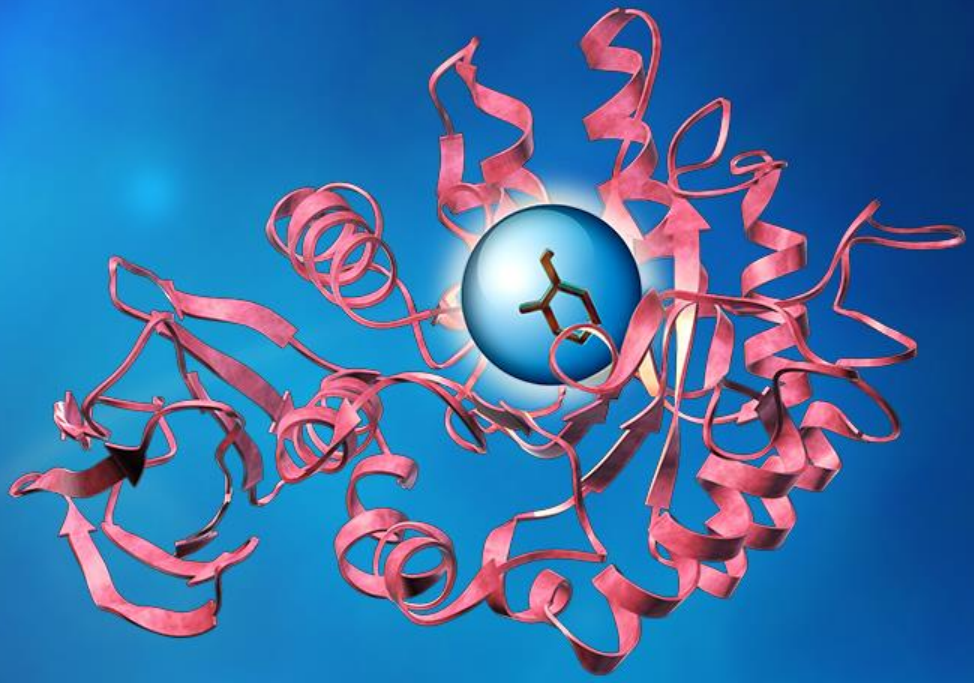
Amicus Therapeutics



Goldman Sachs US Emerging & SMID Cap Growth Conference

Chip Baird, Chief Financial Officer

November 19, 2015

at the forefront of therapies for rare and orphan diseases

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 September 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.

Company Mission

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

Advanced Product Pipeline

PRODUCT/PLATFORM	DISCOVERY	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	Regulatory
Fabry Franchise						
Migalastat Pharmacological Chaperone Monotherapy & Combination w/ ERT						
Galafold® (migalastat) Personalized Medicine						
Co-Administration with ERT						
Novel ERT						
Epidermolysis Bullosa						
Zorblisa (SD-101) Proprietary Topical Skin Treatment						
Novel ERTs						
Pompe Disease ATB200 (rhGAA) + Chaperone						
Other LSDs						

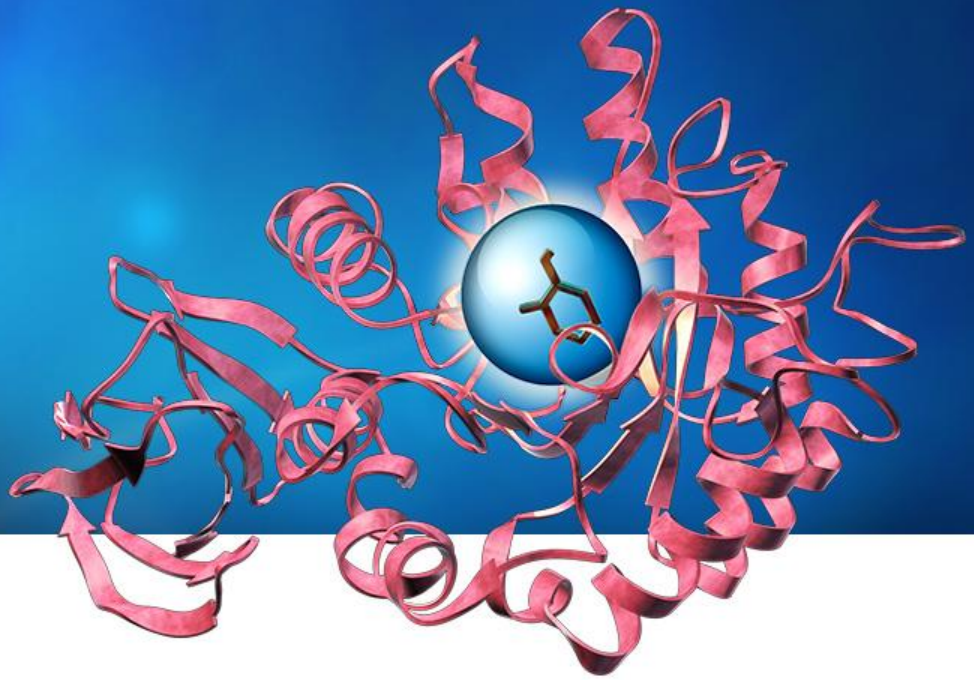
Amicus Therapeutics

Corporate and Program Highlights

Focus on Execution Around 4 Strategic Priorities

- EU Regulatory Process on Track for Galafold™ (migalastat HCl) for Fabry
- Working to determine optimal U.S. approval pathway for migalastat
- Planning to initiate Phase 1/2 study of novel ERT (ATB200 + chaperone) for Pompe
- Significant momentum for Zorblisa™ (SD-101) Phase 3 study - rolling NDA initiated

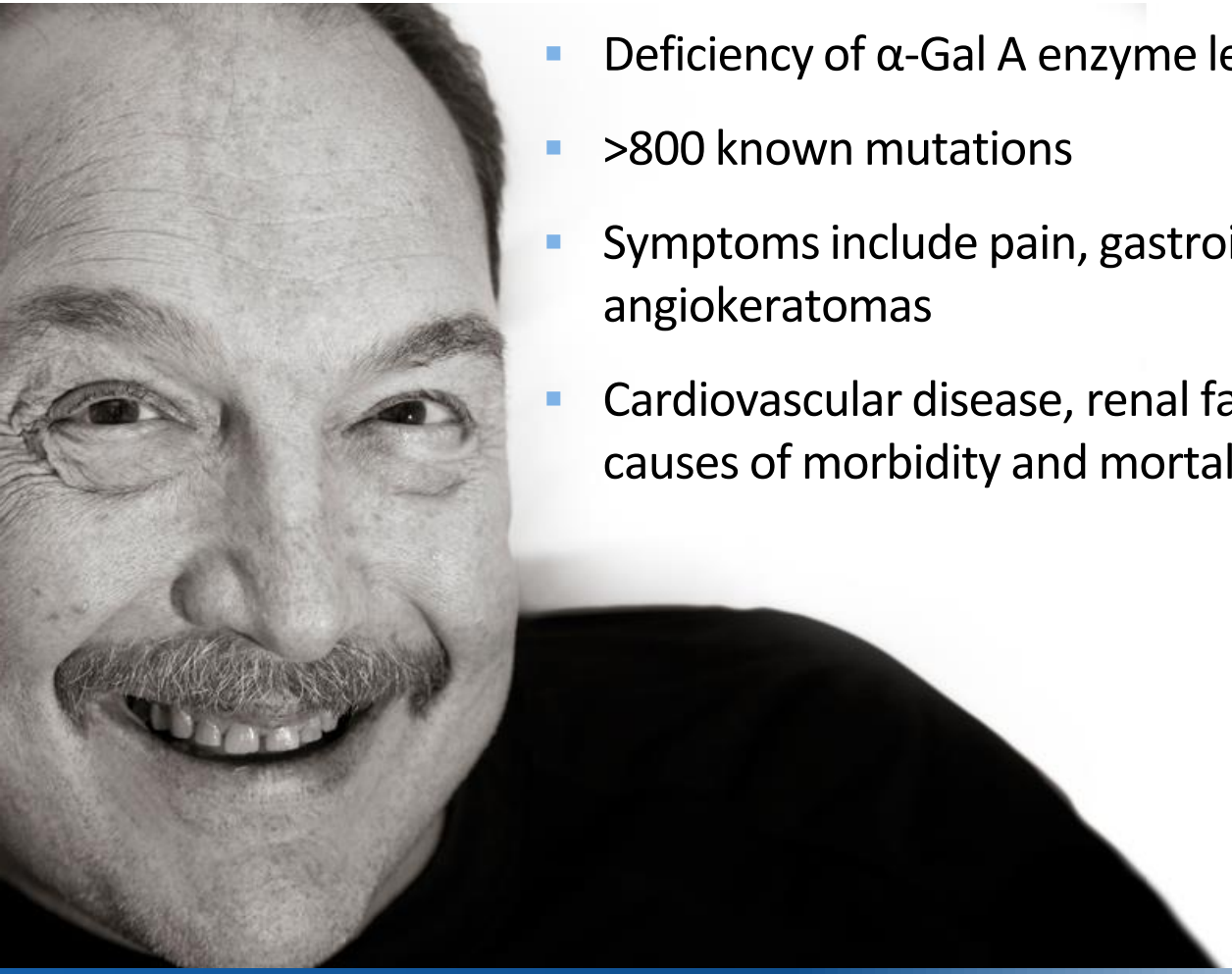
Amicus Therapeutics



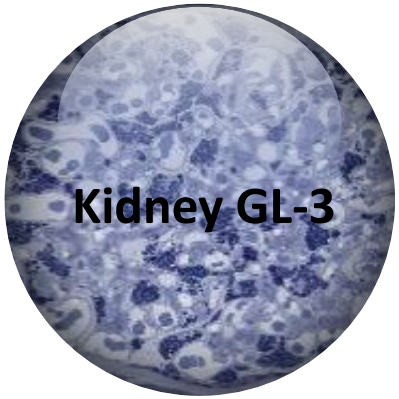
Galafold™ Personalized Medicine for Fabry Disease

Fabry Disease Overview

Fatal Lysosomal Storage Disorder with Significant Unmet Needs



- Deficiency of α-Gal A enzyme leading to GL-3 accumulation
- >800 known mutations
- Symptoms include pain, gastrointestinal problems, angiokeratomas
- Cardiovascular disease, renal failure, and stroke are leading causes of morbidity and mortality

