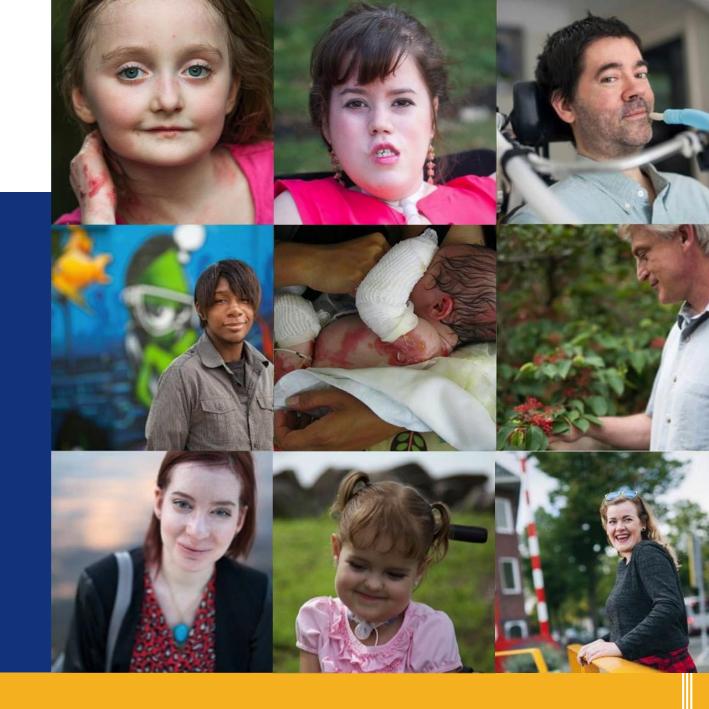


Corporate Overview



**September 2016** 

Introduction

#### Safe Harbor

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, financing plans, and the projected cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; and the potential that we will need additional funding to complete all of our studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2015. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.



### Amicus 2016 – Looking Back

Amicus Has Greatly Expanded Product Pipeline, Technologies and Geographies

2014

2013

2012

- Chaperone Technology for LSDs
- Small molecules
- U.S. rights to Galafold™ (migalastat)
- Callidus acquisition
- Biologics
- Global rights to migalastat

- Positive Phase 3 data for migalastat
- Biologics scaleup

International HQ

2015

- MAA Submission
- Scioderm acquisition
- Pompe ERT in clinic



### Amicus 2016 – Continuing the Momentum

### **Significant Milestones in 2016**

2016-17
Target

Milestones

- ✓ Galafold EU approval for Fabry
- FDA regulatory clarity for Galafold
- EB Phase 3 data
- Pompe clinical data

2014

2013

2012

- Chaperone Technology for LSDs
- Small molecules
- U.S. rights to Galafold™ (migalastat)
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- Global rights to migalastat

- Positive Phase 3 data for migalastat
- Biologics scaleup

International HQ

2015

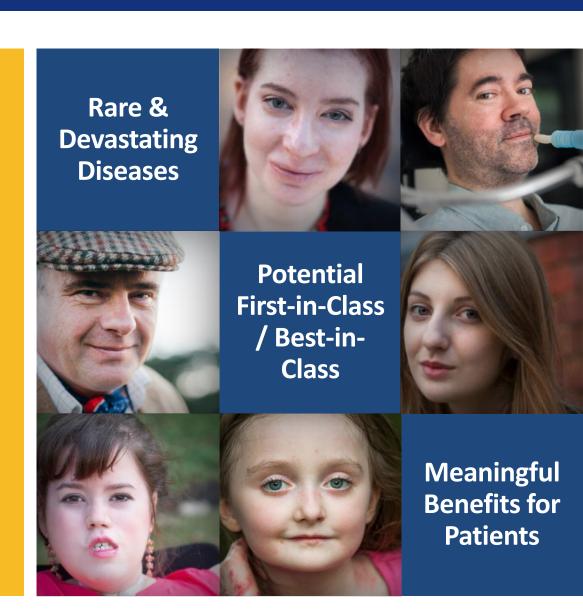
- MAA
   Submission
- Scioderm acquisition
- Pompe ERT in clinic



