UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT PURSUANT TO
SECTION 13 OR 15(D) OF THE
SECURITIES EXCHANGE ACT OF 1934

Date of Report (Date of earliest event reported): **October 4, 2017**

AMICUS THERAPEUTICS, INC.
(Exact Name of Registrant as Specified in Its Charter)

Delaware
(State or Other Jurisdiction of Incorporation)

001-33497	**71-0869350**
(Commission File Number)	(IRS Employer Identification No.)
1 Cedar Brook Drive, Cranbury, NJ	**08512**
(Address of Principal Executive Offices)	(Zip Code)

Registrant's telephone number, including area code: **(609) 662-2000**

(Former Name or Former Address, if Changed Since Last Report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company o

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. o

Item 8.01. Other Events.

On October 4, 2017, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing that additional positive data in its Pompe Disease Phase 1/2 study will be presented at the 22nd International Congress of the World Muscle Society in a late-breaker poster. A copy of this press release and poster is attached hereto as Exhibit 99.1. The Company will also host a conference call and webcast on October 4, 2017 to discuss these results. A copy of the conference call presentation materials is also attached hereto as Exhibit 99.2.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits: The Exhibit Index annexed hereto is incorporated herein by reference.

Exhibit No.	Description
99.1	Press Release dated October 4, 2017 titled "Amicus Therapeutics Announces Additional Positive Data in Pompe Disease Phase 1/2 Study at World Muscle Society."
99.2	Conference Call Presentation Materials

EXHIBIT INDEX

Exhibit No.	Description
99.1	Press Release dated October 4, 2017 titled "Amicus Therapeutics Announces Additional Positive Data in Pompe Disease Phase 1/2 Study at World Muscle Society."
99.2	Conference Call Presentation Materials

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Amicus Therapeutics, Inc.

Date: October 4, 2017

By: /s/ ELLEN S. ROSENBERG
Ellen S. Rosenberg
General Counsel and Corporate Secretary

Amicus Therapeutics Announces Additional Positive Data in Pompe Disease Phase 1/2 Study at World Muscle Society

Mean Six-Minute Walk Distance Improved in ERT-Naive Patients (+42 Meters at 6 Months, +75 Meters at 9 Months) and ERT-Switch Patients (+35 Meters at 6 Months, +37 Meters at 9 Months)

Persistent & Durable Reductions in Key Disease Biomarkers

Pulmonary Function Generally Improved or Remained Stable at 6 and 9 Months

Very Low Number (<1%) of Infusion Associated Reactions Observed After 400+ Infusions

Conference Call at 8:30am ET

CRANBURY, NJ, October 4, 2017 — Amicus Therapeutics (Nasdaq: FOLD) today announced additional positive results from a global Phase 1/2 clinical study (ATB200-02) to investigate ATB200/AT2221 in patients with Pompe disease, an inherited lysosomal storage disorder caused by an enzyme deficiency that leads to accumulation of glycogen (disease substrate) in cells. Consistent with previous results, patients who completed six months of treatment with ATB200/AT2221 showed improvements in six-minute walk test (6MWT) distance and other measures of motor function, stability or increases in forced vital capacity (FVC), and further reductions in biomarkers of muscle damage and disease substrate, with consistent results reported in initial patients who completed nine months of treatment. These clinical results were featured at the 22nd International Congress of the World Muscle Society in a late-breaker poster(1). With these data, Amicus plans to continue a series of collaborative discussions with regulators in the US and EU, and expects to provide an update in the first half of 2018.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics stated, "These remarkable data from our Pompe clinical study of ATB200/AT2221 have once again exceeded our expectations. The consistency, durability and magnitude of the functional outcomes align with significant and continued reductions in key biomarkers of muscle damage and disease substrate, across patients, across cohorts and over significant periods of time. Taken together the strength of these results suggest the effect of ATB200/AT2221 may be very clinically meaningful for people living with Pompe disease. We are committed to working collaboratively with regulators to determine the fastest regulatory pathways that may be available to bring this new treatment paradigm to as many patients living with Pompe disease globally, as quickly as possible."

Mark Roberts, MD, Department of Neurology, Salford Royal NHS Foundation Trust and Principal Investigator in the ATB200-02 study stated, "I believe that the results from this Phase 1/2 clinical study show striking improvements in functional measures and key biomarkers during the first six months of treatment, in addition to continued, further benefit out to nine months. I am especially intrigued by the six-minute walk distance and other motor function tests in the ERT-switch patients who historically have declining motor function following two or more years of treatment. These clinical data are compelling and suggest that ATB200/AT2221 has the potential to shift the treatment paradigm for Pompe disease."

ATB200-02 Full Study Data — Highlights in ERT-Switch and ERT-Naive Patients

Safety, Tolerability & Pharmacokinetics (PK) (n=20)

Safety and tolerability data in all 20 patients reflect a maximum of 72 weeks of treatment. To date, adverse events have been generally mild and transient. Importantly, ATB200/AT2221 has resulted in a low rate of infusion-associated reactions

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(IARs) following 400+ infusions (three events of IARs in two patients; <1% of all 400+ infusions with an IAR). As previously reported, the clinical pharmacokinetic profile has been consistent with previously reported preclinical data.

Pharmacodynamic (PD) Data on Muscle Damage and Disease Substrate Biomarkers (n=20)

Treatment with ATB200/AT2221 resulted in reductions in key disease biomarkers across all patient cohorts after up to 58 weeks and continue to suggest a positive effect on muscle cells.

- **Muscle damage biomarkers**: Creatine kinase (CK) enzyme, alanine aminotransferase (ALT), and aspartate aminotransferase (AST), are biomarkers indicative of muscle damage in Pompe disease. Mean reductions from baseline were approximately 25-35%, 5-25% and 40-55% for the ambulatory ERT-switch (n=11), non-ambulatory ERT-switch (n=4) and ERT-naïve (n=5) patients, respectively.

- **Disease substrate biomarker**: Urine hexose tetrasaccharide (Hex4) is a biomarker of glycogen build-up. Mean reductions from baseline were approximately 40%, 35% and 55% for the ambulatory ERT-switch (n=11), non-ambulatory ERT-switch (n=4) and ERT-naïve (n=5) patients, respectively.

Functional Outcomes (n=18)

Data on 6-month functional outcomes are available for 18 of the 20 patients enrolled (one patient dropped out of the extension study due to travel burden and family considerations, while month 6 assessments are pending in one patient due to an incomplete visit). Muscle function improved in 16 patients and was stable in two patients at month 6. Muscle function improved in 10 out of 10 patients with available data at month 9.

