Amicus Therapeutics®

Cowen and Company 36th Annual Health Care Conference

Bradley L. Campbell, President and Chief Operating Officer

March 9, 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 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

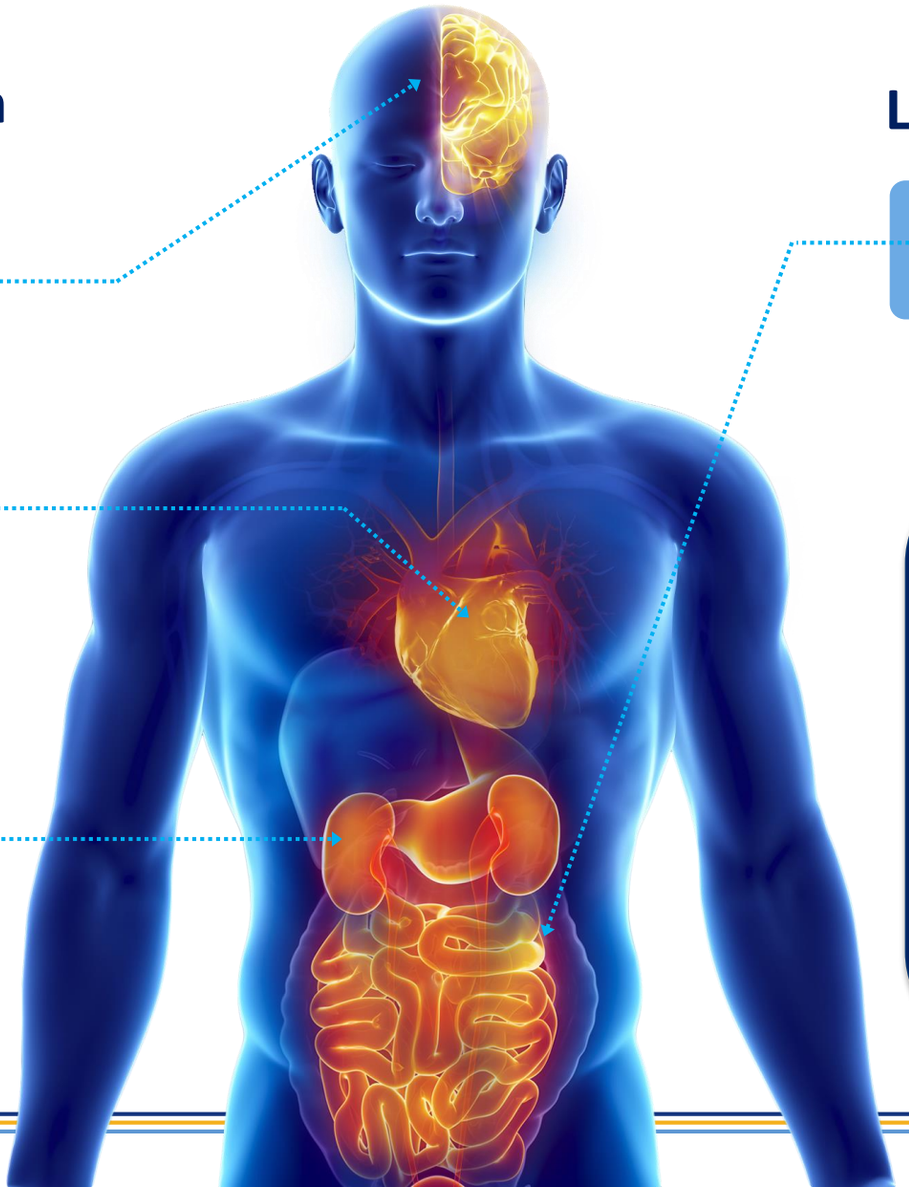
Migalastat Personalized Medicine for Fabry Disease

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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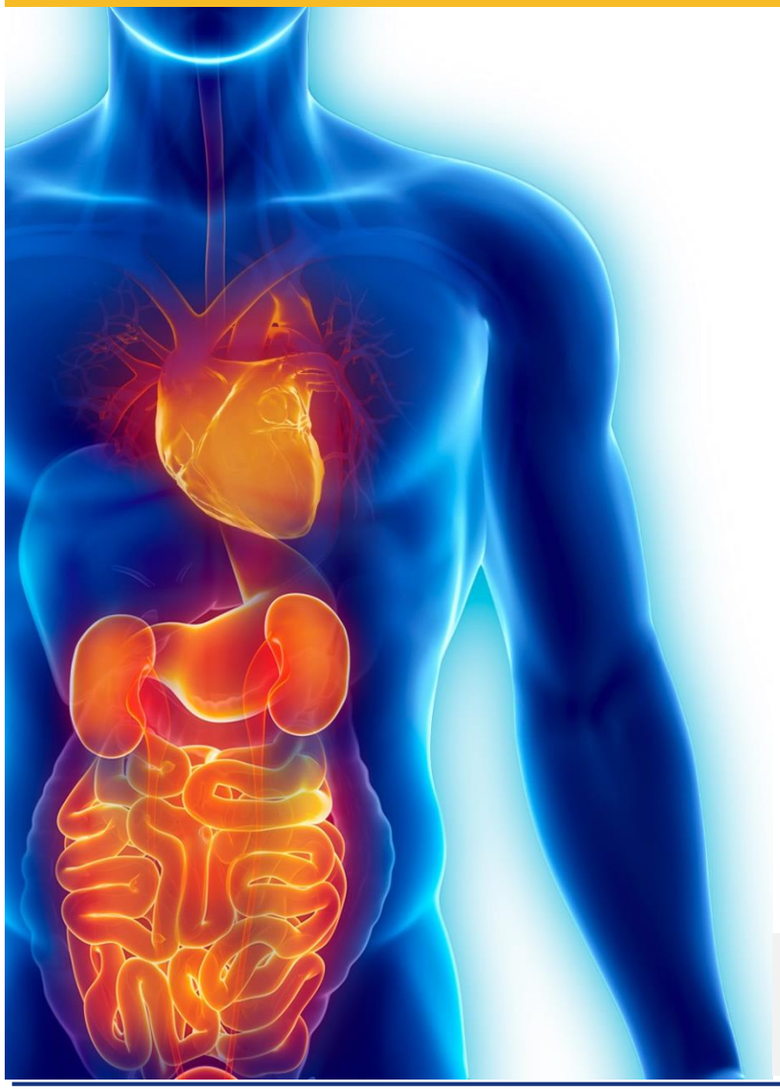
Summary of Clinical Data