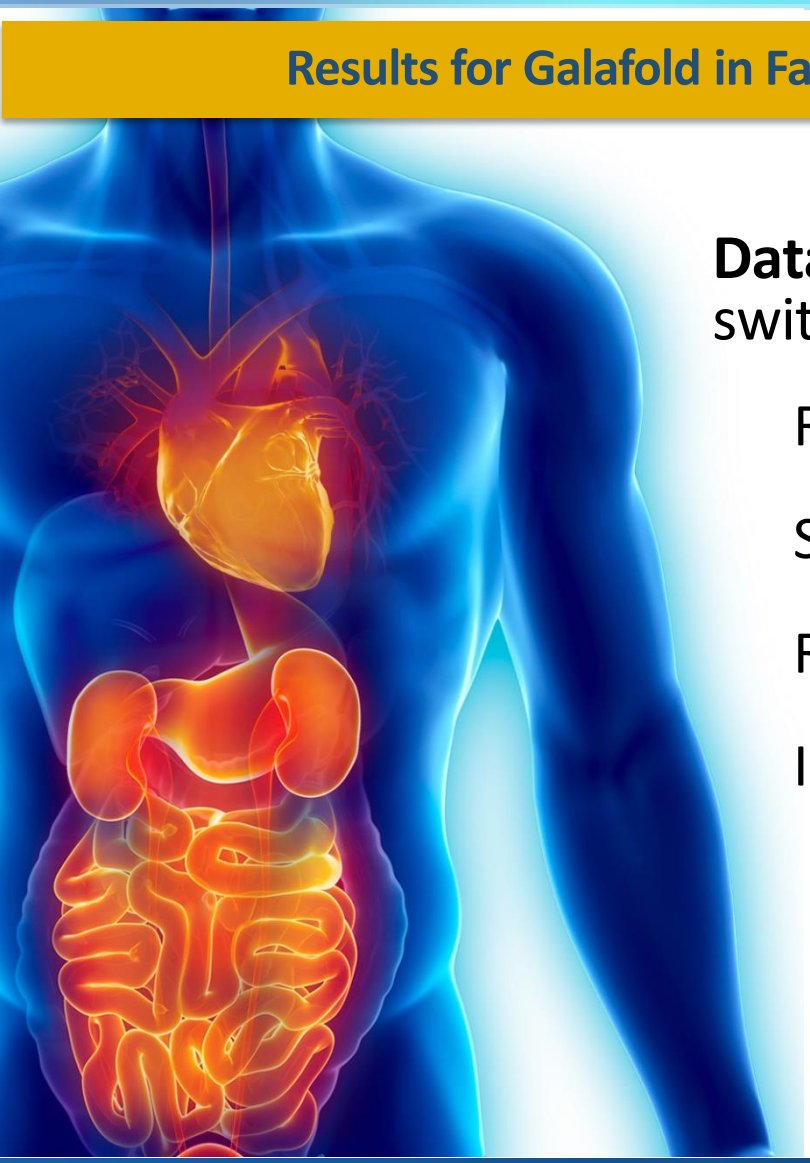


[1]Mehta 2009, [2]Waldek 2009, [3]Patel 2011, [4]Kampmann 2008, [5]Germain 2013

Amicus Therapeutics

Two Global Registration Studies

Results for Galafold in Fabry Patients with Amenable Mutations



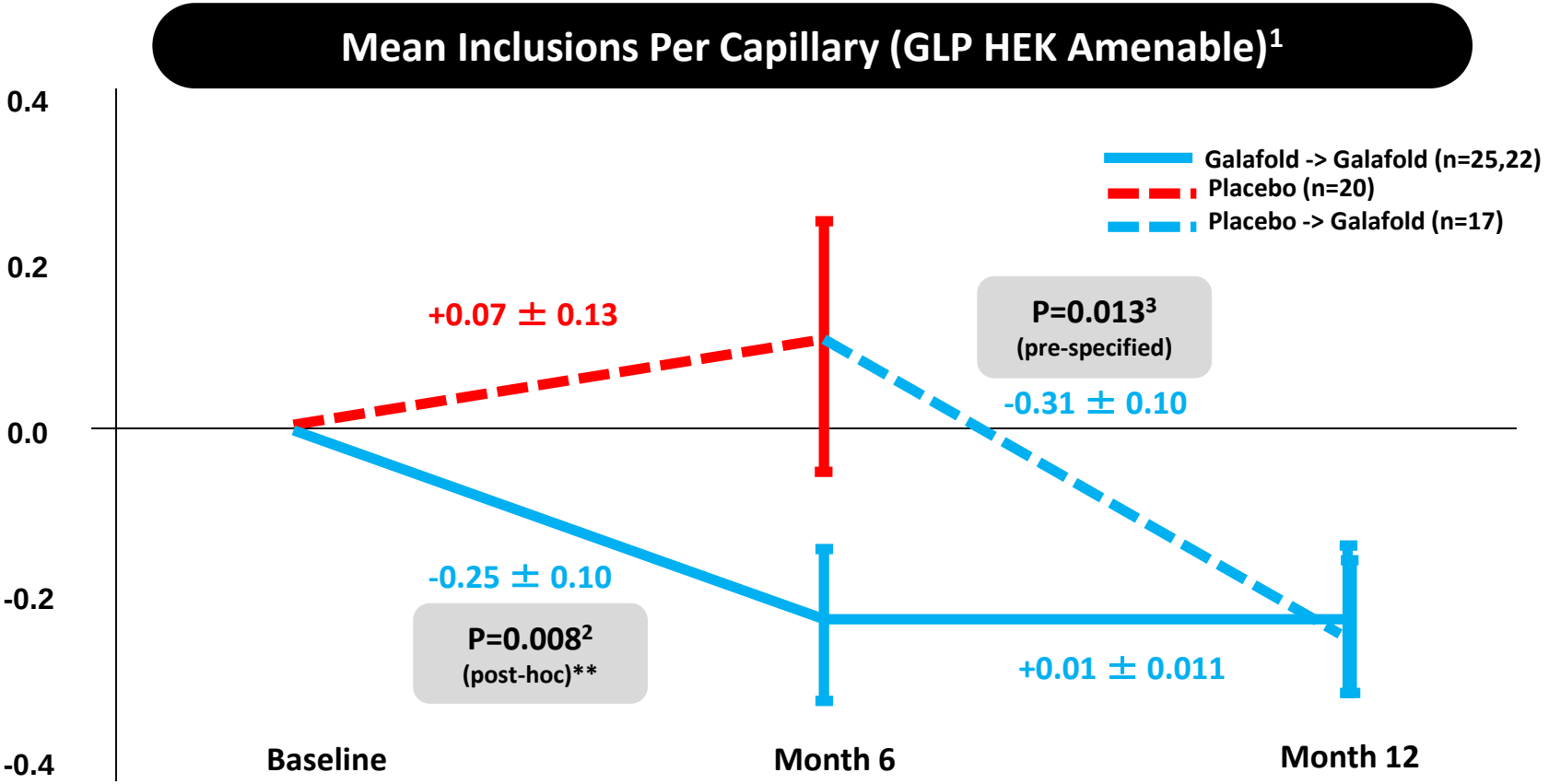
Data in ERT-naïve (Study 011) and ERT switch (Study 012) patients show:

- Reduction in **disease substrate**
- Stability of **kidney function**
- Reduction in **cardiac mass** (LVMi)
- Improvement in **gastrointestinal symptoms**[1]

[1] Study 011 (not evaluated in Study 012)

Phase 3 (Study 011) Primary Efficacy Endpoint

Statistically Significant Reduction in Disease Substrate (Kidney IC GL-3)*



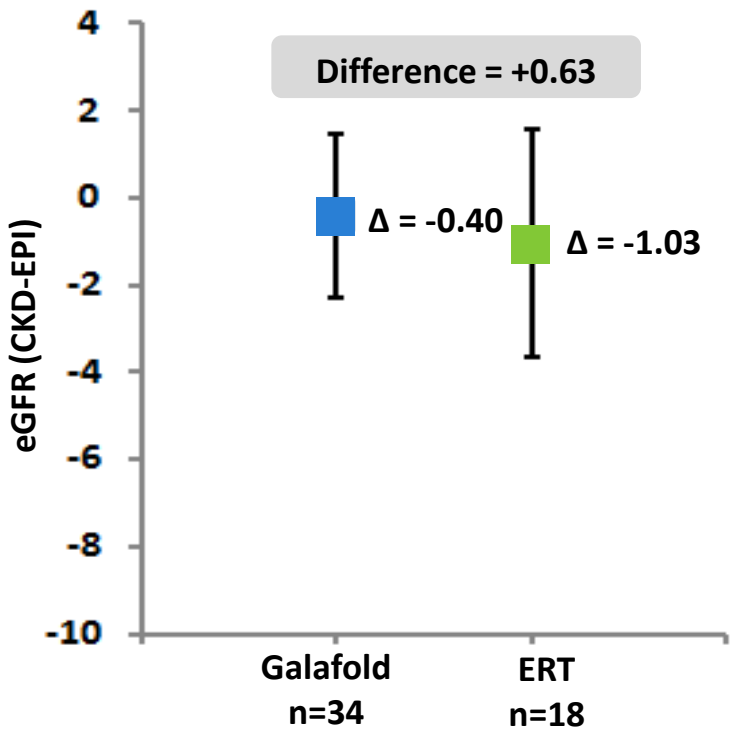
*All patients with evaluable paired biopsies and amenable GLA mutations in GLP-validated HEK assay – post hoc at month 6 and pre-specified at month 12 [1]Data points are baseline corrected; represent mean ± standard error (SEM) change from baseline in the mean number of GL-3 inclusions per capillary after 6 months of treatment with Galafold or placebo. [2]Analysis of covariance (ANCOVA) model with covariate adjustment for baseline value and factors for treatment group and treatment by baseline interaction. P-value corresponding to least-square mean difference between Galafold and placebo is displayed. [3]MMRM Pbo change M6 to M12. **Pre-specified Stage 1 primary analysis was a responder analysis; migalastat 41%, placebo 28%, p=NS

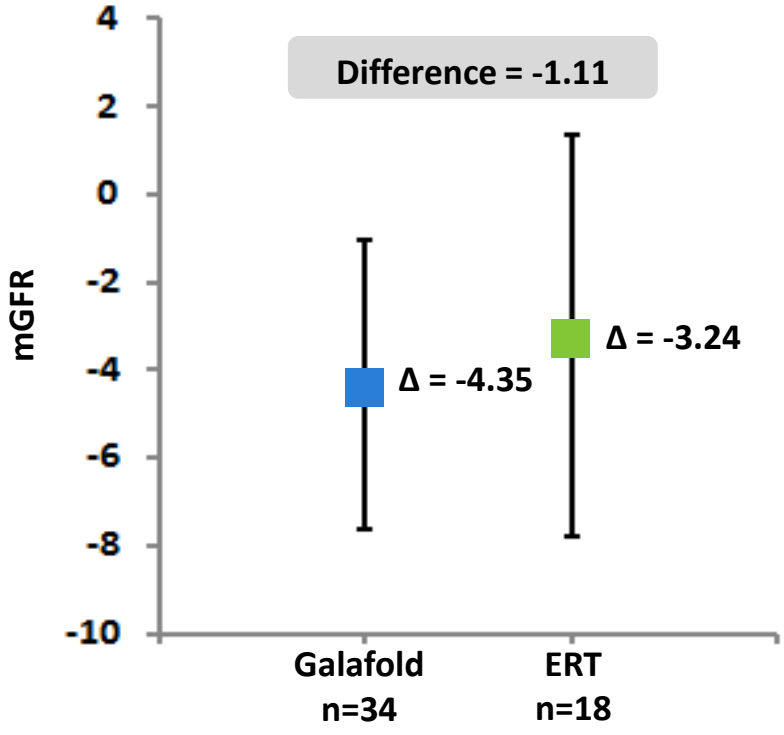
Amicus Therapeutics

Phase 3 (Study 012) Primary Efficacy Endpoint

Met Co-Primary Endpoints Showing Comparability of Kidney Function in Patients Switched from ERT to Galafold

Annualized Rate of Change in eGFR and mGFR at Month 18 (ml/min/1.73 m^2)



