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): **February 16, 2017**

AMICUS THERAPEUTICS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**001-33497**	**71-0869350**
(State or other Jurisdiction of Incorporation)	(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))

Item 8.01. Other Events.

The senior management of Amicus Therapeutics, Inc. (the "Company") is using the presentations attached as Exhibit 99.1 and Exhibit 99.2 to this Current Report in its current meetings with investors and analysts.

Item 9.01. Financial Statements and Exhibits.

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

Exhibit No.	Description
99.1	Presentation Materials — Corporate Overview (February 2017)
99.2	Presentation Materials — Pompe Disease: A New Understanding and A New Approach (February 2017)

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: February 16, 2017

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

Exhibit 99.1

Amicus Therapeutics®

Corporate Overview

February 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. 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 our cash position, actual results may differ based on market factors and our ability to execute 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 presentation to reflect events or circumstances after the date hereof.

Building a Top Global Biotech in Devastating Rare Diseases

Galafold (migalastat)
FIRST ORAL PRECISION MEDICINE FOR FABRY DISEASE

3 PROGRAMS
IN CLINIC IN 3 RARE DISEASES

1 BREAKTHROUGH
THERAPY DESIGNATION

WORLD CLASS SCIENCE & DRUG DEVELOPMENT

ATB200/AT2221
NOVEL TREATMENT PARADIGM FOR POMPE IN PHASE 1/2

TREATING PATIENTS IN 24 COUNTRIES

Two Phase 3 PROGRAMS
(FABRY & EB)

$3B+ MARKET OPPORTUNITY FOR CURRENT PIPELINE

PROTEIN ENGINEERING & GLYCOBIOLOGY

$331M CASH BALANCE

Key Accomplishments in 2016

	2016
Fabry Disease (Galafold™)	• EU approval • International launch success • Regulatory progress
Pompe Disease (ATB200/AT2221)	• Positive preliminary data in Phase 1/2 study in Pompe patients
Epidermolysis Bullosa (EB) (SD-101)	• Phase 3 enrollment near complete
Strong Balance Sheet	• $331M in cash (12/31/16)

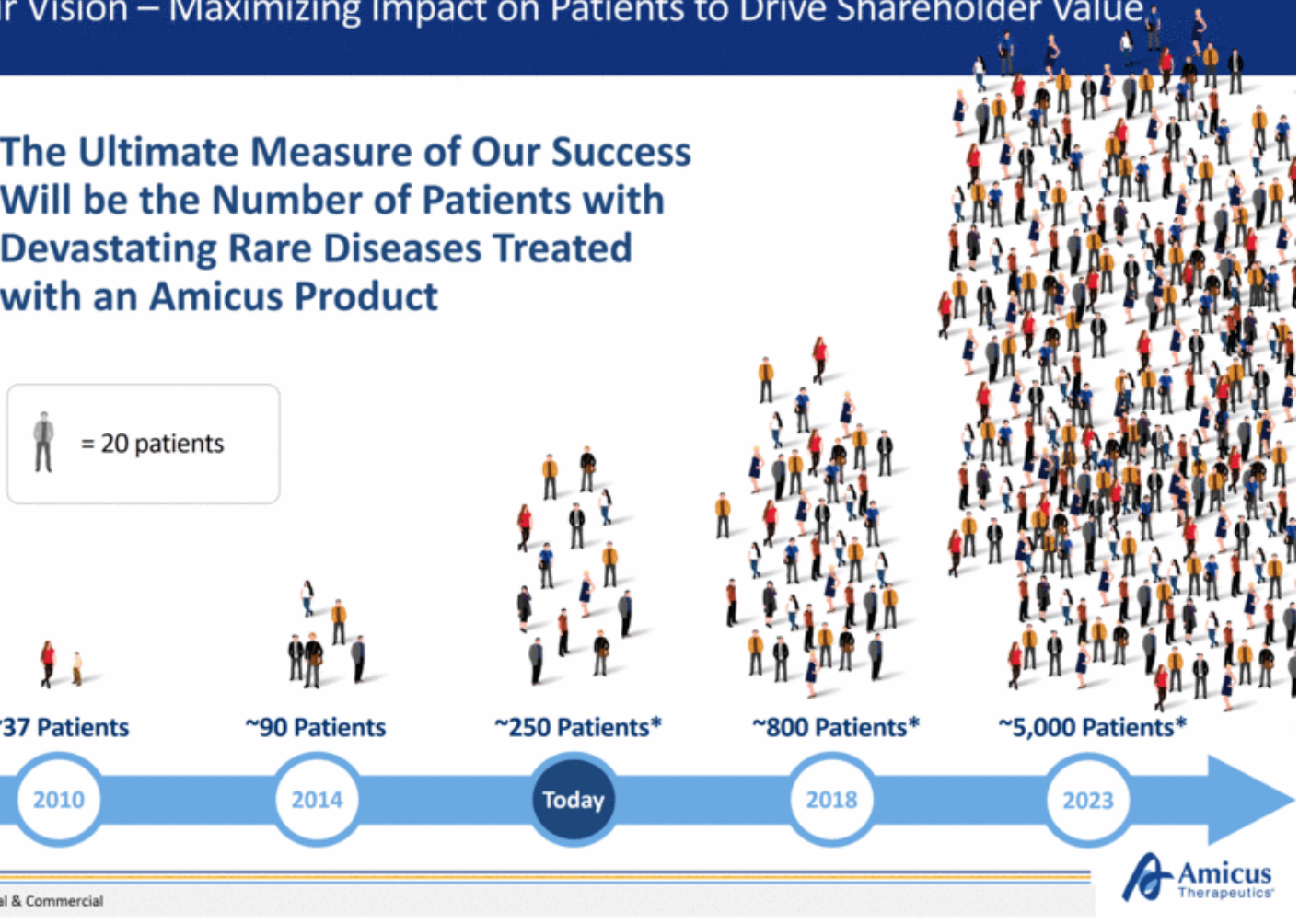
2017 Key Strategic Priorities

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

- Advance International Galafold Launch
- Submit Japanese New Drug Application (J-NDA) for Migalastat
- Establish Definitive Proof of Concept for ATB200/AT2221 with Clear Path to Registration for Pompe Disease
- Successfully Complete Phase 3 EB Study
- Maintain Financial Strength

Our Vision – Maximizing Impact on Patients to Drive Shareholder Value

The Ultimate Measure of Our Success Will be the Number of Patients with Devastating Rare Diseases Treated with an Amicus Product



*Clinical & Commercial

Amicus Therapeutics

Galafold™ (Migalastat) Precision Medicine for Fabry Disease

Continue Launch Execution and Geographic Expansion

Fabry Disease Overview

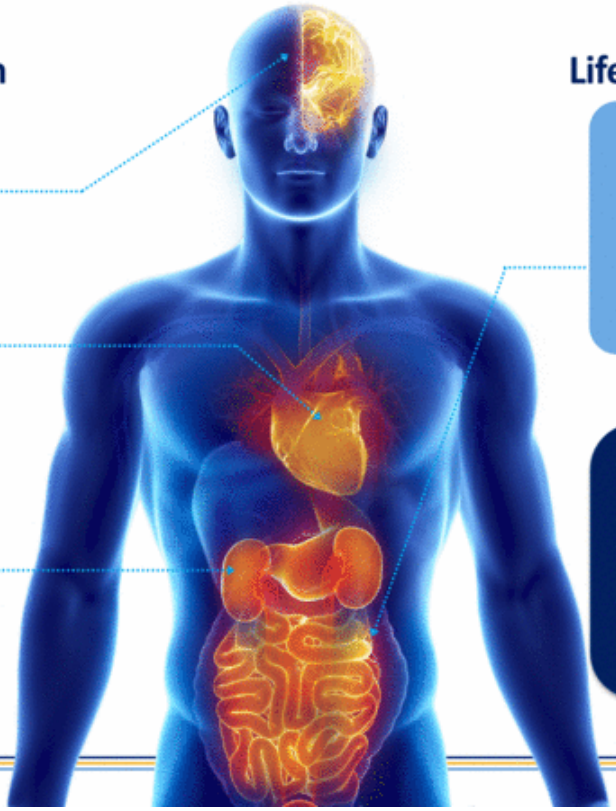
Fabry Disease is a Fatal Genetic Disorder that Affects Multiple Organ Systems

Leading Causes of Death

TRANSIENT ISCHEMIC ATTACK (TIA) & STROKE[1]

HEART DISEASE[2]
- Irregular heartbeat (fast or slow)
- Heart attack or heart failure
- Enlarged heart

KIDNEY DISEASE[3]
- Protein in the urine
- Decreased kidney function
- Kidney failure



Life-Limiting Symptoms

GASTROINTESTINAL[3]
- Nausea, vomiting, cramping, and diarrhea
- Pain/bloating after eating, feeling full
- Constipation
- Difficulty managing weight

Key Facts

- Deficiency of α-Gal A enzyme leading to GL-3 accumulation
- >900 known mutations
- 5-10K diagnosed WW (51% female/49% male[4])
- Newborn screening studies suggest prevalence of ~1:1000 to ~1:4000

1. Desnick R, *et al.* **Ann Intern Med. 2003** 2. Yousef Z, *et al.* **Eur Heart J. 2013** 3. Germain D. **Orphanet J Rare Dis. 2010** 4. Fabry Registry 2011

Amicus Therapeutics

Precision Medicine Driven by a Patient's Genotype

Amicus Therapeutics is Committed to Innovative R&D to Develop the Highest Quality Therapies for ALL Fabry Patients

Today

Migalastat

Oral precision medicine

~$1.2B

Global Fabry market today

Growing to

~$2B

Global Fabry market

Future Vision

Novel ERT co-formulated with migalastat

*Artist rendering, not actual product image

Amicus Therapeutics

Full EU Approval as First Oral Precision Medicine for Fabry Disease

Galafold Indicated for Long-Term Treatment of Adults and Adolescents Aged ≥ 16 years with a Confirmed Diagnosis of Fabry Disease and Who have an Amenable Mutation[3]

- **Approved May 30, 2016**
- **Launch exceeding expectations**

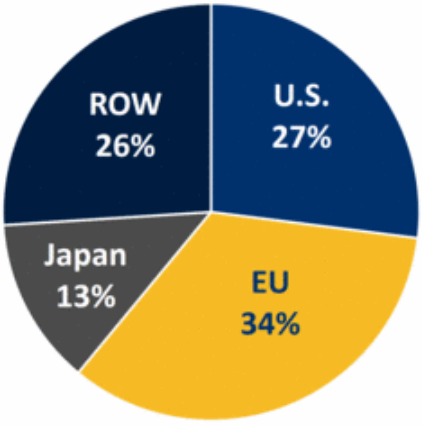
1. Germain, DP et al., New England Journal of Medicine. 2. Hughes, et al., Journal of Medical Genetics. 3. For important safety information for Galafold visit www.ema.europa.eu.

