Amicus Therapeutics®

34th Annual J.P. Morgan Healthcare Conference

John F. Crowley, Chairman and Chief Executive Officer

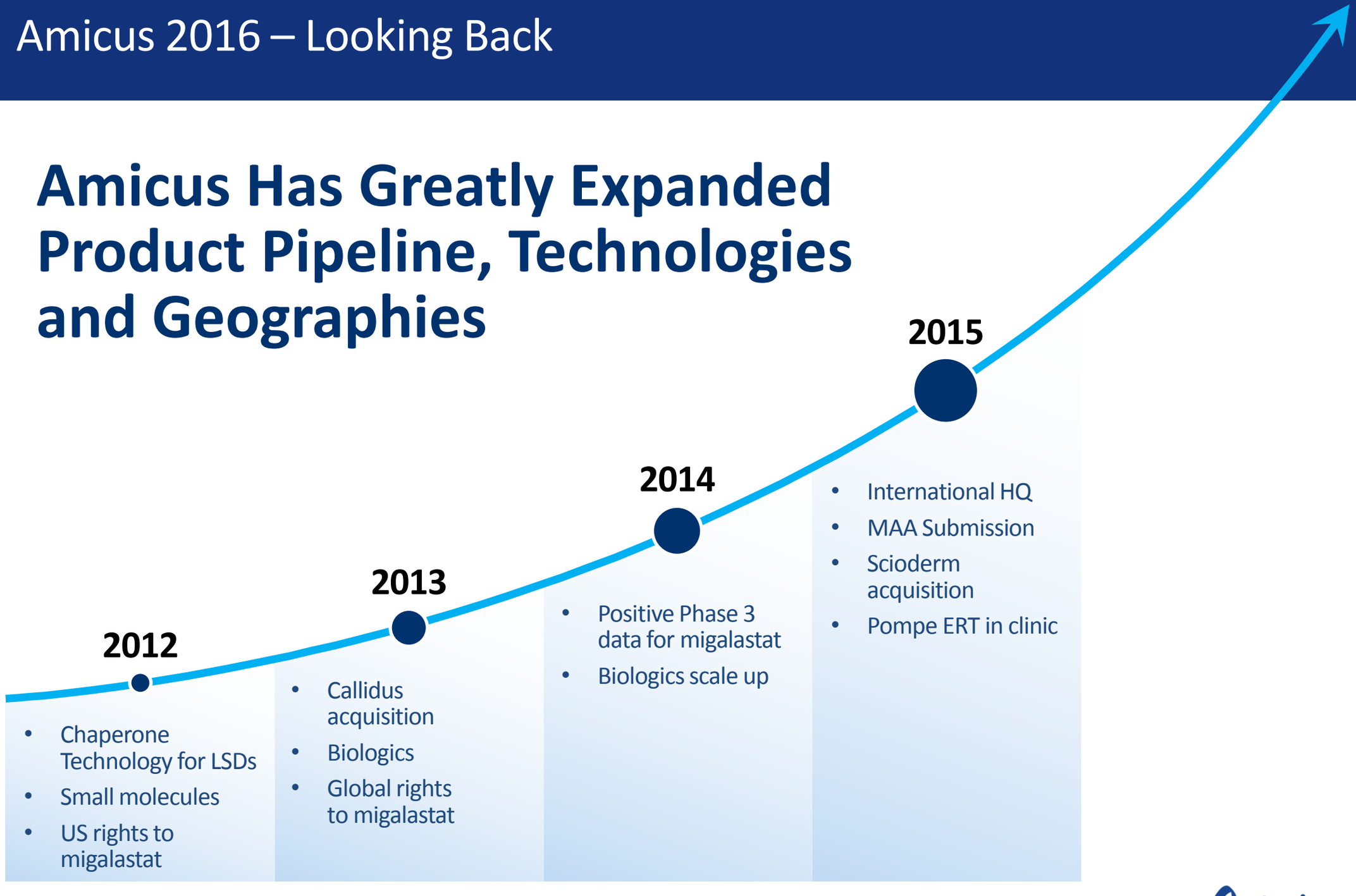
January 12, 2016

Safe Harbor

This presentation will contain, "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of Amicus' candidate drug products, the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus' candidate drug products, financing plans, and the projected cash position for the Company. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. The inclusion of forward-looking statements should not be regarded as a representation by Amicus that any of its plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong. They can be affected by inaccurate assumptions Amicus 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 timing of an NDA submission for migalastat monotherapy, and 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 the business of Amicus, including, without limitation: the potential that results of clinical or pre-clinical 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 may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we will need additional funding to complete all of our studies and, our dependence on third parties in the conduct of our clinical 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, 2014 and Form 10-Q for the quarter ended June 30, 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 Amicus undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

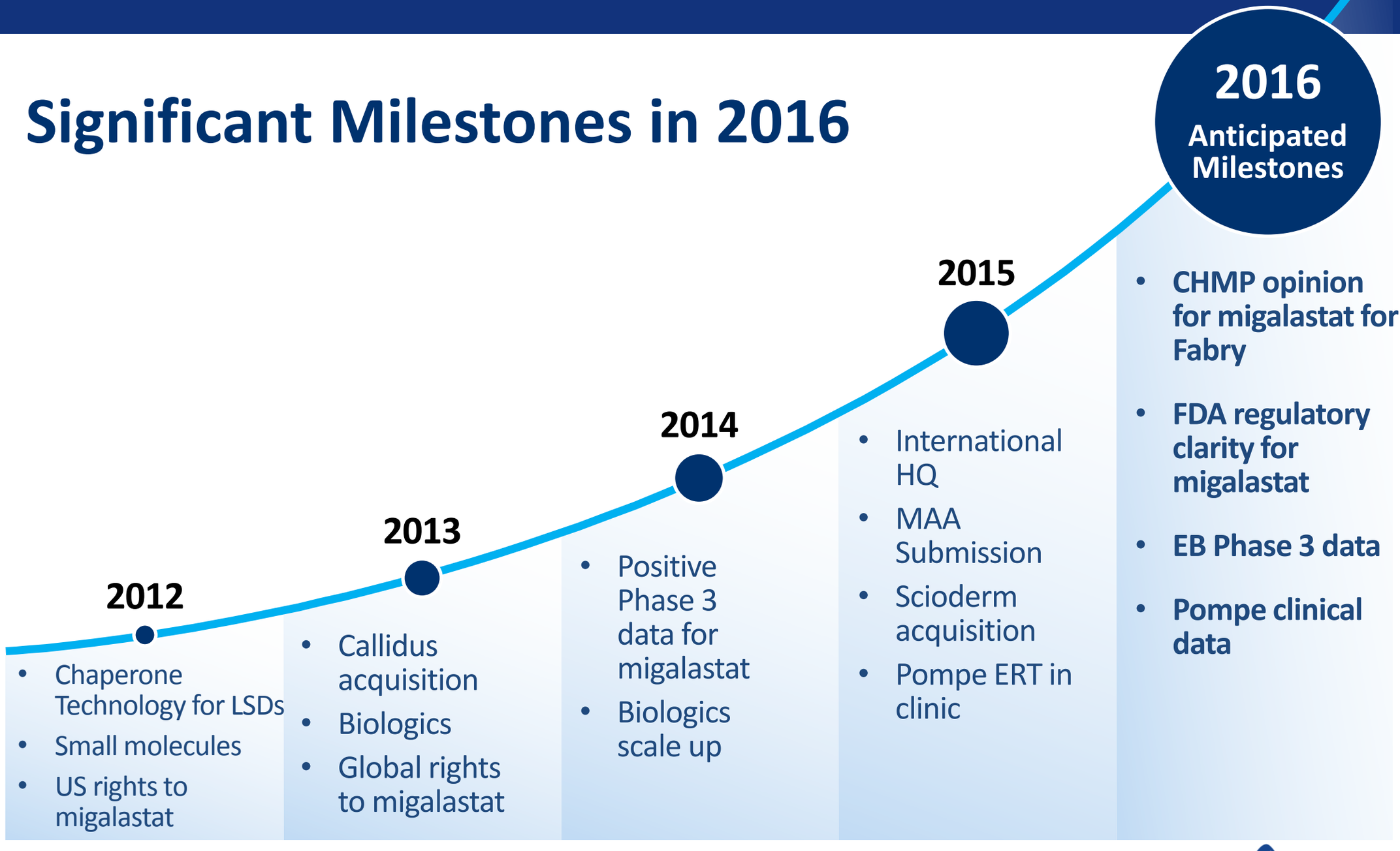
Amicus 2016 – Looking Back

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

Key Drivers of Value

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

Fabry

- Migalastat Personalized Medicine (Small Molecule)
- MAA Submitted
- CHMP Opinion Anticipated Early 2016
- Prepared for EU Launch*

