



37th Annual Cowen & Company Health Care Conference

John F. Crowley, Chairman and Chief Executive Officer
March 8, 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



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

\$330M CASH
BALANCE

Key Accomplishments in 2016

2016

Fabry Disease (Galafold™)

- EU approval
- International launch success
- Regulatory progress

Pompe Disease (ATB200/AT2221)

- Positive preliminary data in Phase 1/2 study in Pompe patients

Epidermolysis Bullosa (EB) (SD-101)

- Phase 3 enrollment near complete

Strong Balance Sheet

- \$330M in cash (12/31/16)

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

2010

~90 Patients

2014

~250 Patients*

Today

~800 Patients*

2018

~5,000 Patients*

2023



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) & STROKE¹

HEART DISEASE²

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

KIDNEY DISEASE³

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

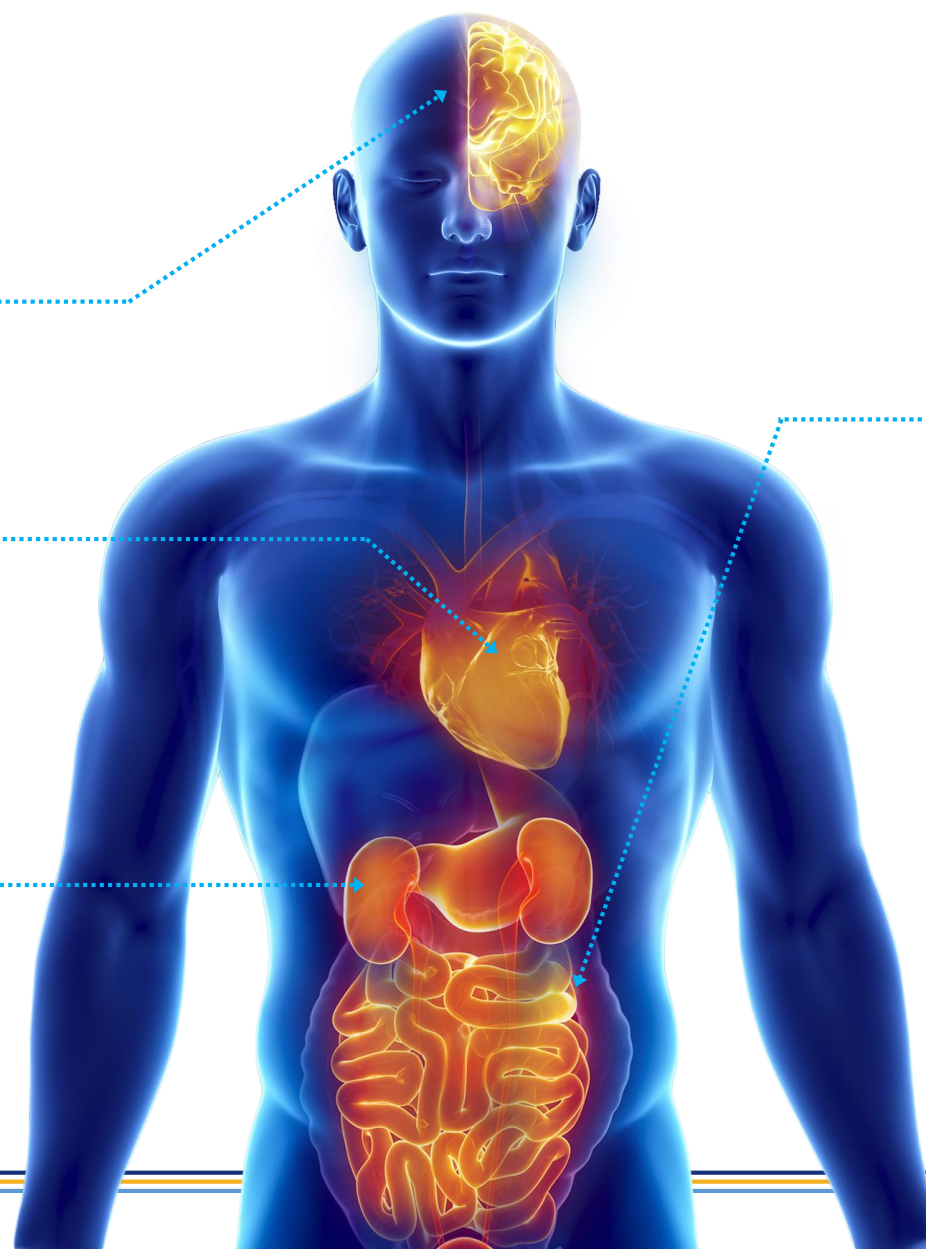
Life-Limiting Symptoms

GASTROINTESTINAL³

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

Key Facts

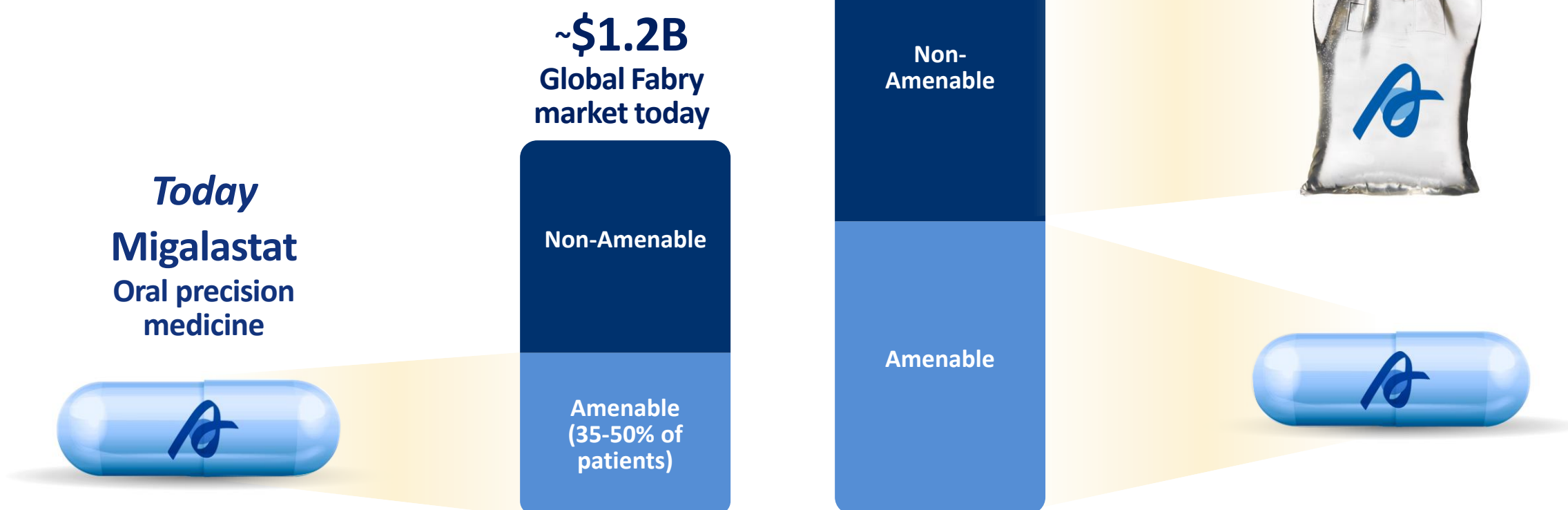
- Deficiency of α -Gal A enzyme leading to GL-3 accumulation
- >900 known mutations
- 5-10K diagnosed WW (51% female/49% male⁴)
- Newborn screening studies suggest prevalence of ~1:1000 to ~1:4000