Amicus Therapeutics

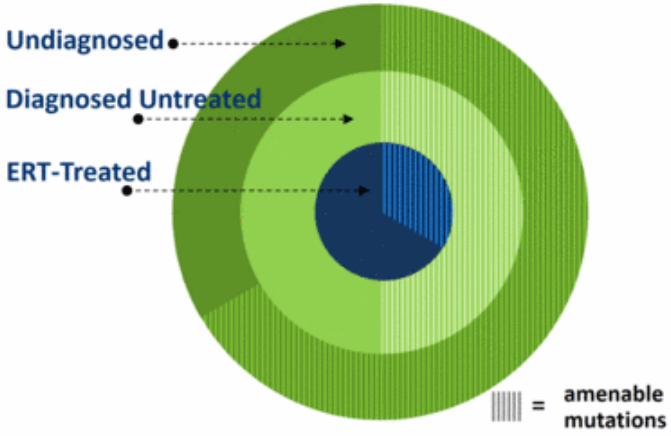
Galafold Commercial Opportunity

Prioritizing EU, Japan, and Other Large Fabry Markets to Address Patients with Amenable Mutations (35%-50% of Fabry Population)

Geographic Segments



Patient Segments



- 5k-10k Patients Diagnosed WW
- 40%-50% of Diagnosed Patients not on ERT
- Newborn Screening Studies Suggest Prevalence of ~1:1000 to ~1:4000[1]

1. Burton, **LDN WORLD Symposium,** 2012 Feb. Mechtler *et al.*, **The Lancet**, 2011 Dec. Hwu *et al.*, **Hum Mutation**, 2009 Jun. Spada *et al.*, **Am J Human Genet.**, 2006 Jul

Amicus Therapeutics

Early Success with International Launch (as of 12/31/16)

Initial Launch Success Driven by Germany with ERT-Switch & Naïve Patients, Reimbursement Now Available in 6 Countries*

- **61** Patients (Switch & Naïve) on reimbursed Galafold (12/31/16)
- **6** Countries with available reimbursement*
- **18** Countries with pricing discussions ongoing
- **22** Countries with Amicus footprint

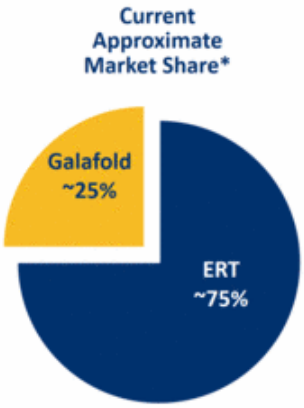
Target Number of Patients on Reimbursed Galafold by YE17

*Commercial and Expanded Access Programs (EAPs)

Amicus Therapeutics

German Launch Update (as of 12/31/16)

Germany is an Important Indicator for EU Launch Success



IMPORTANT EARLY INDICATORS IN GERMANY

- Vast majority switch patients
- ~25% of eligible switch patients now on Galafold*
- All newly experienced patients & physicians
- Majority of switches from Replagal™
- Male / female mix
- 13 unique prescribers

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

Amicus Therapeutics

EU Launch Strategy

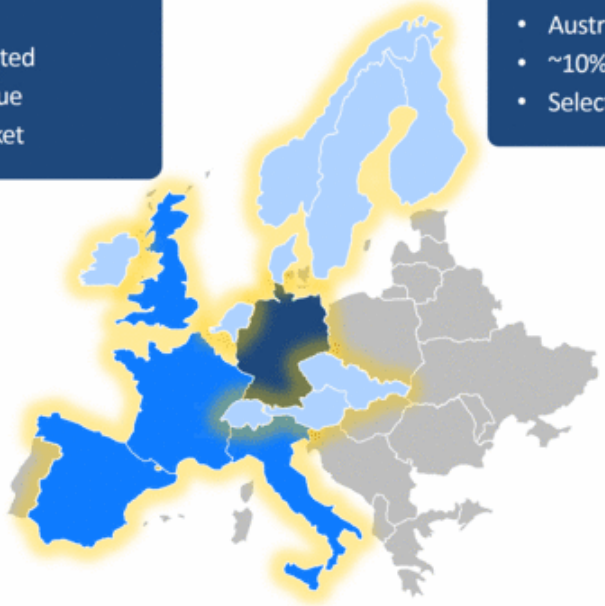
Focus on EU Top 5 Plus Key Mid-Sized EU Markets in 2017

INITIAL FOCUS ON TOP 5 COUNTRIES

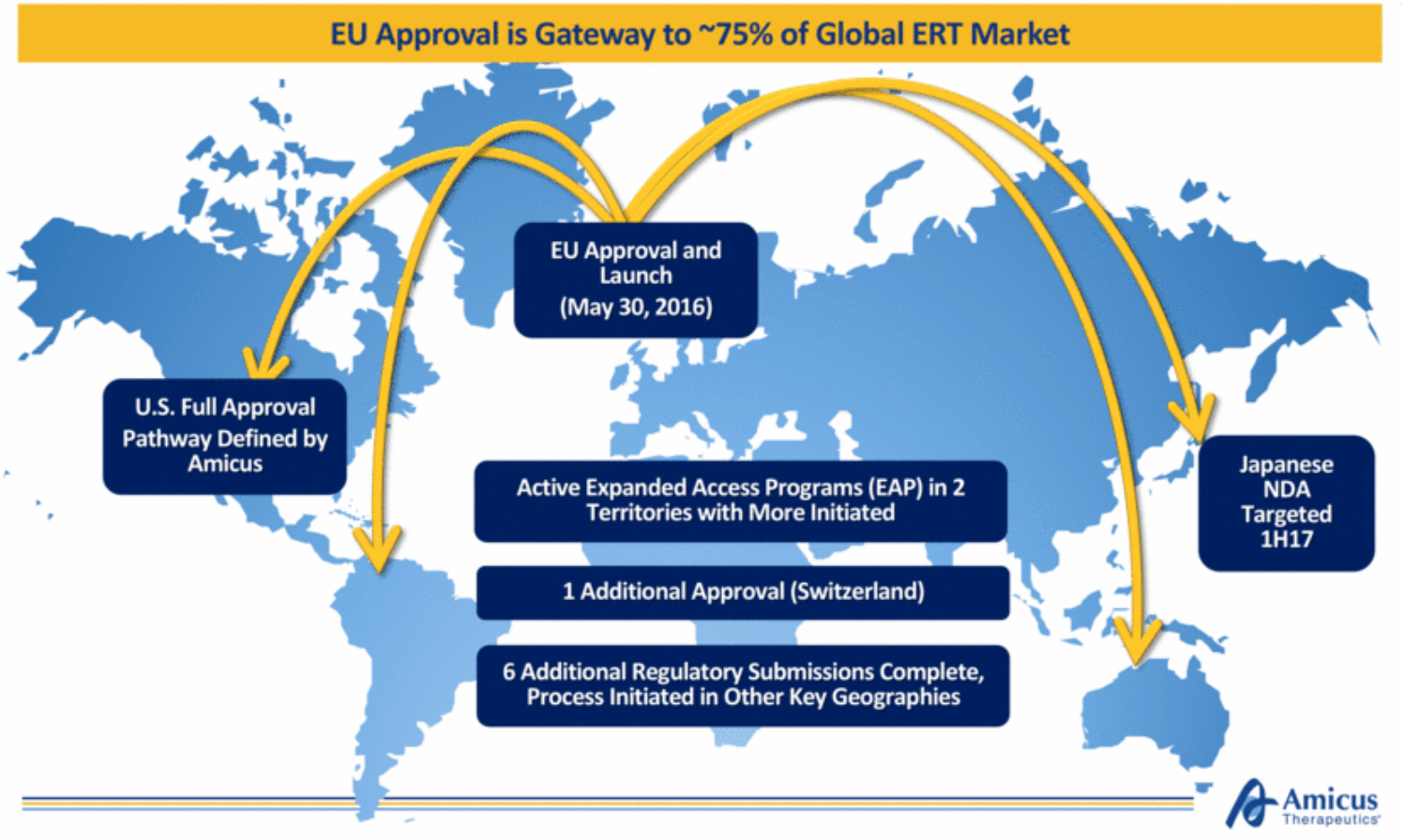
- Germany
- France, Italy, Spain, UK
- ~2,000 Fabry patients treated
- ~70-75% of EU market value
- ~25% of global Fabry market

INVEST IN KEY MID-SIZED EU COUNTRIES AND SELECT EAP OPPORTUNITIES

- Austria, Nordics (4), Netherlands, Belgium, etc.
- ~10% of EU market value
- Selectively invest in key EAP markets



Global Regulatory Strategy to Reach More Patients



Amicus Proprietary Fabry ERT

Building on Biologics Capabilities and CHART™ Platform to Develop Differentiated Novel ERT

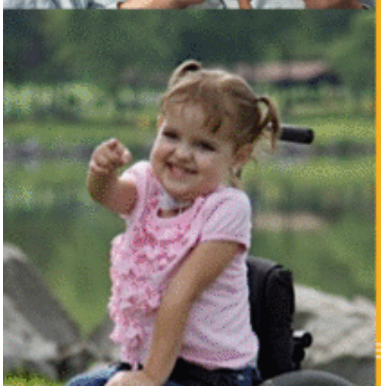
Development status:

- Cell line transferred to manufacturer
- Preclinical data update in 2017

Fabry ERT Target Product Profile:

- Improved drug targeting to key tissues
- Significantly more potent dose delivery
- Co-formulation with chaperone to enhance stability
- Dosing flexibility

Amicus Therapeutics



ATB200 Novel ERT for Pompe Disease

Establishing Human Proof of Concept and Validating Biologics Platform in 2017

Pompe Disease Overview

Devastating Disease Symptoms Persist Across a Broad Spectrum of Patients Despite Available Therapy

