

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, D.C. 20549**

**FORM 8-K**

**CURRENT REPORT PURSUANT TO  
SECTION 13 OR 15(D) OF THE  
SECURITIES EXCHANGE ACT OF 1934**

Date of Report (Date of earliest event reported): **May 22, 2017**

**AMICUS THERAPEUTICS, INC.**  
(Exact Name of Registrant as Specified in Its Charter)

**Delaware**  
(State or Other Jurisdiction of  
Incorporation)

**001-33497**  
(Commission File Number)

**71-0869350**  
(IRS Employer Identification No.)

**1 Cedar Brook Drive, Cranbury, NJ**  
(Address of Principal Executive Offices)

**08512**  
(Zip Code)

Registrant's telephone number, including area code: **(609) 662-2000**

(Former Name or Former Address, if Changed Since Last Report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

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**Item 8.01. Other Events.**

The senior management of Amicus Therapeutics, Inc. (the "Company") is using the presentation attached as Exhibit 99.1 to this Current Report in its current meetings with investors and analysts.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits: The Exhibit Index annexed hereto is incorporated herein by reference.

Exhibit No.	Description
99.1	Presentation Materials — Corporate Overview (May 22, 2017)

## SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Amicus Therapeutics, Inc.

Date: May 22, 2017

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

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## EXHIBIT INDEX

<u>Exhibit No.</u>	<u>Description</u>
99.1	Presentation Materials — Corporate Overview (May 22, 2017)

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# Corporate Overview

May 22, 2017

## 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. In particular, this presentation relates to the preclinical and preliminary clinical data from a global Phase 1/2 study (ATB200-02) to investigate ATB200/AT2221. The inclusion of forward-looking statements arising from this preliminary data and study 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, 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 for any of our product candidates, including ATB200/AT2221 and SD-101. The preliminary data and Phase 1/2 study investigating ATB200/AT2221 discussed herein is inherently preliminary and early in the study, derived from a limited patient set, and later trial results with this patient set or others may not be consistent with these preliminary results. With respect to statements regarding projections of our cash position, actual results may differ based on market factors and our ability to execute operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our previous filings with the SEC and in our Annual Report on Form 10-K for the year ended December 31, 2016. 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.*

# Building a Top Global Biotech in Devastating Rare Diseases

 **Galafold**  
(migalastat)  
FIRST ORAL PRECISION MEDICINE  
FOR FABRY DISEASE

**3**  
**PROGRAMS**  
IN CLINIC IN 3 RARE  
DISEASES

**1**  
**BREAKTHROUGH**  
THERAPY DESIGNATION

WORLD CLASS  
SCIENCE &  
DRUG  
DEVELOPMENT

**ATB200/AT2221**  
NOVEL TREATMENT PARADIGM  
FOR POMPE IN PHASE 1/2

TREATING  
PATIENTS IN  
24 COUNTRIES

**Two Phase 3**  
**PROGRAMS**  
(FABRY & EB)

**\$3B+** MARKET  
OPPORTUNITY FOR  
CURRENT PIPELINE

PROTEIN  
ENGINEERING &  
GLYCOBIOLOGY

**~\$280M CASH**  
**BALANCE**





## 2017 Key Strategic Priorities

We Remain Sharply Focused on FIVE Key Strategic Priorities as We Continue to Build a Top Global Biotechnology Company Focused on Rare Devastating Diseases

**Advance International Galafold Launch**

**Submit Japanese New Drug Application (J-NDA) for Migalastat**

**Establish Definitive Proof of Concept for ATB200/AT2221 with Clear Path to Registration for Pompe Disease**

**Successfully Complete Phase 3 EB Study**

**Maintain Financial Strength**

# Our Vision – Maximizing Impact on Patients to Drive Shareholder Value

## The Ultimate Measure of Our Success Will be the Number of Patients with Devastating Rare Diseases Treated with an Amicus Product



= 20 patients

~37 Patients

~90 Patients

~250 Patients\*

~800 Patients\*

~5,000 Patients\*

2010

2014

Today

2018

2023

\*Clinical & Commercial





# Galafold™ (Migalastat) Precision Medicine for Fabry Disease

Continue Launch Execution and Geographic Expansion



# 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

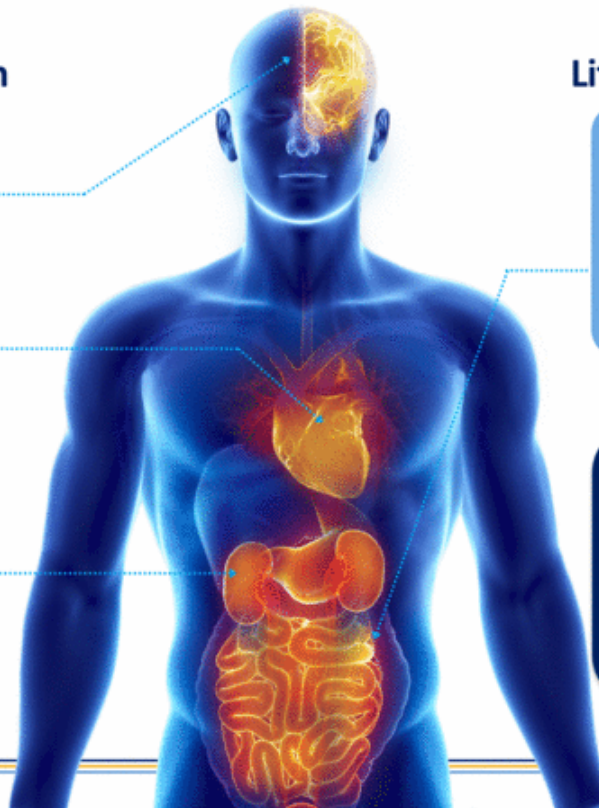
### Life-Limiting Symptoms

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

- Nausea, vomiting, cramping, and diarrhea
- Pain/bloating after eating, feeling full
- Constipation
- Difficulty managing weight