- **Muscle Function:**
  - **Motor function (n=14):** Six-minute walk test (6MWT) distance, a primary measure of motor function in Pompe disease patients, improved in both ERT-naive and ERT-switch patients and was durable to month 9. ERT-naive patients showed mean increases of 42 meters at month 6 (n=5) and 75 meters at month 9 (n=2). ERT-switch patients showed mean increases of 35 meters at month 6 (n=9) and 37 meters at month 9 (n=8). Other motor function tests showed mean improvements consistent with 6MWT distance.
  - **Muscle Strength (n=4):** All four non-ambulatory ERT-switch patients showed improvements in upper extremity strength (elbow and shoulder) from baseline to month 6, as measured by quantitative muscle testing (QMT) and manual muscle testing (MMT).
- **Pulmonary Function:** Forced vital capacity (FVC), the primary measure of pulmonary function in Pompe disease, improved in ERT-naïve patients, with mean absolute change in percent predicted FVC of +4.2% at month 6 (n=5) and +5.0% at month 9 (n=2). FVC was generally stable in ERT-switch patients with mean absolute change in percent predicted FVC of -1.0% at month 6 (n=8) and -2.0% at month 9 (n=7). Other pulmonary tests included maximal inspiratory pressure (MIP), a measure of inhalation, and maximal expiratory pressure (MEP), a measure of exhalation. MIP and MEP were generally stable or increased in both ERT-naïve and ERT-switch patients.

Summary of Functional Outcomes from Baseline to Month 6

Cohort 1 ERT-Switch Patients: Functional Outcomes on ATB200/AT2221 from Baseline to Month 6 and 9

Motor Function Tests						Pulmonary Function Tests		
6MWT (m)	4 Stair Climb	Timed up and	10m walk	Gowers#	GSGC Score	FVC (%)	MIP	MEP

		(sec)	go (sec)	(sec)					
Baseline Mean (SD) (n=10)	**397.2 (96.8)**	**4.1** (2.7)	**10.5** (6.6)	**7.4** (3.0)	**7.9** (2.8)	**12.6** (4.8)	**52.6** (14.7)	**35.7** (11.0)	**72.6** (32.6)
Change at Month 6 (SD) (n=9)	**+35.3** (40.1)	**-1.0** (1.2)	**-2.2** (3.4)	**-0.3** (1.6)	**-2.2** (2.0)	**-0.8** (3.0)	**-1.0** (4.2)	**+0.9** (4.5)	**+20.3** (42.4)
Change at Month 9 (SD) (n=8)	**+37.2** (33.8)	**-0.9** (1.3)	**-0.6** (2.5)	**0.1** (1.3)	**-2.1** (1.3)	**-0.9** (3.5)	**-2.0** (3.6)	**-1.4** (2.7)	**+31.1** (39.3)

Cohort 3 ERT-Naïve Patients: Functional Outcomes on ATB200/AT2221 from Baseline to Month 6 and 9

	Motor Function Tests						Pulmonary Function Tests		
	6MWT (m)	4 Stair Climb (sec)	Timed up and go (sec)	10m walk (sec)	Gowers#	GSGC Score	FVC (%)	MIP	MEP
Baseline Mean (SD) (n=5)	**399.5 (83.5)**	**4.2** (1.5)	**9.4** (2.9)	**7.9** (3.0)	**13.9** (11.0)	**12.2** (3.6)	**53.4** (20.3)	**32.6** (18.5)	**60.6** (8.3)
Change at Month 6 (SD) (n=5)	**+41.8** (29.4)	**-0.6** (0.3)	**-1.0** (1.1)	**-0.7** (1.1)	**7.9*** (21.0)	**-1.8** (3.8)	**+4.2** (5.6)	**+11.0** (5.0)	**-0.4** (12.4)
Change at Month 9 (SD) (n=2)	**+74.9** (4.0)	**-0.8** (0.3)	**-1.6** (1.0)	**-1.0** (0.1)	**-1.3** (0.0)	**-4.0** (1.4)	**+5.0** (1.4)	**+1.5** (0.7)	**-1.0** (19.8)

*Median change from baseline was -0.8 and 4/5 had decrease; # N=9 Missing values not obtained due to patient refusal to perform test

Conference Call and Webcast

Amicus Therapeutics will host a conference call and webcast today, October 4, 2017 at 8:30 a.m. ET. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international); conference ID 96220532. The slide presentation to accompany this conference call and webcast will be available at http://ir.amicusrx.com/events.cfm.

An audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at http://ir.amicusrx.com/events.cfm, and will be archived for 30 days. Web participants are encouraged to go to the web site 15 minutes prior to the start of the call to register, download and install any necessary software. A telephonic replay of the call will be available for seven days beginning at 11:30 a.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID 96220532.

About ATB200-02 Clinical Study

The primary objectives of the open-label ATB200-02 clinical study are to evaluate the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of ATB200/AT2221 over an 18-week primary treatment period followed by a long-term extension. Sixteen clinical sites in five countries participated in the ATB200-02 clinical study. The study enrolled a total of 20 patients at 16 participating sites in five countries across three patient cohorts: ambulatory ERT-switch (Cohort 1, n=11), non-ambulatory ERT-switch (Cohort 2, n=4) and ERT-naïve (Cohort 3, n=5). Patients in Cohort 1 received escalating doses of ATB200 (5, 10, 20 mg/kg), followed by 3 doses of 20 mg/kg ATB200 plus low dose AT2221, followed by ongoing doses of 20 mg/kg ATB200 plus high dose AT2221. Patients in Cohort 2 and 3 patients have all received 20 mg/kg ATB200 plus high dose AT2221.

About ATB200/AT2221

ATB200/AT2221 is a novel treatment paradigm that consists of ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose-6 phosphate (M6P), to enhance uptake, co-administered with AT2221, a pharmacological chaperone. In preclinical studies, ATB200 was associated with increased tissue enzyme levels and reduced glycogen levels in muscle, which was further improved when AT2221 was co-administered with ATB200. Amicus Therapeutics is currently conducting a global Phase 1/2 study (ATB200-02) to evaluate the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics of ATB200/AT2221.

About Pompe Disease

Pompe disease is an inherited lysosomal storage disorder caused by deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA leads to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. Pompe disease can be debilitating, and is characterized by severe muscle weakness that worsens over time. Pompe disease ranges from a rapidly fatal infantile form with significant impacts to heart function to a more slowly progressive, late-onset form primarily affecting skeletal muscle. It is estimated that Pompe disease affects approximately 5,000 to 10,000 people worldwide.