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

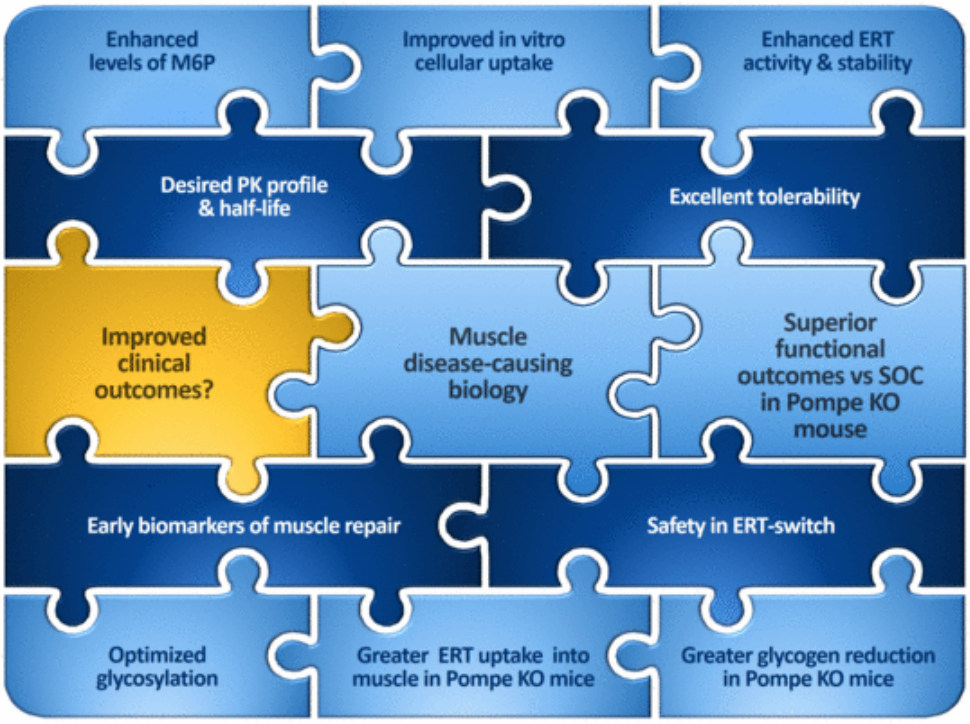
~$800M+ Global Pompe ERT sales in FY15[2]

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

Amicus Therapeutics

Pompe Disease: A Complex Disease with Significant Unmet Needs

We've Made Great Strides and Expect to Address Key Remaining Questions in 2017

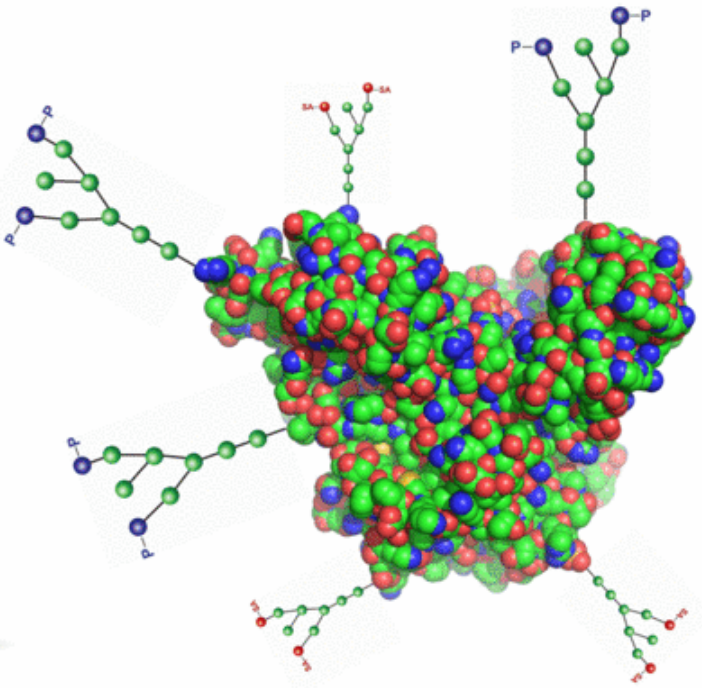


Amicus Therapeutics

ATB200 + Chaperone: A Highly Differentiated Approach

Novel Pompe Treatment Paradigm with Three Key Differentiators

ATB200 (Novel ERT)



Optimized mixture of glycans

High levels of M6P and bis M6P

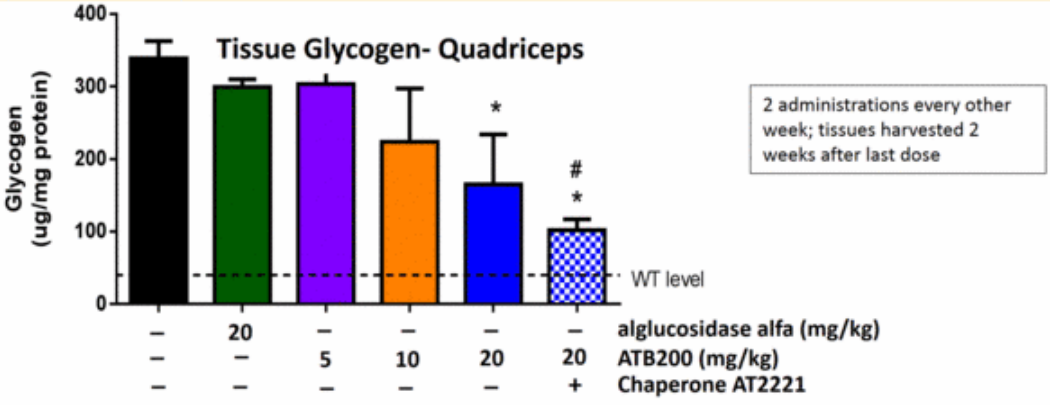
Chaperone addition

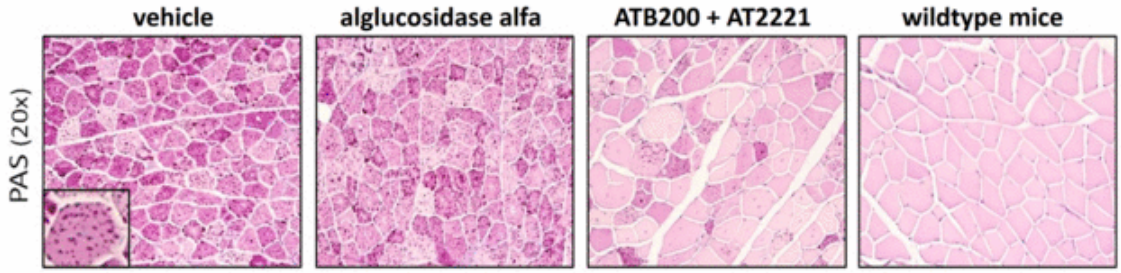
*Artist rendering, not actual product image

Amicus Therapeutics

Substrate Clearance & Cellular Physiology

ATB200/AT2221 Improved Substrate Clearance and Cellular Physiology in Preclinical Models[1]



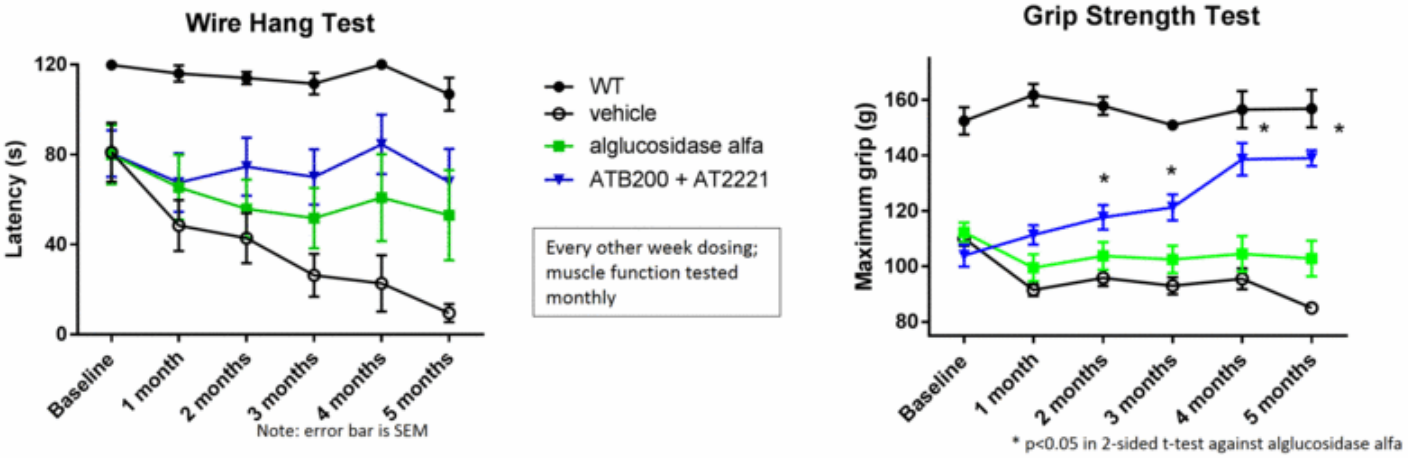


1. Following 2 doses of 20mg/kg alglucosidase alfa or ATB200 + AT2221 in Gaa KO mice, skeletal muscles evaluated for glycogen clearance and proliferated lysosomes. Treatment with alglucosidase alfa modestly reduced glycogen or proliferated lysosomes while ATB200, co-administered with AT2221 significantly decreased the muscle pathology associated with Pompe disease.

Amicus Therapeutics

Functional Muscle Strength

ATB200/AT2221 Progressively Increased Muscle Function and Appears to Induce Muscle Repair and/or Regeneration Rather than Just Maintenance of Damaged Muscle



Amicus Therapeutics

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)

18-Week Primary Treatment Period with Long-Term Extension (n ~20)

Cohort 1 (Ambulatory ERT-Switch)

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) & Cohort 3 (ERT-Naive)

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

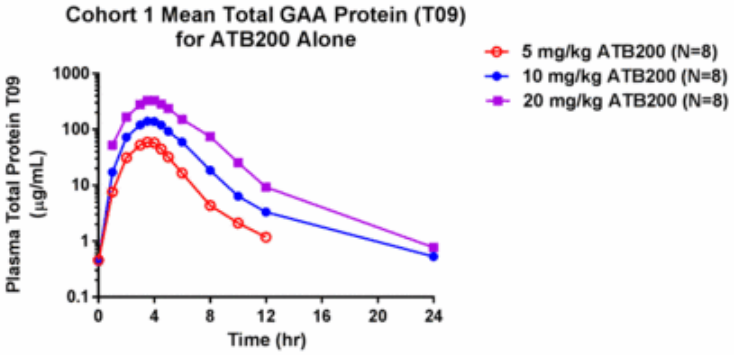
Assessments:

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

Pharmacokinetics in ERT-experienced patients (Cohort 1 N=8)*

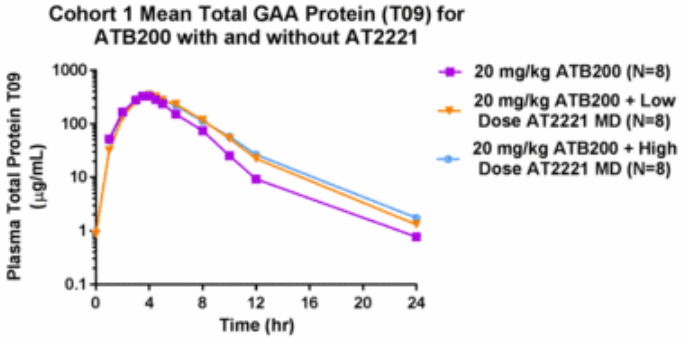
ATB200 Clinical PK Profile as Predicted Based on Preclinical Studies with Greater than Dose Proportional Increases in Exposure that were Enhanced by AT2221