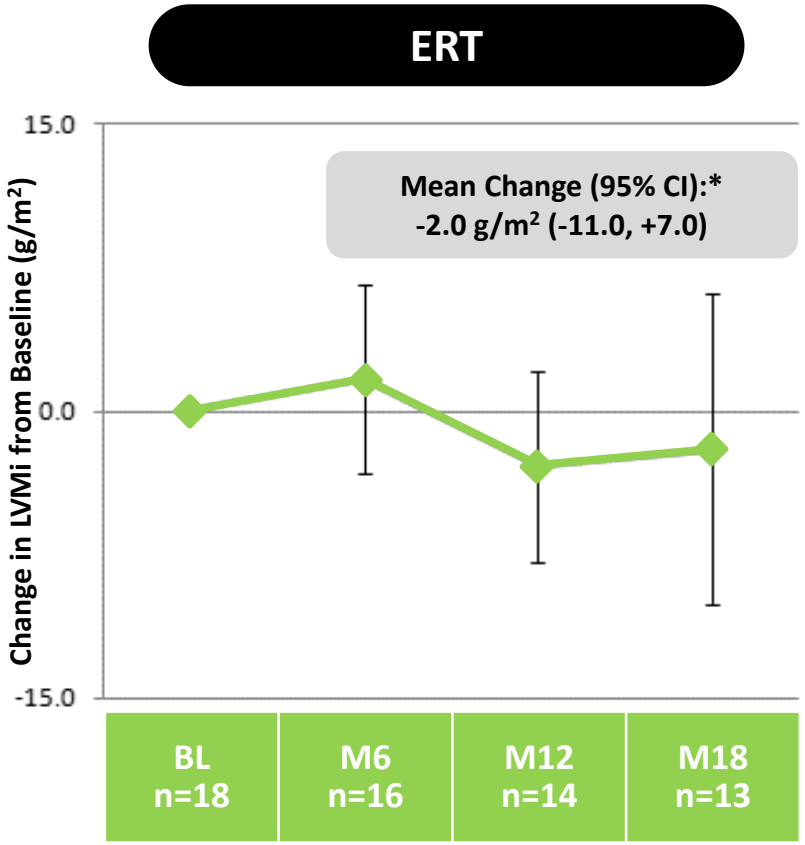


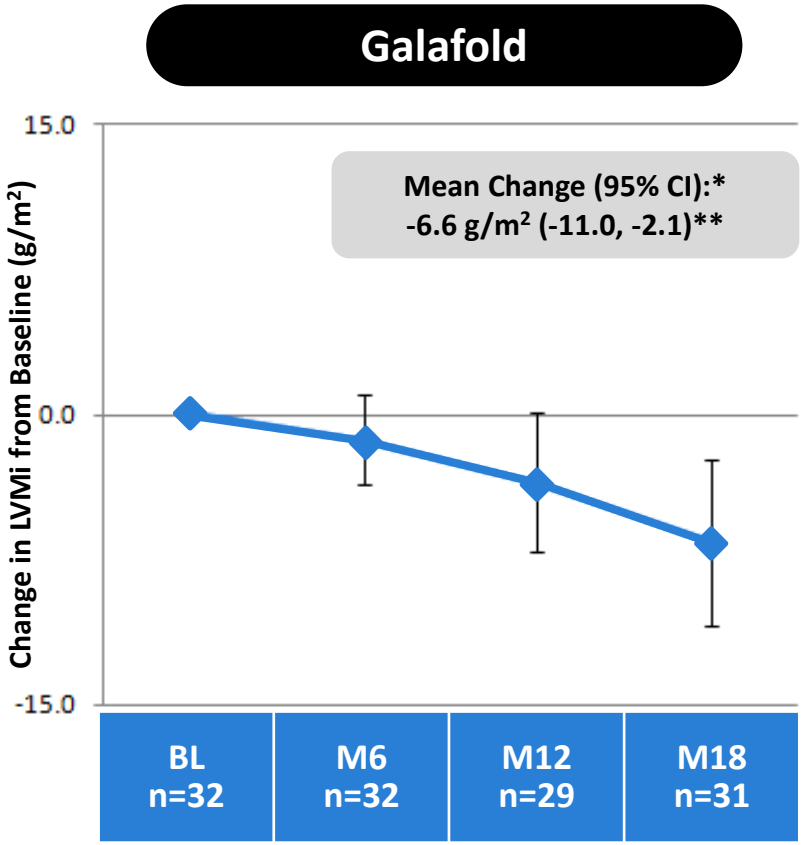
ANCOVA model [mITT]. Data represent LS means and 95% confidence intervals

Amicus Therapeutics

Phase 3 (Study 012) Cardiac Data

Reductions in LVMi Observed in Patients Switched from ERT Through Month 18 *





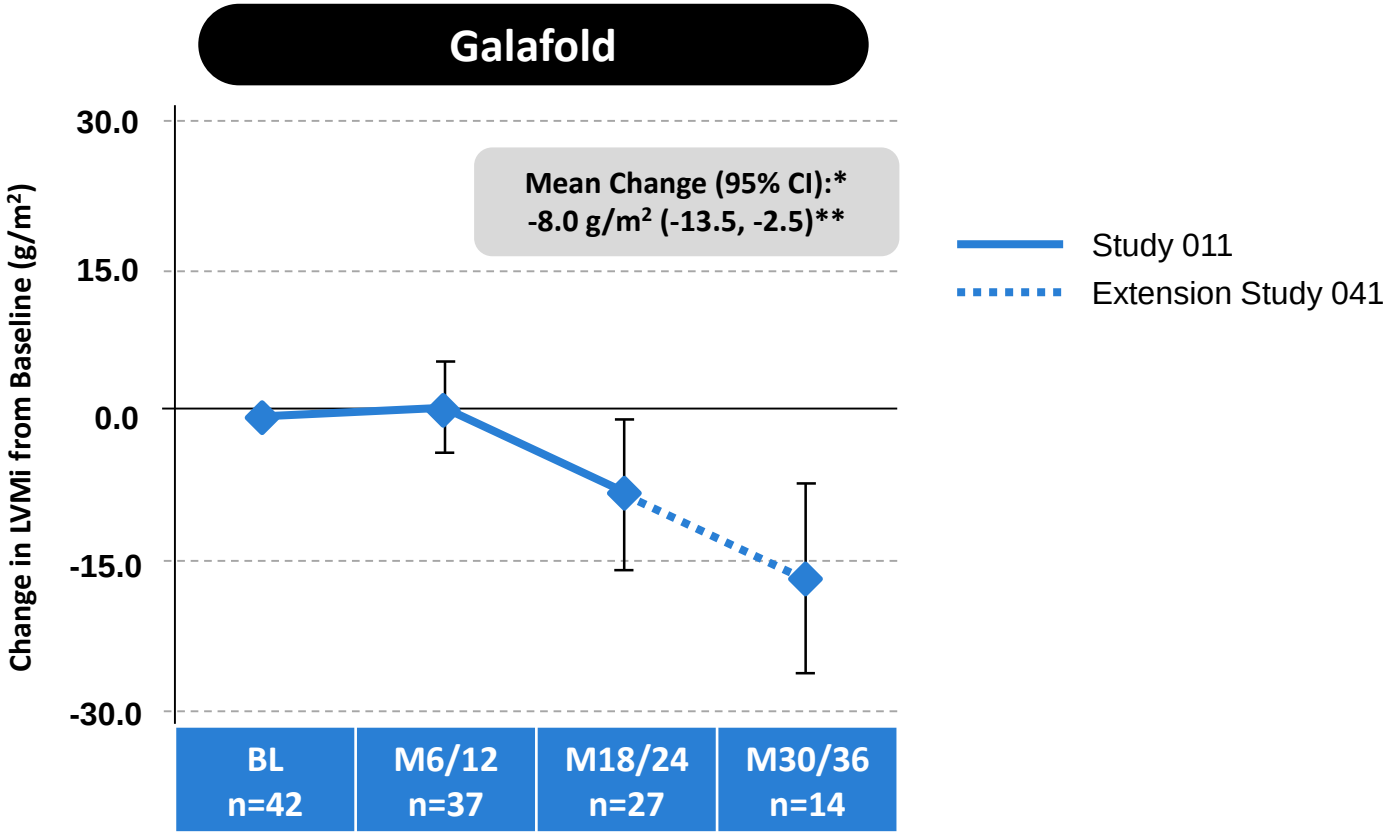
*Mean change to month 18 (mITT; amenable mutations) **Statistically significant (95% CI does not overlap zero)

Note: Mean and 95% confidence intervals on change from baseline are plotted

Amicus Therapeutics

Phase 3 (Study 011+041) Cardiac Data

Galafold also has Persistent and Increasing Effect on LVMi Over Longer Periods of Time (Up to 36 Months)



*Mean change to last available time point (average 22 months) in all patients with amenable mutations with baseline and post-baseline values.
**Statistically significant (95% CI does not overlap zero)
Sample size differences due to subjects not yet reaching a given timepoint or due to missing Echos

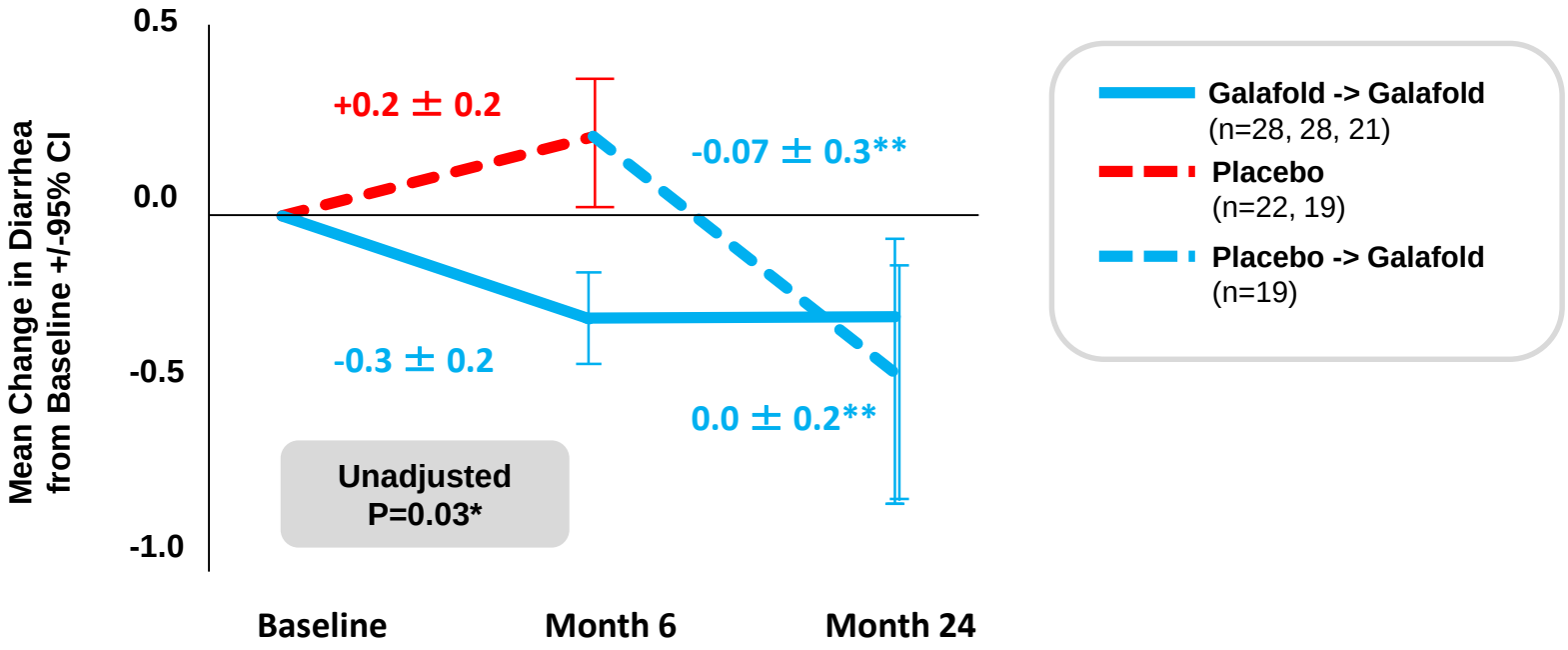
Note: Mean and 95% confidence intervals on change from baseline are plotted

