

**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 8, 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 November 8, 2017, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the third quarter ended September 30, 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 November 8, 2017 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.

**EXHIBIT INDEX**

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#">Press Release dated November 8, 2017</a>
99.2	<a href="#">November 8, 2017 Conference Call Presentation Materials</a>

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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.

Amicus Therapeutics, Inc.

Date: November 8, 2017

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

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

**260+ Fabry Patients on Reimbursed Galafold™ (Migalastat) and  
On Target to Reach 300 Patients by Year-End 2017**

**U.S. NDA Submission for Migalastat Planned in 4Q17**

**Pompe Retrospective and Prospective Data Collection Studies Initiated**

**Additional Pompe Clinical Data to be Presented at WORLDSymposium™ in February 2018**

**CRANBURY, NJ, November 8, 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 third quarter ended September 30, 2017. The Company also summarized recent program updates and reiterated full-year 2017 financial guidance.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, stated, “During the third quarter we continued to successfully execute across our strategic priorities for our core programs in Fabry and Pompe. We are pleased with the significant growth and expansion of our international Galafold launch, and the number of patients treated with this precision medicine for Fabry disease. In Pompe disease, the data cascade for our novel treatment paradigm ATB200/AT2221 has continued to exceed our expectations in terms of the consistency, durability and magnitude of effect across patients and across functional outcomes, key disease biomarkers and safety. There has been extraordinary patient and physician demand for this important potential treatment option. We are committed to increasing access to this investigational medicine for as many people as possible living with Pompe as soon as we can.”

Amicus is on track to achieve four key strategic priorities between now and the end of 2017:

- 1) Target of 300 patients on reimbursed Galafold;
- 2) New Drug Application (NDA) submission for migalastat to the U.S. FDA;
- 3) Continued advancements with the Pompe program, including now ongoing collaborative discussions with regulators to determine the best and fastest pathway; and
- 4) A strong balance sheet with more than 18 months of cash at year-end.

**Third Quarter 2017 Financial Results**

- Total revenue in the third quarter 2017 was approximately \$10.9 million, a sequential increase of 51.4% from total revenue of \$7.2 million in the second quarter 2017, and a year-over-year increase of 419% from total revenue of \$2.1 million in the third quarter 2016. Total revenue 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 \$426.6 million at September 30, 2017 compared to \$330.4 million at December 31, 2016.
- Total operating expenses increased to \$284.3 million compared to \$46.7 million for the third quarter 2016 primarily from non-cash charges related to the Phase 3 ESSENCE study in epidermolysis bullosa (EB).
- Operating expenses, as adjusted, excludes the impact of the non-cash charges related to the ESSENCE study, were \$73.5 million, representing a \$26.8 million increase over the third quarter of 2016 primarily due to increased investments in the Pompe and EB programs as well as increased investment in the Galafold commercial launch.
- Net cash spend was \$147.3 million for the nine months ending September 30, 2017.
- Net loss was \$111.7 million, or \$0.69 per share, compared to a net loss of \$46.7 million, or \$0.33 per share, for the third quarter 2016. Net loss, as adjusted excludes the impact of the non-cash charges related to the ESSENCE study was \$65.6 million or \$0.41 per share.

**2017 Financial Guidance**

Cash, cash equivalents, and marketable securities totaled \$426.6 million at September 30, 2017 compared to \$330.4 million at December 31, 2016. The current cash position includes \$243.0 million in net proceeds from a follow on public offering in July 2017.

Amicus 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 at least the second half of 2019.

**Program Highlights**

**Migalastat for Fabry Disease**

Migalastat is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic mutations. Regulatory authorities in the European Union, Switzerland, Israel, Canada and Australia have granted full approval for migalastat under the trade name Galafold. The EU 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. In the U.S., as previously announced, the FDA has confirmed that Amicus may submit a new drug application (NDA) for migalastat.