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[1])*
Plasma Lyso Gb-3 (Study 011[2,1] and 012[3])*

Stability of Kidney Function
Estimated Glomerular Filtration Rate (eGFR) and Measured GFR (Study 011[4] and Study 012[4,3])

Reduction in Cardiac Mass
Left Ventricular Mass Index (LVMI) (Study 011[2] and 012)*

Improvement in GI Symptoms
Gastrointestinal Symptoms Rating Scale (GSRS) (Study 011[1])*

Low Rate of Fabry-Associated Clinical Events
Renal, Cardiac and Cerebro-Vascular Events (Study 012[3])

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

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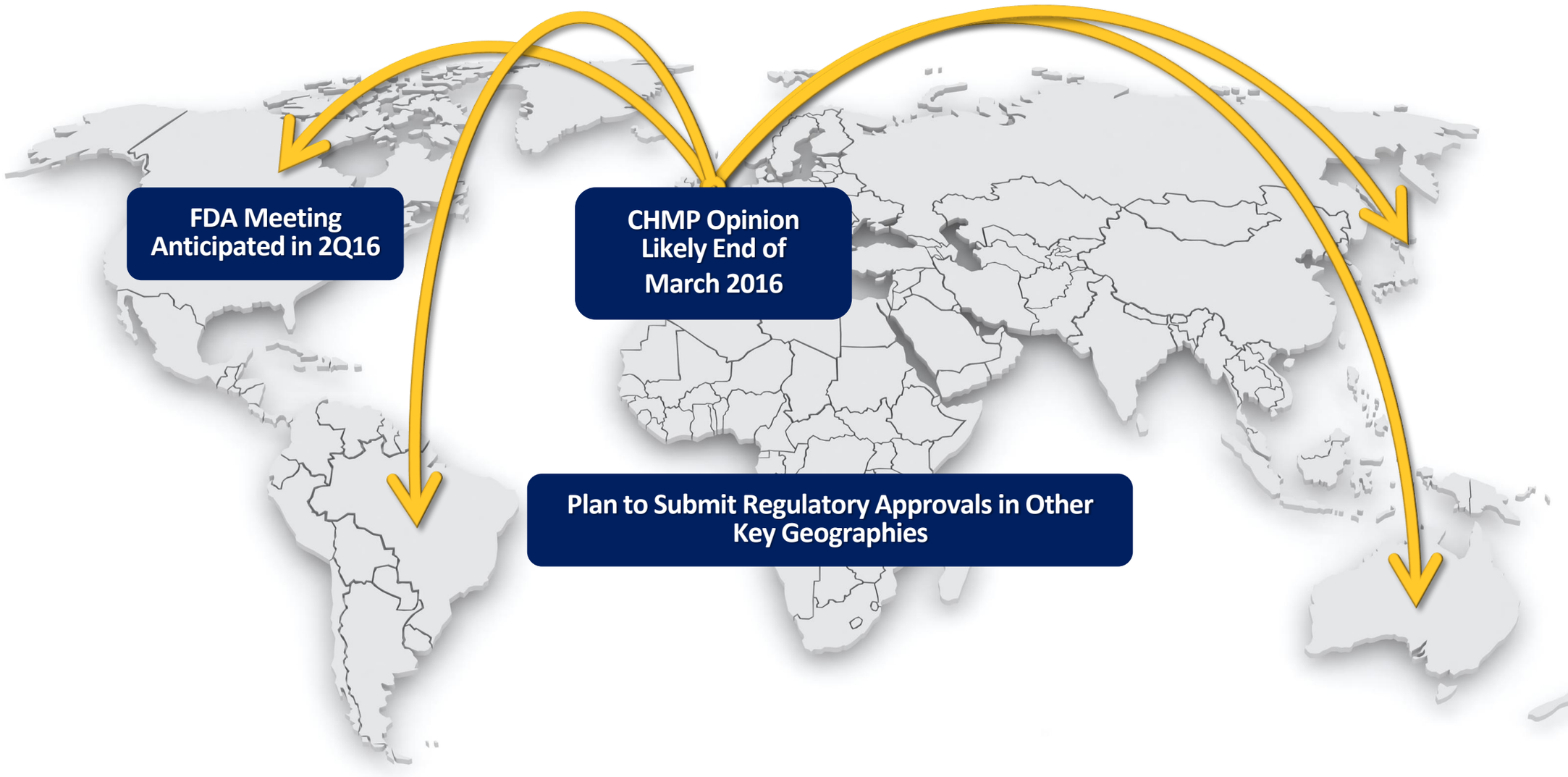
Global Regulatory Strategy