Introduction

#### **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**

- Galafold Precision Medicine (Small Molecule)
- EU Full Approval
- Launched in Germany (May 30, 2016)
- U.S. regulatory update anticipated 4Q16

### **Epidermolysis** Bullosa (EB)

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

#### **Pompe**

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

**R&D Engine and Continued Business Development Activity** 





Galafold™
(Migalastat)
Precision Medicine
for Fabry Disease

### European Commission Granted Full Approval for Galafold

Galafold Indicated for Long-Term Treatment of Adults and Adolescents Aged ≥ 16 years with a Confirmed Diagnosis of Fabry Disease and Who have an Amenable Mutation\*



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

The most common side effect reported in clinical trials was headache.



<sup>\*</sup>For important safety information for Galafold, including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at <a href="https://www.ema.europa.eu">www.ema.europa.eu</a>

### **Fabry Disease Overview**

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

**Leading Causes of Death** 

TRANSIENT ISCHEMIC ATTACK (TIA) & STROKE<sup>1</sup>

#### **HEART DISEASE<sup>2</sup>**

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

#### **KIDNEY DISEASE<sup>3</sup>**

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

Life-Limiting Symptoms

#### **GASTROINTESTINAL**<sup>3</sup>

- 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% male<sup>4</sup>)
- 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**

# **Two Largest Phase 3 Studies Ever Completed in Fabry Disease**



#### **Reduction in Disease Substrate**

IC GL-3 (Study 011<sup>1</sup>)\*
Plasma Lyso Gb-3 (Study 011<sup>2,1</sup> and 012<sup>3</sup>)\*

#### **Stability of Kidney Function**

Estimated Glomerular Filtration Rate (eGFR) and Measured GFR (Study 011<sup>4</sup> and Study 012<sup>4,3</sup>)

#### **Reduction in Cardiac Mass**

Left Ventricular Mass Index (LVMI) (Study 011<sup>2</sup> 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)

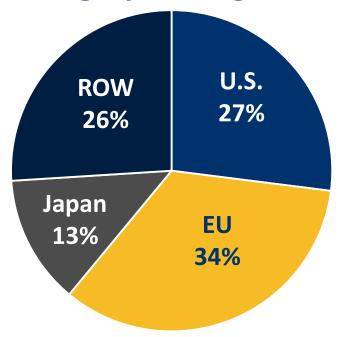
- 1: Improvement versus placebo over 6 months in amenable patients
- 2: Improvement from baseline over 18+ months
- 3: Comparable to ERT over 18 months
- 4: Stabilization from baseline over 18 months with favorable comparison to natural history in literature



### **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

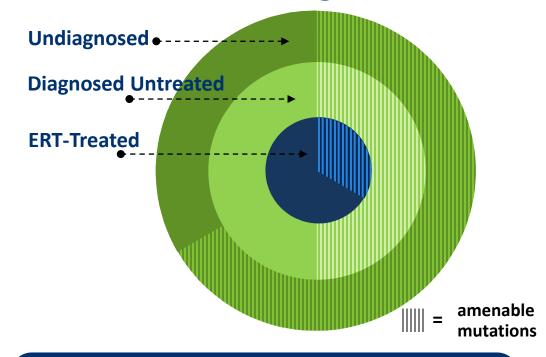
### **Geographic Segments**



#### • \$1.2B in FY15 ERT Sales<sup>1</sup>

- 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



### International Launch Update

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

#### **GERMANY**

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

#### **FRANCE**

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

#### **UNITED KINGDOM**

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



### **EU Launch Update**

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

21



reimbursed Galafold WW (7/31/16)

