

**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): **May 9, 2017**

**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))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

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

On May 9, 2017, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the first quarter ended March 31, 2017. A copy of this press release is attached hereto as Exhibit 99.1. The Company will also host a conference call and webcast on May 9, 2017 to discuss its first 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: May 9, 2017

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 May 9, 2017
99.2	May 9, 2017 Conference Call Presentation Materials

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**Amicus Therapeutics Announces First Quarter 2017  
Financial Results and Corporate Updates**

*100+ Fabry Disease Patients Now on Reimbursed Galafold (migalastat) in the EU*

*On Target to Reach 300 Patients on Galafold by Year-End 2017*

*Additional Important Phase 1/2 Pompe Clinical Data Expected in 2Q17 and 3Q17*

*Phase 3 EB Topline Data on Track for 3Q17*

**CRANBURY, NJ, May 9, 2017** — Amicus Therapeutics (Nasdaq: FOLD), a global biotechnology company at the forefront of therapies for rare and orphan diseases, today announced financial results for the first quarter ended March 31, 2017. The Company also provided program updates and reiterated full-year 2017 financial guidance.

“During the first quarter we continued to advance the international commercial launch of our oral precision medicine Galafold for Fabry disease, as we brought Galafold to more Fabry patients in Germany and other European markets with both new ERT switch patients as well as a notable proportion of ERT-naïve patients,” stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. “We are extremely pleased with the progress of our Galafold launch in terms of patient and physician adoption, as well as pricing and reimbursement. With launch now just initiating this month in the UK, Italy, France, and additional markets, we are confident in achieving our target of 300 patients on Galafold by the end of this year. In addition to the launch, we have also made significant progress on our other four key strategic priorities for 2017: 1) targeting a Japanese new drug application (J-NDA) submission for migalastat in the second quarter, 2) establishing definitive clinical proof of concept for our novel Pompe treatment paradigm ATB200/AT2221, 3) completing our Phase 3 clinical study in patients with epidermolysis bullosa, and 4) maintaining our financial strength. We are particularly looking forward to sharing additional, important Pompe clinical data this quarter. We continue to believe that our Pompe program will be a crucial driver toward our vision of becoming one of the world’s leading global rare disease biotechnology companies.”

**First Quarter 2017 Financial Results**

- Total product revenue in the first quarter 2017 was approximately \$4.2 million, a sequential increase of 50% over total product revenue of \$2.8 million in the fourth quarter 2016. Total product revenue for both periods represents commercial sales of Galafold (migalastat) which commenced in May 2016, as well as reimbursed Expanded Access Programs (EAPs).
- Cash, cash equivalents, and marketable securities totaled \$279.8 million at March 31, 2017 compared to \$330.4 million at December 31, 2016.
- Total operating expenses increased to \$55.4 million compared to \$43.0 million for the first quarter 2016 primarily due to increases in commercial costs of the Fabry monotherapy program and manufacturing scale-up on the Pompe program.
- Net cash spend was \$50.5 million.
- Net loss was \$55.0 million, or \$0.39 per share, compared to a net loss of \$43.7 million, or \$0.35 per share, for the first quarter 2016.

**2017 Financial Guidance**

Cash, cash equivalents, and marketable securities totaled \$279.8 million at March 31, 2017. The Company continues to expect full-year 2017 net operating cash spend of between \$175 million to \$200 million and full-year 2017 total net cash spend (including third-party milestone payments and capital expenditures) of between \$200 million and \$225 million. The current cash position is anticipated to fund ongoing operations into the second half of 2018.

**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 (EC) has granted full approval for migalastat under the trade name

Galafold. The EC approval may serve as the basis for regulatory approvals in more than two-thirds of the global Fabry market that is outside the U.S. The Company has also defined a U.S. pathway to support full approval.

