

**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): **August 7, 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 August 7, 2018, Amicus Therapeutics, Inc. (the “Company”) issued a press release announcing its financial results for the second fiscal quarter, ended June 30, 2018. A copy of this press release is attached hereto as Exhibit 99.1. The Company will host a conference call and webcast on August 7, 2018 to discuss its second 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 Exhibits 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
<u>99.1</u>	<u>Press Release dated August 7, 2018.</u>
<u>99.2</u>	<u>August 7, 2018 Conference Call Presentation Materials</u>

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: August 7, 2018

AMICUS THERAPEUTICS, INC.

By: /s/ Ellen S. Rosenberg

Name: Ellen S. Rosenberg

Title: General Counsel and Corporate Secretary



Amicus Therapeutics Announces Second Quarter 2018 Financial Results and Corporate Updates

2Q18 Net Product Sales of \$21.3M Driven by Galafold® (Migalastat) Expansion

Company Increases FY18 Global Galafold Revenue Guidance to \$80M-\$90M

Pompe Clinical, Regulatory and Manufacturing Activities Continue to Advance

Conference Call and Webcast Today at 8:30am ET

CRANBURY, NJ, August 7, 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 second quarter ended June 30, 2018. The Company also summarized recent program updates, raised its full-year 2018 revenue guidance and updated its net cash spend guidance for the year.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, "In the first half of 2018, we have made tremendous progress advancing Galafold, expanding access to patients with amenable mutations across the world. Given the continued strong momentum of the Galafold launch, the early commercial launch in Japan, and the pending accelerated approval in the U.S., we are raising our 2018 revenue guidance to \$80 million to \$90 million. We have also continued to advance our Pompe clinical, manufacturing and regulatory activities and look forward to providing an update later this quarter on the pivotal study design and best and fastest regulatory path in the United States for this important program. Additionally, we are also extraordinarily focused on assembling a portfolio of technologies, programs and partnerships in the gene therapy space. Our goal beginning in the second half of this year is a bold one: to build one of the most robust gene therapy pipelines in the field of rare, metabolic disorders. 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."

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

- Total revenue in the second quarter 2018 was \$21.3 million, a year-over-year increase of 198% from total revenue of \$7.2 million in the second quarter of 2017.
- Cash, cash equivalents, and marketable securities totaled \$552.8 million at June 30, 2018, compared to \$358.6 million at December 31, 2017.
- Total operating expenses increased to \$65.1 million for the second quarter 2018 compared to \$53.2 million in the second quarter 2017, reflecting an increased investment in Pompe clinical and manufacturing activities as well as Galafold commercial launch and launch preparations.
- Net cash spend was \$51.0 million for the second quarter 2018. Net loss was \$61.8 million, or \$0.33 per share, for the second quarter 2018 compared to a net loss of \$48.1 million, or \$0.34 per share, for the second quarter 2017.

"The second quarter of 2018 marked another period of continued growth for Galafold," said Bradley L. Campbell, President and COO of Amicus Therapeutics. "We are raising our full-year 2018 Galafold global revenue guidance given the current and anticipated increase in patient and physician adoption in our existing markets, the ongoing launch in Japan, and our anticipated PDUFA date and launch in the U.S. this quarter. And we're pleased to announce that we've hired 100% of our US launch team comprised of passionate professionals with significant rare disease experience."

2018 Financial Guidance

For the full-year 2018 the Company is increasing its total Galafold revenue guidance to \$80 to \$90 million from the previous range of \$75 million to \$85 million. This reflects global revenue from all expected 2018 commercial markets.

Based on the increase in expected 2018 revenue and operating expenses that are trending favorably to budget, the Company is updating its full-year 2018 net cash spend to \$220 to \$250 million from the previous range of \$230 to \$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](#), the Company's first therapy, is an oral precision medicine. Regulatory authorities in Australia, Canada, EU, Israel, Japan, South Korea and Switzerland have granted full approval for migalastat under the trade name Galafold to treat Fabry disease in patients 16 years or older who have amenable genetic mutations. In the U.S., the FDA accepted the Company's new drug application (NDA) for migalastat under Subpart H 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 19 countries with first commercial patients treated in multiple new countries in 2018
- Approvals secured in Australia, Canada, EU, Israel, Japan, South Korea and Switzerland
- Commercial launch initiated in Japan during the second quarter
- U.S. leadership and field team now in place to support planned U.S. launch

Anticipated Milestones:

- U.S. FDA regulatory decision (3Q18)

AT-GAA 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 to stabilize ATB200 while in the circulation to deliver active therapeutic enzyme.

