Amicus Therapeutics®

Corporate Overview

May 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

2010

~90 Patients

2014

~250 Patients*

Today

~800 Patients*

2018

~5,000 Patients*

2023

*Clinical & Commercial

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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

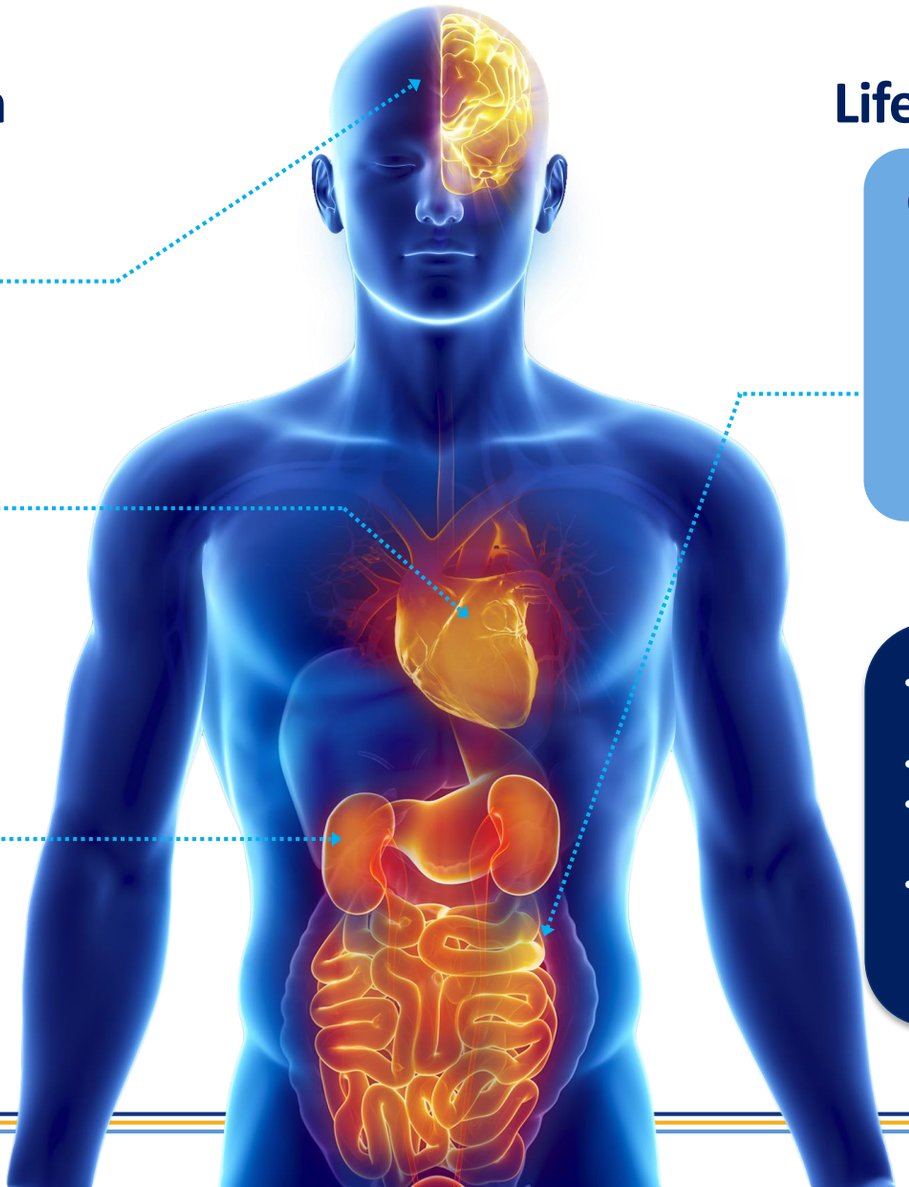
TRANSIENT ISCHEMIC ATTACK (TIA) & STROKE[1]

