

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): **March 1, 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))

Item 2.02. Results of Operations and Financial Condition.

On March 1, 2017, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the full year ended December 31, 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 March 1, 2017 to discuss its full year 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: March 1, 2017

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

3

EXHIBIT INDEX

| <u>Exhibit No.</u> | <u>Description</u> |
|--------------------|--|
| 99.1 | Press Release dated March 1, 2017 |
| 99.2 | March 1, 2017 Conference Call Presentation Materials |

4



**Amicus Therapeutics Announces Full-Year 2016
Financial Results and Corporate Updates**

Growing Momentum for EU Galafold (Migalastat) Launch for Fabry Disease Tracking Toward 300 Patients by Year-End 2017

Target Enrollment Achieved in Phase 1/2 Pompe Study — Additional Data Expected in 2Q17 and 3Q17

Phase 3 EB Program Remains on Track for Topline Data in Mid-2017

CRANBURY, NJ, March 1, 2017 — Amicus Therapeutics (Nasdaq: FOLD), a biotechnology company at the forefront of therapies for rare and orphan diseases, today announced financial results for the full year ended December 31, 2016. The Company also provided program updates and reiterated full-year 2017 financial guidance.

“Throughout 2016 we made significant progress with the international launch of our first commercial product Galafold and continued to advance and expand our robust pipeline of first- and/or best-in-class medicines for people living with devastating rare diseases,” stated John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. “During 2017 we are laser focused on five key strategic priorities to advance our vision to develop and deliver great medicines for patients and to create significant shareholder value: 1) advancing the international launch of Galafold for Fabry disease, 2) completing our regulatory submission for migalastat in Japan (J-NDA), 3) establishing our novel Pompe treatment paradigm ATB200/AT2221 as a highly differentiated therapy, 4) successfully completing our Phase 3 clinical study in patients with epidermolysis bullosa, and 5) maintaining our financial strength. With one commercial-stage medicine and two medicines in clinical development, as well as a biologics platform for future growth, we are building a leading global biotechnology company focused on delivering meaningful benefits for patients living with devastating rare diseases.”

2016 Full-Year Financial Results

- Total product revenue in the full year 2016 was approximately \$5.0 million, which represents commercial sales of Galafold (migalastat) in Germany as well as reimbursed Expanded Access Programs (EAPs) in two countries during the third and fourth quarter of 2016.
- Cash, cash equivalents, and marketable securities totaled \$330.4 million at December 31, 2016 compared to \$214.0 million at December 31, 2015.
- Total operating expenses increased to \$186.0 million compared to \$130.4 million for the full year 2015 primarily due to increases in commercial costs of the Fabry monotherapy program and manufacturing scale-up on the Pompe program.
- Net cash spend was \$154.3 million, within the full-year 2016 guidance of \$135-155 million.
- Net loss was \$200.0 million, or \$1.49 per share, compared to a net loss of \$132.1 million, or \$1.20 per share, for the full year 2015.

2017 Financial Guidance

Cash, cash equivalents, and marketable securities totaled \$330.4 million at December 31, 2016. As previously announced, the Company strengthened the balance sheet during 2016 with a \$100 million at-the-market (ATM) equity financing as well as a \$250 million convertible debt offering. The Company expects full-year 2017 net operating cash spend of between \$175 million to \$200 million and expects 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:

- 75 patients (naïve and ERT-switch) on reimbursed Galafold as of February 28, 2017
- 10 countries with reimbursement (commercial or EAP)
- Reimbursement dossiers submitted and pricing discussions are now underway in 14 countries
- Launch commenced in United Kingdom following National Institute for Health and Care Excellence (NICE) publication of final guidance recommending reimbursement of Galafold in England
- 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

- Phase 3 gastrointestinal (GI) symptoms study protocol nearly complete and detailed feasibility study underway to support U.S. full approval pathway

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) targeted for 1H17
- U.S. intermediate EAP
- Phase 3 gastrointestinal (GI) symptoms study
- Fabry ERT cell line development and program update

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 in the fourth quarter of 2016 and during the 13th Annual WORLDSymposium™ in San Diego, CA in February 2017 from a global clinical study (ATB200-02) to evaluate safety, tolerability, PK, and pharmacodynamics (PD) of ATB200/AT2221. 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).