Mean GAA Total Protein (n=8)
5, 10, 20 mg/kg ATB200 Alone



Treatment	Mean $AUC_{0-\infty}$ (hr*µg/ml)	Mean Half-Life (hr)
5 mg/kg	218	1.1
10 mg/kg	584	1.3
20 mg/kg	1512	1.5

Mean GAA Total Protein (n=8)
20 mg/kg ATB200 + AT2221



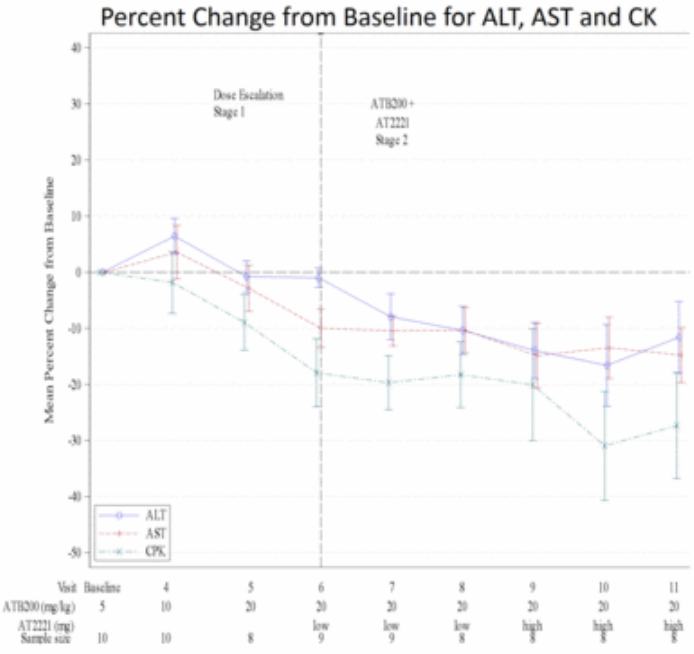
Treatment	Mean $AUC_{0-\infty}$ (hr*µg/ml)	Mean Half-Life (hr)
20 mg/kg	1512	1.5
+low dose AT2221	1808	1.9
+high dose AT2221	1901	2.3

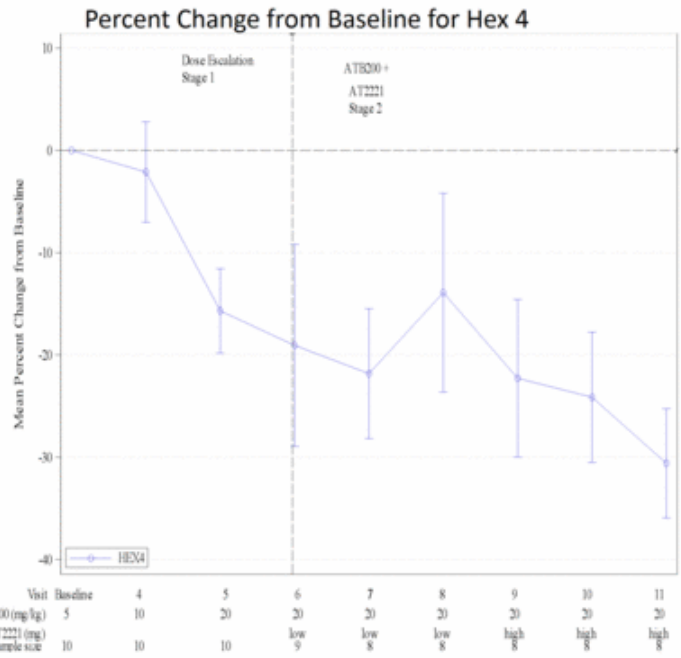
2 patients excluded that did not have full PK data at time of data cut

Amicus Therapeutics

Cohort 1: Biomarkers at Week 18 (N=8)

After Switching from SOC to ATB200/A2221 Patients Generally Demonstrated an Improvement in Biomarkers of Muscle Damage (ALT, AST, CK) and Biomarkers of Disease Substrate (Hex4)

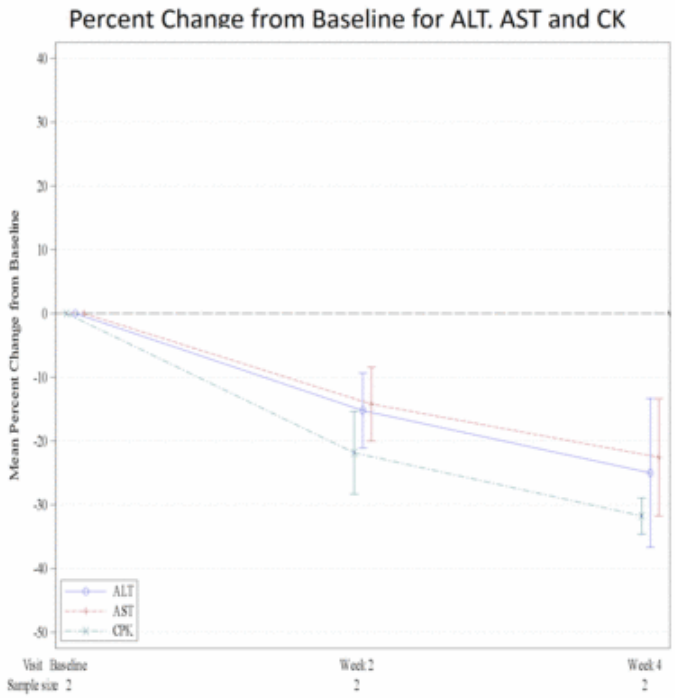


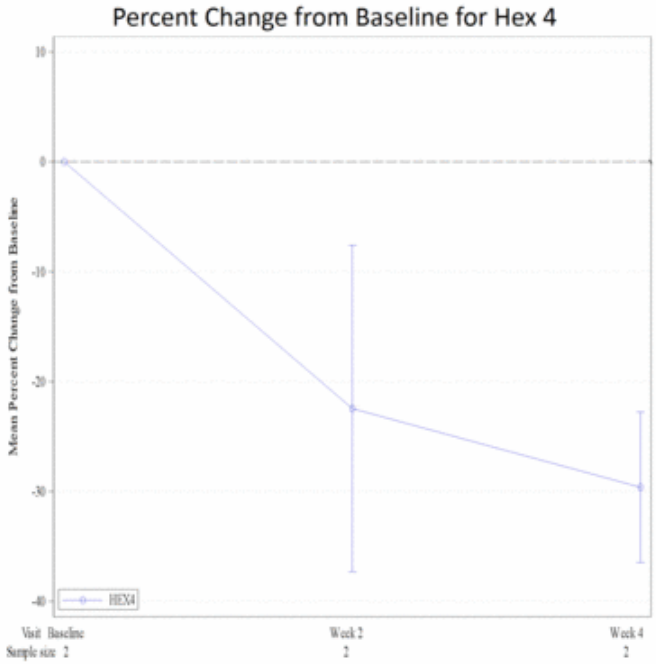


Amicus Therapeutics

Cohort 3: Biomarkers at Week 4 (N=2)

Initial Two Naïve Patients Treated with ATB200/AT2221 Demonstrated Robust Reduction in Biomarkers of Muscle Damage (ALT, AST, CK) and Biomarkers of Disease Substrate (Hex4)





Amicus Therapeutics

Muscle Biomarkers and Urine Hex 4 (N=10)*

- In ERT-switch patients (Week 18):
  - ALT decreased in 5 of 8 patients; 4/4 patients with elevated baseline levels normalized
  - AST decreased in 6 of 8 patients; 3/4 patients with elevated baseline levels normalized
  - CK decreased in 6 of 8 patients; 2/6 patients with elevated baseline levels normalized
  - ALT, AST, CK generally remained stable for patients not demonstrating a decrease
  - Urine Hexose Tetrasaccharide (Hex4) decreased in 8 of 8 patients; overall reduction approximately 30%

- In ERT- naïve patients (Week 4):
  - ALT, AST, CK and Urine Hex4 decreased in 2 of 2 patients.

**N=10 includes 8 switch and 2 naïve patients*

Preliminary Data Summary

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

- Safety (N=13)*
  - No serious adverse events (SAEs)
  - AEs were generally mild and transient
- Tolerability
  - No infusion-associated reactions following 150+ infusions in all patients enrolled to date
- PK (N=10)**
  - 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) and substrate biomarker (urine Hex4) (N=10)**
  - Decrease/normalization of muscle injury biomarkers and biomarker of substrate following a switch from SOC to ATB200/AT2220, and in ERT-naïve patients, suggests positive effect of the new therapy on muscle cells

*N=10 from Cohort 1 (Ambulatory ERT-Switch); N=1 from Cohort 2 (Non-Ambulatory ERT-Switch); N=2 from Cohort 3 (Naïve)
**N=8 from Cohort 1 & N=2 from Cohort 3

Amicus Therapeutics

Biologics Manufacturing Capabilities

Highly Successful Biologics Manufacturing Scale-up in Three Years

Proprietary Process

Research Scale / MCB

5L (Bench Scale) 2013

250L (Clinical Scale) 2014-2015+

1000L (Registration Trial & Commercial Scale) 2016-2017+

Amicus Therapeutics

Pompe Clinical Study ATB200-02 Data Cascade

A Cascade of Additional Data Points During 2017 to Demonstrate 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)
- Meeting with U.S. and EU regulators

18-WEEK DATA

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

EXTENSION DATA

- Motor/pulmonary function

Amicus Therapeutics

SD-101 for Epidermolysis Bullosa

Potential First-in-Class Treatment with Phase 3 Data Anticipated Mid-2017

EB Disease Overview

Rare, Devastating, Connective Tissue Disorder with No Approved Treatments

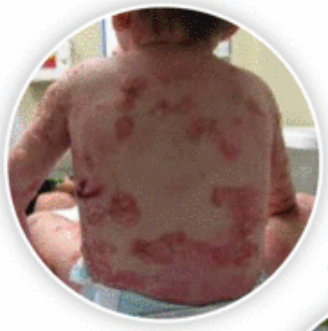
Disease Overview

- Multiple genes cause disease
- Can affect internal organs
- Can be fatal
- Wounds can lead to life-threatening infections
- Diagnosis: infancy to adulthood
- 30,000 – 40,000+ diagnosed in major global regions
- $1B+ potential market