The Company is engaged in collaborative discussions with U.S. and EU regulators regarding a number of key topics including a registration-directed study for full approval, manufacturing activities, and the best and fastest pathway forward for this novel treatment regimen. The Company believes that the evolving regulatory paths in both the U.S. and EU will include a series of iterative discussions with regulators as the program advances and as additional data are collected, including data from existing patients in ongoing studies, data from new patients being enrolled into ongoing studies, and the results of a formal retrospective natural history study of Pompe patients receiving current standard of care ERT. Amicus expects to provide an FDA update in the third quarter 2018 after receipt of written minutes from a scheduled Type C meeting.

Manufacturing activities to support the needs of the Pompe community are also ongoing.

Pompe Manufacturing Updates:

- Release of 1,000L GMP material for use in the planned pivotal study.
- Feedback from German regulatory authorities (BfArM) indicating general agreement with the manufacturing strategy for ATB200, including the strategy to demonstrate comparability of drug substance and drug product between the 1,000 liter scale and the 250 liter scale.

Anticipated Pompe Program Milestones in 2H18:

- Pompe US regulatory update (3Q18).
- Completion of a retrospective natural history study in ~100 ERT-treated Pompe patients.
- 18-month data from ATB200-02 clinical study to be presented at the 23rd International Congress of the World Muscle Society (4Q18).
- Commence pivotal study in 2H18.

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, August 7, 2018, at 8:30 a.m. ET to discuss the second 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: 5887047.

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 August 7, 2018. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 5887047.

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-centric 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 Galafold, an oral precision medicine for people living with Fabry disease who have amenable genetic mutations. The lead biologics program in the Amicus pipeline is AT-GAA, a novel, late-stage treatment for Pompe disease. Amicus 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 as well as our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018 to be filed August 7, 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 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)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Revenue:				
Net product sales	\$ 21,309	\$ 7,158	\$ 38,005	\$ 11,327
Cost of goods sold	3,135	1,061	5,750	1,836
Gross Profit	<u>18,174</u>	<u>6,097</u>	<u>32,255</u>	<u>9,491</u>
Operating Expenses:				
Research and development	34,660	31,985	75,458	62,861
Selling, general and administrative	29,172	19,311	56,568	38,443
Changes in fair value of contingent consideration payable	300	1,050	1,400	5,628
Depreciation	973	812	1,942	1,636
Total operating expenses	<u>65,105</u>	<u>53,158</u>	<u>135,368</u>	<u>108,568</u>
Loss from operations	<u>(46,931)</u>	<u>(47,061)</u>	<u>(103,113)</u>	<u>(99,077)</u>
Other income (expense):				
Interest income	2,913	753	4,650	1,512
Interest expense	(4,560)	(4,179)	(9,048)	(8,469)
Change in fair value of derivatives	(7,600)	—	(2,739)	—
Other (expense) income	(5,316)	2,400	(2,554)	3,010
Loss before income tax	<u>(61,494)</u>	<u>(48,087)</u>	<u>(112,804)</u>	<u>(103,024)</u>
Income tax benefit (expense)	(339)	(49)	1,053	(105)
Net loss attributable to common stockholders	<u>\$ (61,833)</u>	<u>\$ (48,136)</u>	<u>\$ (111,751)</u>	<u>\$ (103,129)</u>
Net loss attributable to common stockholders per common share — basic and diluted	<u>\$ (0.33)</u>	<u>\$ (0.34)</u>	<u>\$ (0.61)</u>	<u>\$ (0.72)</u>
Weighted-average common shares outstanding — basic and diluted	188,621,423	143,000,718	182,303,128	142,886,614