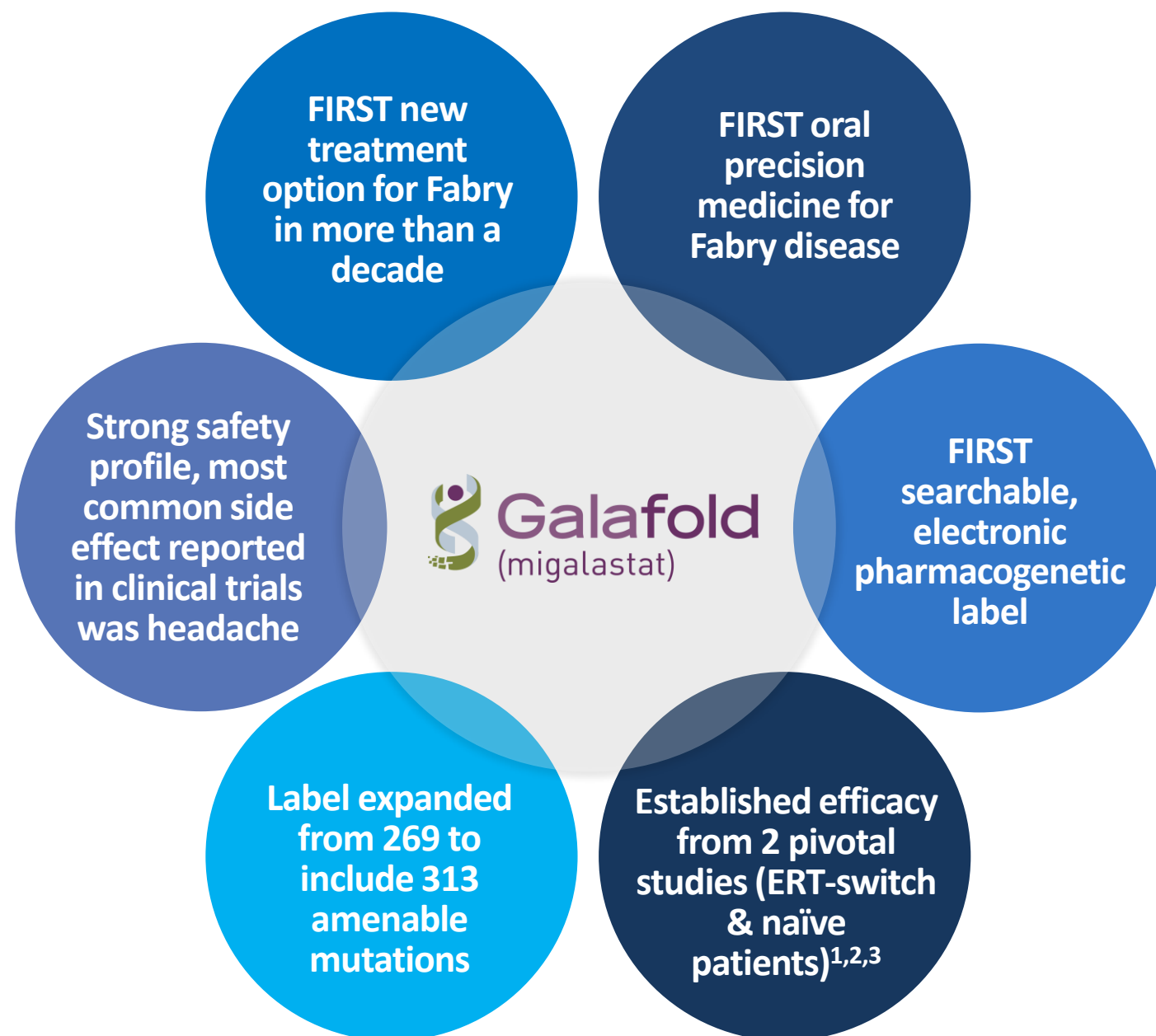
1. Desnick R, et al. *Ann Intern Med.* 2003 2. Yousef Z, et al. *Eur Heart J.* 2013 3. Germain D. *Orphanet J Rare Dis.* 2010 4. Fabry Registry 2011

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



Full EU Approval as First Oral Precision Medicine for Fabry Disease



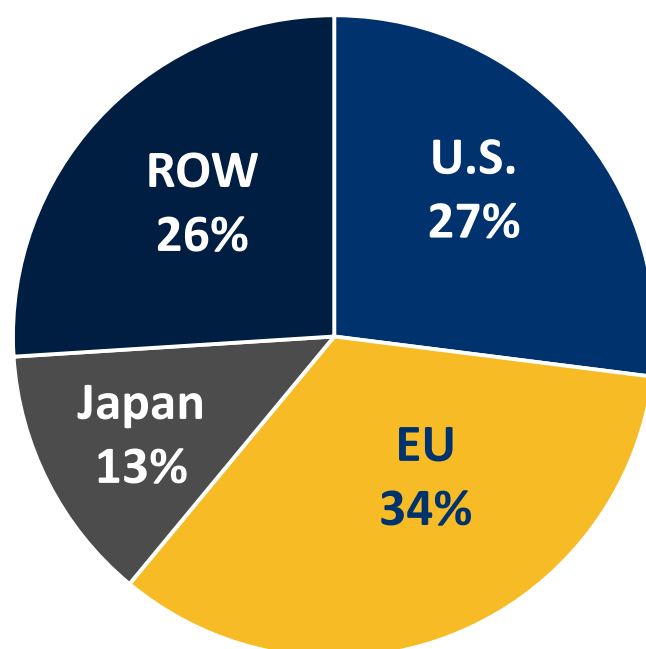
Galafold Indicated for Long-Term Treatment of Adults and Adolescents Aged ≥ 16 years with a Confirmed Diagnosis of Fabry Disease and Who have an Amenable Mutation³

