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 5, 2018**

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 5, 2018, Amicus Therapeutics, Inc. issued a press release announcing that additional positive data in its Pompe disease phase 1/2 study (the "Study") will be presented at the 23rd International Annual Congress of the World Muscle Society. A copy of this press release is attached hereto as Exhibit 99.1 and a slide deck with the latest clinical results from the Study is attached hereto as Exhibit 99.2.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits:

Exhibit No.	Description
99.1	Press release dated October 5, 2018 titled "Amicus Therapeutics Announces Positive 18-Month Data in Pompe Disease Phase 1/2 Study

	at 23rd International Annual Congress of the World Muscle Society”.
99.2	October 5, 2018 Slide Deck

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.

Date: October 5, 2018

AMICUS THERAPEUTICS, INC.
By: /s/ Ellen S. Rosenberg
Name: Ellen S. Rosenberg
Title: General Counsel and Corporate Secretary

Amicus Therapeutics Announces Positive 18-Month Data in Pompe Disease Phase 1/2 Study at 23rd International Annual Congress of the World Muscle Society

Consistent and Durable Responses Across Key Measures of Safety, Functional Outcomes and Biomarkers Continue Out to Month 18 for both ERT-Naïve and ERT-Switch Patients

Very Low Rate (<1%) of Infusion Associated Reactions Maintained After ~900 Infusions

CRANBURY, NJ, and Mendoza, ARGENTINA, October 5, 2018 — Amicus Therapeutics (Nasdaq: FOLD) today announced additional positive results from a global Phase 1/2 clinical study (ATB200-02) to investigate AT-GAA 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. Patients treated with AT-GAA for up to 18 months showed improvements in six-minute walk test (6MWT) distance and other measures of motor function and muscle strength, stability or increases in forced vital capacity (FVC), and durable reductions in biomarkers of muscle damage and disease substrate. These clinical results are being featured at the 23rd International Annual Congress of the World Muscle Society in an oral platform presentation today, Friday October 5, 2018 at 12:20am ART (11:20am EDT). The presentation will be given by Professor Benedikt Schoser, senior consultant at the Friedrich-Baur-Institute, Dept. of Neurology at the Ludwig-Maximilians-University of Munich, Germany and Principal Investigator in the ATB200-02 study.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics stated, “The clinical data for our investigational therapy for Pompe disease AT-GAA are very compelling and consistent across patients and in multiple endpoints now for up to 18 months on treatment. These latest data continue to show meaningful and very durable improvements in functional outcomes in nearly all patients, in addition to persistent and durable reductions in key biomarkers of muscle damage and disease substrate. These new results further support our strategy to significantly enhance the body of clinical data for AT-GAA through our ongoing clinical and natural history studies, as well as our upcoming pivotal study, as we seek to deliver this potential new therapy to as many people living with Pompe disease as soon as possible.”

Professor Schoser stated, “The 18-month results from this Phase 1/2 clinical study of AT-GAA continue to demonstrate a robust effect in adult people living with Pompe disease, including those who had switched from ERT and those who had not previously been treated. I am glad to see the meaningful improvements in muscle strength in most of the patients. Since entering the study, the ambulatory ERT-experienced and ERT-naïve cohorts have walked farther in the 6-minute walking test, and the non-ambulatory ERT-experienced cohort have demonstrated sustained positive changes in their arm and shoulder movements. Overall, the safety and functional data suggest that AT-GAA has the potential to improve treatment for people living with Pompe disease.”

ATB200-02 Study Data Highlights in ERT-Switch and ERT-Naive Patients Out to Month 18

The slide deck with the latest clinical results from the ATB200-02 clinical study presented at World Muscle Society is available at www.amicusrx.com. These results, including additional detailed results at month 18, will be highlighted by Mark Roberts, MD, Department of Neurology, Salford Royal NHS Foundation Trust and Principal Investigator in the ATB200-02 study, at the upcoming Amicus Analyst Day on October 11, 2018.

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

Safety and tolerability data in all 20 patients reflect a maximum of 28+ months of treatment. To date, adverse events have been generally mild and transient. Importantly, AT-GAA has resulted in a low rate of infusion-associated reactions (IARs)

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following 890+ infusions (7 events of IARs in 5 patients; <1% of all 890+ infusions with an IAR). The clinical pharmacokinetic profile has been consistent with previously reported preclinical data.

