Amicus Therapeutics®

Pompe Phase 1/2 Study (ATB200-02) Preliminary Data

Conference Call & Webcast

December 8, 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. 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. 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 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.

Pompe Disease Overview

Severe, Fatal, Genetic Disorder with Significant Unmet Medical Need

- Deficiency of acid alpha-glucosidase (GAA) leading to glycogen accumulation
- Age of onset ranges from infancy to adulthood
- Symptoms include muscle weakness, respiratory failure, and cardiomyopathy
- Respiratory and cardiac failure are leading causes of morbidity and mortality
- 5,000 – 10,000 patients diagnosed WW[1]
- ~$800M+ Global Pompe ERT sales in FY15[2]

1. National Institute of Neurological Disorders and Stroke (NIH). 2. Sanofi Press Release & 10-K

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 blood[1]

Protein Aggregation

CHART™ CHAPERONE-ADVANCED REPLACEMENT THERAPY

Tolerability / Immunogenicity

Infusion-associated reactions in >50% of late-onset patients[3]

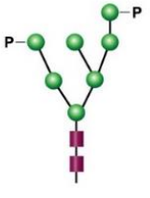
Antibody titers shown to affect treatment outcomes[4,5]

CHART™ CHAPERONE-ADVANCED REPLACEMENT THERAPY

Uptake/ Targeting

Low M6P receptor uptake into skeletal muscle[2]

Vast majority of rhGAA not delivered to lysosomes[2]



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.

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Study Design

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

Stage 1 (Single Ascending Dose)

Single Dose ATB200 Every Other Week

- ATB200 5 mg/kg — Week 2
- ATB200 10 mg/kg — Week 4
- ATB200 20 mg/kg — Week 6

Stage 2 (Multiple Ascending Dose)

Fixed Dose ATB200 + Chaperone (AT2221) Every Other Week

- ATB200 20mg/kg + AT2221 (Low Dose) — Weeks 8, 10, 12
- ATB200 20mg/kg + AT2221 (High Dose) — Weeks 14, 16, 18

Long-Term Extension

Fixed Dose ATB200 + Chaperone (AT2221) Every Other Week

Assessments:

- Plasma PK (Enzyme Activity & Total protein)
- Safety/Tolerability
- Antibodies
- Infusion-Associated Reactions
- Pharmacodynamics
- Efficacy (Long-Term Extension)

Baseline Characteristics (n=8)*

Initial ERT-Switch Patients are Representative of Pompe Population with Mean 5.4 Years on Myozyme/Lumizyme

Baseline Characteristics	ATB200-02 (N=8)
Time on Lumizyme® – mean years (SD)	5.41 (0.991)
Age – mean years (range)	49.8 (42 , 59)
Sex M/F %	75/25
6MWT mean meters (SD)	377.5 (96.12)
FVC Upright mean % predicted (SD)	50.6 (14.43)

*N = 8 from Cohort 1 (Ambulatory ERT-Switch) through interim data analysis (maximum 24 weeks)

Safety Summary (n=9)*

Preliminary Safety Data for ATB200/AT2221 in Initial Patients Showed No Infusion-Associated Reactions Following 100+ Infusions

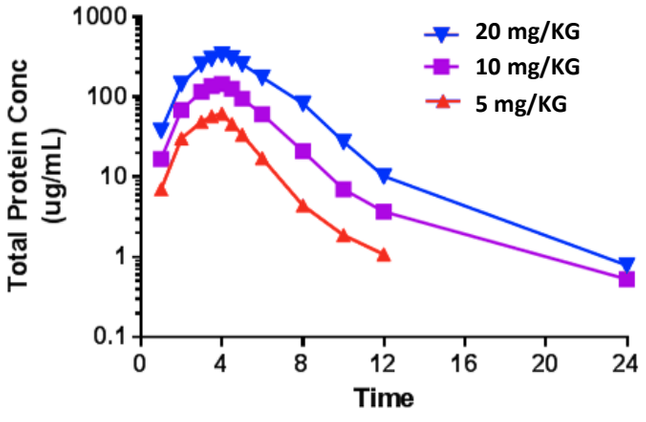
- No Serious Adverse Events (SAEs)
- AEs were generally mild and transient:
  - AEs reported in 6/9 (67%) patients
- Treatment-related AEs reported in 2/9 (22%) patients
  - Headache, acne and nausea
- Safety Steering Committee reviewed initial safety data
- No infusion-associated reactions reported after 100+ total infusions in all patients

