

**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 7, 2016**

**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))

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**Item 2.02. Results of Operations and Financial Condition.**

On November 7, 2016, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the third quarter ended September 30, 2016. 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 7, 2016 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.

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**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.

Date: November 7, 2016

By: /s/ ELLEN S. ROSENBERG  
Ellen S. Rosenberg  
General Counsel and Corporate Secretary

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**EXHIBIT INDEX**

<u>Exhibit No.</u>	<u>Description</u>
99.1	Press Release dated November 7, 2016
99.2	November 7, 2016 Conference Call Presentation Materials

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**Amicus Therapeutics Announces Third Quarter 2016  
Financial Results and Corporate Updates**

***Strong Momentum for Galafold Launch in Europe and Further Global Regulatory Submissions***

***Initial Data from Pompe Phase 1/2 Clinical Study on Track for 4Q16***

**CRANBURY, NJ, November 7, 2016** — Amicus Therapeutics (Nasdaq: FOLD), a global biotechnology company at the forefront of therapies for rare and orphan diseases, today announced financial results for the third quarter ended September 30, 2016. The Company also provided program updates and reiterated full-year 2016 net cash spend guidance.

“During the third quarter we continued to execute toward our vision to build a leading global biotechnology company delivering meaningful benefits to people living with devastating rare diseases,” stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. “We are most pleased with the continued strong momentum in the very early stages of our product launch in Europe. Fifty patients, most of whom have switched from existing approved ERT products, have been prescribed Galafold as of the end of October. The vast majority of these new Galafold patients are in Germany, which is the only nation in the EU where Galafold is fully commercially available. It is wonderful to be able to begin to offer a new therapeutic option to Fabry patients and physicians with our oral, precision small molecule medicine, Galafold. We also remain sharply focused on additional key strategic priorities, including: 1) further global regulatory submissions for migalastat, including a Japanese NDA and clarity on the optimal U.S. regulatory pathway; 2) the advancement of our clinical programs in Pompe and epidermolysis bullosa (EB); 3) a strong balance sheet, and 4) the expansion of our biologics pipeline. We continue to believe that we have one of the best portfolios within the rare and orphan diseases that is uniquely differentiated by our strong science, novel technology platforms, and our incorporation of the patient’s perspective at every stage of the drug development process.”

**Third Quarter 2016 Financial Results**

- Total product revenue in the third quarter of 2016 was approximately \$2.1 million, which represents commercial sales of Galafold (migalastat) in Germany as well as reimbursed Expanded Access Programs (EAPs) in two countries.
- Cash, cash equivalents, and marketable securities totaled \$212.4 million at September 30, 2016 compared to \$214.2 million at June 30, 2016.
- Total operating expenses in the third quarter of 2016 increased to \$46.7 million compared to \$38.0 million for the third quarter 2015 primarily due to increases in commercial costs for the Fabry monotherapy program and the addition of the Phase 3 SD-101 program for EB.
- Net loss was \$46.7 million, or \$0.33 per share in the third quarter of 2016, compared to a net loss of \$37.8 million, or \$0.32 per share, for the third quarter of 2015.

**2016 Financial Guidance**

Cash, cash equivalents, and marketable securities totaled \$212.4 million at September 30, 2016. As previously announced, the Company strengthened the balance sheet during the third quarter of 2016 with \$39.3 million in net proceeds through the at-the-market (ATM) facility and has raised the full \$100 million allotted for the ATM facility. The Company expects to remain within the original 2016 net cash spend guidance of between \$135 million and \$155 million.

**Program Highlights**

**Migalastat for Fabry Disease**

Migalastat is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic mutations. As previously announced, the European Commission has granted full approval for migalastat, under the trade name Galafold™, as a first line therapy for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (alpha-galactosidase A deficiency) and who have an amenable mutation.

**International Launch and Expanded Access Programs (EAP):**

- 50 patients (naïve and ERT-switch) on reimbursed Galafold as of October 31, 2016
- 5 countries with reimbursement (commercial or EAP)
- Reimbursement dossiers submitted and pricing discussions are now underway in 15 countries.