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

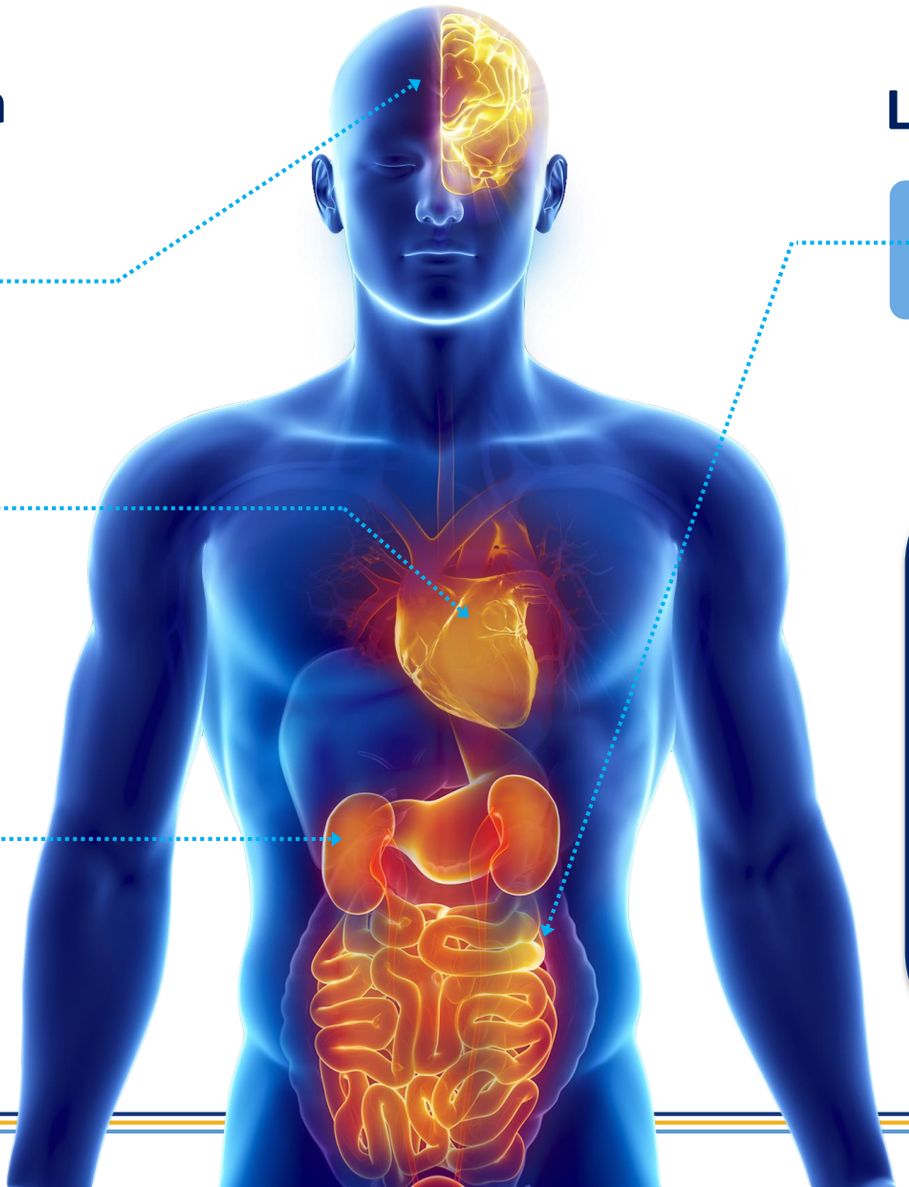
Migalastat Personalized Medicine for Fabry Disease

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]
- **KIDNEY DISEASE**[3]



Life-Limiting Symptoms

- **GASTROINTESTINAL**[3]

Key Facts

- Deficiency of α-Gal A enzyme leading to GL-3 accumulation
- >800 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

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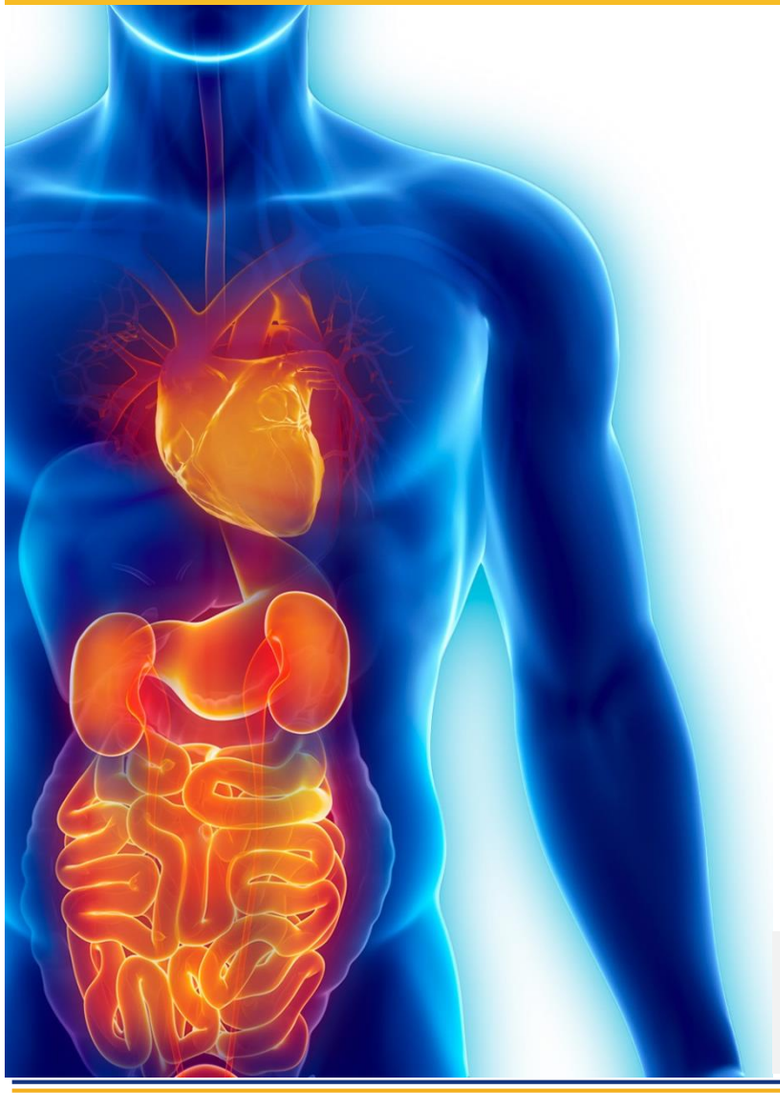
Summary of Clinical Data

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



Reduction in Disease Substrate
IC GL-3 (Study 011[4])
Plasma Lyso Gb-3 (Study 011[3,4] and 012[2])

Stability of Kidney Function
Estimated Glomerular Filtration Rate (eGFR) and measured GFR (Study 011[1] and Study 012[1,2])

Reduction in Cardiac Mass
Left Ventricular Mass Index (LVMI) (Study 011[3] and 012)

Improvement in GI Symptoms
Gastrointestinal Symptoms Rating Scale (GSRS) (Study 011[4])

Low Rate of Fabry-Associated Clinical Events
Renal, Cardiac and Cerebro-Vascular Events (Study 012[2])

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

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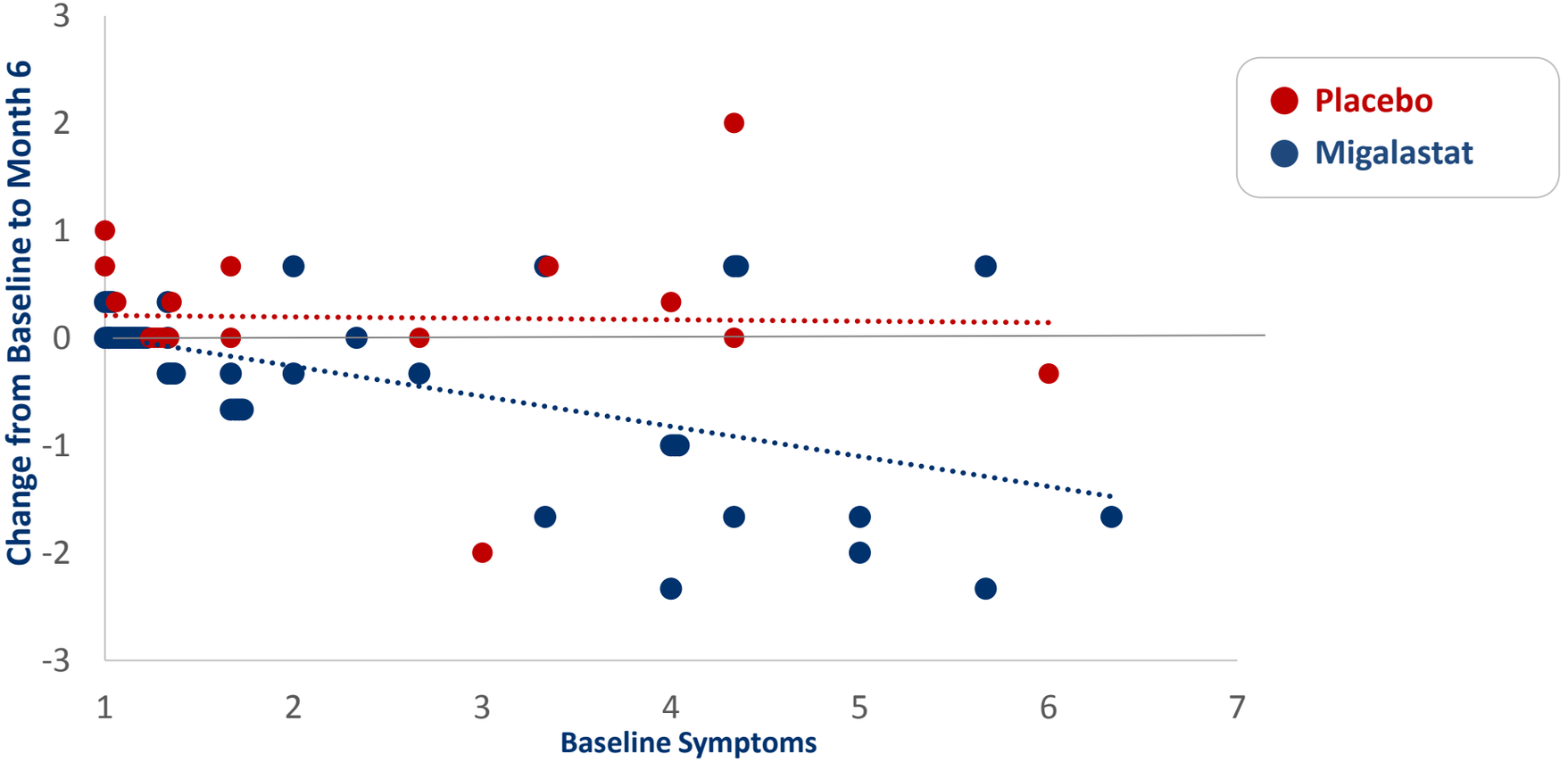
Additional Phase 3 Data on Diarrhea Symptoms