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

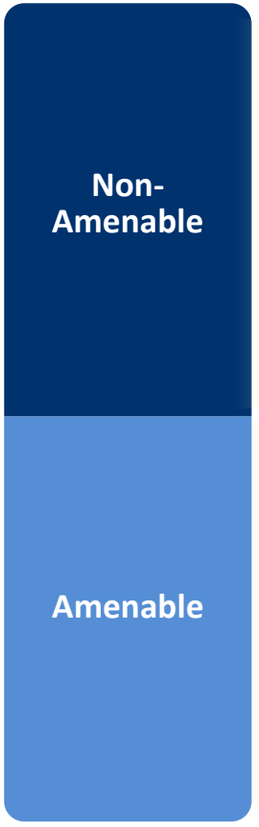
Migalastat

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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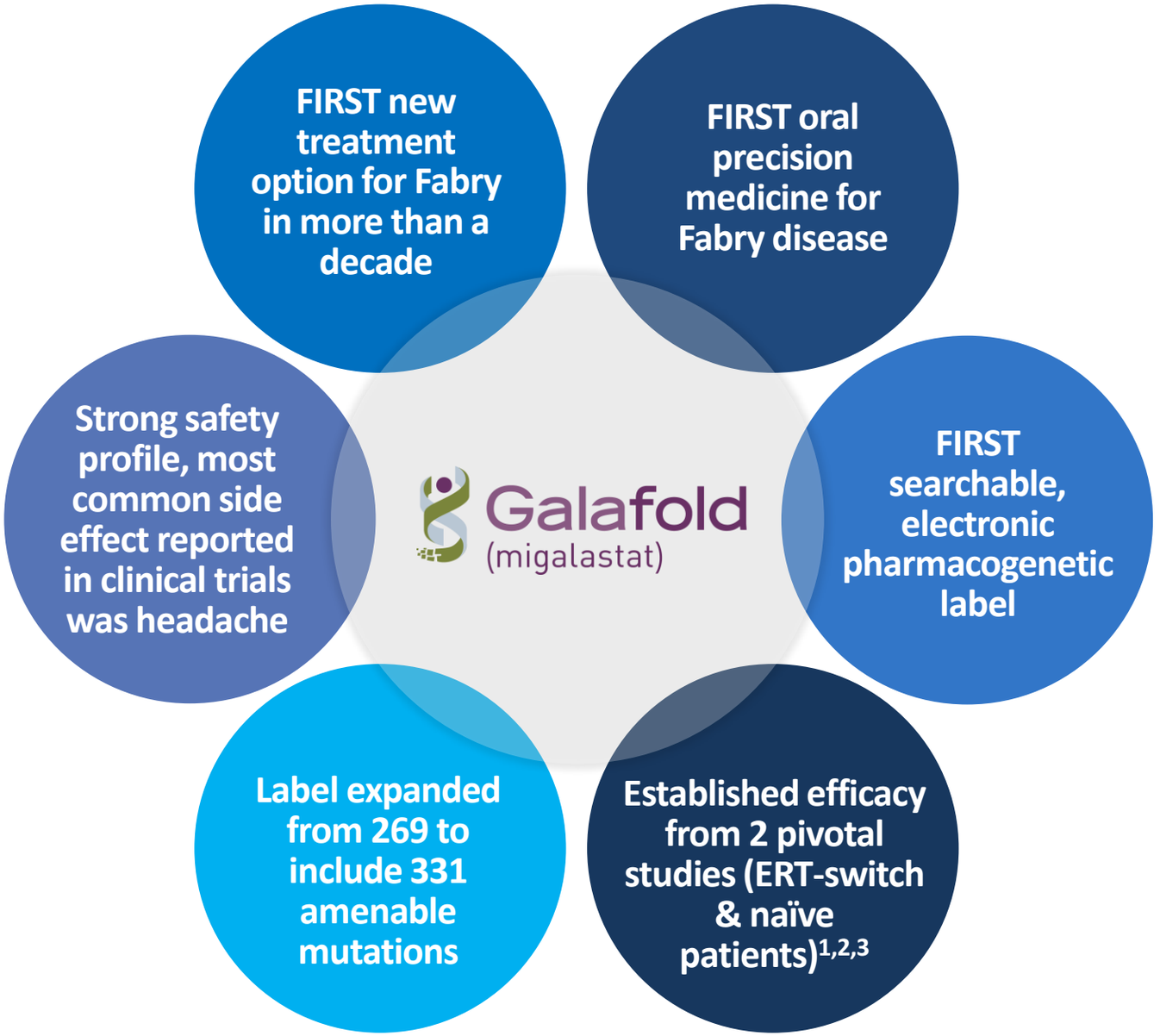
Full EU Approval as First Oral Precision Medicine for Fabry Disease