### Key Facts

- Deficiency of  $\alpha$ -Gal A enzyme leading to GL-3 accumulation
- >900 known mutations
- 5-10K diagnosed WW (51% female/49% male<sup>4</sup>)
- Newborn screening studies suggest prevalence of ~1:1000 to ~1:4000

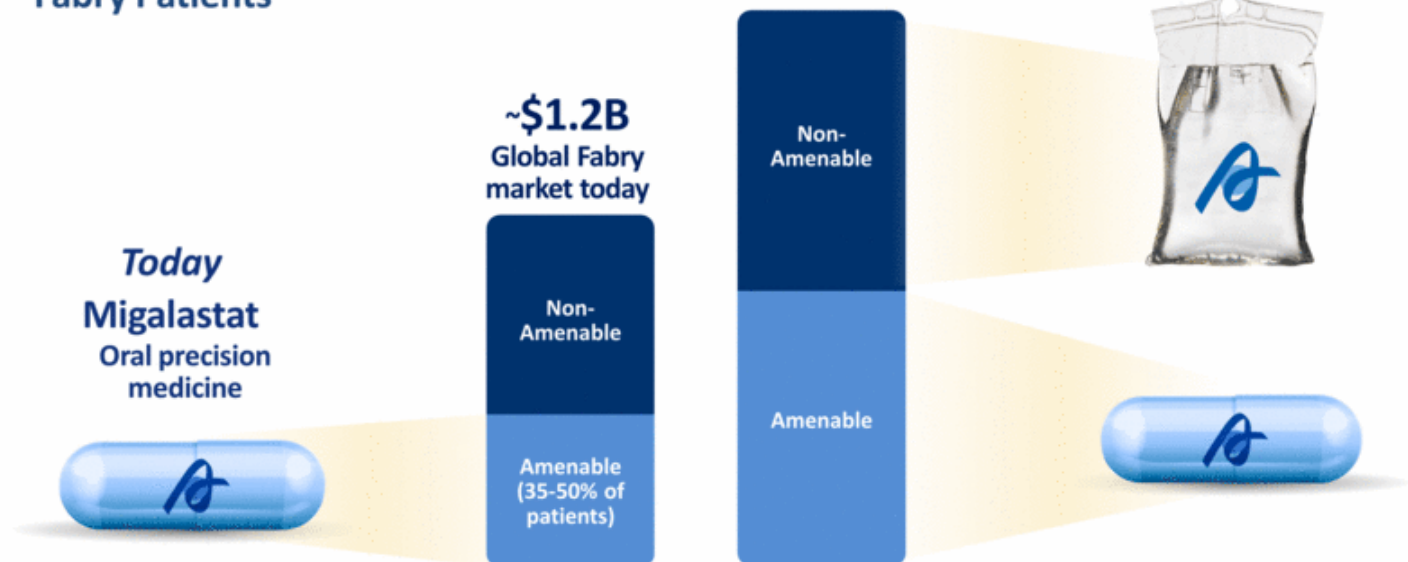


1. Desnick R, et al. *Ann Intern Med.* 2003 2. Yousef Z, et al. *Eur Heart J.* 2013 3. Germain D. *Orphanet J Rare Dis.* 2010 4. Fabry Registry 2011

# Precision Medicine Driven by a Patient's Genotype

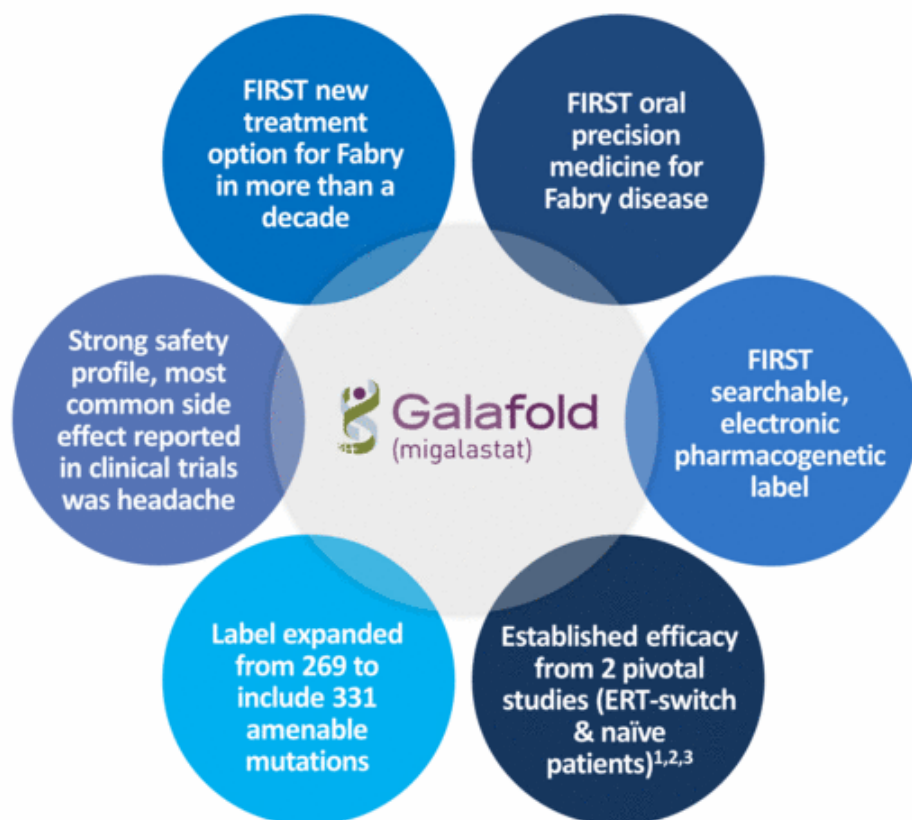
Amicus Therapeutics is Committed to Innovative R&D to Develop the Highest Quality Therapies for ALL Fabry Patients