Amicus Therapeutics

Phase 3 (Study 011) Patient-Reported Outcomes

Statistically Significant Reduction in Diarrhea Reported with Galafold vs. Placebo at Month 6 was Persistent and Durable Through Month 24

Mean Decrease in Diarrhea (GSRS) in Study 011 (All Subjects)[1]



Improvements in Indigestion and Favorable Trends in Reflux and Constipation Also Observed with Galafold

[1]Schiffmann, *et al.*, **WORLD*Symposium*™ 2015** **GSRS is Gastrointestinal Symptoms Rating Scale**

*ANCOVA, **Statistically significant (95% CI does not overlap zero)

Amicus Therapeutics

Safety Summary – Study 011

Most Common Treatment Emergent Adverse Events (≥ 10% of Subjects)						
Adverse event	Baseline to Month 6		Months 7-12		Open-Label Extension (Months 13-24)	
	Placebo* (n=33)	Migalastat (n=34)	Placebo-Migalastat* (n=30)	Migalastat (n=33)	Placebo-Migalastat* (n=28)	Migalastat (n=29)
Any Event	91%	91%	80%	79%	86%	83%
Headache	21%	35%			11%	10%
Fatigue	12%	12%				
Nausea	9%	12%				
Nasopharyngitis	6%	15%				
Paresthesia	12%	9%				
Procedural Pain			10%	12%		
Proteinuria					18%	14%
Bronchitis					11%	10%

*Subjects Received Placebo from Baseline to Month 6, Switched to Migalastat After Month 6

Safety Summary – Study 012
Common AEs (≥10%)

	Migalastat	ERT
N subjects	36	21
n subjects with TEAEs (%)	34 (94%)	20 (95%)
Nasopharyngitis	33%	33%
Headache	25%	24%
Dizziness	17%	10%
Influenza	14%	19%
Abdominal Pain	14%	10%
Diarrhea	14%	10%
Nausea	14%	10%
Back Pain	11%	14%
Upper Respiratory Tract Infection	11%	5%
Urinary Tract Infection	11%	5%
Cough	8%	24%
Vomiting	8%	14%
Sinusitis	8%	14%
Arthralgia	8%	10%
Bronchitis	6%	14%
Edema Peripheral	6%	10%
Vertigo	3%	10%
Dry Mouth	3%	10%
Gastritis	3%	10%
Pain In Extremity	3%	10%
Dyspnea	3%	10%
Procedural Pain	-	10%

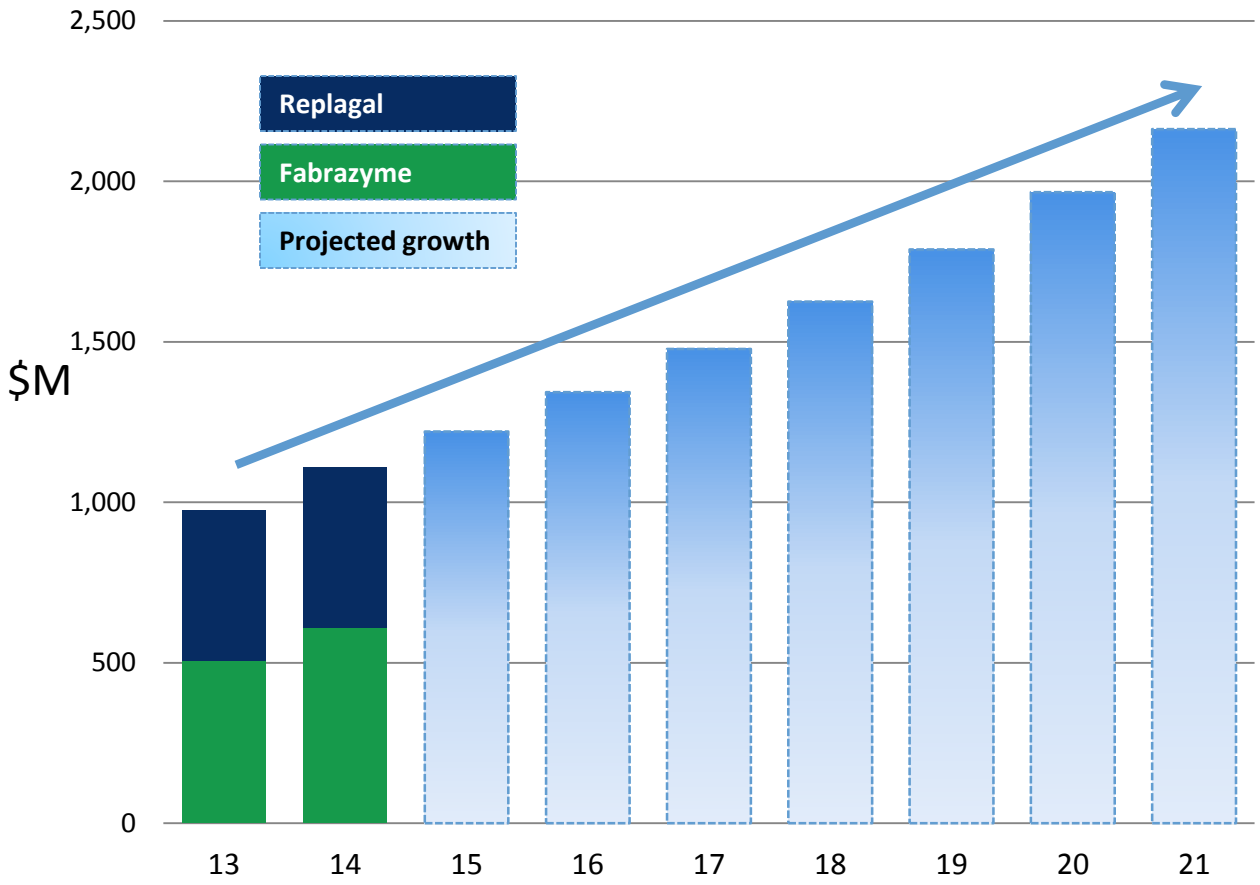
Galafold Regulatory Status

EU Timelines Under Accelerated Assessment on Track to Support Year-End 2015/Early 2016 CHMP Opinion – Working to Determine Optimal U.S. Approval Pathway

Anticipated Timing	Milestone	
2Q15	Accelerated Assessment Granted (150 day review)	✓
2Q15	MAA Submitted	✓
2Q15	MAA Validated	✓
4Q15	Day 120 questions	✓
Late 2015/Early 2016	**CHMP opinion**	
1H16	**Final EU decision**	

Global Fabry Market

Global Fabry Market Exceeded $1.1B in FY14 and Tracking Toward $2B by 2021



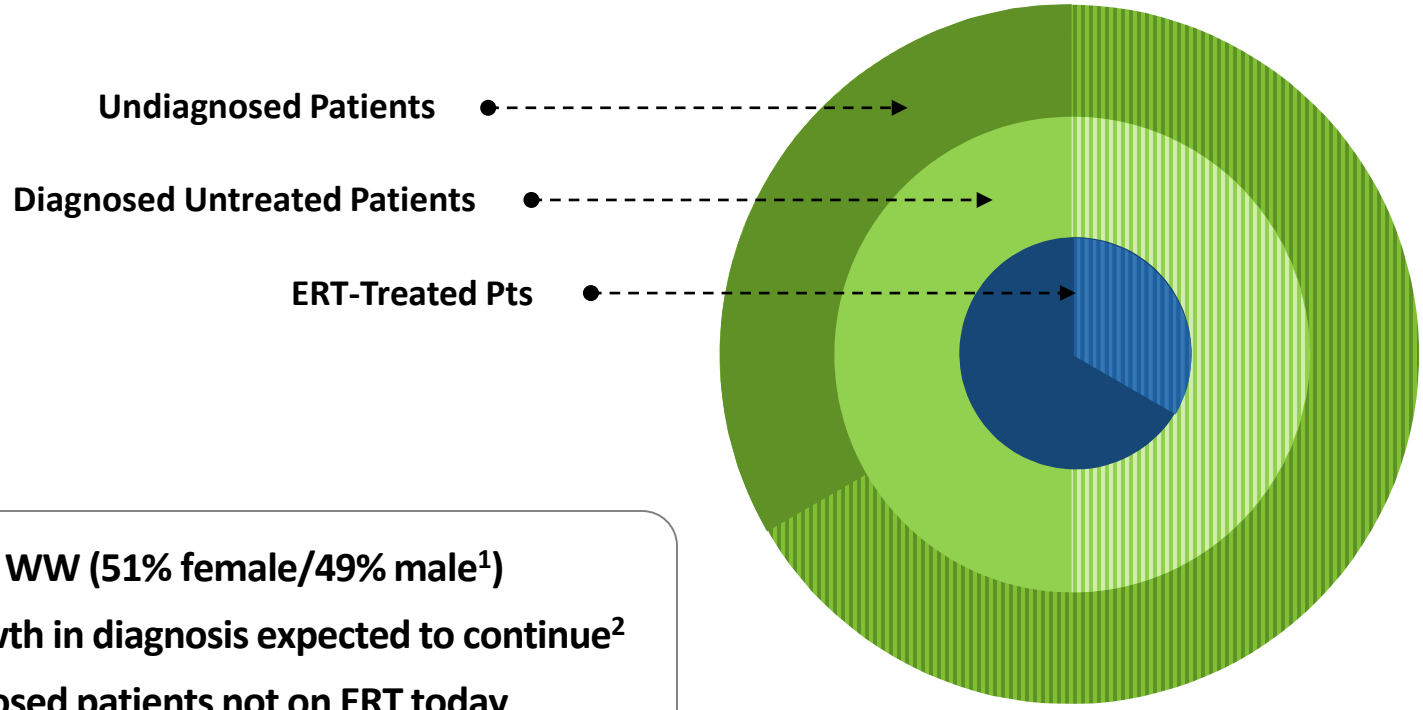
Fabry ERT sales increased **13.8% in 2014**, continuing trend of double-digit annual growth[1]

[1]Genzyme and Shire 10-Ks

Amicus Therapeutics

Galafold Commercial Opportunity

Attractive Commercial Opportunity with Significant Number of Patients with Amenable Mutations