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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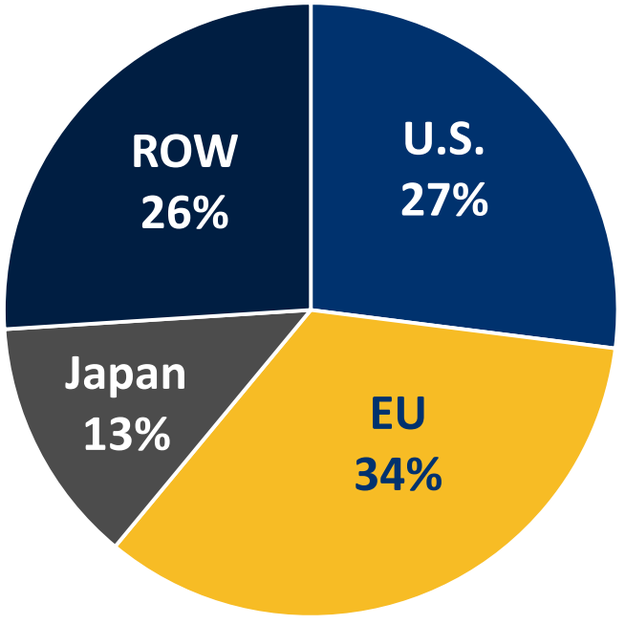
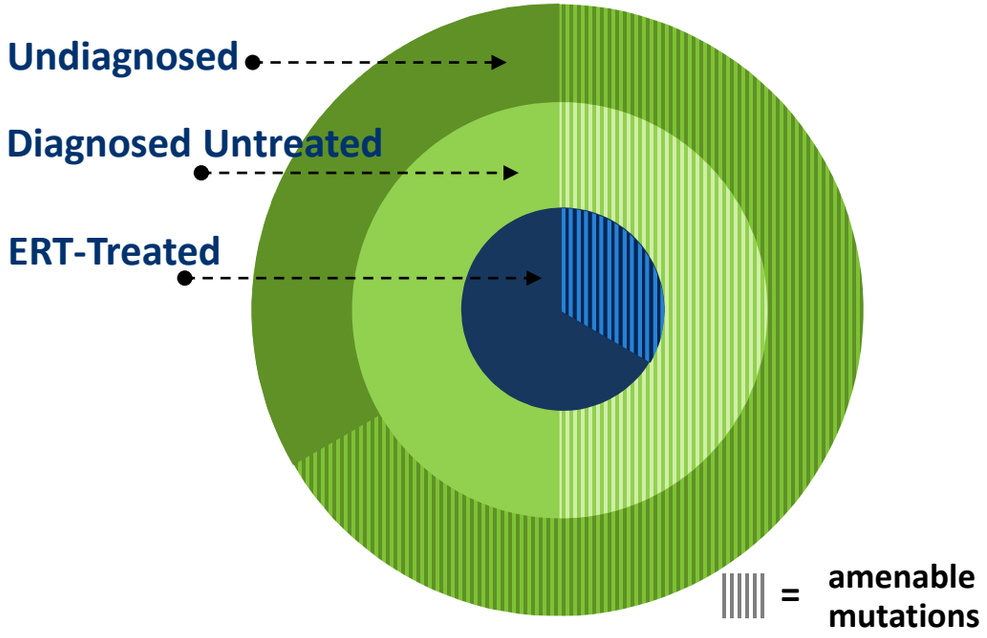
Galafold Commercial Opportunity

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[1]

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

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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

Target Number of Patients on Reimbursed Galafold by YE17

*Commercial and Expanded Access Programs (EAPs)

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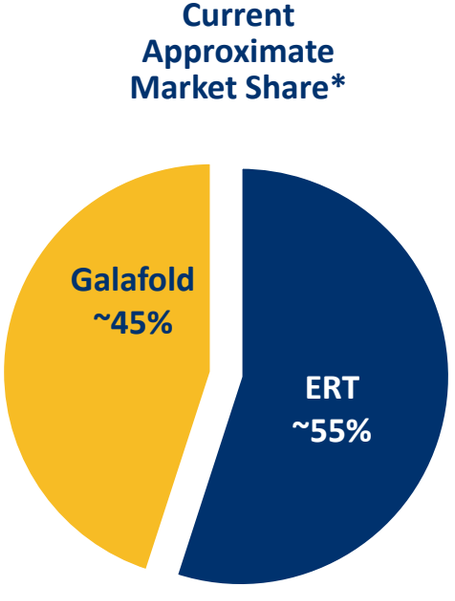
German Launch Update (as of 4/30/17)

