Amicus Therapeutics®

3Q17 Financial Results Conference Call & Webcast

November 8, 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 September 30, 2017, 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.

2017 Key Strategic Priorities

We Remain Sharply Focused on FOUR 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**
- **Maintain Financial Strength**

Galafold™ (Migalastat) Precision Medicine for Fabry Disease

Continue Successful Launch Execution and Geographic Expansion

Successful International Launch Underway (as of 10/31/17)

Driven by Top EU5 Countries, Galafold is Quickly Reaching ERT-Switch & Naïve Patients, Reimbursement Now Available in 13 Countries*

>260 Patients (Switch & Naïve) on reimbursed Galafold (10/31/17)

13 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)

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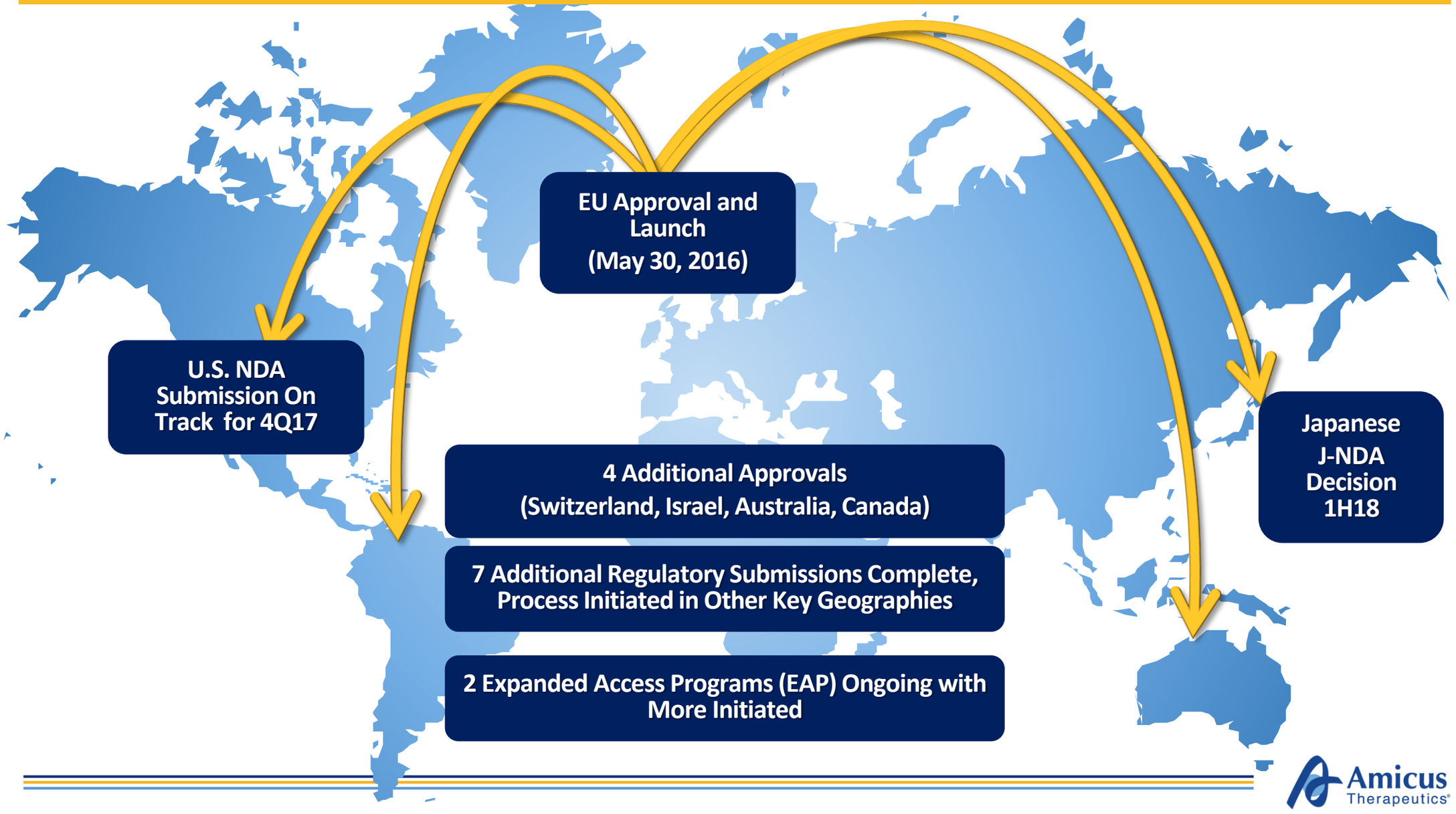
Global Regulatory Strategy to Reach More Patients (as of 10/31/17)

