

**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): **June 7, 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.

2

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: June 7, 2016

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

EXHIBIT INDEX

Exhibit No.	Description
99.1	Presentation Materials



Goldman Sachs 37th
Annual Healthcare
Conference

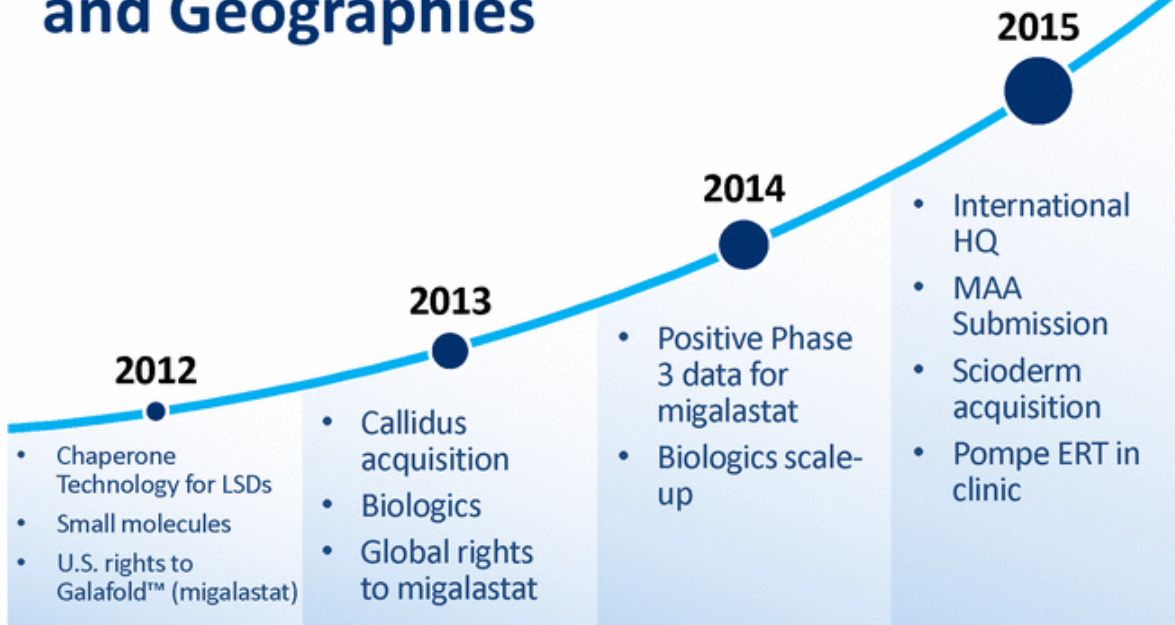
June 7, 2016

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 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 and the expected timing of the EMA's final decision with respect to regulatory approval of migalastat in the European Union, actual results may differ materially from those set forth in this presentation 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 EMA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing our 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 presentation to reflect events or circumstances after the date hereof.

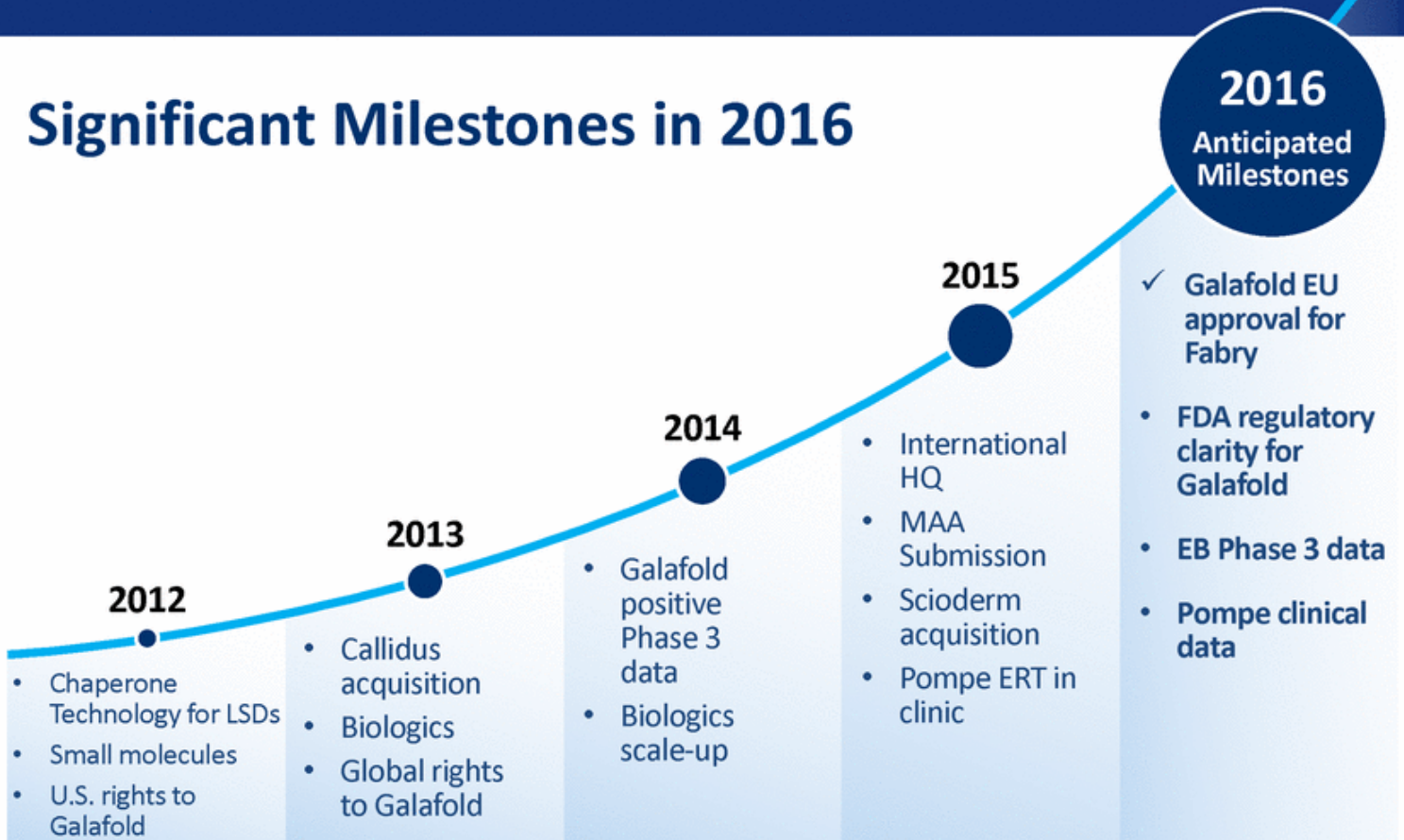
Amicus 2016 – Looking Back

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)
- FDA Meeting anticipated mid-year

Epidermolysis Bullosa (EB)

- Phase 3 Novel Topical Cream (SD-101)
- U.S. Breakthrough Therapy Designation
- Rolling NDA
- Phase 3 Data Expected in 2H16

Pompe

- Novel ERT + Chaperone Treatment Paradigm
- Biologics Manufacturing
- Clinical Study Initiated with Data Anticipated in 2016

R&D Engine and Continued Business Development Activity

*Pending Approval



**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™ 123 mg
(migalastat)
Hard Capsules

Oral Use
14 Hard Capsules

Amicus Therapeutics

Lift to open

Galafold™ (migalastat)

AMENABILITY TABLE

Mutation Search:

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 (α-galactosidase A 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.

