Amicus Therapeutics®

35th Annual J.P. Morgan Healthcare Conference

John F. Crowley, Chairman and Chief Executive Officer

January 10, 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 preliminary 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. The preliminary data and Phase 1/2 study 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 Annual Report on Form 10-K for the year ended December 31, 2015 and Quarterly Report on Form 10-Q for the quarter ended September 30, 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

$331M 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	• $331M 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

2010	2014	Today	2018	2023
~37 Patients	~90 Patients	~250 Patients*	~800 Patients*	~5,000 Patients*

*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

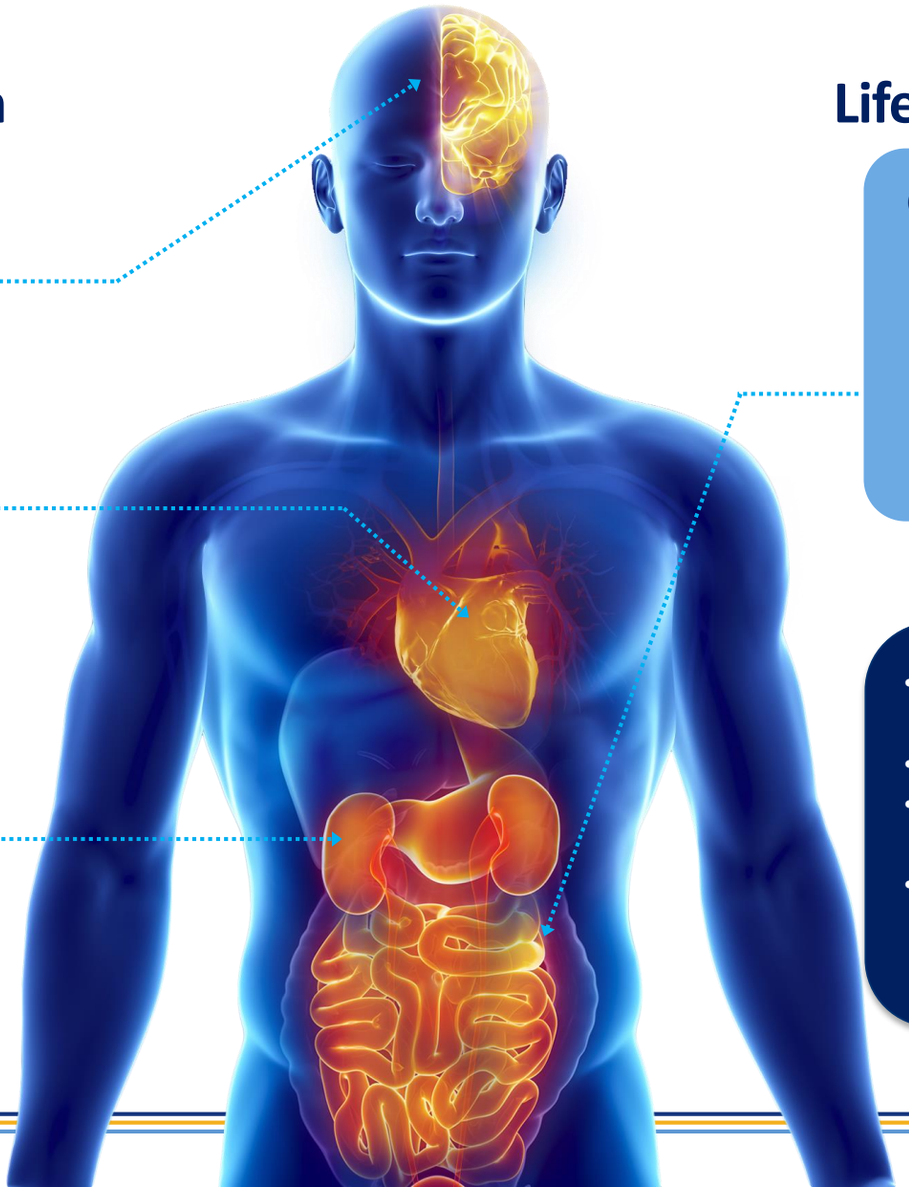
TRANSIENT ISCHEMIC ATTACK (TIA) & STROKE[1]

HEART DISEASE[2]

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

KIDNEY DISEASE[3]

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



Life-Limiting Symptoms

GASTROINTESTINAL[3]

- 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% male[4])
- Newborn screening studies suggest prevalence of ~1:1000 to ~1:4000

