

**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): **August 10, 2016**

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

Delaware
(State or Other Jurisdiction of
Incorporation)

001-33497
(Commission File Number)

71-0869350
(IRS Employer Identification No.)

1 Cedar Brook Drive, Cranbury, NJ
(Address of Principal Executive Offices)

08512
(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:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- 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 presentation attached as Exhibit 99.1 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.

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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.

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

Date: August 10, 2016

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EXHIBIT INDEX

Exhibit No.	Description
99.1	Presentation Materials



Corporate Overview

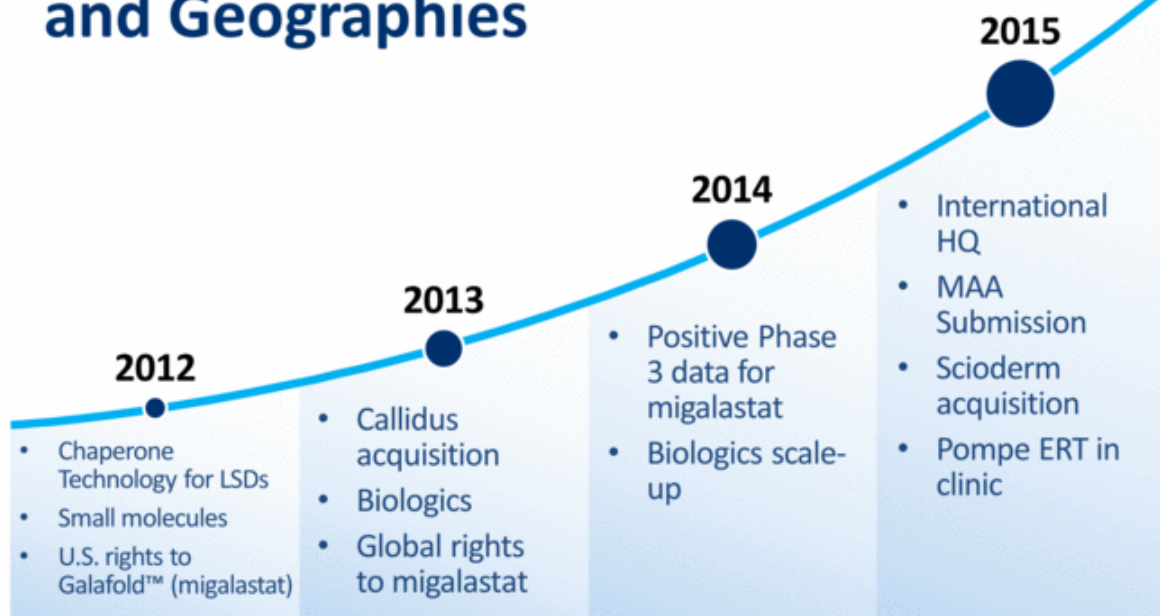
August 2016

Safe Harbor

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, financing plans, and the projected cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release 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. With respect to statements regarding projections of the Company's cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2015. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

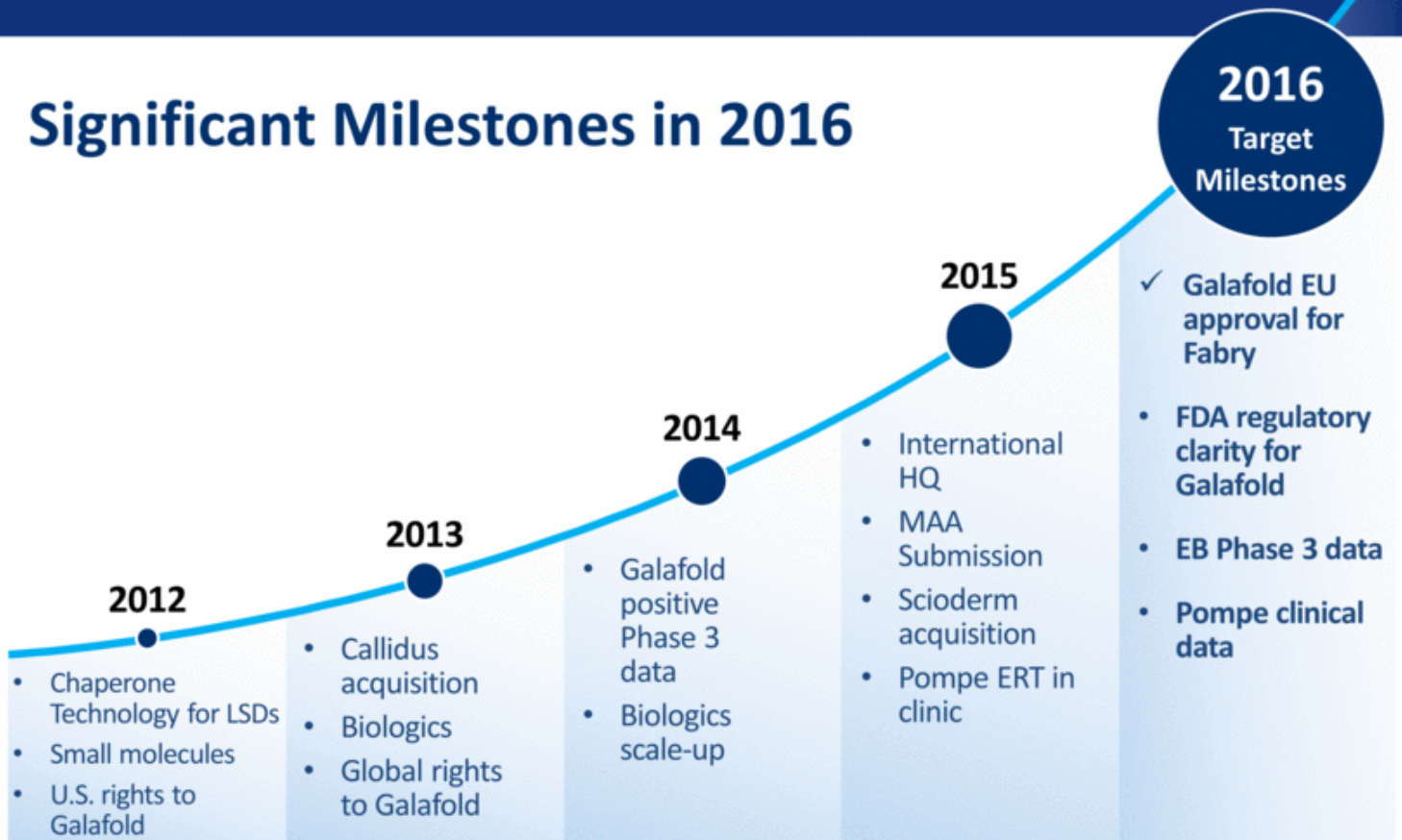
Amicus 2016 – Looking Back

Amicus Has Greatly Expanded Product Pipeline, Technologies and Geographies



Amicus 2016 – Continuing the Momentum

Significant Milestones in 2016



Amicus Vision

Amicus Therapeutics is a global biotechnology company at the forefront of developing advanced therapies to treat a range of devastating rare and orphan diseases