*Future Vision*  
Novel ERT co-formulated with migalastat



\*Artist rendering, not actual product image

# Full EU Approval as First Oral Precision Medicine for Fabry Disease



Galafold Indicated for Long-Term Treatment of Adults and Adolescents Aged  $\geq 16$  years with a Confirmed Diagnosis of Fabry Disease and Who have an Amenable Mutation<sup>3</sup>

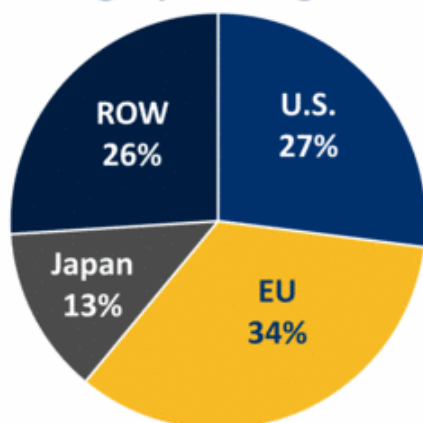
- Approved May 30, 2016
- Launch exceeding expectations

1. Germain, DP et al., New England Journal of Medicine. 2. Hughes, et al., Journal of Medical Genetics. 3. For important safety information for Galafold visit [www.ema.europa.eu](http://www.ema.europa.eu).

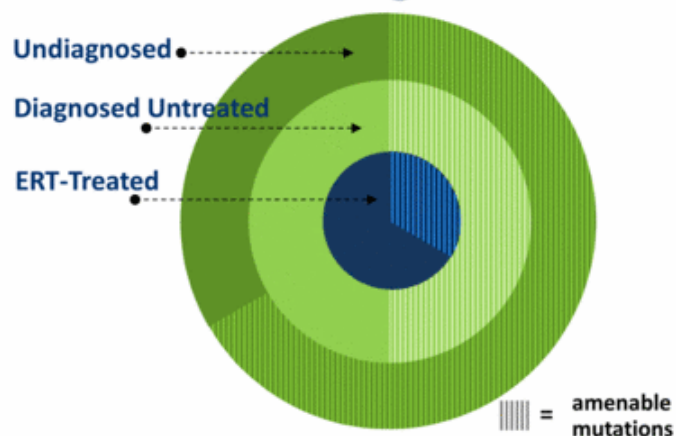
# Galafold Commercial Opportunity

Prioritizing EU, Japan, and Other Large Fabry Markets to Address Patients with Amenable Mutations (35%-50% of Fabry Population)

## Geographic Segments



## Patient Segments



- 5k-10k Patients Diagnosed WW
- 40%-50% of Diagnosed Patients not on ERT
- Newborn Screening Studies Suggest Prevalence of ~1:1000 to ~1:4000<sup>1</sup>

1. Burton, LDN WORLD Symposium, 2012 Feb. Mechtler et al., *The Lancet*, 2011 Dec. Hwu et al., *Hum Mutation*, 2009 Jun. Spada et al., *Am J Human Genet.*, 2006 Jul

## Successful International Launch Underway (as of 4/30/17)

**Initial Launch Success Driven by Germany with ERT-Switch & Naïve Patients, Reimbursement Now Available in 12 Countries Including Four of Top EU5\***

101

Patients (Switch & Naïve) on reimbursed Galafold (4/30/17)

11

Countries with available reimbursement\*

12

Countries with pricing discussions ongoing

27

Countries with Amicus footprint



\*Commercial and Expanded Access Programs (EAPs)

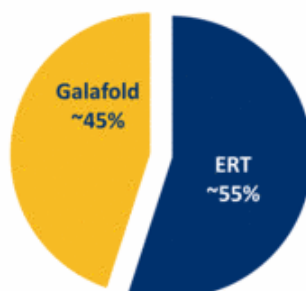


# German Launch Update (as of 4/30/17)

## Germany is an Important Indicator for EU Launch Success



Current  
Approximate  
Market Share\*



### IMPORTANT EARLY INDICATORS IN GERMANY

- Majority switch patients, but growing naïve segment
- ~45% share of amenable patients (switch and naïve)\*
- Switches from both Fabrazyme & Replagal™ commensurate with market share
- Male / female mix
- Most major centers prescribing
- Final price to be effective in 2Q17

\*Market share assumptions based on estimated number of ERT-treated patients and naïve patients with amenable mutations in Germany as of April 2017

## UK Market Dynamics

**Galafold Positioned for Success Following Positive Final NICE Publication and more than a Decade of Clinical Experience Among Largest Treatment Centers**



### MARKET DYNAMICS IN THE UK

- Funding effective May 23, 2017
- Highly concentrated at major centers
- Clinical experience at multiple sites
- ~450 ERT-treated patients
- 50%+ amenability rate projected\*