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

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

Today

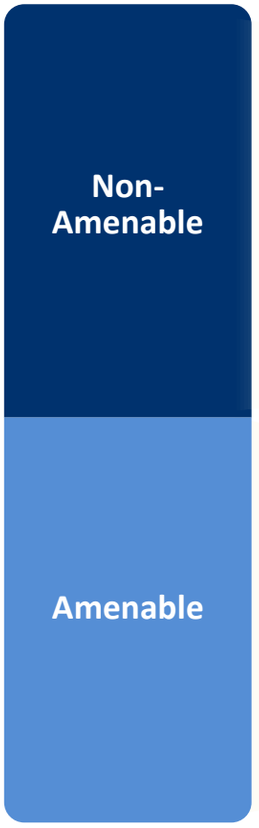
Migalastat

Oral precision medicine

~$1.2B

Global Fabry market today

Growing to ~$2B Global Fabry market



Future Vision

Novel ERT co-formulated with migalastat

*Artist rendering, not actual product image

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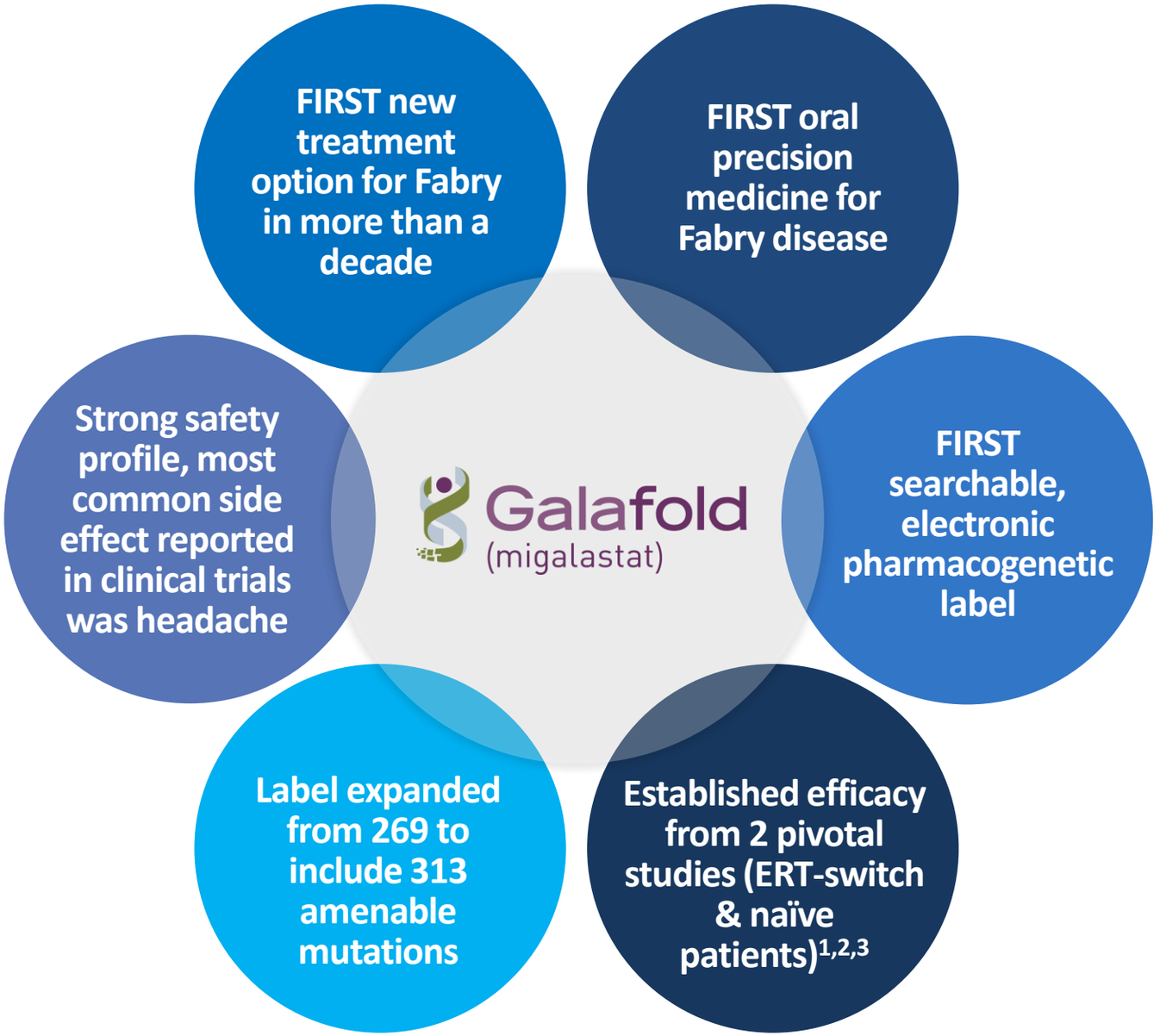
Full EU Approval as First Oral Precision Medicine for Fabry Disease



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 Mutation[3]

- **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.

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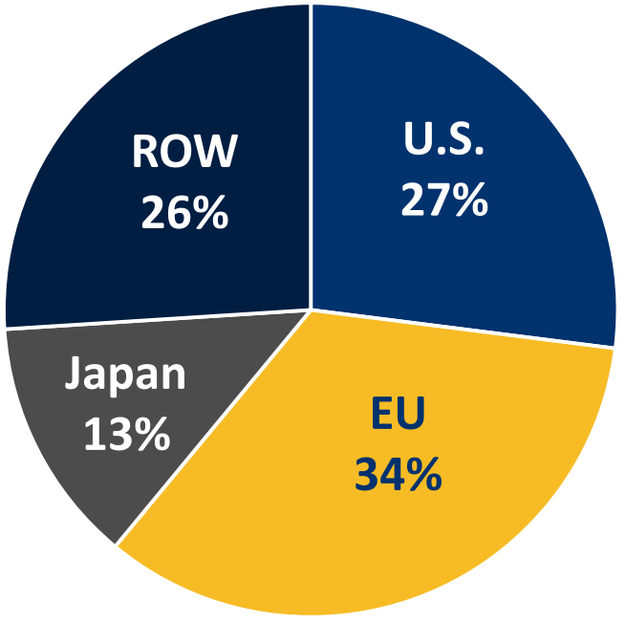
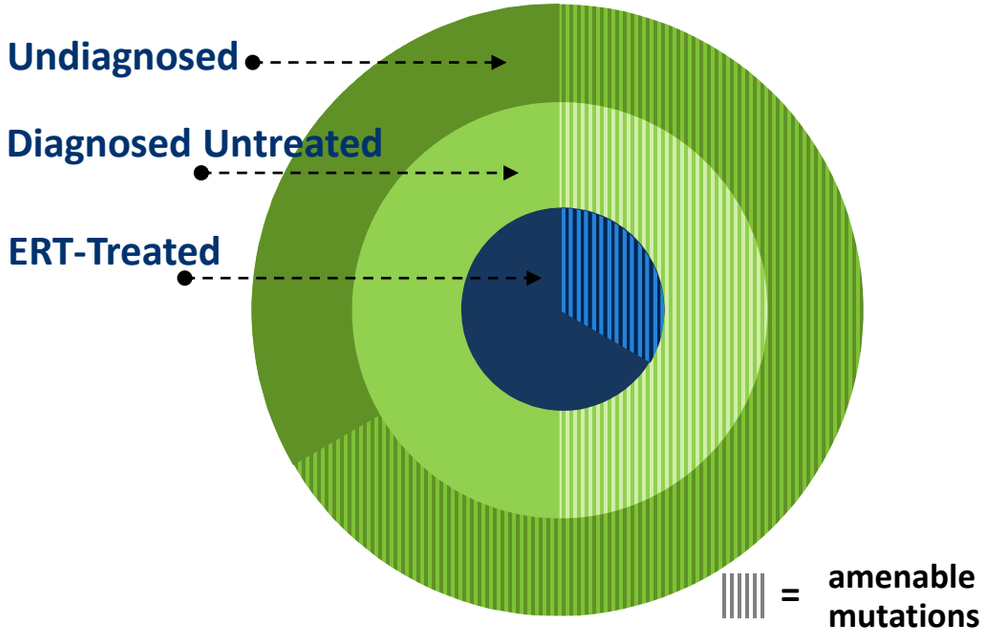
Galafold Commercial Opportunity