Key Preliminary Data Highlights from ATB200-02 Study in Initial ERT-Switch and ERT-Naïve Patients:

- No infusion-associated reactions following 150+ infusions in initial patients treated for a maximum of 36 weeks (n=13)
 - Available PK and PD (muscle and glycogen biomarkers) data through week 18 in eight initial ERT-switch patients and two ERT-naïve showed:
 - The desired PK profile
 - Improvements in key muscle damage biomarkers (creatinine kinase (CK) enzyme, alanine aminotransferase (ALT), and aspartate aminotransferase (AST)) in a majority of ERT-switch patients and both ERT-naïve patients
 - Reductions in a biomarker of glycogen substrate urine hexose tetrasaccharide (Hex4) in all patients
 - Target enrollment achieved across all patient cohorts
-

Anticipated Upcoming Pompe Disease Program Milestones:

- ATB200-02 study data in additional naïve and non-ambulatory patients, as well as extension-phase data on all patient cohorts, in the second and third quarter of 2017
- 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:

- Significant momentum enrolling patients diagnosed with Simplex, Recessive Dystrophic, or Junctional non-Herlitz EB
- More than 95% of patients completing the primary treatment period have elected to continue in the open-label extension study

Anticipated EB Program Milestones:

- Top-line Phase 3 data anticipated mid-2017

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, March 1, 2017 at 8:30 a.m. ET to discuss full-year 2016 financial results and corporate updates. Interested participants and investors may access the conference call at 8:00 a.m. ET by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international) participant code 77299407.

An audio webcast 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 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 77299407.

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 m²). 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.

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. In particular, this press release relates to the preliminary data from a global Phase 1/2 study (ATB200-02) to investigate ATB200/AT2221. The inclusion of forward-looking statements arising from this preliminary data and study 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 for any of our product candidates. The preliminary data and Phase 1/2 study discussed herein is inherently preliminary and early in the study, derived from a limited patient set, and later trial results with this patient set or others may not be consistent with these preliminary 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 previous filings with the SEC and in our Annual Report on Form 10-K for the year ended December 31, 2016 to be filed later today. 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:

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TABLE 1

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

| | Years Ended December 31, | | |
|---|--------------------------|---------------------|--------------------|
| | 2016 | 2015 | 2014 |
| Revenue: | | | |
| Net Product Sales | \$ 4,958 | \$ — | \$ — |
| Research revenue | — | — | 1,224 |
| Total revenue | 4,958 | — | 1,224 |
| Cost of goods sold | 833 | — | — |
| Gross Profit | 4,125 | — | 1,224 |
| Operating Expenses: | | | |
| Research and development | 104,793 | 76,943 | 47,624 |
| Selling, general and administrative | 71,151 | 47,269 | 20,717 |
| Changes in fair value of contingent consideration payable | 6,760 | 4,377 | 100 |
| Restructuring charges | 69 | 15 | (63) |
| Depreciation | 3,242 | 1,833 | 1,547 |
| Total operating expenses | 186,015 | 130,437 | 69,925 |
| Loss from operations | (181,890) | (130,437) | (68,701) |
| Other income (expenses): | | | |
| Interest income | 1,602 | 929 | 223 |
| Interest expense | (5,398) | (1,578) | (1,484) |
| Loss on extinguishment of debt | (13,302) | (952) | — |
| Other expense | (4,793) | (80) | (77) |
| Loss before income tax benefit | (203,781) | (132,118) | (70,039) |
| Income tax benefit | 3,739 | — | 1,113 |
| Net loss attributable to common stockholders | \$ (200,042) | \$ (132,118) | \$ (68,926) |
| Net loss attributable to common stockholders per common share — basic and diluted | \$ (1.49) | \$ (1.20) | \$ (0.93) |
| Weighted-average common shares outstanding — basic and diluted | 134,401,588 | 109,923,815 | 74,444,157 |