Functional Outcomes (n=19)

Data on functional outcomes are available for 19 of the 20 patients enrolled (one patient dropped out of the extension study due to travel burden and family considerations). Muscle function improved in 17 of 19 patients at month 12. Muscle function improved in 17 out of 18 patients with available data at month 18.

- **Motor function (n=15):** 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 with continued benefit observed out to month 18. Improvements were generally consistent across both cohorts.
  - All 5 ERT-naive patients showed increases in 6MWT distance at all time points out to month 18. The ERT-naïve patients showed mean increases of 42 meters at month 6 (n=5), 63 meters at month 12 (n=5), and 49 meters at month 18 (n=5).
  - 6MWT increased in 7/10, 9/10, and 9/9 ERT-switch patients at Months 6, 12, and 18, respectively. The ERT-switch patients showed mean increases of 24 meters at month 6 (n=10), 42 meters at month 12 (n=10), and 52 meters at month 18 (n=9).
  - Other motor function tests generally showed mean improvements consistent with 6MWT distance out to month 18 in both ambulatory cohorts.

- **Muscle Strength (n=4):** three of the four non-ambulatory ERT-switch patients showed improvements in upper extremity strength (which includes elbow and shoulder) from baseline to month 18, as measured by quantitative muscle testing (QMT) and manual muscle testing (MMT). Further details are provided in the slide deck.

- **Pulmonary Function (n=14):** Pulmonary function improved in ERT-naïve patients and was generally stable in ERT-switch patients. In ERT-naïve patients, mean absolute change in forced vital capacity (FVC), one of the main measures of pulmonary function in Pompe disease, was +4.2% at month 6 (n=5), +4.4% at month 12 (n=5), and +5.0% at month 18 (n=5). In ERT-switch patients mean absolute change in FVC was -1.3% at month

6 (n=9), -3.3% at month 12 (n=9), and -3.7% at month 18 (n=8). Overall, other pulmonary tests of maximal inspiratory pressure (MIP), a measure of inhalation, and maximal expiratory pressure (MEP), a measure of exhalation, were stable or increased in both ERT-naïve and ERT-switch patients.

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

Treatment with AT-GAA resulted in persistent and durable reductions in key biomarkers of muscle damage (creatine kinase, or CK) and disease substrate (urine hexose tetrasaccharide, or Hex4) across all patient cohorts out to month 18 and continue to suggest a positive effect on muscle tissue. Further details are provided in the slide deck.

AT-GAA Development and Regulatory Strategy

As previously announced, Amicus is building a robust data set for AT-GAA across several studies including the ongoing ATB200-02 clinical study, a retrospective natural history study, and the upcoming pivotal study.

Anticipated milestones:

- Initiation of pivotal study to support full approval in U.S. and EU, as well as other geographies (2H18)
- Complete a retrospective natural history study in approximately 100 ERT-treated Pompe patients (2H18)
- Additional ATB200-02 study data from up to 10 additional ERT-switch patients in a new Cohort 4 (2019)
- Initiation of studies in additional patient populations, including pediatric patients (2019)

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 originally enrolled a total of 20 patients across three patient cohorts: ambulatory ERT-experienced (Cohort 1, n=11), non-ambulatory ERT-experienced (Cohort 2, n=4) and ERT-naïve (Cohort 3, n=5). A fourth cohort of ambulatory ERT-switch patients is also currently enrolling to double the number of patient data in this ambulatory ERT-switch population. 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 Cohorts 2, 3, and 4 have all received 20 mg/kg ATB200 plus high dose AT2221.

About AT-GAA

AT-GAA is an investigational therapy 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, AT-GAA was associated with

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increased tissue enzyme levels, reduced glycogen levels in muscle, and improvements in muscle strength. Amicus Therapeutics is currently conducting a global Phase 1/2 study (ATB200-02) to evaluate the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics of AT-GAA.

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.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-centric biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases. For more information please visit the company's website at www.amicusrx.com.