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



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ATB200 Novel ERT for Pompe Disease

Establishing Human Proof of Concept and Validating Biologics Platform in 2017

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) at 16 Sites in 5 Countries

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:

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

Summary of Available Data (as of 10/4/17)

	Cohort 1 (ERT-Switch, n=11)	Cohort 2 (Non-ambulatory ERT-Switch, n=4)	Cohort 3 (ERT-Naive, n=5)
Safety and tolerability	n=11	n=4	n=5
Biomarkers	n=11	n=4	n=5
Functional assessments at month	Month 6 (n=9)* Month 9 (n=8)	Month 6 (n=4)	Month 6 (n=5) Month 9 (n=2)
Functional assessments	6MWT Other motor function tests Pulmonary function (FVC, MIP/MEP)	Muscle Strength Tests	6MWT Other motor function tests Pulmonary function (FVC, MIP/MEP)

*One patient discontinued after completing Stage 1 (week 18) due to travel burden/family considerations and one patient's month 6 assessments pending due to an incomplete visit

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Study ATB200-02 Data Summary (as of 10/4/17)

Consistent and Durable Improvement in Muscle Function and Key Biomarkers as well as Stabilization or Improvement in Respiratory Function

- AEs generally mild and transient
- Very low rate of IARs (<1%) after 400+ total infusions
- Six-minute walk test (6MWT) distance increased out to Month 9
  - ERT-naïve patients: mean increases of +42m (Month 6) and +75m (Month 9)
  - ERT-switch patients: mean increases of +35m (Month 6) and +37m (Month 9)
- Muscle strength tests increased in non-ambulatory ERT-switch patients at Month 6
- Muscle function improved in 16/18 and 10/10 patients at months 6 and 9 respectively; other motor function tests consistent with 6MWT for ERT-naïve and ERT-switch patients
- Pulmonary function (FVC) generally stable in ERT-switch patients and increased in ERT-naïve patients
- Consistent and durable improvement in biomarkers of muscle damage (CK, ALT, AST) and disease substrate (Hex4) across all cohorts

Key Pompe Program Updates (as of November 2017)

Building a Robust Data Set in Pompe Patients

- Initiated retrospective natural history study (POM-002 Study)
- Commenced prospective observational study (POM-003 Study)
- Continuing collaborative discussions with U.S. and EU regulators
- Additional Phase 1/2 clinical data at WORLD*Symposium™* in February 2018

Biologics Manufacturing Capabilities

Scaling Up Manufacturing to Meet the Needs of the Pompe Community

Research Scale / MCB → **5L (Bench Scale)** → **250L (Clinical)** → **1000L** (Registration & Commercial)

Analytical and *in vivo* comparability studies completed between 250L and 1000L

- **Continued Success**
- **Ongoing clinical supply**

- **All engineering runs complete**
- **GMP production commenced**
- **Planned capacity flexibility**

Financial Summary

3Q17 Select Financial Results

3Q17 Revenue of $10.9M from Sales of Galafold (419% Increase Year-Over-Year)

($000s) except per share data	September 30, 2017	September 30, 2016
Product revenue	$10,874	$2,127
R&D Expense	40,641	32,457
SG&A Expense	21,647	17,469
Changes in fair value of contingent consideration	(244,250)	(4,110)
Loss on impairment of assets	465,427	-
Loss from operations	(275,232)	(44,940)
Income tax benefit	164,683	253
Net Loss	(111,666)	(46,654)
Net Loss Per Share	$(0.69)	$(0.33)

Financial Summary & Guidance

Strong Balance Sheet with $426.6 Cash at 9/30/17 and Cash Runway Into 2H19

Financial Position	September 30, 2017
Cash	$426.6M
Debt	$250M
FY17 Net Operating Cash Spend Guidance	$175-$200M
FY17 Net Cash Spend Guidance*	$200-$225M
Cash Runway	2H19
Capitalization	**September 30, 2017**
Shares Outstanding	165,491,141

*Includes third party milestone payments and capital expenditures.

Closing Remarks

Key Anticipated Milestones

2017/1H18

Fabry Disease (Galafold)

- U.S. NDA submission in 4Q17
- 300 patients on reimbursed Galafold by YE17*
- Decision on Japanese regulatory submission (J-NDA) in 1H18

Pompe Disease (ATB200/AT2221)

- Ongoing discussions with U.S. and EU regulators
- Additional clinical data at WORLD*Symposium* (February 2018)
- Regulatory update in 1H18

Strong Balance Sheet

- Significant revenue contribution
- Cash runway into 2H19

*Commercial and Expanded Access Programs (EAPs)

Thank You

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