

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

**CURRENT REPORT PURSUANT TO
SECTION 13 OR 15(D) OF THE
SECURITIES EXCHANGE ACT OF 1934**

Date of Report (Date of earliest event reported): **November 3, 2015**

AMICUS THERAPEUTICS, INC.

(Exact Name of Registrant as Specified in Its Charter)

Delaware

(State or Other Jurisdiction of
Incorporation)

001-33497

(Commission File Number)

71-0869350

(IRS Employer Identification No.)

1 Cedar Brook Drive, Cranbury, NJ

(Address of Principal Executive Offices)

08512

(Zip Code)

Registrant's telephone number, including area code: **(609) 662-2000**

(Former Name or Former Address, if Changed Since Last Report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Item 2.02. Results of Operations and Financial Condition.

On November 3, 2015, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the quarter ended September 30, 2015. A copy of this press release is attached hereto as Exhibit 99.1. The Company will also host a conference call and webcast on November 3, 2015 to discuss its third quarter results of operations. A copy of the conference call presentation materials is also attached hereto as Exhibit 99.2.

In accordance with General Instruction B.2. of Form 8-K, the information in this Current Report on Form 8-K and the Exhibit shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits: The Exhibit Index annexed hereto is incorporated herein by reference.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Amicus Therapeutics, Inc.

Date: November 3, 2015

By: /s/ William D. Baird III
William D. Baird III
Chief Financial Officer

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EXHIBIT INDEX

| Exhibit No. | Description |
|--------------------|---|
| 99.1 | Press Release dated November 3, 2015 |
| 99.2 | November 3, 2015 Conference Call Presentation Materials |

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Amicus Therapeutics Announces Third Quarter 2015 Financial Results and Corporate Updates

Migalastat for Fabry Disease Moving Forward to CHMP Opinion in European Union

Novel Pompe ERT Advancing to Enter Phase 1/2 Study in Pompe Patients

Rolling NDA Now Initiated for SD-101 for Epidermolysis Bullosa

Conference Call at 4:30 p.m. ET Today

CRANBURY, NJ, November 3, 2015 – Amicus Therapeutics (Nasdaq: FOLD), a biotechnology company at the forefront of therapies for rare and orphan diseases, today announced financial results for the third quarter ended September 30, 2015. The Company also provided program updates and reiterated financial guidance for 2015 year ending cash balance of \$200-\$225 million.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “During the third quarter we made significant progress in executing our patient-centric vision to build one of the world’s leading biotechnology companies focused on devastating rare and orphan diseases. We are sharply focused on four key business priorities ahead that we believe have the potential to create substantial shareholder value and to deliver upon our mission for rare disease patients – approval and commercialization of our personalized medicine migalastat for Fabry patients in Europe, development of an optimal U.S. approval pathway for migalastat, initiation of clinical studies in Pompe patients with our novel Pompe ERT ATB200, and completion of our Phase 3 study and rolling NDA submission for SD-101 for Epidermolysis Bullosa.”

Financial Highlights for Third Quarter Ended September 30, 2015

- Cash, cash equivalents, and marketable securities totaled \$251.9 million at September 30, 2015, compared to \$169.1 million at December 31, 2014.
- Completed acquisition of Scioderm Inc. for approximately \$224 million (approximately \$141 million paid in cash and approximately \$83 million paid through issuance of 5.9 million newly issued Amicus shares)
- Total operating expenses increased to \$38.0 million compared to \$17.1 million for the third quarter 2014, primarily due to increases in preclinical and clinical development costs on the Fabry monotherapy and Pompe ERT programs as well as pre-commercial organizational costs.
- Net loss was \$37.8 million, or \$0.32 per share, compared to a net loss of \$17.1 million, or \$0.22 per share, for the third quarter 2014.

2015 Financial Guidance

Cash, cash equivalents, and marketable securities totaled \$251.9 million at September 30, 2015 compared to \$169.1 million at December 31, 2014. During the third quarter, Amicus successfully completed its acquisition of 100% of the capital stock of Scioderm, Inc. With the closing of the Scioderm acquisition and the forecasted spending on the EB clinical development program, Amicus expects to end 2015 with between \$200 million and \$225 million of cash on hand. Current cash is expected to fund the Company’s operating plan (including SD-101) into 2017.