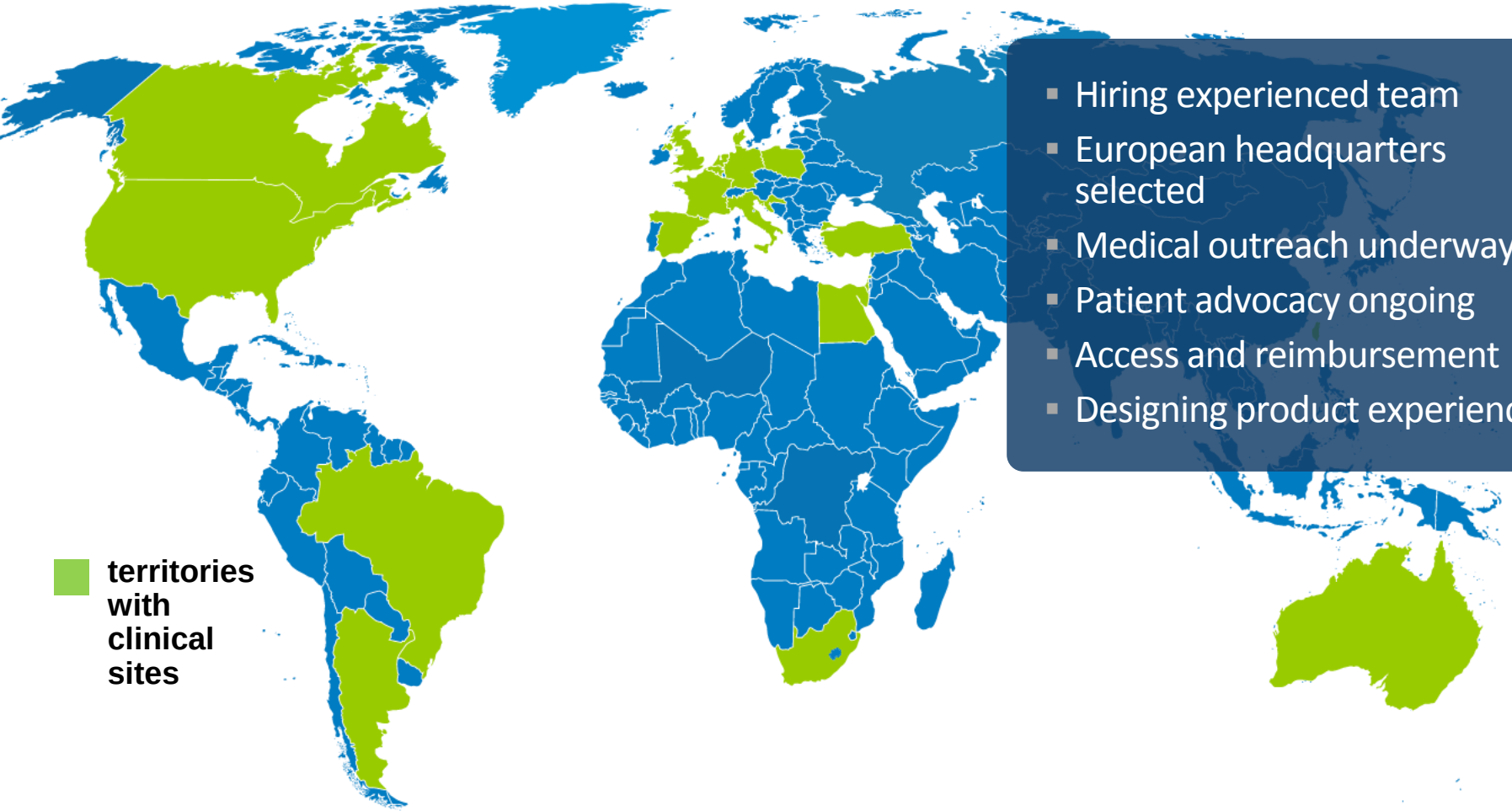


- 5-10K diagnosed WW (51% female/49% male[1])
- 10% annual growth in diagnosis expected to continue[2]
- 40-50% of diagnosed patients not on ERT today
- 30-50% with amenable mutations
- Newborn screening studies suggest prevalence of ~1:1000 to ~1:400

[1]Fabry Registry 2011; [2]Third Party Market Research, 2015

Global Pre-Commercial Activities

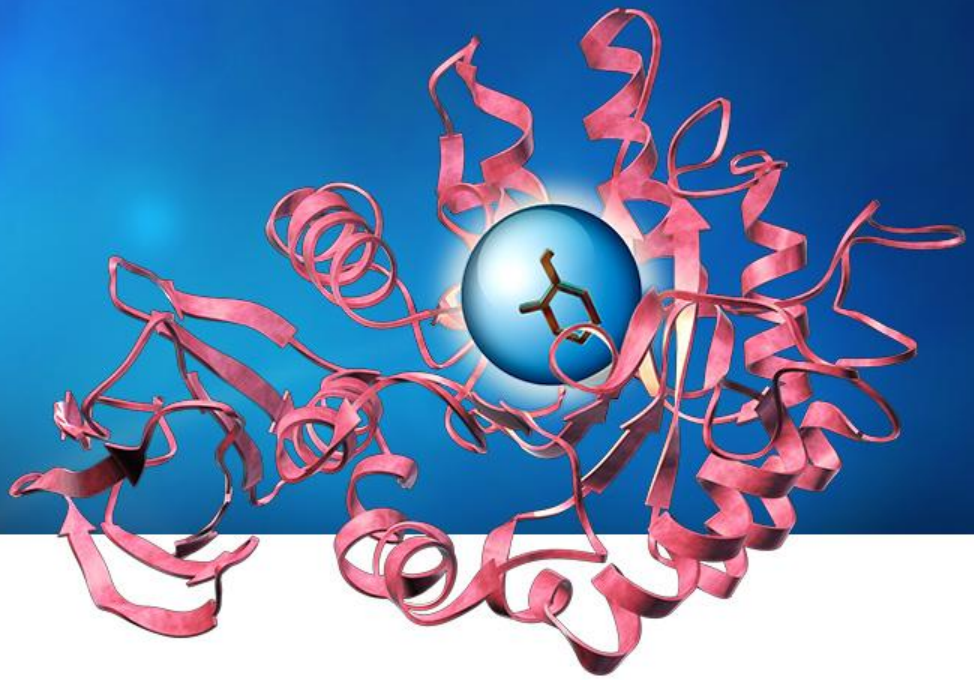
Amicus is Building on Global Galafold Experience to Prepare for Successful Launch



- Hiring experienced team
- European headquarters selected
- Medical outreach underway
- Patient advocacy ongoing
- Access and reimbursement
- Designing product experience

Amicus Therapeutics

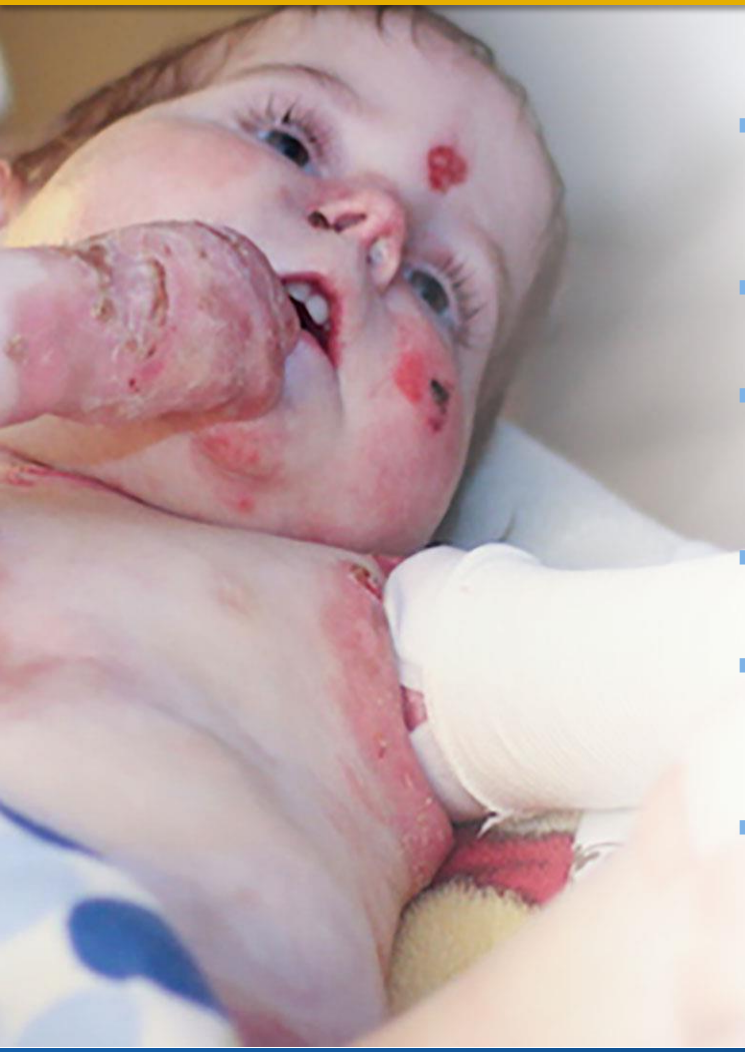
Amicus Therapeutics



Zorblisa™ for Epidermolysis Bullosa (EB)

Epidermolysis Bullosa (EB)

Rare, Devastating, Connective Tissue Disorder with No Approved Treatments



- Multiple genes cause disease which results in fragility of skin and can also 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 lack of treatment, any reduction in disease symptoms would be considered meaningful
- 30,000 – 40,000 **diagnosed** patients in major global regions

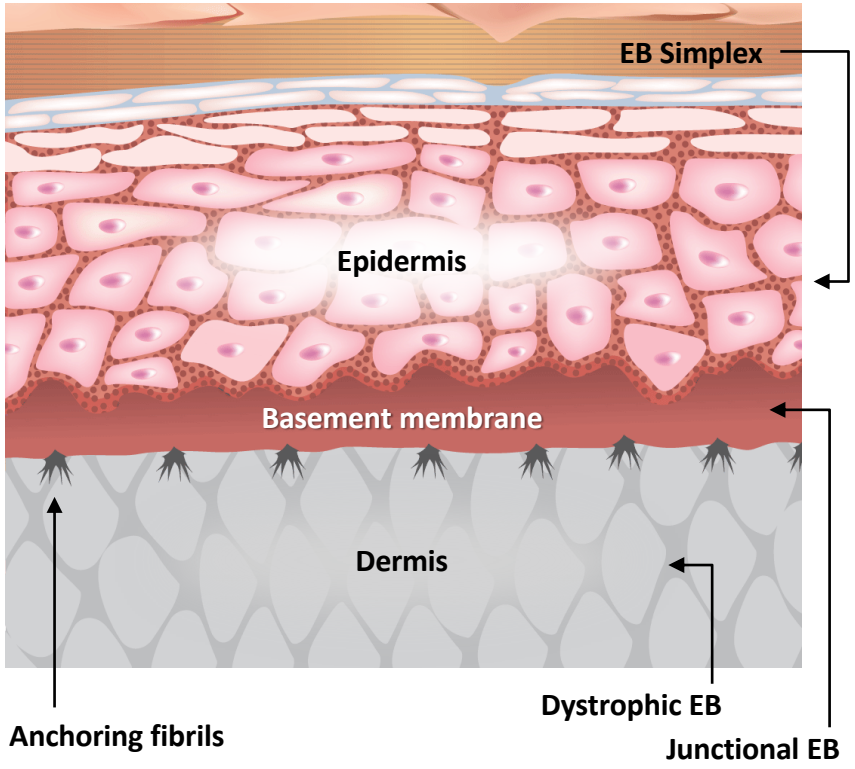
[1] Third party market research

Three Major EB Subtypes

Three Major EB Subtypes Differ By Physical Manifestations, Genetic Makeup, and Prognosis

Skin structure

Sites of primary blister formation



EB subtypes

Represent ~99% of EB Population

Subtypes	Symptoms	Frequency
Junctional	▪ External blistering ▪ Internal blistering (oral tract, internal organs) ▪ Severe complications can become fatal early in life	**~5%**
Dystrophic	▪ External blistering ▪ Narrowing of esophagus ▪ Higher risk of aggressive skin cancer ▪ Associated with mortality	**~20%**
Simplex	▪ Localized and generalized external blistering	**~75%**

Mortality risk

Zorblisa being developed for all major EB subtypes

Source: Adapted from DebRA America

Zorblisa™ Overview

Patented High Concentration Allantoin with Breakthrough Therapy Designation

Novel, Proprietary Topical Cream Promotes Healing of Wounds in EB and is Differentiated by Applicability for All Major EB Subtypes

Active ingredient	▪ Allantoin
RoA	▪ Proprietary topical cream containing 6% allantoin, applied to entire body once daily
Proposed Indication	▪ All major EB subtypes (Simplex, Dystrophic and Junctional)
Phase of development	▪ Phase 3 registration study ongoing
Proposed MOA*	▪ Aids inflammatory response, bactericidal effects, loosens protein bridges, promotes collagen
Formulation	▪ Patented formula to deliver high concentration in highly stable, soluble form

*Margraf and Covey 1977; Meixell and Mecca 1966; Settle 1969; Meixell and Mecca 1966; Flesch 1958, Fisher 1981; Cajkovac et al., 1992, Medda 1976