International Launch and Expanded Access Programs (EAP) Updates:

- More than 260 patients (naïve and ERT-switch) on reimbursed Galafold as of October 31, 2017

- 13 countries with reimbursement (commercial or EAP) including the top four largest EU countries
- Reimbursement dossiers submitted and pricing discussions are now underway in 12 countries
- Target of 300 patients treated with reimbursed Galafold on track for year-end 2017

#### Global Regulatory Updates:

- Four additional approvals secured outside the EU (Switzerland, Israel, Canada and Australia)
- Regulatory submissions completed in seven additional countries outside the EU, including Japan
- NDA submission to U.S. FDA to be based on existing data on track for 4Q17

#### Anticipated Upcoming Fabry Disease Program Milestones:

- Commercial launch and EAPs in additional international countries
- Additional regulatory submissions including a U.S. NDA (4Q17)
- Regulatory decision in Japan (1H18)
- Final preclinical data and announcement of path forward for novel Amicus Fabry ERT cell line for Fabry patients with non-amenable mutations (1Q18)

#### **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. Additional positive data were reported in October 2017 from an ongoing global Phase 1/2 clinical study (ATB200-02) to evaluate safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of ATB200/AT2221 across three cohorts, including ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3). The Company has commenced collaborative discussions with U.S. and EU regulators regarding the best and fastest pathway forward for this novel treatment option, and continues to anticipate a Pompe regulatory pathway update in the first half of 2018.

In order to continue to build a robust data set and to meet the needs of the Pompe community, Amicus announced the following four key status updates for this important program:

- A retrospective natural history study of Pompe patients treated with approved standard of care ERT has been initiated at leading global Pompe disease treatment centers (POM-002 Study)
- A prospective observational study has also been initiated in Pompe patients currently receiving approved standard of care ERT at leading global Pompe disease treatment centers (POM-003 Study)

- All engineering runs successfully completed and GMP production of ATB200 has commenced at the large commercial scale (1,000 Liters)
- Analytical and *in vivo* (preclinical) comparability studies completed between the 250 Liter and 1,000 Liter scale

#### Anticipated Upcoming Pompe Disease Program Milestones:

- Ongoing discussions with U.S. and EU regulators
- Additional data from ATB200-02 clinical study at 14<sup>th</sup> Annual *WORLDSymposium*<sup>™</sup> (February 5-9, 2018)
- Pompe regulatory pathway update (1H18)

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

During the third quarter Amicus reported that top-line data from the randomized, double-blind, placebo-controlled Phase 3 clinical study (ESSENCE, SD-005) to assess the efficacy and safety of the novel topical wound-healing agent SD-101 did not meet the primary endpoints or secondary endpoints in participants with EB. Based on these top-line data Amicus has no current plans to invest in any additional clinical studies or commercial preparation activities for SD-101. The Company continues to make SD-101 available to all patients currently enrolled in the ongoing extension study (SD-006).

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

Amicus Therapeutics will host a conference call and audio webcast today, November 8, 2017 at 8:30 a.m. ET to discuss third 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); conference ID: 5995789.

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); conference ID: 5995789.

### **Non-GAAP Financial Measures**

In addition to the United States generally accepted accounting principles (GAAP) results, this earnings release contains non-GAAP financial measures that we believe, when considered together with the GAAP information, provides useful information to investors that promotes a more complete understanding of our operating results and financial position for the current period. Management uses these non-GAAP financial measures internally for planning, forecasting, evaluating and allocating resources to the Company's programs. The non-GAAP results exclude the impact of the following GAAP items specifically related to the Phase 3 ESSENCE study in EB: changes in fair value of contingent consideration, impairment of assets and adjustments to income tax expense. These non-GAAP financial measures should be considered in addition to, and not as replacements for, or superior to, measures of financial performance prepared in accordance with GAAP. Please refer to the attached Reconciliation of Non-GAAP Financial Measures for explanations of the amounts adjusted to arrive at non-GAAP total operating expense, net loss attributable to common stockholders and net loss attributable to common stockholders per common share — basic and diluted for the three month period ended September 30, 2017.