Rare &
Devastating
Diseases



Potential
First-in-Class
/ Best-in-
Class



Meaningful
Benefits for
Patients



Key Drivers of Value

3 Novel Product Candidates Each with \$500M to \$1B+ Market Potential

Fabry

- Galafold Precision Medicine (Small Molecule)
- EU Full Approval
- Launched in Germany (May 30, 2016)
- U.S. regulatory update anticipated 3Q16

Epidermolysis Bullosa (EB)

- Phase 3 Novel Topical Cream (SD-101)
- U.S. Breakthrough Therapy Designation
- Rolling NDA
- Phase 3 Data targeted in 4Q16/1H17

Pompe

- Novel ERT + Chaperone Treatment Paradigm
- Biologics Manufacturing
- Interim Data Anticipated in 4Q16

R&D Engine and Continued Business Development Activity



Galafold™
(Migalastat)
Precision Medicine
for Fabry Disease

European Commission Granted Full Approval for Galafold

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



Galafold™ (migalastat)

AMENABILITY TABLE

Full prescribing information: EN - English

AMENABILITY TABLE

Search GLA Mutations

You can use this search tool to find out whether a specific GLA mutation has been classified as amenable to treatment with GALAFOLD™ according to the approved SmPC.

GALAFOLD™ is indicated for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (a galactosidase α deficiency) and who have an amenable mutation.

Female patients have two GLA genes on two different chromosomes. The patient is considered amenable if the GLA mutations on either chromosome are amenable. Please utilize the appropriate search function to determine if the mutation or mutations on each chromosome are amenable.

AMENABILITY TABLE

PATIENT HAS SINGLE MUTATION

PATIENT HAS MULTIPLE MUTATIONS

Enter either a nucleotide or amino acid change.

For Nucleotide Change
Please use format c.AA-B or c.AAG for nucleotide sequence changes, where 'c' is optional. If indicates a number: A and B are letters. Examples: L123C or L23C.

For Amino Acid Change
Please use format p.AAG for protein sequence changes, where 'p' is optional. If indicates a number: A and B are letters. Example: pL23E

Download Amenable Reference Table
See the SmPC for full prescribing information.

Last Updated: 28 May 2016

Amicus Therapeutics | MFGAL01/002/02/17/15 | Galafold™ (migalastat)

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“The evaluation of EMA’s Committee for Medicinal Products for Human Use (CHMP) was based on the results of two phase III clinical trials in about 110 patients with Fabry disease who had a genetic mutation which responds to migalastat. Galafold demonstrated its efficacy compared to placebo (a dummy treatment) and to ERT in a long-term comparative study.

- EMA Press Release

Fabry Disease Overview

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

Leading Causes of Death

TRANSIENT ISCHEMIC ATTACK (TIA) & STROKE¹

HEART DISEASE²

- Irregular heartbeat (fast or slow)
- Heart attack or heart failure
- Enlarged heart

KIDNEY DISEASE³

- Protein in the urine
- Decreased kidney function
- Kidney failure

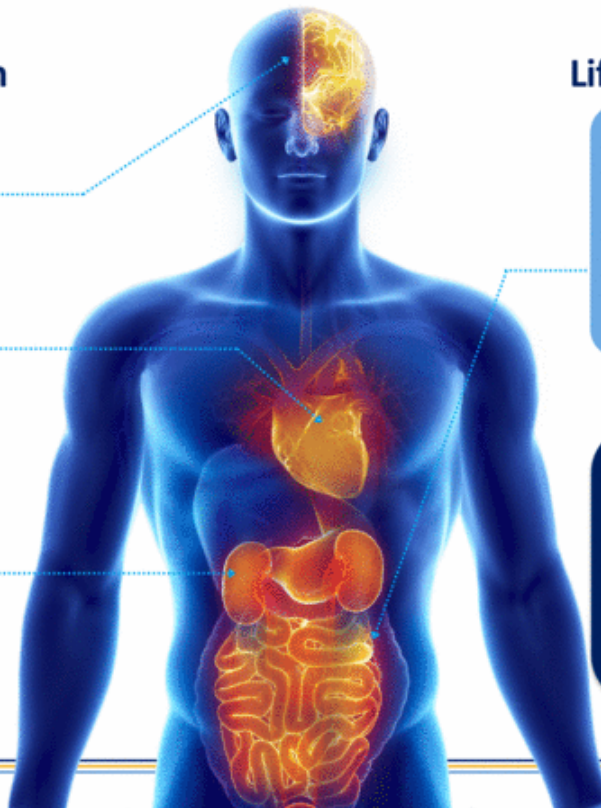
Life-Limiting Symptoms

GASTROINTESTINAL³

- 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
- >800 known mutations
- 5-10K diagnosed WW (51% female/49% male⁴)
- 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

Summary of Clinical Data

Favorable Efficacy and Safety Data in Two Largest Phase 3 Studies Ever Completed in Fabry Disease



Reduction in Disease Substrate

IC GL-3 (Study 011¹)*
Plasma Lyso Gb-3 (Study 011^{2,1} and 012³)*

Stability of Kidney Function

Estimated Glomerular Filtration Rate (eGFR) and Measured GFR
(Study 011⁴ and Study 012^{4,3})

Reduction in Cardiac Mass

Left Ventricular Mass Index (LVMI) (Study 011² and 012)*

Improvement in GI Symptoms

Gastrointestinal Symptoms Rating Scale (GSRS) (Study 011¹)*

Low Rate of Fabry-Associated Clinical Events

Renal, Cardiac and Cerebro-Vascular Events (Study 012³)

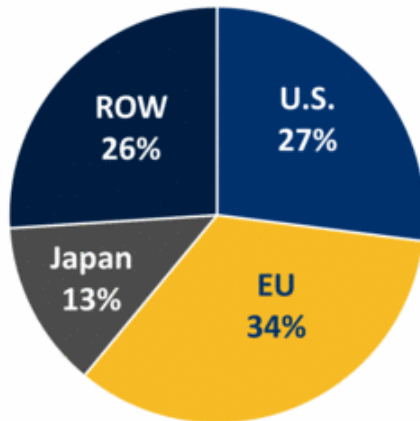
- 1: Improvement versus placebo over 6 months in amenable patients
- 2: Improvement from baseline over 18+ months
- 3: Comparable to ERT over 18 months
- 4: Stabilization from baseline over 18 months with favorable comparison to natural history in literature