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[2]

1. Company filings and Amicus estimates 2. 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

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Early Success with International Launch (as of 12/31/16)

Initial Launch Success Driven by Germany with ERT-Switch & Naïve Patients, Reimbursement Now Available in 6 Countries*

61 Patients (Switch & Naïve) on reimbursed Galafold (12/31/16)

6 Countries with available reimbursement*

18 Countries with pricing discussions ongoing

22 Countries with Amicus footprint

Target Number of Patients on Reimbursed Galafold by YE17

*Commercial and Expanded Access Programs (EAPs)

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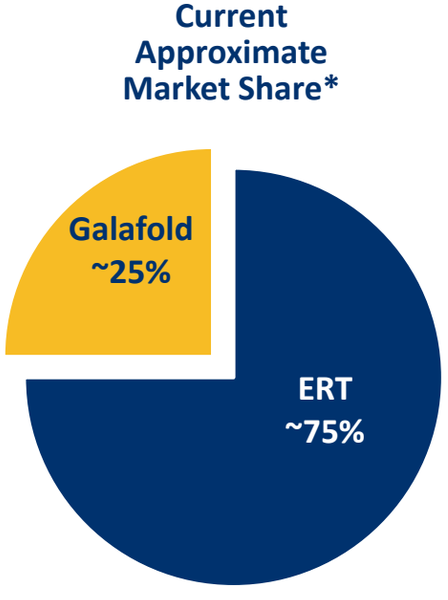
German Launch Update (as of 12/31/16)

Germany is an Important Indicator for EU Launch Success



IMPORTANT EARLY INDICATORS IN GERMANY

- Vast majority switch patients
- ~25% of eligible switch patients now on Galafold*
- All newly experienced patients & physicians
- Majority of switches from Replagal™
- Male / female mix
- 13 unique prescribers

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

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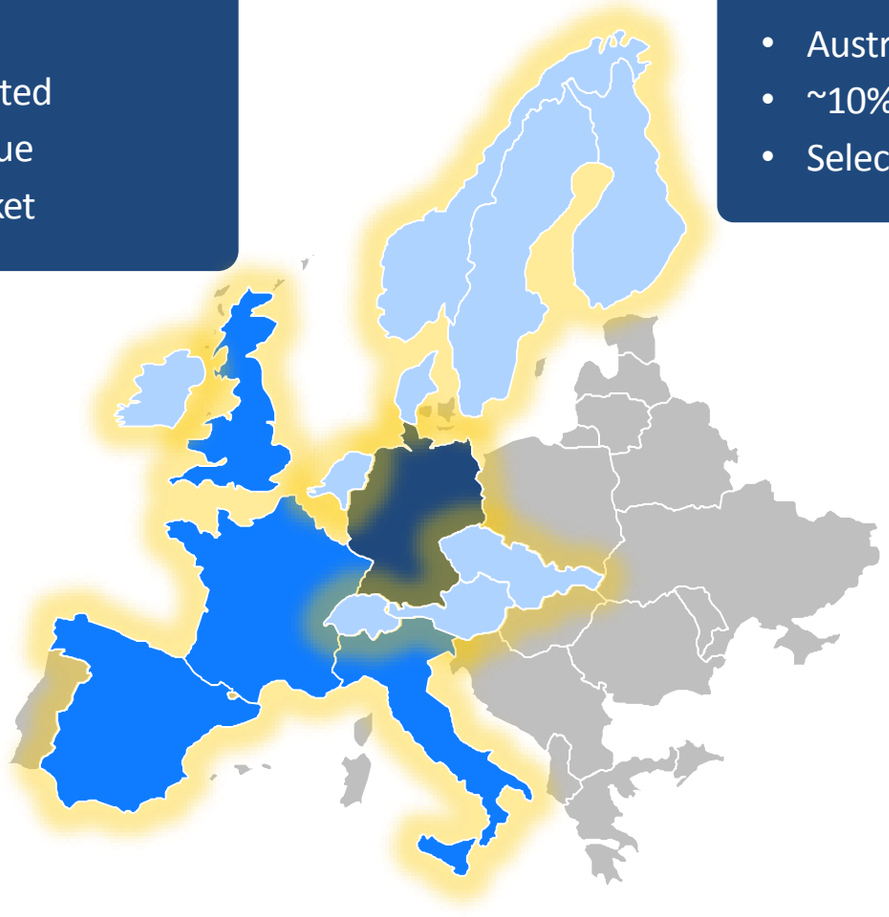
EU Launch Strategy