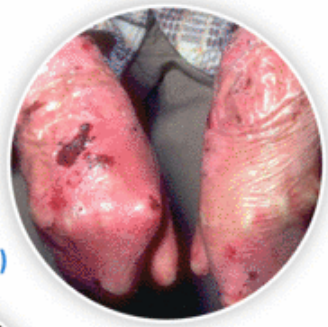
Three Major EB Types

(~99% of EB Population)

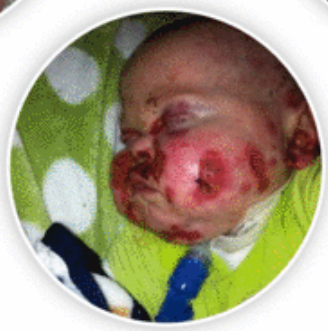
SIMPLEX (75%)



DYSTROPHIC (20%)



JUNCTIONAL (5%)

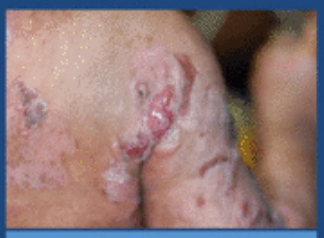


Amicus Therapeutics

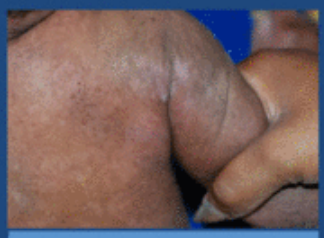
Proof of Concept Findings

Phase 2 Results Informed Phase 3 Design

Phase 2a Key Takeaways (SD-101 3%)



1-Year-Old Girl with EB Simplex at Baseline



Following 2 months of treatment with SD-101

Breakthrough Therapy Designation

Phase 2b Key Takeaways (SD-101 6%)

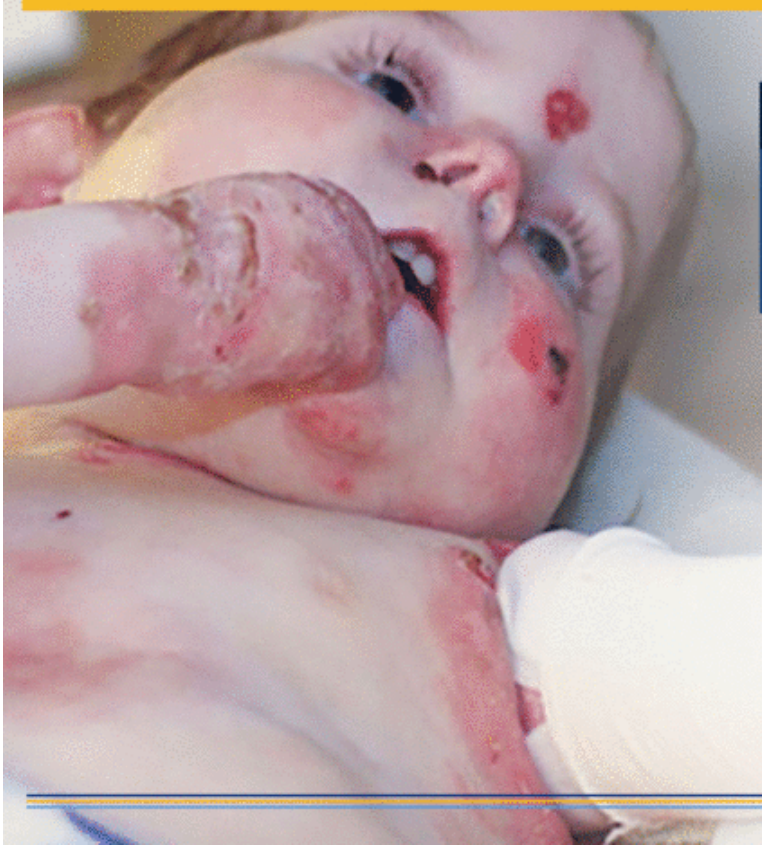
- Faster time to wound closure
- Higher proportion with complete closure
- Reduction in total body surface area (BSA) of wounds
- Larger wounds (>10 cm^2) showed widest separation versus placebo
- Daily administration generally safe and well-tolerated

Informed Phase 3 Study Design

Amicus Therapeutics

Phase 3 Study - Delivering on Our EB Vision

Phase 3 Study Optimized for Success with Top-Line Data Anticipated Mid-2017



SD-005 Study Design Optimized

- Sample size of up to 150 patients
- Larger baseline target wound size
- Time to wound closure endpoint elevated

Status

- 95%+ participation in extension study
- Enrollment near complete
- Top-line data anticipated mid-2017

Amicus Therapeutics

Cyclin-Dependent Kinase-Like 5 (CDKL5) Deficiency

Preclinical Development Underway for a Rare, Devastating, Genetic Neurological Disease with No Approved Treatments

Disease Overview

- Genetic mutations in CDKL5 gene result in deficient protein essential for normal brain development
- Persistent, spontaneous seizures starting in infancy
- Severe impairment in neurological development
- Most affected children cannot walk, talk or care for themselves
- May include scoliosis, visual impairment, sensory issues, and gastrointestinal complications
- >1,200 documented cases worldwide[1]
- Patient identification rising significantly

1. LouLouFoundation.org

Amicus Therapeutics

Financial Summary & Key Milestones

Financial Summary & Guidance

Balance Sheet Strengthened with $331M Cash at 12/31/16 and Cash Runway Into 2H18

Financial Position	December 31, 2016
Cash	$331M
Debt	$250M
FY17 Net Operating Cash Flow Guidance	$175-$200M
FY17 Net Cash Spend Guidance*	$200-$225M
Cash Runway	2H18
Capitalization	**December 31, 2016**
Shares Outstanding	142,691,986

*Includes third party milestone payments and capital expenditures

Amicus Therapeutics

Key Milestones in 2017

	2017
Fabry Disease (Galafold)	• Galafold international launch targeting 300 patients by YE17 • Japan NDA submission • Fabry GI study initiation
Pompe Disease (ATB200/AT2221)	• Phase 1/2 data cascade • Meetings with U.S. and EU regulators
Epidermolysis Bullosa (EB) (SD-101)	• Phase 3 data
Strong Balance Sheet	• Significant revenue contribution in 2017 • Runway into 2H18

Thank You

Amicus Therapeutics®

Exhibit 99.2

Amicus Therapeutics®

Pompe Disease: A New Understanding & A New Approach

February 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 pre-clinical and preliminary clinical 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 belief's which are subject to a number of risks, uncertainties and factors, including that both the preclinical and 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 preclinical, preliminary clinical data or data from the completed study or any future study will not yield results that are consistent with the preclinical and 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, 2015 and Quarterly Report on 10-Q for the Quarter ended September 30, 2016. As a consequence, actual results may differ materially from those set forth in this presentation. 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 presentation to reflect events or circumstances after the date hereof.

Introduction & Pompe Landscape

SUMMARY OF KEY TAKEAWAYS FROM WORLD SYMPOSIUM

Amicus Research is Changing the Way that the Pathophysiology of Pompe Disease is Understood and Preclinical and Clinical Data Suggest that the Amicus Treatment Paradigm of ATB200/AT2221 Has the Potential to Become the New Standard of Care

- **PROFOUND ADVANCEMENT IN POMPE UNDERSTANDING FROM AMICUS SCIENCE**: Amicus research shown at the WORLD*Symposium* significantly advances understanding of the causes of muscle devastation in Pompe disease and potentially links Pompe to other forms of muscular dystrophy
- **LIMITATIONS OF CURRENT ERT STANDARD OF CARE:** The current ERT, while having efficacy, has significant limitations on targeting, activity and tolerability. Many patients continue to decline in muscle function while on current ERT[1]
- **ATB200/AT2221 IS DESIGNED TO BE HIGHLY DIFFERENTIATED FROM CURRENT ERT AND FROM NEO-GAA**
- **PRECLINICAL DATA FROM WORLD SHOW ATB200/AT2221 DISTINGUISHED:** Data demonstrate reversal of cellular damage to enable muscle repair and improve muscle cytoarchitecture, and improved strength in Pompe KO model
- **LATEST RELEASE OF ATB200/AT2221 CLINICAL DATA SHOWS FURTHER TRENDS TOWARD IMPROVEMENT ON ALL KEY BIOMARKERS OF DISEASE ASSESSED, DISTINCT FROM OTHERS' PUBLISHED DATA**

1. Wyatt, *et al.*, HTA. (2012)

Amicus Therapeutics

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

Pompe Development History

20+ Years of Development Have Led to ONE Approved Product. Significant Unmet Need Remains

- **1998** First rhGAA cell line developed
- **2003** Myozyme entered clinic
- **2004** NeoGAA preclinical data first published[1]
- **2006** Myozyme approved (infants)
- **2010** Lumizyme approved (LOPD); BMN-701 entered clinic
- **2013** NeoGAA entered clinic
- **2014** BMN-701 entered Phase 2/3 switch study
- **2016** ATB200/AT2221 entered clinic; NeoGAA Phase 3 study initiated; BMN-701 discontinued

1. Zhu, *et al.*, Biochem J. (2004)

Amicus Therapeutics

Myozyme/Lumizyme Overview

rhGAA Designed to Replace Deficient/Missing GAA Enzyme is First and Only Approved Treatment

- Validated MOA
  - ERT produced by recombinant DNA technology
  - Exogenous source of GAA
  - M6P carbohydrate groups of Lumizyme molecules bind to M6P receptors on cell surface for uptake and delivery to lysosomes
- Infant-onset study: prolonged vent-free survival
- Late-onset study (LOTS): increases in 6MWT, FVC
- ~$800M+ Global sales in FY15[1]
- Key challenges remain with targeting, activity and tolerability

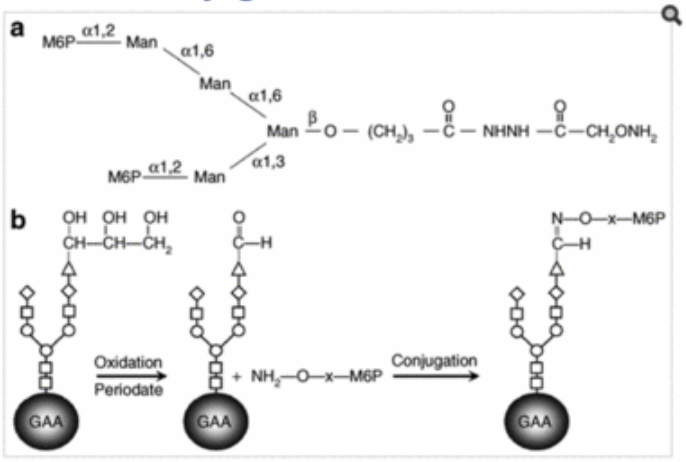
1.. Sanofi 10-K

neoGAA Overview

rhGAA Modified for Higher M6P Levels (After Production) was First Published in 2004 and is Currently in Phase 3 Development