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.AAB for nucleotide sequence changes, where 'c' is optional, # indicates a number, A and B are letters. Examples: GLETC or GLEBC

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

Last Updated: 26 May 2016

Amicus Therapeutics | MFGAL01/082/01/15 | 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

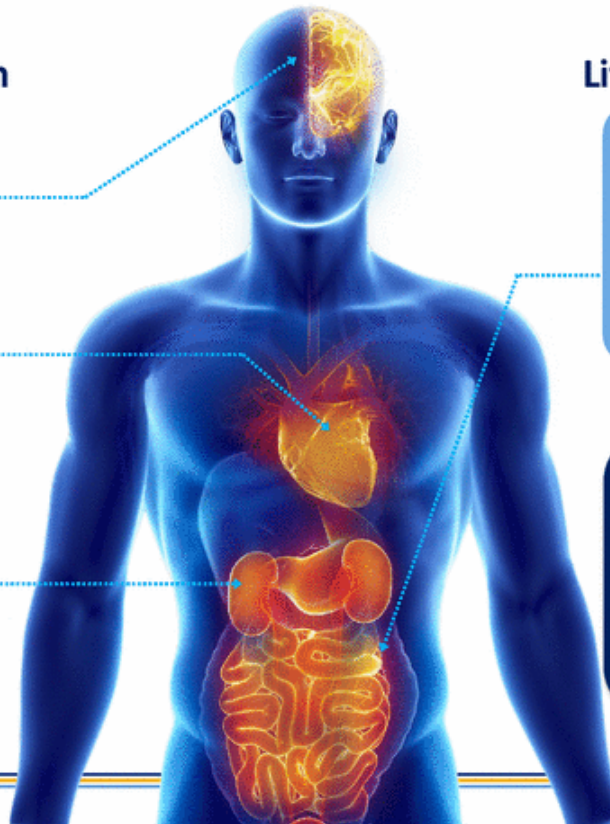
Life-Limiting Symptoms

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

Launch Activities



Medical education and patient advocacy ongoing on behalf of Fabry patients



Experienced commercial leadership team with established international operations



Patient and physician mapping



Global value dossier complete and local submissions in development



International distribution system

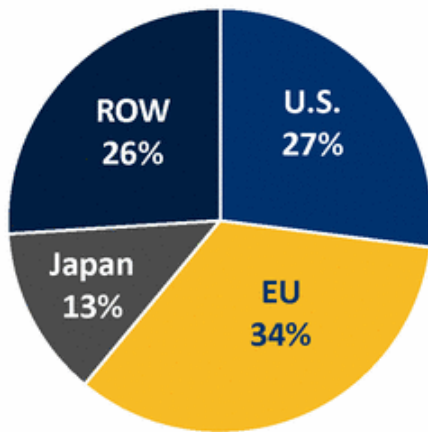
Launch Underway!



Galafold Commercial Opportunity

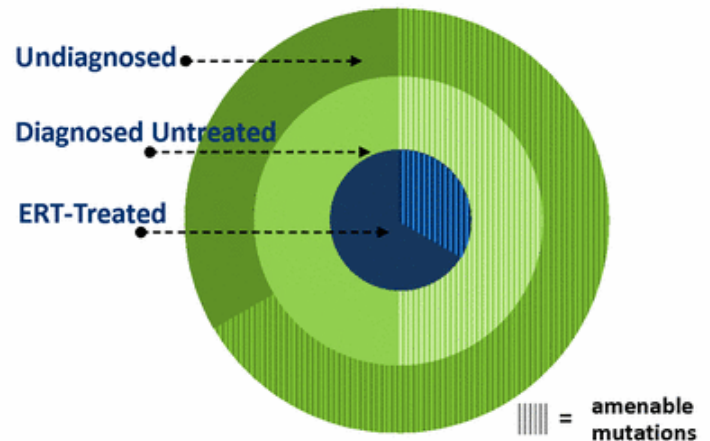
Amicus is Prioritizing EU, Japan, US and Other Large Fabry Markets for Initial Launch
Go To Market Strategy to Address 35%-50% of Patients with Amenable Mutations