TABLE 2

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

| | December 31, | |
|--|---------------------|-------------------|
| | 2016 | 2015 |
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 187,026 | \$ 69,485 |
| Investments in marketable securities | 143,325 | 144,548 |
| Accounts receivable | 1,304 | — |
| Inventories | 3,416 | — |
| Prepaid expenses and other current assets | 4,993 | 2,568 |
| Total current assets | 340,064 | 216,601 |
| Property and equipment, less accumulated depreciation of \$12,495 and \$13,353 at December 31, 2016 and 2015, respectively | 9,816 | 6,178 |
| In-process research & development | 486,700 | 486,700 |
| Goodwill | 197,797 | 197,797 |
| Other non-current assets | 2,468 | 1,108 |
| Total Assets | \$ 1,036,845 | \$ 908,384 |
| Liabilities and Stockholders' Equity | | |
| Current liabilities: | | |
| Accounts payable, accrued expenses, and other current liabilities | \$ 41,008 | \$ 32,216 |
| Deferred reimbursements, current portion | 13,850 | — |
| Contingent consideration payable, current portion | 56,101 | 41,400 |
| Total current liabilities | 110,959 | 73,616 |
| Deferred reimbursements | 21,906 | 35,756 |
| Convertible notes | 154,464 | — |
| Due to related party | — | 41,601 |
| Contingent consideration payable | 213,621 | 232,677 |
| Deferred income taxes | 173,771 | 176,219 |
| Other non-current liability | 1,973 | 681 |
| Commitments and contingencies | — | — |
| Stockholders' equity: | | |
| Common stock, \$.01 par value, 250,000,000 shares authorized, 142,691,986 shares issued and outstanding at December 31, 2016 | 1,480 | 1,306 |
| Common stock, \$.01 par value, 250,000,000 shares authorized, 125,027,034 shares | — | — |

| | | |
|---|---------------------|-------------------|
| issued and outstanding at December 31, 2015, | | |
| Additional paid-in capital | 1,120,156 | 917,454 |
| Accumulated other comprehensive loss: | | |
| Foreign currency translation adjustment, less tax benefit of \$1,293 at December 31, 2016 | 1,945 | — |
| Unrealized gain/ (loss) on available-for securities | 102 | (115) |
| Warrants | 16,076 | 8,755 |
| Accumulated deficit | (779,608) | (579,566) |
| Total stockholders' equity | 360,151 | 347,834 |
| Total Liabilities and Stockholders' Equity | \$ 1,036,845 | \$ 908,384 |

FOLD—G



FY16 Financial Results Conference Call & Webcast

March 1, 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. In particular, this presentation relates to the preliminary data from a global Phase 1/2 study (ATB200-02) to investigate ATB200/AT2221. The inclusion of forward-looking statements arising from this preliminary data and study 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. The preliminary data and Phase 1/2 study discussed herein is inherently preliminary and early in the study, derived from a limited patient set, and later trial results with this patient set or others may not be consistent with these preliminary results. With respect to statements regarding projections of our cash position, actual results may differ based on market factors and our ability to execute 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 to be filed later today. 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 presentation to reflect events or circumstances after the date hereof.



Introduction

Key Accomplishments in 2016

2016

Fabry Disease (Galafold™)

- EU approval
- International launch success
- Regulatory progress

Pompe Disease (ATB200/AT2221)

- Positive preliminary data in Phase 1/2 study in Pompe patients

Epidermolysis Bullosa (EB) (SD-101)

- Phase 3 enrollment near complete

Strong Balance Sheet

- \$330M in cash (12/31/16)

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

Early Success with International Launch (as of 2/28/17)

**Initial Launch Success Driven by Germany with ERT-Switch & Naïve Patients,
Reimbursement Now Available in 10 Countries***

75

Patients (Switch & Naïve) on reimbursed Galafold (2/28/17)

10

Countries with available reimbursement*

14

Countries with pricing discussions ongoing

27

Countries with Amicus footprint



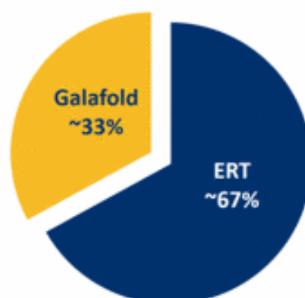
*Commercial and Expanded Access Programs (EAPs)

German Launch Update (as of 2/28/17)