For more information, download our Pompe disease infographic.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a global biotechnology company at the forefront of therapies for rare and orphan diseases. The Company has a robust pipeline of advanced therapies for a broad range of human genetic diseases. Amicus' lead programs in development include the small molecule pharmacological chaperone migalastat as a monotherapy for Fabry disease, as well as novel enzyme replacement therapy (ERT) and biologic products for Fabry disease, Pompe disease, and other rare and devastating diseases.

Forward-Looking Statements

This press release contains "forward- looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to encouraging preliminary data from a global Phase 1/2 study to investigate ATB200/AT2221 for the treatment of Pompe and the potential implications on these data for the future advancement and development of ATB200/AT2221. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "confidence," "encouraged," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. The forward looking statements included in this press release are based on management's current expectations and belief's which are subject to a number of risks, uncertainties and factors, including that the preliminary data based on a small patient sample and reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented, that the Company will be not able to demonstrate the safety and efficacy of ATB200/AT2221, that later study results will not support further development, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize ATB200/AT2221. In addition, all forward looking statements are subject to the other risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2016 and Quarterly Report on 10-Q for the Quarter ended June 30, 2017. As a consequence, actual results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these forward looking statements, which speak only of the date hereof. All forward looking statements are qualified in their entirety by this cautionary statement and we undertake no obligation to revise this press release to reflect events or circumstances after the date hereof.

[1]Roberts, *et. al.,* **22nd International Congress of the World Muscle Society,** First-in-Human Study of ATB200/AT2221 in Patients With Pompe Disease: Interim Results From the ATB200-02 Trial

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First-in-Human Study of ATB200/AT2221 in Patients With Pompe Disease: Interim Results From the ATB200-02 Trial

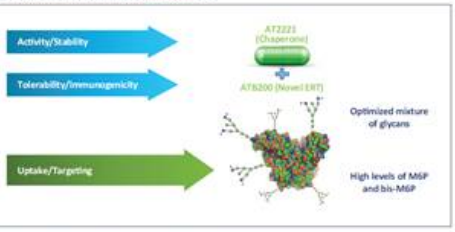
Roberts M[1], Sitaraman S[2], Barth JA[2], Sathe S[2], on behalf of the ATB200-02 Clinical Trial Investigators (Drago Bratkovic, Barry J. Byrne, Paula Clemens, Tarekegn Geberhiwot, Ozlem Goker-Alpan, Priya Kishnani, Xue Ming, Tahseen Mozaffar, Peter Schwenkreis, Kumaraswamy Sivakumar, Ans T. van der Ploeg, Benedikt Schoser)

[1]Salford Royal NHS Foundation Trust, Salford, UK; [2]Amicus Therapeutics, Inc., Cranbury, NJ, USA

INTRODUCTION

- Pompe disease is an inherited metabolic disease of impaired lysosomal glycogen clearance due to acid α-glucosidase (GAA) deficiency, which leads to accumulation of the substrate most prominently in the heart, skeletal muscle, and smooth muscle[1,2]
- Progressive accumulation of glycogen results in a spectrum of disease severity, often leading to organ failure and/or death. Skeletal muscle weakness and progressive respiratory involvement are predominant manifestations of late-onset Pompe disease (LOPD)[1,2]
- Enzyme replacement therapy (ERT) with recombinant human GAA (rhGAA) is the definitive therapy for LOPD[3]
- ATB200 is a next-generation rhGAA ERT designed with optimized glycosylation and high levels of mannose 6-phosphate residues for better uptake in target muscle tissues (**Figure 1**). The pharmacological chaperone AT2221 is co-administered with ATB200 to minimize denaturation of the enzyme in blood and maintain catalytic activity to deliver active ERT to lysosomes[3,4]
- Preclinical studies were conducted in *Gaa* knockout mice to evaluate the pharmacokinetics (PK) and efficiency of glycogen reduction at varying ATB200 ERT and AT2221 chaperone doses. These data were used to estimate the comparable AT2221 chaperone doses in humans[4]
- Study ATB200-02 (NCT02675465) was designed to evaluate the safety, tolerability, PK, pharmacodynamics (PD), and efficacy of ATB200 co-administered with AT2221

Figure 1. Representative Schematic of ATB200/AT2221



ERT=enzyme replacement therapy; M6P=mannose 6-phosphate.

OBJECTIVE

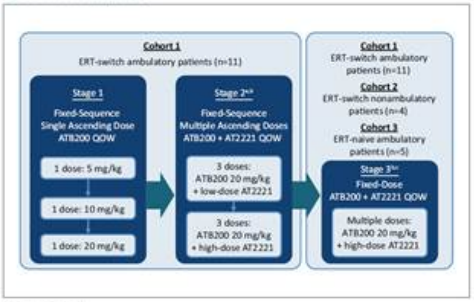
- To evaluate the preliminary safety, PK, PD, and efficacy of ATB200/AT2221 in patients with Pompe disease enrolled in the phase 1/2 ATB200-02 study

METHODS

Study Design

- ATB200-02 is an open-label, fixed-sequence, ascending-dose, first-in-human, phase 1/2 study to assess the safety, tolerability, PK, PD, and efficacy of intravenous infusions of ATB200 co-administered with oral AT2221 in adults with LOPD (**Figure 2**)

Figure 2. ATB200-02 Study Design



QOW=every other week.
[a]Safety data from 2 sentinel patients from Cohort 1 were reviewed at each dose level before dosing in Cohorts 2 and 3.
[b]During stages 2 and 3, AT2221 was orally administered prior to the start of ATB200 intravenous infusion. For all doses, ATB200 was intravenously infused for a 4-hour duration.
[c]The first 2 patients in Cohorts 2 and 3 served as sentinel patients for their respective cohorts.