### **EU 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.

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- 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, 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 other geographies, 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 and our Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, filed 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:

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

Amicus Therapeutics

**TABLE 1**

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
<b>Revenue:</b>				
Net product sales	\$ 10,874	\$ 2,127	\$ 22,201	\$ 2,127
Cost of goods sold	1,790	344	3,626	344
<b>Gross Profit</b>	<b>9,084</b>	<b>1,783</b>	<b>18,575</b>	<b>1,783</b>
<b>Operating Expenses:</b>				
Research and development	40,641	32,457	103,502	74,163
Selling, general and administrative	21,647	17,469	60,090	52,470
Changes in fair value of contingent consideration payable	(244,250)	(4,110)	(238,622)	9,228
Loss on impairment of assets	465,427	—	465,427	—
Restructuring charges	—	11	—	69
Depreciation	851	896	2,486	2,336
Total operating expenses	284,316	46,723	392,883	138,266
Loss from operations	(275,232)	(44,940)	(374,308)	(136,483)
<b>Other income (expenses):</b>				
Interest income	1,190	460	2,702	1,098
Interest expense	(4,351)	(1,517)	(12,820)	(3,517)
Other income (expense)	2,044	(910)	5,054	(3,199)
Loss before income tax benefit	(276,349)	(46,907)	(379,372)	(142,101)
Income tax benefit	164,683	253	164,578	706
<b>Net loss attributable to common stockholders</b>	<b>\$ (111,666)</b>	<b>\$ (46,654)</b>	<b>\$ (214,794)</b>	<b>\$ (141,395)</b>
Net loss attributable to common stockholders per common share — basic and diluted	\$ (0.69)	\$ (0.33)	\$ (1.44)	\$ (1.07)
Weighted-average common shares outstanding — basic and diluted	160,796,841	140,656,109	148,963,864	131,675,690

**TABLE 2**

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

	September 30, 2017	December 31, 2016
<b>Assets</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 64,133	\$ 187,026
Investments in marketable securities, current portion	347,388	143,325
Accounts receivable	5,974	1,304
Inventories	7,272	3,416
Prepaid expenses and other current assets	6,246	4,993
Total current assets	431,013	340,064
Investments in marketable securities	15,109	—
Property and equipment, less accumulated depreciation of \$13,273 and \$12,495 at September 30, 2017 and December 31, 2016, respectively	9,641	9,816
In-process research & development	23,000	486,700
Goodwill	197,797	197,797
Other non-current assets	4,219	2,468
<b>Total Assets</b>	<b>\$ 680,779</b>	<b>\$ 1,036,845</b>

**Liabilities and Stockholders' Equity**

## Current liabilities:

Accounts payable, accrued expenses, and other current liabilities	\$ 53,709	\$ 41,008
Deferred reimbursements, current portion	6,250	13,850
Contingent consideration payable, current portion	8,200	56,101
<b>Total current liabilities</b>	<b>68,159</b>	<b>110,959</b>
Deferred reimbursements	16,906	21,906
Convertible notes	161,635	154,464
Contingent consideration payable	12,900	213,621
Deferred income taxes	9,186	173,771
Other non-current liability	2,313	1,973

## Commitments and contingencies

## Stockholders' equity:

Common stock, \$0.01 par value, 250,000,000 shares authorized 165,491,141 and 142,691,986 shares issued and outstanding at September 30, 2017 and December 31, 2016, respectively	1,707	1,480
Additional paid-in capital	1,387,767	1,120,156
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	(1,367)	1,945
Unrealized gain on available-for-sale securities	(101)	102
Warrants	16,076	16,076
Accumulated deficit	(994,402)	(779,608)
<b>Total stockholders' equity</b>	<b>409,680</b>	<b>360,151</b>
<b>Total Liabilities and Stockholders' Equity</b>	<b>\$ 680,779</b>	<b>\$ 1,036,845</b>

TABLE 3

**Amicus Therapeutics, Inc.**  
**Reconciliation of Non-GAAP Financial Measures**  
**Dollars in Thousands Except Per Share Data**