Germany is an Important Indicator for EU Launch Success



Current
Approximate
Market Share*



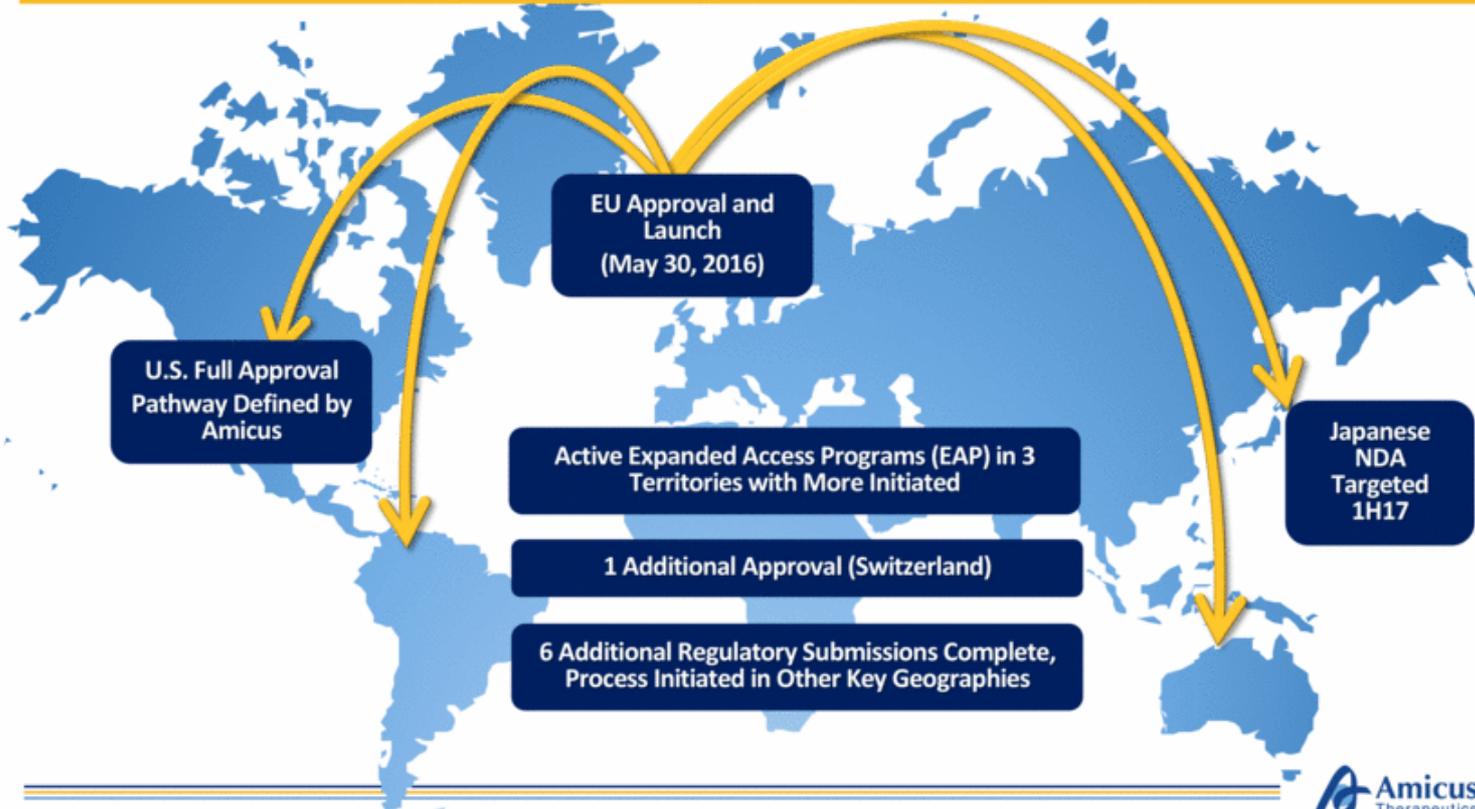
IMPORTANT EARLY INDICATORS IN GERMANY

- Vast majority switch patients
- ~33% of eligible switch patients now on Galafold*
- All newly experienced patients & physicians
- Majority of switches from Replagal™
- Male / female mix
- 16 unique prescribers

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

Global Regulatory Strategy to Reach More Patients

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





ATB200 Novel ERT for Pompe Disease

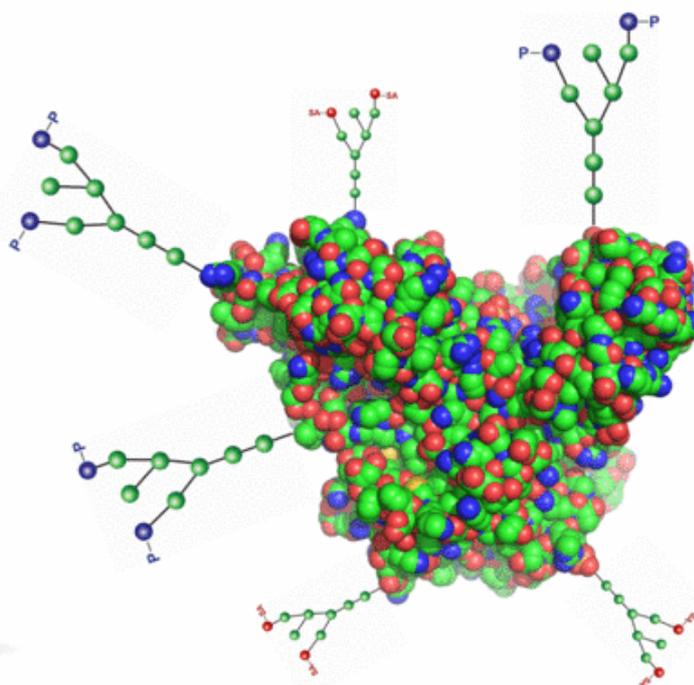
Establishing Human Proof of Concept and Validating
Biologics Platform in 2017