- **Approved May 30, 2016**
- **Launch exceeding expectations**

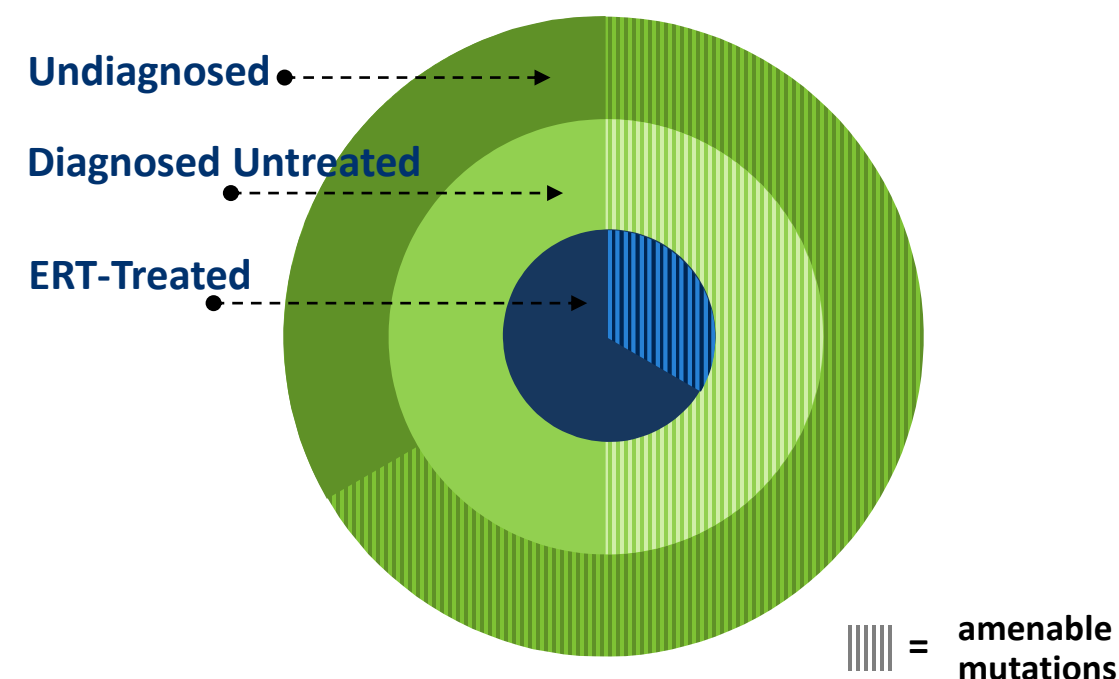
Galafold Commercial Opportunity

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 ~1:4000¹

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

Early Success with International Launch (as of 2/28/17)

**Initial Launch Success Driven by Germany with ERT-Switch & Naïve Patients,
Reimbursement Now Available in 10 Countries***

75

Patients (Switch & Naïve) on
reimbursed Galafold (2/28/17)

10

Countries with available reimbursement*

14

Countries with pricing discussions ongoing

27

Countries with Amicus footprint

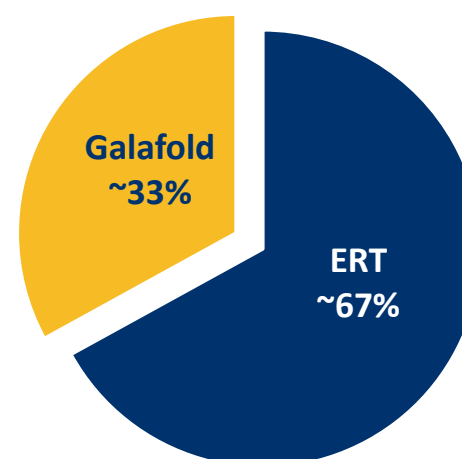


German Launch Update (as of 2/28/17)

Germany is an Important Indicator for EU Launch Success



Current
Approximate
Market Share*



IMPORTANT EARLY INDICATORS IN GERMANY

- Vast majority switch patients
- ~33% of eligible switch patients now on Galafold*
- All newly experienced patients & physicians
- Majority of switches from Replagal™
- Male / female mix
- 16 unique prescribers

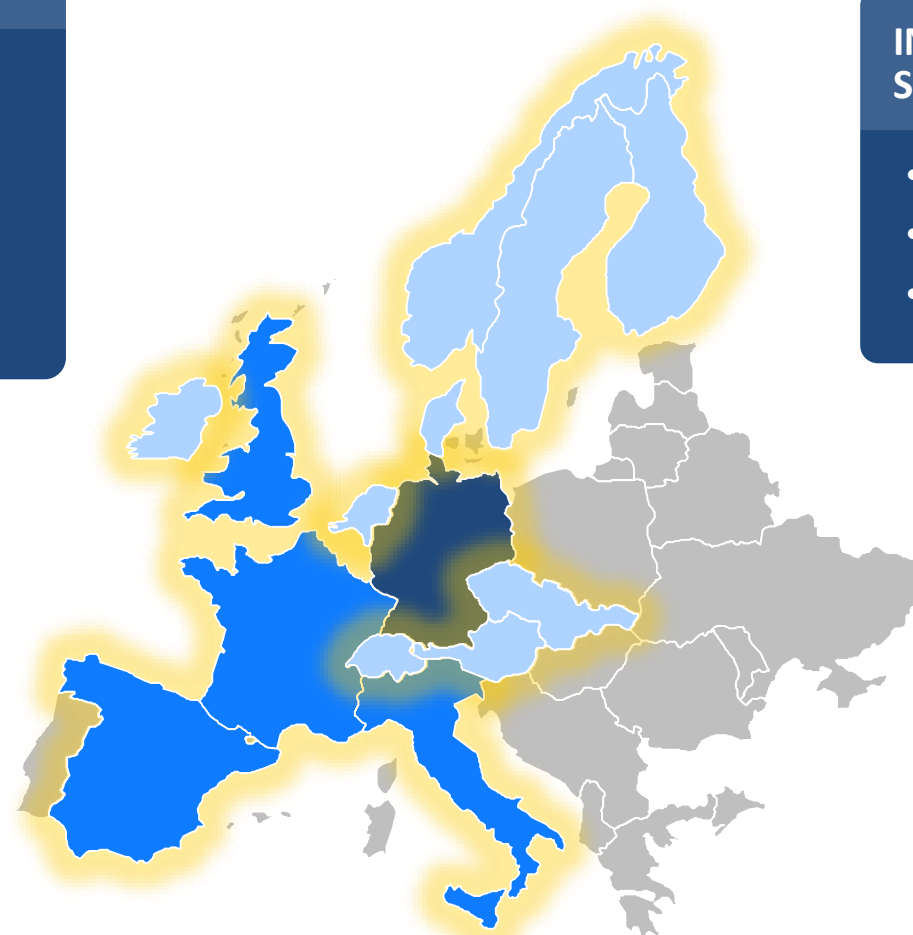
*Market share assumptions based on estimated number of ERT-treated patients with amenable mutations in Germany as of May 2016

EU Launch Strategy

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

INITIAL FOCUS ON TOP 5 COUNTRIES

- Germany
- France, Italy, Spain, UK
- ~2,000 Fabry patients treated
- ~70-75% of EU market value
- ~25% of global Fabry market