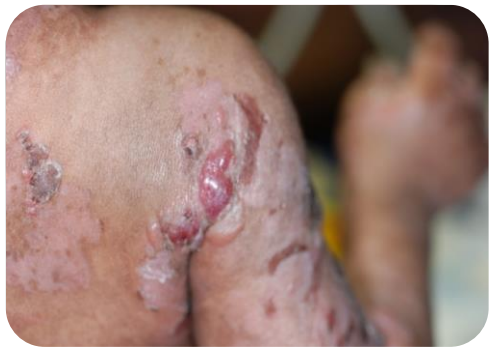
Amicus Therapeutics

Phase 2a Study: Individual Patient Data

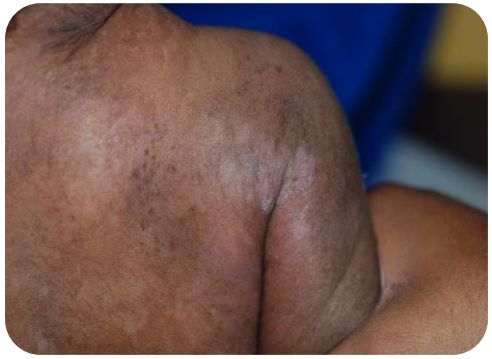
One-year old girl with EB Simplex

As Depicted Below, Phase 2a Study Demonstrated Significant Healing of Wounds

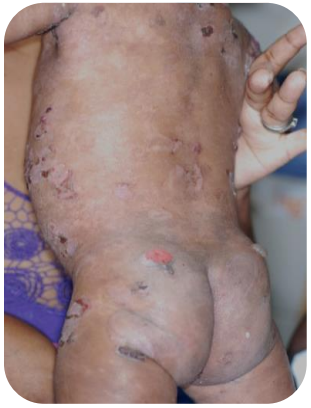
Baseline



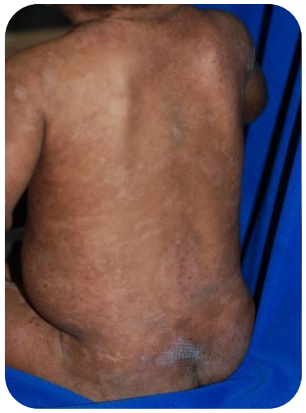
Following 2 months of treatment



Baseline

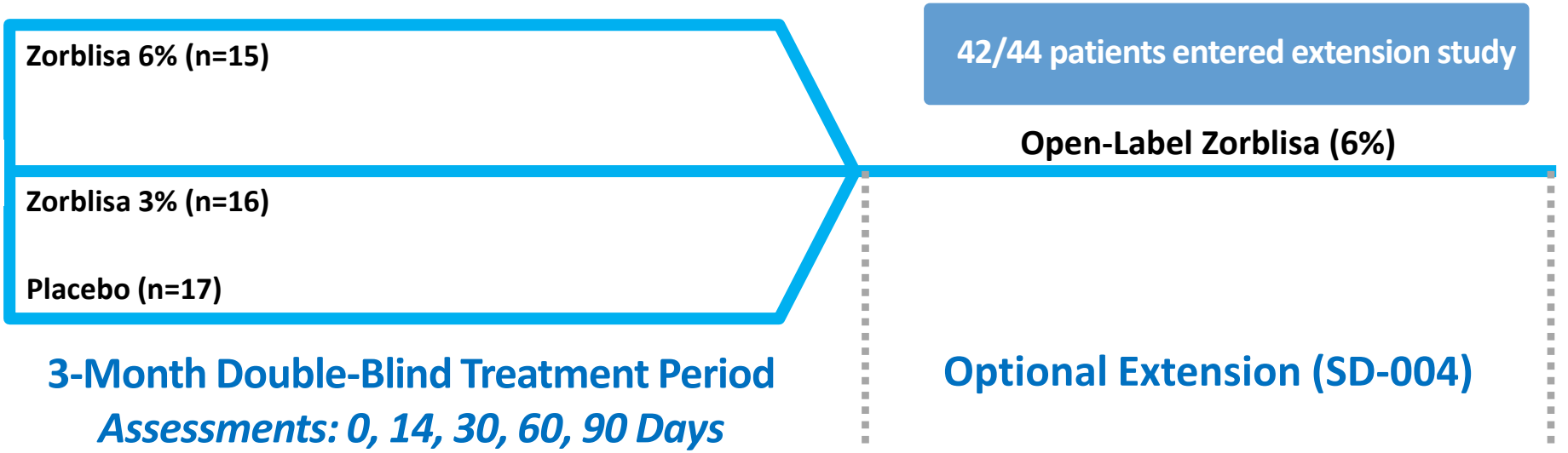


Following 2 months of treatment



Phase 2b (Study 003) Design

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



Primary Efficacy Endpoint:
Target Wound Healing at Month 1

- Baseline wound: Chronic (≥ 21 days), size 5-50 cm^2

Key Statistical Assumptions:

- Placebo response rate: 10%
- Zorblisa response rate: 50%
- 16 patients/arm = 70% power

***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)*

Amicus Therapeutics

Phase 2b (Study 003) Primary Endpoint Results
% Patients with Complete Closure of Target Wounds

Zorblisa 6% Demonstrated Higher Proportion of Complete Target Wound Closure at Pre-Specified Endpoint and Subsequently During the Study

ITT Population (n=48)			
	N	Month 1 (pre-specified primary endpoint)	Month 2 (Phase 3 primary endpoint)
Placebo	17	41%	41%
Zorblisa 3%	16	38%	44%
Zorblisa 6%	15	53%	60%

Evaluable Population (n=45)			
	N	Month 1 (pre-specified primary endpoint)	Month 2 (Phase 3 primary endpoint)
Placebo	17	41%	41%
Zorblisa 3%	16	38%	44%
Zorblisa 6%	12	67%	82% (p=0.04)*

*Zorblisa 6% vs placebo, unadjusted p=0.04

Excluded from Evaluable population: 1 patient (due to lost to follow-up), 2 patients (did not have single identified and qualified target lesion)

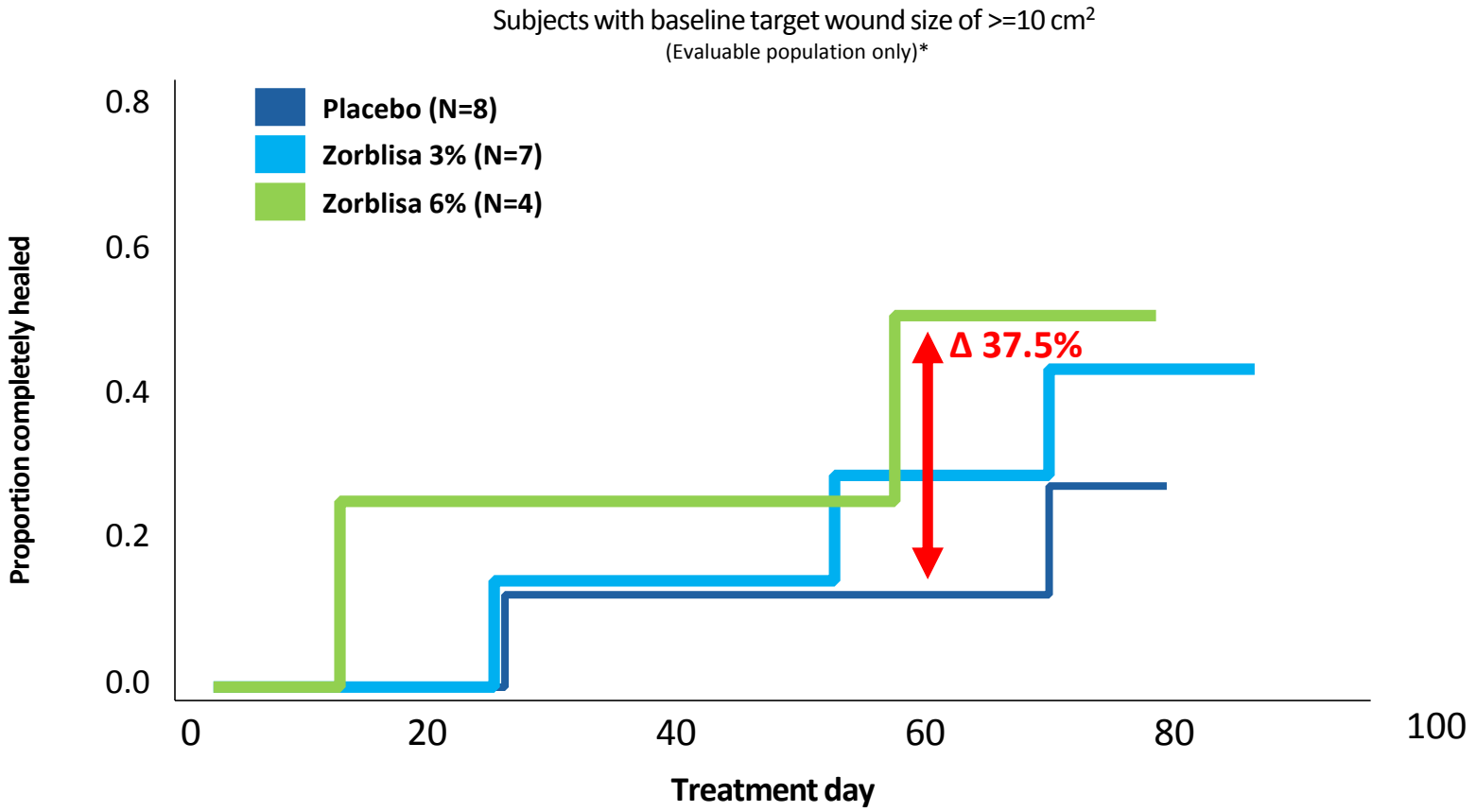
Amicus Therapeutics

Phase 2b (Study 003) Efficacy Results

Subgroup of Patients with Baseline Target Wounds ≥ 10cm² (n=19)

Greatest Separation Between Zorblisa and Placebo at Month 2 in Subjects with Baseline Wounds ≥ 10 cm²

Proportion with Complete Target Wound Closure Over 3 Months

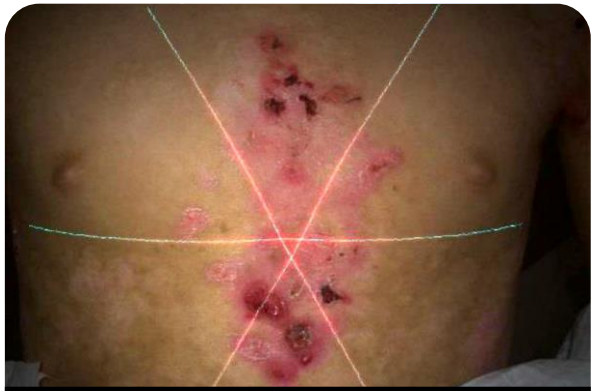


* Subjects 103002, 103004, 104003 are excluded from Evaluable population

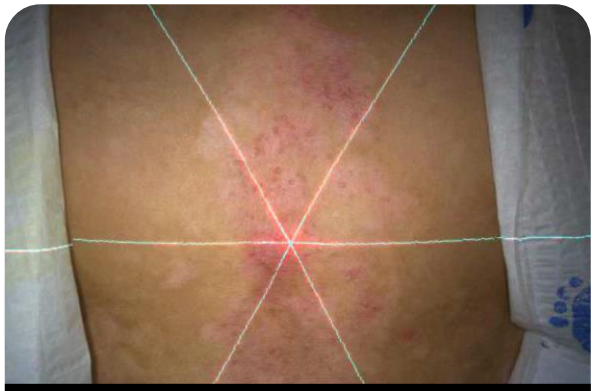
Amicus Therapeutics

Phase 2b (Study 003) Individual Patient Data

8 year old female with EB (Recessive Dystrophic)