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

- 101 patients (naïve and ERT-switch) on reimbursed Galafold as of April 30, 2017
- 11 countries with reimbursement (commercial or EAP) including top four largest EU markets
- Reimbursement dossiers submitted and pricing discussions are now underway in 12 countries
- Target of 300 patients treated with reimbursed Galafold by year-end 2017

**Regulatory Updates:**

- One additional approval secured outside EU (Switzerland)
- Regulatory submissions completed in six additional territories outside EU

**Anticipated Upcoming Fabry Disease Program Milestones:**

- EU commercial launch in additional countries and EAP in additional territories
- Additional regulatory submissions including a Japanese regulatory submission (J-NDA) (2Q17)
- Fabry ERT cell line development and optimization

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

ATB200/AT2221 is a novel treatment paradigm that consists of ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose-6 phosphate (M6P), to enhance uptake, co-administered with AT2221, a pharmacological chaperone. Positive preliminary data were reported from a global Phase 1/2 clinical study (ATB200-02) to evaluate safety, tolerability, PK, and pharmacodynamics (PD) of ATB200/AT2221. The study is fully enrolled with 3 cohorts of patients, including ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3).

Anticipated Upcoming Pompe Disease Program Milestones:

- Additional ATB200-02 study data (2Q17 and 3Q17)
- Meetings with US and EU regulators

### **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.

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.

EB Phase 3 ESSENCE Study Highlights:

- Enrollment complete and target exceeded with more than 160 patients
- More than 95% of patients completing the primary treatment period have elected to continue in the open-label extension study

Anticipated EB Program Milestones:

- Agreement on final statistical analysis plan (SAP) with U.S. Food and Drug Administration (FDA)
- Top-line Phase 3 data (3Q17)

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### **Conference Call and Webcast**

Amicus Therapeutics will host a conference call and audio webcast today, May 9, 2017 at 8:30 a.m. ET to discuss first quarter 2017 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 can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at <http://ir.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 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 16795077.

### **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 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

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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. 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 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 news release to reflect events or circumstances after the date hereof.

## **CONTACTS:**

### **Investors/Media:**

Amicus Therapeutics  
Sara Pellegrino  
Senior Director, Investor Relations  
spellegrino@amicusrx.com  
(609) 662-5044

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**TABLE 1**

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

	<b>Three Months Ended March 31,</b>	
	<b>2017</b>	<b>2016</b>
<b>Revenue:</b>		
Net product sales	\$ 4,169	\$ —
Cost of goods sold	775	—
<b>Gross Profit</b>	<b>3,394</b>	<b>—</b>
<b>Operating Expenses:</b>		
Research and development	30,876	23,425
Selling, general and administrative	19,132	15,701
Changes in fair value of contingent consideration payable	4,578	3,152
Restructuring charges	—	50
Depreciation	823	673
Total operating expenses	55,409	43,001
Loss from operations	(52,015)	(43,001)
<b>Other income (expenses):</b>		
Interest income	759	307
Interest expense	(4,290)	(945)
Other income (expense)	610	(52)
Loss before income tax benefit	(54,936)	(43,691)
Income tax expense	(56)	—
<b>Net loss attributable to common stockholders</b>	<b>\$ (54,992)</b>	<b>\$ (43,691)</b>
Net loss attributable to common stockholders per common share — basic and diluted	\$ (0.39)	\$ (0.35)
Weighted-average common shares outstanding — basic and diluted	142,770,629	125,178,517