**Regulatory Updates:**

- Swissmedic in Switzerland approved Galafold for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease and who have an amenable mutation
- The Committee for Medicinal Products for Human Use (CHMP) agreed to 44 new amenable mutations and the EU label is being updated to include a total of 313 amenable mutations

- Regulatory submissions completed in six additional territories outside the EU

#### Anticipated Upcoming Fabry Disease Program Milestones:

- EU commercial reimbursement and EAP in additional territories
- Regulatory submissions in additional territories that accept the marketing authorization application (MAA) as basis for submission
- U.S. regulatory update on optimal filing pathway for migalastat anticipated by year-end
- Fabry ERT cell line development and program update
- Japanese regulatory submission (J-NDA) targeted for 1H17 on accelerated timeline

#### **ATB200/AT2221 for Pompe Disease**

Patient dosing is underway in a global clinical study (ATB200-02) to investigate ATB200/AT2221, a novel treatment paradigm that consists of ATB200, a uniquely engineered recombinant human acid alpha-glucosidase (rhGAA) enzyme with an optimized carbohydrate structure to enhance uptake, co-administered with AT2221, a pharmacological chaperone. The study is enrolling 3 cohorts of patients, including ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3). The Data Safety Monitoring Board (DSMB) for ATB200-02 completed a safety review of initial patients in Cohort 1 during the third quarter. Following this positive DSMB safety review, enrollment of Cohorts 2-3 is currently underway.

#### Anticipated Upcoming Pompe Disease Program Milestones:

- Data from clinical study ATB200-02 in first four ambulatory ERT-switch patients on track by year-end 2016
- Additional ATB200-02 study data in naïve and non-ambulatory patients, as well as initial extension-phase data on ambulatory ERT-switch patients, throughout 1H17

#### **SD-101 for Epidermolysis Bullosa (EB)**

SD-101 is a novel, late-stage, proprietary topical treatment and potential first-to-market therapy for EB. SD-101 is currently being investigated in a registration-directed Phase 3 study (ESSENCE, also known as SD-005) to support global regulatory submissions. The ESSENCE study is enrolling patients who have a documented diagnosis of Simplex, Recessive Dystrophic, or Junctional non-Herlitz EB. All (100%) patients completing the primary treatment period of the Phase 3 study have elected to continue in the open-label extension study. A total of 28 sites in the U.S., Europe, and Australia are currently open for enrollment.

SD-101 was granted FDA Breakthrough Therapy designation in 2013 based on results from a Phase 2a study for the treatment of lesions in patients suffering with EB. SD-101 is the first-ever treatment in clinical studies to show improvements in wound closure across all major EB types.

#### Anticipated EB Program Milestones:

- Top-line data from the Phase 3 ESSENCE study of SD-101 (1H17)

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#### **Conference Call and Webcast**

Amicus Therapeutics will host a conference call and audio webcast today, November 7, 2016 at 8:30 a.m. ET to discuss third quarter 2016 financial results and corporate updates. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international).

An audio webcast and slides can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at <http://ir.amicusrx.com/events.cfm>, 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 11:30 a.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); participant code 10205490.

#### **About Galafold™ and Amenable Mutations**

Galafold™ (migalastat) is a first-in-class chaperone therapy approved in the EU as a monotherapy for Fabry disease in patients with amenable mutations. Galafold works by stabilizing the body's own dysfunctional enzyme, so it can clear the accumulation of disease substrate in patients who have amenable mutations. A proprietary *in vitro* assay (Galafold Amenability Assay) was used to classify more than 800 known GLA mutations as "amenable" or "not amenable" to treatment with Galafold. The current label includes all 269 GLA mutations that have been identified and determined to be amenable based on the Galafold Amenability Assay, which represent between 35% and 50% of the currently diagnosed Fabry population.

Healthcare providers in the EU may access the website [www.galafoldamenabilitytable.com](http://www.galafoldamenabilitytable.com) to quickly and accurately identify which mutations are categorized as "amenable" or "not amenable" to Galafold. Amicus expects to submit updates to the label as additional GLA mutations are identified and tested in the Galafold Amenability Assay.

#### **Important Safety Information**

Treatment with GALAFOLD should be initiated and supervised by specialists experienced in the diagnosis and treatment of Fabry disease. GALAFOLD is not recommended for use in patients with a nonamenable mutation.

- GALAFOLD is not intended for concomitant use with enzyme replacement therapy.

- GALAFOLD is not recommended for use in patients with Fabry disease who have severe renal impairment (<30 mL/min/1.73 m<sup>2</sup>). The safety and efficacy of GALAFOLD in children 0—15 years of age have not yet been established.
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.
- While taking GALAFOLD, effective birth control should be used. It is not known whether GALAFOLD is excreted in human milk.
- Contraindications to GALAFOLD include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on GALAFOLD or switched to GALAFOLD.
- OVERDOSE: General medical care is recommended in the case of GALAFOLD overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received GALAFOLD. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at [www.ema.europa.eu](http://www.ema.europa.eu).