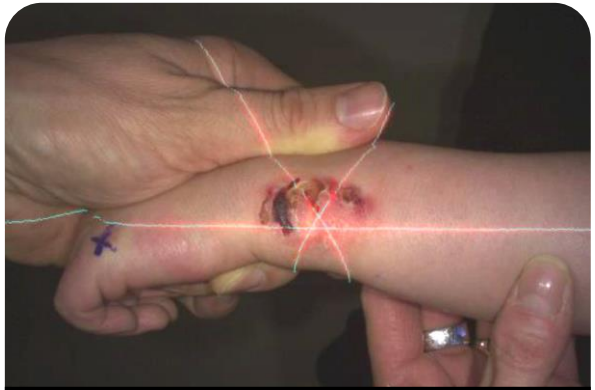


Baseline

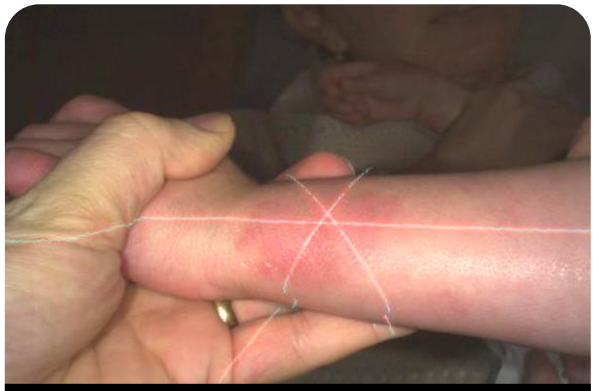


2-Months Post-Treatment

3 year old male with EB (Simplex)



Baseline



2-Weeks Post-Treatment

Phase 2b (Study 003) Safety Summary

Adverse Events Similar Across Treatment Arms of Placebo, Zorblisa 3%, and Zorblisa 6%

- Treatment-emergent adverse events (TEAE) generally similar across treatment groups
- No deaths and no severe TEAEs
- No serious adverse events reported in Zorblisa 6% group

Treatment Emergent Adverse Events ≥10% Frequency

	Placebo	Zorblisa 3%	Zorblisa 6%
N subjects	17	16	15
N subjects with TEAEs (%)	12 (70.6)	13 (81.3)	9 (60.0)
Nasopharyngitis	12%	25%	7%
Pyrexia	12%	19%	33%
Application Site Pain	6%	19%	13%
Pain	-	-	13%
Skin and Subcutaneous Tissue Disorders	35%	19%	20%
Pruritus	6%	13%	13%
Rash	12%	-	7%
Rash Erythematous	12%	-	-
Cough	6%	-	13%
Oropharyngeal Pain	12%	-	-
Rhinorrhea	-	-	13%
Vomiting	6%	6%	13%
Headache	12%	-	7%

Pivotal Phase 3 (Study 005) Underway

Phase 3 Initiated in 2Q15 and Currently Enrolling Patients
Top-line data expected 2H 2016

Zorblisa 6%

~150 EB patients (age ≥ 1 month)
1:1 Randomization - Daily Topical Application

Placebo

3-Month Double-Blind Treatment Period
Assessments: 0, 14, 30, 60, 90 Days

Optional Extension (SD-006)

Open-Label Zorblisa (6%)

36/36 Patients Who Completed Study 005 Continued in Open-Label Extension (Nov. 2015)

Primary Efficacy Endpoint: Target Wound Healing at Month 2

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

Secondary Endpoints

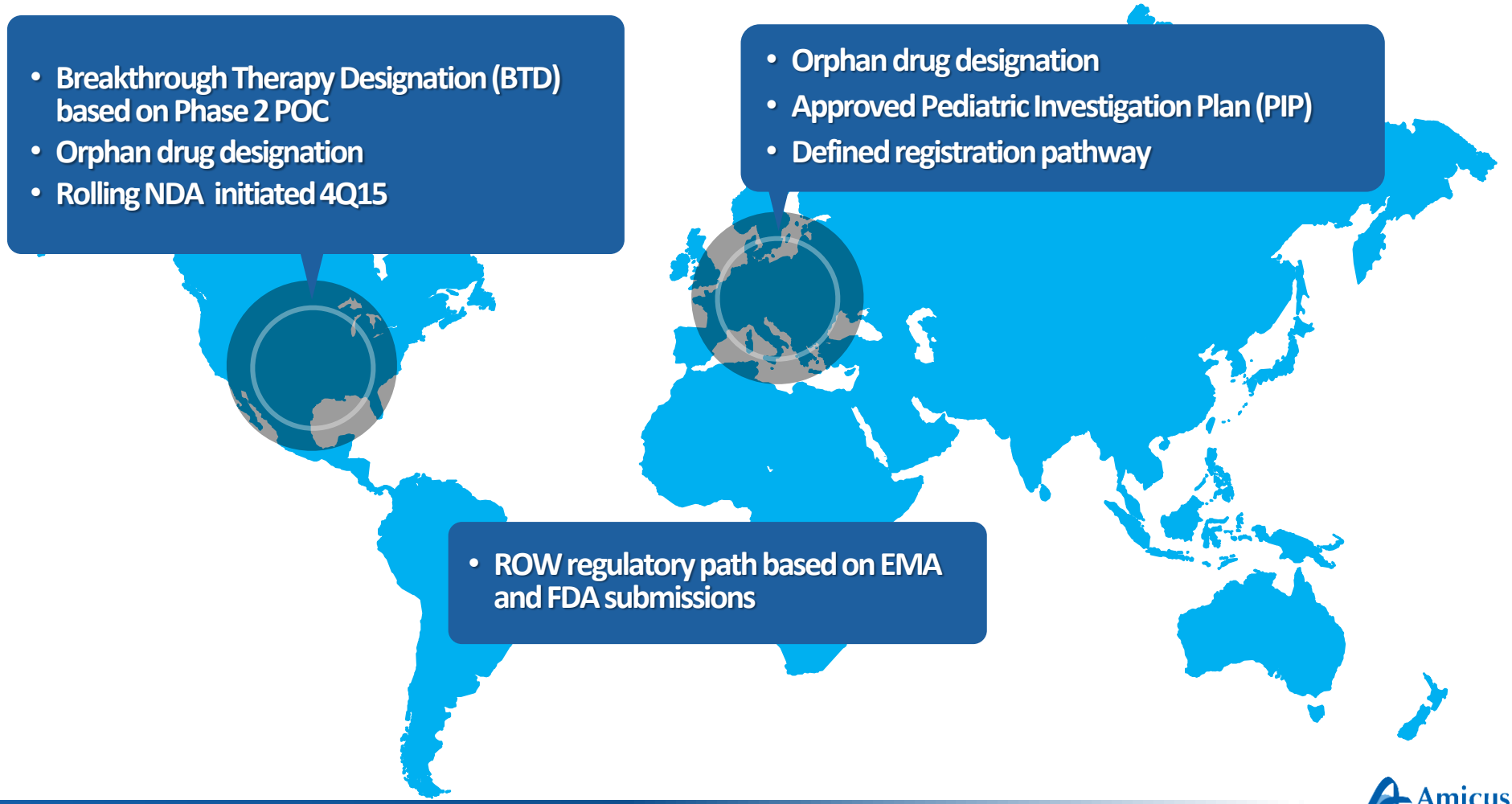
- Time to target wound closure; Change in Body Surface Area (BSA) of lesions and blisters; itching; pain

*Information as of November 3, 2015

Amicus Therapeutics

Zorblisa Regulatory Pathway
Rolling NDA Initiated 4Q15

FDA and EMA Aligned on Phase 3 Study Design



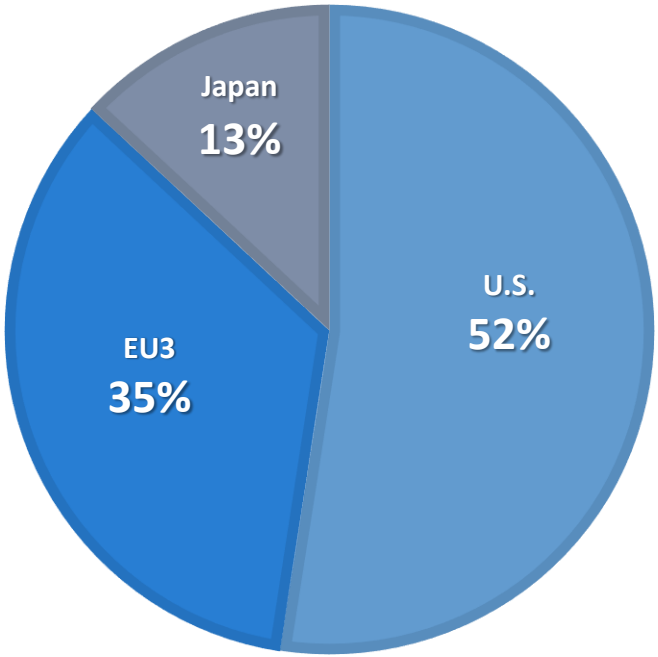
Amicus Therapeutics

Potential $1B+ Global EB Commercial Opportunity for Zorblisa

Significant Global Commercial Opportunity Supported by Profound Unmet Clinical Need, Strong Stakeholder Support and High Orphan Prevalence

Diagnosed EB Patients by Geography

(US, EU3, Japan)



Significant Unmet Clinical Need

- No approved treatments, opportunity for first-in-class
- Compelling proof-of-concept in meaningful endpoints
- Studied in all EB subtypes

Strong Support Among Surveyed Stakeholders

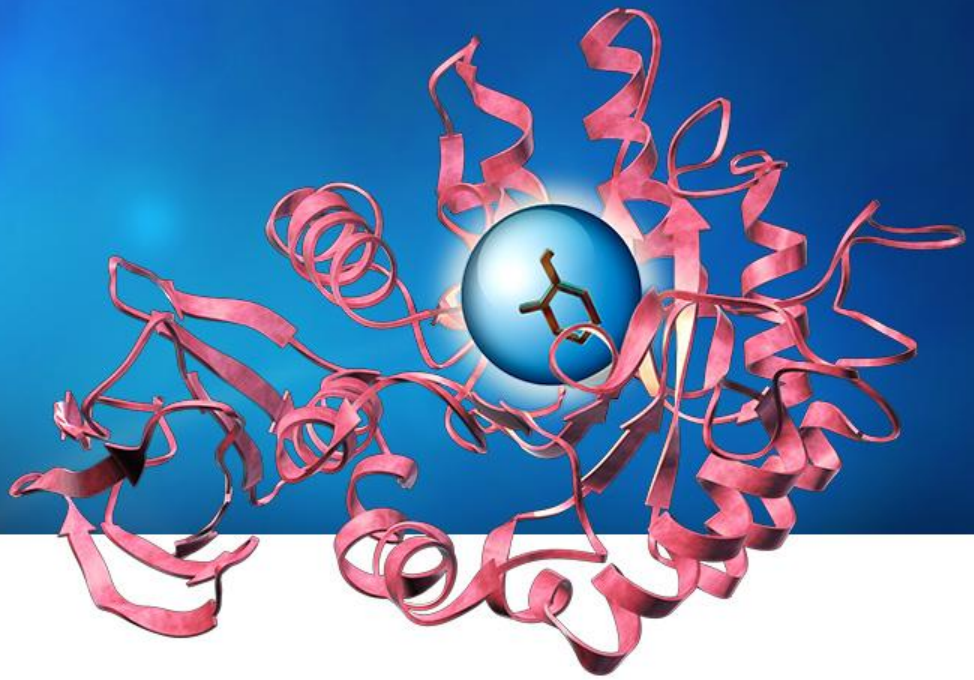
- Physicians indicate usage in 100% patients due to product profile and urgent need
- Payers indicate support for broad reimbursement if approved