TABLE 2

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

	March 31, 2017	December 31, 2016
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 44,755	\$ 187,026
Investments in marketable securities	235,087	143,325
Accounts receivable	1,875	1,304
Inventories	3,698	3,416
Prepaid expenses and other current assets	10,792	4,993
Total current assets	<u>296,207</u>	<u>340,064</u>
Property and equipment, less accumulated depreciation of \$13,316 and \$12,495 at March 31, 2017 and December 31, 2016, respectively	9,745	9,816
In-process research & development	486,700	486,700
Goodwill	197,797	197,797
Other non-current assets	2,932	2,468
<b>Total Assets</b>	<u>\$ 993,381</u>	<u>\$ 1,036,845</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	\$ 39,645	\$ 41,008
Deferred reimbursements, current portion	13,850	13,850
Contingent consideration payable, current portion	56,101	56,101
Total current liabilities	<u>109,596</u>	<u>110,959</u>
Deferred reimbursements	21,906	21,906
Convertible notes	156,859	154,464
Contingent consideration payable	218,199	213,621
Deferred income taxes	173,820	173,771
Other non-current liability	2,223	1,973
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$0.01 par value, 250,000,000 shares authorized, 142,829,530 and 142,691,986 shares issued and outstanding at March 31, 2017 and December 31, 2016, respectively	1,482	1,480
Additional paid-in capital	1,126,148	1,120,156
Accumulated other comprehensive loss:		
Foreign currency translation adjustment, less tax expense of \$1,293 at March 31, 2017 and at December 31, 2016	1,487	1,945
Unrealized gain/ (loss) on available-for securities	185	102
Warrants	16,076	16,076
Accumulated deficit	(834,600)	(779,608)
Total stockholders' equity	<u>310,778</u>	<u>360,151</u>
<b>Total Liabilities and Stockholders' Equity</b>	<u>\$ 993,381</u>	<u>\$ 1,036,845</u>



# 1Q17 Financial Results & Corporate Updates

## Conference Call & Webcast

May 9, 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. 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 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. 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 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 news release to reflect events or circumstances after the date hereof.*



## 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**



# Galafold™ (Migalastat) Precision Medicine for Fabry Disease

Continue Launch Execution and Geographic Expansion

## 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)

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Countries with available reimbursement\*

12

Countries with pricing discussions ongoing

27

Countries with Amicus footprint



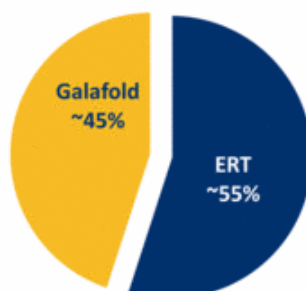
\*Commercial and Expanded Access Programs (EAPs)

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

### Germany is an Important Indicator for EU Launch Success



Current  
Approximate  
Market Share\*



#### 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

## 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

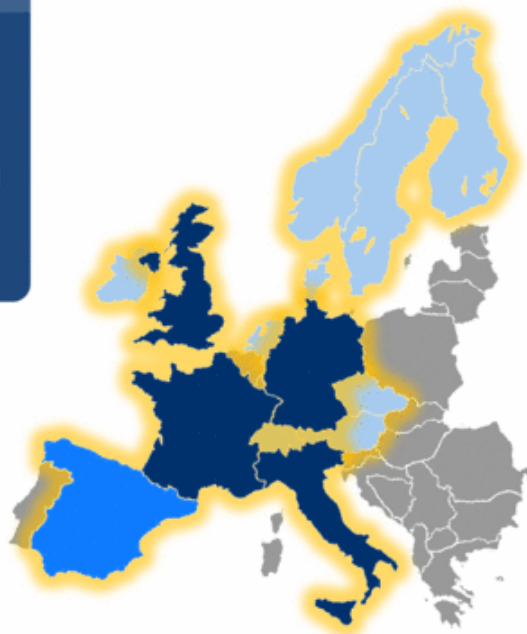
\*\* Evidence-based recommendations on migalastat (Galafold) for treating Fabry disease in people over 16 - [www.nice.org.uk/guidance/hst4](http://www.nice.org.uk/guidance/hst4)