Germany is an Important Indicator for EU Launch Success



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

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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

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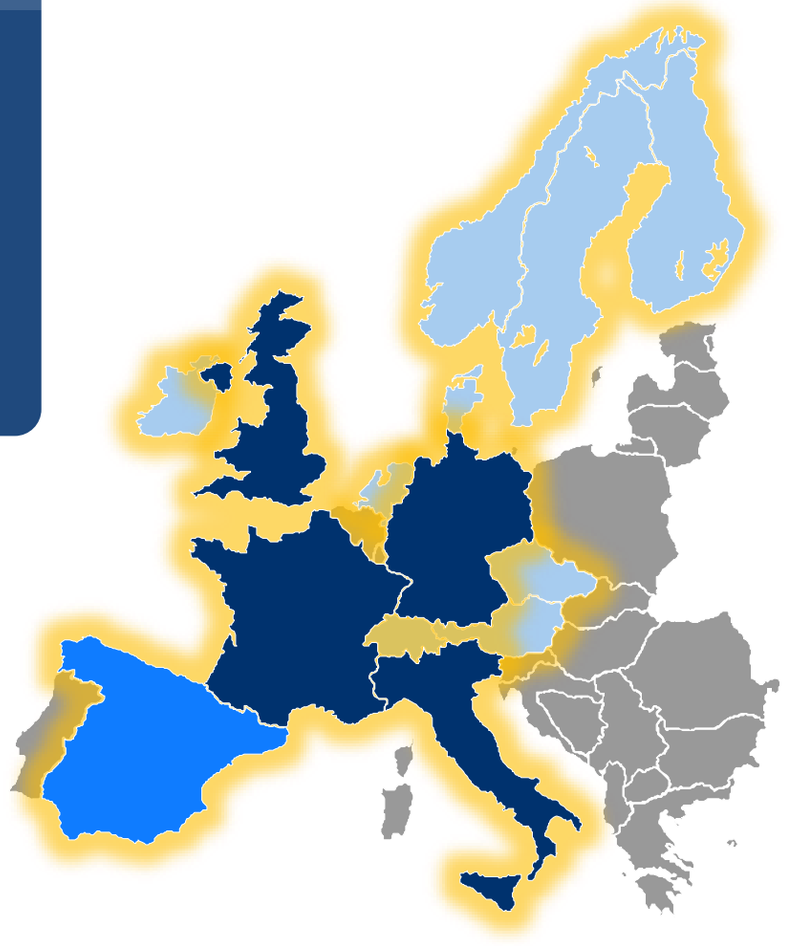
EU Launch Strategy