	<b>Three Months Ended September 30, 2017</b>
Total operating expenses — as reported	\$ 284,316
Loss on impairment of assets related to the Phase 3 ESSENCE study in EB	465,427
Changes in fair value of contingent consideration payable related to the Phase 3 ESSENCE study in EB	(254,650)
<b>Total operating expenses — as adjusted</b>	<b>\$ 73,539</b>
Net loss attributable to common stockholders — as reported	\$ (111,666)
Loss on impairment of assets related to the Phase 3 ESSENCE study in EB	465,427
Changes in fair value of contingent consideration payable related to the Phase 3 ESSENCE study in EB	(254,650)
Income tax benefit (1)	(164,683)
<b>Net loss attributable to common stockholders — as adjusted</b>	<b>\$ (65,572)</b>
Net loss attributable to common stockholders per common share — basic and diluted — as reported	\$ (0.69)
Net loss attributable to common stockholders per common share — basic and diluted — as adjusted	\$ (0.41)
Weighted-average common shares outstanding — basic and diluted — as reported and adjusted	160,796,841

(1) Related to the reversal of the deferred tax liability associated with the Scioderm in process research and development asset.

FOLD—G



# 3Q17 Financial Results Conference Call & Webcast

November 8, 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 and our Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, 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.*

## 2017 Key Strategic Priorities

We Remain Sharply Focused on FOUR Key Strategic Priorities as We Continue to Build a Top Global Biotechnology Company Focused on Rare Devastating Diseases

**Advance International Galafold Launch**

**Submit Japanese and U.S. New Drug Applications for Migalastat**

**Establish Definitive Proof of Concept for ATB200/AT2221 with Clear Path to Registration for Pompe Disease**

**Maintain Financial Strength**



# Galafold™ (Migalastat) Precision Medicine for Fabry Disease

Continue Successful Launch Execution and Geographic Expansion

## Successful International Launch Underway (as of 10/31/17)

Driven by Top EU5 Countries, Galafold is Quickly Reaching ERT-Switch & Naïve Patients,  
Reimbursement Now Available in 13 Countries\*

>260

Patients (Switch & Naïve) on  
reimbursed Galafold (10/31/17)

13

Countries with available reimbursement\*

12

Countries with pricing discussions ongoing

27

Countries with Amicus footprint

300

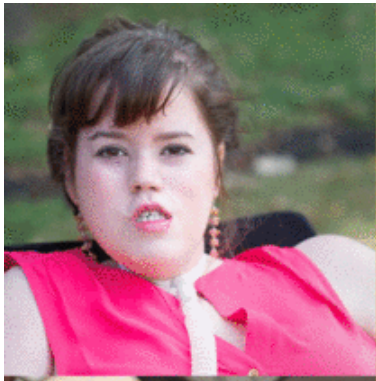
Target Number of  
Patients on Reimbursed  
Galafold by YE17

\*Commercial and Expanded Access Programs (EAPs)

# Global Regulatory Strategy to Reach More Patients (as of 10/31/17)

EU Approval and Planned U.S. NDA Submission Provide Pathway to Reach Global Fabry Market





# ATB200 Novel ERT for Pompe Disease

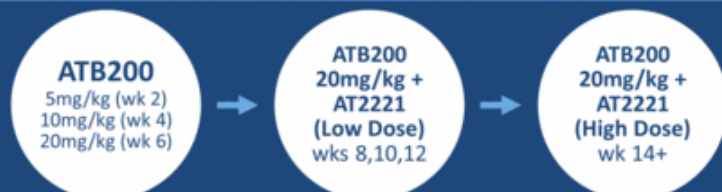
Establishing Human Proof of Concept and Validating  
Biologics Platform in 2017

## Phase 1/2 ATB200-02 Study Design

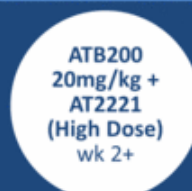
Phase 1/2 Clinical Study to Evaluate Safety, Tolerability, Pharmacokinetics (PK), and Pharmacodynamics (PD) of ATB200 + Chaperone (ATB200/AT2221) at 16 Sites in 5 Countries