- Initial modifications: no difference in glycogen reduction vs. unmodified rhGAA in Pompe mice[1]
- Alternate approach (oxime-neo-rhGAA)[2]
  - Chemically conjugated a synthetic oligosaccharide bearing M6P residues directly onto rhGAA
  - Greater glycogen reduction and corresponding increase in muscle strength and motor function in mice
  - Improved muscle strength observed in preclinical data but not conclusively demonstrated in Phase 1 clinical study[3]

Synthetic M6P-Bearing Oligosaccharide & Scheme for Conjugation to rhGAA



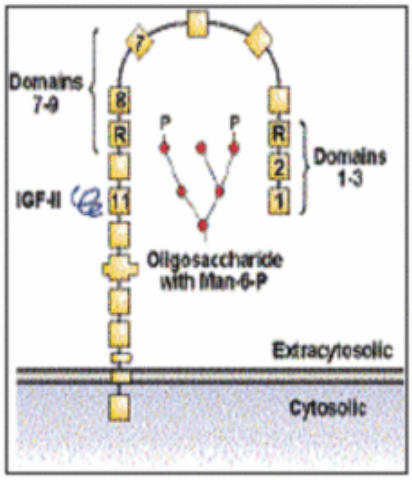
1. Zhu, *et al.*, Biochem J. (2004), 2. Zhu, *et al.*, MolTher. (2009), 3. WORLD*Symposium* 2016

Amicus Therapeutics

BMN-701 Overview

rhGAA with IGF-2 Targeting Moiety to Enhance Cellular Uptake

- Glycosylation-independent insulin-like growth factor 2 (GILT) binding region of CI-MPR designed to enhance uptake
- Off-target effects on IGF-1 and insulin receptor
- Preclinical data on glycogen reduction correlated with improvements in respiratory function
- Clinical data on FVC and 6MWT did not differentiate from standard of care
- Program discontinued in 2016



Adapted from Westlund, *et al.*, J. Biol. Chem. (1991)

Amicus Therapeutics

Data on 6-Minute Walk Test (6MWT)

Next-Generation Therapies Have Not Significantly Differentiated from Standard of Care to Date on 6MWT

Change in 6MWT (m) from Baseline*

Investigational Treatment (Duration)	6MWT (m) (Naives)	6MWT (m) (Switch)
Untreated (LOTS 78 wks)[1] Double Blind Placebo Controlled Study N=30	-3	N/A
Lumizyme 20 mg/kg (LOTS 78 wks)[1] Double Blind Placebo Controlled Study N=60	29	N/A
BMN-701 20 mg/kg (24 Weeks)[2] Open Label Phase 1 Study N=16 Naïve Open Label Phase 2 Study N=18 Switch	22	26
NeoGAA 20 mg/kg (24 Weeks)[3] Open Label Phase 1 Study N=3 Naïve and N=6 Switch	24	-6

*Rounded to Nearest Whole Number. 1. van der Ploeg, *et al.* NEJM (2010) 2. BioMarin press release March 2013, 3. WORLD*Symposium* 2016 Poster and Sanofi press release March 2016

Amicus Therapeutics

Data on Forced Vital Capacity (FVC)

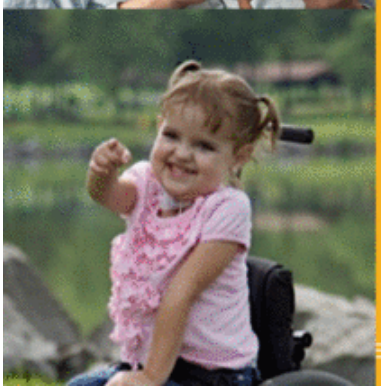
FVC has been a Relevant Endpoint in Previous Pompe Clinical Studies

Change in FVC (%) from Baseline

Investigational Treatment (Duration)	FVC (%) - Naives	FVC (%) - Switch
Untreated (LOTS 78 wks)[1] Double Blind Placebo Controlled Study N=30	-2.2	N/A
Lumizyme 20 mg/kg(LOTS 78 wks)[1] Double Blind Placebo Controlled Study N=30	1.2	N/A
BMN-701 20 mg/kg (24 Weeks)[2] Open Label Phase 1 Study N=16 Naïve Open Label Phase 2 Study N=18 Switch	2.0	-3.7
NeoGAA 20 mg/kg (24 Weeks)[3] Open Label Phase 1 Study N=3 Naïve and N=6 Switch	6.2	1.4

1. van der Ploeg, *et al.* NEJM (2010) 2. BioMarin press release March 2013, 3. WORLD*Symposium* 2016 Poster and Sanofi press release March 2016

Amicus Therapeutics



ATB200/AT2221: a Highly Differentiated Approach

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]

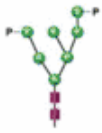
Antibody titers shown to affect treatment outcomes[4,5]

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

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

Amicus Therapeutics

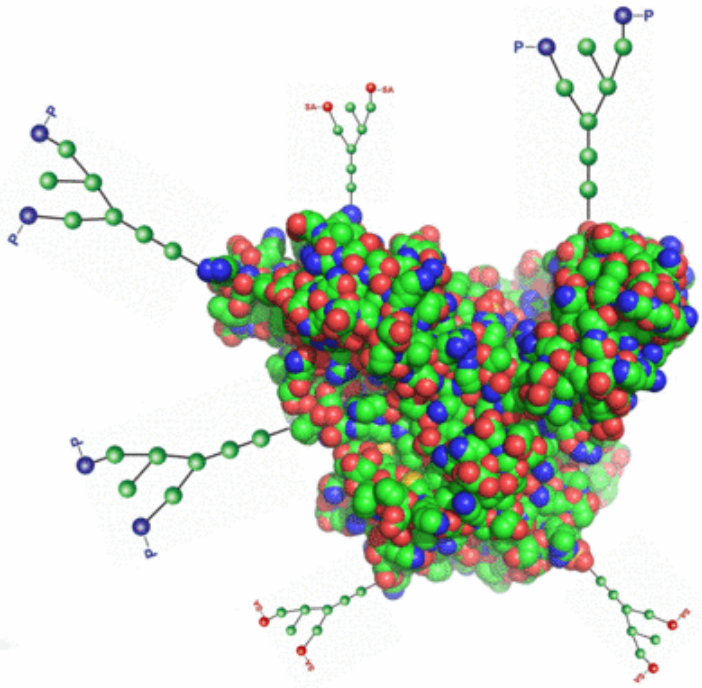
ATB200 + Chaperone: A Highly Differentiated Approach

Novel Pompe Treatment Paradigm with Three Key Differentiators

ATB200 (Novel ERT)

CHART
CHAPERONE-ADVANCED REPLACEMENT THERAPY

Chaperone addition



Optimized mixture of glycans

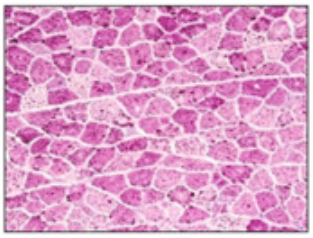
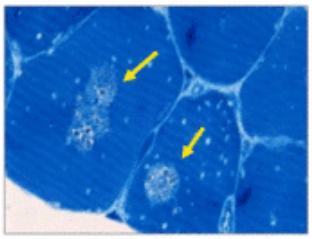
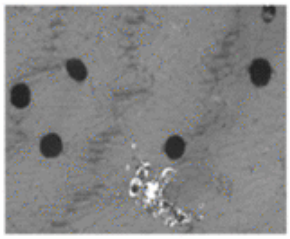
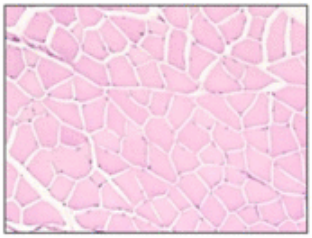
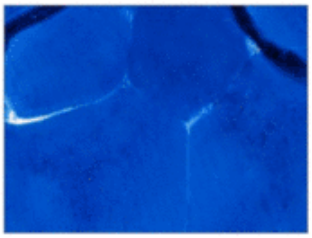
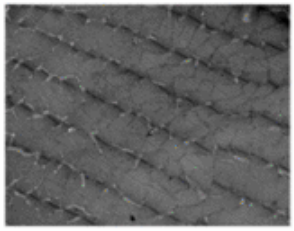
High levels of M6P and bis M6P

*Artist rendering, not actual product image

Amicus Therapeutics

Lysosomal Glycogen Accumulation and Muscle Weakness

Understanding Why a Metabolic Disease Leads to Profound Muscle Weakness

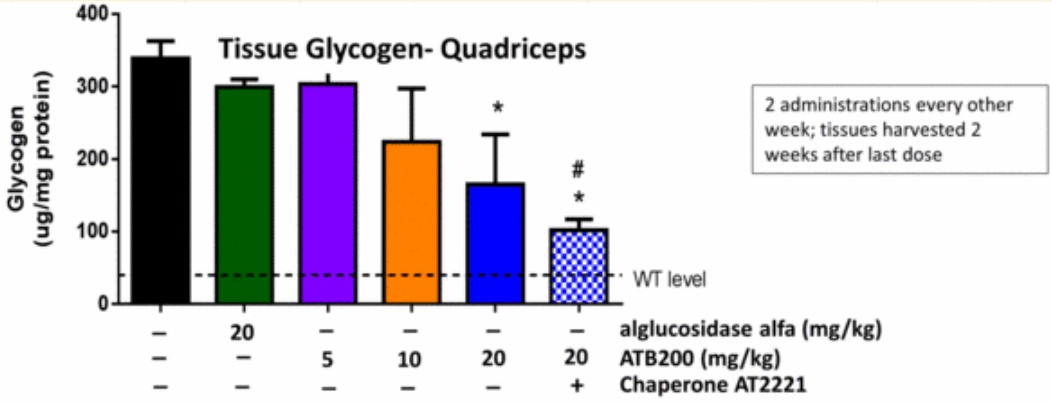
	Glycogen Accumulation (PAS staining)	Cellular Dysfunction (methylene blue staining)	Damaged Cytoarchitecture (electron microscopy)
***Gaa* KO mice**			
wild type mice (normal)			

