

2Q15 Corporate and Program Highlights and Financial Results

August 5, 2015

*at the forefront of therapies
for rare and orphan diseases*

Safe Harbor

This presentation contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to business, operations and financial conditions of Amicus including but not limited to preclinical and clinical development of Amicus’ candidate drug products, cash runway, and the timing and reporting of results from clinical trials evaluating Amicus’ candidate drug products. Words such as, but not limited to, “look forward to,” “believe,” “expect,” “anticipate,” “estimate,” “intend,” “plan,” “would,” “should” and “could,” and similar expressions or words, identify forward-looking statements. Although Amicus believes the expectations reflected in such forward-looking statements are based upon reasonable assumptions, there can be no assurance that its expectations will be realized. Actual results could differ materially from those projected in Amicus’ forward-looking statements due to numerous known and unknown risks and uncertainties, including the “Risk Factors” described in our Annual Report on Form 10-K for the year ended December 31, 2014. All forward-looking statements are qualified in their entirety by this cautionary statement, and Amicus undertakes no obligation to revise or update this presentation to reflect events or circumstances after the date hereof.

Agenda

- 2Q15 corporate and program highlights
 - Fabry market overview
 - Pompe global strategy overview
 - 2Q15 financial results and FY15 guidance
 - Summary and upcoming milestones
- Q&A

2Q15 Corporate and Program Highlights

Successful Achievement of Multiple Corporate and Program Milestones in 2Q15

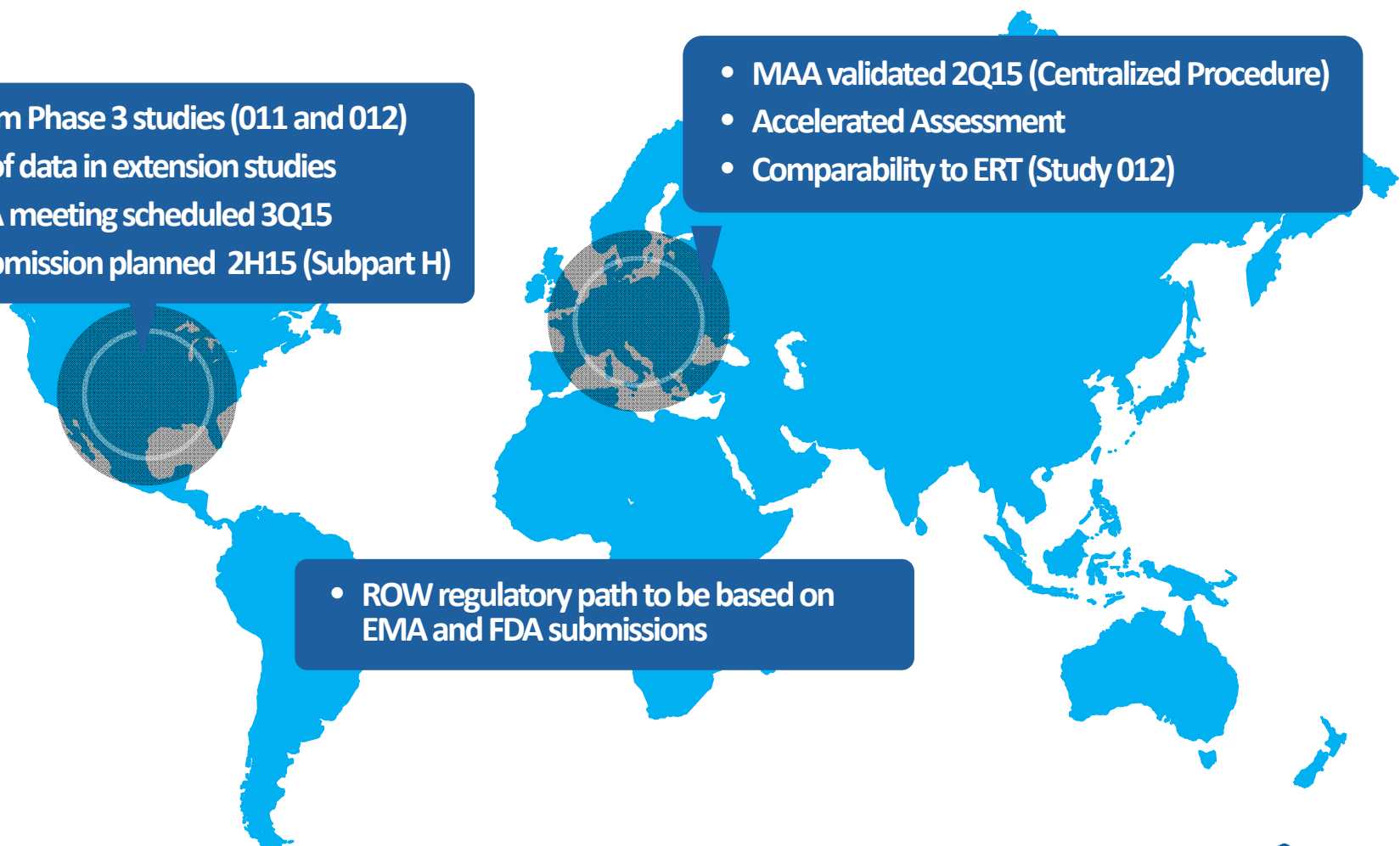
- Galafold® (migalastat HCl) for Fabry
 - MAA submitted and validated (EU review under accelerated assessment)
 - Pre-NDA meeting and NDA submission on track for 2H15 in U.S.
 - Global regulatory process initiated in additional geographies
 - Amicus commercial team in key regions
- Next-generation ERT (ATB200 + chaperone) for Pompe
 - First GMP production run successfully completed
 - IND-enabling studies nearly complete
 - Clinical study initiation on track for 2H15
- Well-capitalized to build leading patient-centric rare disease company
 - \$361.4M cash position on 6/30
 - Balance sheet strengthened with \$258.8M follow-on public offering in 2Q
- International commercial leadership team in place