# 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

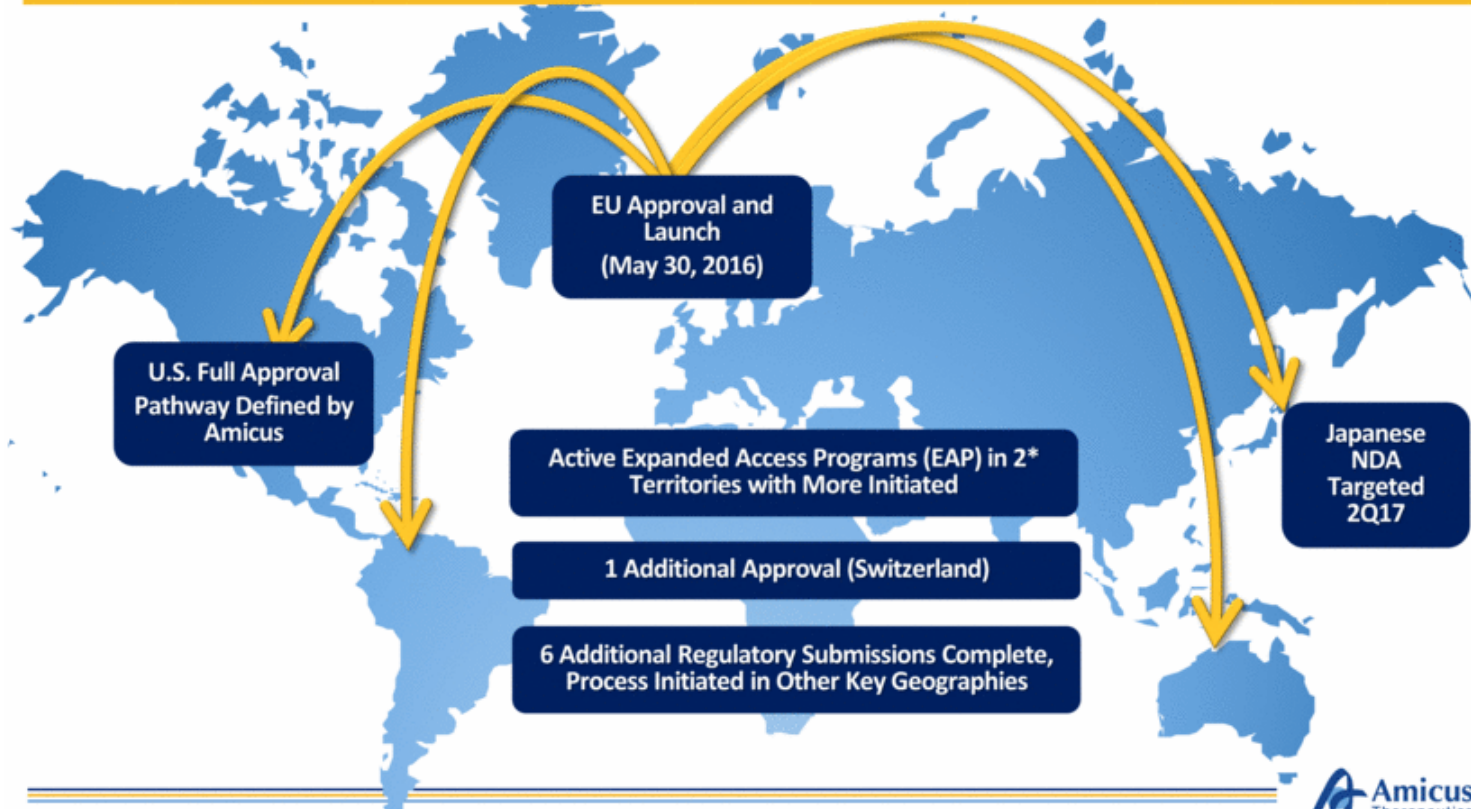


### INVEST IN KEY MID-SIZED EU COUNTRIES AND SELECT EAP OPPORTUNITIES

- Austria, Nordics, Netherlands, Belgium, etc.
- ~10% of EU market value
- Selectively invest in key EAP markets