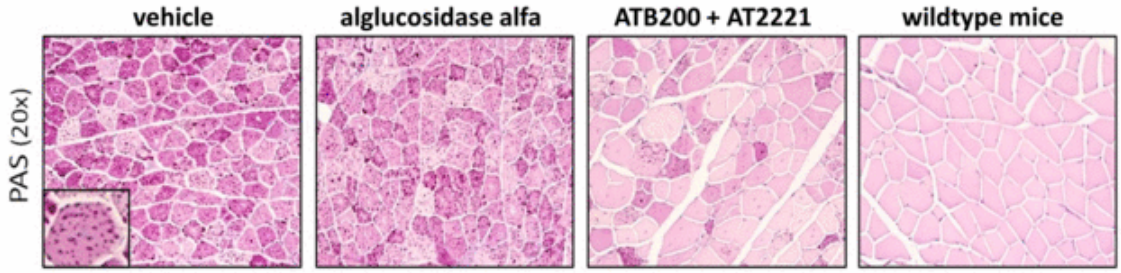
- Gaa deficiency leads to:
  - accumulation of glycogen in lysosomes of muscles
  - significant cellular dysfunction that alters protein trafficking for key muscle proteins
  - Incomplete assembly of critical protein complexes that alters cytoarchitecture and compromises muscle membrane integrity and stability

Amicus Therapeutics

Substrate Clearance & Cellular Physiology

ATB200/AT2221 Improved Substrate Clearance and Cellular Physiology in Preclinical Models[1]



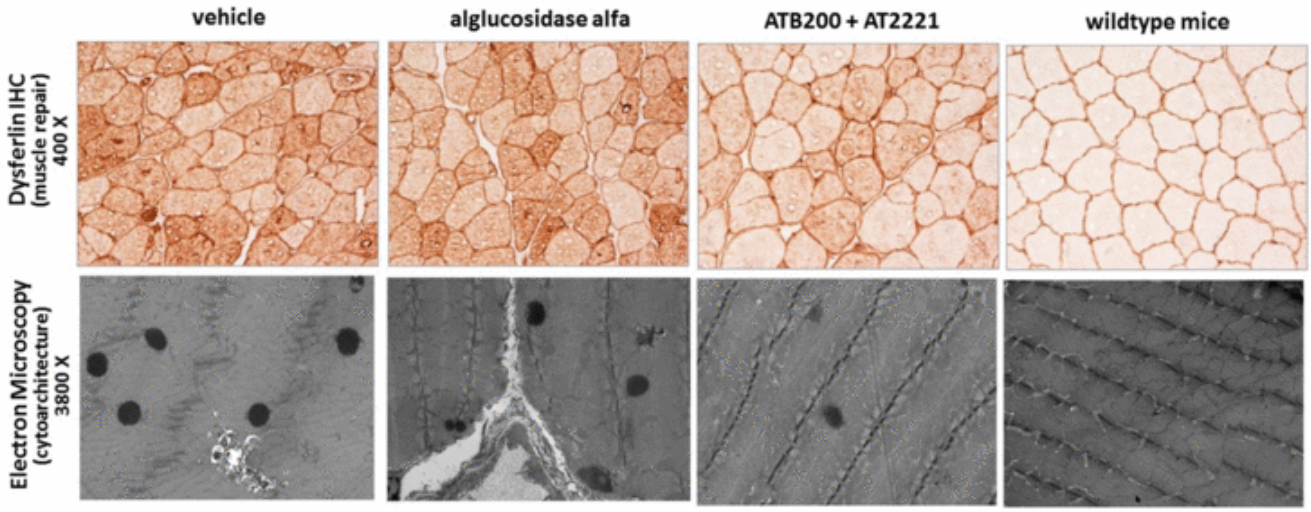


1. Following 2 doses of 20mg/kg alglucosidase alfa or ATB200 + AT2221 in Gaa KO mice, skeletal muscles evaluated for glycogen clearance and proliferated lysosomes. Treatment with alglucosidase alfa modestly reduced glycogen or proliferated lysosomes while ATB200, co-administered with AT2221 significantly decreased the muscle pathology associated with Pompe disease.

Amicus Therapeutics

Cellular Repair & Muscle Strength

ATB200/AT2221 Reversed Cellular Damage to Enable Muscle Repair and Improve Muscle Cytoarchitecture[1]

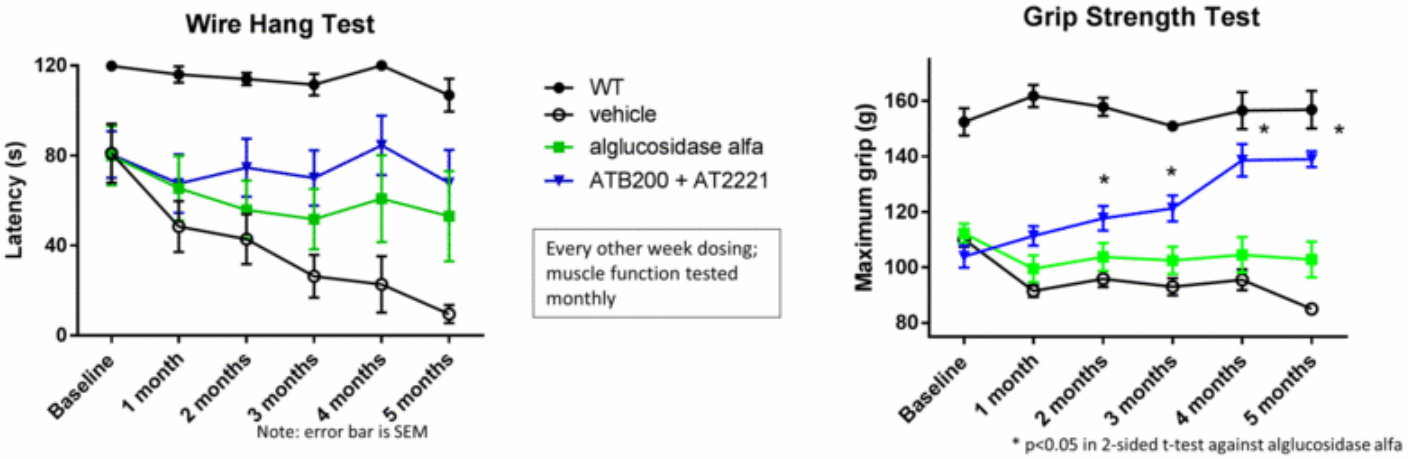


1. Following 2 doses of 20mg/kg alglucosidase alfa or ATB200 + AT2221 in Gaa KO mice, skeletal muscles evaluated for glycogen clearance and proliferated lysosomes. Treatment with alglucosidase alfa modestly reduced glycogen or proliferated lysosomes while ATB200, co-administered with AT2221 significantly decreased the muscle pathology associated with Pompe disease.

Amicus Therapeutics

Functional Muscle Strength

ATB200/AT2221 Progressively Increased Muscle Function and Appears to Induce Muscle Repair and/or Regeneration Rather than Just Maintenance of Damaged Muscle



Amicus Therapeutics

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)

18-Week Primary Treatment Period with Long-Term Extension (n ~20)

Cohort 1 (Ambulatory ERT-Switch)

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) & Cohort 3 (ERT-Naive)

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

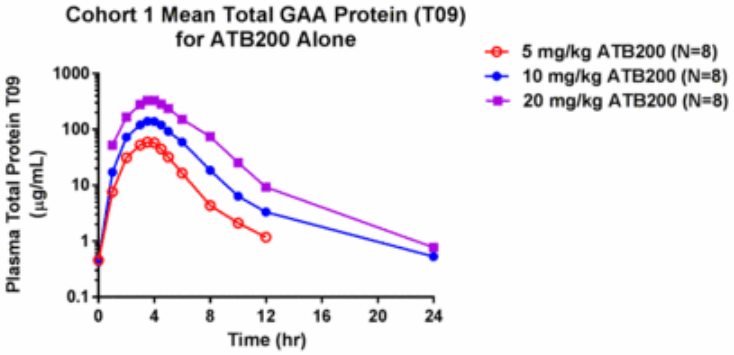
Assessments:

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

Pharmacokinetics in ERT-experienced patients (Cohort 1 N=8)*

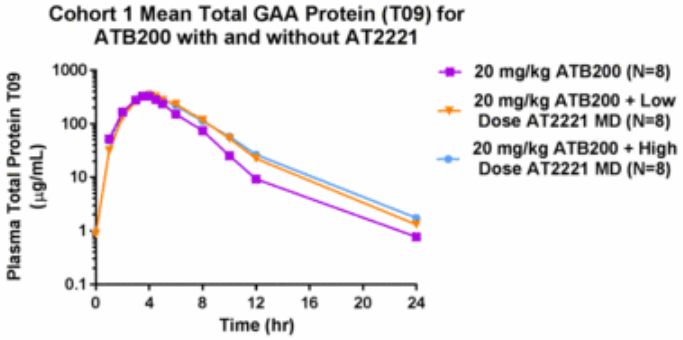
ATB200 Clinical PK Profile as Predicted Based on Preclinical Studies with Greater than Dose Proportional Increases in Exposure that were Enhanced by AT2221

Mean GAA Total Protein (n=8)
5, 10, 20 mg/kg ATB200 Alone



Treatment	Mean $AUC_{0-\infty}$ (hr*µg/ml)	Mean Half-Life (hr)
5 mg/kg	218	1.1
10 mg/kg	584	1.3
20 mg/kg	1512	1.5

Mean GAA Total Protein (n=8)
20 mg/kg ATB200 + AT2221



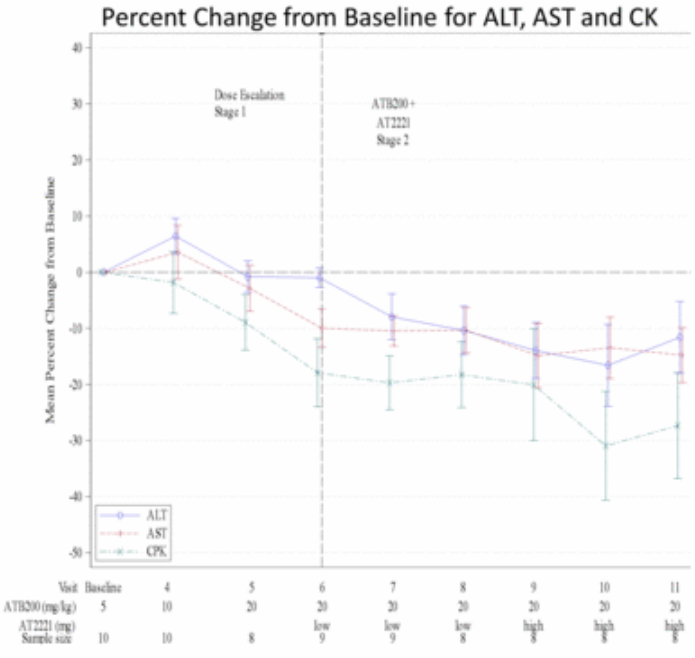
Treatment	Mean $AUC_{0-\infty}$ (hr*µg/ml)	Mean Half-Life (hr)
20 mg/kg	1512	1.5
+low dose AT2221	1808	1.9
+high dose AT2221	1901	2.3