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

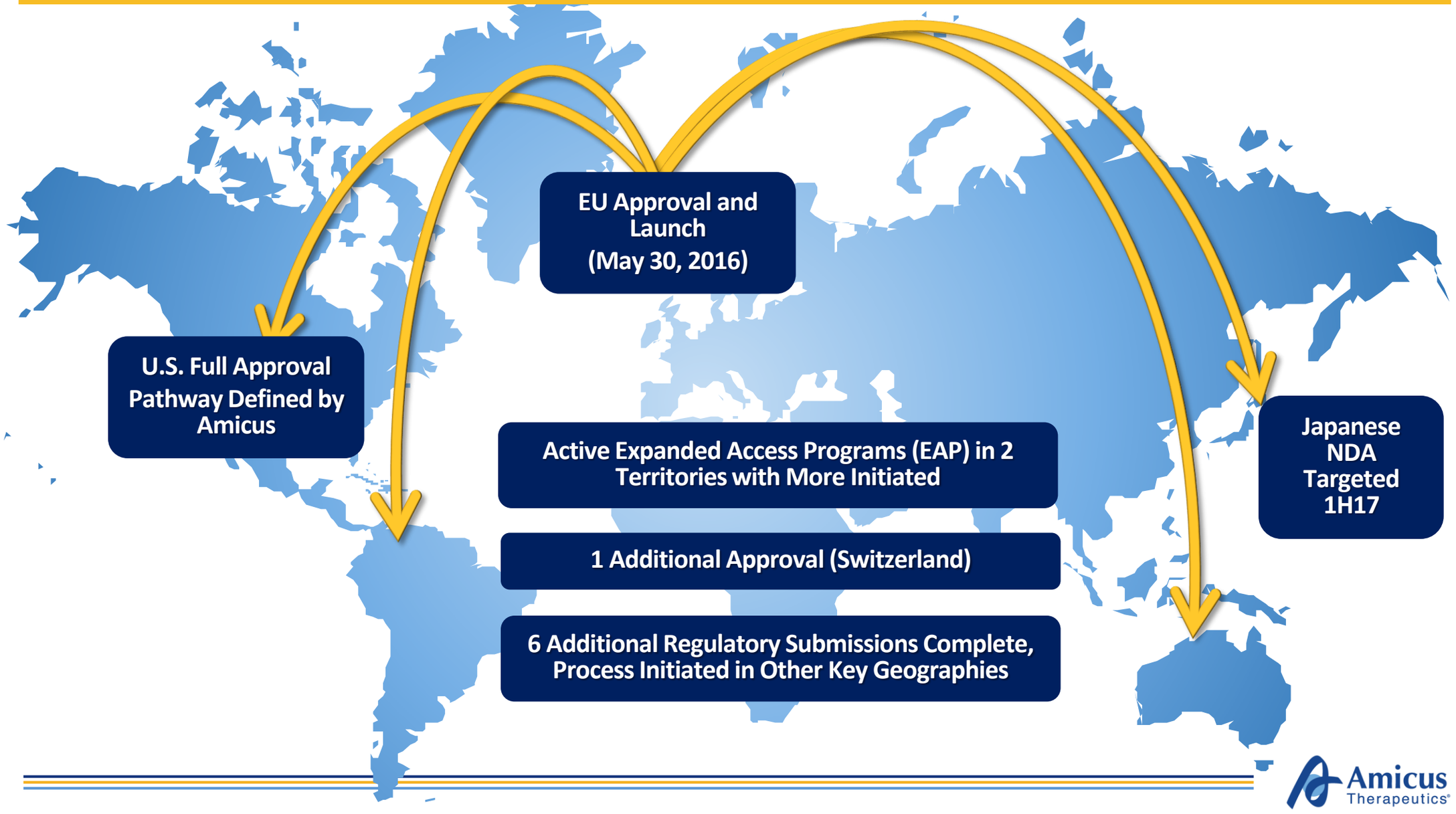


INVEST IN KEY MID-SIZED EU COUNTRIES AND SELECT EAP OPPORTUNITIES

- 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

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ATB200 Novel ERT for Pompe Disease

Establishing Human Proof of Concept and Validating Biologics Platform in 2017

Pompe Disease Overview

Devastating Disease Symptoms Persist Across a Broad Spectrum of Patients Despite Available Therapy

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[1]

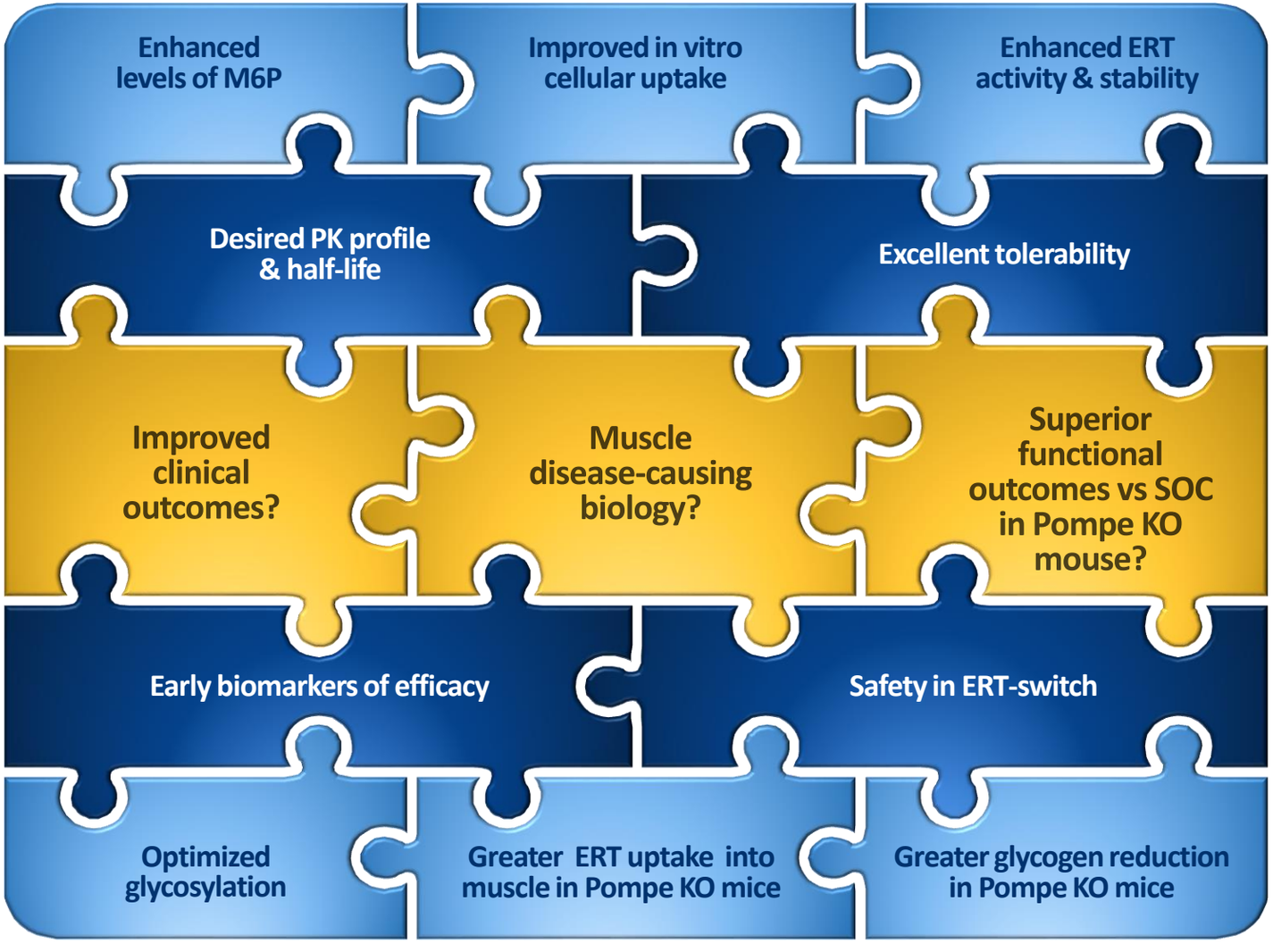
Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