Program Highlights

Migalastat for Fabry Disease

Amicus has made significant progress in building the commercial organization and preparing for the launch of migalastat in Europe and other international geographies. The European Medicines Agency’s (EMA) review of the marketing authorization application (MAA) for migalastat remains on track under accelerated assessment. The Day 120 questions have been received and Amicus continues to expect an opinion from the Committee for Medicinal Products for Human Use (CHMP) in late 2015 or early 2016. As previously reported, the timing of an NDA submission in the U.S. will be based on the determination of the optimal regulatory pathway. An update on the U.S. strategy for migalastat will be provided by Amicus in the first quarter of 2016.

Amicus is developing migalastat in combination with ERT for Fabry patients who do not have amenable mutations and cannot take monotherapy. Amicus is developing a novel Fabry ERT cell line, and preclinical proof-of-concept studies co-formulating Amicus’ Fabry ERT with migalastat will begin this quarter. As the Amicus internal novel ERT for Fabry has continued to advance, the Company plans to focus exclusively on this proprietary cell line for co-formulation with migalastat and no longer plans to conduct a co-administration study with commercially available ERT. Amicus believes that further development of its own proprietary Fabry ERT co-formulated with migalastat represents the fastest and best path for bringing a novel therapy and meaningful improvements to Fabry patients with non-amenable mutations.

Anticipated Fabry Milestones:

- CHMP Opinion for migalastat in Europe by year-end 2015/early 2016
- Internal development underway of novel ERT (Fabry cell line for co-formulation with migalastat)
- 30-month data from Phase 3 Study 012 (ERT-switch patients) in early 2016

Novel ERT for Pompe Disease (ATB200 + Chaperone)

Amicus completed good manufacturing practice (GMP) production runs of ATB200, a novel Pompe ERT, during the third quarter of 2015, and has successfully manufactured sufficient supply for upcoming clinical studies. This novel ERT consists of a uniquely engineered recombinant human acid alpha-glucosidase (rhGAA) enzyme with an optimized carbohydrate structure to enhance uptake, administered with a pharmacological chaperone enhancer to improve activity and stability.

Following a pre-IND meeting held recently with the U.S. Food and Drug Administration (FDA), Amicus plans to move forward with the clinical plan to develop ATB200 with a pharmacological chaperone as a fixed-dose combination. The initial clinical study will be a Phase 1/2 study in Pompe patients. Additional information about the Pompe ERT manufacturing progress and development plan will be discussed on this afternoon's conference call.

Anticipated Pompe Program Milestones:

- Phase 1/2 clinical study initiation
- Interim & full Phase 1/2 data
- End-of-Phase 2 meeting with FDA to finalize Phase 3 pivotal study (2H16)

SD-101 for Epidermolysis Bullosa (EB)

The acquisition of Scioderm strengthened Amicus' pipeline with the addition of SD-101, a novel, late-stage, proprietary topical cream and potential first-to-market therapy for EB. This investigational product was granted FDA breakthrough therapy designation in 2013 based on results from Phase 2 studies for the treatment of lesions in patients suffering with EB. SD-101 was the first-ever treatment in EB clinical studies to show improvements in wound closure across all major EB subtypes.

SD-101 is currently being investigated in a Phase 3 study (SD-005) to support global regulatory submissions. A rolling NDA submission was initiated in October 2015 and is expected to be completed once top-line data are available from the ongoing Phase 3 study.

Anticipated EB Program Milestones:

- Top-line Phase 3 data in 2H16
- Submission of final NDA section

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, November 3, 2015 at 4:30 p.m. ET to discuss third quarter 2015 financial results and program updates. Interested participants and investors may access the conference call at 8:30 a.m. ET by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international).

An audio webcast and slide presentation can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at <http://www.amicusrx.com>, and will be archived for 30 days. Web participants are encouraged to go to the web site 15 minutes prior to the start of the call to register, download and install any necessary software. A telephonic replay of the call will be available for seven days beginning at 8:00 p.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 65781940.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a biotechnology company at the forefront of therapies for rare and orphan diseases. The Company has a robust pipeline of advanced therapies for a broad range of human genetic diseases. Amicus' lead programs in development include the small molecule pharmacological chaperone migalastat as a monotherapy for Fabry disease, SD-101 for Epidermolysis Bullosa (EB), as well as novel enzyme replacement therapy (ERT) products for Fabry disease, Pompe disease, and other lysosomal storage disorders.

Forward-Looking Statements

This press release contains, and the accompanying conference call slides 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.