Large Commercial Opportunity

- 30,000 – 40,000 diagnosed in major markets
- Patients largely seen by neonatal wards, primary care physicians and dermatologists at major medical centers
- KOLs expect diagnosis rates to increase as EB is better characterized and awareness grows

Source: Third Party Market Research and Analysis

Amicus Therapeutics

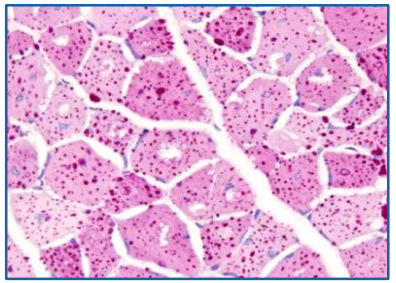


Novel ERT for Pompe Disease

Pompe Disease Overview

Severe, Fatal, Progressive Neuromuscular Disease with Significant Unmet 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
- Incidence 1:28,000[1]

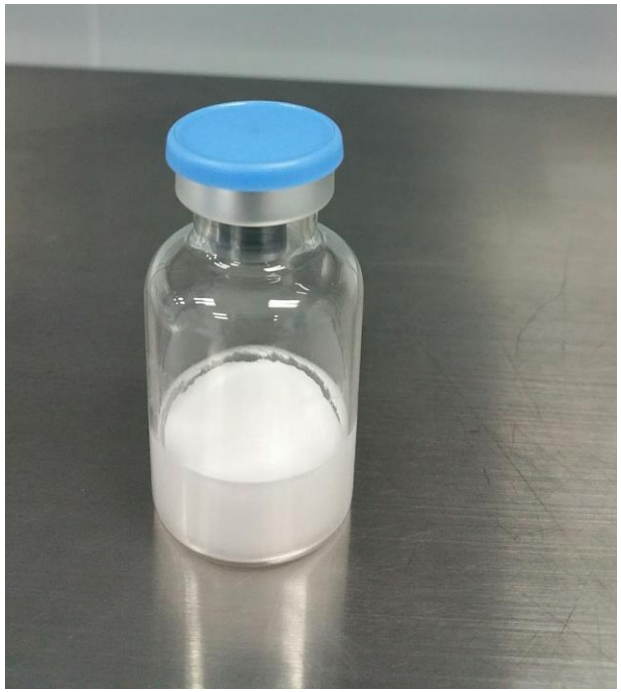


Elevated Glycogen in Muscle

[1]Evidence Report – Newborn Screening for Pompe Disease – June 2013 – HRSA.gov

Amicus Biologics Milestones Achieved

Significant Progress From Pompe Master Cell Banking to GMP Manufacturing in < 2 Years While Maintaining High Levels of M6P and Proper Glycosylation

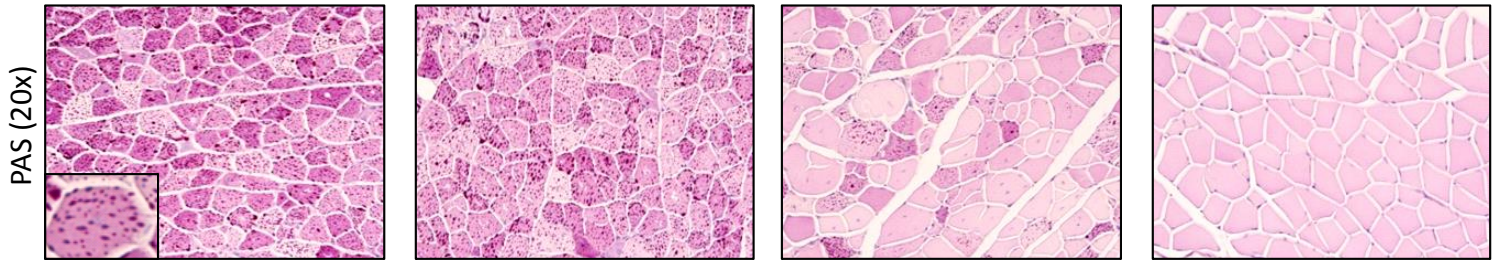


- Master cell banking in 2013
- Cell line scaled to 250 L in 2014
- GMP batches completed 2Q15-3Q15 to initiate upcoming clinical study

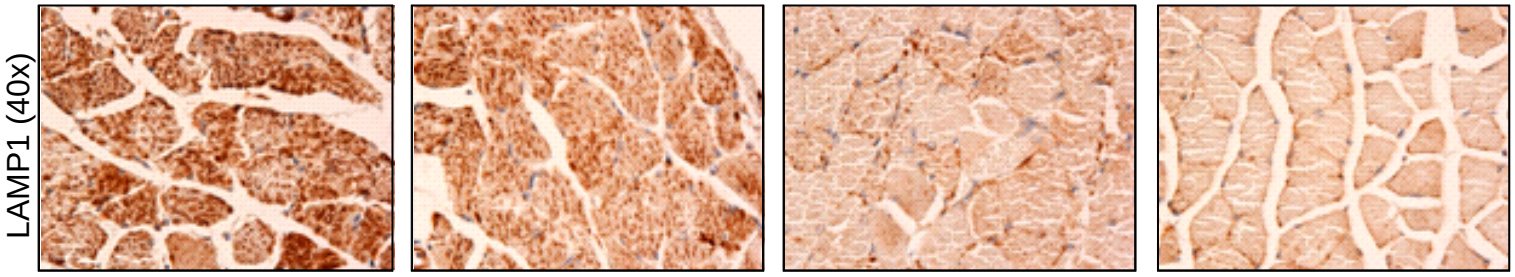
ATB200 + Chaperone Preclinical Proof-of-Concept

Glycogen Clearance Correlates with Endocytic Vesicle Turnover in Skeletal Muscle of *Gaa* KO Mice[1]

PAS-glycogen staining in Quadriceps



LAMP1 Immunohistochemical staining in Soleus



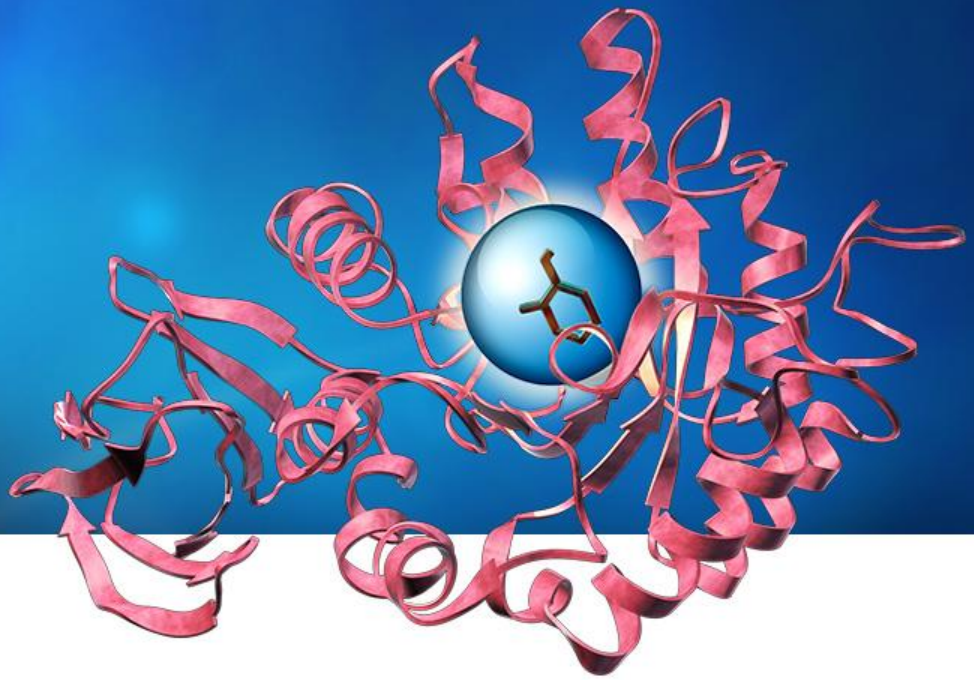
[1]Following 2 doses of 20mg/kg Alglucosidase Alfa or ATB200 + AT2221 in Gaa KO mice, skeletal muscles evaluated for glycogen clearance and proliferated lysosomes. Treatment with Alglucosidase Alfa modestly reduced glycogen or proliferated lysosomes while ATB200, co-administered with AT2221 significantly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

Amicus Therapeutics

ATB200 Summary and Next Steps

- Clinical trial material ready
- Pre-IND meeting completed to discuss Phase 1/2 safety and pharmacokinetic (PK) study in ERT-switch Pompe patients
- On track to initiate Phase 1/2 study pending IND clearance

Amicus Therapeutics



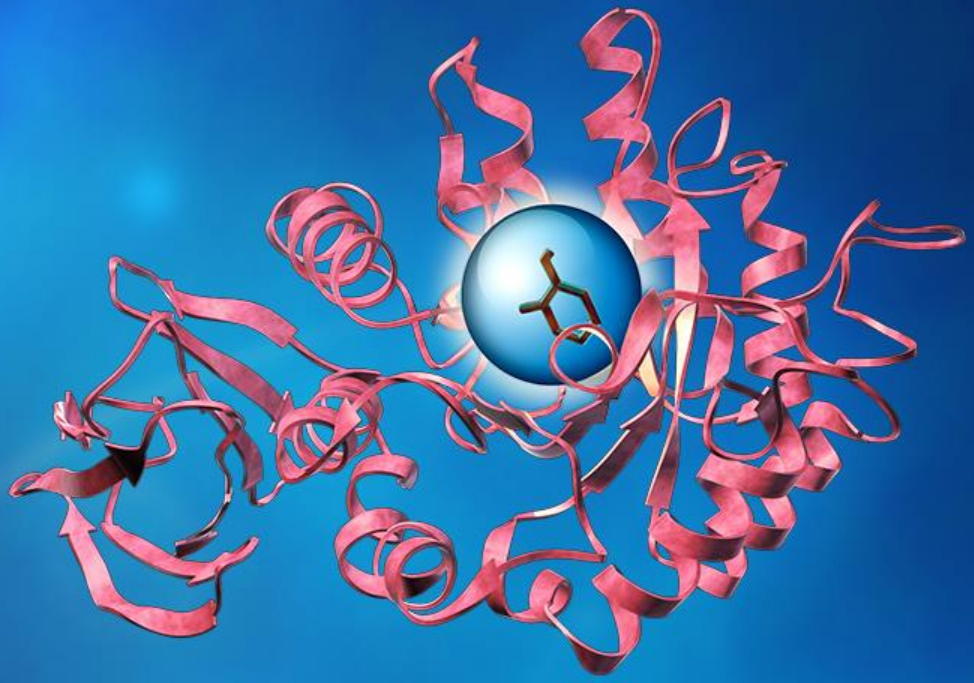
Financial Summary

Financial Summary

Cash Position Provides Runway Under Current Operating Plan Into 1H17

Financial Position	September 30, 2015
Current Cash:	$251.9M
Anticipated Year-end Cash Balance:	$200-$225M
Cash Runway:	1H17
Capitalization	
Shares Outstanding:	124,617,490

Amicus Therapeutics



Goldman Sachs US Emerging & SMID Cap Growth Conference

Chip Baird, Chief Financial Officer

November 19, 2015

at the forefront of therapies for rare and orphan diseases