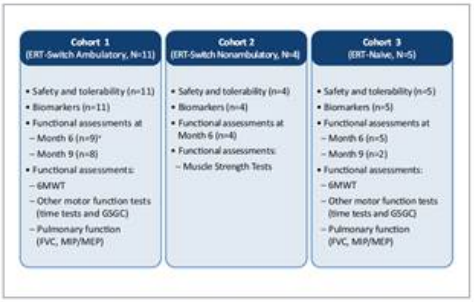
Key Inclusion Criteria

- Males and females aged 18–65 years who were diagnosed with Pompe disease based on documented deficiency of GAA enzyme activity or by GAA genotyping
- Received ERT with alglucosidase alfa for 2-6 years (or ≥2 years for Cohort 2) prior to trial initiation (Cohort 1)
- Currently receiving alglucosidase alfa at a frequency of every other week and having completed the last 2 infusions without a drug-related adverse event (AE) resulting in dose interruption (Cohorts 1 and 2)
- Must be able to walk between 200 and 500 meters on the 6-Minute Walk Test (6MWT) (Cohorts 1 and 3)
- Upright forced vital capacity (FVC) must be 30-80% of predicted normal value (Cohorts 1 and 3)
- Must be wheelchair-bound and unable to walk unassisted (Cohort 2)

Analyses

- Data are from interim analysis 4 (**Figure 3**)

Figure 3. Summary of Analyses and Available Data



6MWT=6-minute walk test; FVC=forced vital capacity; GSGC=Gait, Stair, Gower, Chair; MEP=maximal expiratory pressure; MIP=maximal inspiratory pressure.
*One patient discontinued after completing stage 1 (week 16) due to travel burden and family considerations, and 1 patient's month 6 data are pending.

RESULTS

- Sixteen clinical sites in 5 countries participated in the ATB200-02 clinical study
- Patients were representative of the overall LOPD population (**Table 1**)

Table 1. Baseline Characteristics (N=20)

	Cohort 1 ERT-Switch Ambulatory (n=11)	Cohort 2 ERT-Switch Nonambulatory (n=4)	Cohort 3 ERT-Naive (n=5)
Age, years, mean (range)	49.4 (28-66)	36.0 (18-56)	49.4 (24-65)
Sex, M:F	9:2	3:1	1:4
Time on alglucosidase alfa, years, mean (SD)	4.77 (1.42)*	8.9 (3.8)	NA
6MWT, meters, mean (SD)	392.0 (93.4)	NA	399.5 (83.5)
FVC upright, % predicted, mean (SD)	52.3 (13.2)	NA	53.4 (20.3)

NA=not applicable; SD=standard deviation.
*Cohort 1 patients were required to have been on alglucosidase alfa for 2-6 years at baseline.

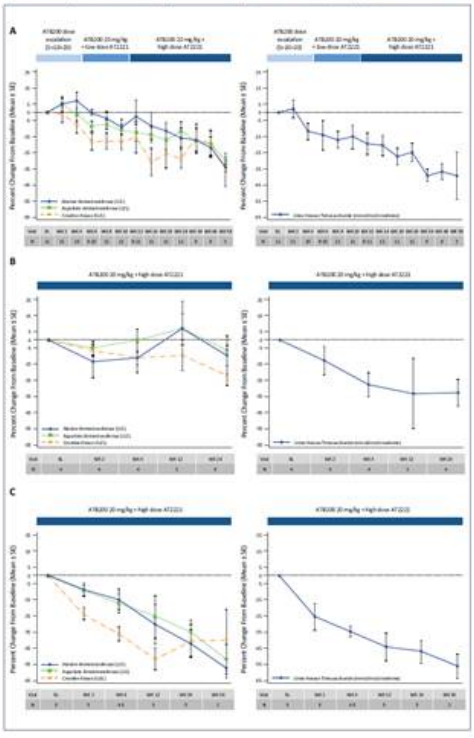
Safety

- AEs were generally mild and transient
  - Most common AEs reported as treatment related were nausea (3/20), tremor (3/20), headache (3/20), fatigue (3/20), diarrhea (2/20), muscle spasm (2/20), and joint swelling (2/20)
- One serious AE was reported, which was unrelated to the study drug (hospitalization for lower respiratory tract infection)
- No patients discontinued the study due to an AE
- There were 3 incidents of infusion-associated reactions (IARs) in 400+ infusions, which were controlled by standard premedication
  - One IAR event in a nonambulatory ERT-switch patient (skin discoloration)
  - Two IAR events in an ERT-naive patient (localized pruritus, erythema and burning sensation)
- Longest duration of treatment was 72 weeks

Markers of Muscle Injury

- Muscle damage markers (creatine kinase enzyme, alanine aminotransferase, and aspartate aminotransferase): Mean reductions from baseline were approximately 30-35%, 5-20%, and 40-55% for ambulatory ERT-switch (n=9), nonambulatory ERT-switch (n=4), and ERT-naive (n=5) patients, respectively (**Figure 4**)
- Glycogen accumulation marker (urine hexose tetrasaccharide [Hex4]): Mean reductions from baseline were approximately 39%, 32%, and 55% for ambulatory ERT-switch (n=9), nonambulatory ERT-switch (n=4), and ERT-naive (n=5) patients, respectively (**Figure 4**)

Figure 4. Mean Percentage Change From Baseline in Markers of Muscle Injury and Disease Substrate in (A) Ambulatory ERT-Switch Patients; (B) Nonambulatory ERT-Switch Patients; and (C) ERT-Naive Patients



Data are reported through an interim data analysis (maximum 58 weeks, 24 weeks, and 36 weeks for cohorts 1, 2, and 3, respectively). Missing values were either unable to be analyzed or not yet analyzed.
BL=baseline; SE=standard error; WK=week.

Efficacy

- 6MWT improved for both ambulatory ERT-switch and ERT-naive patients at month 6, with continued benefit to month 9 (**Table 2**)
  - 6MWT distance increased in 7/9 and 8/8 ERT-switch patients at months 6 and 9, respectively
    - Two patients were stable at month 6; 1 of them reached month 9 and had increased 6MWT
  - 6MWT distance increased in 5/5 and 2/2 ERT-naive patients at months 6 and 9, respectively

Table 2. 6-Minute Walk Test

	Baseline, Mean (SD)	Change From Baseline to Month 6, Mean (SD)	Change From Baseline to Month 9, Mean (SD)
Cohort 1 ERT-Switch	n=10	n=9	n=8
	397.2 (96.8)	+35.3 (40.1)	+37.2 (33.8)
Cohort 3 ERT-Naive	n=5	n=5	n=2
	399.5 (83.5)	+41.8 (29.4)	+74.9 (4.0)

Results are given in meters.

