

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 8, 2018**

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.

Item 2.02. Results of Operations and Financial Condition.

On May 8, 2018, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the first fiscal quarter, ended March 31, 2018. A copy of this press release is attached hereto as Exhibit 99.1. The Company will host a conference call and webcast on May 8, 2018 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 filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits:

Exhibit No.	Description
99.1	Press Release dated May 8, 2018.

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 8, 2018

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



Amicus Therapeutics Announces First Quarter 2018 Financial Results and Corporate Updates

Continued Global Galafold® (Migalastat) Adoption and Expansion — Reaffirms FY18 Revenue Guidance of \$75M-\$85M at Top End of Range

Significant Momentum with Pompe Clinical, Regulatory and Manufacturing Activities - GMP Manufacturing Campaigns of ATB200 Drug Substance and Drug Product Completed at 1000L Scale

\$605.2M Cash Balance to Support Current Operating Plan and Invest in Rare Metabolic Disease Pipeline

Conference Call and Webcast Today at 8:30am ET

CRANBURY, NJ, May 8, 2018 — Amicus Therapeutics (Nasdaq: FOLD), a global biotechnology company focused on discovering, developing and delivering novel medicines for rare metabolic diseases, today announced financial results for the first quarter ended March 31, 2018. The Company also summarized recent program updates and reiterated full-year 2018 revenue and net cash spend guidance.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “Since the beginning of 2018, we have successfully executed across our five key strategic priorities for the year. We are on track to more than double our Galafold® revenue from last year, we secured approval of Galafold in Japan, and we have advanced our Pompe clinical, manufacturing and regulatory activities. Along with our key value drivers in Fabry and Pompe, we continue to evaluate other potential first- or best-in-class technologies that we believe may deliver significant benefits for many more people living with rare metabolic diseases. Today, more than ever, we are well positioned to achieve our vision to impact as many people as possible who are living with rare metabolic diseases as we continue to build a leading global rare disease biotech company.”

2018 Key Strategic Priorities

- Double global revenue for Galafold (\$75 million - \$85 million)
- Secure approvals for migalastat in Japan and the U.S.
- Achieve clinical, manufacturing and regulatory milestones to advance AT-GAA (also known as ATB200/AT2221) toward global regulatory submissions and approvals as soon as possible
- Develop and expand preclinical pipeline to ensure at least one new clinical program in 2019
- Maintain a strong balance sheet

First Quarter 2018 Financial Results and Full-Year 2018 Financial Guidance

- Total revenue in the first quarter 2018 was \$16.7 million, a year-over-year increase of 300% from total revenue of \$4.2 million in the first quarter of 2018.
- Cash, cash equivalents, and marketable securities totaled \$605.2 million at March 31, 2018, compared to \$358.6 million at December 31, 2017.
- Total operating expenses increased to \$70.3 million for the first quarter 2018 compared to \$55.4 million in the first quarter 2017 reflecting increased investment in Pompe clinical and manufacturing activities as well as Galafold commercial launch and launch preparations.

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- Net cash spend was \$48.0 million for the first quarter 2018.
 - Net loss was \$49.9 million, or \$0.28 per share, compared to a net loss of \$55.0 million, or \$0.39 per share, for the first quarter 2017.

“The first quarter of 2018 marked another period of continued growth for Galafold and our strongest balance sheet in our Company’s history,” said Bradley L. Campbell, President and COO of Amicus Therapeutics. “We are confident in our ability to achieve the high end of our full-year 2018 Galafold revenue guidance given the current and anticipated increase in patient and physician adoption; expansion into new countries; and what we are seeing as a very high rate of compliance and adherence for this oral precision medicine.”

2018 Financial Guidance

For the full-year 2018 the Company anticipates total Galafold revenue at the high end of the \$75 million to \$85 million guidance range. The Galafold revenue guidance includes anticipated revenue from countries with final pricing and reimbursement, and does not currently include potential partial year contributions from Japan or the U.S.

The Company continues to expect full-year 2018 net cash spend between \$230 million and \$260 million. The current cash position, including proceeds from the February 2018 equity offering and expected Galafold revenues, is sufficient to fund ongoing Fabry and Pompe program operations into at least 2021. Potential future business development collaborations, pipeline expansion, and investment in biologics manufacturing capabilities could impact the Company’s future capital requirements.