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

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

Pompe Disease: A Complex Disease with Significant Unmet Needs

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

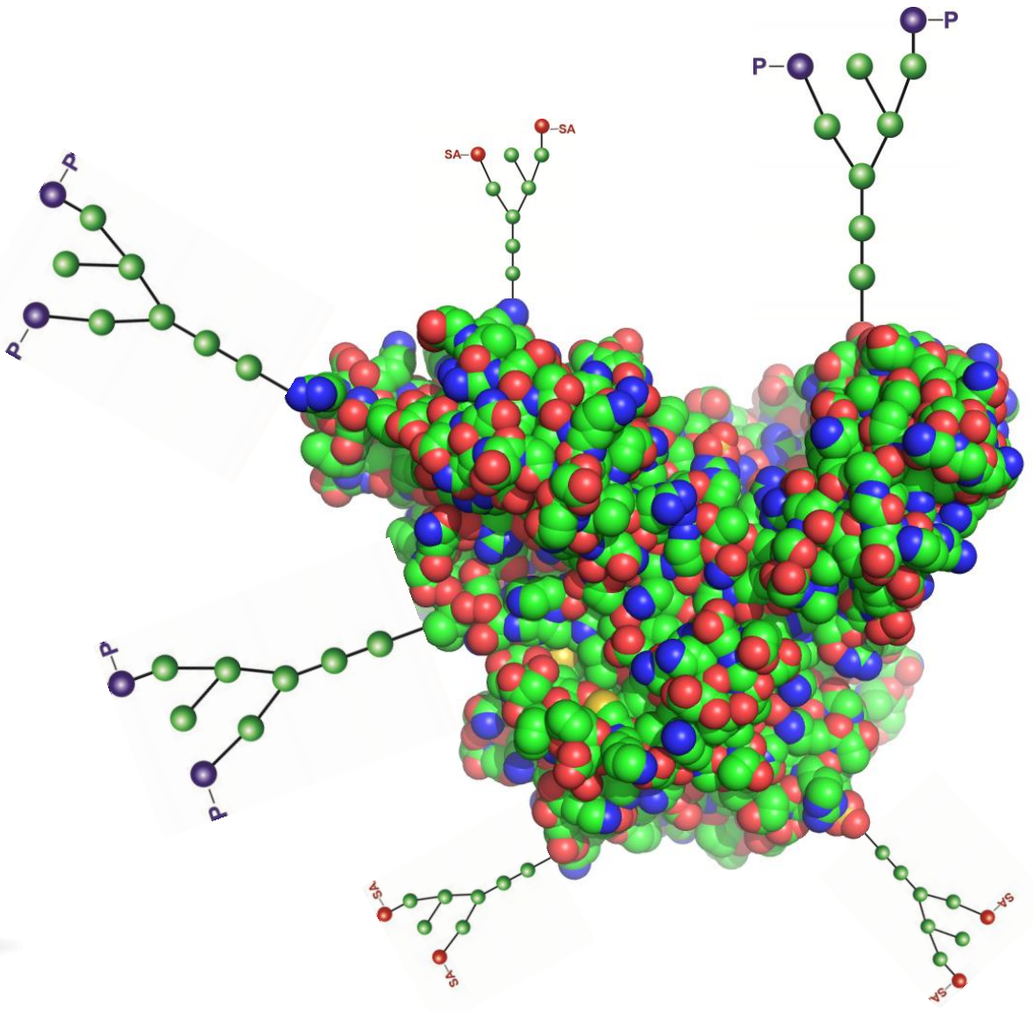


ATB200 + Chaperone: A Highly Differentiated Approach

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

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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+

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)

ATB200
5mg/kg (wk 2)
10mg/kg (wk 4)
20mg/kg (wk 6)

→ **ATB200 20mg/kg + AT2221 (Low Dose)** wks 8,10,12

→ **ATB200 20mg/kg + AT2221 (High Dose)** wk 14+

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

ATB200 20mg/kg + AT2221 (High Dose) wk 2+

Assessments:

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

Preliminary Data Summary

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

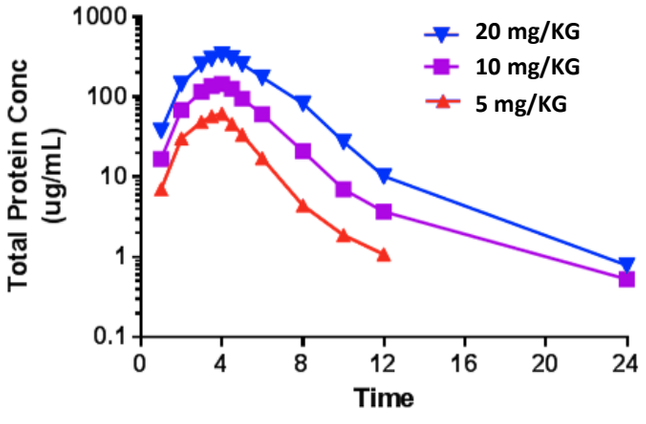
Safety (n=9)*	• No serious adverse events (SAEs) • AEs generally mild and transient
Tolerability	• No infusion-associated reactions following 100+ infusions
PK (n=4)**	• Clinical PK profile as predicted consistent with preclinical data • ATB200 plasma clearance rate suggests efficient tissue uptake • ATB200 alone showed greater than dose-proportional increases in exposure, further enhanced with AT2221
Muscle damage biomarkers (CK, AST, ALT) (n=4)	• Early trend to improvement in 2 patients • Stable in 2 patients
Immunogenicity (n=4)	• Anti-rhGAA antibodies remained generally stable • Cytokines remained low and stable during infusions