### **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) and biologic products for Fabry disease, Pompe disease, and other rare and devastating diseases.

### **Forward-Looking Statements**

This press release and conference call slides contain “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

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for the Company. The inclusion of forward-looking statements 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 press release 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. 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, 2015 as well as our 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 news release to reflect events or circumstances after the date hereof.

### **CONTACTS:**

#### **Investors/Media:**

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**Consolidated Statements of Operations**  
(Unaudited)  
(in thousands, except share and per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Net product sales	\$ 2,127	\$ —	\$ 2,127	\$ —
Cost of goods sold	344	—	344	—
Gross profit	1,783	—	1,783	—
Operating Expenses:				
Research and development	32,457	20,971	74,163	54,318
Selling, general and administrative	17,469	15,372	52,470	30,077
Changes in fair value of contingent consideration payable	(4,110)	1,300	9,228	2,400
Restructuring charges	11	7	69	44
Loss on extinguishment of debt	—	—	—	952
Depreciation	896	395	2,336	1,256
Total operating expenses	46,723	38,045	138,266	89,047
Loss from operations	(44,940)	(38,045)	(136,483)	(89,047)
Other income (expenses):				
Interest income	460	316	1,098	645
Interest expense	(1,517)	(17)	(3,517)	(727)
Other expense	(910)	(54)	(3,199)	(93)
Loss before income tax benefit	(46,907)	(37,800)	(142,101)	(89,222)
Income tax benefit	253	—	706	—
Net loss	(46,654)	(37,800)	(141,395)	(89,222)
Net loss per common share — basic and diluted	\$ (0.33)	\$ (0.32)	\$ (1.07)	\$ (0.85)
Weighted-average common shares outstanding — basic and diluted	140,656,109	118,724,882	131,675,690	104,885,956

**Table 2**

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

	September 30, 2016	December 31, 2015
<b>Assets:</b>		
Current assets:		
Cash and cash equivalents	\$ 33,115	\$ 69,485
Investments in marketable securities	179,284	144,548
Accounts receivable	864	—
Inventories	3,251	—
Prepaid expenses and other current assets	5,198	2,568
Total current assets	221,712	216,601
Property and equipment, less accumulated depreciation of \$15,181 and \$13,353 at September 30, 2016 and December 31, 2015, respectively	10,183	6,178
In-process research & development	486,700	486,700
Goodwill	197,797	197,797
Other non-current assets	1,788	1,108
<b>Total Assets</b>	<b>\$ 918,180</b>	<b>\$ 908,384</b>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable and accrued expenses	\$ 29,013	\$ 32,216
Contingent consideration payable, current portion	55,992	41,400
Other current liabilities	607	—
Total current liabilities	85,612	73,616
Deferred reimbursements	35,756	35,756
Due to related party	44,047	41,601
Unsecured notes payable	21,977	—
Contingent consideration payable, less current portion	216,198	232,677
Deferred tax liability	176,219	176,219
Other non-current liabilities	1,816	681
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$.01 par value, 250,000,000 authorized, 142,273,085 shares issued and outstanding at	1,478	1,306

September 30, 2016, 250,000,000 shares authorized, 125,027,034 shares issued and outstanding at  
December 31, 2015

Additional paid-in capital	1,038,613	917,454
Accumulated other comprehensive loss:		
Foreign currency translation adjustment, less tax benefit of \$706 at September 30, 2016	1,062	—
Unrealized gain/ (loss) on available-for securities	287	(115)
Warrants	16,076	8,755
Accumulated deficit	(720,961)	(579,566)
Total stockholders' equity	<u>336,555</u>	<u>347,834</u>
<b>Total Liabilities and Stockholders' Equity</b>	<b><u>\$ 918,180</u></b>	<b><u>\$ 908,384</u></b>

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# 3Q16 Financial Results and Program Updates

November 7, 2016



## 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. The inclusion of forward-looking statements 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 press release 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. 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, 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 news release to reflect events or circumstances after the date hereof.*

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## 2016: Significant Progress with Key Strategic Priorities

We Remain Sharply Focused on FIVE Strategic Priorities as We Continue to Build a Leading Global Biotechnology Company Focused on Rare and Devastating Diseases