Program Highlights

Migalastat for Fabry Disease

Amicus is committed to advancing the highest quality therapies for all people living with Fabry disease. Migalastat is an oral precision medicine intended to treat Fabry disease in patients 16 years or older who have amenable genetic mutations. Regulatory authorities in the European Union, Japan, Switzerland, Israel, Canada, Australia, and South Korea have granted full approval for migalastat under the trade name Galafold. In the U.S., the FDA accepted the Company's new drug application (NDA) for migalastat under priority review with a six-month PDUFA goal date of August 13, 2018.

For people with non-amenable mutations who are not eligible for migalastat as an oral precision medicine, the strategy is to advance next-generation therapies such as a novel Fabry ERT (ATB101) co-formulated with migalastat or other innovative technologies that continue to be evaluated.

Global Fabry Updates:

- Pricing and reimbursement secured in 18 countries with first commercial patients treated in multiple new countries in 2018
- Approvals secured in EU, Australia, Canada, Japan, Israel, South Korea and Switzerland
- Launch team hired and trained in Japan to execute upcoming launch
- U.S. leadership, including Mike Keavany, to serve as Senior Vice President of the U.S. Business, and majority of field team now in place to support planned U.S. launch

Anticipated Milestones:

- Japanese launch (2Q18)
- U.S. FDA regulatory decision (3Q18)

Advanced and Targeted GAA (AT-GAA, also known as ATB200/AT2221) for Pompe Disease

AT-GAA 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. During *WORLDSymposium™* in February 2018, Amicus presented updated positive data from an ongoing global Phase 1/2 clinical study (ATB200-02) to evaluate safety, tolerability, pharmacokinetics (PK), pharmacodynamics (PD), and functional outcomes of AT-GAA across ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2), and ERT-naïve patients (Cohort 3).

The Company is engaged in ongoing collaborative discussions with U.S. and EU regulators regarding a registration-directed study for full approval, manufacturing activities, and the best and fastest pathway forward for this novel treatment regimen. Amicus is pursuing formal scientific advice from the European Medicines Agency (EMA). A scientific advice meeting with the EMA is scheduled in 2Q18. The Company expects to provide an update on the results from that meeting by the end of 2Q. In the U.S., ongoing interactions on this program include a Type C meeting to occur in early 3Q18. Amicus expects to provide an FDA update in 3Q after receipt of written minutes from this meeting. The Company continues to believe that the evolving regulatory path will include a series of further iterative discussions with regulators as the program advances and as additional data are collected, including data from ongoing studies, data from new patients being enrolled and the results of a formal natural history study of Pompe patients receiving current ERT standard of care.

Amicus also continues to make progress with clinical and manufacturing activities to support the needs of the Pompe community.

Pompe Clinical Activities

- All 19 patients for whom extension study data have been previously reported continue treatment with AT-GAA in the ongoing Phase 1/2 ATB200-02 clinical study
- Currently enrolling Cohort 4 (up to 10 additional ambulatory ERT-switch patients) in ATB200-02 study
- Supportive studies underway including a retrospective study (POM-002) on the natural history of Pompe disease in up to 100 ERT-treated Pompe patients to help provide context for the ATB200-02 clinical study results; and a prospective observational study (POM-003) to assess safety and functional outcomes in patients currently treated with standard of care ERT, and to serve as a potential run-in for a registration study

Pompe Manufacturing Activities:

- As previously announced, FDA agreed on comparability between 250L scale and 1000L engineering batches, as well as the testing strategy for demonstrating comparability between 250L scale and 1000L GMP batches
- GMP production runs of ATB200 drug substance and drug product completed at 1,000L commercial scale

Anticipated Upcoming Pompe Program Milestones:

- Full enrollment of additional patients in ATB200-02 clinical study
- Final demonstration of comparability between 1,000L GMP material and 250L material
- Pompe regulatory updates (2Q18 and 3Q18)
- Release of 1,000L GMP material for initiation of registration-directed study (2H18)
- 18-month data from ATB200-02 clinical study (4Q18)

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, May 8, 2018, at 8:30 a.m. ET to discuss first quarter 2018 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: 6678368.

An audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate website at <http://ir.amicusrx.com/>, and will be archived for 30 days. Web participants are encouraged to go to the website 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 on May 8, 2018. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 6678368.

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.

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 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 global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. The cornerstone of the Amicus portfolio is migalastat, an oral precision medicine for people living with Fabry disease who have amenable genetic mutations. Migalastat is currently approved under

the trade name Galafold™ in the European Union, with additional approvals granted and pending in several geographies. The lead biologics program in the Amicus pipeline is AT-GAA, a novel, late-stage, potential best-in-class treatment paradigm for Pompe disease. The Company is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic 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, manufacturing and supply plans, financing plans, and the projected revenues and 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 and 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; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's revenue and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2017 to be filed on March 1, 2018. 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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FOLD—G

TABLE 1

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

	Three Months Ended March 31,	
	2018	2017
Revenue:		
Net product sales	\$ 16,696	\$ 4,169
Cost of goods sold	2,615	775
Gross Profit	14,081	3,394
Operating Expenses:		
Research and development	40,798	30,876
Selling, general and administrative	27,396	19,132
Changes in fair value of contingent consideration payable	1,100	4,578
Depreciation	969	823
Total operating expenses	70,263	55,409
Loss from operations	(56,182)	(52,015)
Other income (expense):		
Interest income	1,737	759
Interest expense	(4,488)	(4,290)
Change in fair value of derivatives	4,861	—
Other income	2,764	610
Loss before income tax	(51,308)	(54,936)
Income tax benefit (expense)	1,392	(56)
Net loss attributable to common stockholders	\$ (49,916)	\$ (54,992)
Net loss attributable to common stockholders per common share — basic and diluted	\$ (0.28)	\$ (0.39)
Weighted-average common shares outstanding — basic and diluted	175,977,700	142,770,629

TABLE 2

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

	March 31, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	\$ 114,322	\$ 49,060
Investments in marketable securities	448,198	309,502
Accounts receivable	10,836	9,464
Inventories	8,070	4,623
Prepaid expenses and other current assets	10,607	19,316
Total current assets	592,033	391,965
Investments in marketable securities	42,673	—
Property and equipment, less accumulated depreciation of \$13,461 and \$12,515 at March 31, 2018 and December 31, 2017, respectively	8,910	9,062
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	5,592	5,200
Total Assets	\$ 870,005	\$ 627,024

Liabilities and Stockholders' Equity

Current liabilities:

Accounts payable, accrued expenses, and other current liabilities	\$	43,678	\$	53,890
Deferred reimbursements		7,750		7,750
Derivative liability		80,577		—
Contingent consideration payable		8,700		8,400
Total current liabilities		<u>140,705</u>		<u>70,040</u>
Deferred reimbursements		14,156		14,156
Convertible notes		166,768		164,167
Contingent consideration payable		17,800		17,000
Deferred income taxes		6,465		6,465
Other non-current liability		2,494		2,346
Total liabilities		<u>348,388</u>		<u>274,174</u>

Commitments and contingencies

Stockholders' equity:

Common stock, \$0.01 par value, 250,000,000 shares authorized 187,972,218 and 166,989,790 shares issued and outstanding at March 31, 2018 and December 31, 2017, respectively		1,929		1,721
Additional paid-in capital		1,621,479		1,400,758
Accumulated other comprehensive loss:				
Foreign currency translation adjustment		(3,847)		(1,659)
Unrealized loss on available-for-sale securities		(877)		(436)
Warrants		16,076		16,076
Accumulated deficit		<u>(1,113,143)</u>		<u>(1,063,610)</u>
Total stockholders' equity		<u>521,617</u>		<u>352,850</u>
Total Liabilities and Stockholders' Equity	\$	<u>870,005</u>	\$	<u>627,024</u>



1Q18 Financial Results & Corporate Highlights



May 8, 2018

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, manufacturing and supply plans, financing plans, and the projected revenues and 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 and 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; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's revenue and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2017 as well as our Quarterly Report on Form 10-Q for the quarter ended March 31, 2018 to be filed May 9, 2018 with the Securities and Exchange Commission. 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.