*N = 8 from Cohort 1 (Ambulatory ERT-Switch) and 1 from Cohort 1 (Non-Ambulatory ERT-Switch); through interim data analysis (maximum 24 weeks)
**N = 4 from Cohort 1

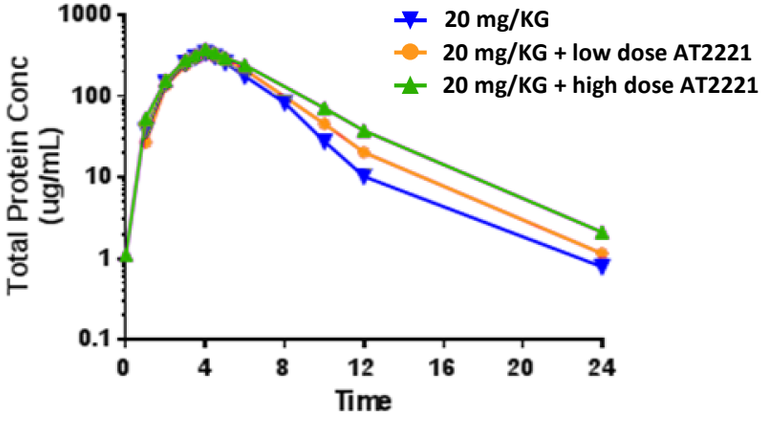
Pharmacokinetics at Week 14: Plasma Exposure (n=4)*

ATB200 Clinical PK Profile as Predicted Based on Preclinical Studies with Greater than Dose Proportional Increases in Exposure that were Enhanced by AT2221

Mean GAA Total Protein (n=4)
5, 10, 20 mg/kg ATB200 Alone



Mean GAA Total Protein (n=4)
20 mg/kg ATB200 + AT2221



Treatment	Mean $AUC_{0-\infty}$ (hr*µg/ml)	Mean Clearance (L/hr)
5 mg/kg	215	1.97
10 mg/kg	589	1.45
20 mg/kg	1547	1.11

Treatment	Mean $AUC_{0-\infty}$ (hr*µg/ml)	Mean Clearance (L/hr)
20 mg/kg	1547	1.11
+low dose AT2221	1676	1.03
+high dose AT2221	1945	0.90

*N = 4 from Cohort 1 (Ambulatory ERT-Switch)

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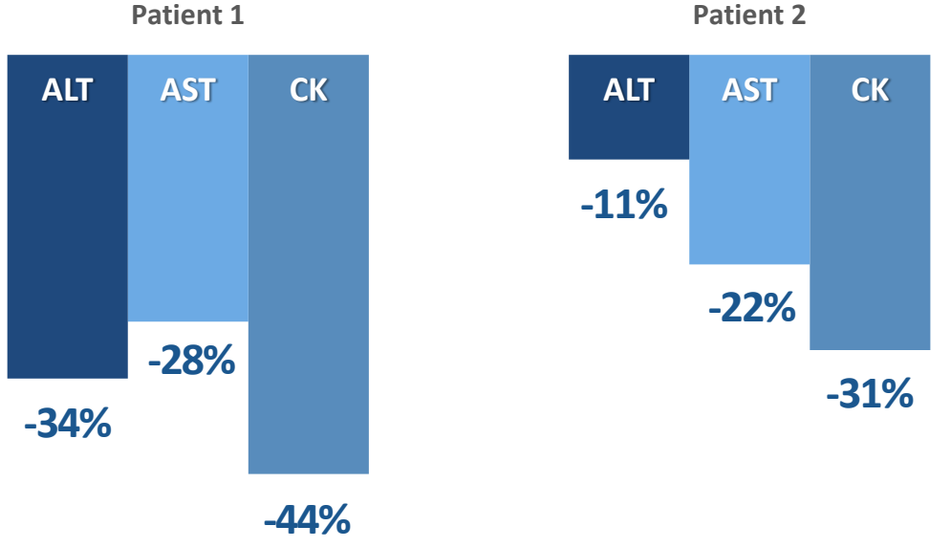
Muscle Damage Biomarkers at Week 14 (n=4)*

After Switching from Lumizyme™ to ATB200/AT2221, Muscle Damage Biomarkers (CK, AST, ALT) Trended Toward Early Improvement in Two Patients and Were Stable in the Other Two Patients

Two patients showed early trend toward improvement in all three biomarkers:



*Elevated **creatine kinase (CK), alanine aminotransferase (ALT)**, and **aspartate aminotransferase (AST)** are indicators of damage to muscle tissue*

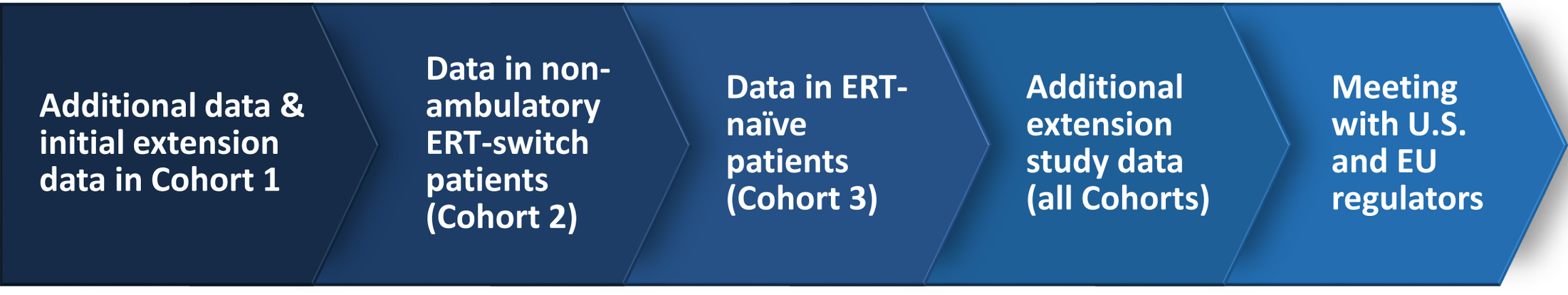
Two patients remained stable

*N = 4 from Cohort 1 (Ambulatory ERT-Switch)