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 AT-GAA for the treatment of Pompe and the potential implications on these data for the future advancement and development of AT-GAA. 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 AT-GAA, 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 AT-GAA. 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, 2017 and Quarterly Report on 10-Q for the Quarter ended June 30, 2018. 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) Shoser, *et. al.*, **23rd Annual Congress of the World Muscle Society, First-in-human Study of ATB200/AT2221 in Patients With Pompe Disease: 18M Safety and Efficacy Data From the ATB200-02 Trial**

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Results From ATB200-02: First-in-Human Study of ATB200 Co-Administered With AT2221 for Pompe Disease (18-Month Results)

Benedikt Schoser,[1] Drago Bratkovic,[2] Barry J. Byrne,[3] Paula Clemens,[4] Tarekegn Geberhiwot,[5] Ozlem Goker-Alpan,[6] Priya Kishnani,[7] Xue Ming,[8] Tahseen Mozaffar,[9] Peter Schwenkreis,[10] Kumaraswamy Sivakumar,[11] Ans T. van der Ploeg,[12] Jacquelyn Wright,[13] Swati Sathe,[13] Sheela Sitaraman,[13] Hjalmar Lagast,[13] Jay A. Barth,[13] Mark Roberts[14]

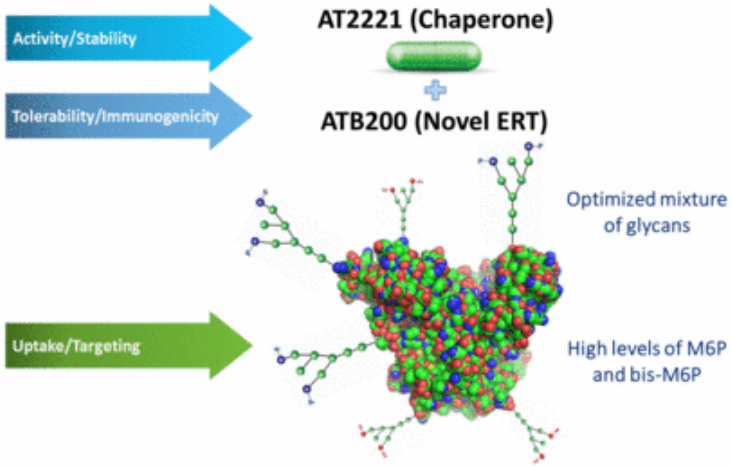
[1]Klinikum der Universität München-Neurologische Klinik, Friedrich-Baur-Institut, Munich, Germany; [2]PARC Research Clinic, Royal Adelaide Hospital, Adelaide, SA, Australia; [3]University of Florida, Gainesville, FL, USA; [4]University of Pittsburgh and Department of Veterans Affairs Medical Center, Pittsburgh, PA, USA; [5]University Hospital Birmingham NHS Foundation Trust, Queen Elizabeth Medical Center, Birmingham, UK; [6]O&O Alpan LLC, Fairfax, VA, USA; [7]Duke University Medical Center, Durham, NC, USA; [8]Rutgers New Jersey Medical School, Newark, NJ, USA; [9]University of California, Irvine, Orange, CA, USA; [10]Neurologische Klinik und Poliklinik des Berufsgenossenschaftlichen, Universitätklinikum Bergmannsheil, Bochum, Germany; [11]Neuromuscular Research Center, Phoenix, AZ, USA; [12]Erasmus MC University Medical Center, Rotterdam, The Netherlands; [13]Amicus Therapeutics, Inc., Cranbury, NJ, USA; [14]Salford Royal NHS Foundation Trust, Salford, UK;

Benedikt Schoser Disclosure Information

- I have the following financial relationships to disclose:
  - Consultant for Amicus Therapeutics, Inc.
  - Consultant and member of speaker bureau for Audentes, Genzyme, Intiva, Lupin, Valerion and Vertex.
- I will discuss the following off-label use and/or investigational use in my presentation:
  - Data from a phase 1/2 trial of ATB200/AT2221 for the treatment of patients with Pompe disease
  - ATB200/AT2221 is an investigational therapy that has not been approved for commercial use

AT-GAA (Acid α-Glucosidase) (ATB200/AT2221)

- AT2221: orally administered investigational chaperone given prior to infusion of ATB200
  - Shown to stabilize ERT in blood and maintain catalytic activity to enhance delivery of active enzyme to lysosomes[1,2]
- ATB200: investigational next-generation ERT
  - Designed with optimized glycosylation and high levels of mannose 6-phosphate residues for better uptake to target tissues



ERT=enzyme replacement therapy; M6P=mannose-6-phosphate.
1. Gotschall R et al. *Mol Genet Metab*. 2015;114(2):S49. Abstract 94. 2. Khanna R et al. Presented at: the 12th Annual WORLD*Symposium*™; February 29-March 4, 2016; San Diego, CA, USA.