2018 Key Strategic Priorities

Focused on FIVE Key Strategic Priorities in 2018

- 1 Double Galafold (migalastat) revenue to \$75-\$85M
- 2 Secure approvals for migalastat in Japan and the U.S.
- 3 Achieve clinical, manufacturing and regulatory milestones to advance AT-GAA* toward global regulatory submissions and approvals
- 4 Develop and expand preclinical pipeline to ensure at least one new clinical program in 2019
- 5 Maintain financial strength

*Advanced and Targeted GAA (AT-GAA, also known as ATB200/AT2221)



Galafold™ (Migalastat) Precision Medicine for Fabry Disease

"We push ideas as far and as fast as possible"
- Amicus Belief Statement

Galafold Snapshot (as of March 31, 2018)



FIRST Oral Precision Medicine for Fabry Disease

Galafold Indicated for Long-Term Treatment of Adults and Adolescents Aged ≥ 16 years with a Confirmed Diagnosis of Fabry Disease and Who Have an Amenable Mutation**

*EU, Australia, Canada, Japan, Israel, Switzerland, South Korea

**For important safety information for Galafold visit www.ema.europa.eu.

Migalastat Launch Progress (3/31/18)

Approved in Japan in 2Q18 – Now Approved in 7 Target Markets



Additional Galafold Launch Dynamics

Key Success Factors Driving Strong Launch Across Geographies and Support \$500M+ Global Peak Sales Potential

- Robust clinical data set^{1,2,3}
- Broad labels for amenable mutations (35%-50% of Fabry patients)
- Growth in patient and physician adoption from existing markets
- Expansion into new countries
- Very high rate of compliance and adherence
- Innovative precision medicine
- Compelling value proposition leading to rapid reimbursement
- Focus on hiring the best and brightest from a range of leading rare disease companies

1. Germain, DP et al., *New England Journal of Medicine*. 2. Hughes, et al., *Journal of Medical Genetics*. 3. For important safety information for Galafold visit www.ema.europa.eu.

U.S. and Japan Demographics for Galafold

U.S. NDA Filed Under Priority Review

Japan Launch Anticipated in 2Q18



US Overview

NDA Filed (Priority Review)

August 13, 2018 PDUFA

Leadership and majority of field team in place

Appropriate stakeholder engagement underway

~3,000 diagnosed (1,500 treated), 35-50% amenable

Orphan Drug and Fast Track designations

Japan Overview

J-NDA Approved March 2018

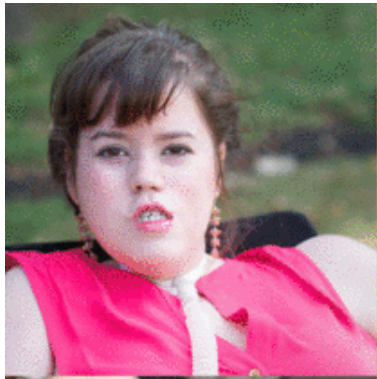
Launch team hired and trained

Launch anticipated 2Q18

~850 patients diagnosed (>700 treated)

No ERT home infusion currently available

Physicians tend to initiate treatment early



AT-GAA Novel ERT for Pompe Disease

"We encourage and embrace constant innovation"
- Amicus Belief Statement

Key Clinical & Manufacturing Activities 2018

Significant Progress Against Clinical and GMP Manufacturing Activities Ongoing in 2018 to Lay Foundation for Most Successful and Fastest Approval Pathways

CLINICAL

- ✓ Additional Phase 1/2 extension data presented at *WORLDSymposium*
- ✓ Additional patients in Phase 1/2 study (ongoing)
- ✓ Retrospective natural history of ERT-treated patients (ongoing)
- ✓ Prospective data collection on current ERT-treated patients (ongoing)
- Initiation of larger registration-directed study planned in 2018



MANUFACTURING

- ✓ Additional 1000L GMP campaigns completed
- ✓ Added capacity to ensure sufficient medicines to supply patient population
- Final regulatory agreement on comparability between 1,000L and 250L GMP scale
- Release for clinic of 1,000L GMP commercial scale material
- Announce plan for long term commercial manufacture and capacity