Pompe Clinical Study ATB200-02 Data Cascade

A Cascade of Additional Data Points During 2017 to Demonstrate Proof of Concept

Pompe Milestones in 2017

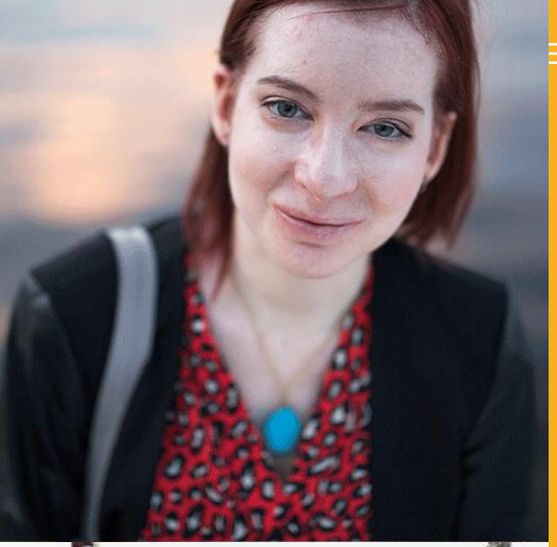


18-WEEK DATA

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

EXTENSION DATA

- Motor/pulmonary function



SD-101 for Epidermolysis Bullosa

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

EB Disease Overview

Rare, Devastating, Connective Tissue Disorder with No Approved Treatments

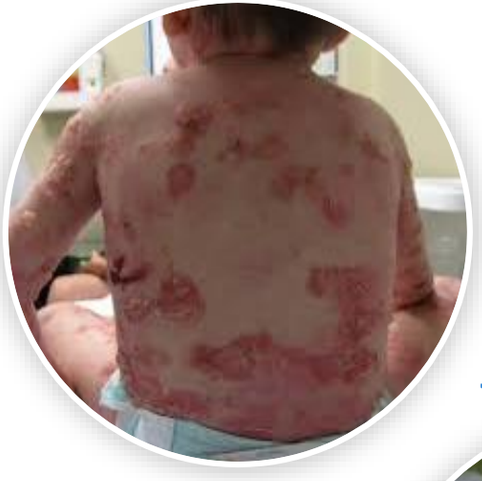
Disease Overview

- 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

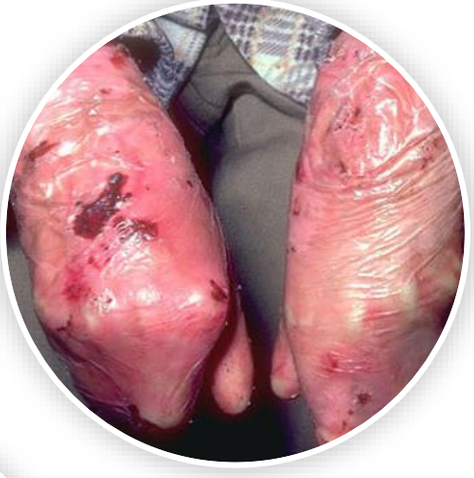
Three Major EB Types

(~99% of EB Population)

SIMPLEX (75%)



DYSTROPHIC (20%)

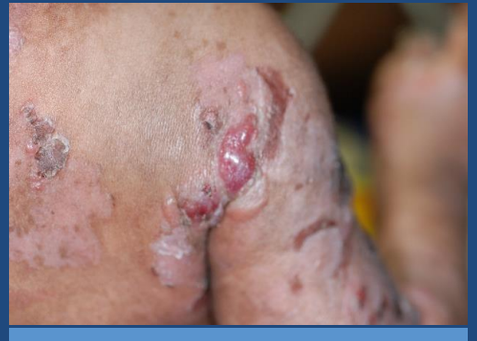


JUNCTIONAL (5%)