*N = 8 from Cohort 1 (Ambulatory ERT-Switch), 1 from Cohort 2 (Non-Ambulatory ERT-Switch) through interim data analysis (maximum 24 weeks)

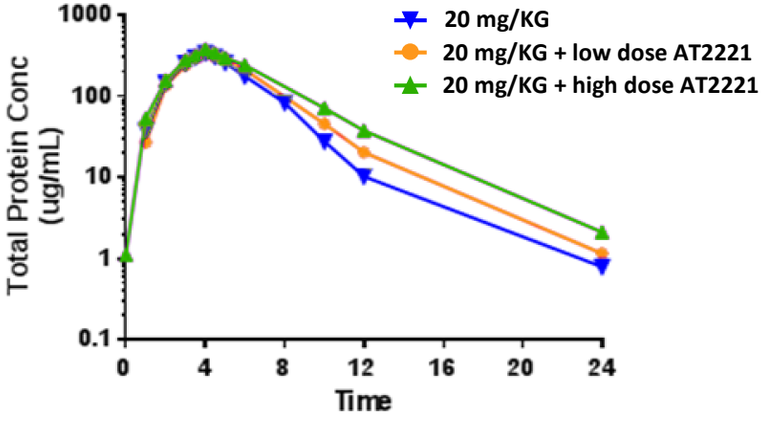
Pharmacokinetics at Week 14: Plasma Exposure (n=4)*

ATB200 Clinical PK Profile as Predicted Based on Preclinical Studies with Greater than Dose Proportional Increases in Exposure that were Enhanced by AT2221

Mean GAA Total Protein (n=4)
5, 10, 20 mg/kg ATB200 Alone



Mean GAA Total Protein (n=4)
20 mg/kg ATB200 + AT2221



Treatment	Mean $AUC_{0-\infty}$ (hr*µg/ml)	Mean Clearance (L/hr)
5 mg/kg	215	1.97
10 mg/kg	589	1.45
20 mg/kg	1547	1.11

Treatment	Mean $AUC_{0-\infty}$ (hr*µg/ml)	Mean Clearance (L/hr)
20 mg/kg	1547	1.11
+low dose AT2221	1676	1.03
+high dose AT2221	1945	0.90

*N = 4 from Cohort 1 (Ambulatory ERT-Switch)

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Muscle Damage Biomarkers at Week 14 (n=4)*

After Switching from Lumizyme to ATB200/AT2221, Muscle Damage Biomarkers (CK, AST, ALT) Trended Toward Early Improvement in Two Patients and Were Stable in the Other Two Patients

- Two patients showed early trend toward improvement in all three biomarkers
  - 44%, 28%, 34% reduction in CK, AST, ALT respectively
  - 31%, 22%, 11% reduction in CK, AST, ALT respectively
- Two patients remained stable

*Elevated **creatine kinase (CK), alanine aminotransferase (ALT),** and **aspartate aminotransferase (AST)** are indicators of damage to muscle tissue*

*N = 4 from Cohort 1 (Ambulatory ERT-Switch)