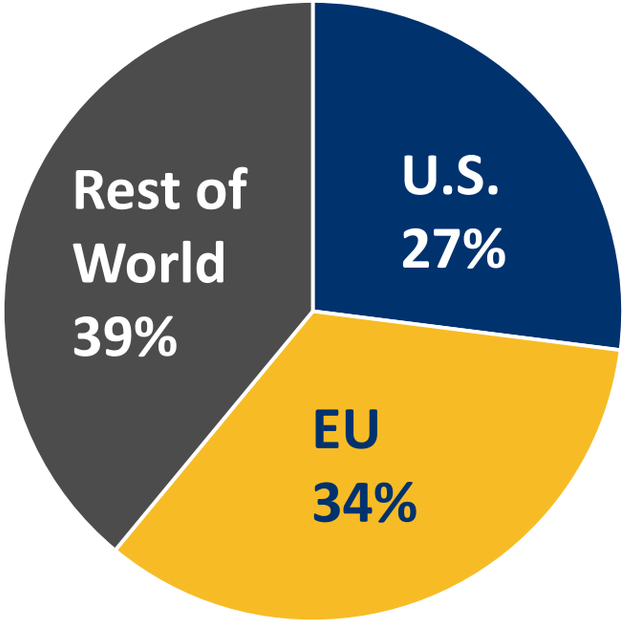
EU Approval Will Lay the Foundation to Address ~70% of Global Fabry Market



Fabry Market Today

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

$1.2B in FY15 ERT Sales[1]



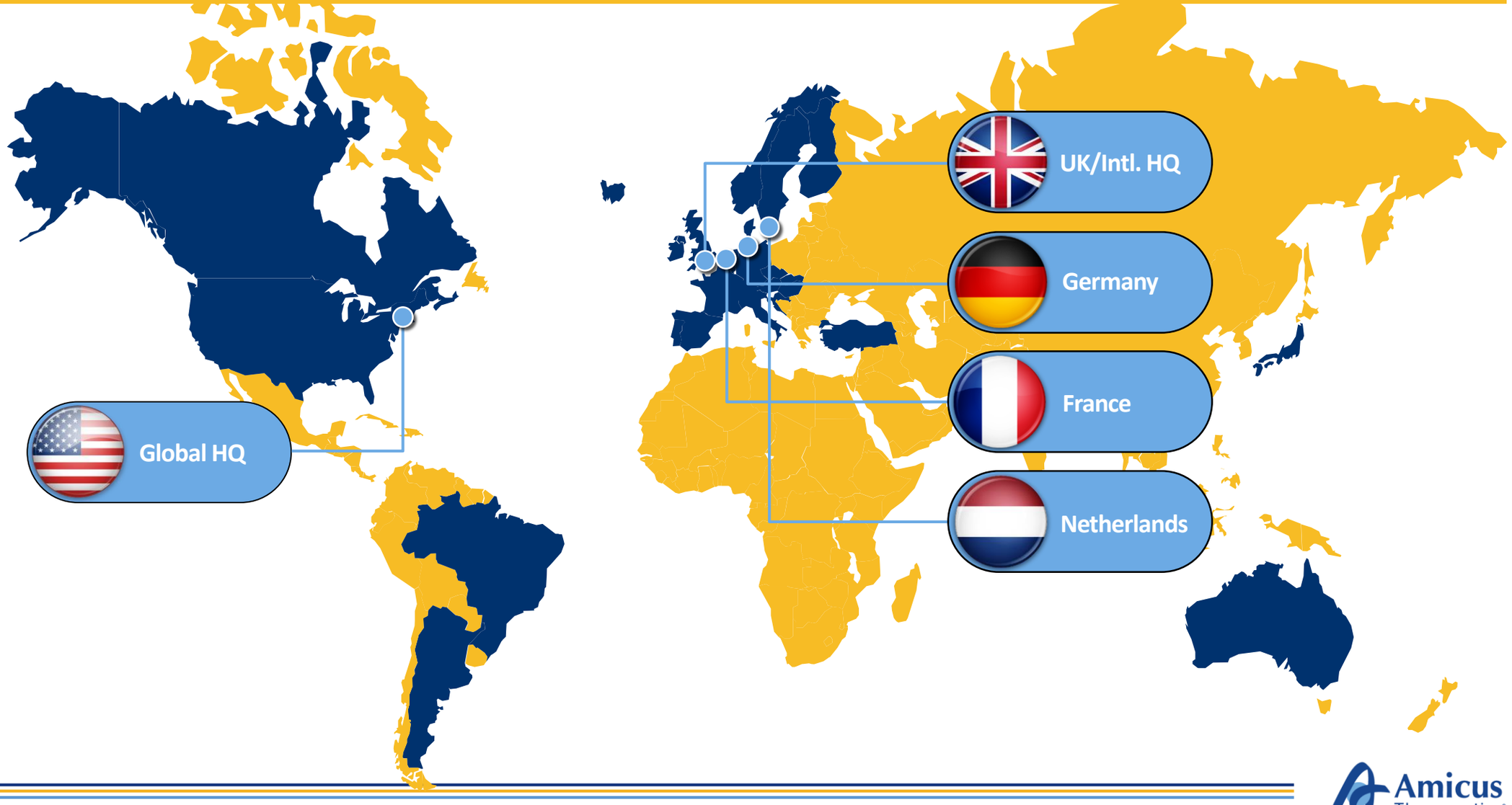
- 40-50% of Diagnosed Patients Not on ERT Today
- Market Continues to Grow > 10% / Year
- ERT Infused Once Every 2 Weeks