### 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:

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

## Summary of Available Data (as of 10/4/17)

	Cohort 1 (ERT-Switch, n=11)	Cohort 2 (Non-ambulatory ERT-Switch, n=4)	Cohort 3 (ERT-Naive, n=5)
<b>Safety and tolerability</b>	n=11	n=4	n=5
<b>Biomarkers</b>	n=11	n=4	n=5
<b>Functional assessments at month</b>	Month 6 (n=9)* Month 9 (n=8)	Month 6 (n=4)	Month 6 (n=5) Month 9 (n=2)
<b>Functional assessments</b>	6MWT Other motor function tests Pulmonary function (FVC, MIP/MEP)	Muscle Strength Tests	6MWT Other motor function tests Pulmonary function (FVC, MIP/MEP)

\*One patient discontinued after completing Stage 1 (week 18) due to travel burden/family considerations and one patient's month 6 assessments pending due to an incomplete visit



## Study ATB200-02 Data Summary (as of 10/4/17)

### Consistent and Durable Improvement in Muscle Function and Key Biomarkers as well as Stabilization or Improvement in Respiratory Function

- AEs generally mild and transient
- Very low rate of IARs (<1%) after 400+ total infusions
- Six-minute walk test (6MWT) distance increased out to Month 9
  - ERT-naïve patients: mean increases of +42m (Month 6) and +75m (Month 9)
  - ERT-switch patients: mean increases of +35m (Month 6) and +37m (Month 9)
- Muscle strength tests increased in non-ambulatory ERT-switch patients at Month 6
- Muscle function improved in 16/18 and 10/10 patients at months 6 and 9 respectively; other motor function tests consistent with 6MWT for ERT-naïve and ERT-switch patients
- Pulmonary function (FVC) generally stable in ERT-switch patients and increased in ERT-naïve patients
- Consistent and durable improvement in biomarkers of muscle damage (CK, ALT, AST) and disease substrate (Hex4) across all cohorts

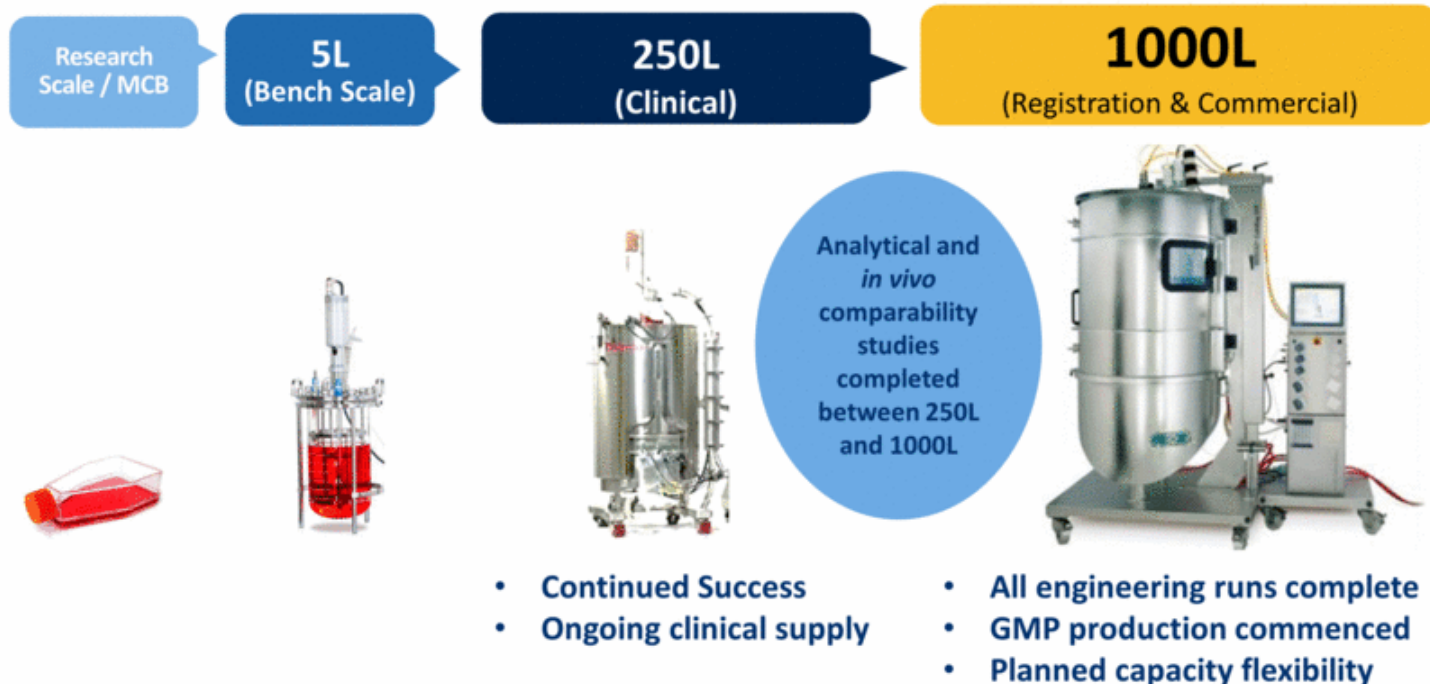
## Key Pompe Program Updates (as of November 2017)