Galafold International Launch

Migalastat Regulatory Approvals

Pompe & EB Clinical Studies

Balance Sheet Strength

Pipeline



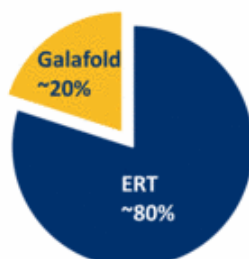
# Galafold™ (Migalastat) Precision Medicine for Fabry Disease

International Launch Underway

# International Launch Update

Successful First Full Quarter Driven by German Launch  
with ERT-Switch and Naïve Patients on Galafold

## German Launch Update



Eligible ERT-Switch Patients<sup>1</sup>

### IMPORTANT EARLY INDICATORS IN GERMANY

- Vast majority switch patients
- ~20% of eligible switch patients now on Galafold<sup>1</sup>
- All newly experienced patients & physicians

50

Patients (Switch & Naïve) on Reimbursed Galafold (10/31/16)

15

Countries with Active Pricing Discussions

5

Countries with Reimbursement (Commercial and EAP)

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

## EU Launch Update

**Galafold Early Launch Strength in EU Market Representing 34% of FY15 ERT Global Sales (\$1.2B) – Focusing on Patient Access and Reimbursement**

### GERMANY

Diagnosed patients : ~1000 (~50% untreated)  
Galafold launched – initial patients on treatment

### FRANCE

ERT-treated patients : ~375 patients  
Multiple patients treated under ATU

### UNITED KINGDOM

ERT-treated patients: ~450  
Highly Specialised Technology (HST)

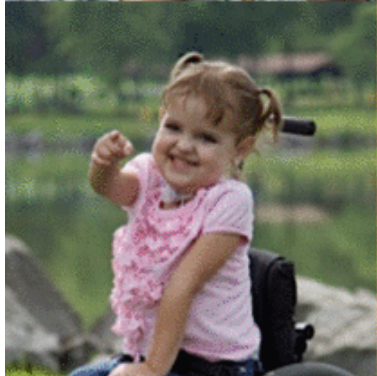
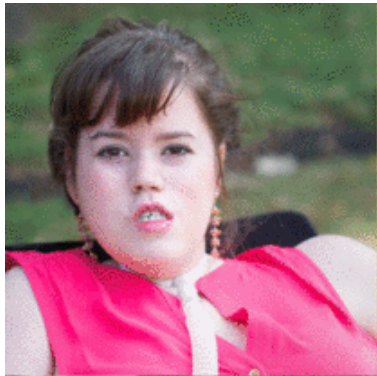




# Global Regulatory Strategy

**Prioritizing Global Regulatory Submissions in Key Markets (US and Japan) with Additional Submissions Completed or Planned Based on EU Approval (MAA)**





# ATB200 Novel ERT for Pompe Disease

A Proprietary, Clinical-Stage Biologics Program

# Pompe ERT - 3 Challenges

## Amicus Technology Platforms with Potential to Address Challenges with Existing Pompe ERT

### Activity/ Stability

Rapid denaturation of ERT in pH of blood<sup>1</sup>

Protein Aggregation



### Tolerability / Immunogenicity

Infusion-associated reactions in >50% of late-onset patients<sup>3</sup>

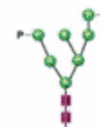
Antibody titers shown to affect treatment outcomes<sup>4,5</sup>



### Uptake/ Targeting

Low M6P receptor uptake into skeletal muscle<sup>2</sup>

Vast majority of rhGAA not delivered to lysosomes<sup>2</sup>



Uniquely Engineered rhGAA Optimized M6P & Carbohydrates

<sup>1</sup>Khanna et al., PLoS ONE, 2012; <sup>2</sup>Zhu et al., Amer. Soc. Gene Therapy, 2009 June; <sup>3</sup>Banati et al., Muscle Nerve, 2011 Dec.; <sup>4</sup>Banugaria et al., Gen. Med., 2011 Aug.; <sup>5</sup>de Vries et al., Mol Genet Metab., 2010 Dec.



# Pompe Clinical Study ATB200-02 Data Cascade

A Cascade of Data Points from 4Q16 through 2017 Offer Clear Parameters to Define Success and Differentiate ATB200/AT2221

## Pompe Data Cascade 4Q16 Through 2017

Data in initial ambulatory ERT-switch patients (Cohort 1, n=4)

Additional data & initial extension data in Cohort 1

Data in non-ambulatory ERT-switch patients (Cohort 2)

Data in ERT-naïve patients (Cohort 3)

Additional extension study data (all Cohorts)

### 18-WEEK DATA

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

### EXTENSION DATA

- Motor/pulmonary function

# Pompe Clinical Study ATB200-02 Parameters for Success

## Key Questions to Determine Potential for ATB200/AT2221 to Address 3 Major ERT Challenges in Initial 18-Week Treatment Period

### CHALLENGES :

### KEY QUESTIONS :

#### Safety

? Do patients safely switch from standard of care to ATB200/AT2221?