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Global Regulatory Strategy

MAA Submitted in Europe and NDA on Track for 2H15 in U.S.

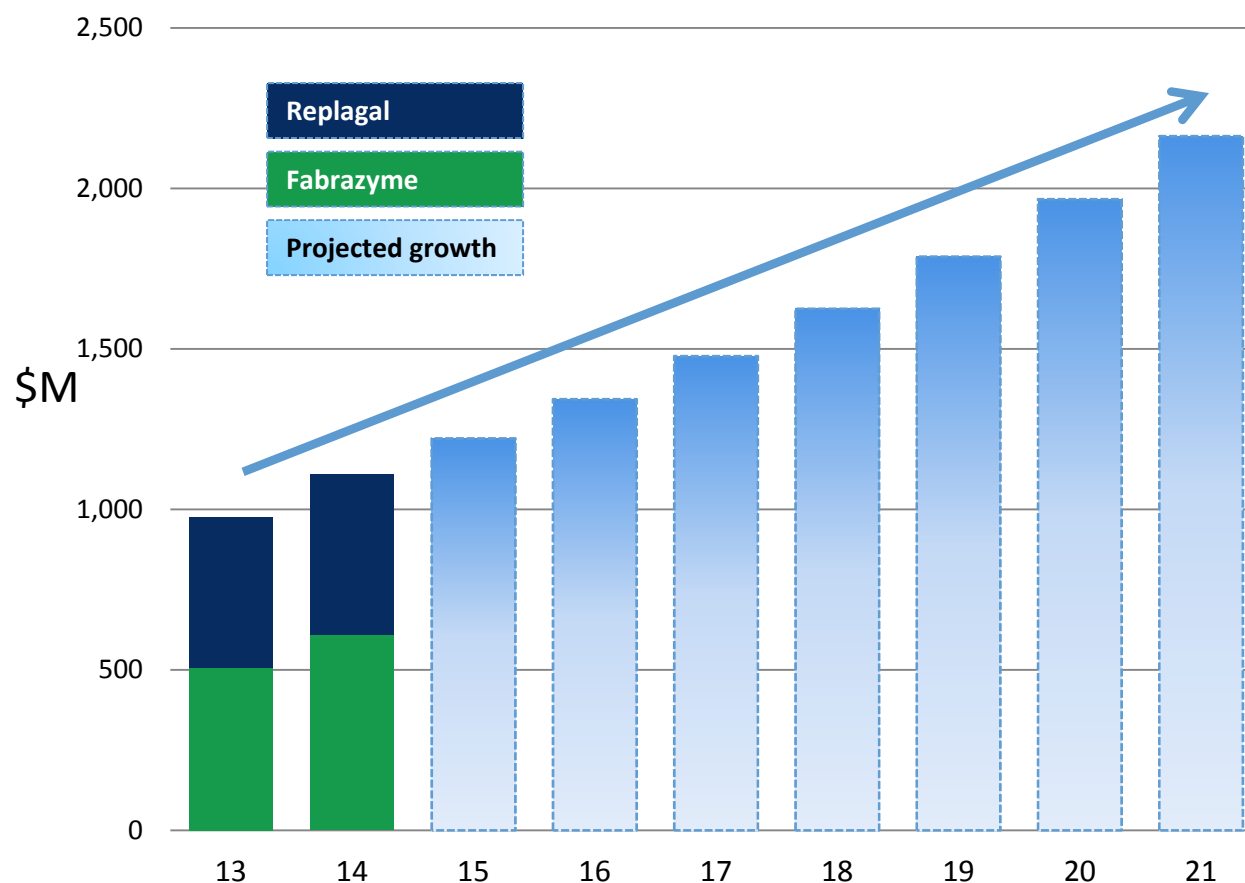
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- A world map with a light blue background. Three circular callouts are placed over North America, Europe, and Asia. Each callout contains a globe icon and a list of regulatory milestones. The callout over North America points to the U.S. regulatory path, the one over Europe points to the MAA submission, and the one over Asia points to the ROW regulatory path.
- Data from Phase 3 studies (011 and 012)
 - 9 years of data in extension studies
 - Pre-NDA meeting scheduled 3Q15
 - NDA submission planned 2H15 (Subpart H)