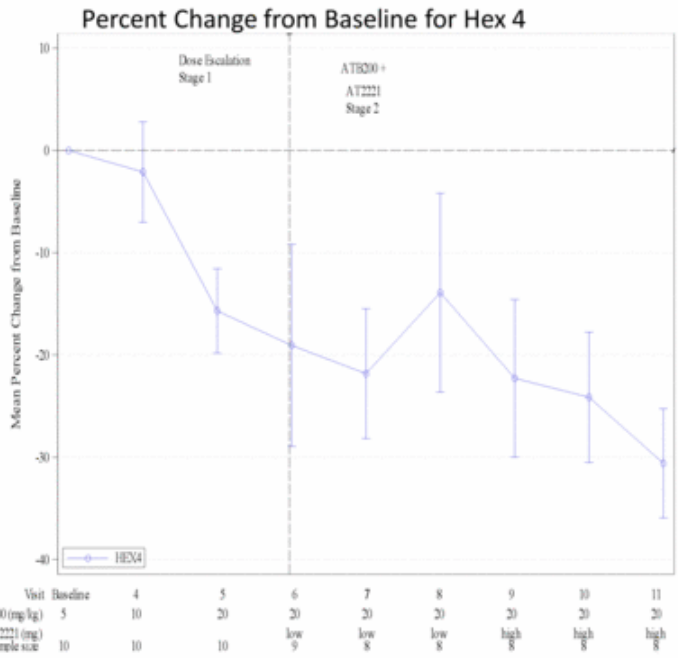
2 patients excluded that did not have full PK data at time of data cut

Amicus Therapeutics

Cohort 1: Biomarkers at Week 18 (N=8)

After Switching from SOC to ATB200/A2221 Patients Generally Demonstrated an Improvement in Biomarkers of Muscle Damage (ALT, AST, CK) and Biomarker of Disease Substrate (Hex4)

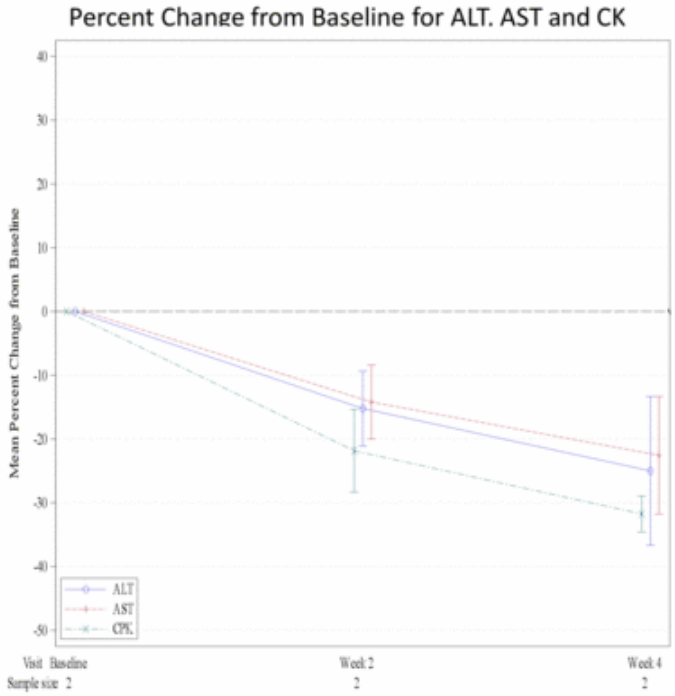


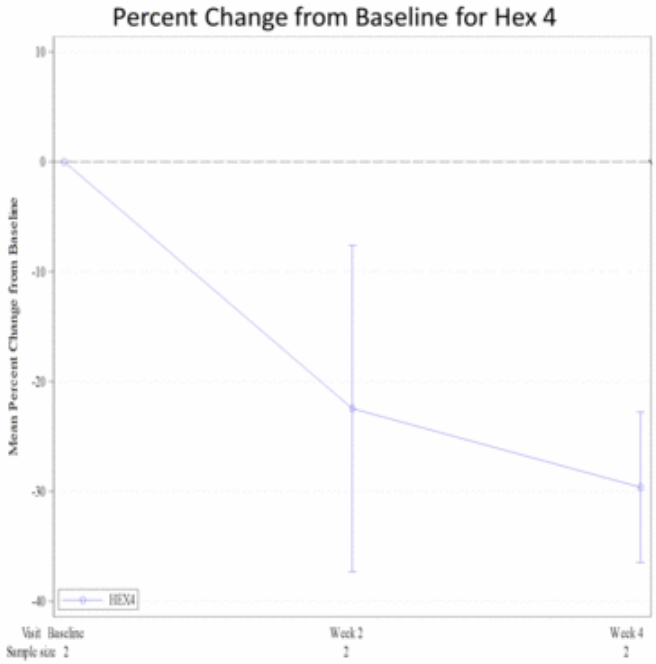


Amicus Therapeutics

Cohort 3: Biomarkers at Week 4 (N=2)

Initial Two Naïve Patients Treated with ATB200/AT2221 Demonstrated Robust Reduction in Biomarkers of Muscle Damage (ALT, AST, CK) and Biomarker of Disease Substrate (Hex4)





Amicus Therapeutics

Muscle Damage Biomarkers and Urine Hex 4 (N=10)*

- In ERT-switch patients (Week 18):
  - ALT decreased in 5 of 8 patients; 4/4 patients with elevated baseline levels normalized
  - AST decreased in 6 of 8 patients; 3/4 patients with elevated baseline levels normalized
  - CK decreased in 6 of 8 patients; 2/6 patients with elevated baseline levels normalized
  - ALT, AST, CK generally remained stable for patients not demonstrating a decrease
  - Urine Hexose Tetrasaccharide (Hex4) decreased in 8 of 8 patients; overall reduction approximately 30%

- In ERT- naïve patients (Week 4):
  - ALT, AST, CK and Urine Hex4 decreased in 2 of 2 patients.

**N=10 includes 8 switch and 2 naïve patients*

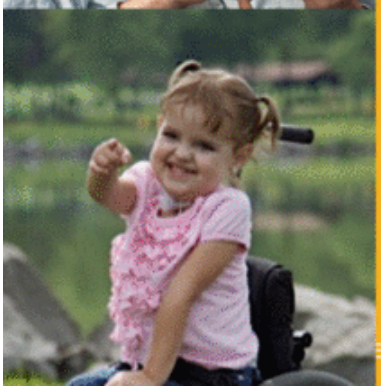
Preliminary Data Summary

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

- Safety (N=13)*
  - No serious adverse events (SAEs)
  - AEs were generally mild and transient
- Tolerability
  - No infusion-associated reactions following 150+ infusions in all patients enrolled to date
- PK (N=10)**
  - 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) and substrate biomarker (urine Hex4) (N=10)**
  - Decrease/normalization of muscle injury biomarkers and biomarker of substrate following a switch from SOC to ATB200/AT2220, and in ERT-naïve patients, suggests positive effect of the new therapy on muscle cells

*N=10 from Cohort 1 (Ambulatory ERT-Switch); N=1 from Cohort 2 (Non-Ambulatory ERT-Switch); N=2 from Cohort 3 (Naive)
**N=8 from Cohort 1 & N=2 from Cohort 3

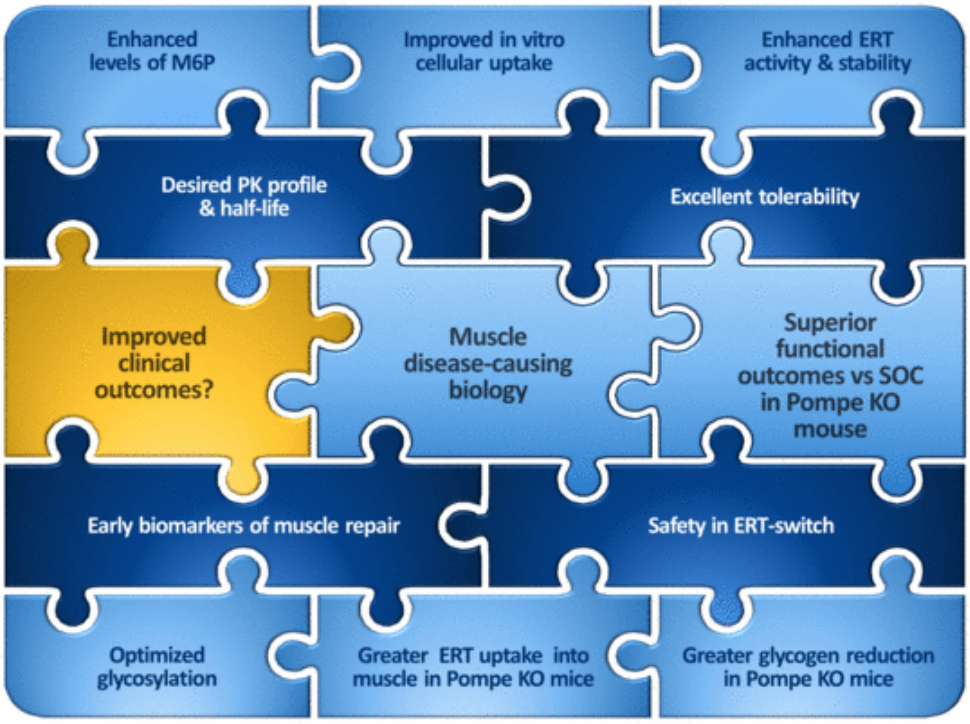
Amicus Therapeutics



Upcoming Pompe Milestones

Pompe Disease: A Complex Disease with Significant Unmet Needs

We've Made Great Strides and Expect to Address Key Remaining Questions in 2017



Amicus Therapeutics

Pompe Clinical Study ATB200-02 Data Cascade

A Cascade of Additional Data Points During 2017 to Demonstrate 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)
- Meeting with U.S. and EU regulators

18-WEEK DATA

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

EXTENSION DATA

- Motor/pulmonary function

Amicus Therapeutics

Biologics Manufacturing Capabilities

Highly Successful Biologics Manufacturing Scale-up in Three Years

Proprietary Process

Research Scale / MCB

5L (Bench Scale) 2013

250L (Clinical Scale) 2014-2015+

1000L (Registration Trial & Commercial Scale) 2016-2017+

Amicus Therapeutics

Thank You

Amicus Therapeutics®