Pompe Regulatory Strategy

A series of regulatory updates regarding a registration-directed study for full approval, manufacturing activities and the best and fastest path forward for AT-GAA

- Significant progress in collaborative discussions with EMA and FDA since 4Q17
- Ongoing interactions include formal meetings scheduled with both agencies
 - EMA - scientific advice meeting and EU update anticipated in 2Q18
 - U.S. FDA – type C meeting and US update anticipated in 3Q18
- Goals of interactions and formal meetings
 - Alignment on design of pivotal and supportive studies for full approval
 - Discussion of potential conditional (EU) and accelerated (US) approval pathways
- A series of further iterative discussions with regulators and additional updates are expected as program advances, new data is collected, and registration-directed study initiated
- Goal continues to remain to determine the best and fastest pathway to approval

Key Clinical Activities 2018

Significant Progress In Clinical Activities in 2018 to Support Fastest and Best Approval Pathways

- ✓ Additional Phase 1/2 ATB200-02 extension data presented at *WORLDSymposium*
- ✓ Additional patients in Phase 1/2 ATB200-02 clinical study (ongoing)
- ✓ Retrospective natural history of ERT-treated patients (ongoing)
- ✓ Prospective data collection on current ERT-treated patients (ongoing)
- Initiation of larger registration-directed study (2H18)
- 18-month data from ATB200-02 clinical study (4Q18)



Biologics Manufacturing Progress & Upcoming Milestones

Maintaining Identical Key Quality Attributes Throughout Scale Up to 1000L

- ✓ Analytical and *in vivo* comparability studies completed between 250L and 1000L engineering batches
- ✓ FDA agreement on comparability between 250L GMP scale and 1000L engineering batches; and testing strategy for demonstrating comparability between 250L scale and 1000L GMP batches
- ✓ GMP Manufacturing Campaigns of Drug Substance and Drug Product at 1000L GMP Scale Successfully Completed
- ❑ Release of 1,000L GMP material for initiation of registration-directed study
- ❑ Final regulatory agreement on comparability between 1,000L and 250L GMP scale



Long-Term Biologics Manufacturing Strategy

Strategy for Added Capacity and Long-Term Commercial Manufacture

- ✓ Additional capacity at WuXi (China & Ireland) to ensure sufficient medicine to supply patient population
- ✓ Diligence ongoing for long-term U.S. commercial manufacture and capacity
- Project update anticipated 2H18