*Analyses in this endpoint achieved statistical significance. For more complete clinical data go to amicusrx.com/posters.aspx

Galafold Commercial Opportunity

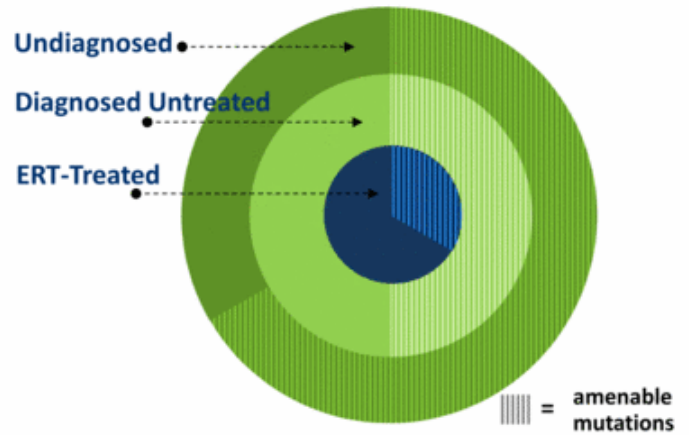
Amicus is Prioritizing EU, Japan, US and Other Large Fabry Markets for Initial Launch
Go To Market Strategy to Address 35%-50% of Patients with Amenable Mutations

Geographic Segments



- \$1.2B in FY15 ERT Sales¹
- Market Continues to Grow > 10% / Year
- ERT Infused Once Every 2 Weeks

Patient Segments



- 5k-10k Patients Diagnosed WW
- 40%-50% of Diagnosed Patients not on ERT
- Newborn screening studies suggest prevalence of ~1:1000 to ~1:400²

1. Company filings and Amicus estimates 2. 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

International Launch Update

EU Market Represents 34% of FY15 ERT Global Sales (\$1.2B)

GERMANY

ERT-treated patients : ~500 patients
~50% of diagnosed patients untreated
Galafold launched – initial patients on treatment

FRANCE

ERT-treated patients : ~375 patients
Multiple patients treated under ATU

UNITED KINGDOM

ERT-treated patients: ~450
Highly Specialised Technology (HST)



EU Launch Update

Successful Early Days of EU Launch with Naïve and Switch Patients on Galafold – Focusing on Patient Access and Country-by-Country Reimbursement Processes



LAUNCH IN GERMANY = IMPORTANT INDICATOR

- First Galafold Rx within 24 hours of EC approval
- Patient support program
- Experienced, high quality team
- Pricing dossier submitted

21

patients (switch & naïve) on reimbursed Galafold WW (7/31/16)

12

countries with active pricing discussions

3

countries with reimbursement (commercial and EAP)

Global Regulatory Strategy

Prioritizing Global Regulatory Submissions in Key Markets (US and Japan) with Additional Submissions Completed or Planned Based on EU Approval (MAA)



Amicus Proprietary Fabry ERT

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

Target Fabry ERT product profile:

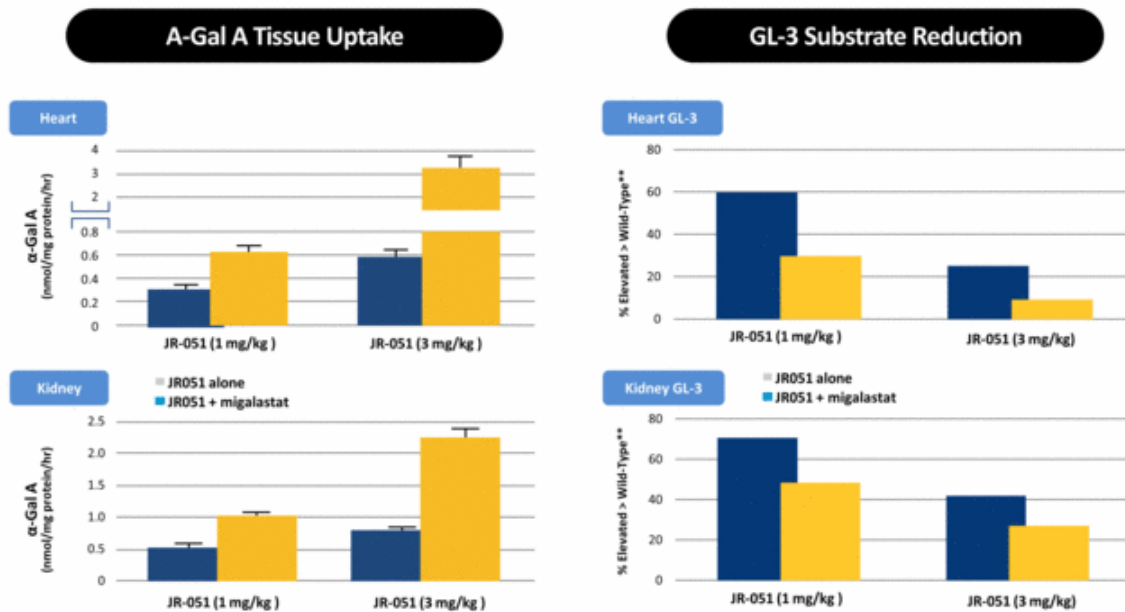
- Improved drug targeting
- Co-formulation with chaperone

Development status:

- Cell line transferred to manufacturer
- Preclinical data update in 2H16

CHART Preclinical Proof-of-Concept for Fabry Co-Formulation

Co-Formulation (ERT + Migalastat) Results in Significantly Greater Tissue Uptake and Further Substrate Reduction*



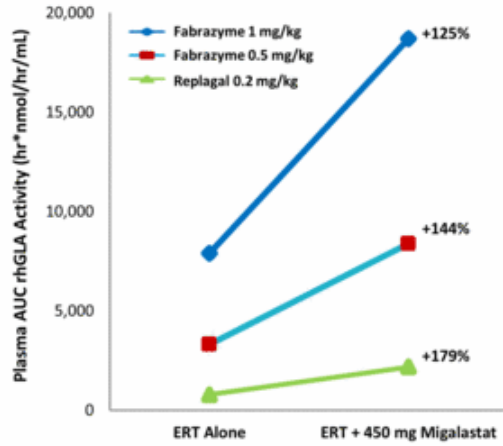
*ERT +/- Migalastat HCl in GLA Knock-Out Mice (Repeat-Dose IV Administration)