- Improvement in motor function tests, along with 6MWT, were consistent with an overall improvement in muscle function for both ambulatory ERT-switch and ERT-naive patients (**Table 3**)

Table 3. Other Motor Function Tests

	Assessment, sec	Baseline, Mean (SD)	Change From Baseline to Month 6, Mean (SD)	Change From Baseline to Month 9, Mean (SD)
Cohort 1 ERT-Switch		n=10	n=9	n=8
	Timed Up and Go	10.5 (6.6)	−2.2 (3.4)	−0.6 (2.5)
	4 Stair Climb	4.1 (2.7)	−1.0 (1.2)	−0.9 (1.3)
	10M Walk	7.4 (3.0)	−0.3 (1.6)	0.1 (1.3)
	Gowers[a]	7.9 (2.8)	−2.2 (2.0)	−2.1 (1.3)
	GSGC Score	12.6 (4.8)	−0.8 (3.0)	−0.9 (3.5)
Cohort 3 ERT-Naive		n=5	n=5	n=2
	Timed Up and Go	9.4 (2.9)	−1.0 (1.1)	−1.6 (1.0)
	4 Stair Climb	4.2 (1.5)	−0.6 (0.3)	−0.8 (0.3)
	10M Walk	7.9 (3.0)	−0.7 (1.1)	−1.0 (0.1)
	Gowers	13.9 (11.0)	7.9[b] (21)	−1.3 (0.0)
	GSGC Score	12.2 (3.6)	−1.8 (3.8)	−4.0 (1.4)

*N=9; one patient refused to perform test.
[b]Median change from baseline was −0.8, and 4/5 patients had a decrease.

- Consistent and substantial increases were observed in upper extremity strength in all nonambulatory ERT-switch patients at month 6 (**Table 4**)

Table 4. Muscle Strength Testing in Nonambulatory ERT-Switch Patients (cohort 2)

Assessment	Muscle Group Tested	Baseline, Mean (SD)		Change From Baseline to Month 6, Mean (SD)	
		Right	Left	Right	Left
Quantitative Muscle Testing—Dynamometer, pounds force	Shoulder Adduction[a]	1.5 (1.9)	4.2 (6.8)	+5.8 (8.4)	+2.3 (4.4)
	Shoulder Abduction	6.9 (7.6)	9.8 (10.9)	+0.8 (1.5)	+0.3 (5.1)
	Elbow Flex	4.9 (5.1)	7.8 (8.7)	+2.4 (6.1)	−0.1 (10.0)
	Elbow Extension	5.0 (5.9)	7.3 (8.1)	+4.1 (2.1)	+1.5 (3.4)
Manual Muscle Testing, manual score[b]	Shoulder Adduction[a]	1.0 (1.0)	1.3 (1.2)	+0.7 (1.2)	+0.7 (1.2)
	Shoulder Abduction[a]	1.3 (1.2)	1.3 (1.2)	0.0 (0.0)	+0.5 (0.7)
	Elbow Flex	2.0 (2.0)	2.3 (2.5)	+1.0 (1.0)	+0.7 (0.6)
	Elbow Extension	2.0 (2.0)	2.0 (2.0)	+1.0 (1.0)	+0.7 (0.6)

[a]n=3 due to assessment not being performed at some visits for some patients.
[b]Scoring: (1) Visible muscle movement, but no movement at the joint; (2) Movement at the joint, but not against gravity; (3) Movement against gravity, but not against added resistance; (4) Movement against resistance, but less than normal; (5) Normal strength.
[a,b]n=2 due to assessment not being performed at some visits for some patients.

- FVC was generally stable in ambulatory ERT-switch patients and increased in ERT-naive patients (**Table 5**)
  - FVC was stable or increased in 5/8 and 5/7 ERT-switch patients at months 6 and 9, respectively
  - FVC increased in 4/5 and 2/2 ERT-naive patients at months 6 and 9, respectively
- Maximal inspiratory pressure (MIP) was stable and maximal expiratory pressure (MEP) increased in ambulatory ERT-switch patients; MIP increased and MEP was stable in ERT-naive patients (**Table 5**)

Table 5. Forced Vital Capacity and Other Pulmonary Function Tests

	Assessment	Baseline, Mean (SD)	Change From Baseline to Month 6, Mean (SD)	Change From Baseline to Month 9, Mean (SD)
Cohort 1 ERT-Switch		n=10	n=9	n=8
	FVC, % predicted*	52.6 (14.7)	−1.0 (4.2)	−2.0 (3.6)
	MIP	35.7 (11.0)	+0.9 (4.5)	−1.4 (2.7)
	MEP	72.6 (32.6)	+20.3 (42.4)	+31.1 (39.3)
Cohort 3 ERT-naive		n=5	n=5	n=2
	FVC, % predicted	53.4 (20.3)	+4.2 (5.6)	+5.0 (1.4)
	MIP	32.6 (18.5)	+11.0 (5.0)	+1.5 (0.7)
	MEP	60.6 (8.3)	−0.4 (12.4)	−1.0 (19.8)

*FVC not available for 1 patient.

SUMMARY AND CONCLUSIONS

- There is concordance in the data showing a parallel improvement in markers of muscle injury and substrate accumulation, motor function (endurance, timed tests, and muscle strength), and stabilization and/or improvement in respiratory function tests across the different cohorts
- Muscle function improved in 16/18 and 10/10 patients at months 6 and 9, respectively
- Increases in 6MWT distance were consistent and durable out to month 9 as were the improvements in other motor function tests in ambulatory ERT-switch and ERT-naive patients
- Qualitative and quantitative measures showed increases in upper extremity strength in nonambulatory ERT-switch patients at month 6
- FVC was generally stable in ambulatory ERT-switch patients and increased in ERT-naive patients
- The multi-dimensional impact of the therapy is suggestive that the combination regimen of ATB200/AT2221 has the potential to be an important treatment option for patients with Pompe disease. Further study of this regimen is ongoing

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1. Kishnani PS et al. *Genet Med.* 2006;8(5):267-288.
2. Bijvoet AGA et al. *Hum Mol Gen.* 1998;7(1):53-62.
3. Gotschall R et al. *Mol Genet Metab.* 2015;114(2):549.
4. Khanna R et al. Presented at the 12th Annual WORLDSymposium™; February 29-March 4, 2016; San Diego, CA, USA.
5. Data on file. Amicus Therapeutics, Inc.

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DISCLOSURES

Conflicts of Interest

M. Roberts has no conflicts of interest to disclose. S. Sitaraman, J.A. Barth, and S. Sathe are employees of and own stock in Amicus Therapeutics.

For questions, please contact Mark Roberts at marksrob@doctors.org.uk.