Geographic Segments



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

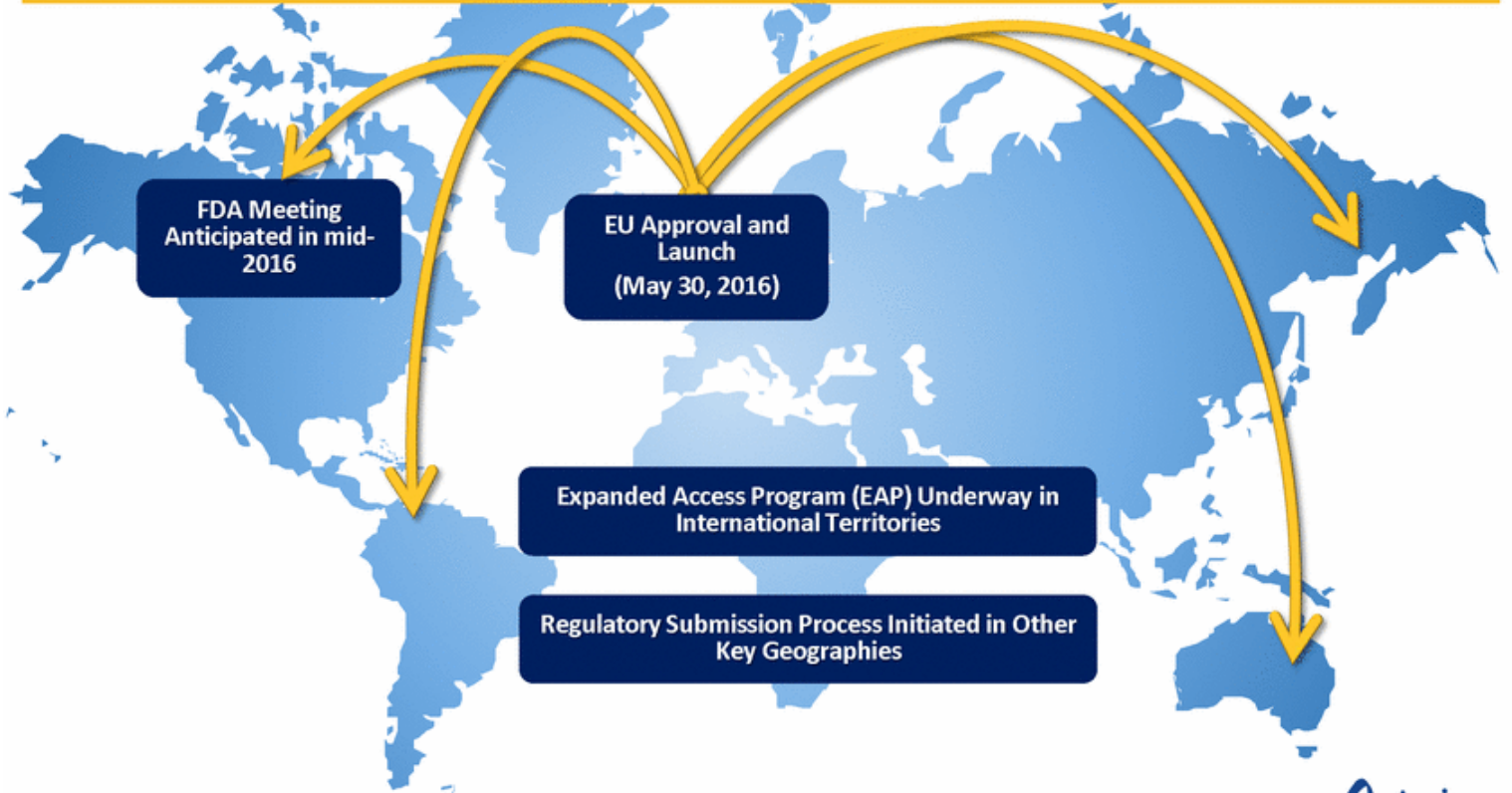
Patient Segments



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

Global Regulatory Strategy

EU Approval Lays the Foundation to Address ~70% of Global Fabry Market



EU Launch Strategy

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

UNITED KINGDOM

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

FRANCE

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

GERMANY

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



German Launch Underway

Drug Shipped to First Patient within 24 Hours

- Experienced, high quality team
- Launch began May 30, 2016
- First prescription successfully delivered
- Patient support program in place
- Pricing dossier ready for submission
- Market mapping complete
- Significant KOL engagement to date



Japan Market Overview

Amicus is Actively Pursuing a Regulatory Pathway in Japan



MARKET OVERVIEW

- ~650 patients treated
- No ERT home infusion currently available
- Physicians tend to initiate treatment early

CLINICAL/REGULATORY STATUS

- Phase 1 PK study completed
- Multiple sites and patients participated in Phase 3 Study 012
- Orphan drug designation
- Regulatory discussions initiated with PMDA

United States Market Overview

FDA Meeting and U.S. Regulatory Update Anticipated Mid-2016



MARKET OVERVIEW

ERT-treated patients: ~1,500
~40%-50% diagnosed and untreated
Fabrazyme only

CLINICAL/REGULATORY STATUS

FDA meeting anticipated mid-2016

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

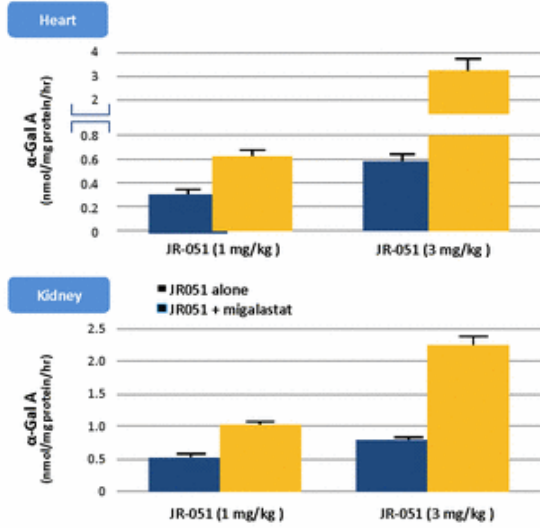
Development status:

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

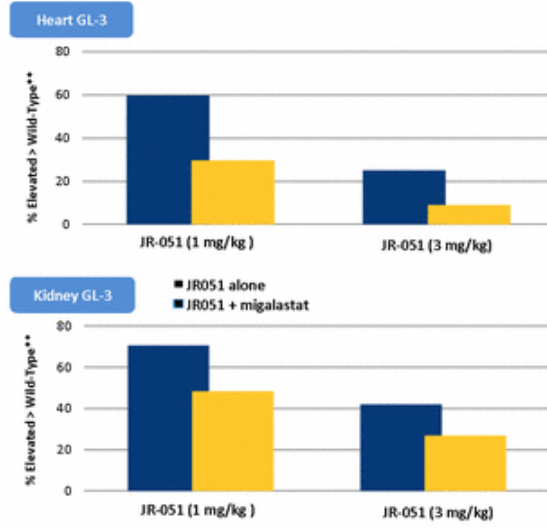
CHART Preclinical Proof-of-Concept for Fabry Co-Formulation

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

A-Gal A Tissue Uptake



GL-3 Substrate Reduction



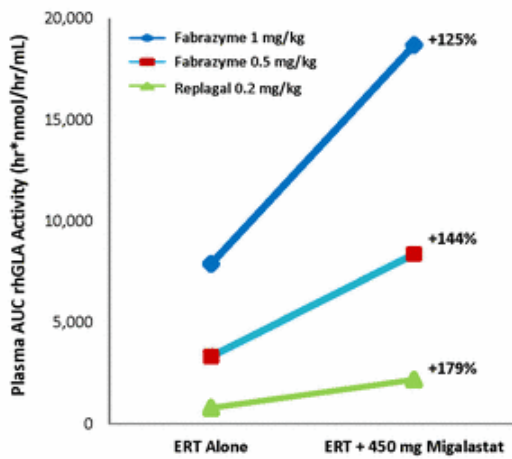
*ERT+/- Migalastat HCl in GLA Knock-Out Mice (Repeat-Dose IV Administration)

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

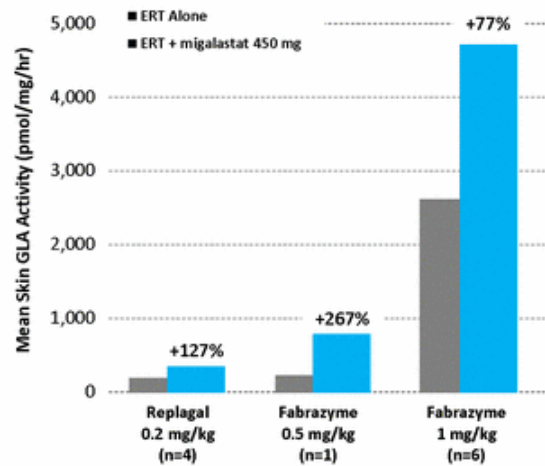
CHART Phase 2a Results for Fabry Co-Administration Study 013

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

Plasma rhGLA Activity (Area Under Curve)