**ERT designed to be biosimilar to Fabrazyme; **0 = wild-type, 100 = untreated KO mouse

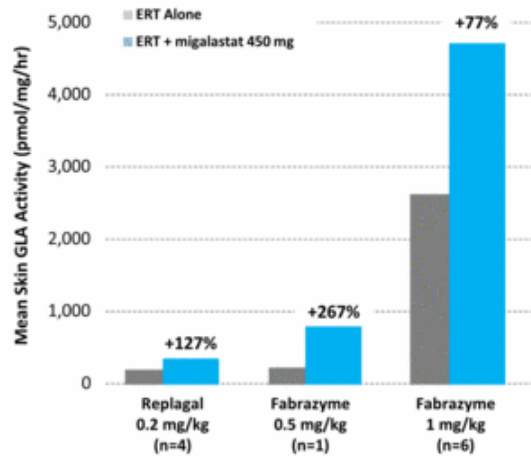
CHART Phase 2a Results for Fabry Co-Administration Study 013

Co-Administration with Fabrazyme or Replagal Leads to Consistent Increases in Active Plasma Enzyme Levels and Tissue Uptake¹

Plasma rhGLA Activity (Area Under Curve)



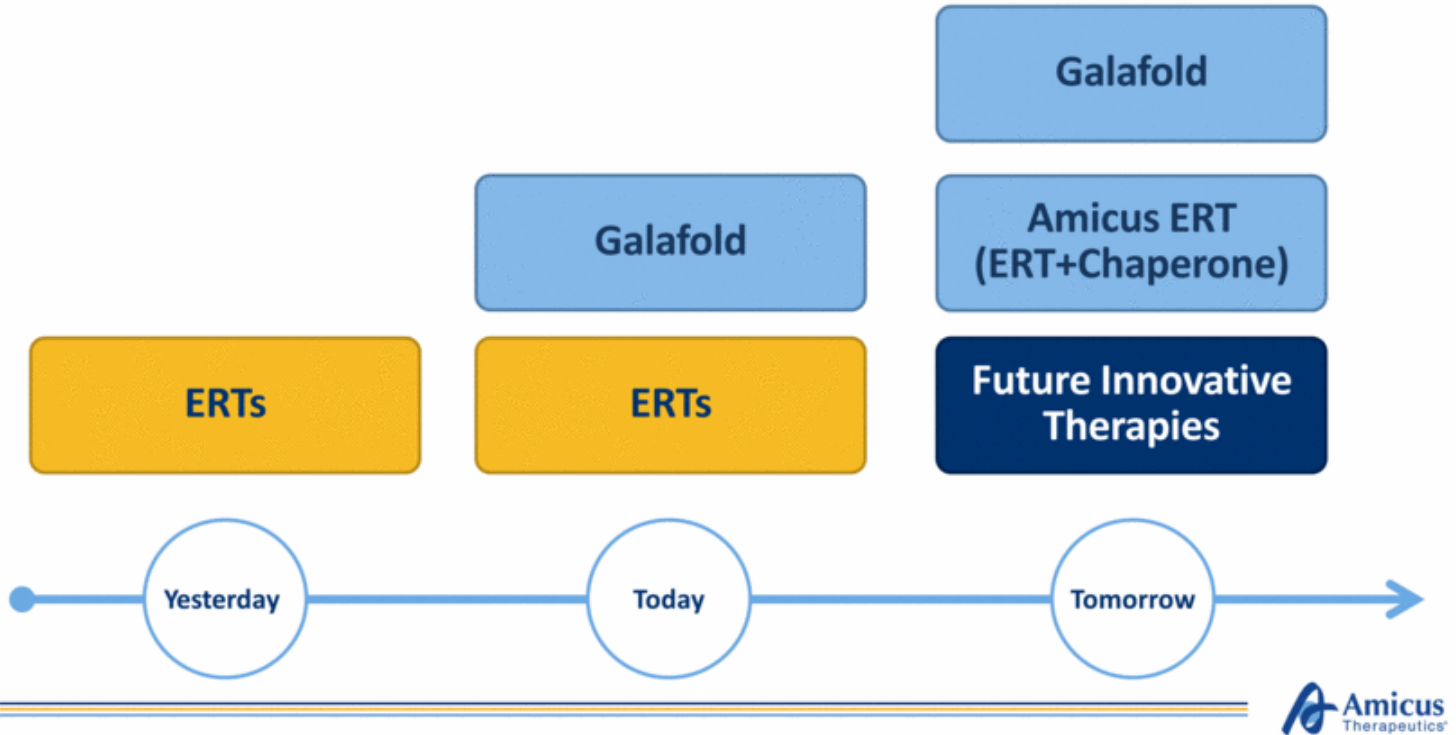
Mean Skin GLA Activity (Day 2)

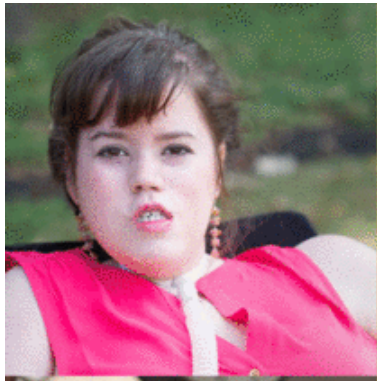


¹ Bichet, et al., A Phase 2a Study to Investigate the Effect of a Single Dose of Migalastat HCl, a Pharmacological Chaperone, on Agalsidase Activity in Subjects with Fabry Disease, LDN WORLD 2013.

Fabry Franchise Strategy

Amicus Therapeutics is Committed to Delivering the Highest Quality Therapies and Future Innovation to Find a Cure for ALL Fabry Patients





ATB200 Novel ERT for Pompe Disease

A Proprietary, Clinical-Stage Biologics Program

Pompe Disease Overview

Severe, Fatal, Genetic Disorder with Significant Unmet Medical Need



- Deficiency of GAA leading to glycogen accumulation
- Age of onset ranges from infancy to adulthood
- Symptoms include muscle weakness, respiratory failure, and cardiomyopathy
- Respiratory and cardiac failure are leading causes of morbidity and mortality
- 5,000 – 10,000 patients diagnosed WW¹
- ~\$800M+ Global Pompe ERT sales in FY15²

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

Pompe ERT - 3 Challenges

Amicus Technology Platforms with Potential to Address Challenges with Existing Pompe ERT

Activity/ Stability

Rapid denaturation of ERT in pH of blood¹

Protein Aggregation



Tolerability / Immunogenicity

Infusion-associated reactions in >50% of late-onset patients³

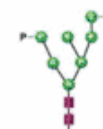
Antibody titers shown to affect treatment outcomes^{4,5}