NEW

Migalastat has Generated Promising Data on Diarrhea Improvement, One of the Most Life-Limiting Symptoms of Fabry Disease

Statistically Significant Change - Migalastat vs. Placebo from Baseline to Month 6 (nominal p=0.026)



Note: Month 6 used as baseline for patients in placebo arm switching to migalastat; MID from Chan 2006 in kidney transplant. Minimal important difference (MID) for the GSRS diarrhea domain is 0.4 (Chan 2006, renal transplant patients)

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Fabry Patient Perspective

Case Report from Long-Term Treatment with Migalastat Shows Improvement in Pain and Return to Everyday Activities[1]

Nine-year follow-up in a patient receiving migalastat pharmacological chaperone therapy for Fabry disease

Schiffmann R[1], Swift C[1], Barth J[2], Skuban N[2], and Castelli J[2]

[1]Baylor Research Institute, Dallas, TX; [2]Amicus Therapeutics, Cranbury, NJ, USA;

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BACKGROUND

Fabry Disease

Fabry disease is a rare, progressive, and devastating X-linked lysosomal storage disorder caused by a deficiency in α-galactosidase A, with an estimated prevalence of 1:117,000 to 1:40,000 (Desnick and Schindler 2001; Meikle et al. 1999; Eurordis 2005).

More than 800 disease-causing mutations in GLA have been identified; ~60% of these are missense mutations.

Affects males and females; females have a mosaic of healthy and diseased cells.

The cardinal presenting features of Fabry disease are intermittent acroparesthesia and episodic crises of pain and fever (especially in childhood), angiokeratomas, hypohidrosis, heat/cold intolerance, and a characteristic "whorled" corneal opacity that generally does not affect vision. In these patients, the major causes of mortality include renal failure, cardiomyopathy, and cerebrovascular accidents.

Globotriaosylceramide (GL-3), a natural substrate of α-Gal A, accumulates and affects multiple organs and organ systems (kidney, heart, brain, gastrointestinal, skin).

Globotriaosylsphingosine (lyso-Gb3) is another substrate of α-Gal A that is elevated in plasma of males and females living with Fabry disease.

MIGALASTAT FOR FABRY DISEASE

Migalastat is an orally administered investigational pharmacological chaperone for patients with amenable mutations; facilitates proper folding and cellular trafficking of amenable mutant forms of α-Gal A to lysosomes where the breakdown of substrate can proceed.

Migalastat
Deoxygalactonojirimycin
AT1001

Amenable mutant forms of α-Gal A are identified using a GLP-validated HEK-293 cell-based assay (GLP HEK assay; see below).

30-50% of patients with Fabry disease are estimated to have amenable mutations; the majority of amenable mutations are associated with the classic phenotype of the disease.

DEFINITION OF AMENABILITY

The GLP HEK assay was used to express each of 600 FD-causing mutations in HEK-293 cells and measure increases in α-Gal A activity in response to migalastat. Amenable mutant forms were defined by a relative increase of ≥1.2-fold and absolute increase of ≥3% wild-type in the presence of 10 μM migalastat.

OBJECTIVE AND METHODS

We describe the long-term effects of migalastat on FD-associated pain.

A 37-year-old male patient with Fabry disease presenting with severe acroparesthesia and sensation of stiffness predominantly in the lower extremities, was evaluated. Clinical and biochemical studies were performed. Use of concomitant pain medications was also monitored

RESULTS: CONCOMITANT PAIN MEDICATION

Phase 2
First 4.5 years migalastat treatment

During the first phase 2 trial (dose finding), α-Gal activity increased 5-fold in leukocytes and 30-fold in skin after 72 weeks of migalastat treatment. Cardiac and renal function, as well as urine GL-3 levels remained normal.

Phase 3 (open label extension)
Years 4.5 to 9

During the phase 3 migalastat treatment (150 mg QOD), paresthesias and sensation of stiffness progressively improved, and he was able to decrease and then discontinue all his pain medications by the 8th year of therapy.

4.5 years — 4.5 years still ongoing

Phase 2 studies various migalastat doses | Phase 3 study migalastat 150 mg QOD

Change in pain meds

Pain meds: Dose XX (TID*)
1. oxycodone, tizanidine, baclofen
2. acetaminophen, naproxen, ibuprofen

Dose XX TID
Dose Y BID
Dose Z Once daily

Patient Journey to Diagnosis

- Chronic pain
- Weakness and fatigue
- Pain medication

Phase 2 Study + OLE for 4.5 Years

- Enzyme activity increased
- Cardiac and renal functions remained normal

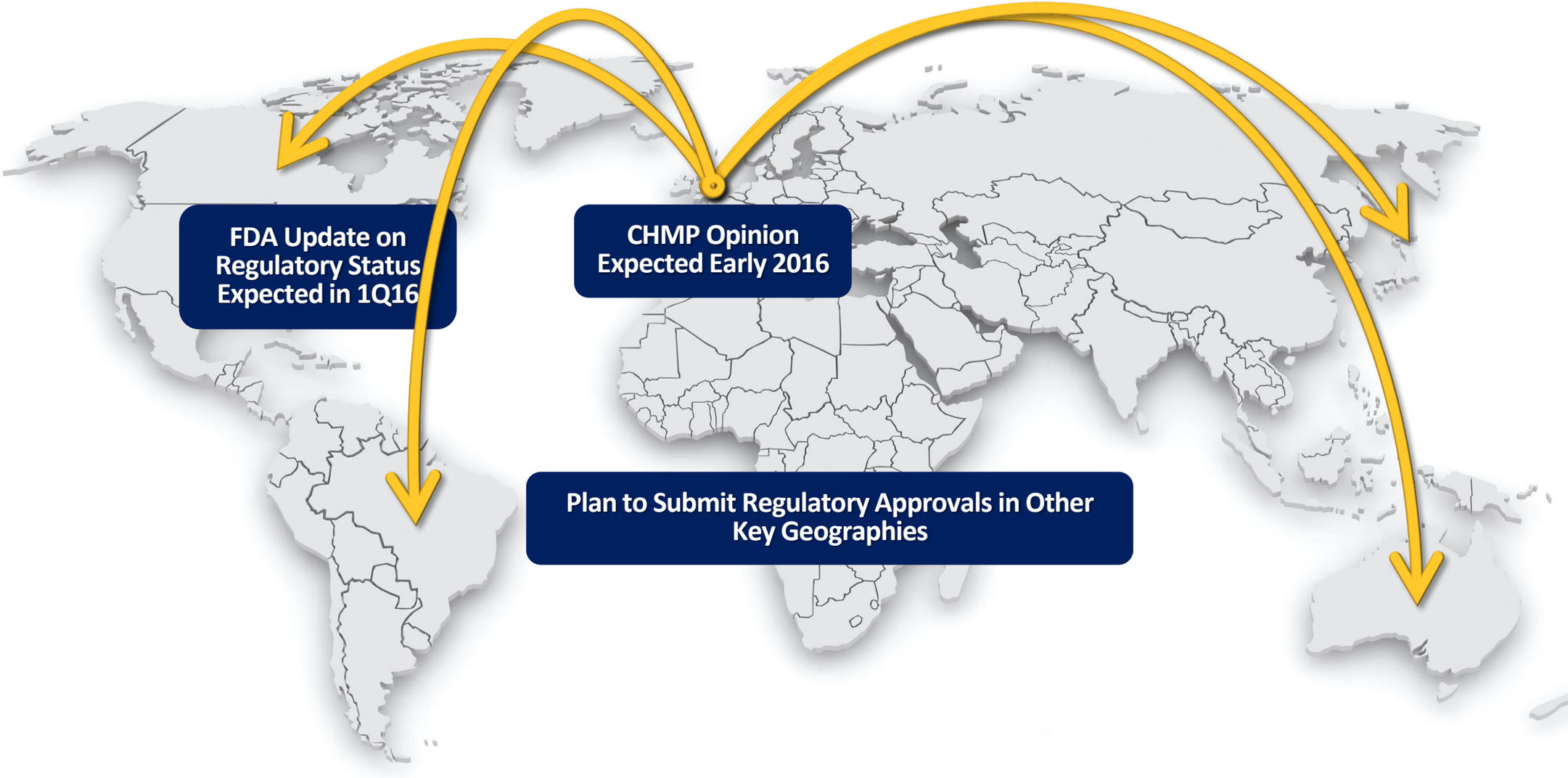
Phase 3 OLE for 4.5 Years (Still Ongoing)

- Paresthesias and sensation of stiffness improved
- Feels well, goes to gym and works
- Discontinued pain medication

1. Schiffmann, *et al.* Nine-year follow-up in a patient receiving migalastat pharmacological chaperone therapy for Fabry disease. **SSIEM 2015**.