Preliminary Data Summary

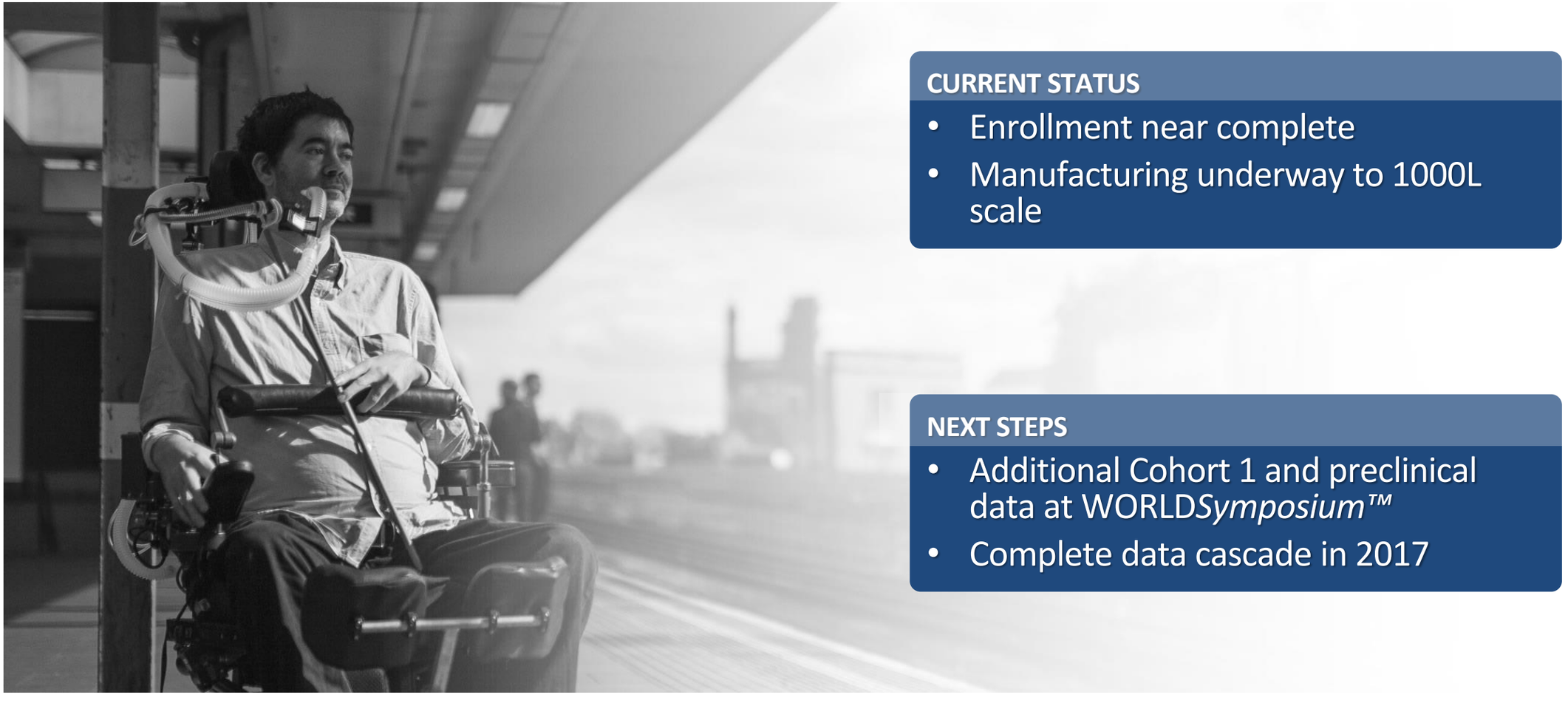
ATB200/AT2221 Demonstrates Promising Preliminary Results in First ERT-Switch Patients at the Targeted Therapeutics Dose

- Safety (n=9)*
  - No serious adverse events (SAEs)
  - AEs were generally mild and transient
- Tolerability
  - No infusion-associated reactions following 100+ infusions in all patients enrolled to date
- PK (n=4)**
  - Clinical PK profile as predicted consistent with previously reported preclinical data
  - ATB200 plasma clearance rate suggests optimized carbohydrate structure provides efficient uptake into tissues
  - ATB200 alone showed greater than dose-proportional increases in exposure, which was further enhanced with AT2221
- Muscle damage biomarkers (CK, AST, ALT) (n=4)
  - Early trend to improvement in 2 patients and stable in 2 patients
- Immunogenicity (n=4)
  - All patients had anti-rhGAA antibodies at baseline which remained generally stable
  - Cytokines remained low and stable during infusions

*N = 8 from Cohort 1 (Ambulatory ERT-Switch) and 1 from Cohort 1 (Non-Ambulatory ERT-Switch); through interim data analysis (maximum 24 weeks)
**N = 4 from Cohort 1

Current Status and Next Steps

Enrollment Near Complete with Manufacturing Scale Up Underway to Support Phase 3/Commercial



CURRENT STATUS

- Enrollment near complete
- Manufacturing underway to 1000L scale

NEXT STEPS

- Additional Cohort 1 and preclinical data at WORLD*Symposium*™
- Complete data cascade in 2017

Pompe Clinical Study ATB200-02 Data Cascade

A Cascade of Additional Data Points During 2017 to Culminate in 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)

End of Phase 2 Meeting

18-WEEK DATA

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

EXTENSION DATA

- Motor/pulmonary function

Thank You

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