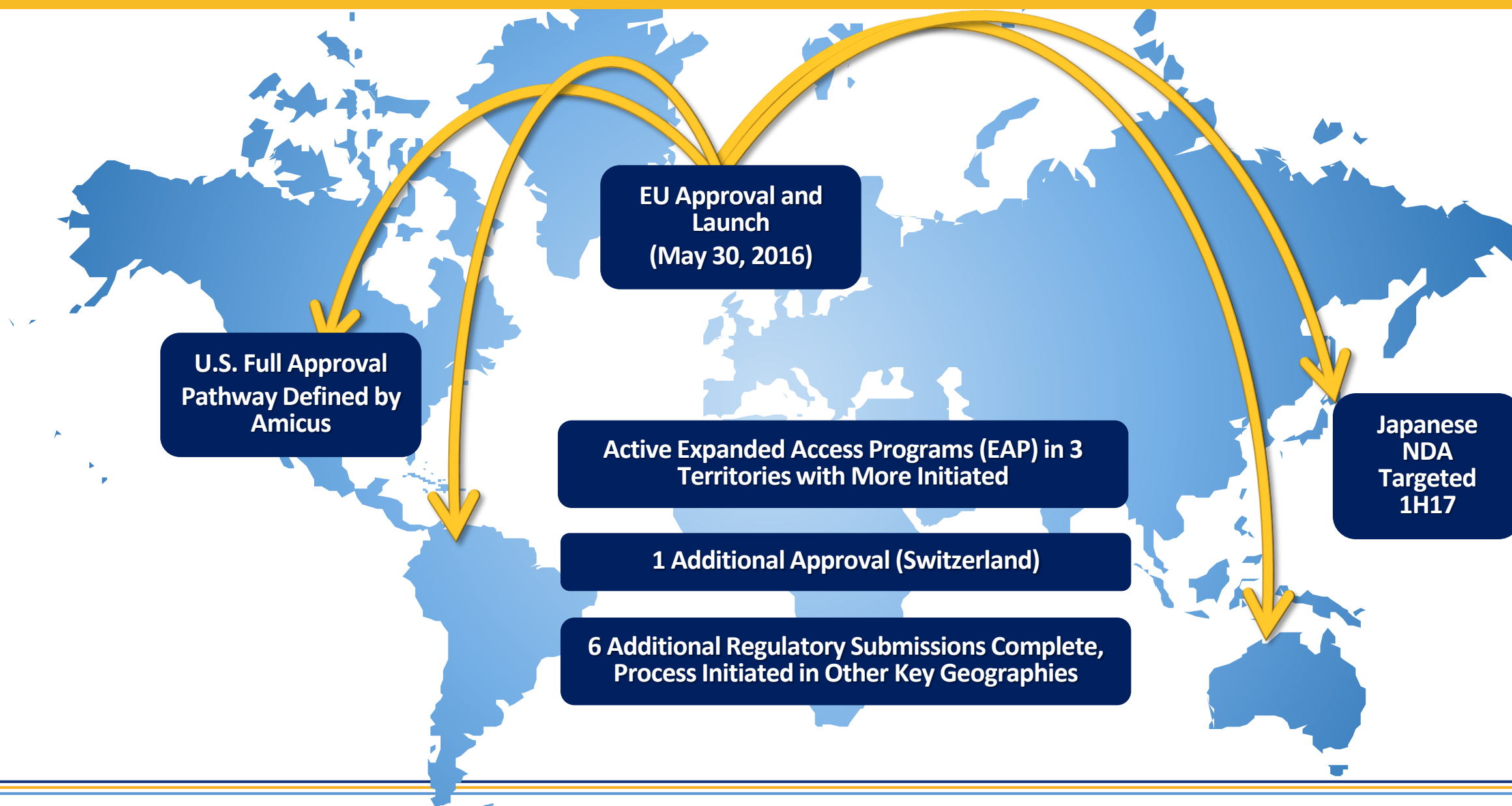


INVEST IN KEY MID-SIZED EU COUNTRIES AND SELECT EAP OPPORTUNITIES

- Austria, Nordics (4), 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



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

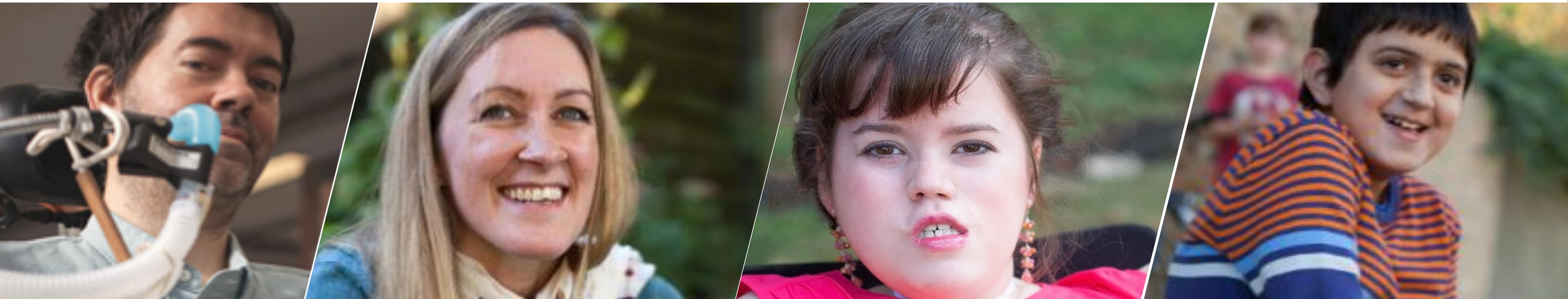
Respiratory and cardiac failure are leading causes of morbidity and mortality