Global Regulatory Strategy

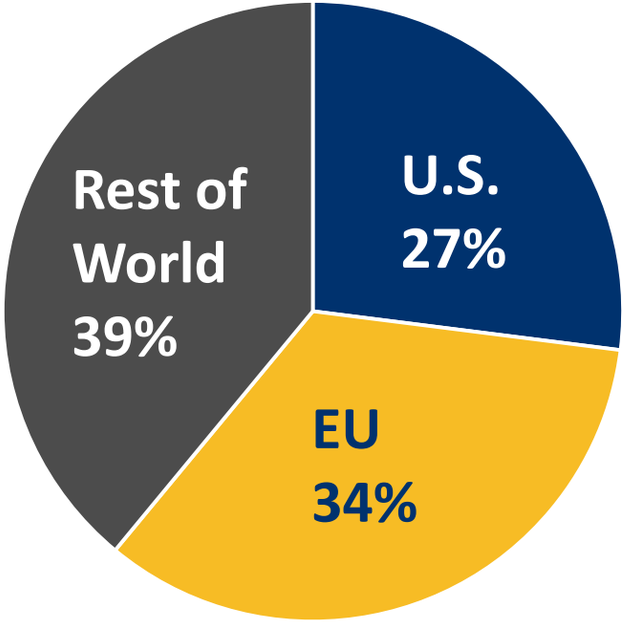
EU Approval Will Lay the Foundation to Address ~70% of Global Fabry Market



Fabry Market Today

Migalastat has Potential to Offer a Number of Important "Firsts" for Fabry Patients

$1.1B in FY14 ERT Sales[1]



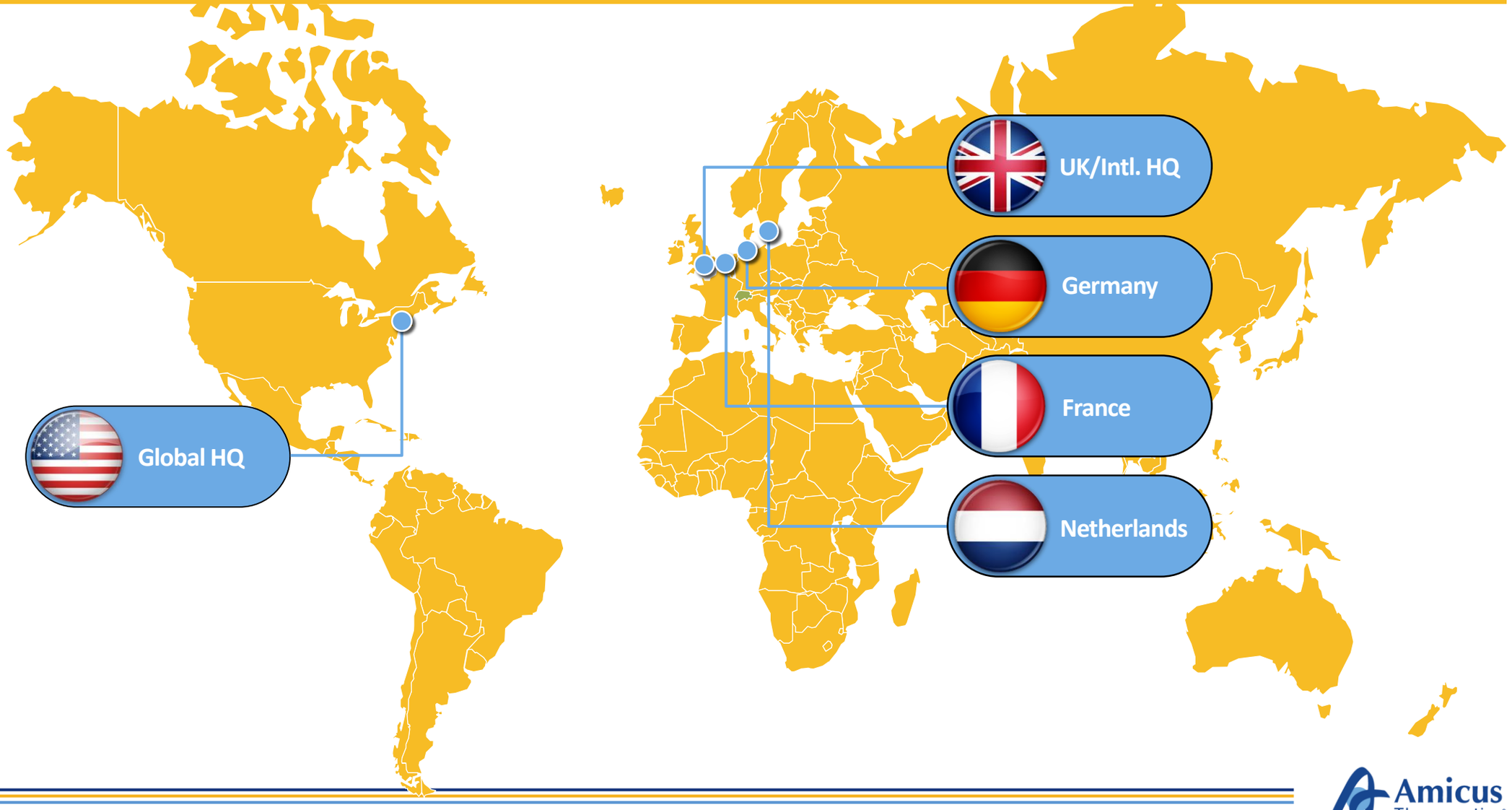
- 40-50% of Diagnosed Patients Not on ERT Today
- Market Continues to Grow > 10% / Year
- ERT Infused Once Every 2 Weeks

- ***First*** new product in > 10 years
- ***First*** oral therapy
- ***First*** targeted therapy for amenable patients (30%-50% of population)

1. Company filings and Amicus estimates

Global Infrastructure and International Team

World-Class Global Commercial Team to Support Migalastat Launch Upon Regulatory Approvals with Further Expansion in 2016



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Global Infrastructure and International Team

World-Class Global Commercial Team to Support Migalastat Launch Upon Regulatory Approvals with Further Expansion in 2016

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Commercial Launch Preparation 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

Amicus is Preparing for Potential Launches in 2016

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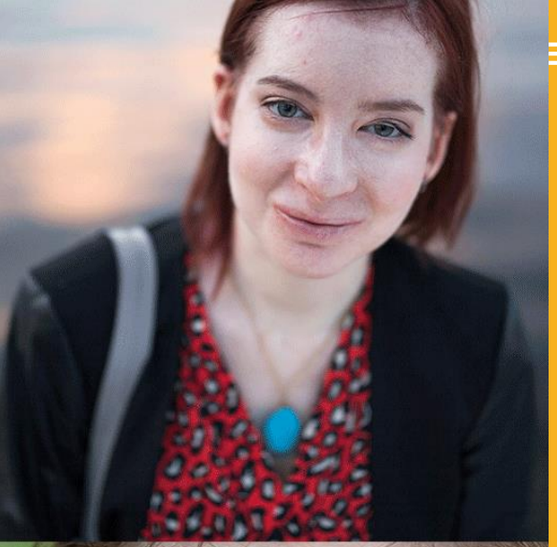
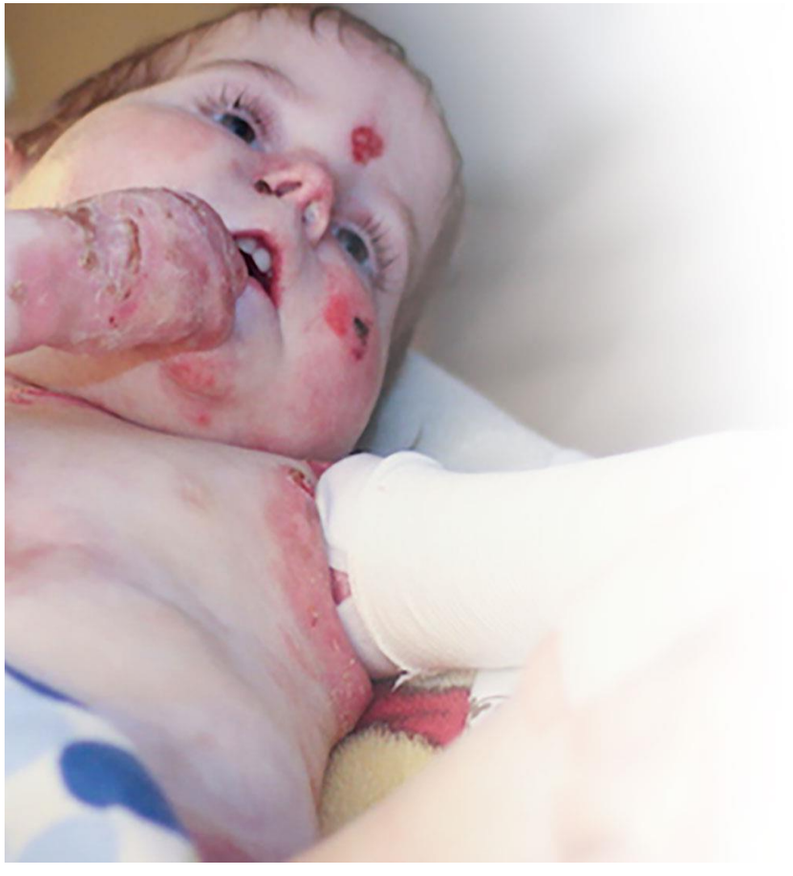