***“Migalastat has a lower total cost than ERT, and potentially provides greater health benefits than ERT.”***

-NICE Highly Specialised Technologies Guidance [HST4]\*\*

\* Estimates based on detailed market mapping and physician chart reviews

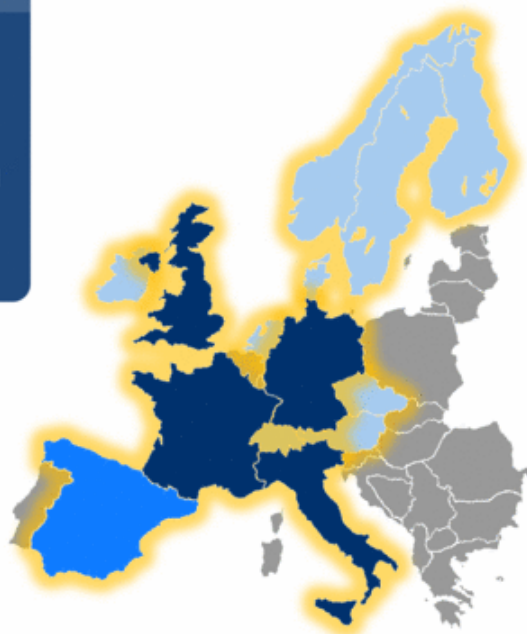
\*\* Evidence-based recommendations on migalastat (Galafold) for treating Fabry disease in people over 16 - [www.nice.org.uk/guidance/hst4](http://www.nice.org.uk/guidance/hst4)

# EU Launch Strategy

## Focus on EU Top 5 Plus Key Mid-Sized EU Markets in 2017

### INITIAL FOCUS ON TOP 5 COUNTRIES

- Launched in Germany, UK, Italy and France
- Spain reimbursement discussions underway
- ~2,000 Fabry patients treated
- ~70-75% of EU market value
- ~25% of global Fabry market

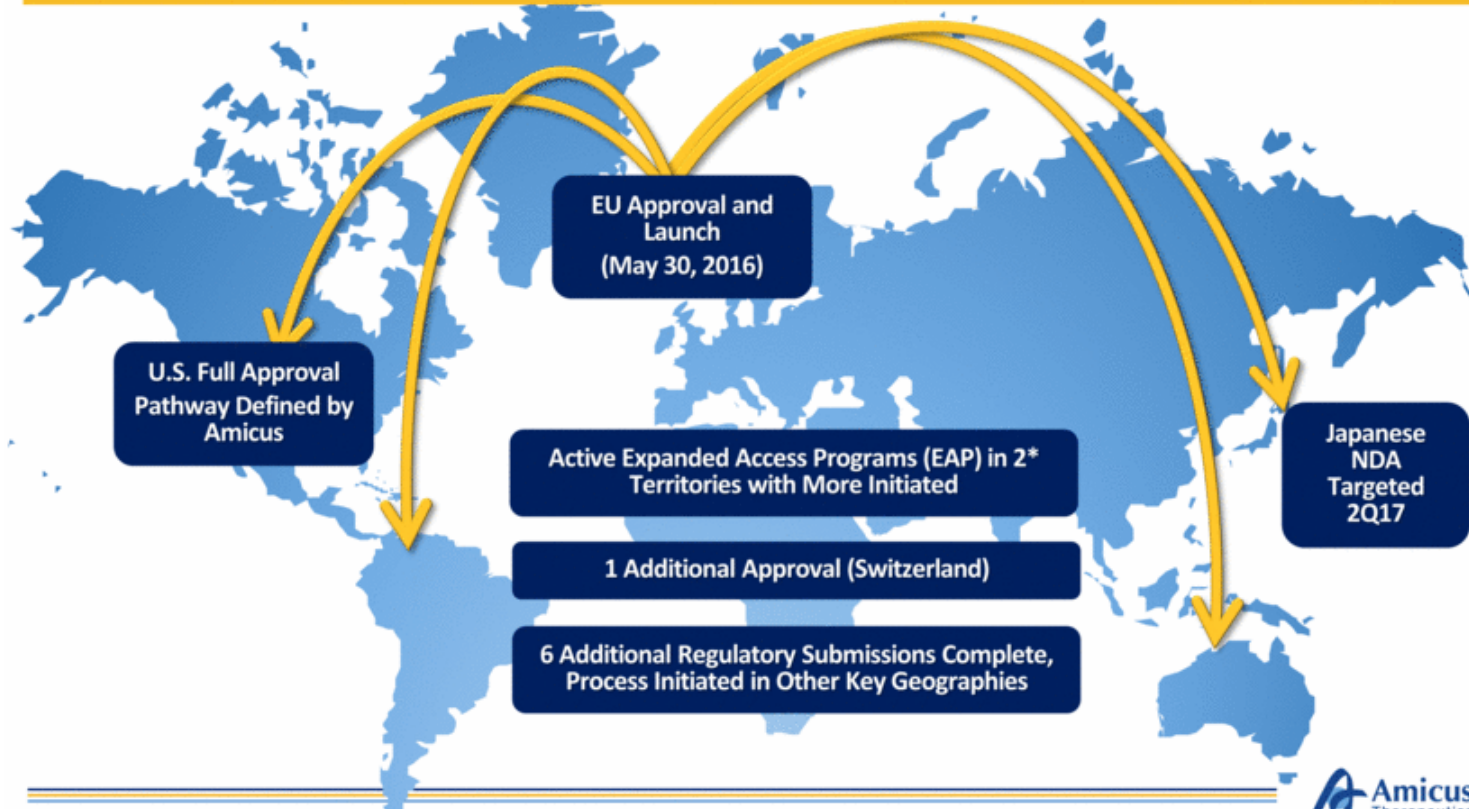


### INVEST IN KEY MID-SIZED EU COUNTRIES AND SELECT EAP OPPORTUNITIES

- Austria, Nordics, Netherlands, Belgium, etc.
- ~10% of EU market value
- Selectively invest in key EAP markets