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Positive Pompe Phase 1/2 Data at World Muscle Society

Conference Call & Webcast

October 4, 2017

Safe Harbor

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to encouraging preliminary data from a global Phase 1/2 study to investigate ATB200/AT2221 for the treatment of Pompe and the potential implications on these data for the future advancement and development of ATB200/AT2221. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "confidence," "encouraged," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. The forward looking statements included in this presentation are based on management's current expectations and beliefs which are subject to a number of risks, uncertainties and factors, including that the preliminary data based on a small patient sample and reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented, that the Company will not be able to demonstrate the safety and efficacy of ATB200/AT2221, that later study results will not support further development, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize ATB200/AT2221. In addition, all forward looking statements are subject to the other risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2016 and Quarterly Report on 10-Q for the Quarter ended June 30, 2017. As a consequence, actual results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these forward looking statements, which speak only of the date hereof. All forward looking statements are qualified in their entirety by this cautionary statement and we undertake no obligation to revise this press release to reflect events or circumstances after the date hereof.

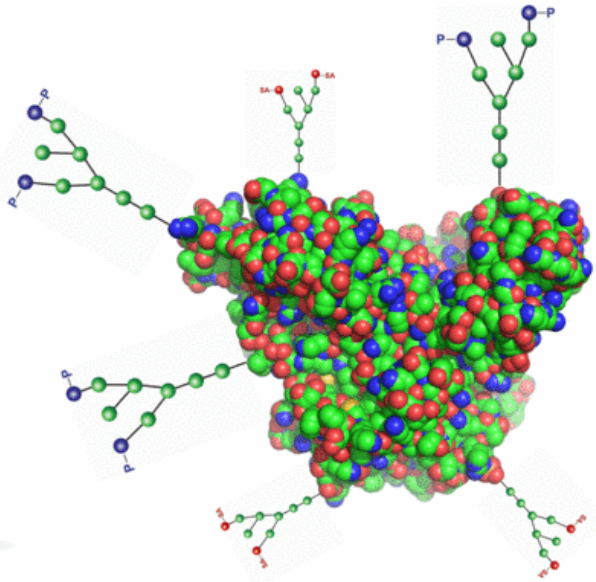
ATB200 + Chaperone: A Highly Differentiated Approach

Novel Pompe Treatment Paradigm with Three Key Differentiators

ATB200 (Novel ERT)

CHART
CHAPERONE-ADVANCED REPLACEMENT THERAPY

Chaperone addition



Optimized mixture of glycans

High levels of M6P and bis M6P

*Artist rendering, not actual product image

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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)

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Baseline Characteristics of Patients in Phase 1/2 ATB200-02 Study (n=20)

Patients Across Three Cohorts Representative of Overall Late-Onset Pompe Population with Impairment at Baseline

Baseline Characteristics (N=20)	Cohort 1: Ambulatory ERT-Switch* (N=11)	Cohort 2: Non-Ambulatory ERT-Switch (N=4)	Cohort 3: ERT-Naïve (N=5)
Time on Standard of Care – mean years (SD)	4.77 (1.4)*	8.9 (3.8)	N/A
Age – mean years (range)	49.4 (28, 66)	36.0 (18, 56)	49.4 (24, 65)
Sex M:F	9:2	3:1	1:4
6MWT – mean meters (SD)	392.0 (93.4)	N/A	399.5 (83.5)
FVC Upright – mean % predicted (SD)	52.3 (13.2)	N/A	53.4 (20.3)

*Cohort 1 patients required to have been on ERT Standard of Care for 2-6 years at baseline

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Summary of Available Data

	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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Safety Summary (n=20)*

AEs Generally Mild and Transient with Very Low (<1%) Rate of Infusion-Associated Reactions After 400+ Total Infusions of ATB200/AT2221 Across All Cohorts

- AEs were generally mild and transient
- Very low number (<1%) of infusion-associated reactions (IARs) after 400+ total infusions
  - One IAR event in one ERT-switch patient (skin discoloration)
  - Two IAR events in one ERT-naïve patient (localized pruritus, erythema and burning sensation)
- Longest duration of treatment is 72 weeks

*Reported through interim data analysis (maximum 72 weeks)

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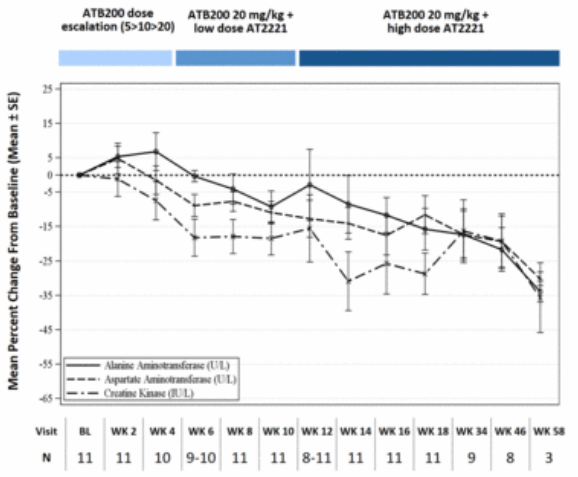
Cohort 1 (ERT-Switch): Biomarkers up to Week 58 (N=11)*

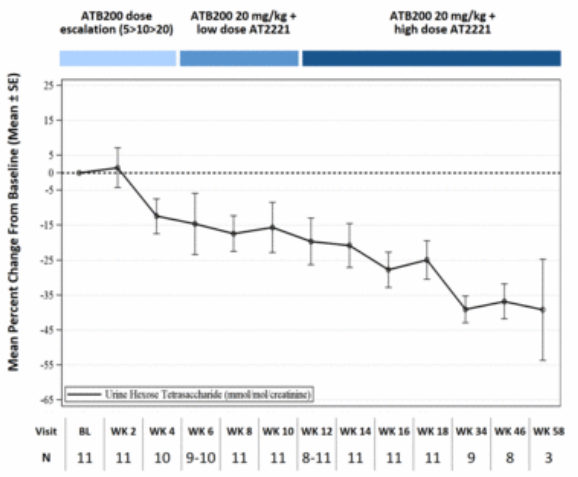
Persistent and Durable Improvement in Biomarkers of Muscle Damage (CK, ALT, AST) and Disease Substrate (Hex4) for up to 58 Weeks on ATB200/AT2221

Muscle Damage Biomarkers (% Change from Baseline for CK, ALT, and AST)



Visit	BL	WK 2	WK 4	WK 6	WK 8	WK 10	WK 12	WK 14	WK 16	WK 18	WK 34	WK 46	WK 58
N	11	11	10	9-10	11	11	8-11	11	11	11	9	8	3

Disease Substrate Biomarker (% Change from Baseline for Hex 4)