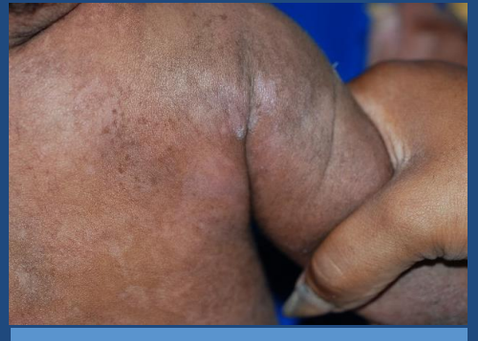
Proof of Concept Findings

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

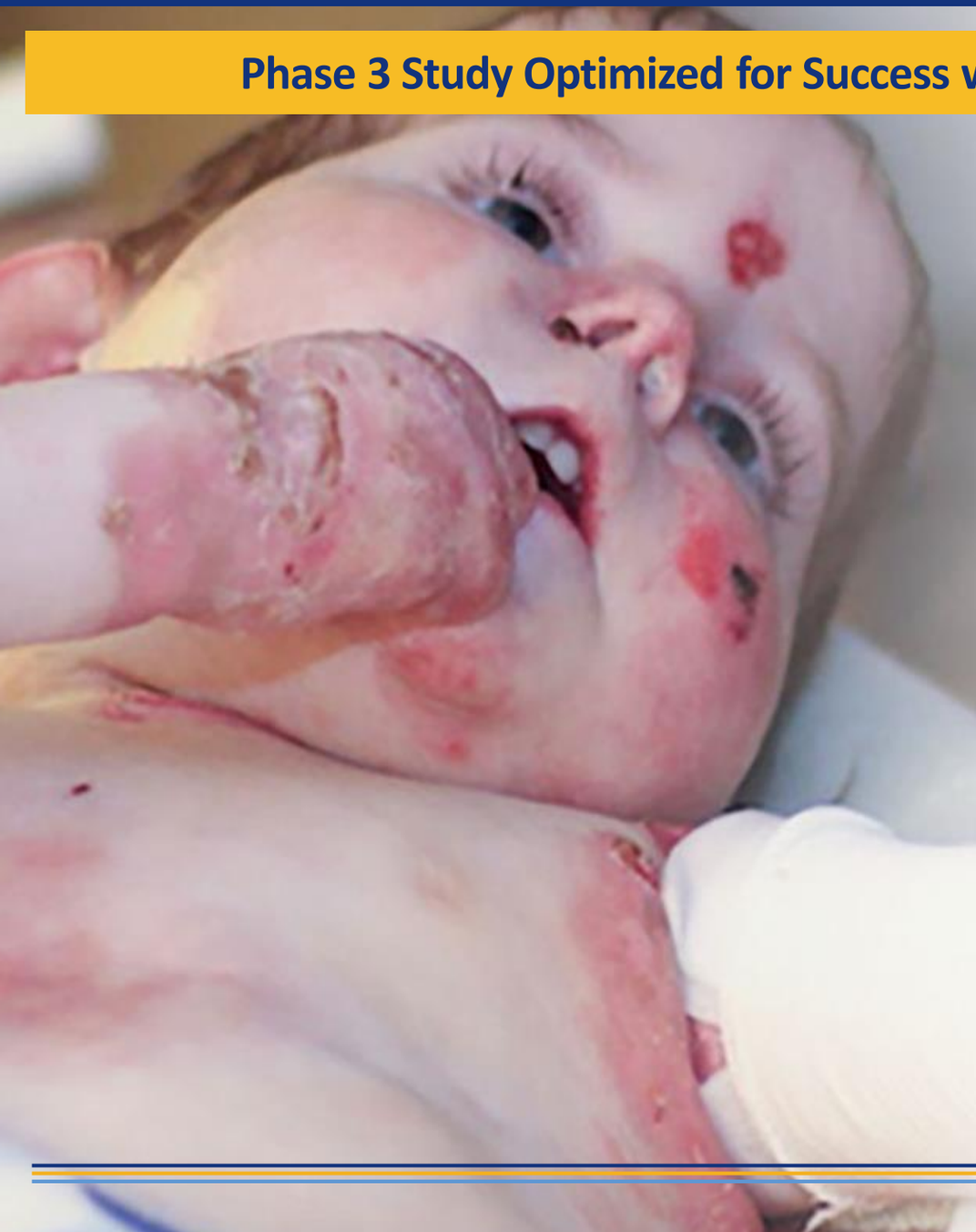
Phase 2b Key Takeaways (SD-101 6%)

- Faster time to wound closure
- Higher proportion with complete closure
- Reduction in total body surface area (BSA) of wounds
- Larger wounds (>10 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 Anticipated 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

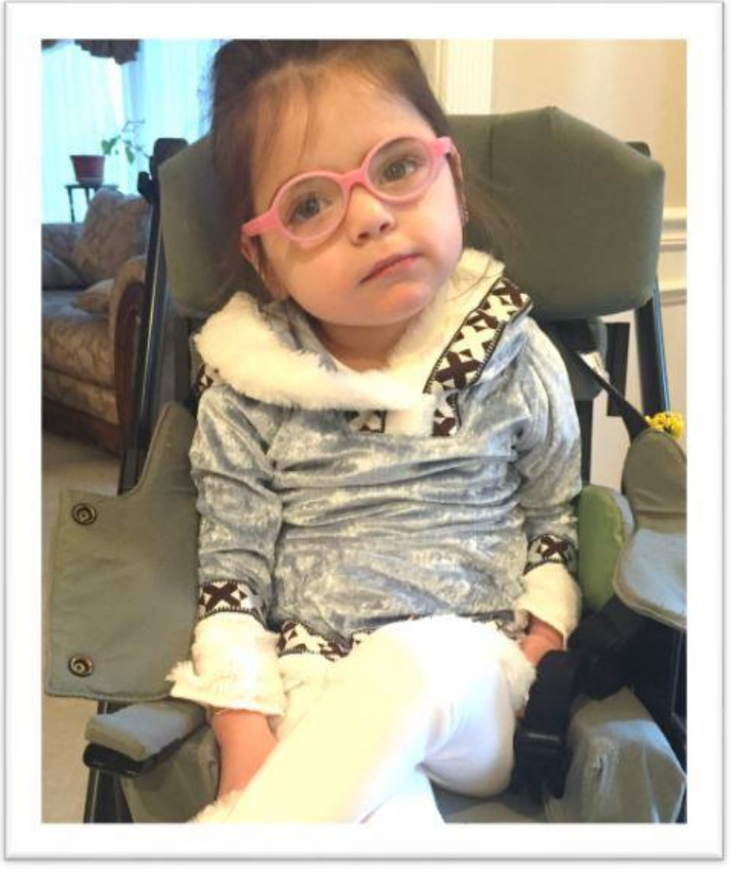
- 95%+ participation in extension study
- Enrollment near complete
- Top-line data anticipated mid-2017

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 worldwide[1]
- Patient identification rising significantly



1. LouLouFoundation.org

Financial Summary & Key Milestones

Financial Summary & Guidance

Balance Sheet Strengthened with $331M Cash at 12/31/16 and Cash Runway Into 2H18

Financial Position	December 31, 2016
Cash	$331M
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 Milestones in 2017

2017	
Fabry Disease (Galafold)	• Galafold international launch targeting 300 patients by YE17 • Japan NDA submission • Fabry GI study initiation
Pompe Disease (ATB200/AT2221)	• Phase 1/2 data cascade • Meetings with U.S. and EU regulators
Epidermolysis Bullosa (EB) (SD-101)	• Phase 3 data
Strong Balance Sheet	• Significant revenue contribution in 2017 • Runway into 2H18

Thank You

Amicus Therapeutics®