- ***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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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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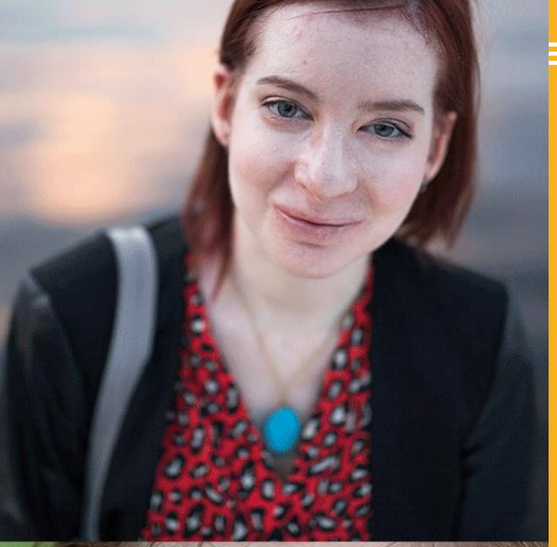
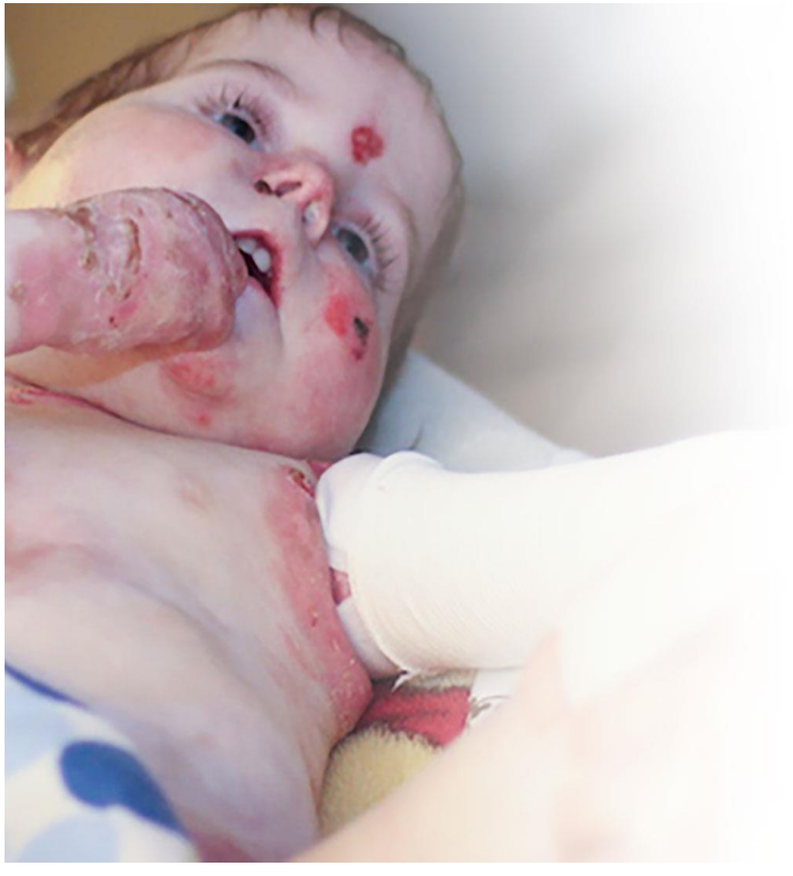


SD-101 for Epidermolysis Bullosa (EB)

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

EB Disease Overview

Rare, Devastating, Connective Tissue Disorder with No Approved Treatments

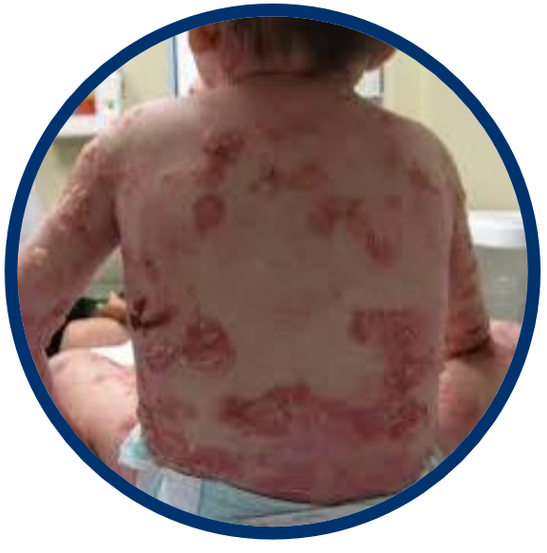


- Multiple genes cause disease which results in fragility of skin and can affect internal organs
- Diagnosed from infancy to adulthood
- Severe blistering, open wounds, and scarring in response to minor friction to the skin
- Disfiguring, excruciatingly painful, and can be fatal
- Given the lack of approved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

Three Major EB Types Represent ~99% of EB Population

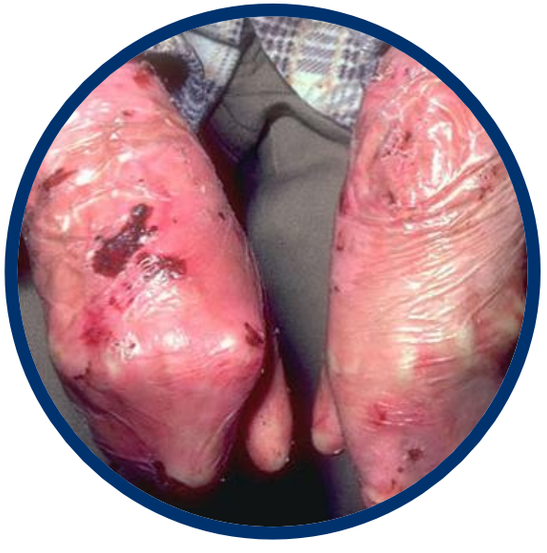
Multiple Types…Single Devastating and Fatal Genetic Disorder