CONTACTS:

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Media:

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Table 1

Amicus Therapeutics, Inc.
Consolidated Statements of Operations
(Unaudited)
(in thousands, except share and per share amounts)

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--|-------------------------------------|--------------------|------------------------------------|--------------------|
| | 2015 | 2014 | 2015 | 2014 |
| Revenue: | | | | |
| Research revenue | — | \$ 293 | — | \$ 1,224 |
| Total revenue | — | 293 | — | 1,224 |
| Operating Expenses: | | | | |
| Research and development | \$ 20,971 | \$ 12,049 | \$ 54,318 | \$ 32,019 |
| General and administrative | 15,372 | 5,270 | 30,077 | 15,199 |
| Changes in fair value of contingent consideration payable | 1,300 | (600) | 2,400 | (400) |
| Restructuring charges | 7 | 15 | 44 | (74) |
| Loss on extinguishment of debt | — | — | 952 | — |
| Depreciation | 395 | 375 | 1,256 | 1,183 |
| Total operating expenses | 38,045 | 17,109 | 89,047 | 47,927 |
| Loss from operations | (38,045) | (16,816) | (89,047) | (46,703) |
| Other income (expenses): | | | | |
| Interest income | 316 | 55 | 645 | 133 |
| Interest expense | (17) | (377) | (727) | (1,106) |
| Other expense | (54) | (11) | (93) | (30) |
| Net loss | <u>\$ (37,800)</u> | <u>\$ (17,149)</u> | <u>\$ (89,222)</u> | <u>\$ (47,706)</u> |
| Net loss per common shares – basic and diluted | \$ (0.32) | \$ (0.22) | \$ (0.85) | \$ (0.68) |
| Weighted-average common shares outstanding – basic and diluted | 118,724,882 | 78,889,346 | 104,885,956 | 70,216,251 |

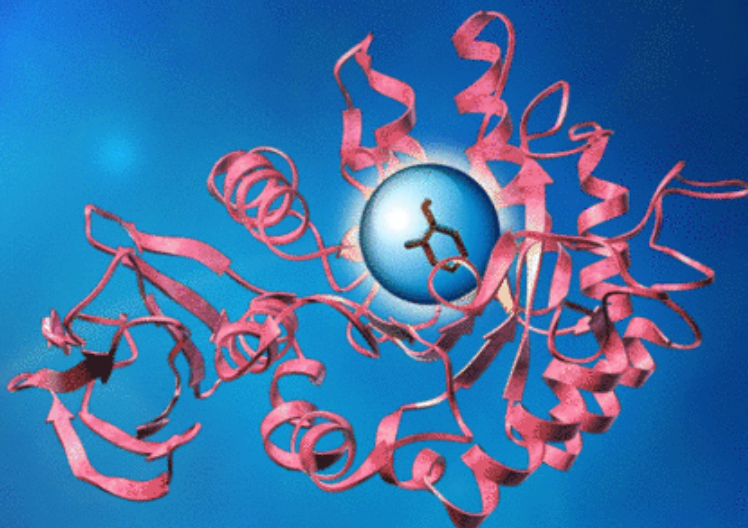
Table 2

Amicus Therapeutics, Inc.
Consolidated Balance Sheets
(Unaudited)
(in thousands, except share and per share amounts)

| | September 30, 2015 | December 31, 2014 |
|--|-----------------------|----------------------|
| Assets: | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 19,439 | \$ 24,074 |
| Investments in marketable securities | 217,070 | 127,601 |
| Prepaid expenses and other current assets | 3,544 | 2,902 |
| Total current assets | 240,053 | 154,577 |
| Investments in marketable securities | 15,428 | 17,464 |
| Property and equipment, less accumulated depreciation of \$12,776 and \$11,520 at September 30, 2015 and December 31, 2014, respectively | 3,855 | 2,811 |
| In-process research & development | 518,810 | 23,000 |