Age of onset ranges from infancy to adulthood

5,000 – 10,000 patients diagnosed WW¹

Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

~\$800M+ Global Pompe ERT sales in FY15²



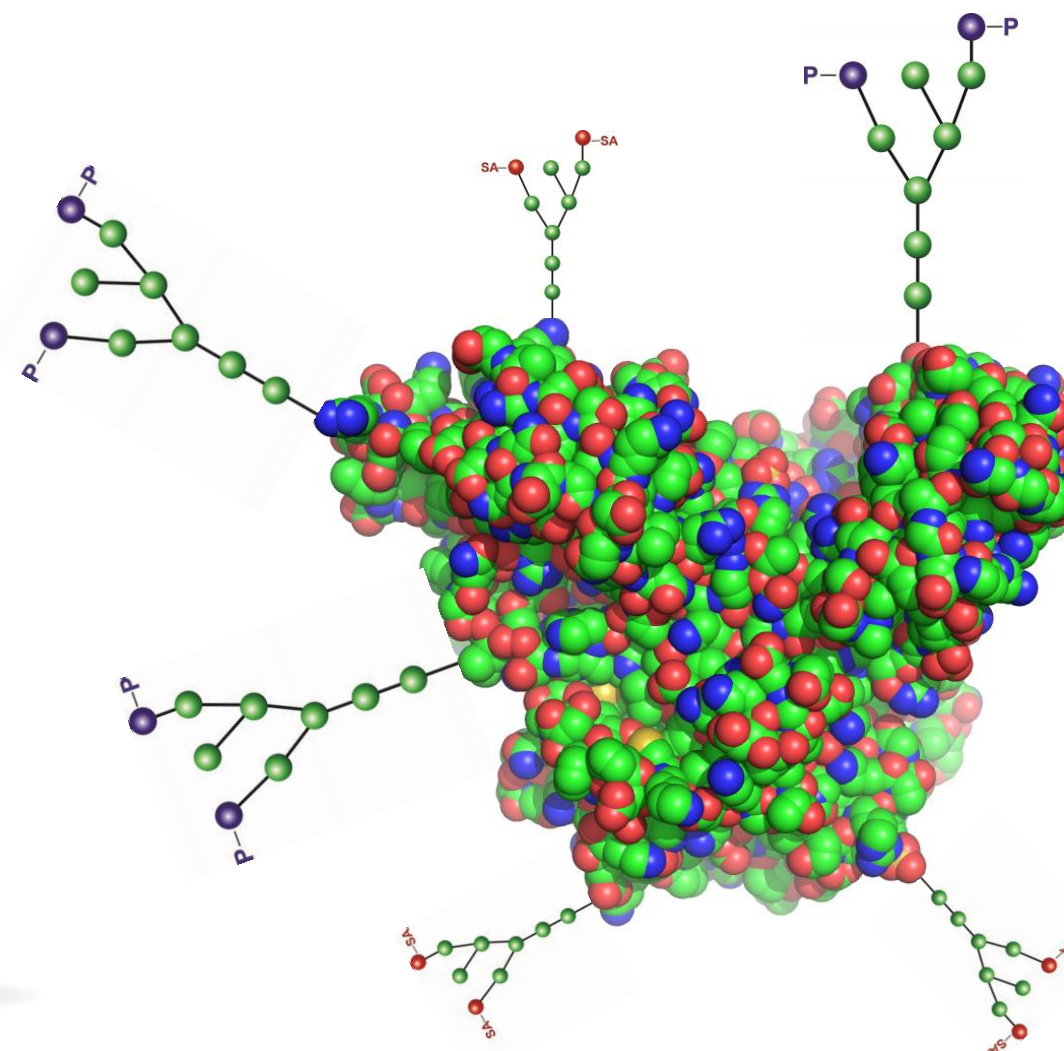
ATB200 + Chaperone: A Highly Differentiated Approach

Novel Pompe Treatment Paradigm with Three Key Differentiators

**ATB200
(Novel ERT)**



**Chaperone
addition**

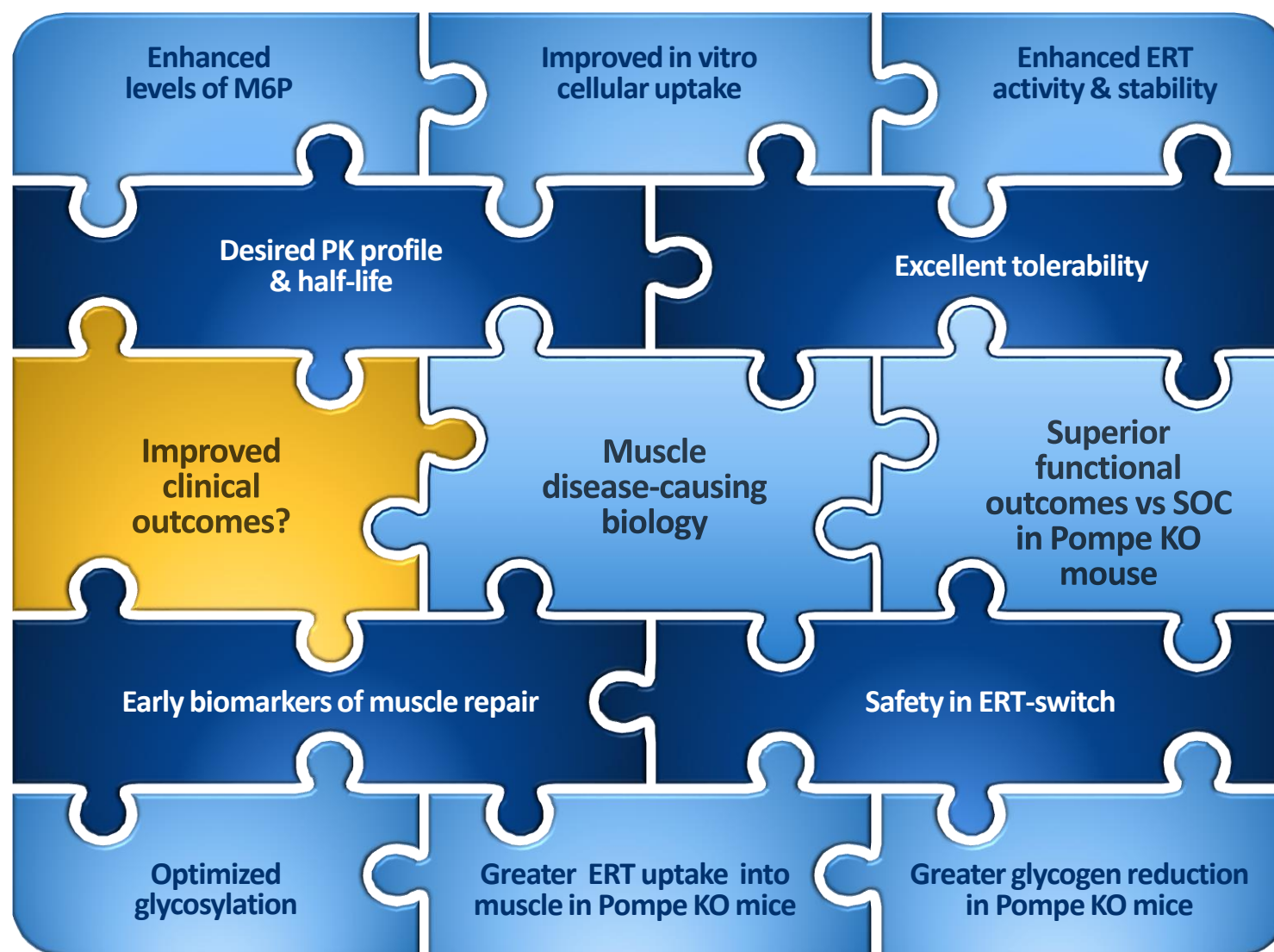


**Optimized
mixture of
glycans**

**High levels of
M6P and bis
M6P**

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."