patients (switch & naïve) on

countries with active pricing discussions

#### **LAUNCH IN GERMANY = IMPORTANT INDICATOR**

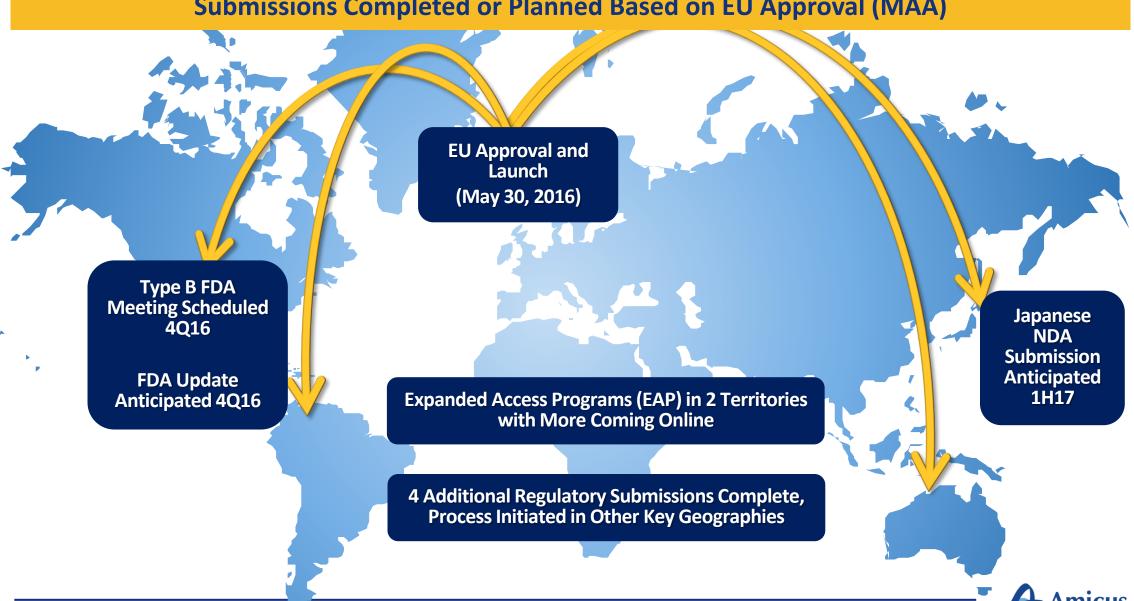
- First Galafold Rx within 24 hours of EC approval
- Patient support program
- Experienced, high quality team
- Pricing dossier submitted

countries with reimbursement (commercial and EAP)



### **Global Regulatory Strategy**

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





### **Amicus Proprietary Fabry ERT**



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

### Target Fabry ERT product profile:

- Improved drug targeting
- Co-formulation with chaperone

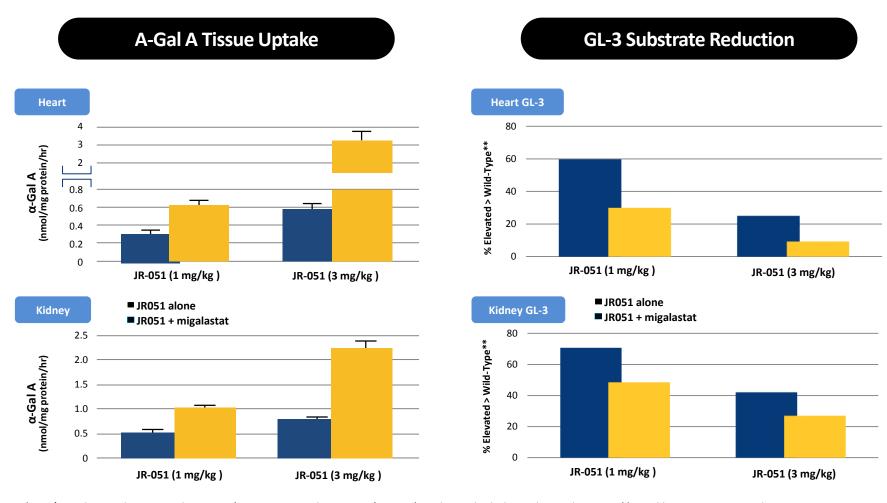
### Development status:

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



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

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



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

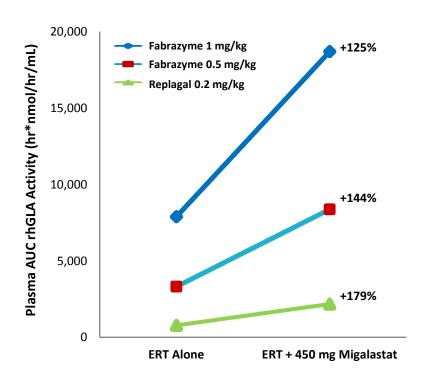
\*ERT designed to be biosimilar to Fabrazyme; \*\*0 = wild-type, 100 = untreated KO mouse