# Global Regulatory Strategy to Reach More Patients

EU Approval is Gateway to ~75% of Global ERT Market



\* Two EAPs converted to commercial reimbursement





## Amicus Proprietary Fabry ERT

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

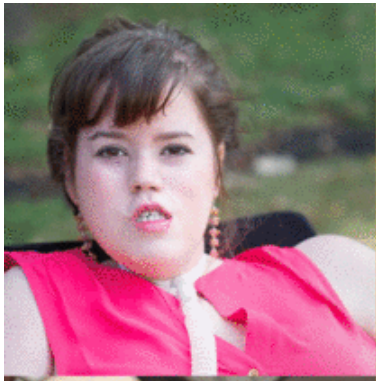
### **Development status:**

- Cell line transferred to manufacturer
- Preclinical data update in 2017

### **Fabry ERT Target Product Profile:**

- Improved drug targeting to key tissues
- Significantly more potent dose delivery
- Co-formulation with chaperone to enhance stability
- Dosing flexibility





# ATB200 Novel ERT for Pompe Disease

Establishing Human Proof of Concept and Validating  
Biologics Platform in 2017

# Pompe Disease Overview

## Pompe Disease is Heterogeneous Across a Broad Spectrum of Patients

Deficiency of GAA leading to glycogen accumulation

Respiratory and cardiac failure are leading causes of morbidity and mortality

Age of onset ranges from infancy to adulthood

5,000 – 10,000 patients diagnosed WW<sup>1</sup>

Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

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



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

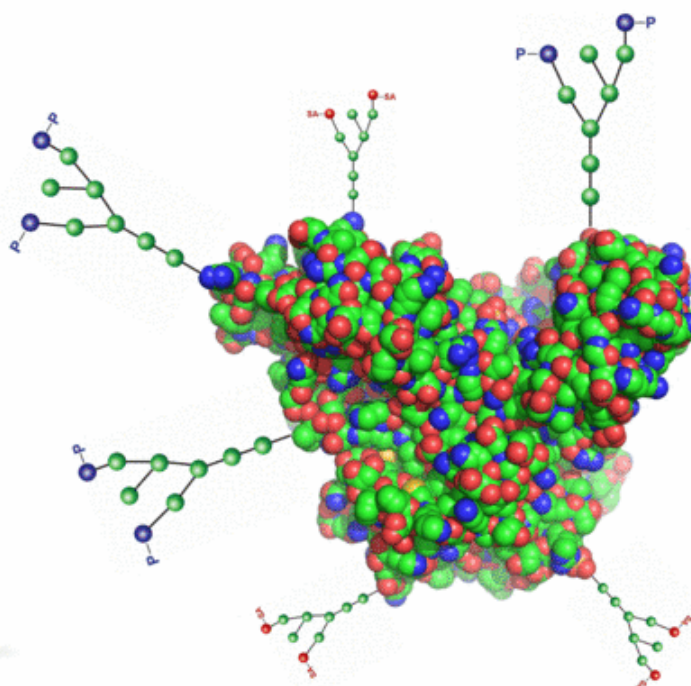
# ATB200 + Chaperone: A Highly Differentiated Approach

## Novel Pompe Treatment Paradigm with Three Key Differentiators

**ATB200  
(Novel ERT)**



**Chaperone  
addition**



**Optimized  
mixture of  
glycans**

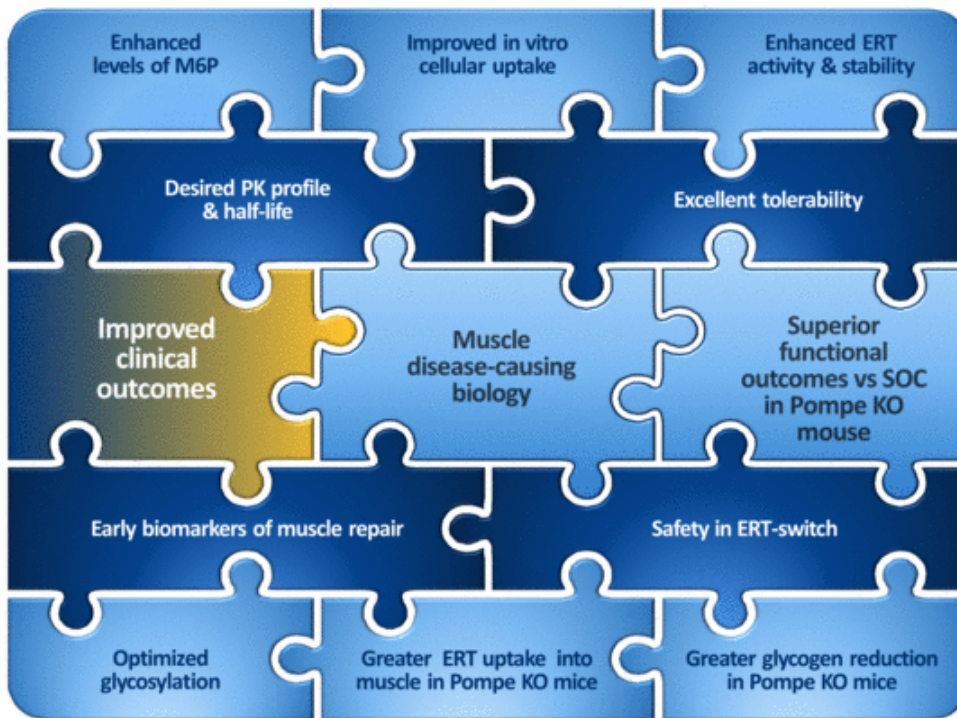
**High levels of  
M6P and bis  
M6P**

\*Artist rendering, not actual product image



# Pompe Disease: A Complex Disease with Significant Unmet Needs

We've Made Great Strides and Expect to Address Key Remaining Questions in 2017



preclinical

clinical

key question

*"The scientific findings and preclinical data are profound and shed new light on questions about the underlying cause of muscle damage and weakness in Pompe patients. Furthermore, these results provide a window into a potential underlying link among key muscular dystrophies, such as Pompe, Limb Girdle, and Duchenne. Amicus has been a pioneer in advancing the scientific understanding of Pompe disease and in developing next-generation therapies for patients."*