ATB200-02 Study Design (NCT02675465)

Phase 1/2 Clinical Study to Evaluate Safety, Tolerability, PK, and PD of AT-GAA (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
5 mg/kg (wk 2)
10 mg/kg (wk 4)
20 mg/kg (wk 6)
→ **ATB200 20 mg/kg + AT2221 (Sub-Optimal Dose)** wks 8,10,12
→ **ATB200 20 mg/kg + AT2221 (Optimal Dose)** wk 14+

Cohort 2 (Non-ambulatory ERT-Switch, n=4) & Cohort 3 (ERT-Naive, n=5)

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

Assessments:

- Safety/Tolerability
- Plasma PK
- Infusion-Associated Reactions
- Antibody & Cytokine Levels
- Pharmacodynamics
- Efficacy (long-term extension)

ERT=enzyme replacement therapy; PD=pharmacodynamics; PK=pharmacokinetics, wk=week.

Baseline Characteristics

Patients (N=20) enrolled across the 3 cohorts were representative of the overall LOPD population, with significant impairment at baseline

	Cohort 1 ERT-Switch Ambulatory n=11[a]	Cohort 2 ERT-Switch Nonambulatory n=4	Cohort 3 ERT-Naive n=5
Age, mean years (min, max)	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, mean years (SD)	4.8 (1.4)[b]	8.9 (3.8)	NA
6MWT, mean meters (SD)	392.0 (93.4)	NA	399.5 (83.5)
Upright FVC, mean % predicted (SD)	52.3 (13.2)	NA	53.4 (20.3)

6MWT=6-minute walk test; ERT=enzyme replacement therapy; FVC=forced vital capacity; LOPD=late-onset Pompe disease; NA=not applicable; SD=standard deviation.
[a]One patient in Cohort 1 discontinued after 18 weeks due to burden of travel; [b]Cohort 1 patients were required to have been on alglucosidase alfa for 2-6 years at baseline.

6-Minute Walk Test

6MWT improved for both ERT-switch ambulatory and ERT-naive patients at Month 6 with continued benefit observed out to Month 18

All results are mean (SD), meter	Baseline	Change From Baseline		
		Month 6	Month 12	Month 18
Cohort 1 ERT-Switch Ambulatory	n=10	n=10	n=10	n=9[a]
	397.2 (96.8)	**+23.9** (52.2)	**+42.2** (46.5)	**+51.7** (45.9)
Cohort 3 ERT-Naive	n=5	n=5	n=5	n=5
	399.5 (83.5)	**+41.8** (29.4)	**+63.1** (29.1)	**+49.0** (28.3)

- 6MWT increased in 7/10, 9/10, and 9/9 ERT-switch patients at Months 6, 12, and 18, respectively
- 6MWT increased in 5/5, 5/5, and 5/5 ERT-naive patients at Months 6, 12, and 18, respectively
- Timed motor function tests were consistent with 6MWT (not shown)

6MWT=6-minute walk test; ERT=enzyme replacement therapy; SD=standard deviation.
[a]Data for one patient is pending (visit had not occurred at time of interim data cut).

Manual Muscle Test Score

Increases were observed in manual muscle strength[a] in all patients at Months 6, 12, and 18

	Body Area	Baseline		Change From Baseline					
				Month 6		Month 12		Month 18	
		mean (SD)	n	mean (SD)	n	mean (SD)	n	mean (SD)	n
ERT-switch Ambulatory	Total Body Max score 80	**66.4** (8.1)	10	**+2.5** (3.2)	9	**+3.3** (3.4)	9	**+4.5** (3.2)	9
ERT-switch Non-Ambulatory	Upper Body Max score 40	**13.3** (12.2)	3[b]	**+4.5** (0.7)	2[bc]	**+2.7** (2.3)	3[b]	**+4.3** (3.5)	3[b]
ERT-Naive	Total Body Max score 80	**66.9** (3.7)	5	**+0.3** (2.8)	5	**+1.1** (3.1)	5	**+2.0** (2.9)	4[d]

- Quantitative muscle strength testing[e] results were generally consistent with manual muscle test results

ERT=enzyme replacement therapy; SD=standard deviation. [a]Measured via the Medical Research Criteria (MRC) scale; [b]Baseline data missing for 1 patient; [c]One patient did not complete Month 6 assessment; [d]Manual muscle testing not completed for one patient; [e]Measured via hand-held dynamometer.