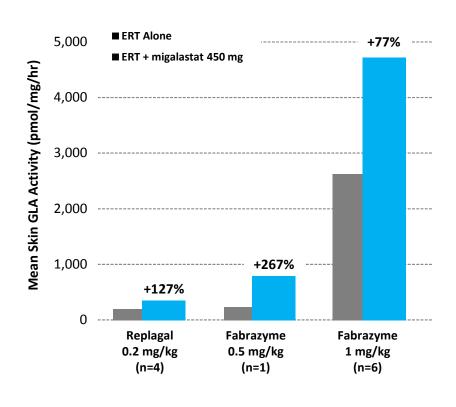
### CHART Phase 2a Results for Fabry Co-Administration Study 013

# Co-Administration with Fabrazyme or Replagal Leads to Consistent Increases in Active Plasma Enzyme Levels and Tissue Uptake<sup>1</sup>

#### Plasma alpha-Gal A Activity (Area Under Curve)



#### Mean alpha-Gal A Activity (Day 2)

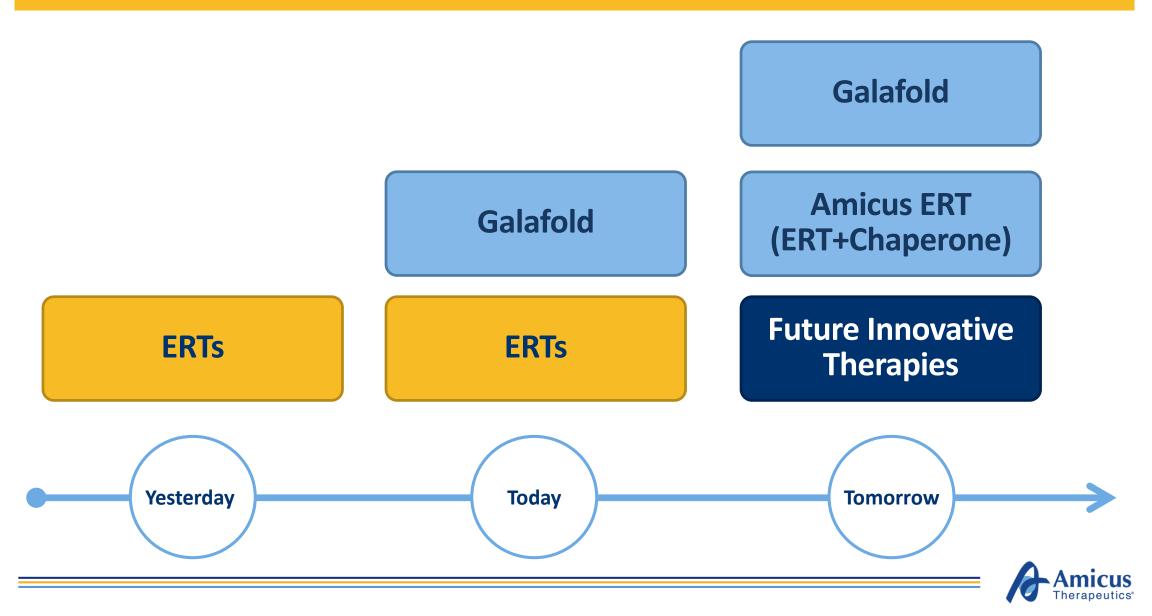


<sup>&</sup>lt;sup>1</sup> Bichet, et al., A Phase 2a Study to Investigate the Effect of a Single Dose of Migalastat HCl, a Pharmacological Chaperone, on Agalsidase Activity in Subjects with Fabry Disease, LDN WORLD 2013.