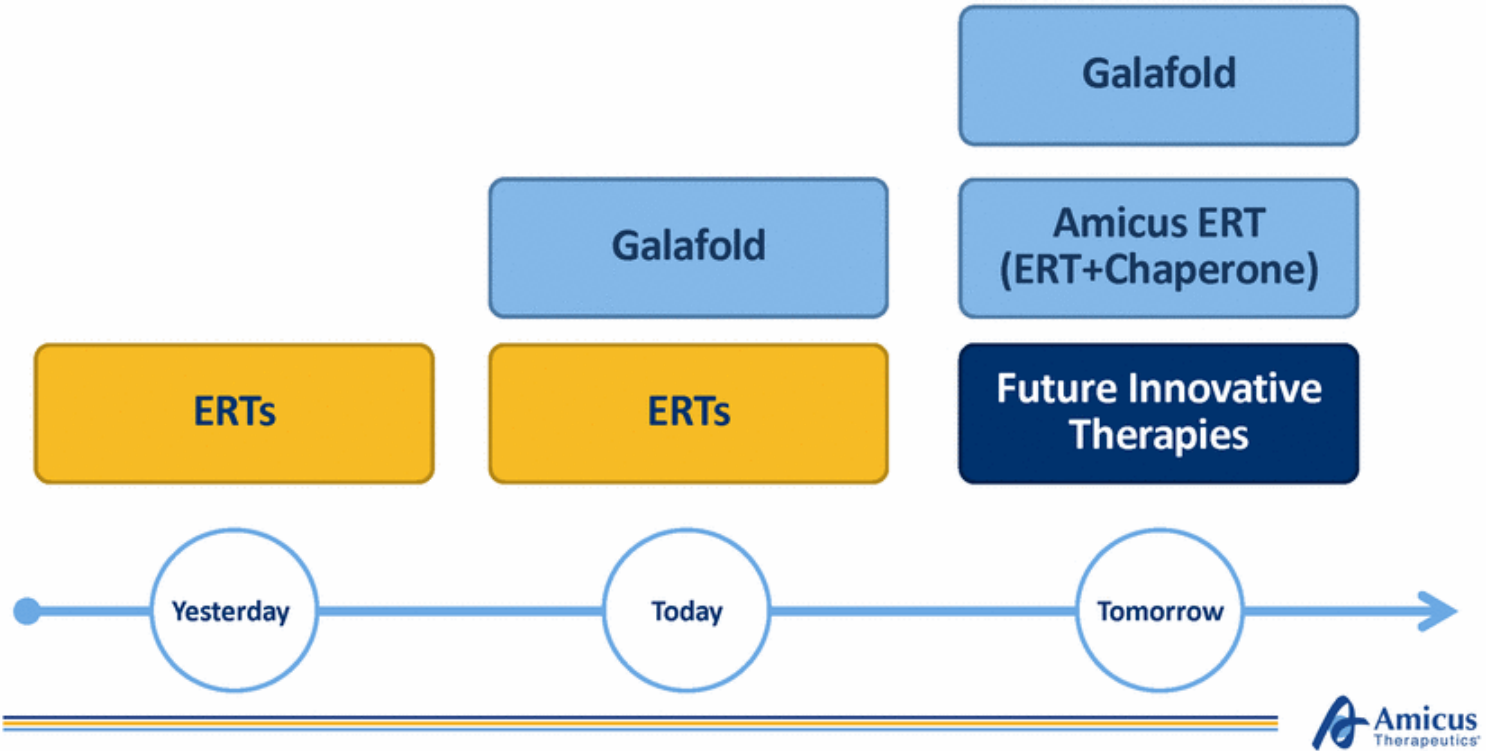
Mean Skin GLA Activity (Day 2)

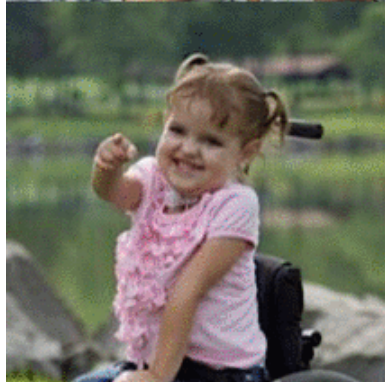
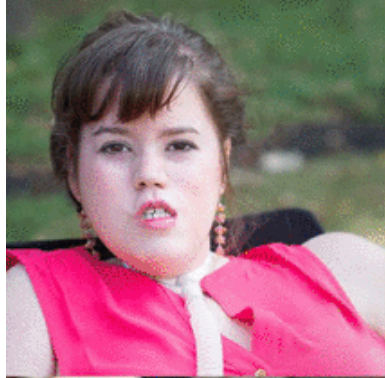


¹ Bichet, et al., A Phase 2a Study to Investigate the Effect of a Single Dose of Migalastat HCl, a Pharmacological Chaperone, on Agalsidase Activity in Subjects with Fabry Disease, LDN WORLD 2013.

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

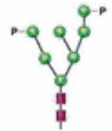
Antibody titers shown to affect treatment outcomes^{4,5}



Uptake/ Targeting

Low M6P receptor uptake into skeletal muscle²

Vast majority of rhGAA not delivered to lysosomes²



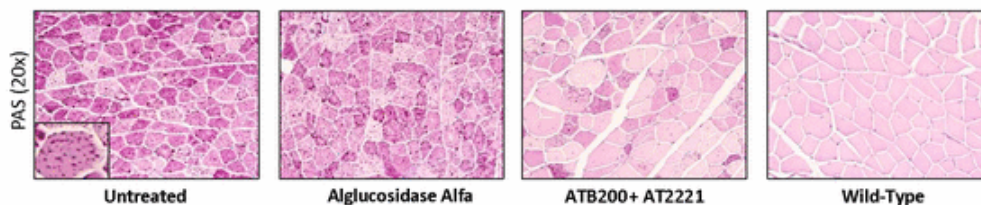
Uniquely Engineered rhGAA Optimized M6P & Carbohydrates

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.

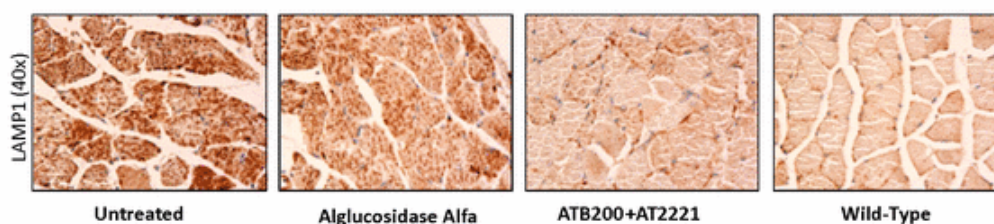
Preclinical Proof of Concept

ATB200 + Chaperone Results in Improved Substrate Clearance in Preclinical Models¹

PAS-glycogen staining in Quadriceps



LAMP1 Immunohistochemical staining in Soleus

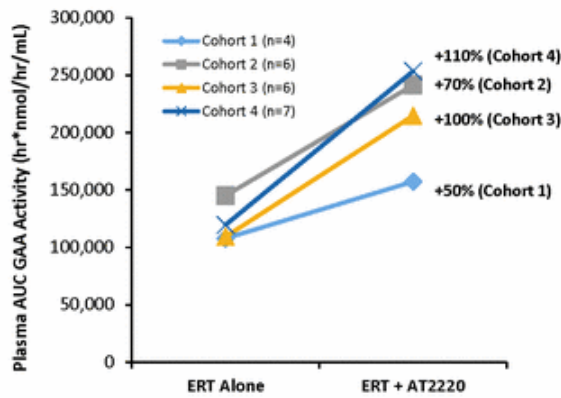


1. Following 2 doses of 20mg/kg alglucosidase alfa or ATB200 + AT2221 in Gaa KO mice, skeletal muscles evaluated for glycogen clearance and proliferated lysosomes. Treatment with alglucosidase alfa modestly reduced glycogen or proliferated lysosomes while ATB200, co-administered with AT2221 significantly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