Simplex



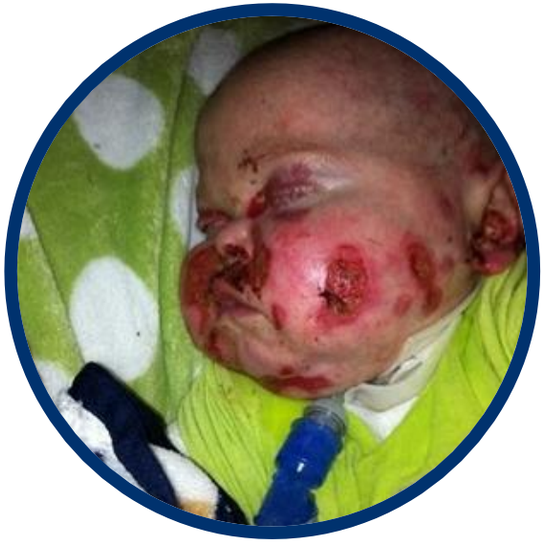
~75% of EB Population

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

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 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 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

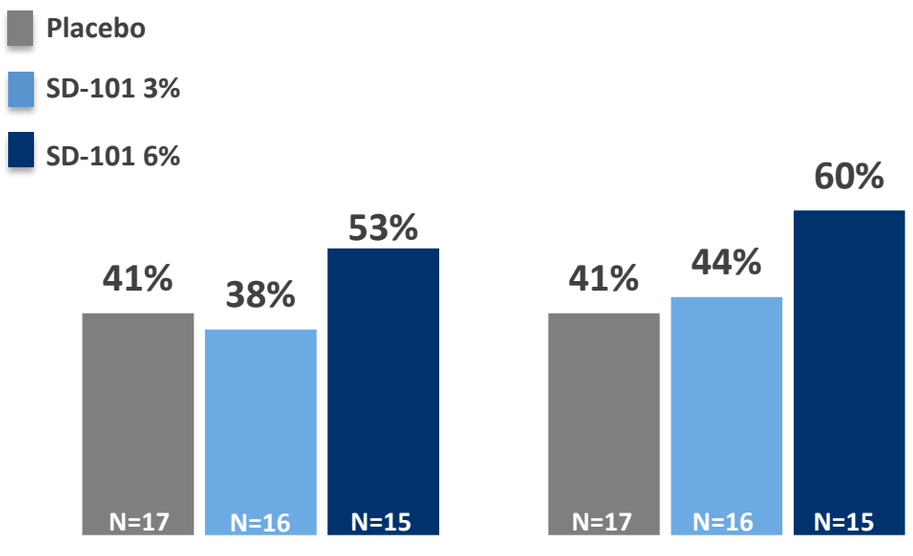
1. Assessments: 0, 14, 30, 60, 90 Days. 2. Initial Disease Severity: Mean target lesion size (cm^2) 14.0 (range 5-39); mean lesional BSA: 19.4% (range 0.4-48%); mean wound age (days): 182 (range 21-1,639). EB types enrolled: Simplex (n=11), Dystrophic (n=29), and Junctional (n=8)

Phase 2b Results

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

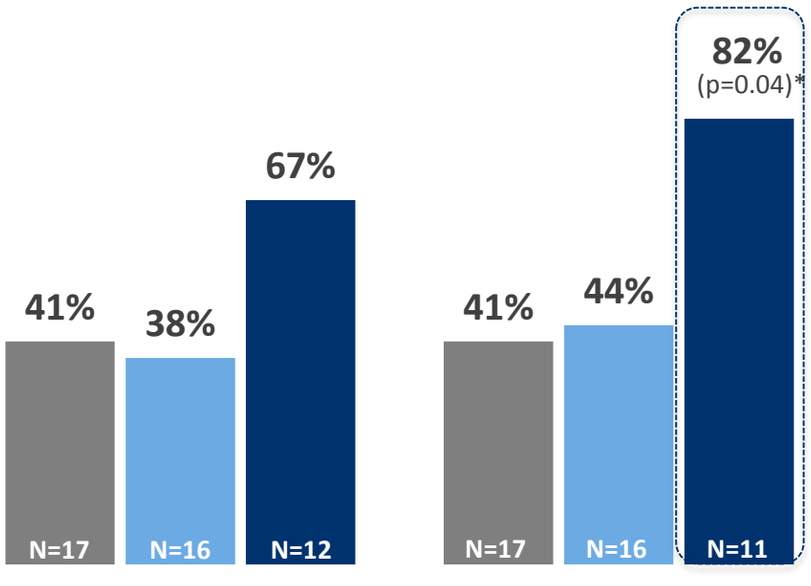
ITT Population (n=48)

Proportion of Complete Target Wound Closure (%)



Evaluable 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 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 SD-101 6%