Uptake/ Targeting

Low M6P receptor uptake into skeletal muscle²

Vast majority of rhGAA not delivered to lysosomes²



Uniquely Engineered rhGAA Optimized M6P & Carbohydrates

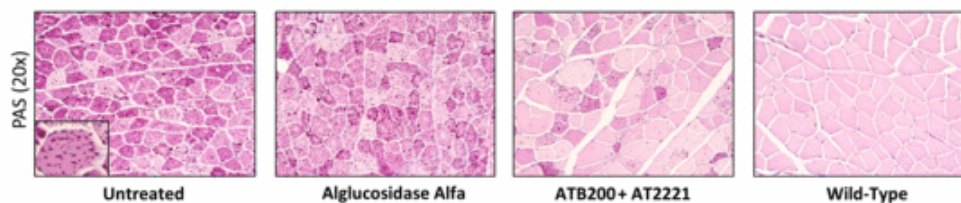
¹Khanna et al., PLoS ONE, 2012; ²Zhu et al., Amer. Soc. Gene Therapy, 2009 June; ³Banati et al., Muscle Nerve, 2011 Dec.; ⁴Banugaria et al., Gen. Med., 2011 Aug.; ⁵de Vries et al., Mol Genet Metab., 2010 Dec.



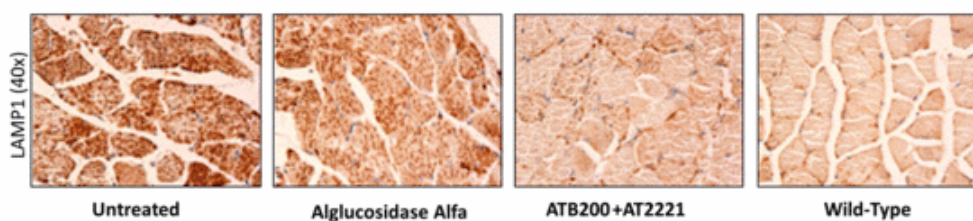
Preclinical Proof of Concept

ATB200 + Chaperone Results in Improved Substrate Clearance in Preclinical Models¹

PAS-glycogen staining in Quadriceps



LAMP1 Immunohistochemical staining in Soleus

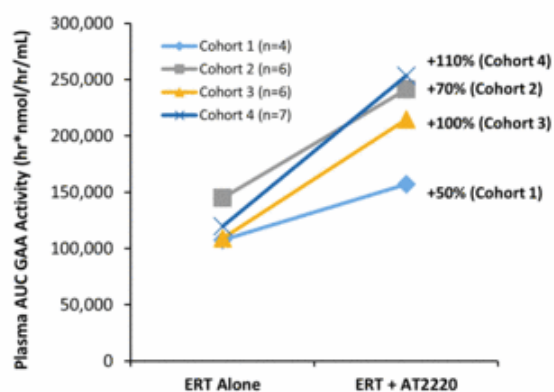


1. Following 2 doses of 20mg/kg alglucosidase alfa or ATB200 + AT2221 in Gaa KO mice, skeletal muscles evaluated for glycogen clearance and proliferated lysosomes. Treatment with alglucosidase alfa modestly reduced glycogen or proliferated lysosomes while ATB200, co-administered with AT2221 significantly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

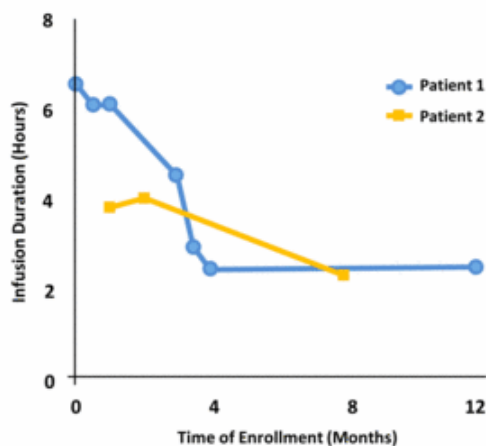
Human Proof-of-Concept: Currently Marketed ERT + Chaperones

ERT Activity Increased and Infusion Time Decreased with ERT + Chaperone

**Amicus Phase 2 Study 010
Enzyme Activity¹**



**Investigator-Initiated Study
Infusion Time²**



¹ Kishnani, et al., LDN WORLD 2013

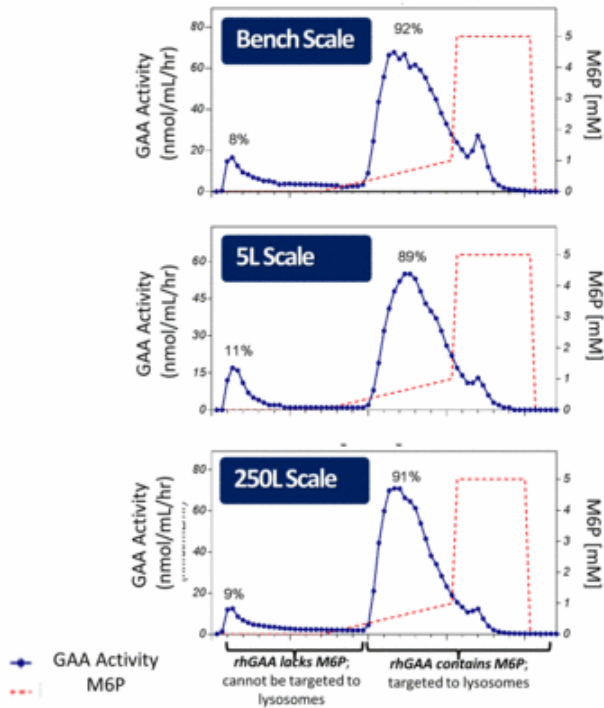
² Doerfler, et al. WORLD 2014

* Cohort 1 (AT2220 50 mg) muscle GAA activity not shown; 50 mg dose did not demonstrate meaningful change in tissue uptake (muscle)