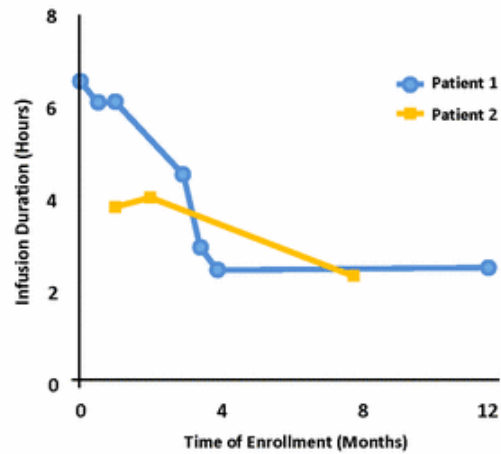
Human Proof-of-Concept: Currently Marketed ERT + Chaperones

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

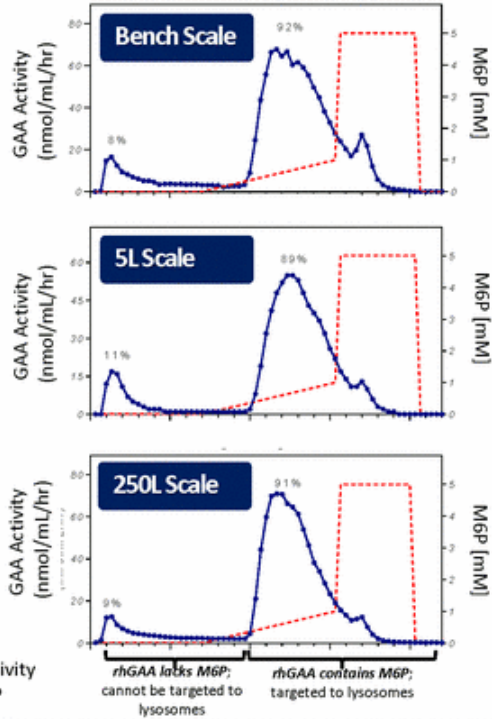
² Doerfler, et al. WORLD 2014

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

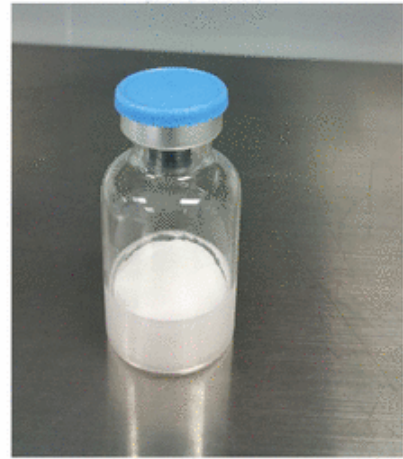
Biologics Manufacturing Capabilities

Optimized Glycosylation and Key Quality Attributes Maintained Through Scale Up

CI-MPR Receptor Chromatography

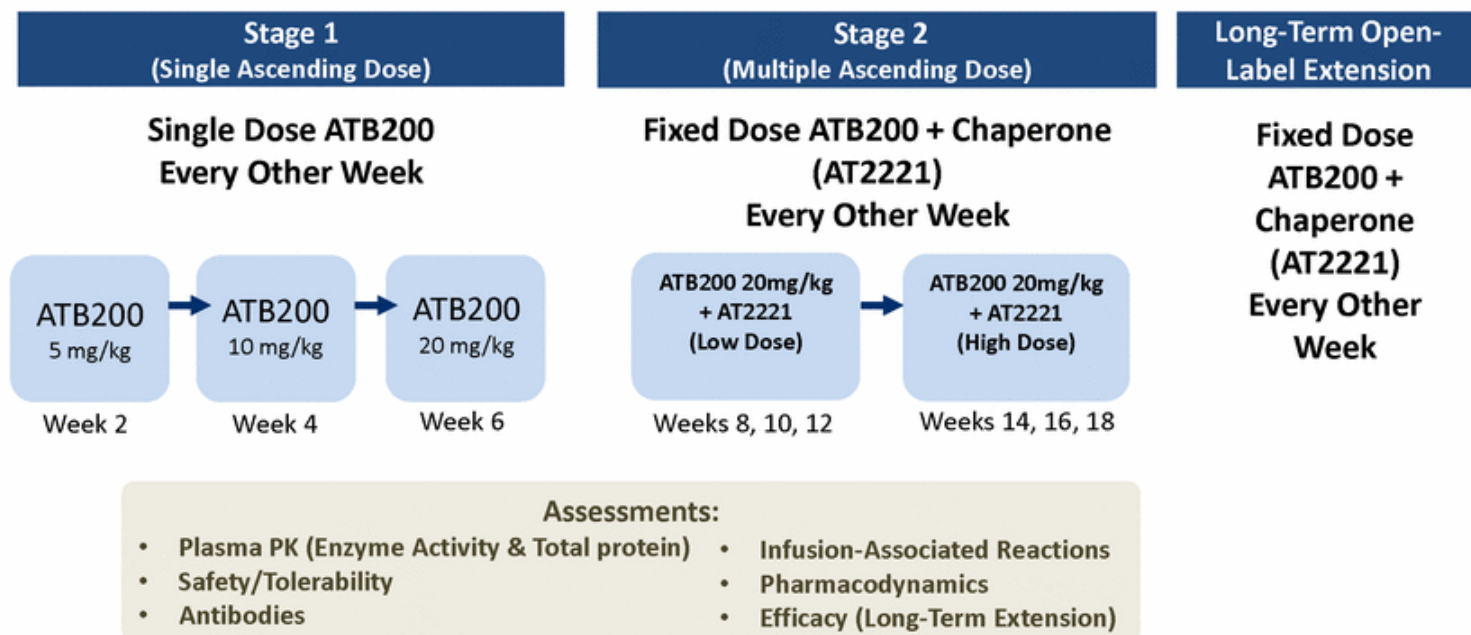


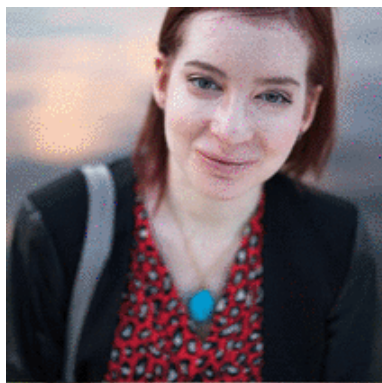
Lyophilized Vial of ATB200



Clinical Study in Pompe Patients

Patient Dosing Underway and Enrollment Ongoing at Multiple Sites





SD-101 for Epidermolysis Bullosa (EB)

Poised to deliver pivotal data for a
devastating rare disease in 2016

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

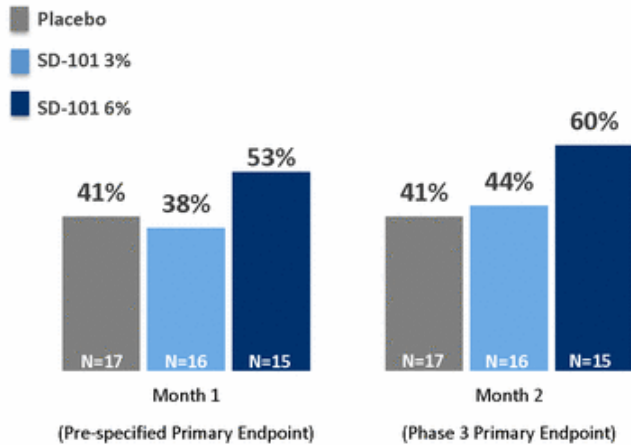
1. Assessments: 0, 14, 30, 60, 90 Days. 2. Initial Disease Severity: Mean target lesion size (cm²) 14.0 (range 5-39); mean lesional BSA: 19.4% (range 0.4-48%); mean wound age (days): 182 (range 21-1,639). EB types enrolled: Simplex (n=11), Dystrophic (n=29), and Junctional (n=8)