53/53 Patients Have Continued in Open-Label Extension

(Feb. 25, 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 cm^2

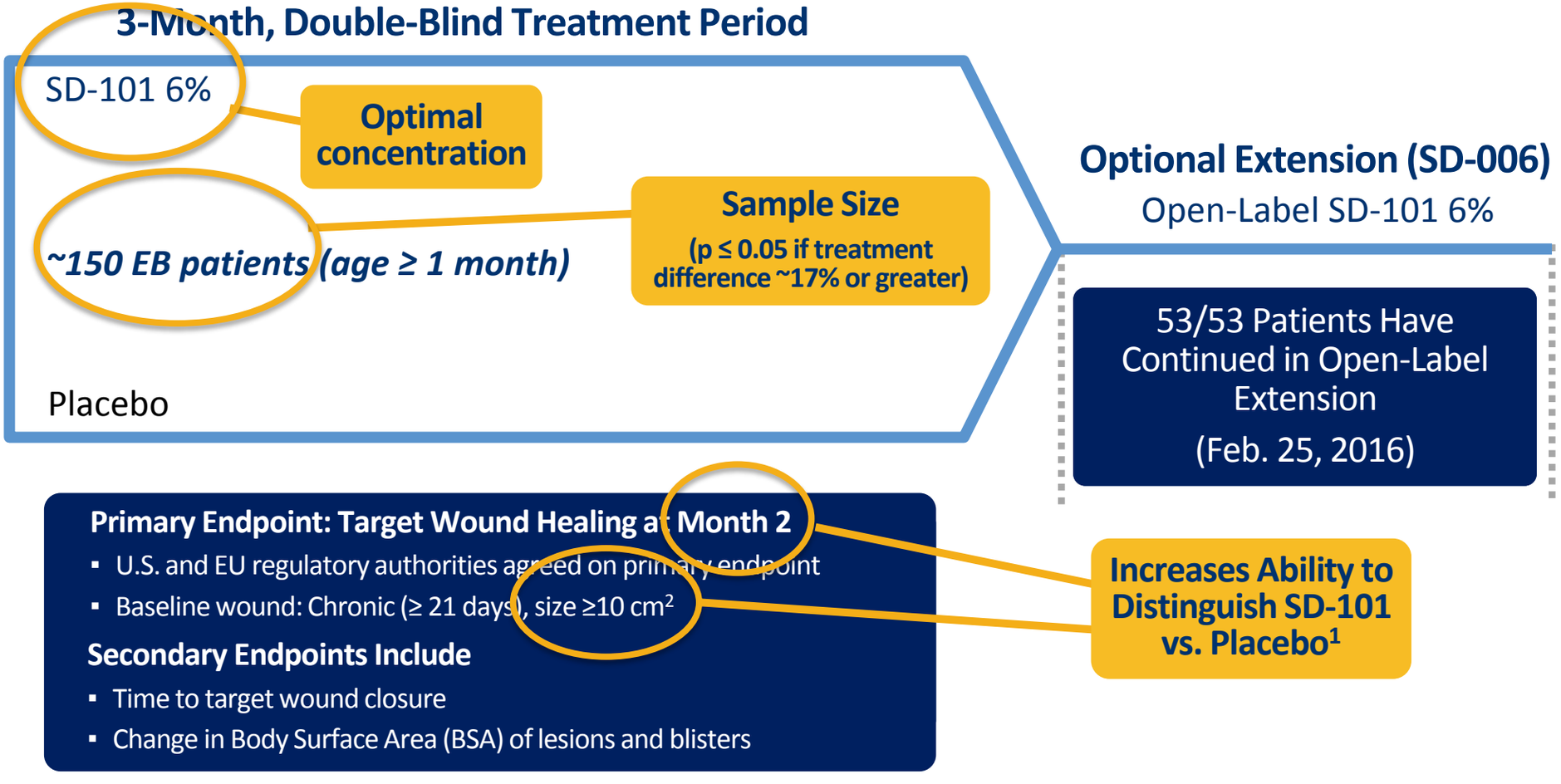
Secondary Endpoints Include

- 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)