TABLE 2

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

	June 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	\$ 73,311	\$ 49,060
Investments in marketable securities	465,641	309,502
Accounts receivable	15,077	9,464
Inventories	7,769	4,623
Prepaid expenses and other current assets	14,432	19,316
Total current assets	<u>576,230</u>	<u>391,965</u>
Investments in marketable securities	13,836	—
Property and equipment, less accumulated depreciation of \$14,415 and \$12,515 at June 30, 2018 and December 31, 2017, respectively	9,111	9,062
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	5,915	5,200
Total Assets	<u>\$ 825,889</u>	<u>\$ 627,024</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	\$ 41,613	\$ 53,890
Deferred reimbursements	2,750	7,750
Contingent consideration payable	8,600	8,400
Total current liabilities	<u>52,963</u>	<u>70,040</u>
Deferred reimbursements	14,156	14,156
Convertible notes	169,440	164,167
Contingent consideration payable	18,200	17,000
Deferred income taxes	6,465	6,465
Other non-current liability	2,770	2,346
Total liabilities	<u>263,994</u>	<u>274,174</u>
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$0.01 par value, 500,000,000 and 250,000,000 shares authorized, 189,053,214 and 166,989,790 shares issued and outstanding at June 30, 2018 and December 31, 2017, respectively	1,939	1,721
Additional paid-in capital	1,723,865	1,400,758
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	(1,539)	(1,659)
Unrealized gain on available-for-sale securities	(455)	(436)
Warrants	13,063	16,076
Accumulated deficit	<u>(1,174,978)</u>	<u>(1,063,610)</u>
Total stockholders' equity	<u>561,895</u>	<u>352,850</u>
Total Liabilities and Stockholders' Equity	<u>\$ 825,889</u>	<u>\$ 627,024</u>



2Q18 Financial Results & Corporate Highlights

August 7, 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 June 30, 2018 to be filed August 7, 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

As of January 2018

Focused on FIVE Key Strategic Priorities in 2018

- 1 Double Galafold (migalastat) revenue to \$75-\$85M (Now \$80-\$90M)
- 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

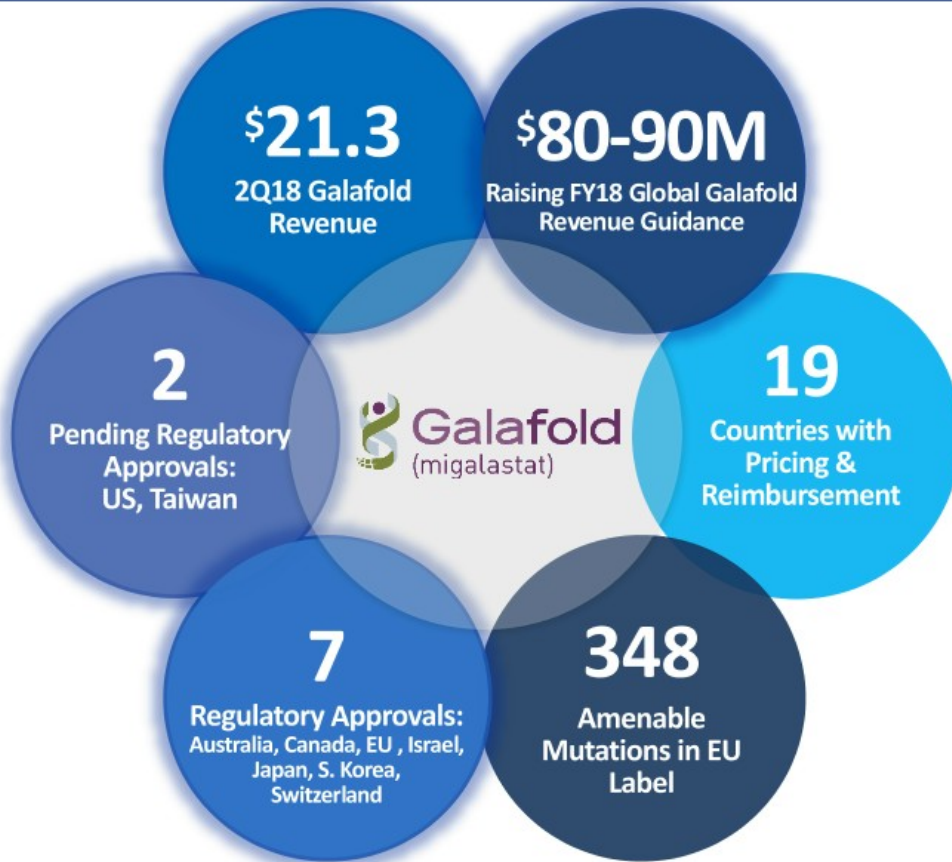
* AT-GAA, also known as ATB200/AT2221



Galafold™ (Migalastat) Precision Medicine for Fabry Disease

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

Galafold Snapshot (as of June 30, 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 -For important safety information for Galafold visit www.ema.europa.eu

Japan Launch Update

Japan Launch of Galafold Underway

- Full commercial team hired and trained
- First commercial patients commenced treatment in late 2Q18
- Successful launch symposium attended by 100+ HCPs
- >850 patients diagnosed (>700 treated with an ERT)*
- No ERT home infusion currently available
- Physicians tend to initiate treatment early