| | | |
|---|-------------------|-------------------|
| Goodwill | 207,564 | 11,613 |
| Other non-current assets | 982 | 502 |
| Total Assets | \$ 986,692 | \$ 209,967 |
| Liabilities and Stockholders' Equity | | |
| Current liabilities: | | |
| Accounts payable and accrued expenses | \$ 30,565 | \$ 16,345 |
| Current portion of contingent consideration payable | 5,300 | — |
| Current portion of secured loan | — | 3,840 |
| Total current liabilities | 35,865 | 20,185 |
| Deferred reimbursements | 36,620 | 36,620 |
| Secured loan, less current portion | — | 10,510 |
| Due to related party | 50,000 | — |
| Contingent consideration payable, less current portion | 277,684 | 10,700 |
| Deferred tax liability | 207,213 | 9,186 |
| Other non-current liabilities | 555 | 588 |
| Commitments and contingencies | | |
| Stockholders' equity: | | |
| Common stock, \$.01 par value, 250,000,000 shares authorized, 124,617,490 shares issued and outstanding at September 30, 2015, 125,000,000 shares authorized, 95,556,277 shares issued and outstanding at December 31, 2014 | 1,304 | 1,015 |
| Additional paid-in capital | 914,263 | 568,743 |
| Accumulated other comprehensive loss | (142) | (132) |
| Accumulated deficit | (536,670) | (447,448) |
| Total stockholders' equity | 378,755 | 122,178 |
| Total Liabilities and Stockholders' Equity | \$ 986,692 | \$ 209,967 |

FOLD-G



***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.

Agenda

- 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
- Q&A

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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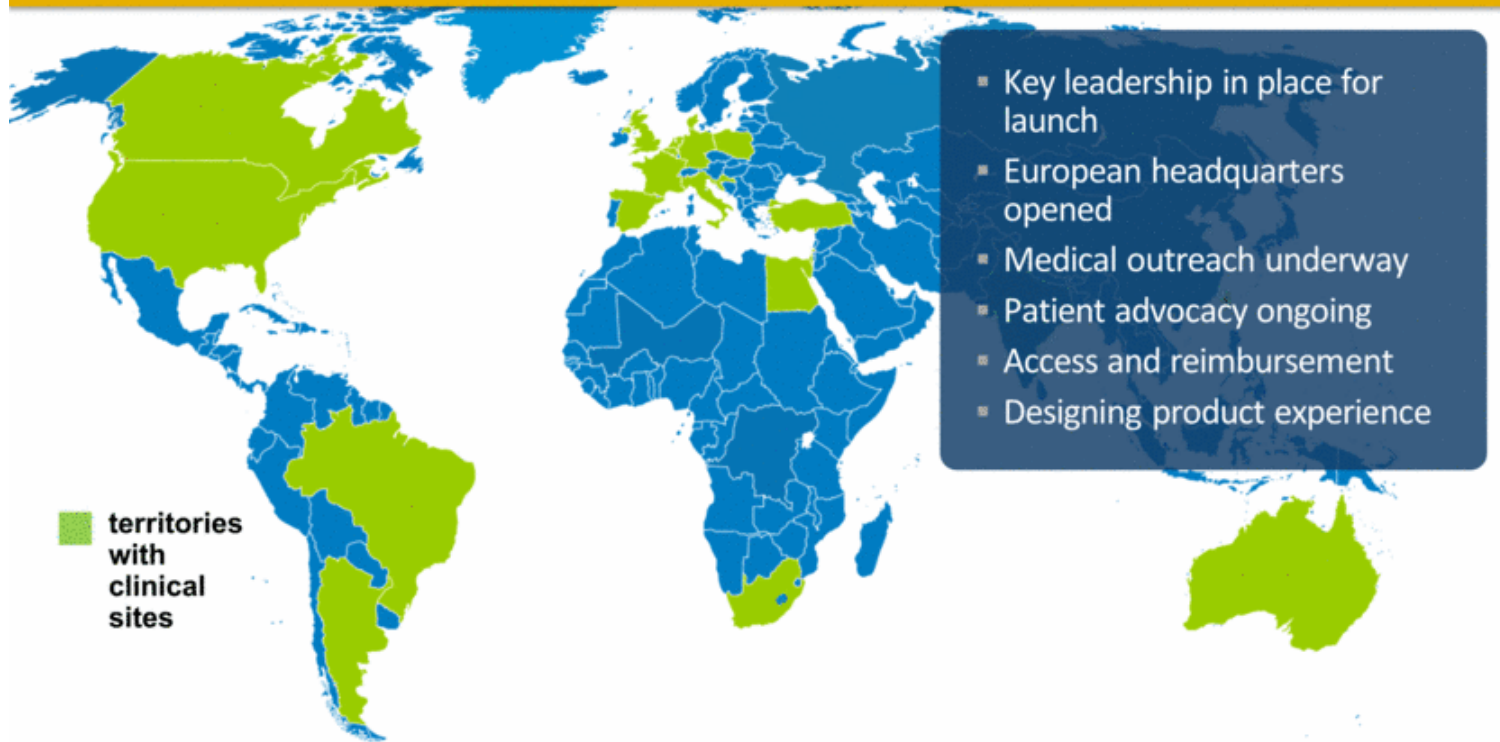
Galafold EU Regulatory Update