- MAA validated 2Q15 (Centralized Procedure)
- Accelerated Assessment
- Comparability to ERT (Study 012)

- ROW regulatory path to be based on EMA and FDA submissions

Global Fabry Market

Global Fabry Market Exceeded \$1.1B in FY14 and Tracking Toward \$2B by 2021



Fabry ERT sales increased
13.8% in 2014,
continuing trend of double-
digit annual growth¹

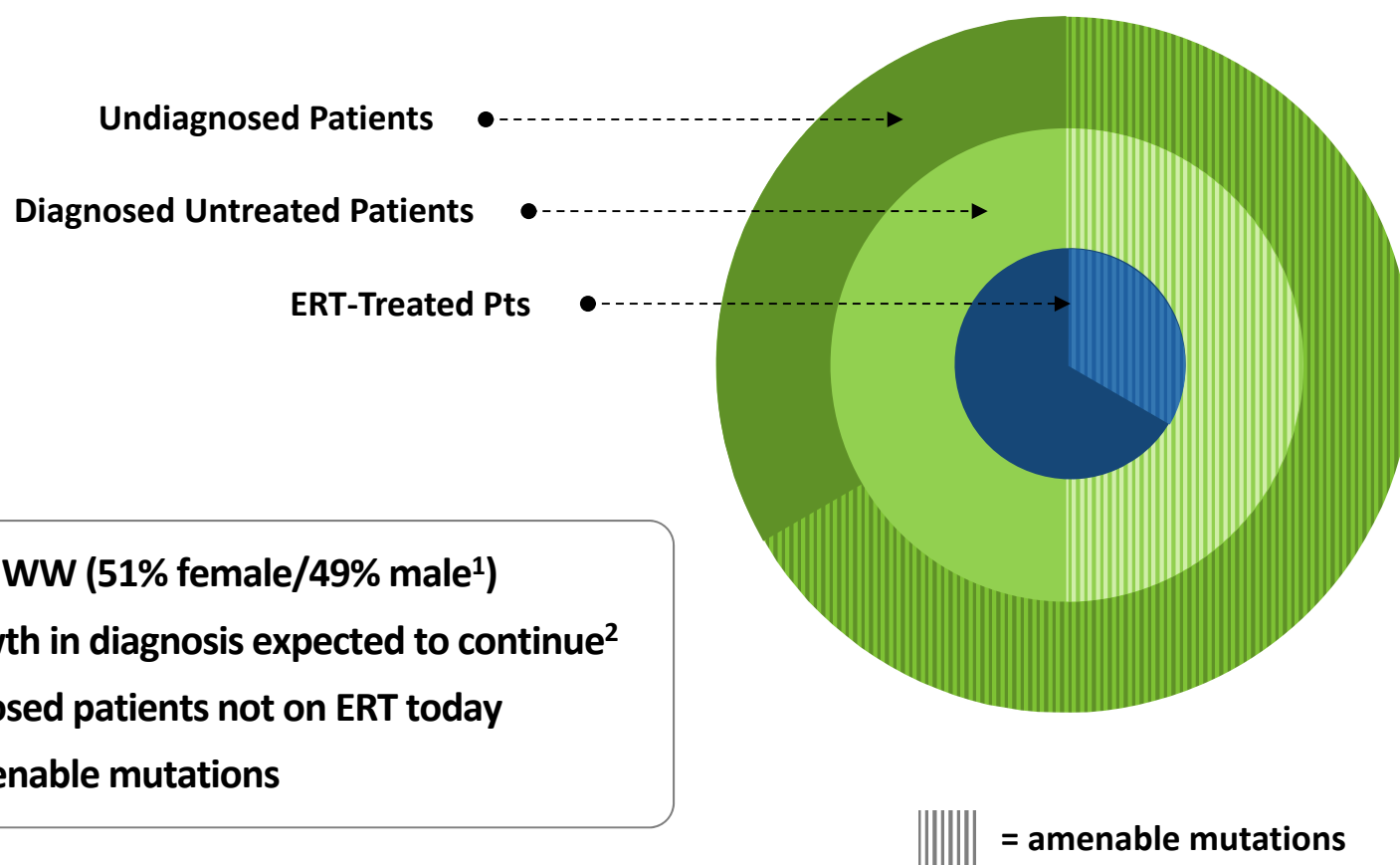
U.S. and Western Europe KOLs
expect continued market
growth:

*"The number of diagnosed patients
will increase. We keep identifying
new patients, and this number is not
decreasing year on year. I would not
be surprised if it gets close to
doubling in next 10 years."*

– UK Fabry KOL

Galafold Commercial Opportunity

Attractive Commercial Opportunity with Significant Number of Patients with Amenable Mutations

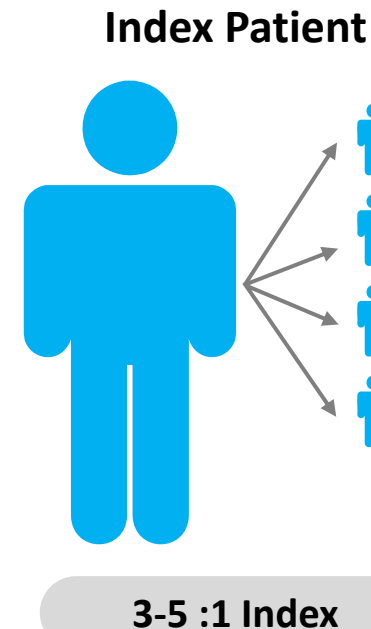


- 5-10K diagnosed WW (51% female/49% male¹)
- 10% annual growth in diagnosis expected to continue²
- 40-50% of diagnosed patients not on ERT today
- 30-50% with amenable mutations

Significant Underdiagnosis of Fabry Disease

Large Number of Patients Identified Through Newborn Screening Suggests Fabry Could Be One of the More Prevalent Human Genetic Diseases

Newborn Screening Study	# Newborns Screened	# Confirmed Fabry Mutations	% Amenable
Burton, 2012, US	8,012	7 [1: ~1100]	TBD
Mechtler, 2011, Austria	34,736	9 [1: ~3,800]	100%
Hwu, 2009, Taiwan	171,977	75 [1: ~2300]	75%
Spada, 2006, Italy	37,104	12 [1: ~3100]	86%
Historic published incidence		1:40,000 to 1:60,000	



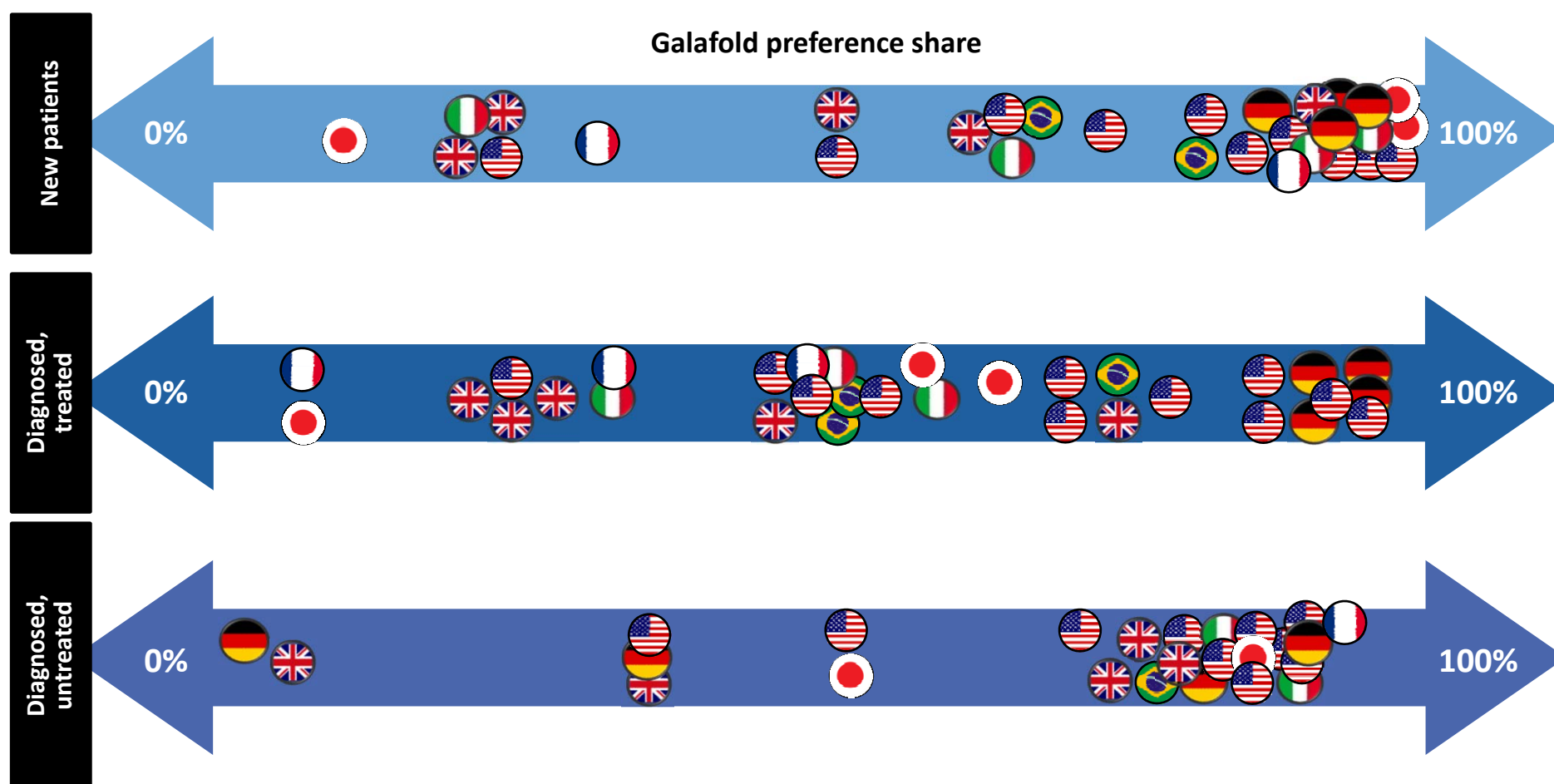
Majority Diagnosed through Newborn Screening Have Amenable Mutations