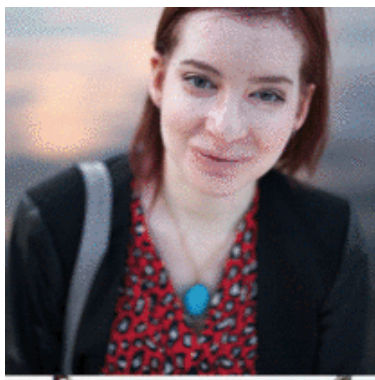
? Do patients tolerate ATB200/AT2221 with limited infusion-associated reactions?

#### Exposure, Targeting & Uptake

? Is PK profile of ATB200/AT2221 differentiated and in optimal range consistent with preclinical studies?

#### Tolerability & Immunogenicity

? Do antibodies in switch patients remain the same on ATB200/AT2221?



# SD-101 for Epidermolysis Bullosa

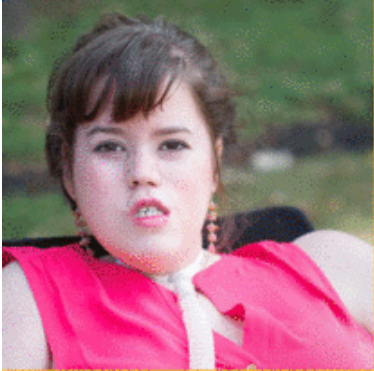
## EB Program Update - Phase 3 ESSENCE Study (SD-005)

Significant Momentum for Ongoing Study with Data on Track for 1H17



### PHASE 3 ESSENCE STUDY STATUS

- 28 sites activated as of October 31, 2016
- 100% conversion to extension study (SD-006)
- SAP submitted to FDA for finalization
- Top-line Phase 3 data anticipated 1H17



# Financial Summary

## 3Q16 Select Financial Results

First-Ever Quarter to Report Product Revenue of \$2.1M from Sales of Galafold

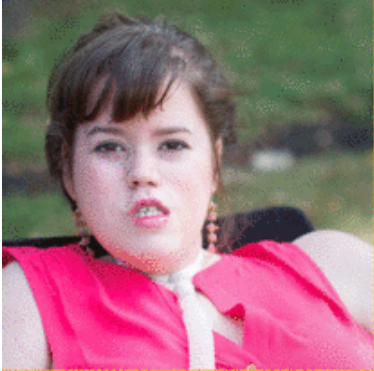
(\$000s) except per share data	September 30, 2016	September 30, 2015
Product revenue	2,127	-
R&D Expense	32,457	20,971
SG&A Expense	17,469	15,372
Net Loss	(46,654)	(37,800)
Net Loss Per Share	(0.33)	(0.32)



## Strong Balance Sheet

**Balance Sheet Strengthened with ~\$39M in Equity  
Since June 30 with Cash Runway Through Late 2017**

Financial Position	September 30, 2016
Cash:	\$212.4M
Debt	\$80.0M (\$66.0M net of discount for warrants issued)
FY16 Net Cash Spend Guidance:	\$135-\$155M (maintained)
Cash Runway	Late 2017
Full Allotment Raised in ATM (average price per share: \$6.67)	\$100M (\$61.7M in 2Q; \$39.3M in 3Q)
Capitalization	September 30, 2016
Shares Outstanding	142,273,085



## Closing Remarks



## Key Drivers of Value

### 3 Novel Product Candidates Each with \$500M to \$1B+ Market Potential

#### Fabry

- Galafold Precision Medicine (Small Molecule)
- EU Full Approval
- Launched in Germany (May 30, 2016)
- U.S. regulatory update on track for 4Q16

#### Epidermolysis Bullosa (EB)

- Phase 3 Novel Topical Medicine (SD-101)
- U.S. Breakthrough Therapy Designation
- Rolling NDA
- Phase 3 Data Targeted in 1H17

#### Pompe

- Novel ERT + Chaperone Treatment Paradigm
- Biologics Manufacturing
- Interim Data Anticipated in 4Q16

**R&D Engine and Continued Business Development Activity**