Visit	BL	WK 2	WK 4	WK 6	WK 8	WK 10	WK 12	WK 14	WK 16	WK 18	WK 34	WK 46	WK 58
N	11	11	10	9-10	11	11	8-11	11	11	11	9	8	3

*Reported through interim data analysis (maximum 58 weeks); Missing values either unable to be analyzed or not yet analyzed

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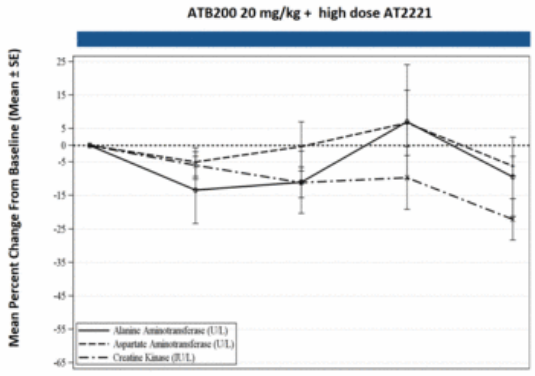
Cohort 2 (Non-Ambulatory ERT-Switch): Biomarkers up to Week 24 (N=4)*

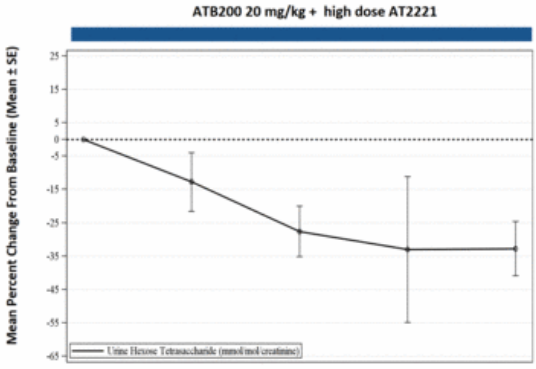
Improvement in Biomarkers of Muscle Damage (CK) and Disease Substrate for up to 24 Weeks on ATB200/AT2221

Muscle Damage Biomarkers (% Change from Baseline for CK, ALT, and AST)



Visit	BL	WK 2	WK 4	WK 12	WK 24
N	4	4	4	3	4

Disease Substrate Biomarker (% Change from Baseline for Hex 4)



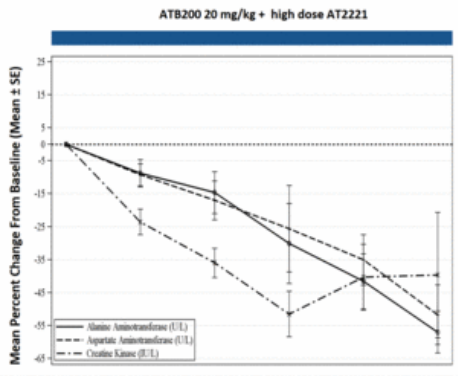
Visit	BL	WK 2	WK 4	WK 12	WK 24
N	4	4	4	3	4

*Reported through interim data analysis (maximum 24 weeks); Missing values either unable to be analyzed or not yet analyzed

Cohort 3 (ERT-Naïve): Biomarkers up to Week 36 (N=6)*

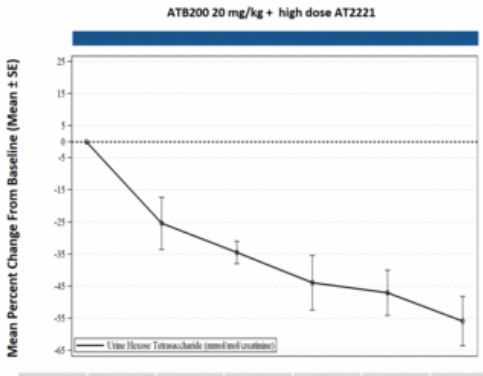
Improvement in Biomarkers of Muscle Damage and Disease Substrate for up to 36 Weeks on ATB200/AT2221

Muscle Damage Biomarkers (% Change from Baseline for CK, ALT, and AST)



Visit	BL	WK 2	WK 4	WK 12	WK 24	WK 36
N	5	5	4-5	5	5	2

Disease Substrate Biomarker (% Change from Baseline for Hex 4)



Visit	BL	WK 2	WK 4	WK 12	WK 24	WK 36
N	5	5	4-5	5	5	2

*Reported through interim data analysis (maximum 36 weeks); Missing values either unable to be analyzed or not yet analyzed

Cohort 1 and 3: 6-Minute Walk Test (6MWT) (n=14)

Mean 6MWT Distance Improved for Both ERT-Naïve Patients (+41.8 Meters) and ERT-Switch Patients (+35.3 Meters) at Month 6 with Continued Benefit Out to Month 9

6-Minute Walk Test (m): Month 6 and 9

Cohort	Baseline (n=10) Mean (SD)	Change at Month 6 (n=9) Mean (SD)	Change at Month 9 (n=8) Mean (SD)
Cohort 1 ERT-Switch	397.2 (96.8)	+35.3 (40.1)	+37.2 (33.8)
Cohort	**Baseline (n=5) Mean (SD)**	**Change at Month 6 (n=5) Mean (SD)**	**Change at Month 9 (n=2) Mean (SD)**
Cohort 3 ERT-Naïve	399.5 (83.5)	+41.8 (29.4)	+74.9 (4.0)

6MWT distance increased in 7/9 and 8/8 ERT-switch patients at Month 6 and 9, respectively

- Two patients stable at Month 6, one of these patients reached Month 9 and had increased walking distance

6MWT Increased in 5/5 and 2/2 ERT-Naïve Patients at Month 6 and Month 9, Respectively

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Cohort 1 and 3: Other Motor Function Tests (n=14)

Improvement in Nearly All Motor Function Tests with 6MWT Consistent with Overall Improvement in Motor Performance for Both ERT-Switch and ERT-Naïve Patients at Month 6 and 9