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

Positive KOL Feedback

Based on Target Product Profile, KOLs Would Use Galafold in Most Naïve and Switch Patients with Amenable Mutations with Signs and Symptoms if Approved



Payor Feedback Supports Reimbursement

Interviews with 20 Payors in Major Markets Suggest Broad Reimbursement and Coverage for Amenable Patients if Approved

Coverage supported by clinical trial data...

Based on Target Product Profile, payors interviewed in all studied countries believe there is sufficient evidence to support reimbursement of Galafold

- Payor, UK: I think the level of evidence is good enough here for reimbursement, at least at [pricing] parity to ERT

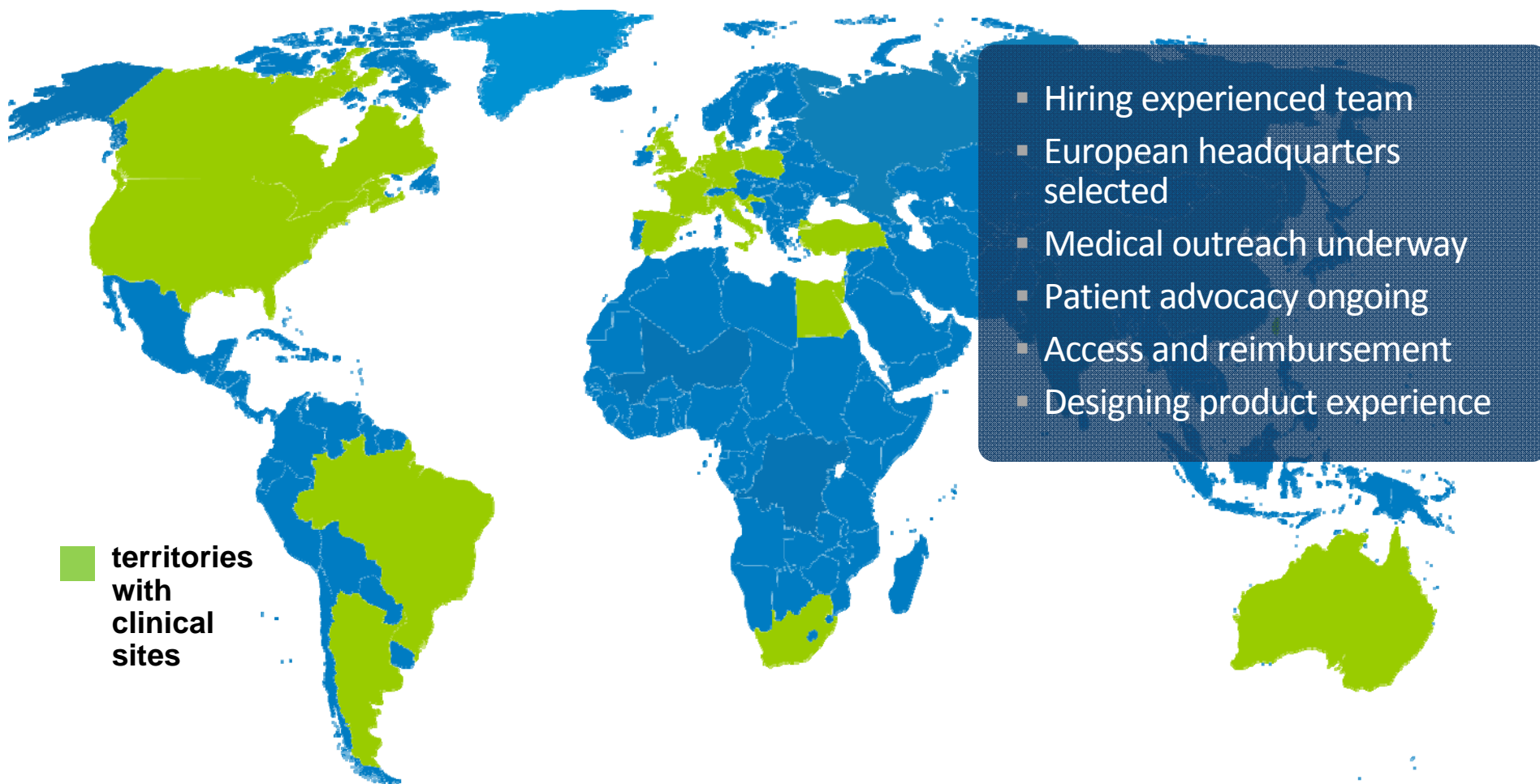
...and more convenient route of administration