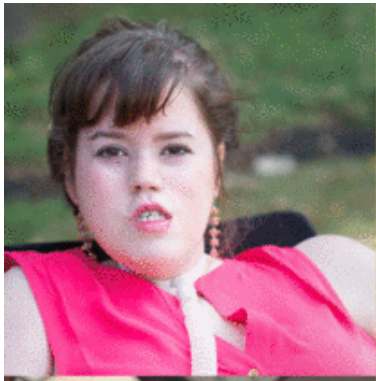
# Global Regulatory Strategy to Reach More Patients

EU Approval is Gateway to ~75% of Global ERT Market



\* Two EAPs converted to commercial reimbursement





# ATB200 Novel ERT for Pompe Disease

Establishing Human Proof of Concept and Validating  
Biologics Platform in 2017

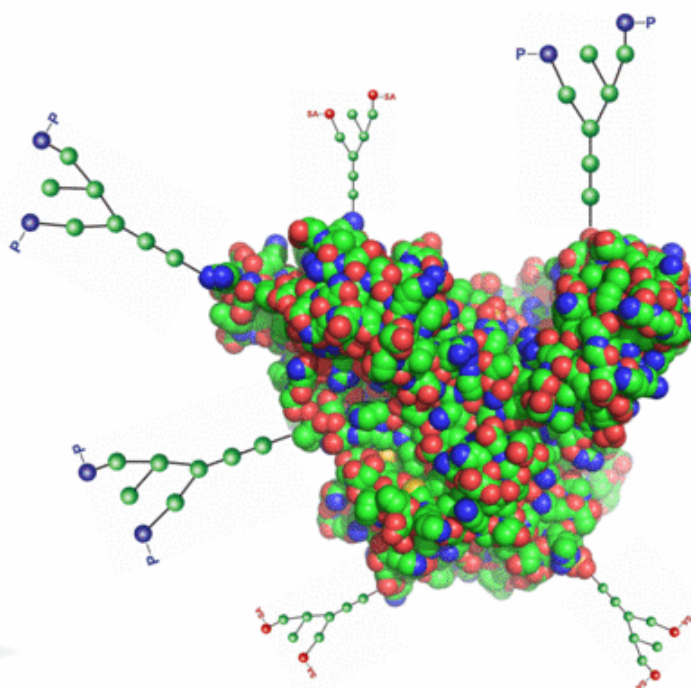


# ATB200 + Chaperone: A Highly Differentiated Approach

## Novel Pompe Treatment Paradigm with Three Key Differentiators

**ATB200  
(Novel ERT)**

**Chaperone  
addition**



**Optimized  
mixture of  
glycans**

**High levels of  
M6P and bis  
M6P**

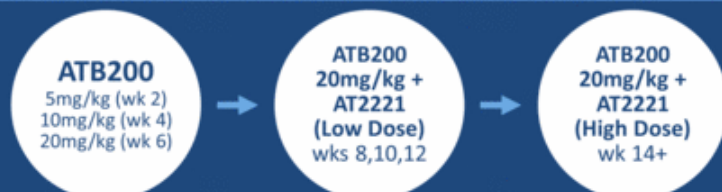
\*Artist rendering, not actual product image

# Phase 1/2 ATB200-02 Study Design

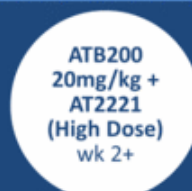
Phase 1/2 Clinical Study to Evaluate Safety, Tolerability, Pharmacokinetics (PK), and Pharmacodynamics (PD) of ATB200 + Chaperone (ATB200/AT2221)

## 18-Week Primary Treatment Period with Long-Term Extension (n=20)

Cohort 1 (Ambulatory ERT-Switch, n=11)



Cohort 2 (Non-Ambulatory ERT-Switch, n=4) & Cohort 3 (ERT-Naïve, n=5)