Sitting Forced Vital Capacity (FVC, % Predicted)

FVC was generally stable in ERT-switch ambulatory patients and increased in ERT-naive patients

	Baseline, mean (SD)	Change From Baseline, mean (SD)		
		Month 6	Month 12	Month 18
Cohort 1 ERT-Switch Ambulatory	n=9[a]	n=9[a]	n=9[a]	n=8[a,b]
	52.6 (14.7)	**−1.3** (4.1)	**−3.3** (6.1)	**−3.7** (7.0)
Cohort 3 ERT-Naive	n=5	n=5	n=5	n=5
	53.4 (20.3)	**+4.2** (5.6)	**+4.4** (8.6)	**+5.0** (2.9)

- FVC was stable or increased in 5/9, 6/9, and 5/8 ERT-switch patients at Months 6, 12, and 18, respectively
- FVC was stable or increased in 5/5, 4/5, and 5/5 ERT-naive patients at Months 6, 12, and 18, respectively

ERT=enzyme replacement therapy; SD=standard deviation.
[a]Baseline FVC not available for 1 patient in Cohort 1; [b]FVC for one patient in Cohort 1 pending (visit had not occurred at time of interim data cut). .

Other Pulmonary Function Tests: MIP and MEP

MIP was stable and MEP increased in ERT-switch ambulatory patients; MIP and MEP increased in ERT-naive patients

	Assessment	Baseline, mean (SD)	Change From Baseline, mean (SD)		
			Month 6	Month 12	Month 18
Cohort 1 ERT-Switch Ambulatory		n=10	n=10	n=10	n=9[a]
	MIP	**35.7** (11.0)	**+0.3** (4.6)	**0.0** (3.2)	**−2.8** (4.4)
	MEP	**72.6** (32.6)	**+16.1** (42.1)	**+28.6** (44.0)	**+30.2** (43.0)
Cohort 3 ERT-Naive		n=5	n=5	n=5	n=5
	MIP	**32.6** (18.5)	**+11.0** (5.0)	**+5.2** (12.2)	**+6.2** (11.5)
	MEP	**60.6** (8.3)	**−0.4** (12.4)	**+8.6** (16.3)	**+9.8** (19.6)

ERT=enzyme replacement therapy; MEP=maximal expiratory pressure; MIP=maximal inspiratory pressure; SD=standard deviation.
MIP and MEP measured in centimeters of water.
[a]Data for one patient in Cohort 1 pending (visit had not occurred at time of interim data cut).

Fatigue Severity Scale (FSS)

All cohorts were significantly impacted by fatigue at baseline and demonstrated a mean improvement in fatigue

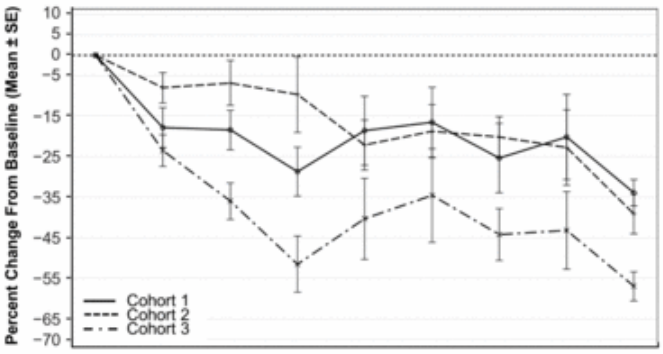
	Baseline, mean (SD)	Change From Baseline, mean (SD)		
		Month 6	Month 12	Month 18
Cohort 1 ERT-Switch Ambulatory	n=10	n=10	n=10	n=9
	53.5 (7.7)	**−8.0** (10.7)	**−8.0** (6.5)	**−3.8** (12.2)
Cohort 2 ERT-Switch Nonambulatory	n=4	n=4	n=4	n=3
	42.3 (14.6)	**+2.3** (8.7)	**−12.5** (10.0)	**−13.3** (2.1)
Cohort 3 ERT Naive	n=5	n=5	n=5	n=5
	39.2 (12.7)	**−5.2** (11.7)	**−7.2** (7.5)	**−2.0** (7.5)