Additionally, assuming parity pricing to ERT, payors generally expressed high interest in including Galafold in their formulary as they believe most patients would prefer oral route of administration over infusion

- Payor, U.S: If it was priced at parity with ERT, there would be zero restrictions on its use

Global Pre-Commercial Activities

Amicus is Building on Global Galafold Experience to Prepare for Successful Launch



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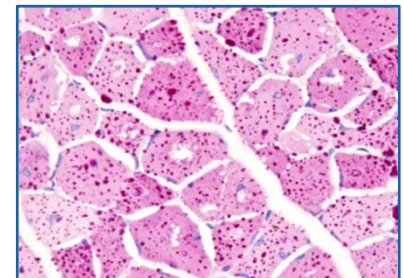
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Pompe Disease Overview

Severe, Fatal, Progressive Neuromuscular Disease with Significant Unmet Need Despite Availability of ERT



- Deficiency of GAA leading to glycogen accumulation
- Age of onset ranges from infancy to adulthood
- Symptoms include muscle weakness, respiratory failure and cardiomyopathy
- Respiratory and cardiac failure are leading causes of morbidity and mortality
- Incidence 1:28,000¹



Elevated Glycogen
in Muscle

Amicus Biologics Platform Technologies

Multiple Complementary Amicus Platform Technologies
With Potential to Address The Challenges with Existing ERTs Today

Activity/
Stability



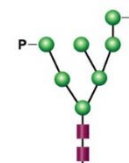
CHAPERONE-ADVANCED
REPLACEMENT THERAPY

Tolerability /
Immunogenicity



CHAPERONE-ADVANCED
REPLACEMENT THERAPY

Uptake/
Targeting



Uniquely Engineered rhGAA
Optimized M6P & Carbohydrates

Amicus Biologics Capabilities

Significant Progress From Pompe Master Cell Banking to GMP Manufacturing in < 2 Years While Maintaining High Levels of M6P and Proper Glycosylation



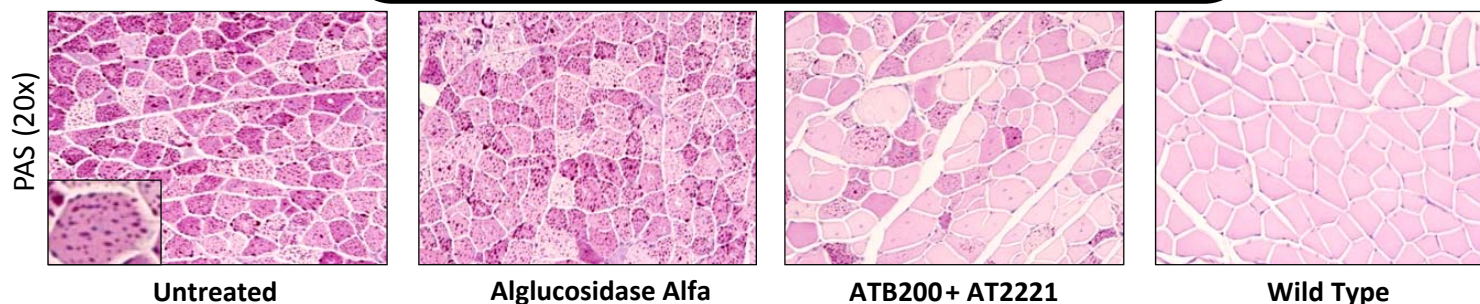
- Master cell banking in 2013
- Cell line scaled to 250 L in 2014
- First GMP batch completed 2Q15
- Additional GMP runs underway for clinical supply
- IND-enabling tox studies nearing completion by 4Q15

