in 1H17

Pompe

- Novel ERT + Chaperone Treatment Paradigm
- Biologics Manufacturing
- Interim Data Anticipated in 4Q16

R&D Engine and Continued Business Development Activity