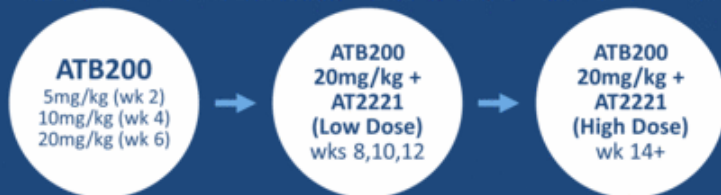
Grace K. Pavlath, Ph.D., Senior Vice President, Scientific Program Director of Muscular Dystrophy Association

# Phase 1/2 ATB200-02 Study Design

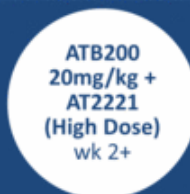
**Phase 1/2 Clinical Study to Evaluate Safety, Tolerability, Pharmacokinetics (PK), and Pharmacodynamics (PD) of ATB200 + Chaperone (ATB200/AT2221)**

## 18-Week Primary Treatment Period with Long-Term Extension (n=20)

Cohort 1 (Ambulatory ERT-Switch, n=11)



Cohort 2 (Non-Ambulatory ERT-Switch, n=4) & Cohort 3 (ERT-Naïve, n=5)



### Assessments:

- Safety/Tolerability
- Plasma PK
- Infusion-Associated Reactions
- Antibody & Cytokine Levels
- Pharmacodynamics
- Efficacy (Long-Term Extension)



## 6-Minute Walk Test (6MWT) Summary at Month 6 (n=9)

6MWT Distance Improved for Both ERT-Naïve Patients (Mean +52 Meters)  
and ERT-Switch Patients (Mean +38 Meters) at Month 6

### 6-Minute Walk Test (m): Month 6

Cohort	Baseline Mean (SD)	Change at Month 6 Mean (SD)
Cohort 3 ERT Naïve (n=2)	432 (68)	+52 (15)
Cohort 1 ERT Switch (n=7)	383 (103)	+38 (43)

6MWT Increased in 2/2 ERT-Naïve Patients and 6/7 ERT-Switch Patients

## Other Motor Function Tests at Month 6 (n=9)

Other Motor Function Tests Show Improvements for Both ERT-Naïve and ERT-Switch Patients, Consistent With 6MWT

### Other Motor Function Tests: Month 6

Patients	Timepoint	4 Stair Climb Mean (SD) (sec)	Timed Up and Go Mean (SD) (sec)	10M walk Mean (SD) (sec)
Cohort 3: ERT Naïve (n=2)	Baseline	3.9 (0.6)	8.9 (0.9)	6.9 (0.8)
	Change at Month 6	-0.3 (0.0)	-1.4 (0.4)	-0.5 (0.2)
Cohort 1: ERT Switch (n=7)	Baseline	4.4 (3.1)	11.0 (7.7)	7.5 (3.5)
	Change at Month 6	-1.1 (1.3)	-1.9 (2.8)	-0.04 (1.6)

## Cohort 2 Muscle Strength Testing at Month 6 (n=1)

Substantial Improvement Observed in Shoulder and Elbow Strength in First Non-Ambulatory ERT-Switch Patient with Available Data at Month 6

### Quantitative Muscle Testing (QMT) - Dynamometer

Assessment	Elbow Flex		Elbow Extension		Shoulder Adduction		Shoulder Abduction		<b>Scoring</b> Measurement of force production in pounds as measured by dynamometer
	Right	Left	Right	Left	Right	Left	Right	Left	
Baseline	1.0	0.9	1.2	1.1	0.8	0.5	1.3	0.9	
Month 6	4.1	3.3	3.5	3.2	2.8	0.0	3.3	3.6	
CFBL	+3.1	+2.4	+2.3	+2.1	+2.0	-0.5	+2.0	+2.7	

### Manual Muscle Testing (MMT)\*

Assessment	Elbow Flex		Elbow Extension		Shoulder Adduction		<b>Scoring</b> 1. Visible muscle movement, but no movement at the joint 2. Movement at the joint, but not against gravity 3. Movement against gravity, but not against added resistance 4. Movement against resistance, but less than normal 5. Normal strength
	Right	Left	Right	Left	Right	Left	
Baseline	2	2	2	2	2	2	
Month 6	4	3	4	3	2	2	
CFBL	+2	+1	+2	+1	0	0	

\*R/L shoulder abduction by MMT not assessed at M6

## Forced Vital Capacity (FVC) Summary at Month 6 (n=8)\*

FVC Results Show Improvement in ERT-Naïve Patients (Mean +3.0%) and Stability in ERT-Switch Patients (Mean +0.3%) at Month 6

### FVC (% Predicted): Month 6

Cohort	Baseline Mean (SD)	Absolute Change at Month 6 Mean (SD)
Cohort 3 ERT Naïve (n=2)	51 (27)	+3 (0)
Cohort 1 ERT Switch (n=6)*	51 (17)	+0.3 (3)

**FVC increased in 2/2 ERT-Naïve patients and 3/6 ERT-Switch patients**

\*FVC results not available for 1 subject at month 6

## Other Pulmonary Function Tests at Month 6 (n=8-9)\*

MIP increased and MEP decreased in ERT-naïve patients,  
MIP and MEP both increased in ERT-switch patients