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) | ✓ |
| 2Q15 | MAA Submitted | ✓ |
| 2Q15 | MAA Validated | ✓ |
| 4Q15 | Day 120 questions | ✓ |
| 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) | | | |
|-----------------------|----|---|---------------------------------------|
| | N | 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) | | | |
|-----------------------------|----|---|---------------------------------------|
| | N | 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
 - $p \leq 0.05$ if treatment difference $\sim 17\%$ or greater
- Placebo response minimized in baseline target wounds size $\geq 10 \text{ cm}^2$
- 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

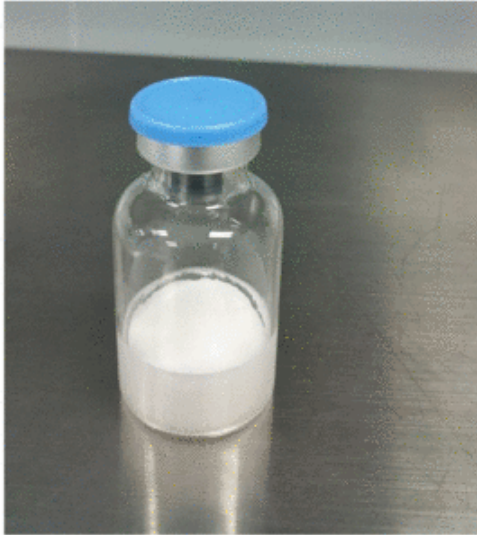
- ROW regulatory path based on EMA and FDA submissions

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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



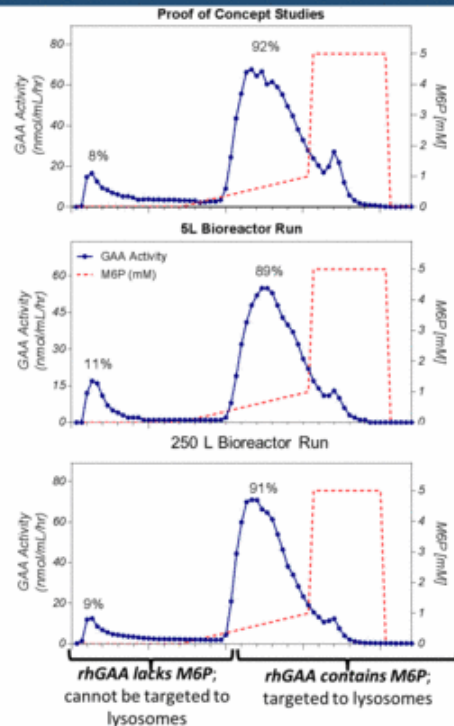
- Master cell banking in 2013
- Cell line scaled to 250 L in 2014
- GMP batches completed 2Q15-3Q15 to initiate upcoming clinical study

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

CI-MPR Receptor Chromatography

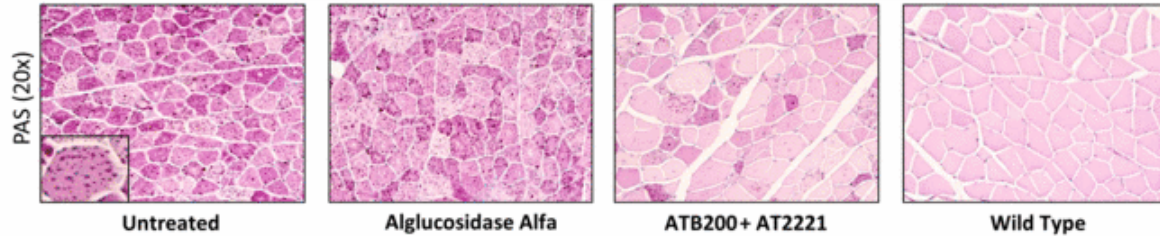
- Proprietary cell line produces a well phosphorylated rhGAA
- Current manufacturing process produces ATB200 with naturally high M6P content
- Cell line and process scaled up from 2 L to 250L bioreactors while maintaining all critical quality attributes to enable efficient drug targeting