Phase 2b Results

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

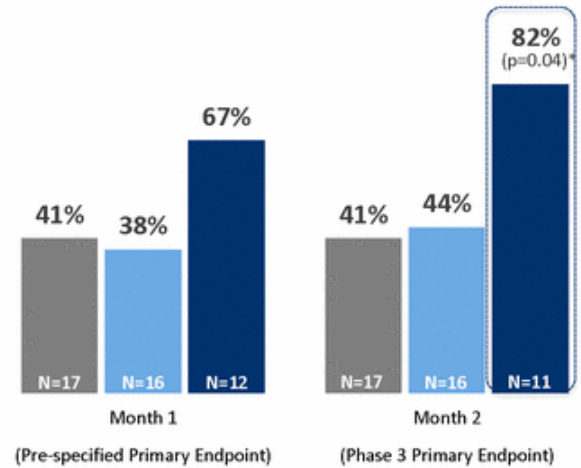
ITT Population (n=48)

Proportion of Complete Target Wound Closure (%)



Evaluable Population¹ (n=45)

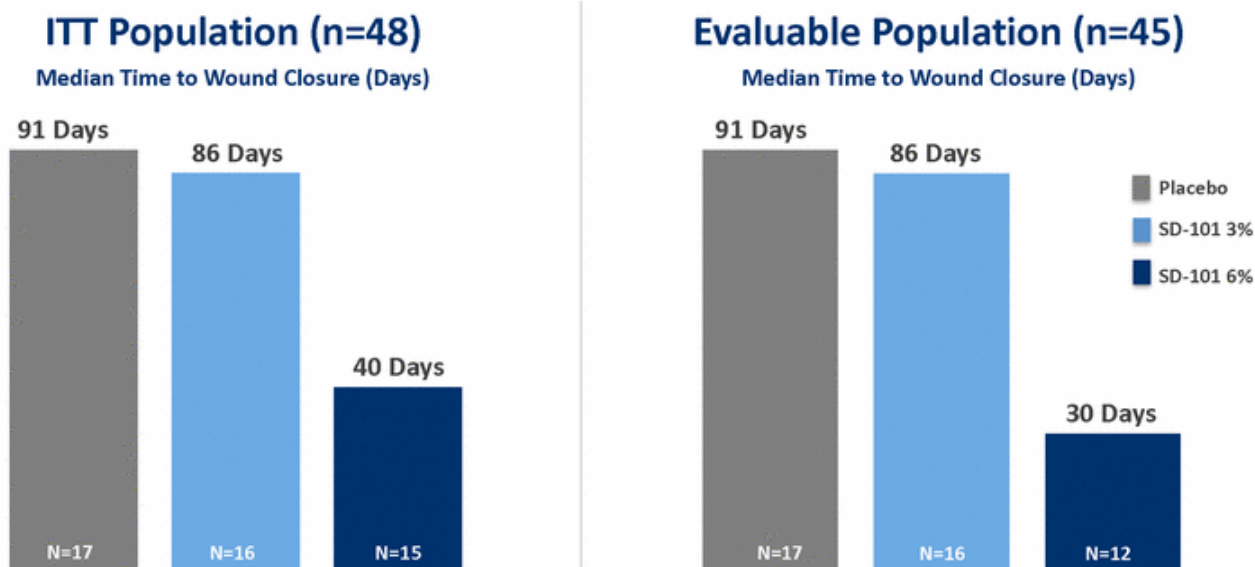
Proportion of Complete Target Wound Closure (%)



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 Results – Secondary Endpoint

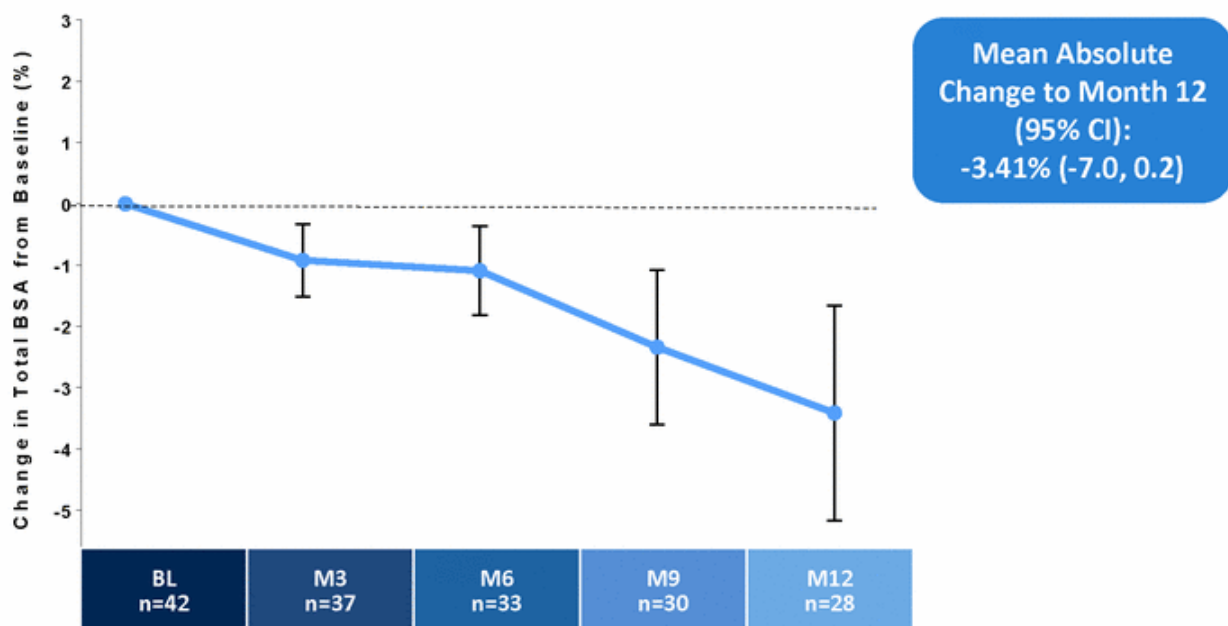
**SD-101 6% Showed Fastest Time to Wound Closure;
SD-101 Generally Safe and Well-Tolerated**



Adverse Events Similar Across Treatment Arms of Placebo, SD-101 3%, and SD-101 6%

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

Phase 3 Design (SD-005)

Phase 3 Initiated in 2Q15 and ~50% Enrolled
Top-Line Data Expected 2H16

3-Month, Double-Blind Treatment Period¹

SD-101 6%

~150 EB patients (age ≥ 1 month)

Placebo

Optional Extension (SD-006)

Open-Label SD-101 6%

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

Primary Endpoint: Target Wound Healing at Month 2

- U.S. and EU regulatory authorities agreed on primary endpoint
- Baseline wound: Chronic (≥ 21 days), size ≥10 cm²