Other Motor Function Tests: Month 6 and 9

Cohort	Assessment (sec)	Baseline (n=10) Mean (SD)	Change at Month 6 (n=9) Mean (SD)	Change at Month 9 (n=8) Mean (SD)
Cohort 1: ERT-Switch	Timed up and Go	10.5 (6.6)	-2.2 (3.4)	-0.6 (2.5)
	4 Stair Climb	4.1 (2.7)	-1.0 (1.2)	-0.9 (1.3)
	10M walk	7.4 (3.0)	-0.3 (1.6)	0.1 (1.3)
	Gowers#	7.9 (2.8)	-2.2 (2.0)	-2.1 (1.3)
	GSGC Score	12.6 (4.8)	-0.8 (3.0)	-0.9 (3.5)
Cohort	**Assessment (sec)**	**Baseline (n=5) Mean (SD)**	**Change at Month 6 (n=5) Mean (SD)**	**Change at Month 9 (n=2) Mean (SD)**
Cohort 3: ERT-Naïve	Timed up and Go	9.4 (2.9)	-1.0 (1.1)	-1.6 (1.0)
	4 Stair Climb	4.2 (1.5)	-0.6 (0.3)	-0.8 (0.3)
	10M walk	7.9 (3.0)	-0.7 (1.1)	-1.0 (0.1)
	Gowers	13.9 (11.0)	7.9* (21.0)	-1.3 (0.0)
	GSGC Score	12.2 (3.6)	-1.8 (3.8)	-4.0 (1.4)

Notes: * Median change from baseline was -0.8 and 4/5 had decrease; # N=9 Missing values not obtained due to patient refusal to perform test

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Cohort 2: Muscle Strength Testing at Month 6 (n=4)

Substantial and Consistent Improvement in Upper Extremity Strength in Non-Ambulatory ERT-Switch Patients in Nearly All Quantitative and Qualitative Measures at Month 6

Assessment	Muscle Group Tested	Baseline		Change to Month 6	
		Left Mean (SD)	Right Mean (SD)	Left Mean (SD)	Right Mean (SD)
QMT- Quantitative Muscle Testing - Dynamometer (pounds force)	Shoulder Adduction*	4.2 (6.8)	1.5 (1.9)	+2.3 (4.4)	+5.8 (8.4)
	Shoulder Abduction	9.8 (10.9)	6.9 (7.6)	+0.3 (5.1)	+0.8 (1.5)
	Elbow Flex	7.8 (8.7)	4.9 (5.1)	-0.1 (10.0)	+2.4 (6.1)
	Elbow Extension	7.3 (8.1)	5.0 (5.9)	+1.5 (3.4)	+4.1 (2.1)
MMT - Manual Muscle Testing (manual score)	Shoulder Adduction*	1.3 (1.2)	1.0 (1.0)	+0.7 (1.2)	+0.7 (1.2)
	Shoulder Abduction**	1.3 (1.2)	1.3 (1.2)	+0.5 (0.7)	0.0 (0.0)
	Elbow Flex	2.3 (2.5)	2.0 (2.0)	+0.7 (0.6)	+1.0 (1.0)
	Elbow Extension	2.0 (2.0)	2.0 (2.0)	+0.7 (0.6)	+1.0 (1.0)

Note: MMT Scoring: 1) Visible muscle movement, but no movement at the joint, 2) Movement at the joint, but not against gravity, 3) Movement against gravity, but not against added resistance, 4) Movement against resistance, but less than normal, 5) Normal strength
*N=3 or **N=2 due to assessment not being performed at some visits for some patients

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Forced Vital Capacity (FVC) Summary (n=13)*

FVC Increased in ERT-Naïve Patients and was Generally Stable in ERT-Switch Patients

FVC (% Predicted): Month 6 and 9

Cohort	Baseline (n=9) Mean (SD)	Change at Month 6 (n=8) Mean (SD)	Change at Month 9 (n=7) Mean (SD)
Cohort 1 ERT-Switch Ambulatory*	52.6 (14.7)	-1.0 (4.2)	-2.0 (3.6)
Cohort	**Baseline (n=5) Mean (SD)**	**Change at Month 6 (n=5) Mean (SD)**	**Change at Month 9 (n=2) Mean (SD)**
Cohort 3 ERT-Naïve	53.4 (20.3)	+4.2 (5.6)	+5.0 (1.4)

FVC stable or increased in 5/8 and 5/7 ERT-switch patients at Month 6 and Month 9 respectively

FVC increased in 4/5 and 2/2 ERT-naïve patients at Month 6 and Month 9 respectively

*FVC results not available for 1 subject at Month 6 and 9

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Other Pulmonary Function Tests at Month 6 (n=14)

MIP and MEP Generally Stable or Increased in Both ERT-Naïve and ERT-Switch Patients

Other Pulmonary Function Tests: MIP and MEP

Patients	Assessment	Baseline (n=10) Mean (SD)	Change at Month 6 (n=9) Mean (SD)	Change at Month 9 (n=8) Mean (SD)
Cohort 1: ERT-Switch	MIP	35.7 (11.0)	+0.9 (4.5)	-1.4 (2.7)
	MEP	72.6 (32.6)	+20.3 (42.4)	+31.1 (39.3)
Patients	**Assessment**	**Baseline (n=5) Mean (SD)**	**Change at Month 6 (n=5) Mean (SD)**	**Change at Month 9 (n=2) Mean (SD)**
Cohort 3: ERT-Naive	MIP	32.6 (18.5)	+11.0 (5.0)	+1.5 (0.7)
	MEP	60.6 (8.3)	-0.4 (12.4)	-1.0 (19.8)

Study ATB200-02 Data Summary

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

Muscle Function (All Cohorts)

- Muscle function improved in 16/18 and 10/10 patients at Month 6 and 9, respectively
- 6MWT distance increased to Month 9
  - ERT-naïve: mean increases of +42m (Month 6) and +75m (Month 9)
  - ERT-switch: mean increases of +35m (Month 6), +37m (Month 9)
- Improvement in other motor function tests consistent with 6MWT for both ERT-naïve and ERT-switch patients
- 4/4 non-ambulatory ERT-switch patients showed significant increase in muscle strength tests at Month 6

Pulmonary Function (Cohorts 1 and 3)

- FVC generally stable in ERT-switch patients
- FVC increased in a majority of ERT-naïve patients
- MIP and MEP generally stable or improved in both ERT-switch and ERT-naïve patients

Pompe Phase 1/2 Study ATB200-02 Data Cascade

Continuing Collaborative Discussions with Regulators in the U.S. and EU
Update Anticipated in the First Half of 2018

Pompe Milestones in 2017

- **Preliminary 18-Week Data at WORLD*Symposium***
- **Additional 18-Week & Initial Extension Data**
- **18-Week & Extension Data Presentation at World Muscle Society**
- **Discussions with U.S. and EU regulators**

18-WEEK DATA

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

EXTENSION DATA

- Motor/pulmonary function

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Thank you

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Appendix

Pompe Disease Overview

Pompe Disease is Heterogeneous Across a Broad Spectrum of Patients

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

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