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

INITIAL FOCUS ON TOP 5 COUNTRIES

- 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

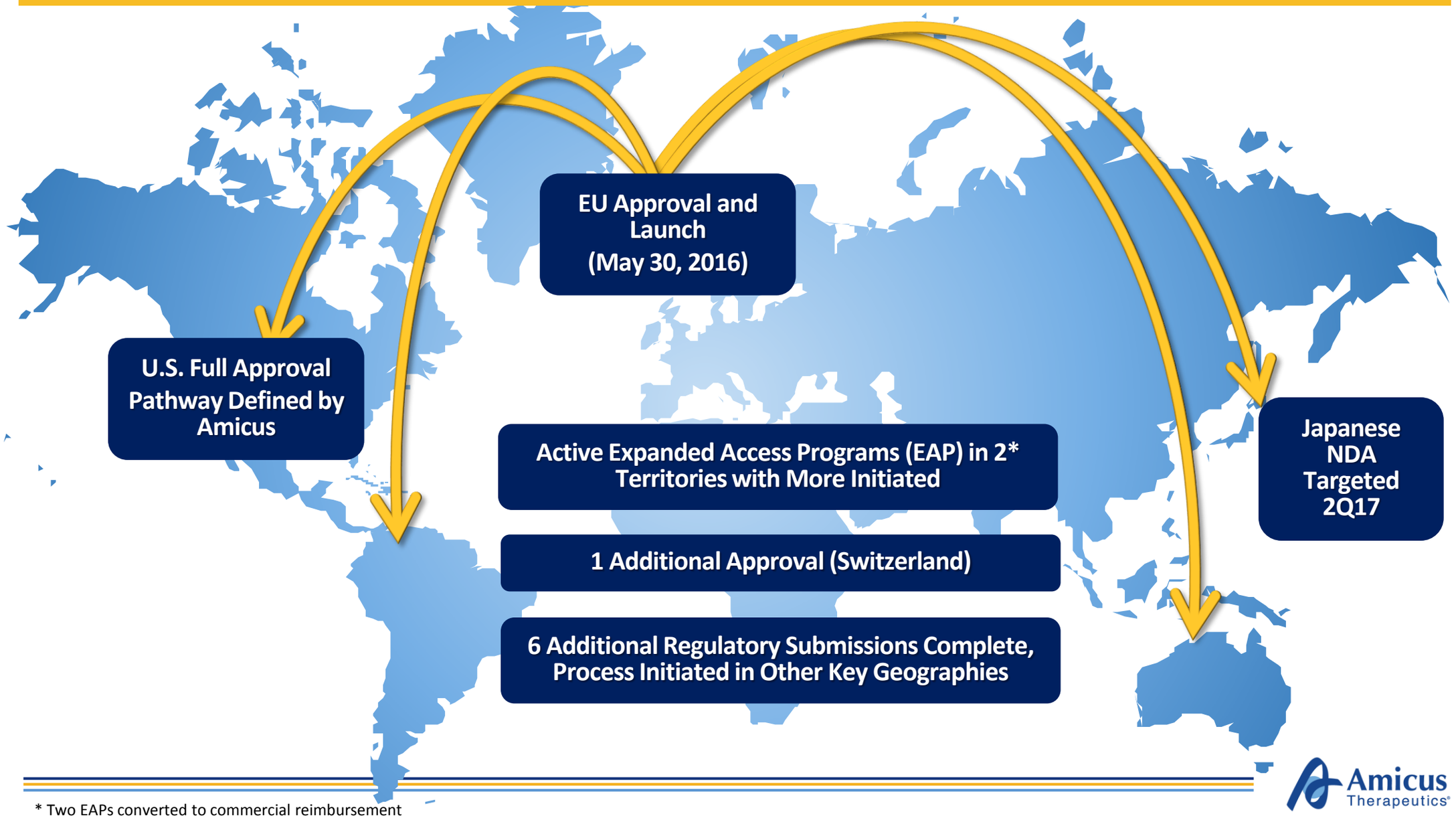


INVEST IN KEY MID-SIZED EU COUNTRIES AND SELECT EAP OPPORTUNITIES

- 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