Biologics Manufacturing Capabilities

Optimized Glycosylation and Key Quality Attributes Maintained Through Scale Up

CI-MPR Receptor Chromatography

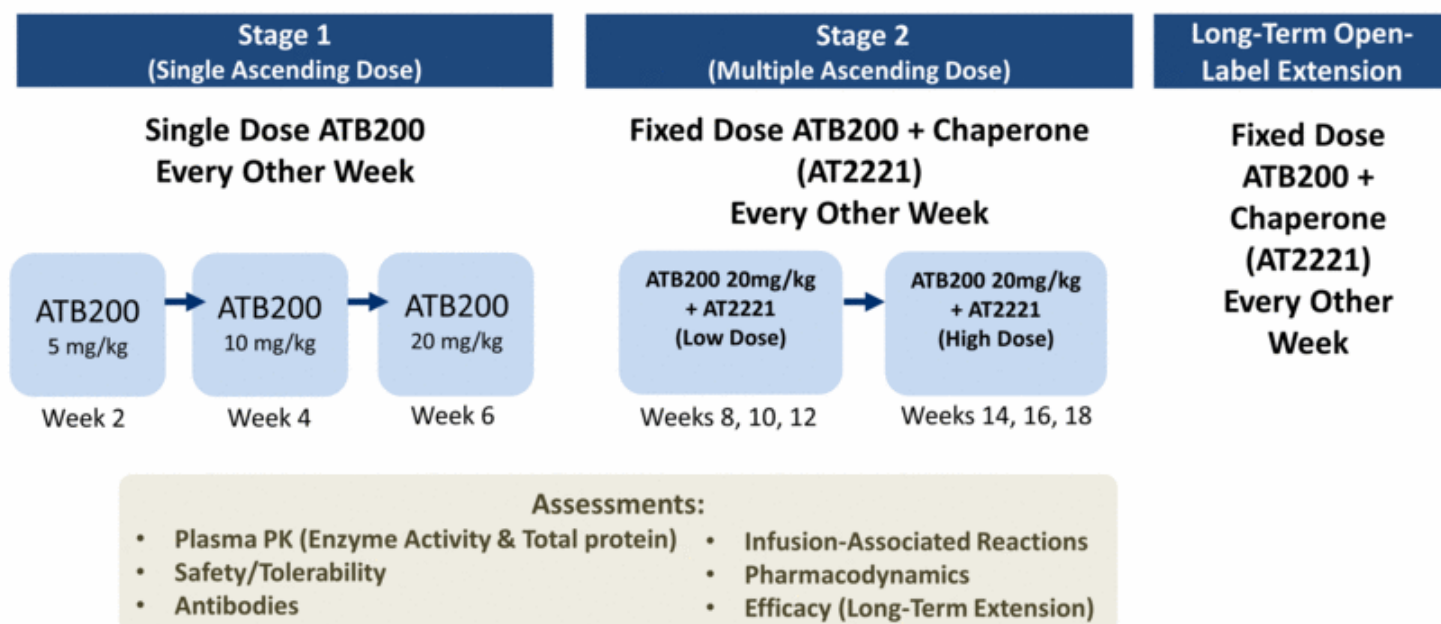


Lyophilized Vial of ATB200



Clinical Study in Pompe Patients

Patient Dosing Underway and Enrollment Ongoing at Multiple Sites



Pompe Clinical Study ATB200-02 Data Cascade

A Cascade of Data Points from 4Q16 through 2017 Offer Clear Parameters to Define Success and Differentiate ATB200/AT2221

Pompe Data Cascade 4Q16 Through 2017

Data in initial ambulatory ERT-switch patients (N=~4, Cohort 1)

Additional data & initial extension data in Cohort 1

Data in ERT-naïve patients (Cohort 2)

Data in non-ambulatory ERT-switch patients (Cohort 3)

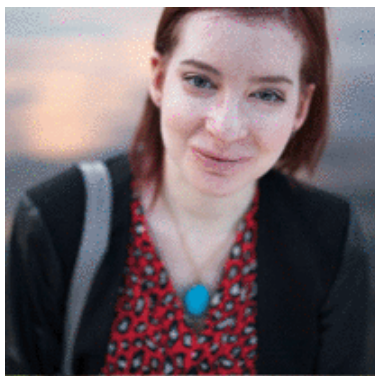
Additional extension study data (all Cohorts)

18-WEEK DATA

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

EXTENSION DATA

- Motor/pulmonary function



SD-101 for Epidermolysis Bullosa (EB)

Poised to deliver pivotal data for a
devastating rare disease

EB Disease Overview

Rare, Devastating, Connective Tissue Disorder with No Approved Treatments



- Multiple genes cause disease which results in fragility of skin and can affect internal organs
- Diagnosed from infancy to adulthood
- Severe blistering, open wounds, and scarring in response to minor friction to the skin
- Disfiguring, excruciatingly painful, and can be fatal
- Given the lack of approved treatment options, any reduction in disease symptoms would be considered meaningful
- 30,000 – 40,000 diagnosed patients in major global regions

Three Major EB Types Represent ~99% of EB Population

Multiple Types...Single Devastating and Fatal Genetic Disorder

Simplex



~75% of EB Population

Dystrophic



~20% of EB Population

Junctional



~5% of EB Population

INCREASING SEVERITY

No Approved Therapies Today

SD-101 in Development for All 3 Major Types

30,000 - 40,000 Diagnosed in Major Markets

U.S. Breakthrough Therapy Designation

Positive Early Results from Phase 2a Study Led to Breakthrough Therapy Designation

- Open-label, 8-patient proof of concept study¹
- Ages 6 months – 9 years
- All baseline target wounds $\geq 10 \text{ cm}^2$
- SD-101 3% applied once daily for 3 months

Key Findings

87.5%

of patients experienced complete closure of target wounds within 1 month

57%

reduction in affected body surface area by month 3

Daily administration generally safe and well-tolerated

1-Year-Old Girl with EB Simplex



Baseline



Following 2 months of treatment

1. Simplex (n=3), Junctional (n=3), Dystrophic (n=2)

Phase 2b Design (Study 003)

3-Month, Double-Blind Treatment Period¹

SD-101 6% (n=15)

SD-101 3% (n=16)

Placebo (n=17)

Optional Extension (SD-004)

Open-Label SD-101 6%

42/44 Patients entered extension study

\$400K FDA Grant for Extension Study

Primary Efficacy Endpoint: Target Wound Healing at Month 1

- Baseline wound: Chronic (≥ 21 days), size 5-50 cm²

Secondary Efficacy Endpoints Include:

- Time to target wound closure
- Change in Body Surface Area (BSA) of lesional skin