### Assessments:

- Plasma PK
- Safety/Tolerability
- Infusion-Associated Reactions
- Antibody & Cytokine Levels
- Pharmacodynamics
- Efficacy (Long-Term Extension)

## Preliminary Clinical Data Summary (as of February 2017)

### ATB200/AT2221 Demonstrates Promising Preliminary Results in Initial ERT-Switch and Naïve Patients at the Targeted Therapeutic Dose

- **Safety (N=13)\***
  - No serious adverse events (SAEs) related to ATB200/AT2221
  - AEs were generally mild and transient
- **Tolerability**
  - No infusion-associated reactions following 150+ infusions in all patients enrolled to date
- **PK (N=10)\*\***
  - Clinical PK profile as predicted consistent with previously reported preclinical data
- **Biomarkers of muscle damage (CK, AST, ALT) and substrate (urine Hex4) (N=10)\*\***
  - Decrease or normalization of muscle injury biomarkers in a majority of patients
  - Decreases in urine Hex4 in all patients
  - Improvement in all biomarkers suggests positive effect of ATB200/AT2221 on muscle cells

\*N=10 from Cohort 1 (Ambulatory ERT-Switch); N=1 from Cohort 2 (Non-Ambulatory ERT-Switch); N=2 from Cohort 3 (Naïve)

\*\*N=8 from Cohort 1 & N=2 from Cohort 3

# Pompe Clinical Study ATB200-02 Data Cascade

A Cascade of Additional Data Points on Track for 2Q17 and 3Q17  
to Demonstrate Proof of Concept

## Pompe Milestones in 2017

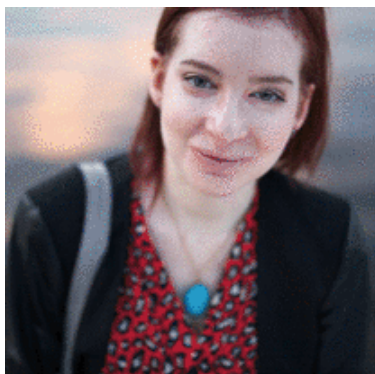


### 18-WEEK DATA

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

### EXTENSION DATA

- Motor/pulmonary function



# SD-101 for Epidermolysis Bullosa

Potential First-in-Class Treatment  
with Phase 3 Data Anticipated 3Q17

## 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 up to 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



# Financial Summary

# 1Q17 Select Financial Results

Continue to Focus on Revenue of \$4.2M from Sales of Galafold

	March 31, 2017	March 31, 2016
Product revenue	\$4.2m	-
R&D Expense	\$30.9m	\$23.4m
SG&A Expense	\$19.1m	\$15.7m
Net Loss	(\$55.0)	(\$43.7)
Net Loss Per Share	(\$0.39)	(\$0.35)

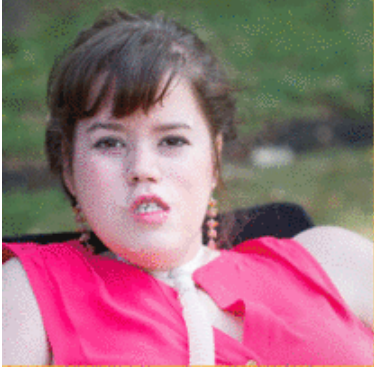


## Financial Summary & Guidance

**Strong Balance Sheet with \$279.8M Cash at 3/31/17 and Cash Runway Into 2H18**

<b>Financial Position</b>	<b>March 31, 2017</b>
Cash	\$279.8M
Debt	\$250M
FY17 Net Operating Cash Flow Guidance	\$175-\$200M
FY17 Net Cash Spend Guidance*	\$200-\$225M
Cash Runway	2H18
<b>Capitalization</b>	<b>March 31, 2017</b>
Shares Outstanding	142,829,530

\*Includes third party milestone payments and capital expenditures



## Closing Remarks

## 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)

# Building a Top Global Biotech in Devastating Rare Diseases



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**

Thank You