### Other Pulmonary Function Tests: Month 6

Patients	Timepoint	MIP Mean (SD)	MEP Mean (SD)
Cohort 3: ERT Naïve (n=2)	Baseline	45.5 (27.6)	57.5 (9.2)
	Change at Month 6	+8.5 (3.5)	-4.5 (17.7)
Cohort 1: ERT Switch (n=6-7)*	Baseline	35.4 (11.3)	69.5 (21.2)
	Change at Month 6	+1.0 (5.2)	+15.5 (25.4)

\*MEP results not available for 1 patient at month 6



## Functional Data Summary (n=10)

- **Muscle function at Month 6**

- Muscle function improved in 9/10 patients
- Mean 6MWT distance improved in both naïve (+52 Meters) and ERT-switch (+38 Meters) patients (8 out of 9)
- Other motor function tests in ambulatory patients consistent with 6MWT
- First non-ambulatory patient showed significant improvements in muscle strength tests

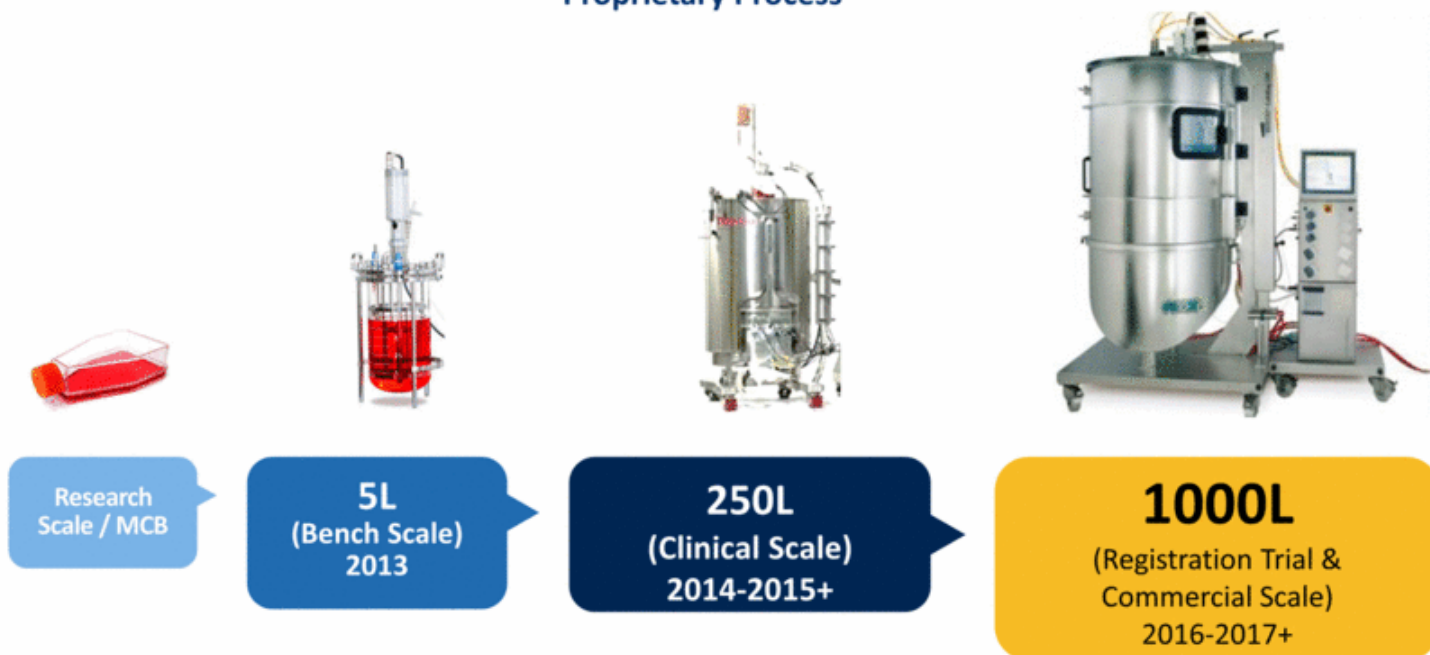
- **Pulmonary function at Month 6**

- FVC increased in ERT-naïve patients (mean +3.0%) and was stable in ERT-switch patients (mean +0.3%)
- MIP and MEP generally consistent with FVC

# Biologics Manufacturing Capabilities

## Highly Successful Biologics Manufacturing Scale-up in Three Years

### Proprietary Process



# Pompe Phase 1/2 Study ATB200-02 Data Cascade

On Track to Report Full Data Set in 3Q17

## Pompe Milestones in 2017



### 18-WEEK DATA

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

### EXTENSION DATA

- Motor/pulmonary function



# SD-101 for Epidermolysis Bullosa

Potential First-in-Class Treatment

# EB Disease Overview

Rare, Devastating, Connective Tissue Disorder with No Approved Treatments

## Disease Overview

- Multiple genes cause disease
- Can affect internal organs
- Can be fatal
- Wounds can lead to life-threatening infections
- Diagnosis: infancy to adulthood
- 30,000 – 40,000+ diagnosed in major global regions
- \$1B+ potential market

## Three Major EB Types

(~99% of EB Population)

SIMPLEX (75%)



DYSTROPHIC (20%)



JUNCTIONAL (5%)





# Proof of Concept Findings

## Phase 2 Results Informed Phase 3 Design

### Phase 2a Key Takeaways (SD-101 3%)



1-Year-Old Girl with EB Simplex at Baseline



Following 2 months of treatment with SD-101

**Breakthrough  
Therapy  
Designation**

### Phase 2b Key Takeaways (SD-101 6%)

- Faster time to wound closure
- Higher proportion with complete closure
- Reduction in total body surface area (BSA) of wounds
- Larger wounds (>10 cm<sup>2</sup>) showed widest separation versus placebo
- Daily administration generally safe and well-tolerated