SD-101 for Epidermolysis Bullosa (EB)

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

EB Disease Overview

Rare, Devastating, Connective Tissue Disorder with No Approved Treatments

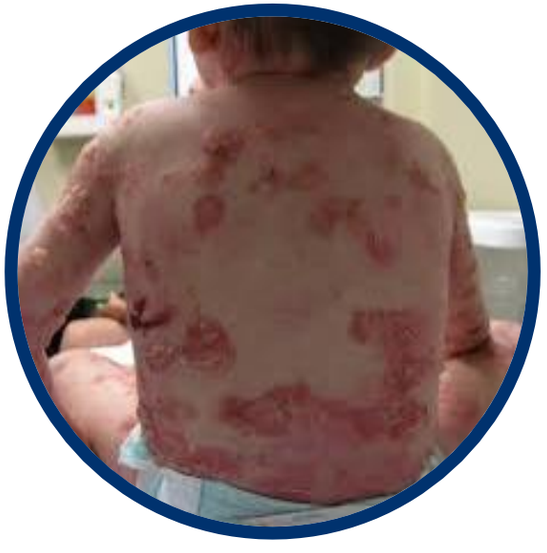


- Multiple genes cause disease which results in fragility of skin and can affect internal organs
- Diagnosed from infancy to adulthood
- Severe blistering, open wounds and scarring in response to minor friction to the skin
- Disfiguring, excruciatingly painful, and can be fatal
- Given the lack of approved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

Three Major EB Subtypes Represent ~99% of EB Population

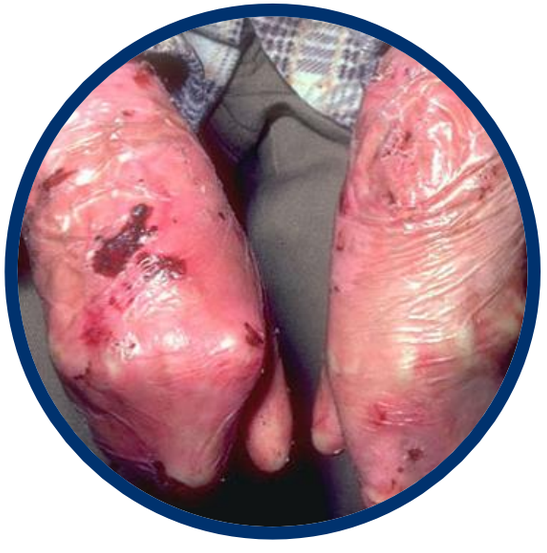
Multiple Subtypes…Single Devastating and Fatal Genetic Disorder

Simplex



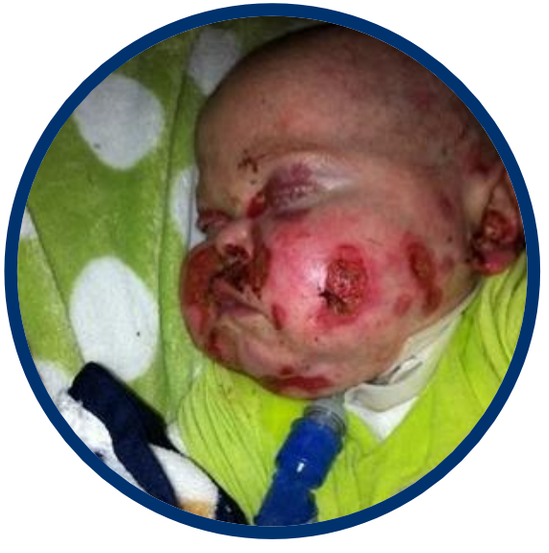
~75% of EB Population

Dystrophic



~20% of EB Population

Junctional



~5% of EB Population

INCREASING SEVERITY

No Approved Therapies Today

SD-101 in Development for All 3 Major Subtypes

30,000 - 40,000+ Diagnosed in Major Markets

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U.S. Breakthrough Therapy Designation

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

- Open-label, 8 patient proof-of-concept study[1]
- Ages 6 months – 9 years
- All baseline target wounds ≥ 10 cm^2
- SD-101 3% applied once daily for 3 months

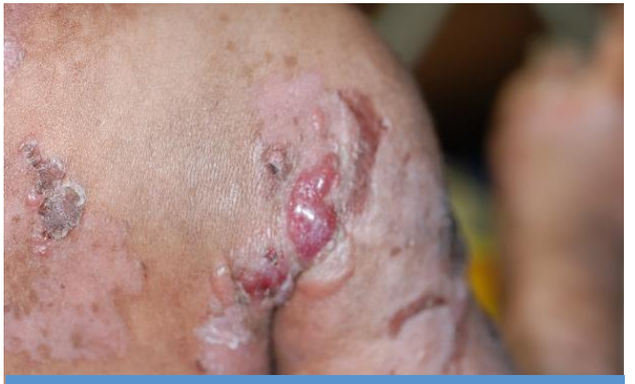
Key Findings

87.5%
of patients experienced complete closure of target wounds within 1 month

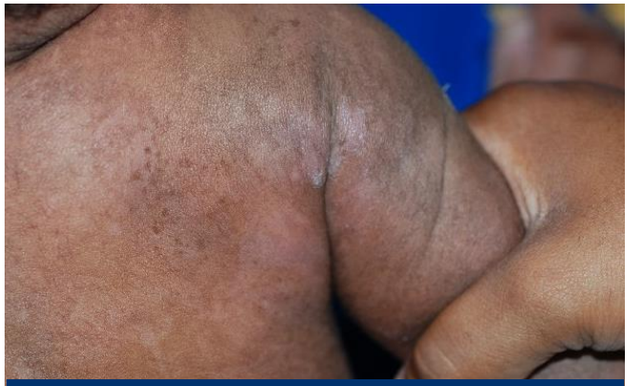
57%
reduction in affected body surface area by month 3

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), Recessive Dystrophic (n=2)

Phase 2b Design (Study 003)

3-Month Double-Blind Treatment Period[1]

SD-101 6% (n=15)

SD-101 3% (n=16)

Placebo (n=17)

Optional Extension (SD-004)

Open-Label Zorblisa (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 cm^2

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

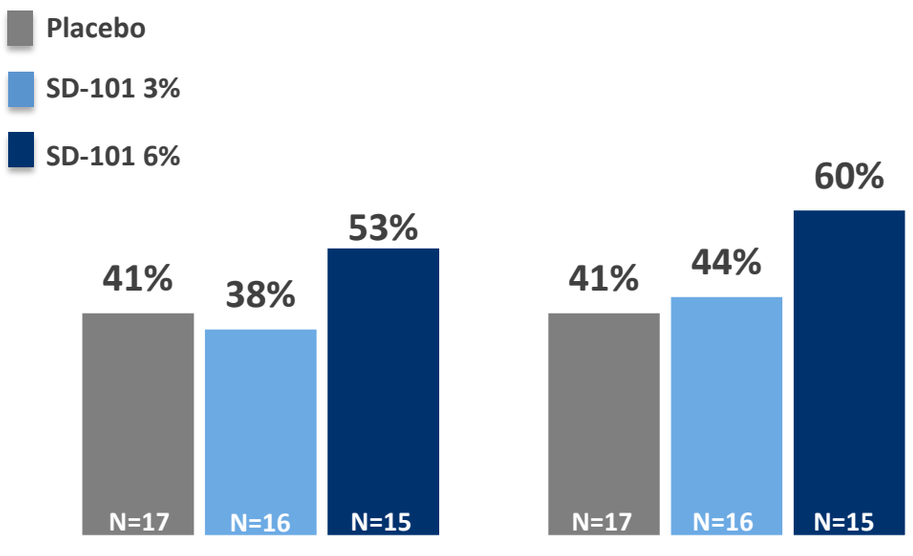
1. Assessments: 0, 14, 30, 60, 90 Days. 2. Initial Disease Severity: Mean target lesion size (cm^2) 14.0 (range 5-39); mean lesional BSA: 19.4% (range 0.4-48%); mean wound age (days): 182 (range 21-1,639). EB Subtypes enrolled: Simplex (n=11), Recessive Dystrophic (n=29), and Junctional (n=8)