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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

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[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

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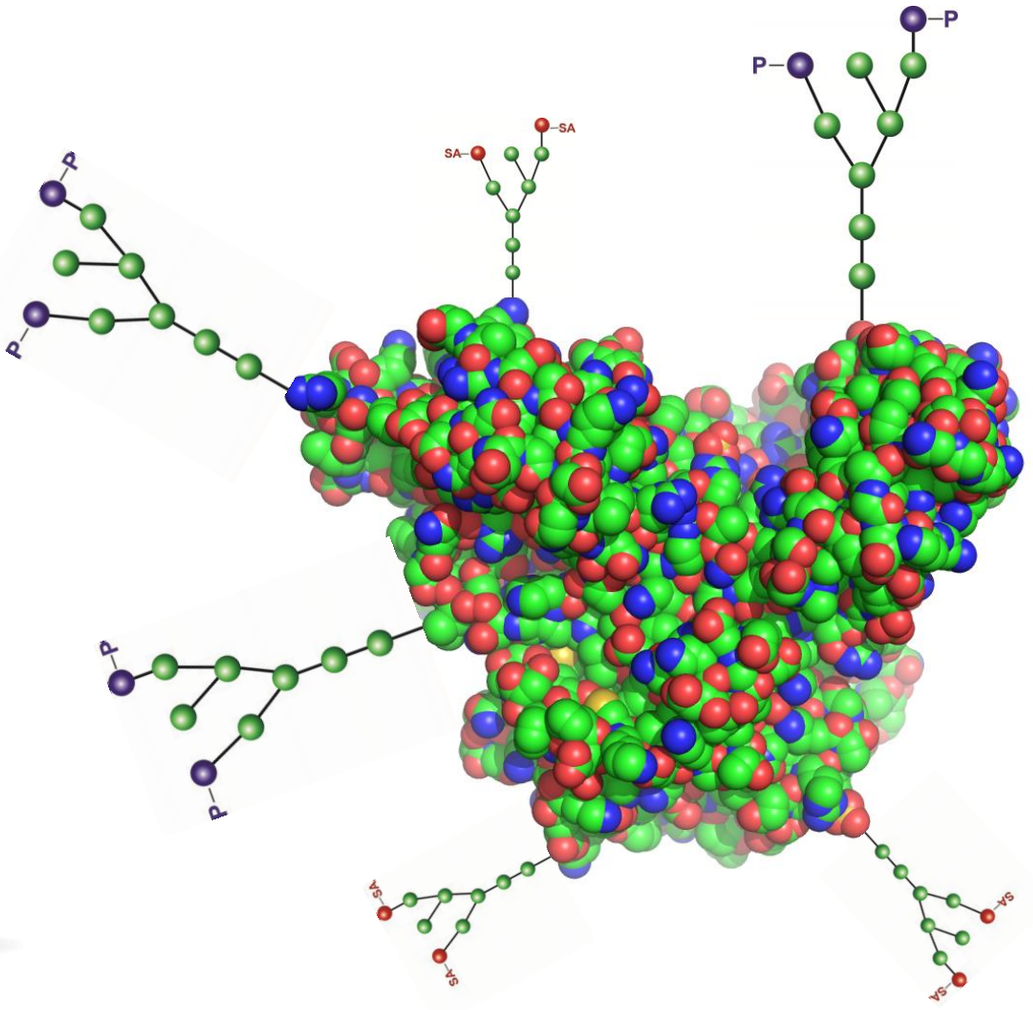
ATB200 + Chaperone: A Highly Differentiated Approach