ATB200 + Chaperone: A Highly Differentiated Approach

Novel Pompe Treatment Paradigm with Three Key Differentiators

**ATB200
(Novel ERT)**

**Chaperone
addition**



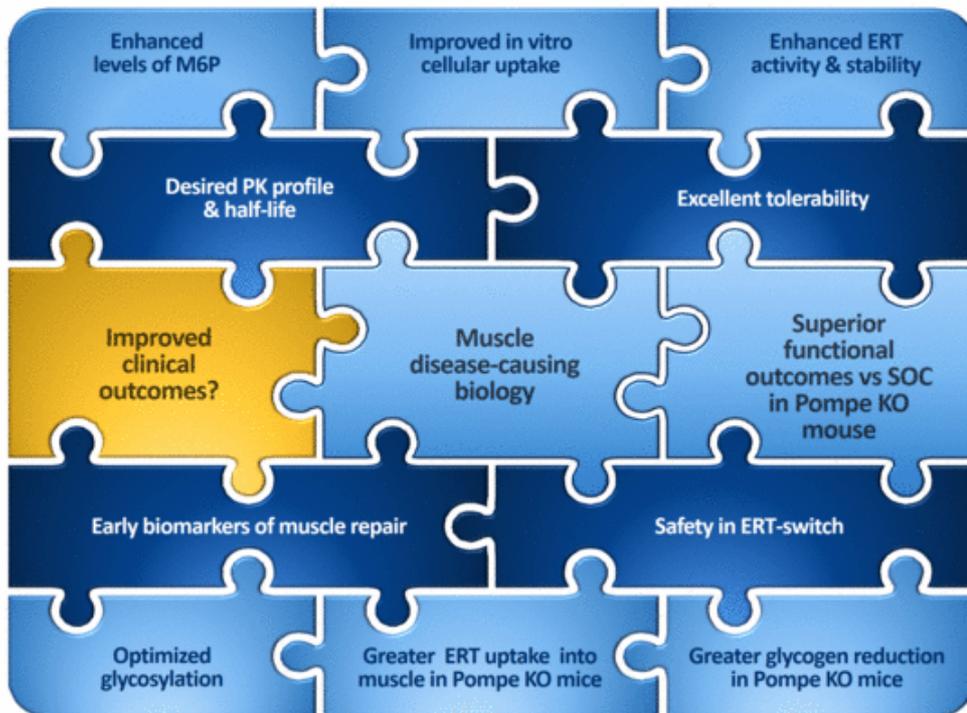
**Optimized
mixture of
glycans**

**High levels of
M6P and bis
M6P**

*Artist rendering, not actual product image

Pompe Disease: A Complex Disease with Significant Unmet Needs

We've Made Great Strides and Expect to Address Key Remaining Questions in 2017



preclinical clinical key question

"The scientific findings and preclinical data are profound and shed new light on questions about the underlying cause of muscle damage and weakness in Pompe patients. Furthermore, these results provide a window into a potential underlying link among key muscular dystrophies, such as Pompe, Limb Girdle, and Duchenne. Amicus has been a pioneer in advancing the scientific understanding of Pompe disease and in developing next-generation therapies for patients."