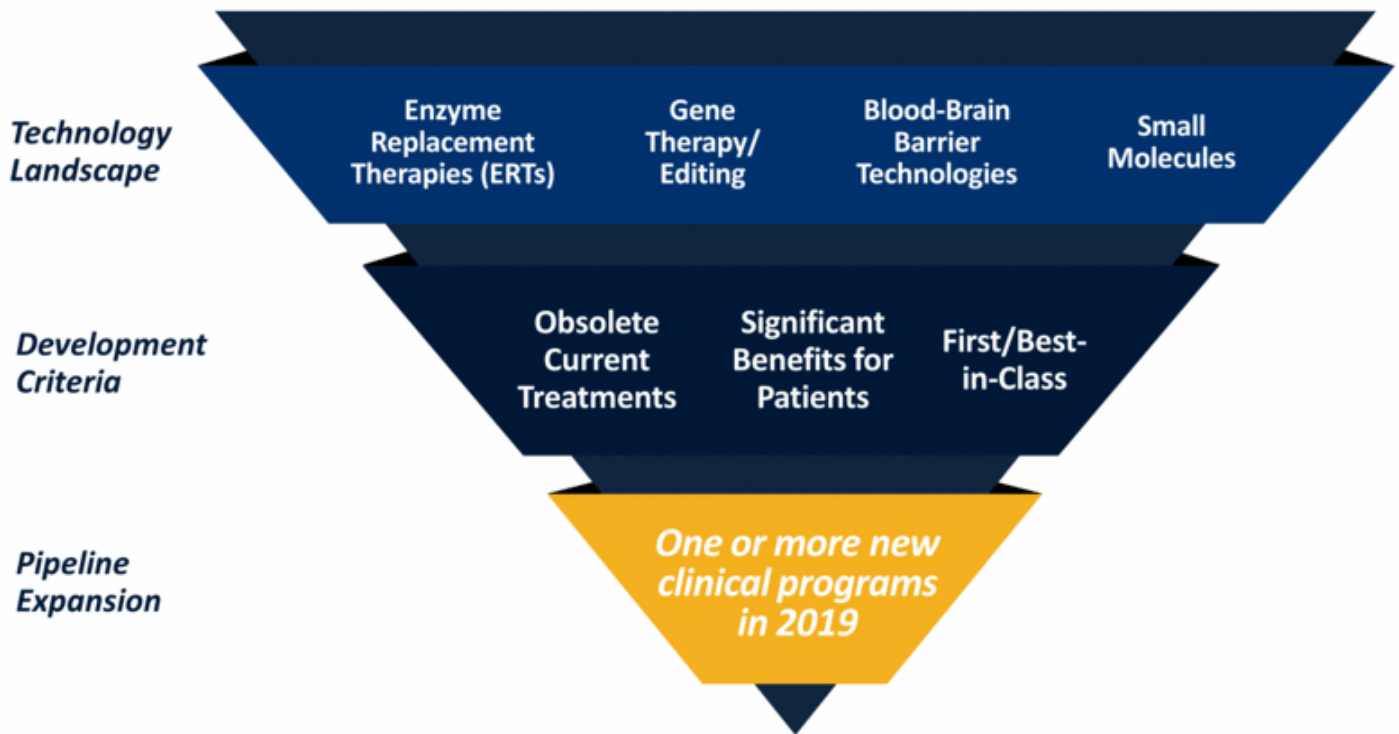


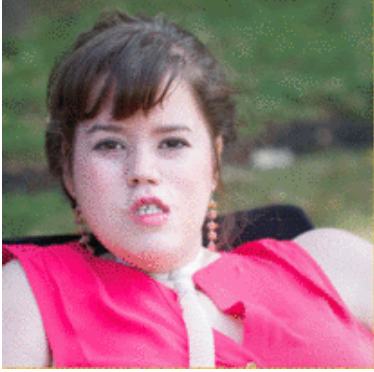
Pipeline Strategy

"We have a duty to obsolete our own technologies"
- Amicus Belief Statement

Pipeline Strategy

Sharply Focused on Developing Therapies for People Living with Rare Metabolic Diseases





Financial Summary and Upcoming Milestones

1Q18 Select Financial Results

1Q18 Revenue of \$16.7M from Sales of Galafold

(\$000s) except per share data	March 31, 2018	March 31, 2017
Product revenue	16,696	4,169
Cost of Goods Sold	2,615	775
R&D Expense	40,798	30,876
SG&A Expense	27,396	19,132
Changes in fair value of contingent consideration	1,100	4,578
Loss from operations	56,182	52,015
Change in fair value of derivatives	4,861	-
Income tax benefit (expense)	1,392	(56)
Net Loss	49,916	54,992
Net Loss Per Share	0.28	0.39

Financial Summary & Guidance

Strong Balance Sheet with \$605M Cash at 3/31/18 - Cash Runway into at Least 2021

FINANCIAL POSITION

March 31, 2018

Cash	\$605M
Debt	\$250M
Cash Runway¹	Into at least 2021

CAPITALIZATION

Shares Outstanding²	187,972,218
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FINANCIAL GUIDANCE

FY18 Net Cash Spend Guidance	\$230-\$260M
Galafold Revenue Guidance	\$75-\$85M

¹Based on existing operating plan for Fabry and Pompe programs. ²Includes shares from the February 2018 equity offering

2018 Key Strategic Priorities

Focused on FIVE Key Strategic Priorities in 2018

- 1 Double Galafold (migalastat) revenue to \$75-\$85M
- 2 Secure approvals for migalastat in Japan and the U.S.
- 3 Achieve clinical, manufacturing and regulatory milestones to advance AT-GAA* toward global regulatory submissions and approvals
- 4 Develop and expand preclinical pipeline to ensure at least one new clinical program in 2019
- 5 Maintain financial strength

*Advanced and Targeted GAA (AT-GAA, also known as ATB200/AT2221)

Thank You

“Our passion for making a difference unites us”

-Amicus Belief Statement