Novel Pompe Treatment Paradigm with Three Key Differentiators

ATB200 (Novel ERT)

CHART™
CHAPERONE-ADVANCED REPLACEMENT THERAPY

Chaperone addition



Optimized mixture of glycans

High levels of M6P and bis M6P

*Artist rendering, not actual product image

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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)

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, n=4) & Cohort 3 (ERT-Naïve, n=5)

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

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	
	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**

Scoring

Measurement of force production in pounds as measured by dynamometer

Manual Muscle Testing (MMT)*

Assessment	Elbow Flex		Elbow Extension		Shoulder Adduction	
	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**

Scoring

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

*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

Research Scale / MCB

5L
(Bench Scale)
2013

250L
(Clinical Scale)
2014-2015+

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

Pompe Phase 1/2 Study ATB200-02 Data Cascade

On Track to Report Full Data Set in 3Q17

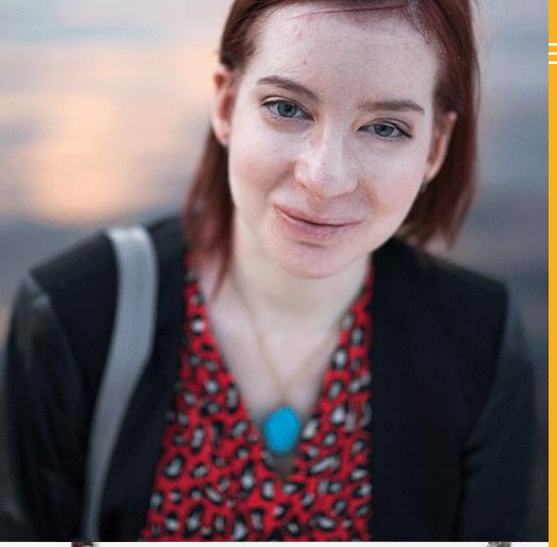
Pompe Milestones in 2017

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

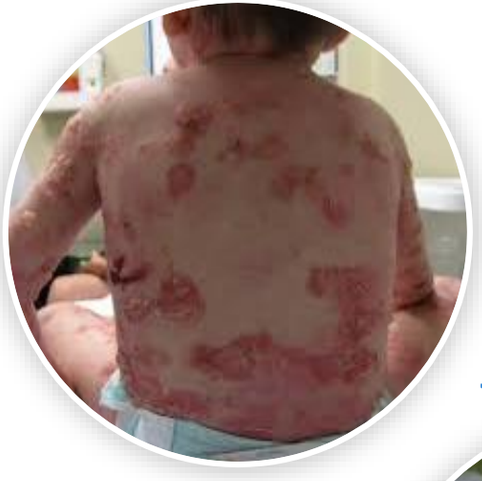
Disease Overview

- 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

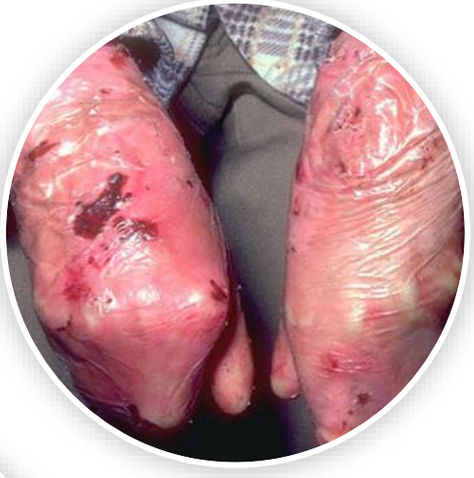
Three Major EB Types

(~99% of EB Population)

SIMPLEX (75%)



DYSTROPHIC (20%)

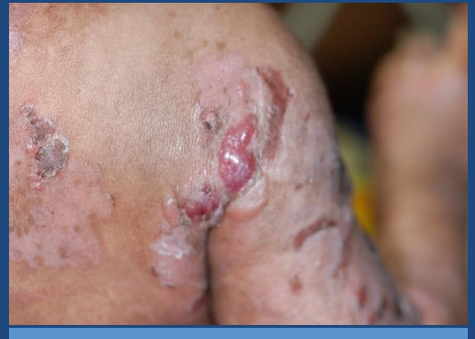


JUNCTIONAL (5%)

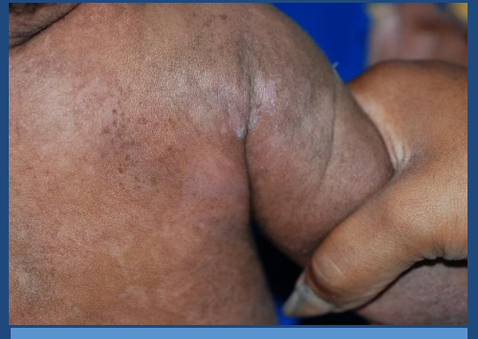
Proof of Concept Findings

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^2) 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^2

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:

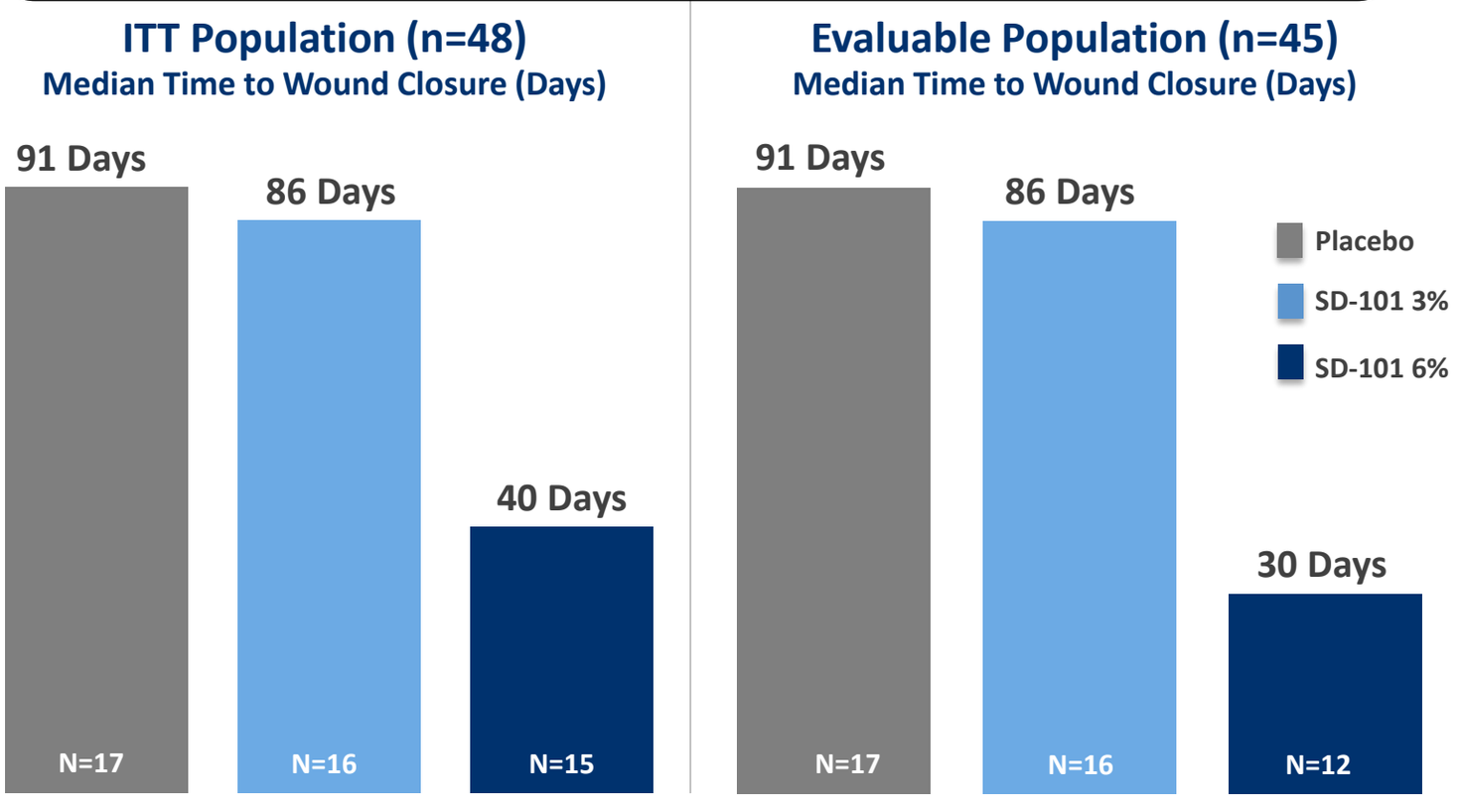
- Proportion of patients with target wound closure (at earlier timepoints)
- Change in Body Surface Area (BSA) of lesions and blisters
- Change in BSA of wounds
- Patient-reported itching
- Patient-reported pain

*Study success based on time to target would 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

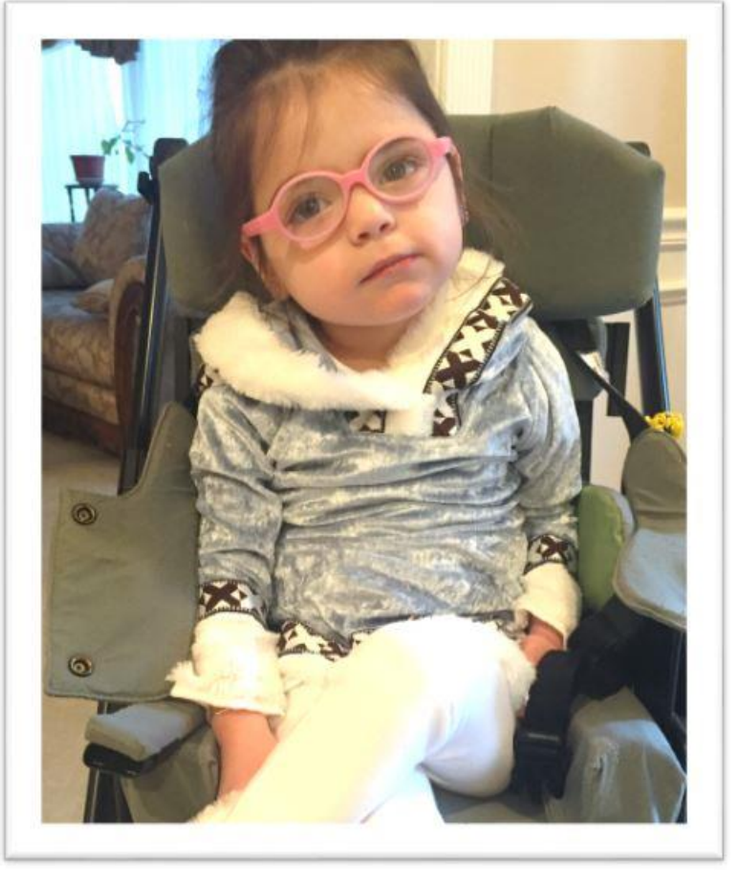
http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm071324.pdf

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[1]
- 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

Amicus Therapeutics®