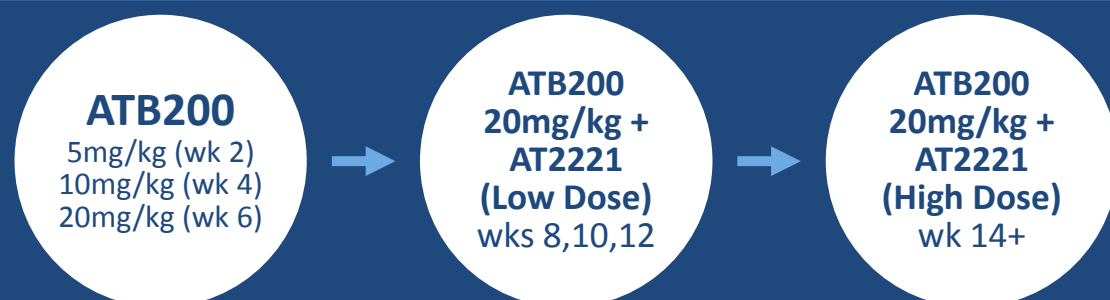
Grace K. Pavlath, Ph.D., Senior Vice President,
Scientific Program Director of Muscular Dystrophy Association

Phase 1/2 ATB200-02 Study Design

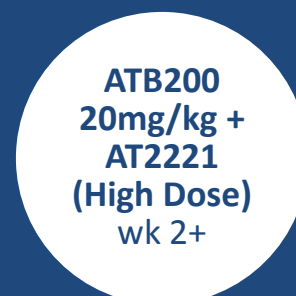
Phase 1/2 Clinical Study to Evaluate Safety, Tolerability, Pharmacokinetics (PK), and Pharmacodynamics (PD) of ATB200 + Chaperone (ATB200/AT2221)

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

Cohort 1 (Ambulatory ERT-Switch)



Cohort 2 (Non-Ambulatory ERT-Switch) & Cohort 3 (ERT-Naive)



Assessments:

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

Preliminary Clinical Data Summary

ATB200/AT2221 Demonstrates Promising Preliminary Results in Initial ERT-Switch and Naïve Patients at the Targeted Therapeutic Dose

- **Safety (N=13)***
 - No serious adverse events (SAEs)
 - AEs were generally mild and transient
- **Tolerability**
 - No infusion-associated reactions following 150+ infusions in all patients enrolled to date
- **PK (N=10)****
 - Clinical PK profile as predicted consistent with previously reported preclinical data
- **Biomarkers of muscle damage (CK, AST, ALT) and substrate (urine Hex4) (N=10)****
 - Decrease or normalization of muscle injury biomarkers in a majority of patients
 - Decreases in urine Hex4 in all patients
 - Improvement in all biomarkers suggests positive effect of ATB200/AT2221 on muscle cells

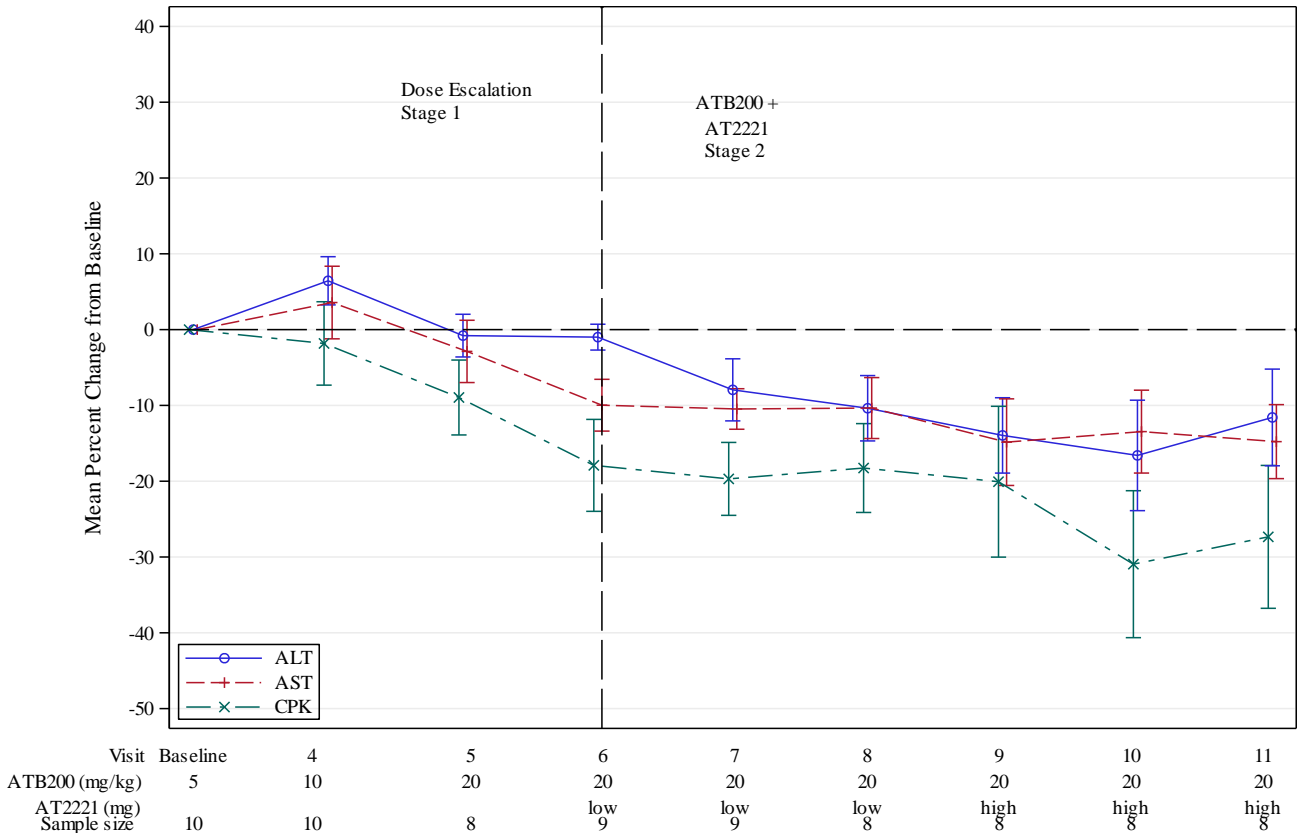
*N=10 from Cohort 1 (Ambulatory ERT-Switch); N=1 from Cohort 2 (Non-Ambulatory ERT-Switch); N=2 from Cohort 3 (Naïve)