Grace K. Pavlath, Ph.D., Senior Vice President, Scientific Program Director of Muscular Dystrophy Association



Preliminary Clinical Data Summary

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

- **Safety (N=13)***
 - No serious adverse events (SAEs)
 - 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

Target Enrollment Achieved with a Cascade of Additional Data Points During 2Q17 and 3Q17 to Demonstrate Proof of Concept

Pompe Milestones in 2017

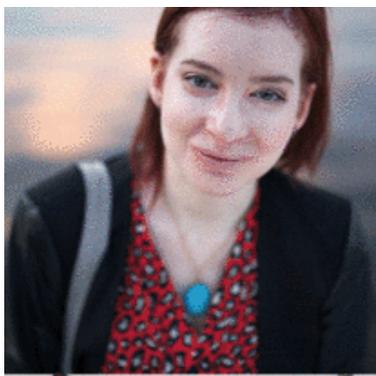


18-WEEK DATA

- 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 Mid-2017

Phase 3 Study - Delivering on Our EB Vision

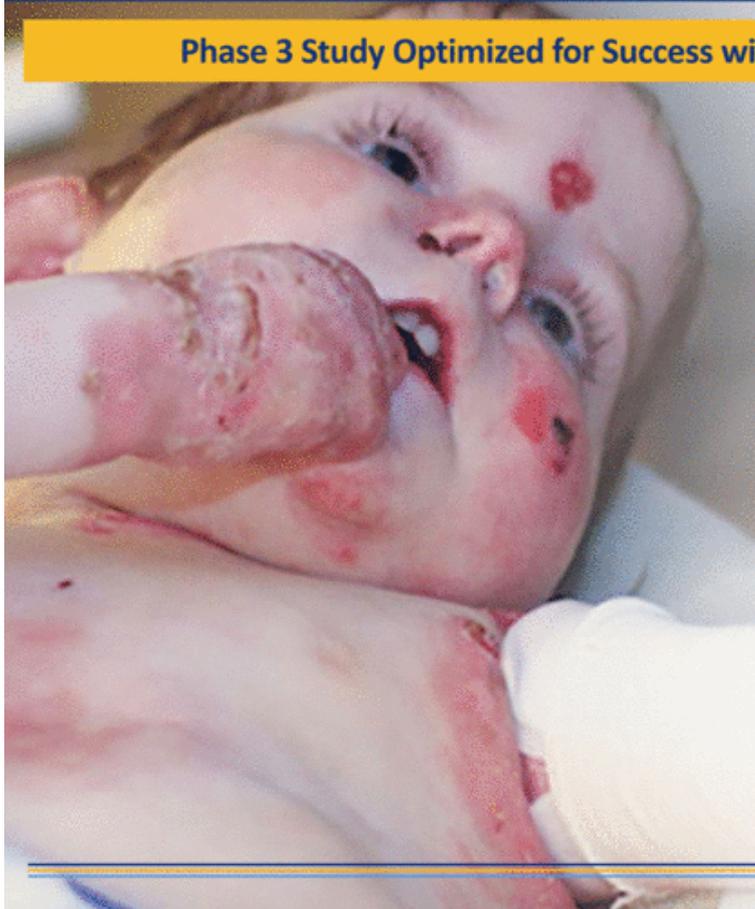
Phase 3 Study Optimized for Success with Top-Line Data On Track for Mid-2017

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
- Enrollment near complete
- Top-line data anticipated mid-2017





Financial Summary

FY16 Select Financial Results

First-Ever Year to Report Product Revenue of \$5M from Sales of Galafold

| | December 31, 2016 | December 31, 2015 |
|--------------------|-------------------|-------------------|
| Product revenue | \$5.0M | - |
| R&D Expense | \$104.8M | \$76.9M |
| G&A Expense | \$71.2M | \$47.3M |
| Net Loss | (\$200.0M) | (\$132.1M) |
| Net Loss Per Share | (1.49) | (1.20) |

Financial Summary & Guidance

Balance Sheet Strengthened with \$330M Cash at 12/31/16 and Cash Runway Into 2H18

| Financial Position | December 31, 2016 |
|---------------------------------------|-------------------|
| Cash | \$330M |
| Debt | \$250M |
| FY17 Net Operating Cash Flow Guidance | \$175-\$200M |
| FY17 Net Cash Spend Guidance* | \$200-\$225M |
| Cash Runway | 2H18 |
| Capitalization | December 31, 2016 |
| Shares Outstanding | 142,691,986 |

*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 1H17
- Fabry GI study

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 mid-2017

Strong Balance Sheet

- Significant revenue contribution
- Cash runway into 2H18

*Commercial and Expanded Access Programs (EAPs)

Our Vision – Maximizing Impact on Patients to Drive Shareholder Value

The Ultimate Measure of Our Success Will be the Number of Patients with Devastating Rare Diseases Treated with an Amicus Product



= 20 patients



~37 Patients

~90 Patients

~250 Patients*

~800 Patients*

~5,000 Patients*

2010

2014

YE16

2018

2023

*Clinical & Commercial



Thank You