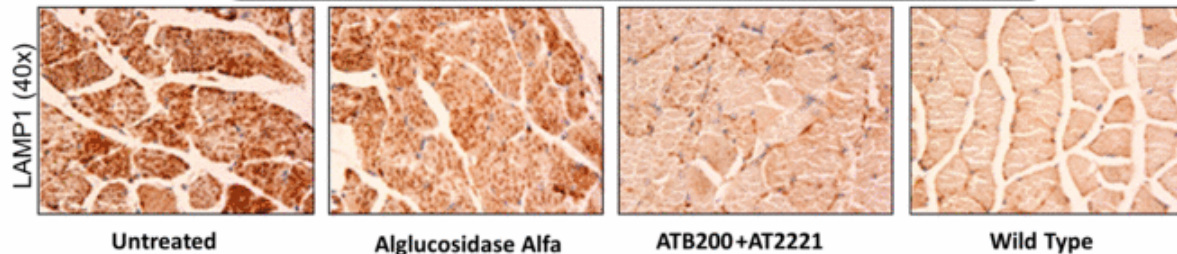
ATB200 + Chaperone Preclinical Proof-of-Concept

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

PAS-glycogen staining in Quadriceps



LAMP1 Immunohistochemical staining in Soleus



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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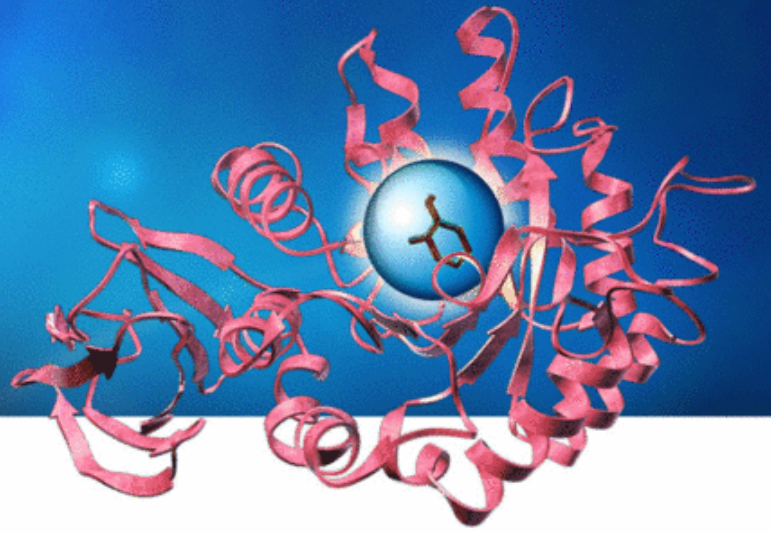
3Q15 Financial Summary

Cash Position Provides Runway Under Current Operating Plan Into 1H17

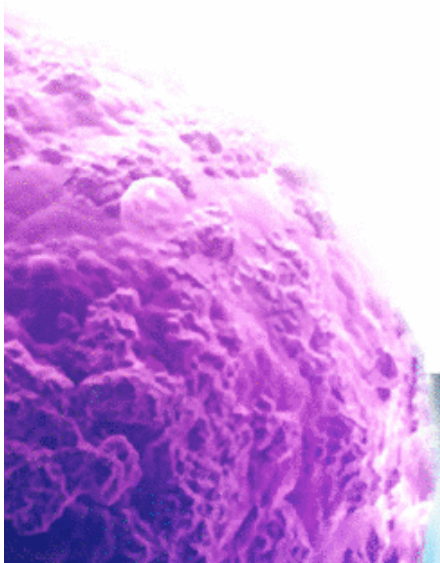
| Financial Position | September 30, 2015 |
|------------------------------------|---------------------------|
| Current Cash: | \$251.9M |
| Anticipated Year-end Cash Balance: | \$200-\$225M |
| Cash Runway: | 1H17 |
| Capitalization | |
| Shares Outstanding: | 124,617,490 |

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)