Phase 2b Results

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

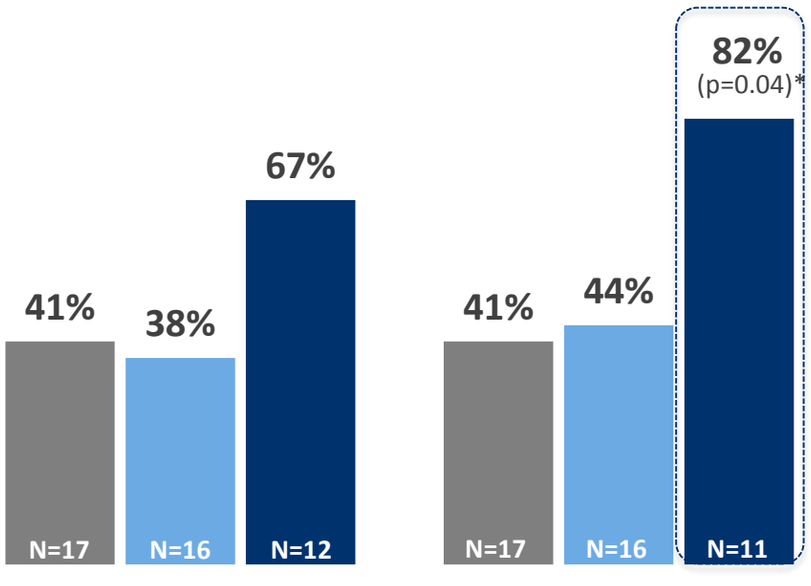
ITT Population (n=48)

Proportion of Complete Target Wound Closure (%)



Evaluable Population[1] (n=45)

Proportion of Complete Target Wound Closure (%)



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

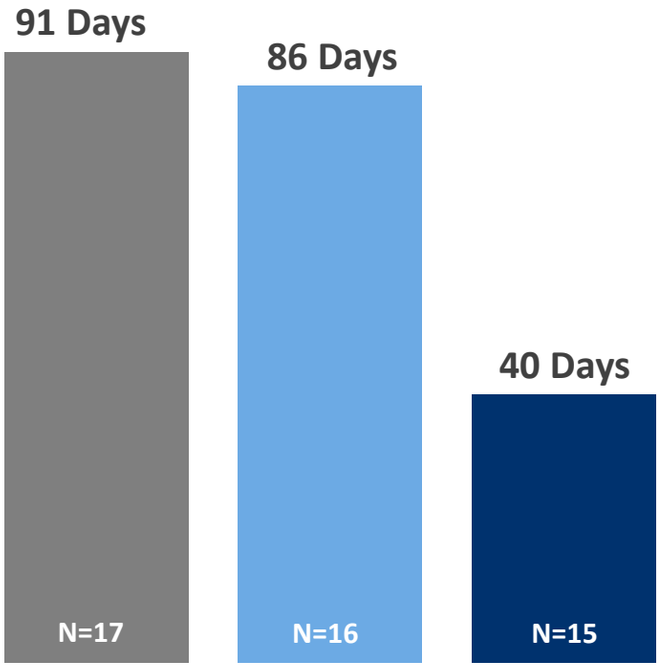
1. Excluded from Evaluable population: 1 patient (due to lost to follow-up), 2 patients (did not have single identified and qualified target lesion). 1 additional patient lost to follow up after Month 1 visit and is excluded from target wound assessment at later time points

Phase 2b Results – Secondary Endpoint

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

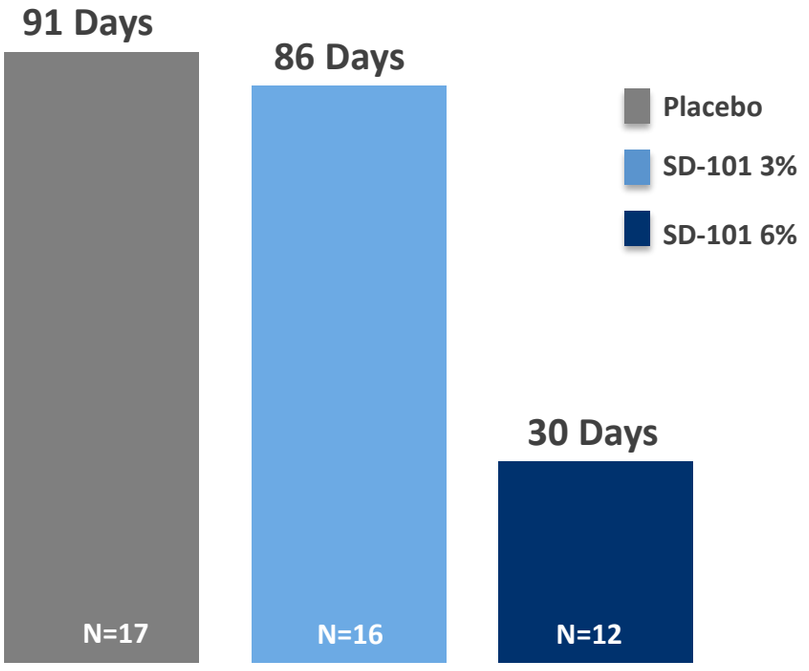
ITT Population (n=48)

Median Time to Wound Closure (Days)



Evaluable Population (n=45)

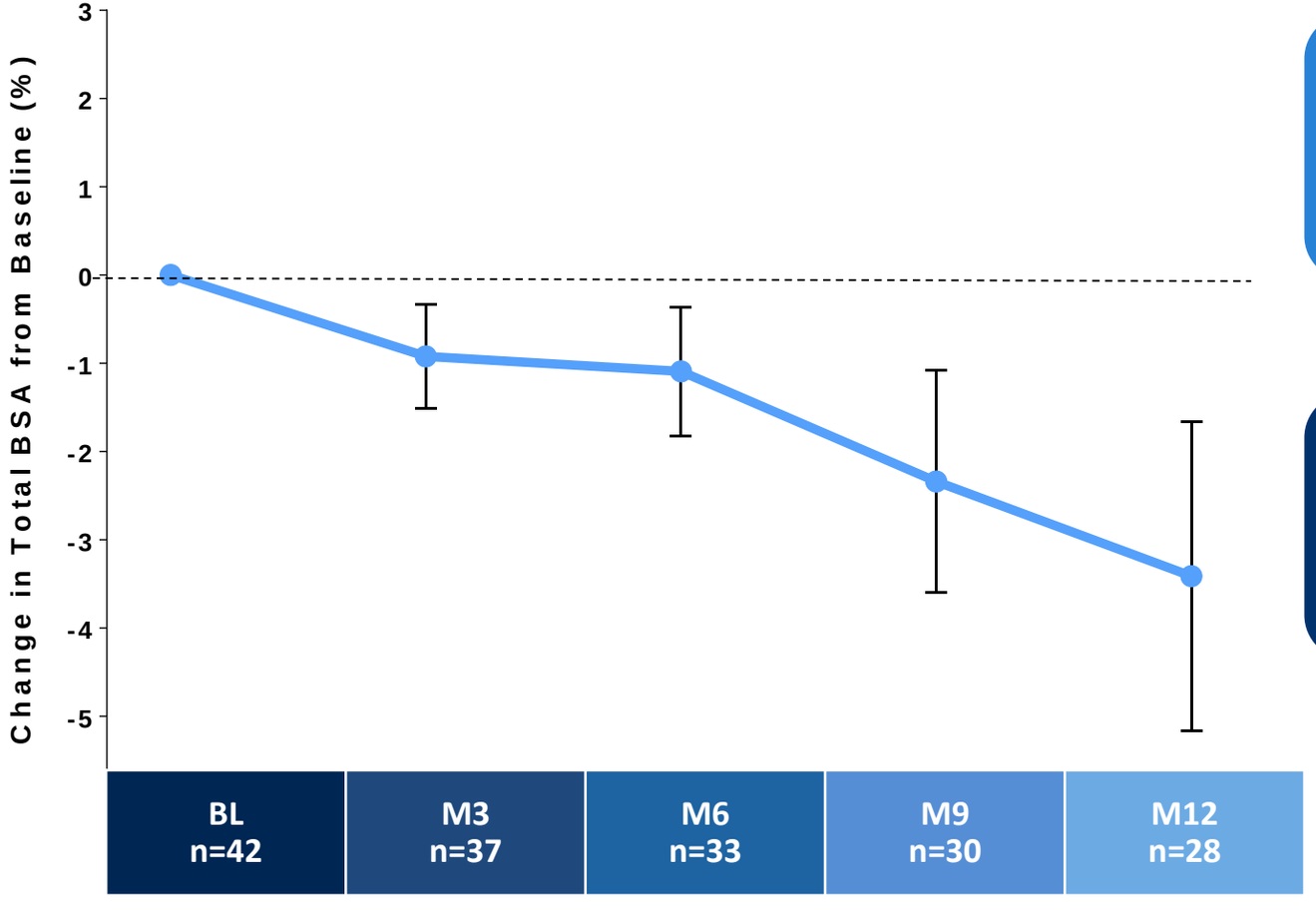
Median Time to Wound Closure (Days)



