

3Q15 Corporate and Program Highlights and Financial Results

November 3, 2015

at the forefront of therapies for rare and orphan diseases

Safe Harbor

This presentation will contain, "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of Amicus' candidate drug products, the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus' candidate drug products, financing plans, and the projected cash position for the Company. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. The inclusion of forward-looking statements should not be regarded as a representation by Amicus that any of its plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong. They can be affected by inaccurate assumptions Amicus 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 timing of an NDA submission for migalastat monotherapy, and 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 the business of Amicus, including, without limitation: the potential that results of clinical or pre-clinical 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 may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we will need additional funding to complete all of our studies and, our dependence on third parties in the conduct of our clinical studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's cash position, actual results may differ based on market factors and the Company's ability to execute its 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, 2014 and Form 10-Q for the quarter ended June 30, 2015. 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 Amicus undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.





3Q15 corporate and program highlights

- Galafold EU regulatory update
- Epidermolysis Bullosa (EB) program update
- Pompe clinical update
- 3Q15 financial results and FY15 guidance
- Summary and upcoming milestones





3Q15 Corporate and Program Highlights

Focus on Execution Around 4 Strategic Priorities

- EU Regulatory Process on Track for Galafold[™] (migalastat HCl) for Fabry
- Working to determine optimal U.S. approval pathway for migalastat
- Planning to initiate Phase 1/2 study of novel ERT (ATB200 + chaperone) for Pompe
- Significant momentum for Zorblisa™ (SD-101) Phase 3 study rolling NDA initiated





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Galafold EU Regulatory Update

EU Timelines Under Accelerated Assessment on Track to Support Year-End 2015/Early 2016 CHMP Opinion

Anticipated Timing	Milestone	
2Q15	Accelerated Assessment Granted (150 day review)	\checkmark
2Q15	MAA Submitted	\checkmark
2Q15	MAA Validated	\checkmark
4Q15	Day 120 questions	\checkmark
Late 2015/Early 2016	CHMP opinion	
1H16	Final EU decision	

Global Pre-Commercial Activities

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





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Phase 2b (Study 003) Primary Endpoint Results % Patients with Complete Closure of Target Wounds

Zorblisa 6% Demonstrated Higher Proportion of Complete Target Wound Closure at Pre-Specified Endpoint and Subsequently During the Study

ITT Population (n=48)			
	Ν	Month 1 (pre-specified primary endpoint)	Month 2 (Phase 3 primary endpoint)
Placebo	17	41%	41%
Zorblisa 3%	16	38%	44%
Zorblisa 6%	15	53%	60%

Evaluable Population (n=45)			
	Ν	Month 1 (pre-specified primary endpoint)	Month 2 (Phase 3 primary endpoint)
Placebo	17	41%	41%
Zorblisa 3%	16	38%	44%
Zorblisa 6%	12	67%	82% (p=0.04)*

*Zorblisa 6% vs placebo, unadjusted p=0.04

Excluded from Evaluable population: 1 patient (due to lost to follow-up), 2 patients (did not have single identified and qualified target lesion)



Phase 2b (Study 003) Secondary Endpoint Median Time to Wound Closure

Zorblisa 6% Showed Fastest Time to Wound Closure in Both ITT and Evaluable Populations

	Median Time to Wound Closure (Days)		
	ITT Population (n=48)	Evaluable Population (n=45)	
Placebo	91 Days	91 Days	
Zorblisa 3%	86 Days	86 Days	
Zorblisa 6%	40 Days	30 Days	



Phase 2b (Study 003) Safety Summary

Adverse Events Similar Across Treatment Arms of Placebo, Zorblisa 3%, and Zorblisa 6%

- Treatment-emergent adverse events (TEAE) generally similar across treatment groups
- No deaths and no severe TEAEs
- No serious adverse events reported in Zorblisa 6% group

Treatment Emergent Adverse Events ≥10% Frequency

	Placebo	Zorblisa 3%	Zorblisa 6%
N subjects	17	16	15
N subjects with TEAEs (%)	12 (70.6)	13 (81.3)	9 (60.0)
Nasopharyngitis	12%	25%	7%
Pyrexia	12%	19%	33%
Application Site Pain	6%	19%	13%
Pain	-	-	13%
Skin and Subcutaneous Tissue Disorders	35%	19%	20%
Pruritus	6%	13%	13%
Rash	12%	-	7%
Rash Erythematous	12%	-	-
Cough	6%	-	13%
Oropharyngeal Pain	12%	-	-
Rhinorrhea	-	-	13%
Vomiting	6%	6%	13%
Headache	12%	-	7%



Phase 2b (Study 003): Results Summary and Key Learnings

Phase 2b Learnings Informed Dose Selection, Patient Population, and Primary Endpoint for Phase 3 Trial

- Proof of concept with similar TEAEs across treatment groups
- Clear dose response at 6% concentration
- Phase 2b results used to calculate adequate sample size in Phase 3 study
 p ≤ 0.05 if treatment difference ~17% or greater
- Placebo response minimized in baseline target wounds size ≥10 cm²
- Wound closure within 2 months (versus 1 month) is optimal time to measure primary endpoint
 - Increases ability to distinguish Zorblisa vs placebo
 - Endpoint accepted by FDA and EU regulators
- Defined approval pathway with Phase 3 study design based on EMA and FDA feedback