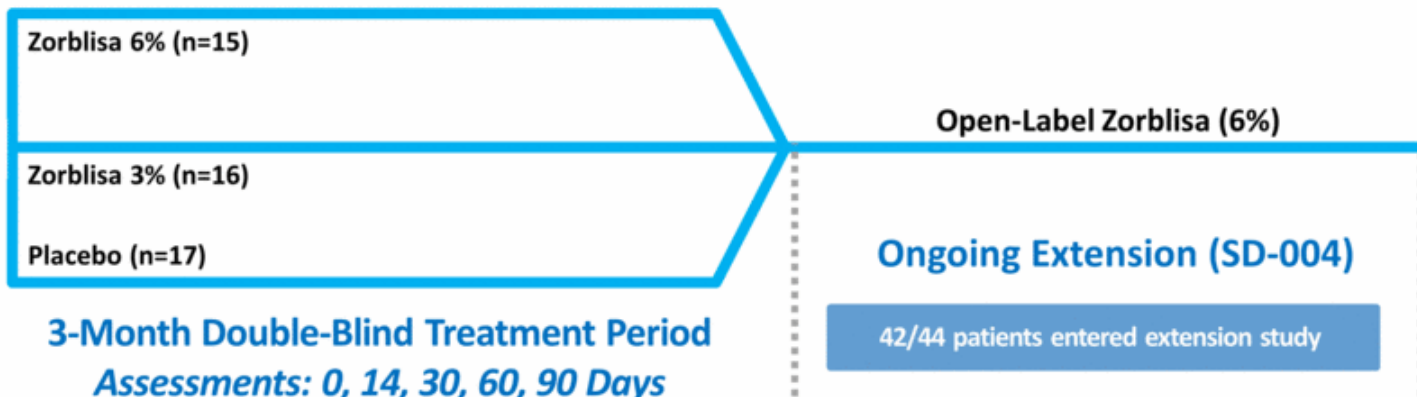
Rare, Devastating, Connective Tissue Disorder with No Approved Treatments



- 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 **diagnosed** patients in major global regions

Phase 2b (Study 003) Design

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



Zorblisa 6% (n=15)

Zorblisa 3% (n=16)

Placebo (n=17)

3-Month Double-Blind Treatment Period
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

Open-Label Zorblisa (6%)

Ongoing Extension (SD-004)

42/44 patients entered extension study

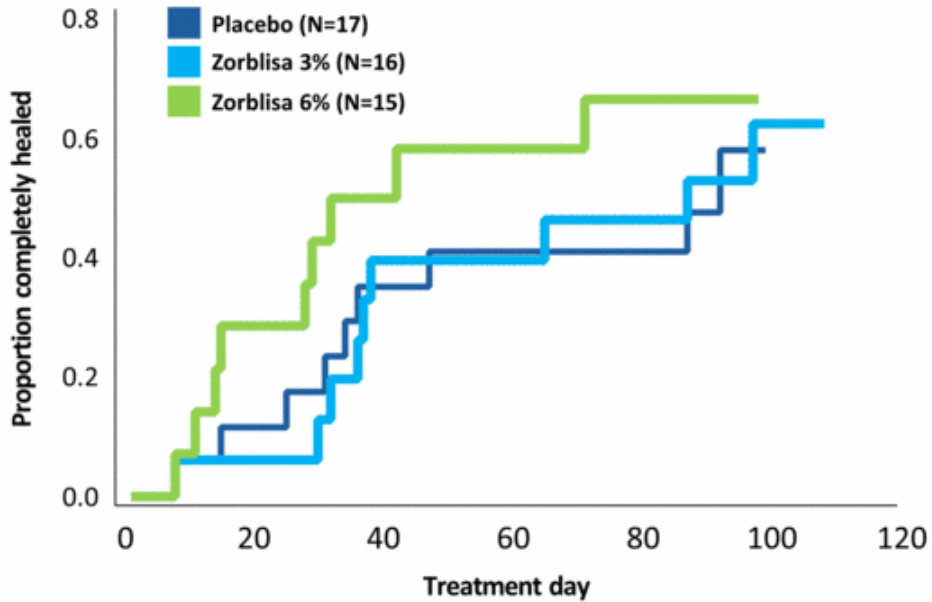
**Initial Disease Severity: Mean target lesion size (cm²) 14.0 (range 5-39); mean lesional BSA: 19.4% (range 0.4-48%); mean wound age (days): 182 (range 21-1,639)*
EB Subtypes enrolled: Simplex (n=11), Recessive Dystrophic (n=29), and Junctional (n=8)

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

Proportion with Complete Target Wound Closure Over 3 Months

(ITT population)