### Fabry Franchise Strategy

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



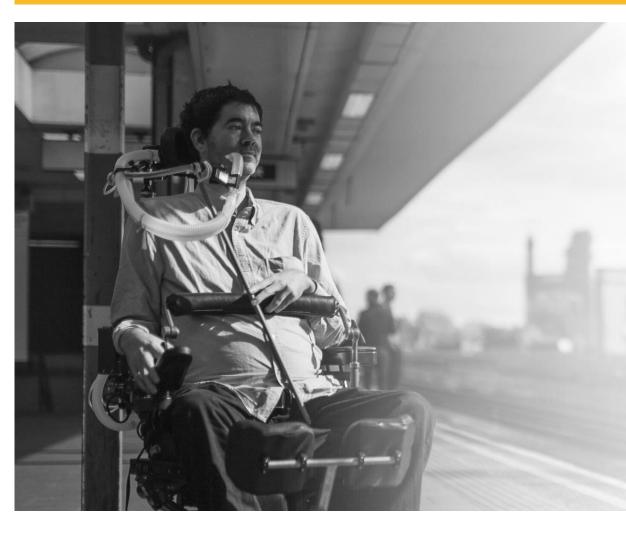


# ATB200 Novel ERT for Pompe Disease

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 WW<sup>1</sup>
- ~\$800M+ Global Pompe ERT sales in FY15<sup>2</sup>



### 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<sup>1</sup>

Protein Aggregation



Tolerability / Immunogenicity

Infusion-associated reactions in >50% of late-onset patients<sup>3</sup>

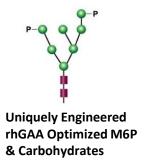
Antibody titers shown to affect treatment outcomes<sup>4,5</sup>



Uptake/ Targeting

Low M6P receptor uptake into skeletal muscle<sup>2</sup>

Vast majority of rhGAA not delivered to lysosomes<sup>2</sup>



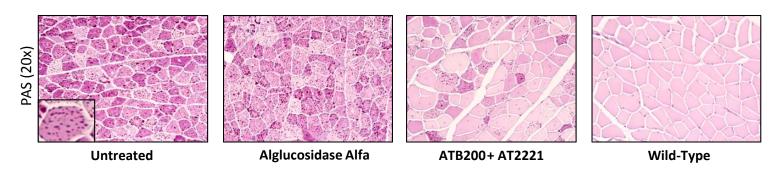
1Khanna et al., PLoS ONE, 2012; 2Zhu et al., Amer. Soc. Gene Therapy, 2009 June; 3Banati et al., Muscle Nerve, 2011 Dec.; 4Banugaria et al., Gen. Med., 2011 Aug.; 5de Vries et al., Mol Genet Metab., 2010 Dec.



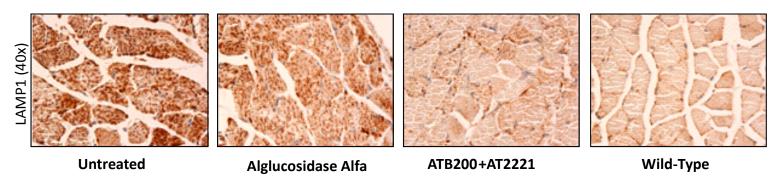
### **Preclinical Proof of Concept**

#### ATB200 + Chaperone Results in Improved Substrate Clearance in Preclinical Models<sup>1</sup>

#### PAS-glycogen staining in Quadriceps



### **LAMP1** Immunohistochemical staining in Soleus

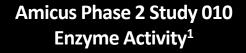


1. Following 2 doses of 20mg/kg alglucosidase alfa or ATB200 + AT2221 in Gaa KO mice, skeletal muscles evaluated for glycogen clearance and proliferated lysosomes. Treatment with alglucosidase alfa modestly reduced glycogen or proliferated lysosomes while ATB200, co-administered with AT2221 significantly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