**Informed  
Phase 3  
Study Design**

## Phase 3 ESSENCE Study - Delivering on Our EB Vision

Phase 3 Study Overenrolled (>160 Patients) with Top-Line Data On Track for 3Q17



### SD-005 Study Design Optimized

- Sample size of ~150 patients
- Larger baseline target wound size
- Time to wound closure endpoint elevated

### Status

- 95%+ participation in extension study
- Study overenrolled (>160 patients)
- Top-line data anticipated 3Q17

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

### Study Design Optimized for Success – Data on Track for 3Q17

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

SD-101 6%

**>160 EB patients enrolled (age ≥ 1 month)**

*Baseline wound: Chronic (≥ 21 days), size 10 - 50 cm<sup>2</sup>*

Placebo

#### Optional Extension (SD-006)

Open-Label SD-101 6%

>95% Participation in  
Extension Study  
(May 1, 2017)

#### Primary Endpoints at Month 3 (Sequential Testing):

1. Time to target wound closure (statistical significance at  $p \leq 0.05$ )
2. Proportion of patients with target wound closure (statistical significance at  $p \leq 0.05$ )\*

#### Secondary Endpoints Include:

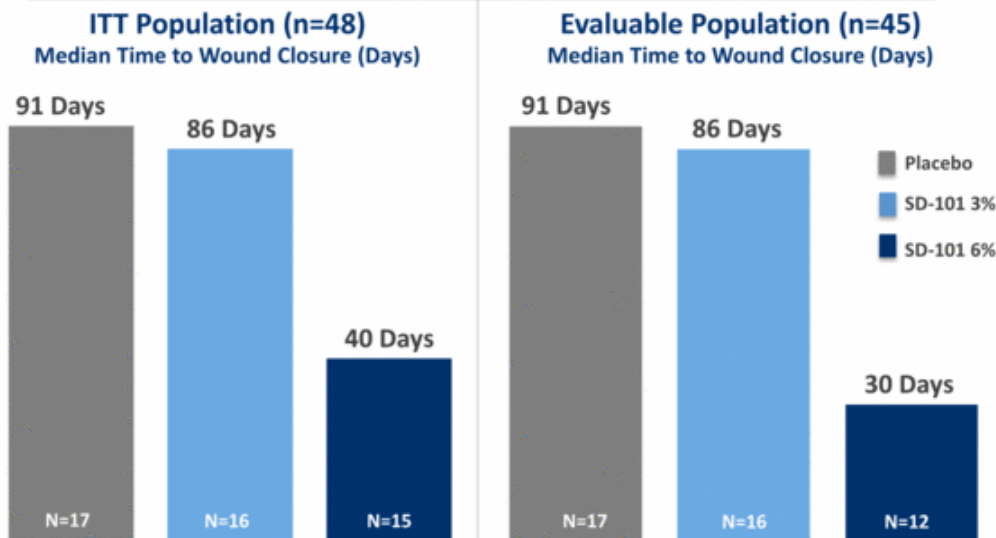
- Proportion of patients with target wound closure (at earlier timepoints)
- Change in Body Surface Area (BSA) of lesions and blisters
- Change in BSA of wounds
- Patient-reported itching
- Patient-reported pain

\*Study success based on time to target wound closure (TTWC) of  $p \leq 0.05$ . If TTWC  $p \leq 0.05$  then proportion of patients with target wound closure will be formally tested

# Elevation of Time to Wound Closure Endpoint

If Difference in Time to Wound Closure Between SD-101 6% and Placebo is Statistically Significant ( $p \leq 0.05$ ) then Phase 3 ESSENCE Study will be Considered a Success

## Time to Wound Closure in Phase 2b Study



### Time to Wound Closure

- Encouraging results in SD-101 Phase 2b study
- Time to wound closure has more power than proportion of patients with target wound closure
- Results correlate with proportion of patients with target wound closure
- Statistical simulations indicate elevation of time to wound closure increases probability of study success

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



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

**Preclinical Development Underway for a Rare, Devastating, Genetic Neurological Disease with No Approved Treatments**

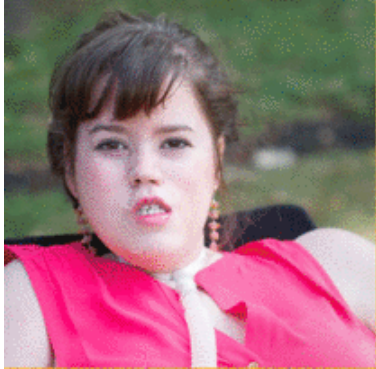
## Disease Overview

- 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



1. LouLouFoundation.org





# Financial Summary & Key Milestones

# Financial Summary & Guidance

## Strong Balance Sheet with \$279.8M Cash at 3/31/17 and Cash Runway Into 2H18

Financial Position	March 31, 2017
Cash	\$279.8M
Debt	\$250M
FY17 Net Operating Cash Flow Guidance	\$175-\$200M
FY17 Net Cash Spend Guidance*	\$200-\$225M
Cash Runway	2H18
Capitalization	March 31, 2017
Shares Outstanding	142,829,530

\*Includes third party milestone payments and capital expenditures

## Key Anticipated Milestones in 2017

### 2017

#### Fabry Disease (Galafold)

- 300 patients on reimbursed Galafold by YE17\*
- Japan NDA submission in 2Q17

#### Pompe Disease (ATB200/AT2221)

- Phase 1/2 data cascade in 2Q and 3Q
- Meetings with U.S. and EU regulators

#### Epidermolysis Bullosa (EB) (SD-101)

- Phase 3 top-line data 3Q17

#### Strong Balance Sheet

- Significant revenue contribution
- Cash runway into 2H18

\*Commercial and Expanded Access Programs (EAPs)

Thank You