48 EB patients (age ≥ 6 months)¹ - 1:1:1 Randomization - Daily Topical Application

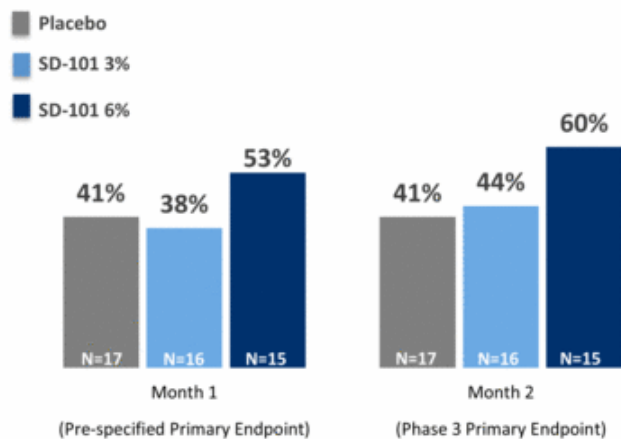
1. Assessments: 0, 14, 30, 60, 90 Days. 2. Initial Disease Severity: Mean target lesion size (cm²) 14.0 (range 5-39); mean lesional BSA: 19.4% (range 0.4-48%); mean wound age (days): 182 (range 21-1,639). EB types enrolled: Simplex (n=11), Dystrophic (n=29), and Junctional (n=8)

Phase 2b Results

SD-101 6% Demonstrated Higher Proportion of Complete Target Wound Closure

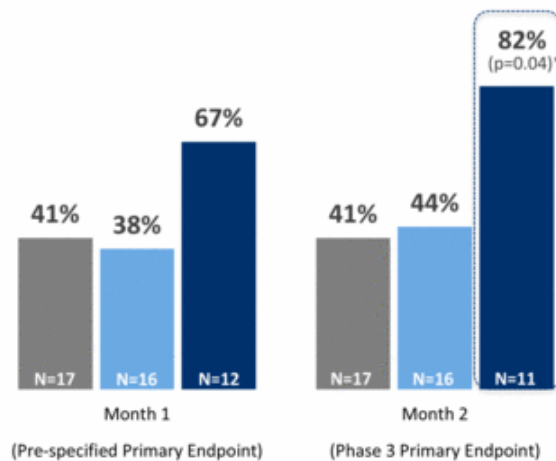
ITT Population (n=48)

Proportion of Complete Target Wound Closure (%)



Evaluable Population¹ (n=45)

Proportion of Complete Target Wound Closure (%)

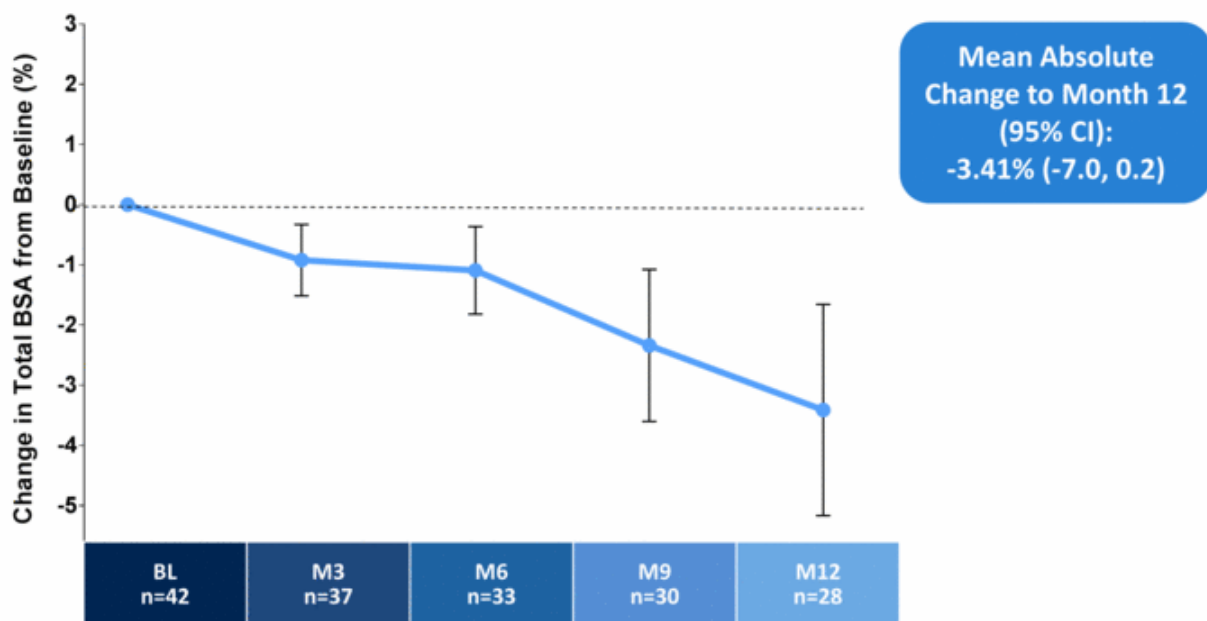


*SD-101 6% vs placebo, unadjusted p=0.04

1. Excluded from Evaluable population: 1 patient (due to lost to follow-up), 2 patients (did not have single identified and qualified target lesion). 1 additional patient lost to follow up after Month 1 visit and is excluded from target wound assessment at later time points

Phase 2b Extension (Study 004) Results

Results on Total Body Surface Area (BSA) Affected by Wounds and Lesions



Note: Mean and SEM on change from baseline are plotted. Study 004 Total BSA baseline values are: N=42 Baseline population: 11.3. N=28 population used for Month 12 comparison: 10.9

EB Program Update - Phase 3 ESSENCE Study (SD-005)

Following Recent Meeting with FDA, Amicus has Elevated Time to Wound Closure From Secondary to Co-Primary Endpoint. We Believe This Change Improves the Overall Likelihood of Study Success while ESSENCE Study Remains Blinded.



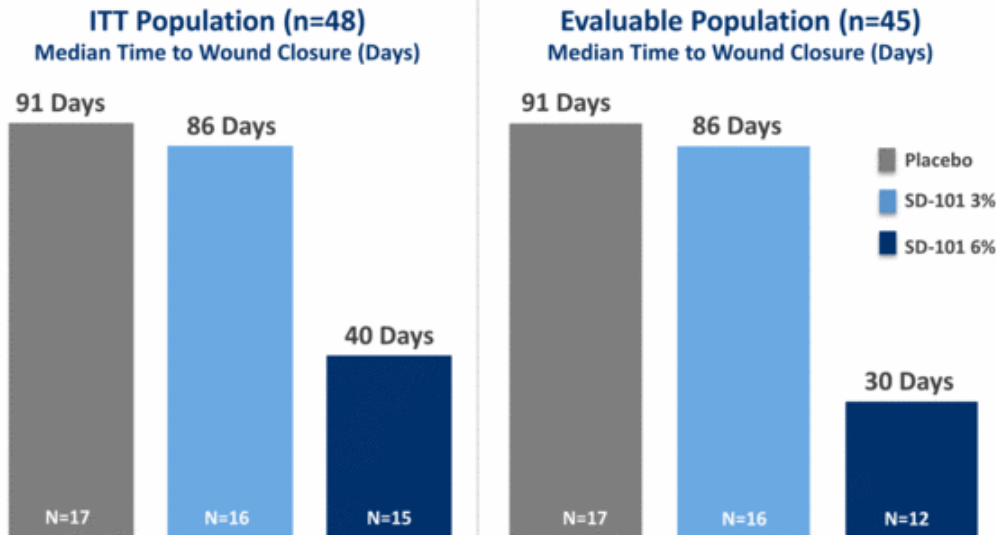
PHASE 3 ESSENCE STUDY STATUS

- >50% of target enrollment achieved
- 100% conversion to extension study (SD-006)
- Top-line Phase 3 data anticipated 4Q16/1H17