Image from Sartorius Stedim

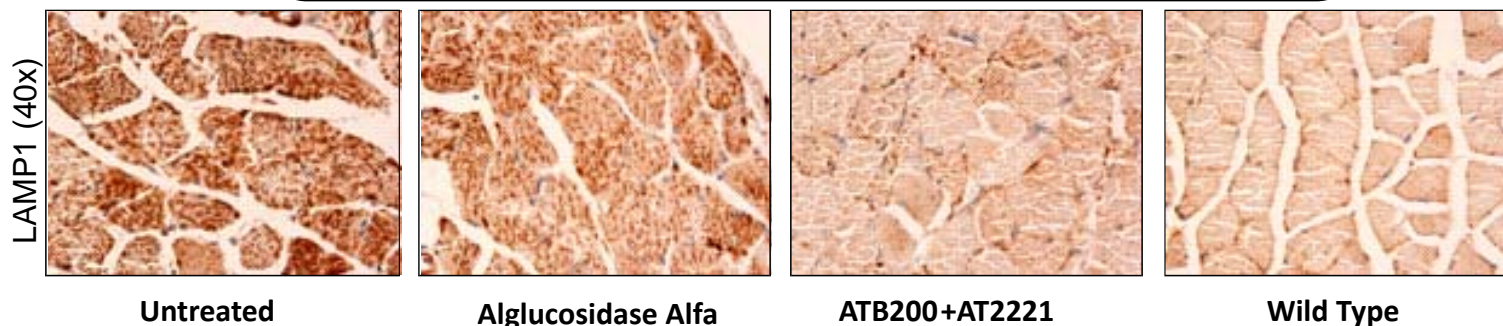
ATB200 + Chaperone Preclinical Proof-of-Concept

Glycogen Clearance Correlates with Endocytic Vesicle Turnover in Skeletal Muscle of *Gaa* KO Mice¹