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



Mean Absolute Change to Month 12 (95% CI): -3.41% (-7.0, 0.2)

Mean Relative Change to Month 12 (95% CI): -12.92% (-37.8, 11.9)

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 Period[1]

SD-101 6%

~150 EB patients (age ≥ 1 month)

Placebo

Optional Extension (SD-006)

Open-Label Zorblisa (6%)

44/44 Patients Have Continued in Open-Label Extension

(Jan. 8, 2015)

Primary Endpoint: Target Wound Healing at Month 2

- US and EU regulatory authorities agreed on primary endpoint
- Baseline wound: Chronic (≥ 21 days), size ≥10 cm^2

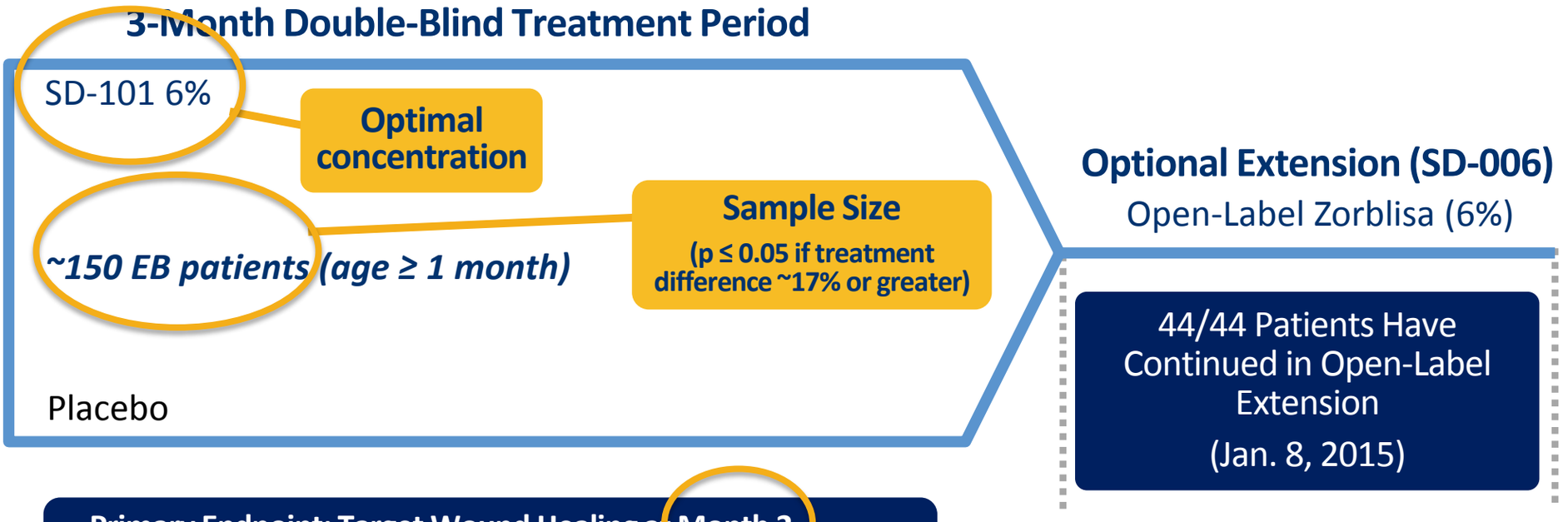
Secondary Endpoints Include

- 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



Primary Endpoint: Target Wound Healing at Month 2

- US and EU regulatory authorities agreed on primary endpoint
- Baseline wound: Chronic (≥ 21 days), size ≥10 cm^2

Secondary Endpoints Include

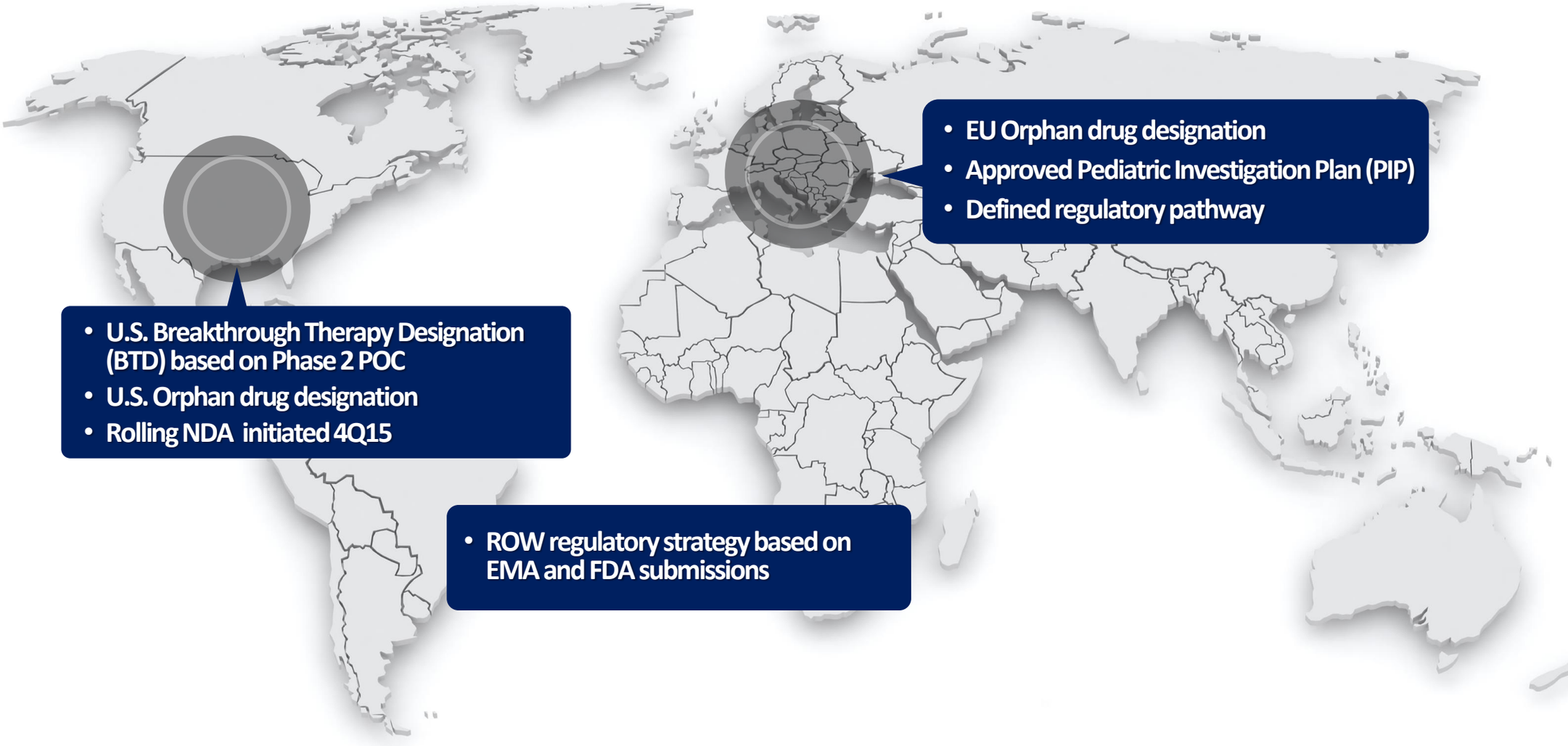
- Time to target wound closure
- Change in Body Surface Area (BSA) of lesions and blisters

Increases Ability to Distinguish SD-101 vs. Placebo[1]

1. Complete target wound closure in patients with target wounds ≥ 10 cm^2 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

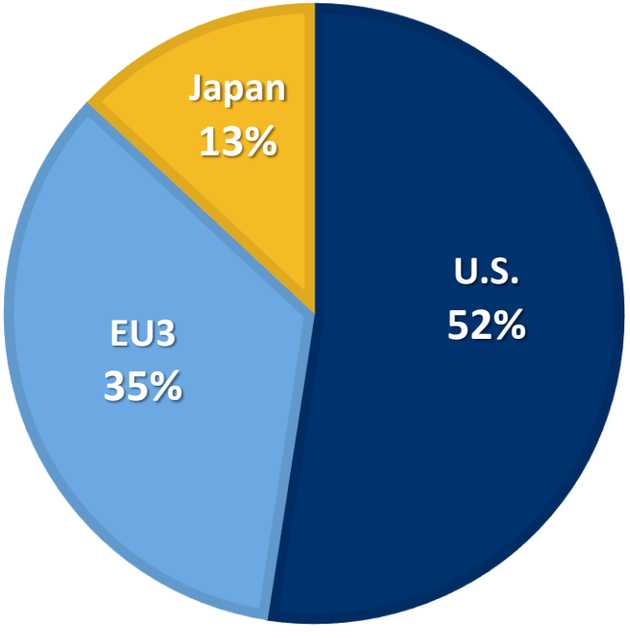


$1B+ Commercial Potential

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

Diagnosed EB Patients by Geography

(US, EU3, Japan)



Significant Unmet Clinical Need

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

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

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 WW[1]
- ~$700M+ Global Pompe ERT sales in FY14[2]