**N=8 from Cohort 1 & N=2 from Cohort 3

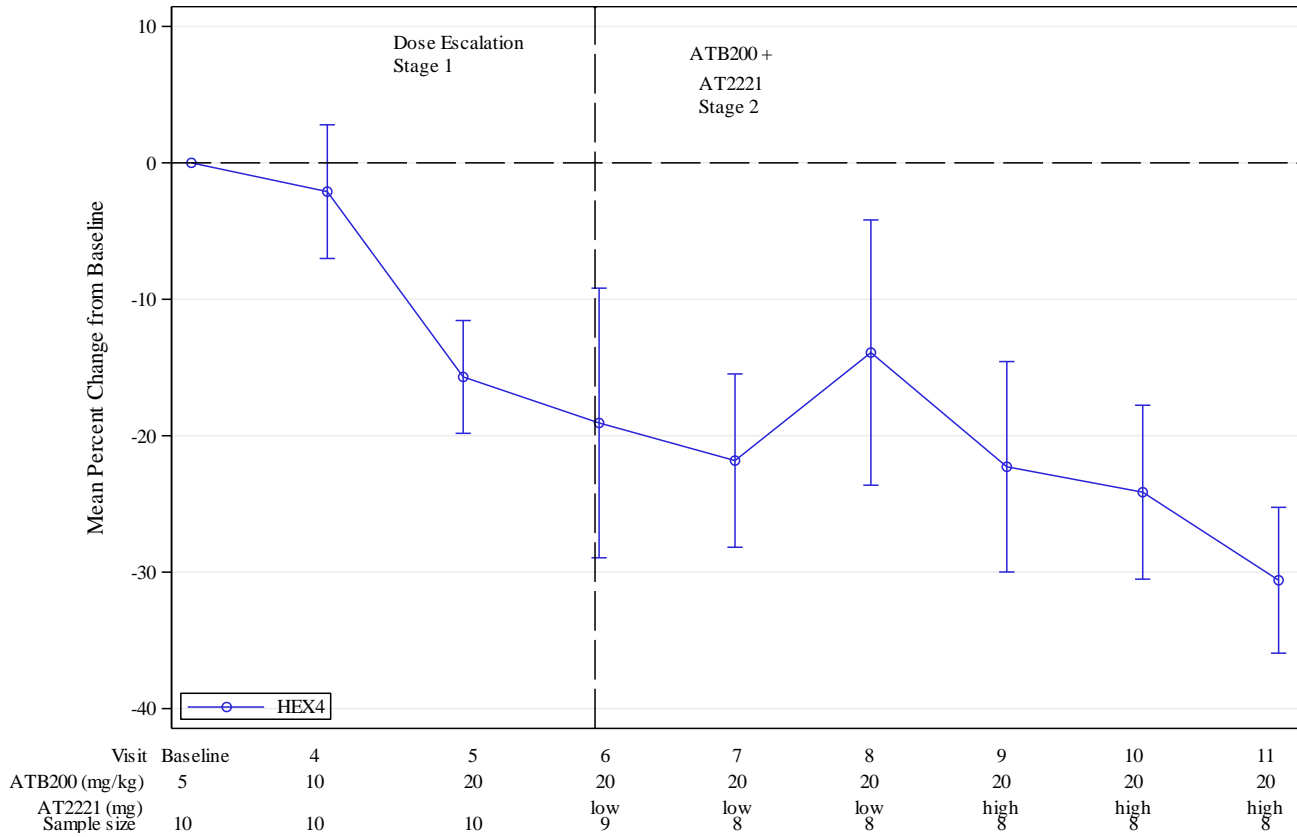
Cohort 1: Biomarkers at Week 18 (N=8)

After Switching from SOC to ATB200/A2221 Patients Generally Demonstrated an Improvement in Biomarkers of Muscle Damage (ALT, AST, CK) and Biomarker of Disease Substrate (Hex4)

Percent Change from Baseline for ALT, AST and CK



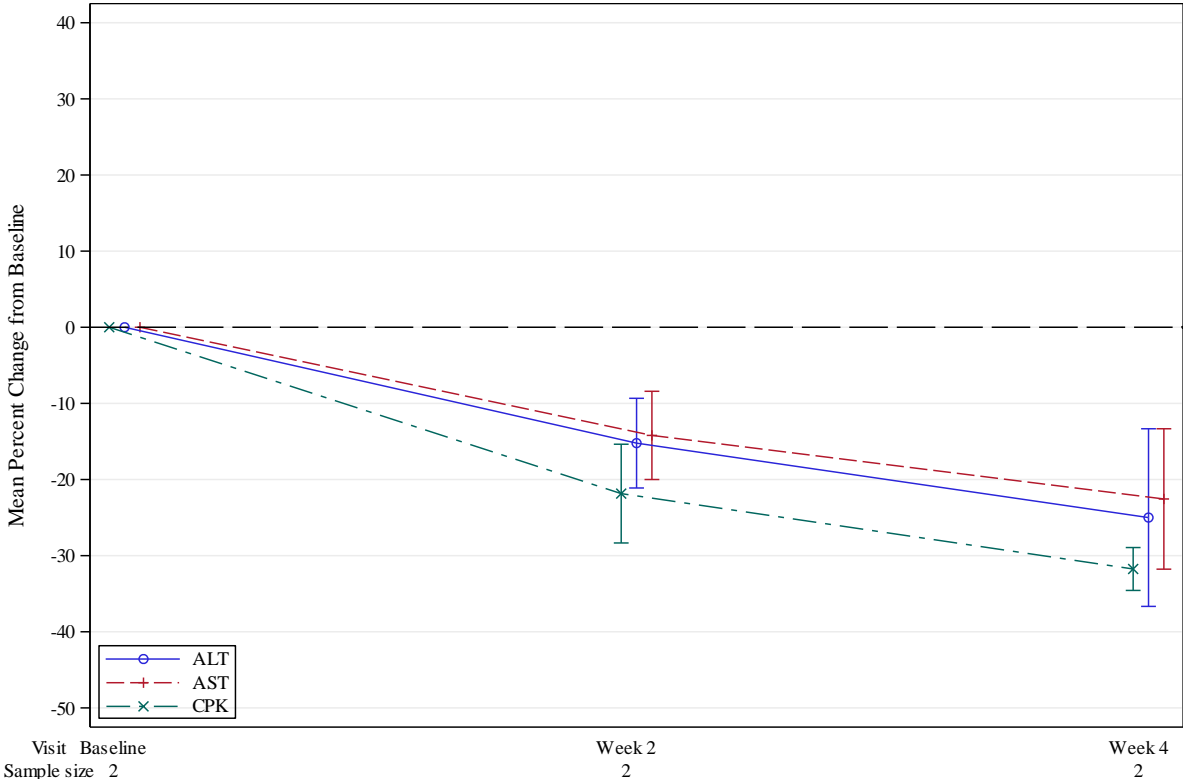
Percent Change from Baseline for Hex 4



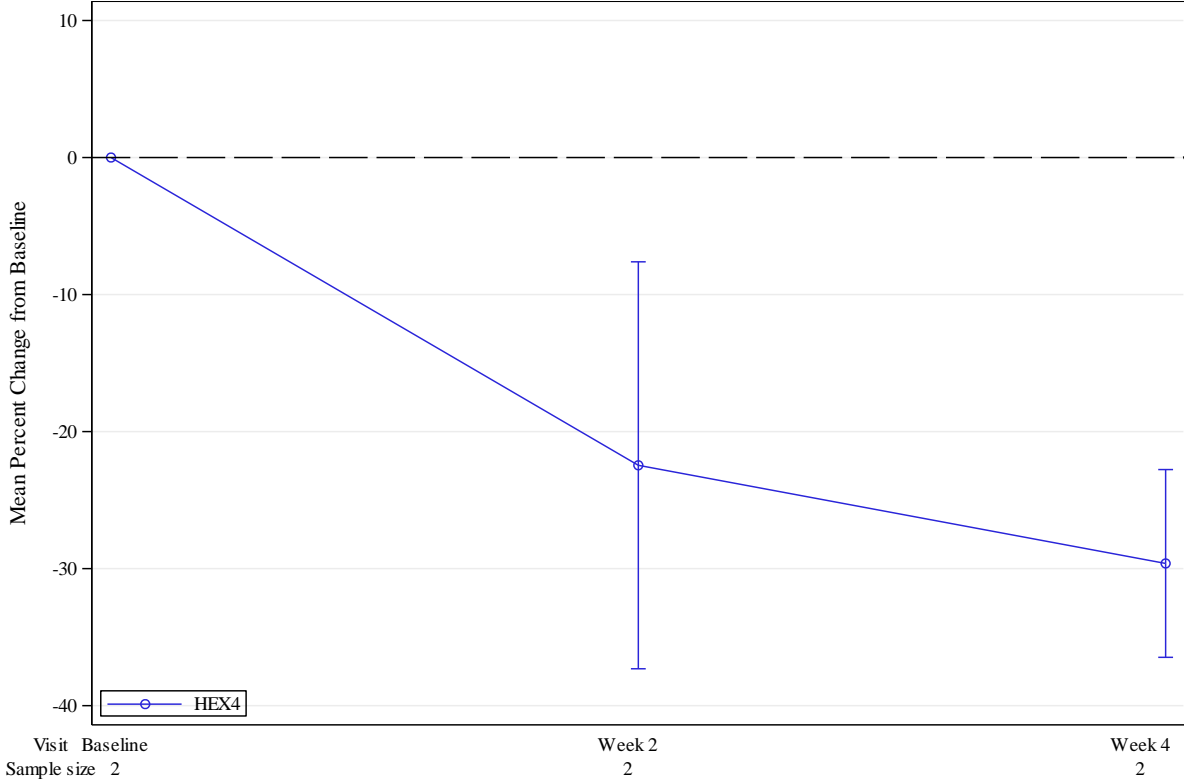
Cohort 3: Biomarkers at Week 4 (N=2)