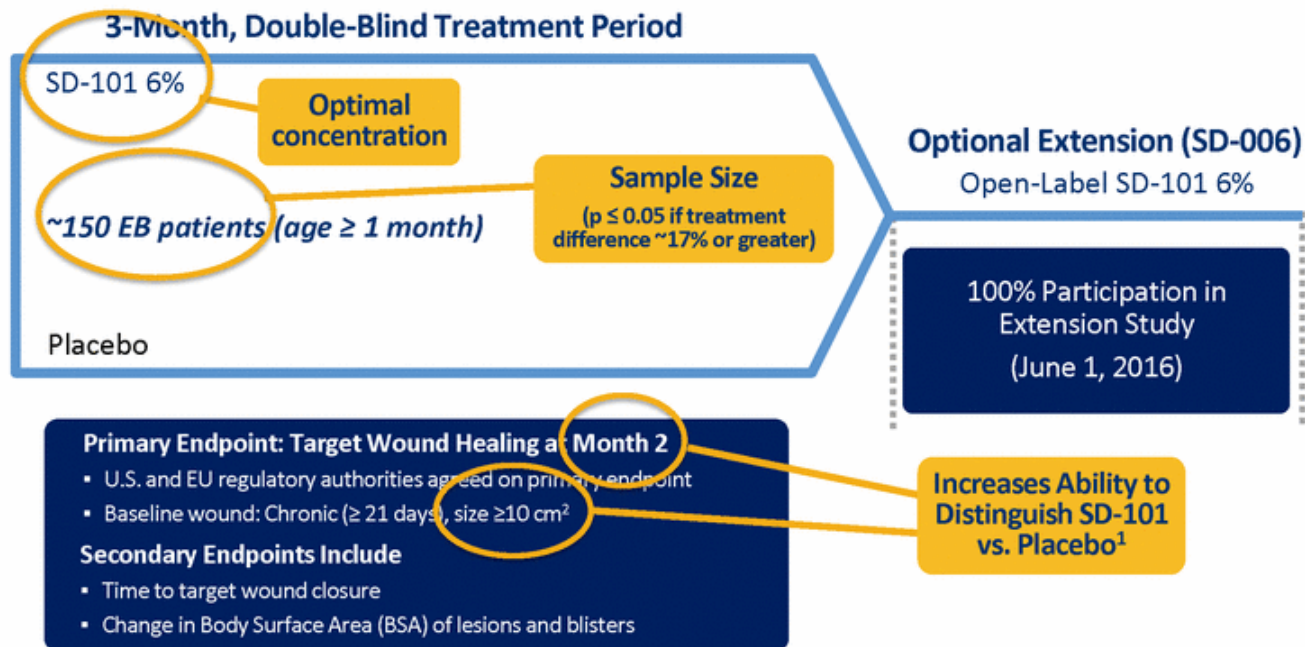
Secondary Endpoints Include

- Time to target wound closure
- Change in Body Surface Area (BSA) of lesions and blisters

1. Assessments: 0, 14, 30, 60, 90 Days. 1:1 randomization, daily topical application

Phase 3 Design (SD-005)

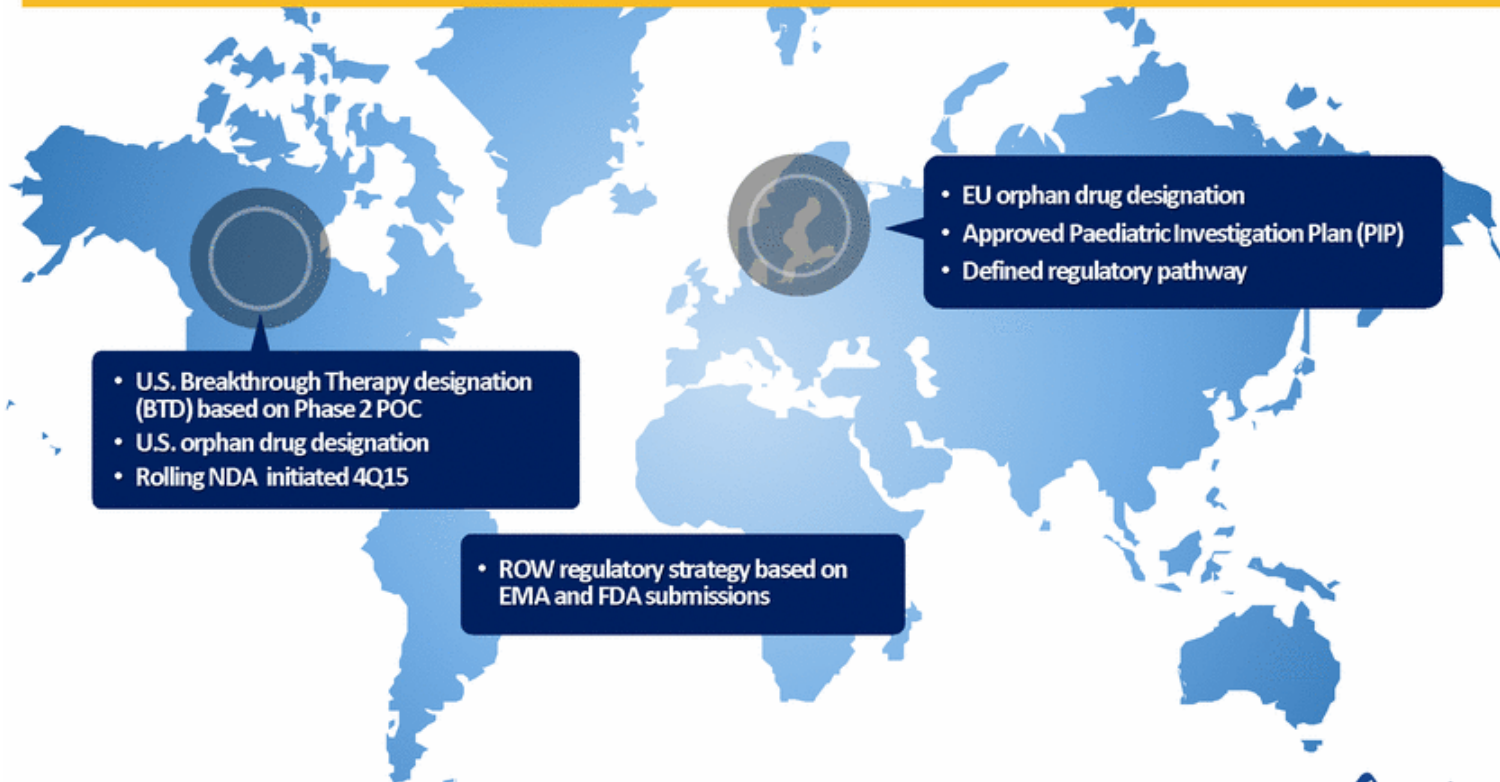
Study Design Incorporates Key Learnings from Phase 2b Study



1. Complete target wound closure in patients with target wounds ≥ 10 cm² at Month 2 in Phase 2b: SD-101 6% - 50% (n= 4) vs. Placebo - 12.5% (n=8)