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

Pompe Patient Perspectives

Very Significant Unmet Need Despite Availability of Currently Marketed Therapy

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]

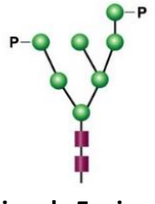
Antibody titers shown to affect treatment outcomes[4,5]

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

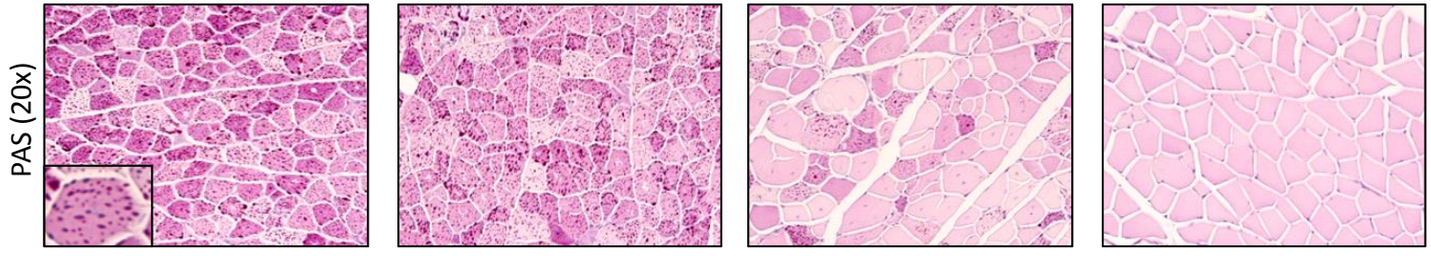
1Khanna et al., PLoS ONE, 2012; 2Zhu et al., Amer. Soc. Gene Therapy, 2009 June; 3Banati et al., Muscle Nerve, 2011 Dec.; 4Banugaria et al., Gen. Med., 2011 Aug.; 5de Vries et al., Mol Genet Metab., 2010 Dec.

Amicus Therapeutics

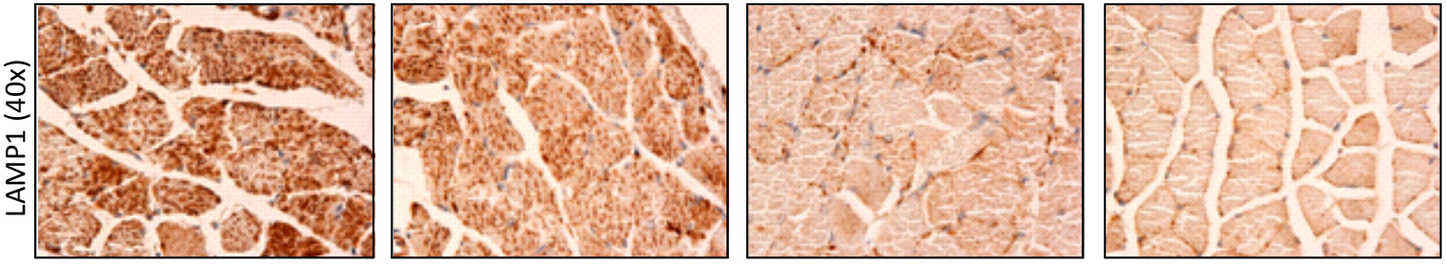
Preclinical Proof-of-Concept

ATB200 + Chaperone Results in Improved Substrate Clearance in Preclinical Models[1]

PAS-glycogen staining in Quadriceps



LAMP1 Immunohistochemical staining in Soleus

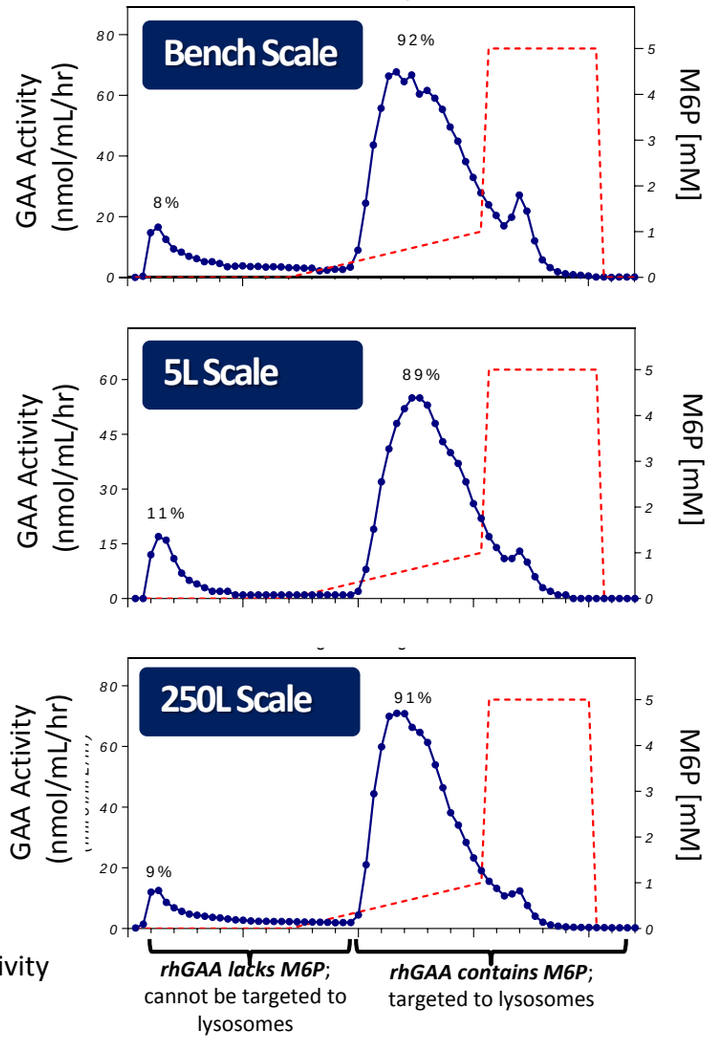


1. Following 2 doses of 20mg/kg alglucosidase alfa or ATB200 + AT2221 in Gaa KO mice, skeletal muscles evaluated for glycogen clearance and proliferated lysosomes. Treatment with alglucosidase alfa modestly reduced glycogen or proliferated lysosomes while ATB200, co-administered with AT2221 significantly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

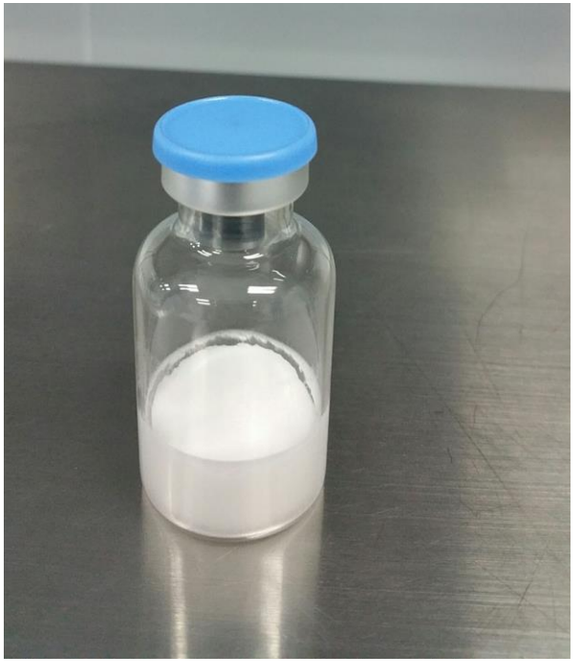
Biologics Manufacturing Capabilities

Optimized Glycosylation and Key Quality Attributes Maintained Through Scale Up

CI-MPR Receptor Chromatography



Lyophilized Vial of ATB200



Clinical Study in Pompe Patients

Study Design Supported by US and EU Regulators

Stage 1 (Single Ascending Dose)

Single Dose ATB200 Every Other Week

ATB200 5 mg/kg → ATB200 10 mg/kg → ATB200 20 mg/kg

Stage 2 (Multiple Ascending Dose)

Fixed Dose ATB200 + Chaperone (AT2221) Every Other Week

ATB200 20mg/kg + AT2221 (Low Dose) 3 doses → **ATB200 20mg/kg + AT2221 (High Dose)** 3 doses

Long-Term Open Label Extension

Fixed Dose ATB200 + Chaperone (AT2221) Every Other Week

Assessments:

- Plasma PK (Enzyme Activity & Total protein)
- Safety/Tolerability
- Antibodies
- Infusion-Associated Reactions
- Pharmacodynamics (OLE)

Financial Summary

Strong Balance Sheet to Invest in Rare Disease Pipeline

Strong Balance Sheet

Cash Position Provides Runway Under Current Operating Plan into 1H17

Financial Position	December 31, 2015
Current Cash:	$214M
Current Debt	$50M
FY16 Net Cash Spend Guidance:	$135M-$155M
Cash Runway	Mid-2017
Capitalization	
Shares Outstanding	125,027,034

Key Drivers of Value

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

Fabry

- Migalastat Personalized Medicine (Small Molecule)
- MAA Submitted
- CHMP Opinion Anticipated Early 2016
- Prepared for EU Launch*

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

Amicus Therapeutics®