Zorblisa Regulatory Pathway Rolling NDA Initiated 4Q15

FDA and EMA Aligned on Phase 3 Study Design and Feedback to Date Provides Confidence in Global Approval of Zorblisa in Major Subtypes of EB

- Breakthrough Therapy Designation (BTD) based on Phase 2 POC
- Orphan drug designation
- Rolling NDA initiated 4Q15

- Orphan drug designation
- Approved Pediatric Investigation Plan (PIP)
- Defined registration pathway

 ROW regulatory path based on EMA and FDA submissions





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Amicus Biologics Milestones Achieved

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
- GMP batches completed 2Q15-3Q15 to initiate upcoming clinical study



ATB200: A Pompe ERT Optimized for Lysosomal Targeting via the CI-MPR

ATB200 Produced with Naturally High Amount of M6P Content **Critical Quality Attributes to Enable Efficient Drug Targeting Maintained During Scale Up**

- Proprietary cell line produces a well phosphorylated rhGAA
- Current manufacturing process produces ATB200 with naturally high M6P content
- Cell line and process scaled up from 2 L to 250L bioreactors while maintaining all critical quality attributes to enable efficient drug targeting





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





Untreated



Alglucosidase Alfa



ATB200+ AT2221



Wild Type

LAMP1 Immunohistochemical staining in Soleus



Untreated

Alglucosidase Alfa

ATB200+AT2221

Wild Type

¹Following 2 doses of 20mg/kg Alglucosidase Alfa or ATB200 + AT2221 in Gaa KO mice, skeletal muscles evaluated for glycogen clearance and proliferated lysosomes. Treatment with Alglucosidase Alfa modestly reduced glycogen or proliferated lysosomes while ATB200, co-administered with AT2221 significantly decreased the muscle pathology associated with Pompe disease.



ATB200 Summary and Next Steps

- Clinical trial material ready
- Successful pre-IND meeting to discuss Phase 1/2 safety and PK study in ERT-switch Pompe patients
- On track to initiate Phase 1/2 study pending IND clearance





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3Q15 Financial Summary

Cash Position Provides Runway Under Current Operating Plan Into 1H17

Financial Position	September 30, 2015
Current Cash:	\$251.9M
Anticipated Year-end Cash Balance:	\$200-\$225M
Cash Runway:	1H17
Capitalization	
Shares Outstanding:	124,617,490



3Q15 Financial Results

(\$000s)	Sept. 30, 2015	Sept. 30, 2014
Total Operating Expenses	38,045	17,109
Net Loss	(37,800)	(17,149)
Net Loss Per Share	(0.32)	(0.22)





Appendix

Epidermolysis Bullosa (EB)

Rare, Devastating, Connective Tissue Disorder with No Approved Treatments



- Multiple genes cause disease which results in fragility of skin and can also affect internal organs
- Life-long chronic condition that typically manifests at birth
 - Severe blistering, open wounds and scarring
- Disfiguring, excruciatingly painful, and can be fatal
- 30,000 40,000 <u>diagnosed</u> patients in major global regions



Phase 2b (Study 003) Design

48 EB patients (age \geq 6 months)* - 1:1:1 Randomization - Daily Topical Application

Zorblisa 6% (n=15)	
	Open-Label Zorblisa (6%)
Zorblisa 3% (n=16)	
Placebo (n=17)	Ongoing Extension (SD-004)
3-Month Double-Blind Treatment Period	42/44 patients entered extension study
Assessments: 0, 14, 30, 60, 90 Days	

Primary Efficacy Endpoint: Target Wound Healing at Month 1 Baseline wound: Chronic (≥ 21 days), size 5-50 cm²

Secondary Endpoints:

Change in BSA of lesions and blisters; itching; pain

*Initial Disease Severity: Mean target lesion size (cm²) 14.0 (range 5-39); mean lesional BSA: 19.4% (range 0.4-48%); mean wound age (days): 182 (range 21-1,639) EB Subtypes enrolled: Simplex (n=11), Recessive Dystrophic (n=29), and Junctional (n=8)



Phase 2b (Study 003) Efficacy Results ITT Population (n=48)

Proportion of Complete Target Wound Closure over 3 Months Indicates Early and Sustained Separation Between Zorblisa 6% and Placebo





Phase 2b (Study 003) Efficacy Results *Evaluable Population (n=45)*

Early and Sustained Separation Between Zorblisa 6% and Placebo Also Observed in the Evaluable Population

Proportion with Complete Target Wound Closure Over 3 Months



micus

(Evaluable population only)*

Pivotal Phase 3 (Study 005) Underway Study Design Supported by Both FDA and EMA

Phase 3 Initiated in 2Q15 and Currently Enrolling Patients Top-line data expected 2H 2016

Zorblisa 6%

~150 EB patients (age \geq 1 month) 1:1 Randomization - Daily Topical Application

Placebo

3-Month Double-Blind Treatment Period Assessments: 0, 14, 30, 60, 90 Days

Primary Efficacy Endpoint: Target Wound Healing at Month 2

- US and EU regulatory authorities agreed to target wound healing as primary endpoint
- Baseline wound: Chronic (≥ 21 days), size ≥10 cm²

Secondary Endpoints

 Time to target wound closure; Change in Body Surface Area (BSA) of lesions and blisters; itching; pain

Optional Extension (SD-006)

Open-Label Zorblisa (6%)

36/36 Patients Who Completed Study 005 Continued in Open-Label Extension (Oct. 2015)



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





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