### Building a Robust Data Set in Pompe Patients

- Initiated retrospective natural history study (POM-002 Study)
- Commenced prospective observational study (POM-003 Study)
- Continuing collaborative discussions with U.S. and EU regulators
- Additional Phase 1/2 clinical data at *WORLDSymposium™* in February 2018

# Biologics Manufacturing Capabilities

## Scaling Up Manufacturing to Meet the Needs of the Pompe Community





# Financial Summary

## 3Q17 Select Financial Results

### 3Q17 Revenue of \$10.9M from Sales of Galafold (419% Increase Year-Over-Year)

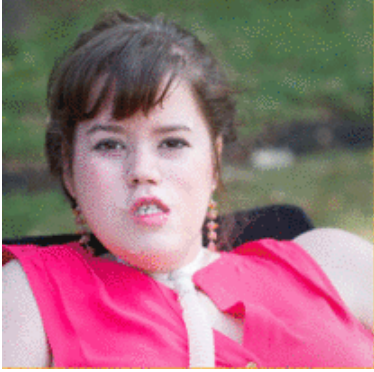
(\$000s) except per share data	September 30, 2017	September 30, 2016
Product revenue	\$10,874	\$2,127
R&D Expense	40,641	32,457
SG&A Expense	21,647	17,469
Changes in fair value of contingent consideration	(244,250)	(4,110)
Loss on impairment of assets	465,427	-
Loss from operations	(275,232)	(44,940)
Income tax benefit	164,683	253
Net Loss	(111,666)	(46,654)
Net Loss Per Share	\$(0.69)	\$(0.33)

# Financial Summary & Guidance

**Strong Balance Sheet with \$426.6 Cash at 9/30/17 and Cash Runway Into 2H19**

<b>Financial Position</b>	<b>September 30, 2017</b>
Cash	\$426.6M
Debt	\$250M
FY17 Net Operating Cash Spend Guidance	\$175-\$200M
FY17 Net Cash Spend Guidance*	\$200-\$225M
Cash Runway	2H19
<b>Capitalization</b>	<b>September 30, 2017</b>
Shares Outstanding	165,491,141

\*Includes third party milestone payments and capital expenditures.



## Closing Remarks

## Key Anticipated Milestones

### 2017/1H18

#### Fabry Disease (Galafold)

- U.S. NDA submission in 4Q17
- 300 patients on reimbursed Galafold by YE17\*
- Decision on Japanese regulatory submission (J-NDA) in 1H18

#### Pompe Disease (ATB200/AT2221)

- Ongoing discussions with U.S. and EU regulators
- Additional clinical data at *WORLDSymposium* (February 2018)
- Regulatory update in 1H18

#### Strong Balance Sheet

- Significant revenue contribution
- Cash runway into 2H19

\*Commercial and Expanded Access Programs (EAPs)



Thank You