*Clinical & commercial, all figures approximate

U.S. Demographics for Galafold



Prepared for Launch Pending FDA Decision

NDA Filed (Priority Review)

August 13, 2018 PDUFA

Full launch team hired and trained

Appropriate stakeholder engagement ongoing

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

Orphan Drug and Fast Track designations

*Clinical & commercial, all figures approximate



Financial Summary

2Q18 Select Financial Results

2Q18 Revenue of \$21.3M from Sales of Galafold

(in thousands, except per share data)

	June 30, 2018	June 30, 2017
Product revenue	21,309	7,158
Cost of goods sold	3,135	1,061
R&D expense	34,660	31,985
SG&A expense	29,172	19,311
Changes in fair value of contingent consideration	300	1,050
Loss from operations	(46,931)	(47,061)
Change in fair value of derivatives	(7,600)	-
Income tax benefit (expense)	(339)	(49)
Net loss	(61,833)	(48,136)
Net loss per share	(0.33)	(0.34)

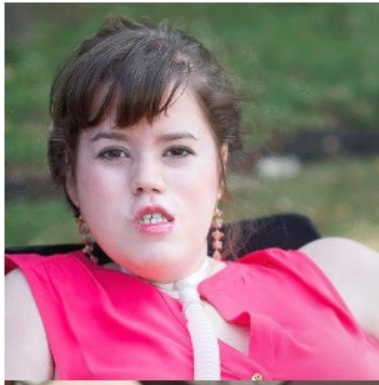
Financial Summary & Guidance

Strong Balance Sheet with \$553M Cash at 6/30/18- Cash Runway into at Least 2021

FINANCIAL POSITION	June 30, 2018
Cash	\$553M
Debt	\$250M
Cash Runway¹	Into at least 2021
CAPITALIZATION	
Shares Outstanding²	189,053,214
FINANCIAL GUIDANCE	
FY18 Net Cash Spend Guidance	\$220-\$250M
Galafold Revenue Guidance	\$80-\$90M

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





AT-GAA Novel ERT for Pompe Disease

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

Key Activities in 2018

Significant Progress toward Clinical, Regulatory, and GMP Manufacturing Activities in 2018 to Lay Foundation for Best and Fastest Approval Pathways

Year to Date Progress

CLINICAL

- ✓ Additional Phase 1/2 ATB200-02 extension data presented at *WORLD Symposium*
- ✓ Additional patients in Phase 1/2 ATB200-02 clinical study
- ✓ Initiation of retrospective natural history of ERT-treated patients
- ✓ Prospective data collection on current ERT-treated patients

REGULATORY

- ✓ EMA: Received Scientific Advice Working Party Guidance

MANUFACTURING

- ✓ Final regulatory agreement on comparability between 1,000L and 250L GMP scale
- ✓ Release for clinic of 1,000L GMP commercial scale material

Upcoming Milestones

CLINICAL

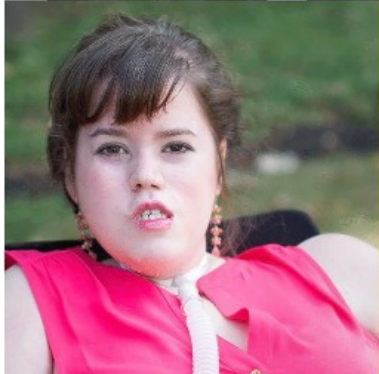
- ❑ 18-month data from ATB200-02 clinical study (4Q18)
- ❑ Completion of a retrospective natural history study (2H18)
- ❑ Initiation of larger registration-directed study (2H18)

REGULATORY

- ❑ U.S. FDA type C meeting and US update (3Q18)

MANUFACTURING

- ❑ Announce plan for long term commercial manufacturing



Pipeline Strategy and Upcoming Milestones

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

Pipeline Strategy

Developing Therapies for People Living with Rare Metabolic Diseases with a New Focus on Gene Therapy

Technology Focus

Gene Therapies

Development Criteria

Obsolete Current Treatments

Significant Benefits for Patients

First/Best-in-Class

Pipeline Expansion

One or more new clinical programs in 2019

2018 Key Strategic Priorities

As of August 2018

Focused on FIVE Key Strategic Priorities in 2018

- 1 Double Galafold (migalastat) revenue to \$80-\$90M
- 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

* AT-GAA, also known as ATB200/AT2221

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement