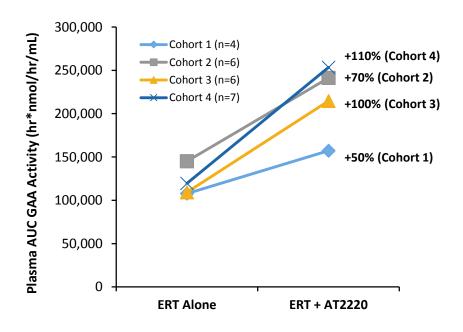


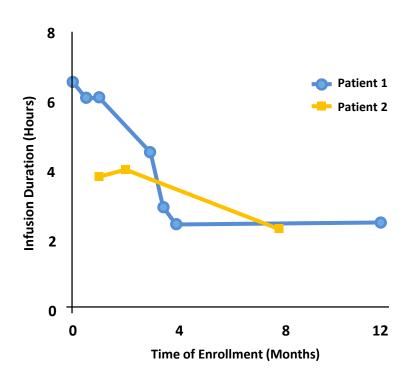
### Human Proof-of-Concept: Currently Marketed ERT + Chaperones

#### **ERT Activity Increased and Infusion Time Decreased with ERT + Chaperone**



### Investigator-Initiated Study Infusion Time<sup>2</sup>







<sup>&</sup>lt;sup>1</sup> Kishnani, et al., LDN WORLD 2013

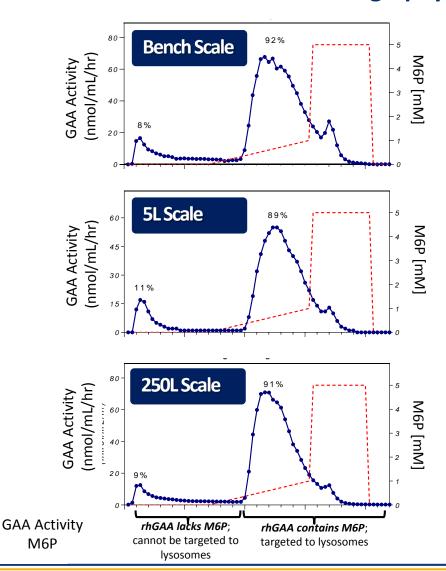
<sup>&</sup>lt;sup>2</sup> Doerfler, et al. WORLD 2014

<sup>\*</sup> 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**

Stage 1 (Single Ascending Dose)

Single Dose ATB200 Every Other Week

ATB200
5 mg/kg

ATB200
10 mg/kg

ATB200
20 mg/kg

Week 2

Week 4

Week 6

Stage 2 (Multiple Ascending Dose)

Fixed Dose ATB200 + Chaperone (AT2221)

Every Other Week

ATB200 20mg/kg + AT2221 (Low Dose) ATB200 20mg/kg + AT2221 (High Dose)

Weeks 8, 10, 12

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

Weeks 14, 16, 18

- Pharmacodynamics
- Efficacy (Long-Term Extension)



### Pompe Clinical Study ATB200-02 Data Cascade

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

### Pompe Data Cascade 4Q16 Through 2017

Data in initial ambulatory ERTswitch patients (N=~4,Cohort 1)

Additional data & initial extension data in Cohort 1

Data in ERT-naïve patients (Cohort 2)

#### 18-WEEK DATA

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

#### **EXTENSION DATA**

Motor/pulmonary function

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

Additional extension study data (all Cohorts)





# SD-101 for Epidermolysis Bullosa (EB)

Poised to deliver pivotal data for a devastating rare disease

#### **EB Disease Overview**

#### Rare, Devastating, Connective Tissue Disorder with No Approved Treatments



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



### Three Major EB Types Represent ~99% of EB Population

#### **Multiple Types...Single Devastating and Fatal Genetic Disorder**

#### **Simplex**



**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 study<sup>1</sup>
- Ages 6 months 9 years
- All baseline target wounds ≥ 10 cm<sup>2</sup>
- 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







### Phase 2b Design (Study 003)

#### 3-Month, Double-Blind Treatment Period<sup>1</sup>

SD-101 6% (n=15)

SD-101 3% (n=16)

Placebo (n=17)

#### Primary Efficacy Endpoint: Target Wound Healing at Month 1

Baseline wound: Chronic (≥ 21 days), size 5-50 cm<sup>2</sup>

#### **Secondary Efficacy Endpoints Include:**

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

**Optional Extension (SD-004)** 

Open-Label SD-101 6%

42/44 Patients entered extension study

\$400K FDA Grant for Extension Study

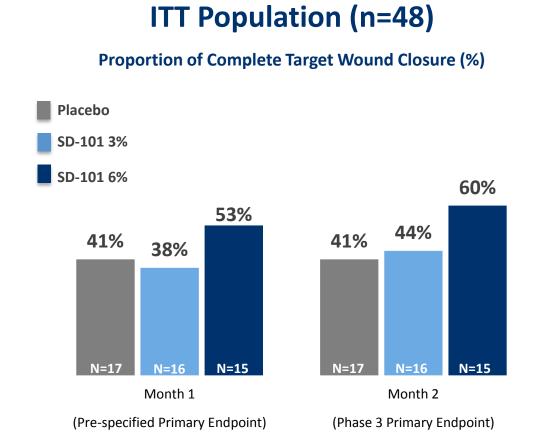
#### 48 EB patients (age ≥ 6 months)¹ - 1:1:1 Randomization - Daily Topical Application

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)



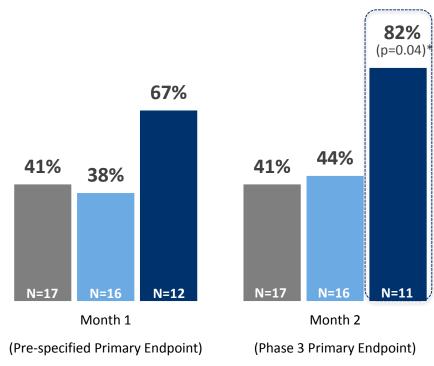
### Phase 2b Results

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



### **Evaluable Population<sup>1</sup> (n=45)**





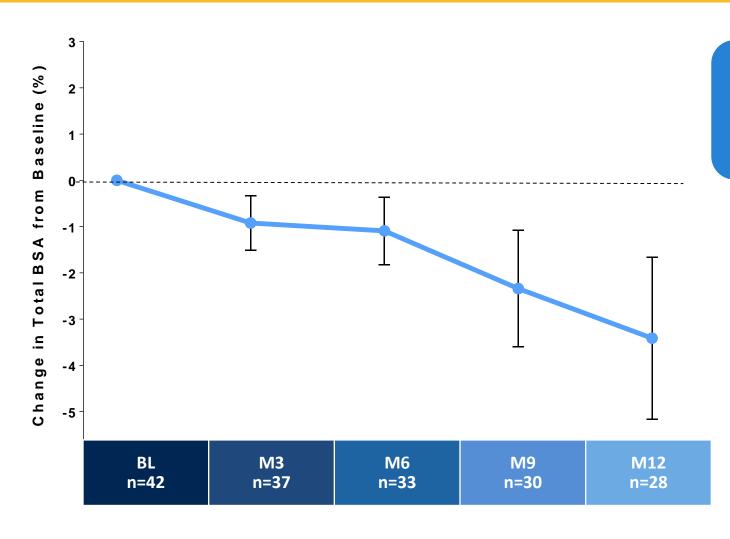
\*SD-101 6% vs placebo, unadjusted p=0.04

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



### Phase 2b Extension (Study 004) Results

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



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

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



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

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



#### PHASE 3 ESSENCE STUDY STATUS

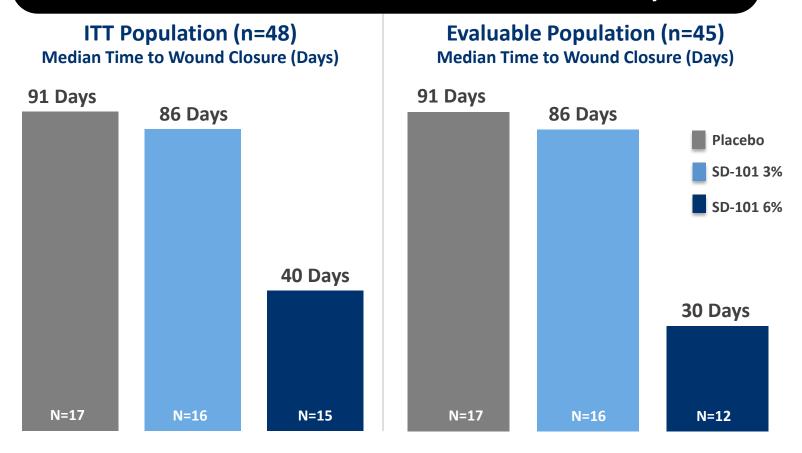
- >50% of target enrollment achieved
- 100% conversion to extension study (SD-006)
- Top-line Phase 3 data anticipated 1H17



### Elevation of Time to Wound Closure as Co-primary Endpoint

FDA 2006 Guidance Document<sup>1</sup> States Time to Wound Closure is an Acceptable Primary Efficacy Endpoint

#### **Median Time to Wound Closure in Phase 2b Study**



#### **Time to Wound Closure**

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

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



### Phase 3 ESSENCE Study Design (SD-005)

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

#### 3-Month, Double-Blind Treatment Period

SD-101 6%

~150 EB patients (age ≥ 1 month)

Baseline wound: Chronic ( $\geq 21$  days), size  $\geq 10$  cm<sup>2</sup>

Placebo

#### **Co-Primary Endpoints**

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

#### Secondary Endpoints Include.

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

**Covariates include age of** patient and size of wound at baseline

**Optional Extension (SD-006)** 

Open-Label SD-101 6%

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

**Average Baseline Target** Wound Size in Phase 3 Population: ~20 cm<sup>2</sup>

(August 1, 2016)

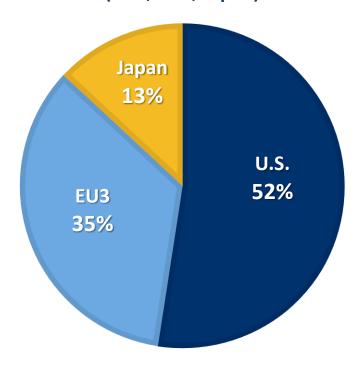


### \$1B+ Commercial Potential

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

## Diagnosed EB Patients by Geography

(U.S., EU3, Japan)



#### **Significant Unmet Clinical Need**

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

#### **Strong Support Among Surveyed Stakeholders**

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

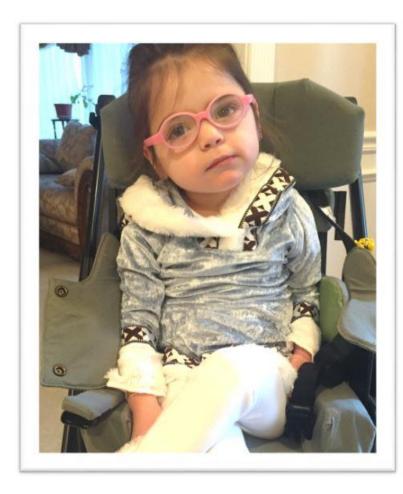
#### **Large Commercial Opportunity**

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



### Cyclin-Dependent Kinase-Like 5 (CDKL5) Deficiency

#### Rare, Devastating, Genetic Neurological Disease with No Approved Treatments



- Genetic mutations in CDKL5 gene result in deficient protein essential for normal brain development
- Persistent, spontaneous seizures starting in infancy
- Severe impairment in neurological development
- Most affected children cannot walk, talk or care for themselves
- May include scoliosis, visual impairment, sensory issues, and gastrointestinal complications
- >1,200 documented cases worldwide<sup>1</sup>
- Patient identification rising significantly



### Strategic Fit with Amicus Vision and Biologics Pipeline

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

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

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

Partnering with CDKL5 community to raise awareness and advance toward treatment

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

-John F. Crowley, Chairman and CEO of Amicus

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

- Michael Jasulavic, Founder of MiaMed

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

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



# **Financial Summary**

**Strong Balance Sheet to Invest in Rare Disease Pipeline** 

### **Strong Balance Sheet**

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

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

### Key Drivers of Value

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

#### **Fabry**

- Galafold Precision Medicine (Small Molecule)
- EU Full Approval
- Launched in Germany (May 30, 2016)
- U.S. regulatory update anticipated 4Q16

### **Epidermolysis** Bullosa (EB)

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

#### **Pompe**

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

**R&D Engine and Continued Business Development Activity** 



# Thank You