PAS-glycogen staining in Quadriceps

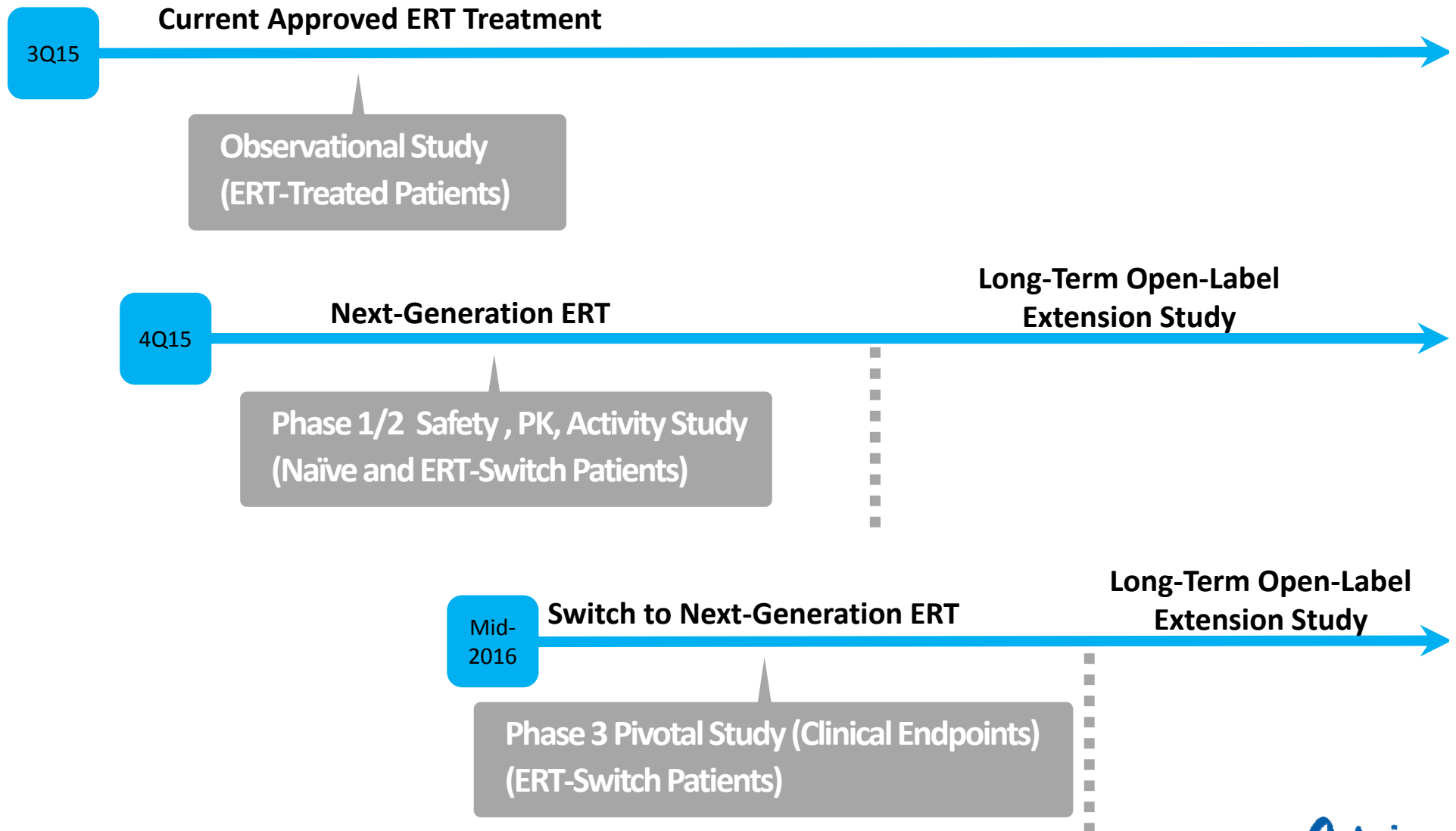


LAMP1 Immunohistochemical staining in Soleus



¹Following 2 doses of 20mg/kg Alglucosidase Alfa and ATB200 +/- AT2221 in *Gaa* KO mice, skeletal muscle evaluated for glycogen clearance and lysosomes. Treatment with ATB200 resulted in greater glycogen reduction and improved muscle physiology. Co-administration of ATB200 with AT2221 had an even greater impact on decreasing the muscle pathology associated with Pompe disease.

Proposed Pompe Clinical Development Plan



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2Q15 Financial Summary

Cash Position Provides Runway Under Current Operating Plan Into 2H17

Financial Position	June 30, 2015
Current Cash:	\$361.4M
Net Proceeds from 2Q Offering	\$258.8M
2015 Net Cash Spend:	\$100-\$110M
Cash Runway:	2H17
Capitalization	
Shares Outstanding:	118,367,319

2Q15 Financial Results

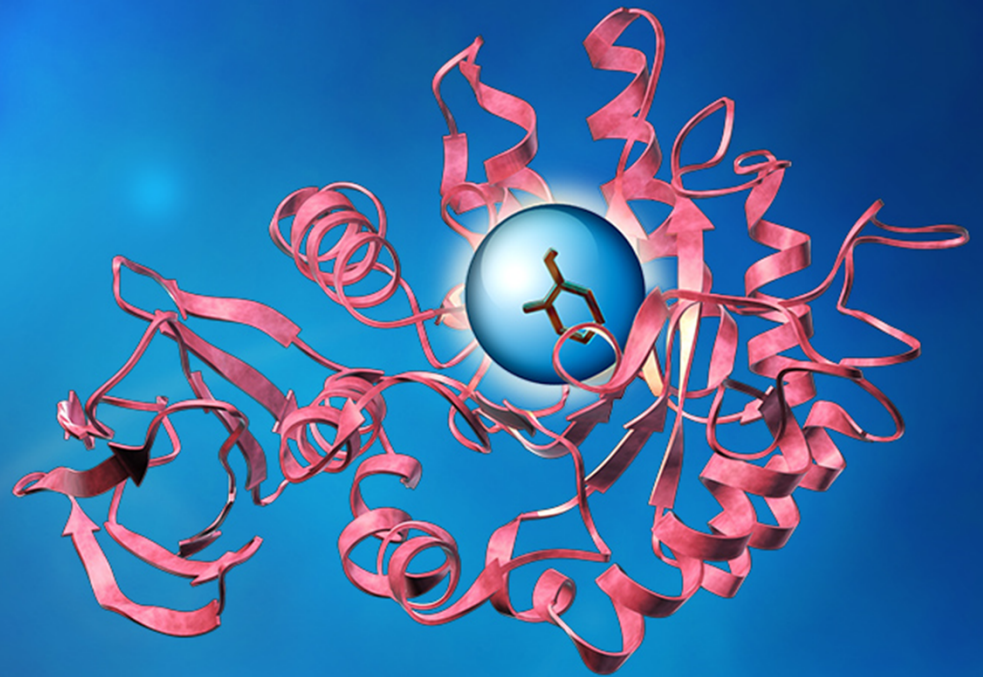
(\$000s)	June 30, 2015	June 30, 2014
Total Operating Expenses	26,943	14,741
Net Loss	(27,133)	(14,614)
Net Loss Per Share	(0.27)	(0.22)

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2H15 Anticipated Milestones

Milestones	Fabry Franchise	Milestones	Next-Generation Pompe ERT
3Q15	Pre-NDA meeting with U.S. FDA	3Q15	Pre-IND and MHRA Meetings
2H15	NDA Submission	3Q15	FPI in observational study in Pompe patients
2H15	Initiation of Phase 2 co-administration study	3Q15	Pre-IND and MHRA Meetings
Ongoing	Internal Fabry ERT cell line development	4Q15	Completion of IND-Tox Studies
		4Q15	Phase 1/2 PK study initiation (ATB200 + chaperone)



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