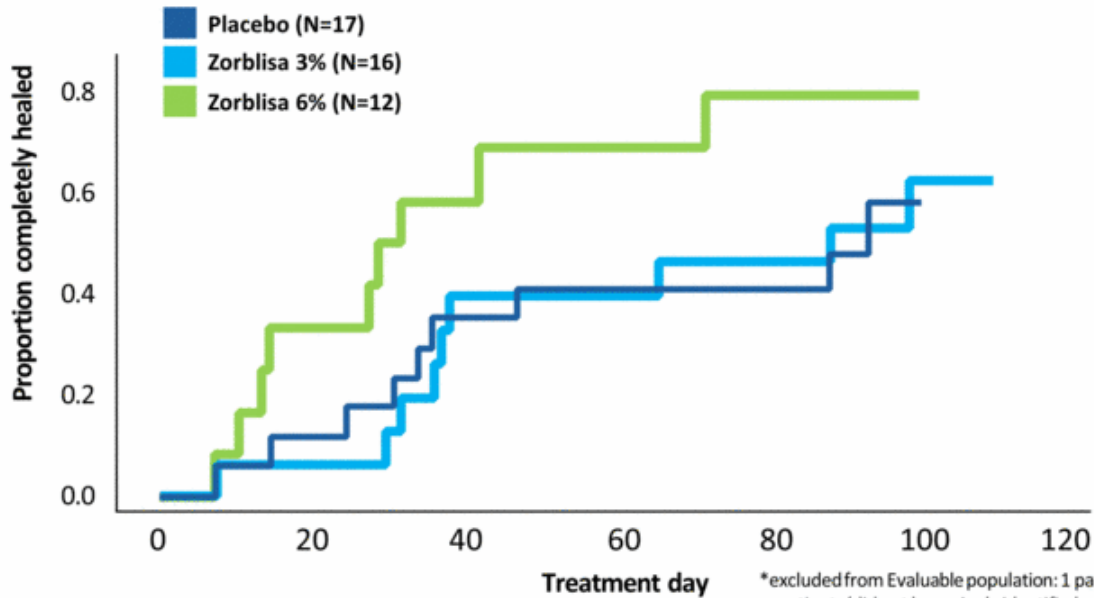
Phase 2b (Study 003) Efficacy Results

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

(Evaluable population only)*



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

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

Optional Extension (SD-006)

Open-Label Zorblisa (6%)

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

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 (\geq 21 days), size \geq 10 cm²

Secondary Endpoints

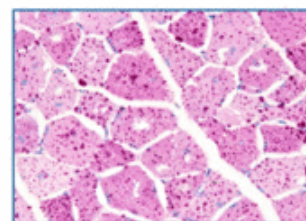
- Time to target wound closure; Change in Body Surface Area (BSA) of lesions and blisters; itching; pain

Pompe Disease Overview

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

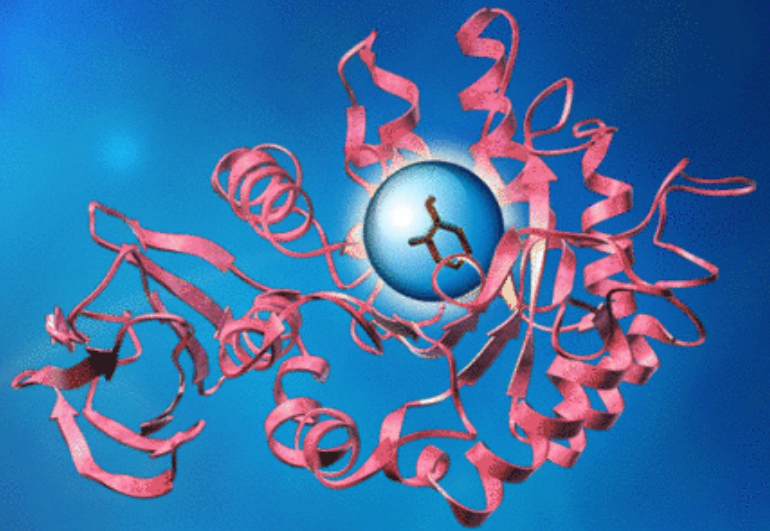


- Deficiency of GAA leading to glycogen accumulation
- Age of onset ranges from infancy to adulthood
- Symptoms include muscle weakness, respiratory failure and cardiomyopathy
- Respiratory and cardiac failure are leading causes of morbidity and mortality
- Incidence 1:28,000¹



Elevated Glycogen
in Muscle





***3Q15 Corporate and Program
Highlights and Financial Results***

November 3, 2015

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