Global Regulatory Strategy

Positive FDA and EMA Feedback on Phase 3 Study Design

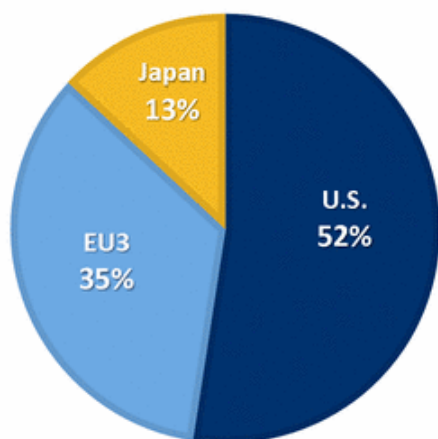


\$1B+ Commercial Potential

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

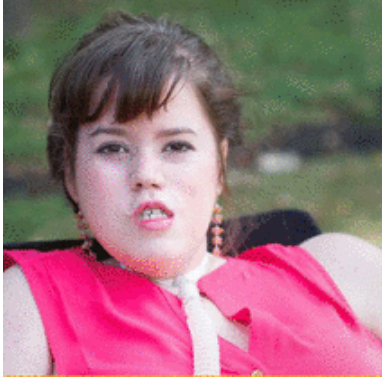
- No approved treatments, opportunity for first-in-class
- Promising proof of concept in all EB types

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



Financial Summary

Strong Balance Sheet to Invest in Rare Disease Pipeline

Strong Balance Sheet

Strong Balance Sheet Provides Cash Runway into Mid-2017

Financial Position	March 31, 2016
Current Cash:	\$165.9M
Current Debt	\$50.0M
FY16 Net Cash Spend Guidance:	\$135-\$155M
Cash Runway	Mid-2017
Total Net Proceeds from ATM as of April 29	\$16.2M
Capitalization	
Shares Outstanding	125,221,637

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)
- FDA Meeting anticipated mid-year

Epidermolysis Bullosa (EB)

- Phase 3 Novel Topical Cream (SD-101)
- U.S. Breakthrough Therapy Designation
- Rolling NDA
- Phase 3 Data Expected in 2H16

Pompe

- Novel ERT + Chaperone Treatment Paradigm
- Biologics Manufacturing
- Clinical Study Initiated with Data Anticipated in 2016

R&D Engine and Continued Business Development Activity

*Pending Approval

Thank You

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Significant Underdiagnosis of Fabry Disease

Larger Number of Patients Identified Through Newborn Screening Suggest Fabry Could be One of the More Prevalent Human Genetic Diseases

Newborn Screening Study	# Newborns Screened	# Confirmed Fabry Mutations	% Amenable
Burton, 2012, US	8,012	7 [1: ~1100]	TBD
Mechtler, 2011, Austria	34,736	9 [1: ~3,800]	100%
Hwu, 2009, Taiwan	171,977	75 [1: ~2300]	75%
Spada, 2006, Italy	37,104	12 [1: ~3100]	86%
Historic published incidence	1:40,000 to 1:60,000		

Index Patient
(3-5:1 Index)



Majority of Newly Diagnosed Patients Have Amenable Mutations

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

30-Month Renal Function (Study 012) at WORLD 2016

Kidney Function (GFR) in Migalastat Group at Month 30 was Comparable to Migalastat and ERT Groups through Month 18

	Mean Annualized Change in GFR (ml/min/m²/yr) (95% CI) with Galafold Baseline to Month 30 in Study 012*
Estimated GFR (eGFR) (CKD-EPI) (n=31)	-1.7 (-2.6, -0.8)
Measured GFR (mGFR) (n=30)	-2.75 (-4.8, -0.7)

*Annualized change in GFR at Month 18 in Study 012: eGFR -1.0 (-3.6, 1.6) for patients on ERT and -0.4 (-2.3, 1.5) for patients on migalastat; mGFR -3.2 (-7.8, 1.3) for patients on ERT and -4.35 (-7.7, -1.1) for patients on migalastat

30-Month Cardiac Function (Study 012) at WORLD 2016

Reductions in LVMI Demonstrated through Month 30 with Statistically Significant Reductions in Patients who had Abnormal Cardiac Mass (left ventricular hypertrophy, or LVH) at Baseline

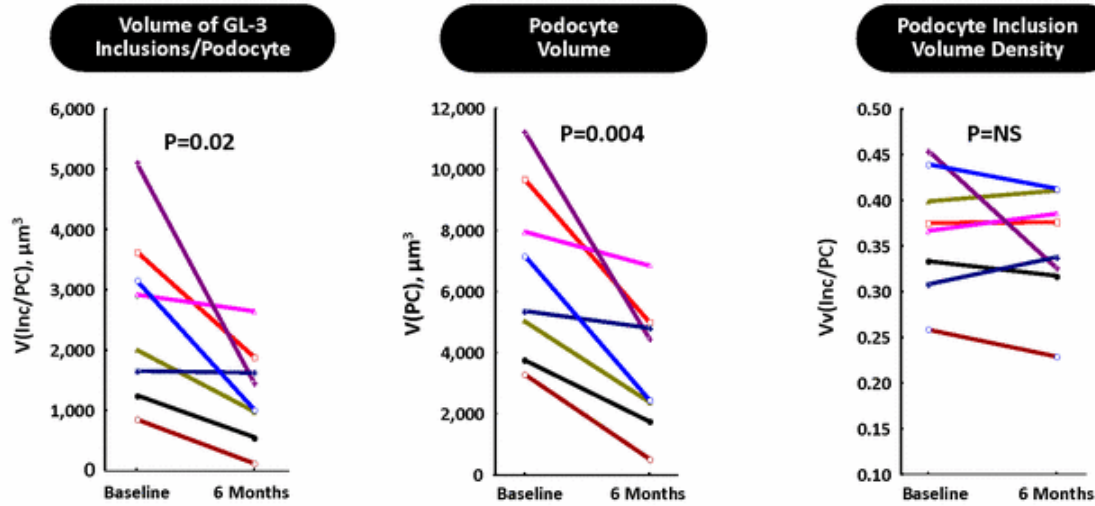
Cardiac ECHO Parameters - Change from Baseline to Month 30

	Migalastat (Overall) n=30	Migalastat Change (Overall) (Mean, 95% CI) n=28	Migalastat (LVH at Baseline) n=11	Migalastat Change (LVH at Baseline) (Mean, SD) N=10
Left Ventricular Mass Index (LVMI) (g/m ²)**	94.6	-3.7 (-8.9, +1.3)	116	-10.0*** (-16.6, -3.3)

Normal LVMI: 43-95 (female), 49-115 (male). Change in LVMI at Month 18 in Study 012: ERT group: -2.0 (-11.0, 7.0) for all patients, +4.5 (-20.9, 29.9) for patients with LVH at baseline. Migalastat group: -6.6 (-11.0, -2.1) for all patients, -8.4 (-14.9, -2.0) for all patients with LVH at baseline. *Statistically significant (95% CI does not overlap zero)

GL-3 Substrate Reduction in Podocytes (Study 011) at WORLD 2016

Migalastat Demonstrated a Consistent and Statistically Significant Reduction in Disease Substrate (GL-3) in Podocytes from Baseline to Month 6 (p=0.02)



P-value based on paired t-test; Spearman correlation analysis