Initial Two Naïve Patients Treated with ATB200/AT2221 Demonstrated Robust Reduction in Biomarkers of Muscle Damage (ALT, AST, CK) and Biomarker of Disease Substrate (Hex4)

Percent Change from Baseline for ALT, AST and CK



Percent Change from Baseline for Hex 4



Muscle Damage Biomarkers and Urine Hex 4 (N=10)*

- **In ERT-switch patients (Week 18):**
 - ALT decreased in 5 of 8 patients; 4/4 patients with elevated baseline levels normalized
 - AST decreased in 6 of 8 patients; 3/4 patients with elevated baseline levels normalized
 - CK decreased in 6 of 8 patients; 2/6 patients with elevated baseline levels normalized
 - ALT, AST, CK generally remained stable for patients not demonstrating a decrease
 - Urine Hexose Tetrasaccharide (Hex4) decreased in 8 of 8 patients; overall reduction approximately 30%
- **In ERT- naïve patients (Week 4):**
 - ALT, AST, CK and Urine Hex4 decreased in 2 of 2 patients.

*N=10 includes 8 switch and 2 naïve patients

Biologics Manufacturing Capabilities

Highly Successful Biologics Manufacturing Scale-up in Three Years

Proprietary Process



Research
Scale / MCB



5L
(Bench Scale)
2013



250L
(Clinical Scale)
2014-2015+



1000L
(Registration Trial &
Commercial Scale)
2016-2017+

Pompe Clinical Study ATB200-02 Data Cascade

A Cascade of Additional Data Points During 2Q17 and 3Q17 to Demonstrate Proof of Concept

Pompe Milestones in 2017

**Additional data
& initial
extension data
in Cohort 1**

**Data in non-
ambulatory
ERT-switch
patients
(Cohort 2)**

**Data in ERT-
naïve patients
(Cohort 3)**

**Additional
extension
study data
(all Cohorts)**

**Meeting
with U.S.
and EU
regulators**

18-WEEK DATA

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

EXTENSION DATA

- Motor/pulmonary function



SD-101 for Epidermolysis Bullosa

**Potential First-in-Class Treatment
with Phase 3 Data Anticipated Mid-2017**

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 ($>10 \text{ cm}^2$) showed widest separation versus placebo
- Daily administration generally safe and well-tolerated

**Informed Phase 3 Study
Design**

Phase 3 Study - Delivering on Our EB Vision

Phase 3 Study Optimized for Success with Top-Line Data On Track for Mid-2017



SD-005 Study Design Optimized

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

Status

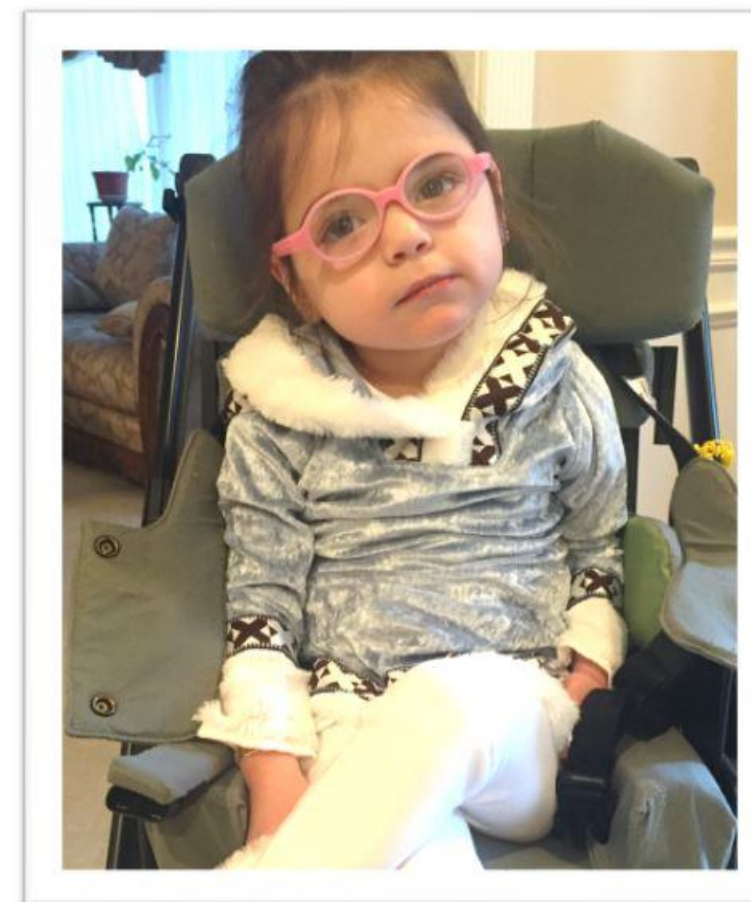
- 95%+ participation in extension study
- Enrollment near complete
- Top-line data anticipated mid-2017

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 worldwide¹
- Patient identification rising significantly





Financial Summary & Key Milestones

Financial Summary & Guidance

Balance Sheet Strengthened with \$330M Cash at 12/31/16 and Cash Runway Into 2H18

Financial Position	December 31, 2016
Cash	\$330M
Debt	\$250M
FY17 Net Operating Cash Flow Guidance	\$175-\$200M
FY17 Net Cash Spend Guidance*	\$200-\$225M
Cash Runway	2H18
Capitalization	December 31, 2016
Shares Outstanding	142,691,986

*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 1H17
- Fabry GI study

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 mid-2017

Strong Balance Sheet

- Significant revenue contribution
- Cash runway into 2H18

*Commercial and Expanded Access Programs (EAPs)

Thank You