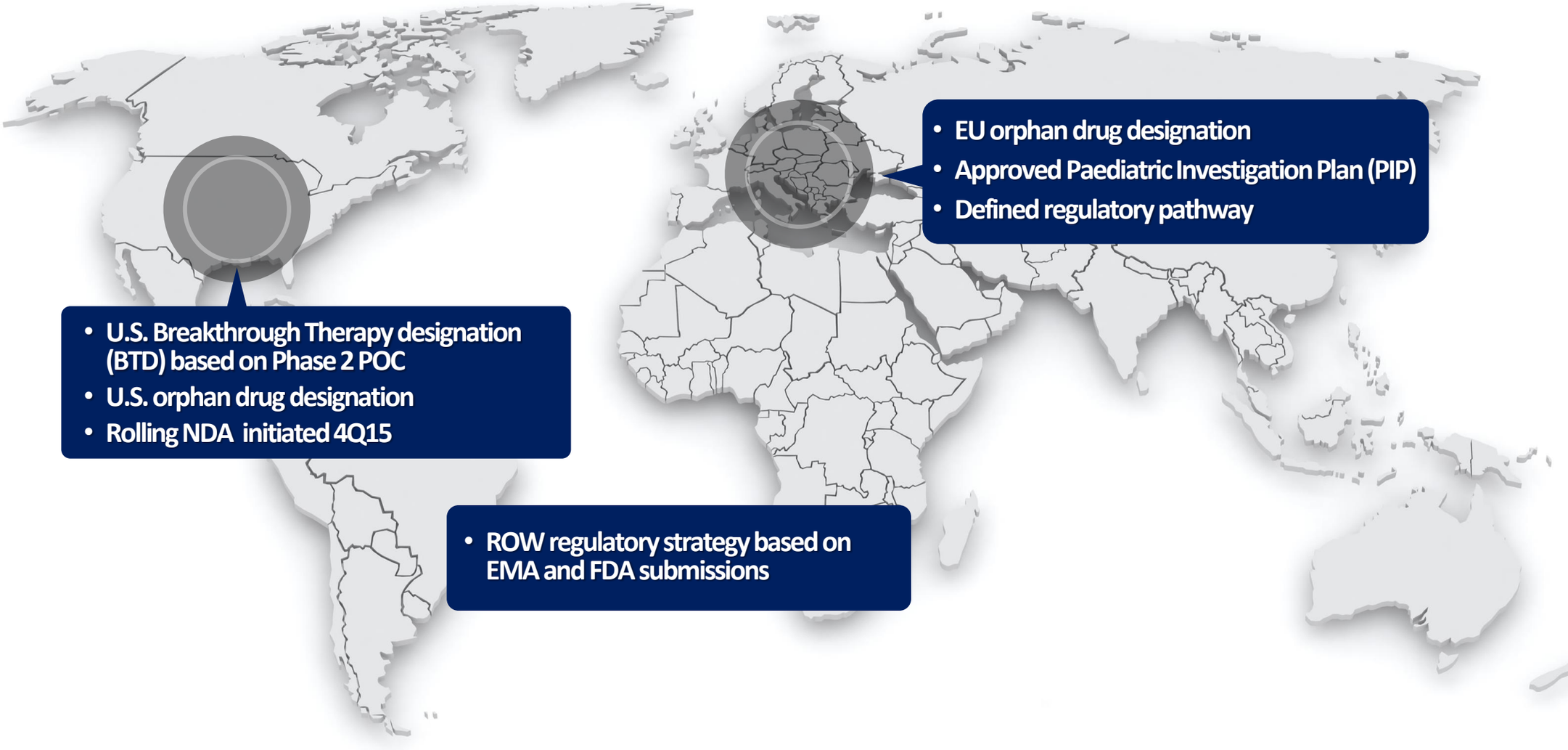
Study Design Incorporates Key Learnings from Phase 2b Study



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

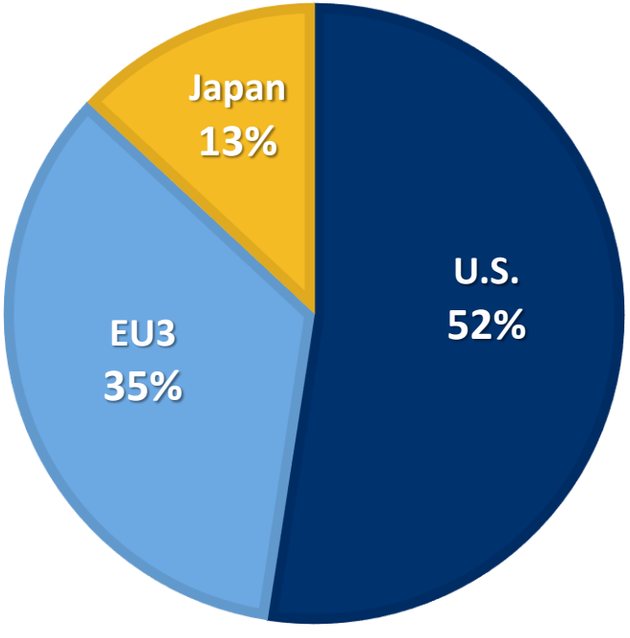


$1B+ Commercial Potential

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

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

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 blood[1]

Protein Aggregation

CHART™
CHAPERONE-ADVANCED REPLACEMENT THERAPY

Tolerability / Immunogenicity

Infusion-associated reactions in >50% of late-onset patients[3]

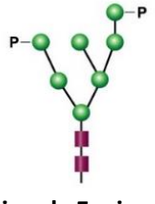
Antibody titers shown to affect treatment outcomes[4,5]

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.

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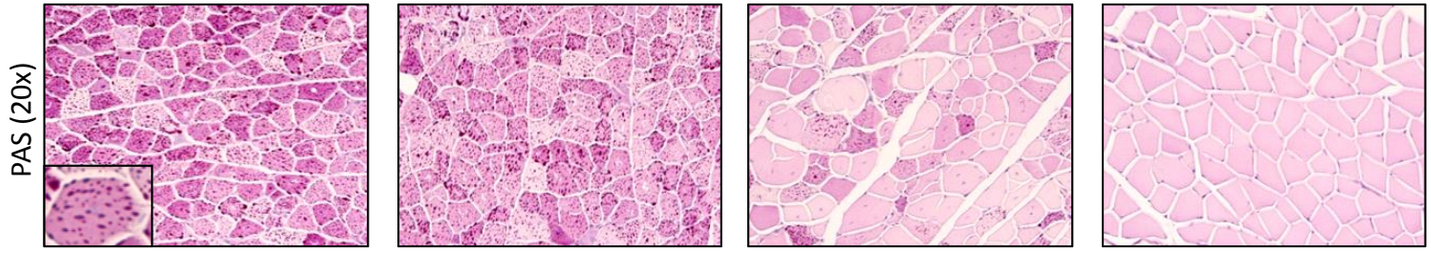
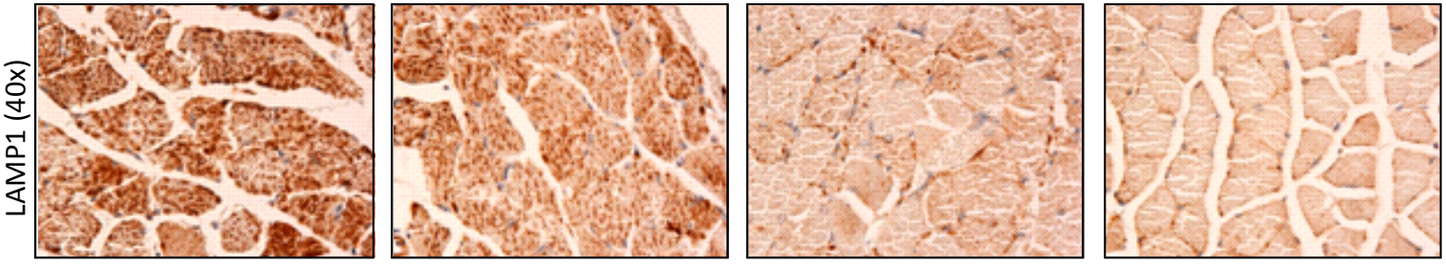
Preclinical Proof of Concept