Elevation of Time to Wound Closure as Co-primary Endpoint

FDA 2006 Guidance Document¹ States Time to Wound Closure is an Acceptable Primary Efficacy Endpoint

Median Time to Wound Closure in Phase 2b Study



Time to Wound Closure

- Encouraging results in SD-101 Phase 2b study
- Measuring healing over time vs. one time point may further control for placebo response
- Results correlate with incidence of complete wound closure
- Statistical simulations indicate addition of time to wound closure increases probability of study success

¹<http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm071324.pdf>

Phase 3 ESSENCE Study Design (SD-005)

Study Success Potentially Based on Achievement of One or Both Co-Primary Endpoints

3-Month, Double-Blind Treatment Period

SD-101 6%

~150 EB patients (age \geq 1 month)

Baseline wound: Chronic (\geq 21 days), size \geq 10 cm²

Placebo

Optional Extension (SD-006)

Open-Label SD-101 6%

100% Participation in
Extension Study
(August 1, 2016)

Average Baseline Target
Wound Size in Phase 3
Population: **~20 cm²**
(August 1, 2016)

Co-Primary Endpoints

- Complete closure of target wound (previously specified primary endpoint)
- Time to target wound closure (elevated from secondary to co-primary)

Secondary Endpoints Include:

- Change in Body Surface Area (BSA) of lesions and blisters
- Patient-reported itching
- Patient-reported pain

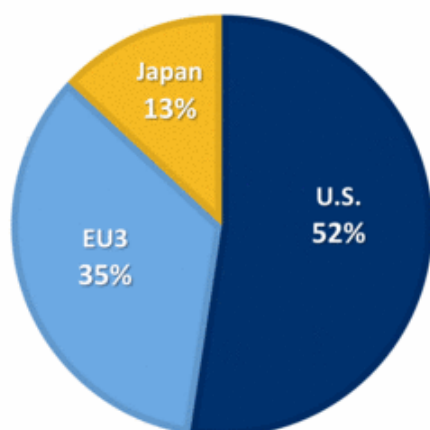
Covariates include age of patient and size of wound at baseline

\$1B+ Commercial Potential

KOL Feedback Supports Profound Unmet Medical Need and Broad Usage in All EB Types

Diagnosed EB Patients by Geography

(U.S., EU3, Japan)



Significant Unmet Clinical Need

- No approved treatments, opportunity for first-in-class
- Promising proof of concept in all EB types

Strong Support Among Surveyed Stakeholders

- Physicians indicate usage in 100% patients
- Payers indicate support for broad reimbursement if approved

Large Commercial Opportunity

- 30,000 – 40,000 diagnosed patients in major markets
- KOLs expect diagnosis rates to increase

Cyclin-Dependent Kinase-Like 5 (CDKL5) Deficiency

Rare, Devastating, Genetic Neurological Disease with No Approved Treatments



- 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 worldwide¹
- Patient identification rising significantly

1. LouLouFoundation.org

Strategic Fit with Amicus Vision and Biologics Pipeline

New CDKL5 Program Expands Biologics Pipeline and Fits with Our Vision to Build a Leading Global Biotechnology Company Focused on Rare and Devastating Diseases

CDKL5 is a rare, devastating genetic neurological disease with no approved treatment

Potential first-in-class CDKL5 protein replacement therapy expands biologics pipeline

Partnering with CDKL5 community to raise awareness and advance toward treatment

"This CDKL5 program is an important investment in our stated strategy to expand our biologics pipeline by integrating new, innovative technologies to develop first- and best-in-class therapies for patients who are in desperate need of new treatments."

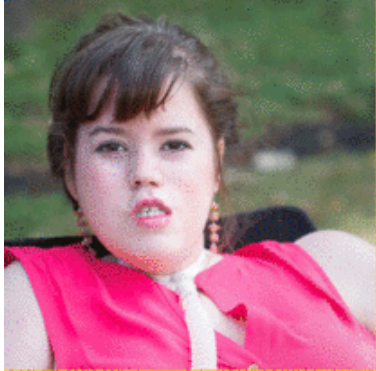
-John F. Crowley, Chairman and CEO of Amicus

"I am confident the Company's advancement of this program will raise CDKL5 awareness and, most importantly, increase the potential for success in developing a CDKL5 protein replacement therapy."

-Michael Jasulavic, Founder of MiaMed

"Today there is no approved treatment for people living with CDKL5 deficiency, and the number of patients diagnosed has been increasing rapidly..."

-Ashley R. Winslow, PhD, Director of Neurogenetics of the Orphan Disease Center at University of Pennsylvania



Financial Summary

Strong Balance Sheet to Invest in Rare Disease Pipeline

Strong Balance Sheet

Balance Sheet Strengthened with \$130M in Equity and Debt Proceeds Since March 31 with Cash Runway into 2H17

Financial Position	June 30, 2016
Cash:	\$214.2M
Debt	\$80.0M
FY16 Net Cash Spend Guidance:	\$135-\$155M (maintained)
Cash Runway	Into 2H17
Full Allotment Raised in ATM (average price per share: \$6.67)	\$100M (\$61.7M in 2Q; \$39.3M in 3Q)
Capitalization	
Shares Outstanding	134,408,526

Key Drivers of Value

3 Novel Product Candidates Each with \$500M to \$1B+ Market Potential

Fabry

- Galafold Precision Medicine (Small Molecule)
- EU Full Approval
- Launched in Germany (May 30, 2016)
- U.S. regulatory update anticipated 3Q16

Epidermolysis Bullosa (EB)

- Phase 3 Novel Topical Cream (SD-101)
- U.S. Breakthrough Therapy Designation
- Rolling NDA
- Phase 3 Data targeted in 4Q16/1H17

Pompe

- Novel ERT + Chaperone Treatment Paradigm
- Biologics Manufacturing
- Interim Data Anticipated in 4Q16

R&D Engine and Continued Business Development Activity

Thank You

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