ERT=enzyme replacement therapy; SD=standard deviation.
1. Grace J et al. *Parkinsonism Relat Disord*. 2007;13(7):442-445.
FSS consists of 9 questions, each scored on a scale from 1 to 7. Total scores range from 9 to 63, with higher values representing higher levels of fatigue due to the disease condition. The normative value in the healthy population is ~21.[1]

CK and Hex4 Biomarkers

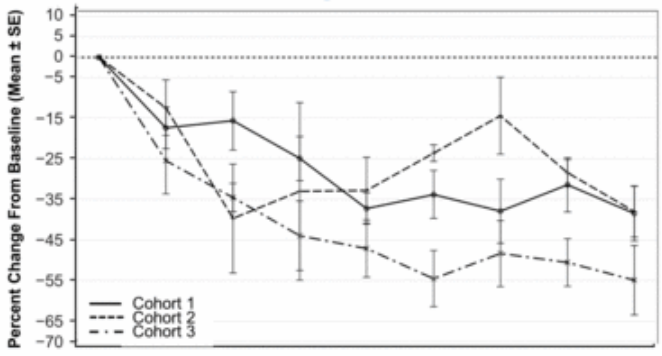
All cohorts demonstrated persistent improvement in biomarkers of muscle damage (CK) and disease substrate (Hex4) for up to 18 months

Percent Change From Baseline for CK



n	BL	W2	W4	M3	M6	M9	M12	M15	M18
Cohort 1	11	11	11	11	10	10	10	9	8
Cohort 2	4	4	5	3	4	4	4	4	3
Cohort 3	5	5	4	5	5	5	5	3	3

Percent Change From Baseline for Hex4



n	BL	W2	W4	M3	M6	M9	M12	M15	M18
Cohort 1	11	11	11	11	10	10	10	10	9
Cohort 2	4	4	4	4	4	4	4	4	2
Cohort 3	5	5	5	5	5	5	5	5	3

BL=baseline; CK=creatine kinase; Hex4=urine hexose tetrasaccharide; M=month; W=week.
Reported through interim data analysis; missing values either unable to be analyzed or not yet analyzed.

Safety Summary at 18 Months of Treatment

Safety data (N=20) for AT-GAA show that AEs have been generally mild and transient with very low rates of IARs (<1%) after 890+ total infusions across all cohorts

- AEs were generally mild and transient
  - The most common treatment-emergent AEs[a] by decreasing frequencies were nasopharyngitis (10/20); fall (9/20); abdominal pain[b] and diarrhea (8/20); upper respiratory tract infection (7/20); arthralgia, nausea, fatigue, pain in extremities, and myalgia (6/20); and headache, tremor, oropharyngeal pain, and muscle spasms (5/20)
- For SAEs, 5 events occurred in 4 patients (severity: 3 moderate, 2 mild) and were unrelated to treatment. SAEs did not lead to treatment interruption or study discontinuation.
- 7 incidents of IARs in 5 patients in 890+ infusions, which were controlled by standard medication or premedication
  - 1 IAR event each in 3 ambulatory ERT-switch patients
  - 1 IAR event in a non-ambulatory ERT-switch patient
  - 3 IAR events in a ERT-naive patient
- Longest duration of treatment is 28+ months

AE, adverse events; ERT=enzyme replacement therapy; IAR, infusion-associated reaction; SAE=serious adverse event.
[a]Number of patients experiencing the AE; [b]Includes upper and lower abdominal pain.

Conclusions at 18 Months of Treatment

6MWT, an integrated measure of motor, cardiac, and pulmonary function, improved in ERT-switch ambulatory and ERT-naive patients out to Month 18

- 6MWT showed continued benefit in ERT-switch and ERT-naive patients
- Timed motor function tests were generally consistent with 6MWT results in both ambulatory cohorts
- Muscle strength increased in all cohorts, including nonambulatory ERT-switch patients
- Pulmonary function
  - FVC, MIP, and MEP generally increased in ERT-naive patients
  - FVC, MIP, and MEP were generally stable in ERT-switch patients
- Fatigue Severity Scale
  - Improvement in fatigue score was observed in all cohorts
- Biomarkers and safety
  - CK and Hex4 levels decreased in all cohorts
  - AT-GAA (ATB200/AT2221) was generally well tolerated

6MWT=6-minute walk test; CK=creatine kinase; ERT=enzyme replacement therapy; FVC=forced vital capacity; Hex4=urine hexose tetrasaccharide; MEP=maximal expiratory pressure; MIP=maximal inspiratory pressure.

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