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

PAS-glycogen staining in Quadriceps



LAMP1 Immunohistochemical staining in Soleus

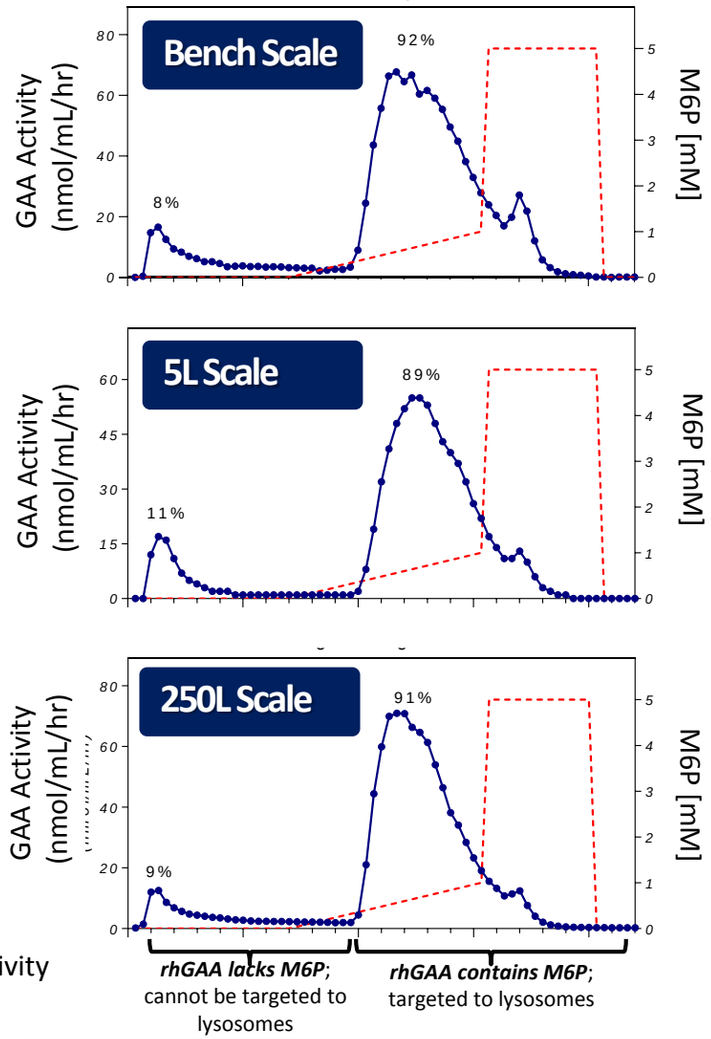


1. Following 2 doses of 20mg/kg alglucosidase alfa or ATB200 + AT2221 in Gaa KO mice, skeletal muscles evaluated for glycogen clearance and proliferated lysosomes. Treatment with alglucosidase alfa modestly reduced glycogen or proliferated lysosomes while ATB200, co-administered with AT2221 significantly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

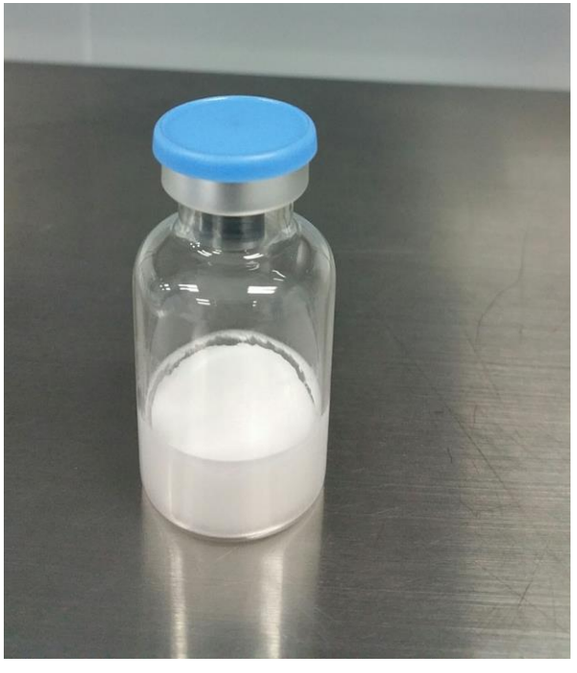
Biologics Manufacturing Capabilities

Optimized Glycosylation and Key Quality Attributes Maintained Through Scale Up

CI-MPR Receptor Chromatography



Lyophilized Vial of ATB200



Clinical Study in Pompe Patients

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
- Efficacy (Long-Term Extension)

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

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Thank You

Amicus Therapeutics®