

**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 5, 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 November 5, 2018, Amicus Therapeutics, Inc. (the “Company”) issued a press release announcing its financial results for the third fiscal quarter, ended September 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 November 5, 2018 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 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:**

<b>Exhibit No.</b>	<b>Description</b>
<a href="#">99.1</a>	<a href="#">Press Release dated November 5, 2018</a>
<a href="#">99.2</a>	<a href="#">November 5, 2018 Conference Call Presentation Materials</a>

**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: November 5, 2018

AMICUS THERAPEUTICS, INC.

By: /s/ Ellen S. Rosenberg

Name: Ellen S. Rosenberg

Title: General Counsel and Corporate Secretary



## Amicus Therapeutics Announces Third Quarter 2018 Financial Results and Corporate Updates

U.S. Galafold® (Migalastat) Fabry Launch Tracking Significantly Ahead of Expectations- 100+ Patients Prescribed Galafold Since August Launch

3Q18 Global Galafold Net Product Sales of \$20.6M Driven by Continued International Growth - 500+ International Fabry Patients Now on Galafold

Reaffirming Higher End of FY18 Revenue Guidance of \$80M-\$90M – Balance Sheet Strength Sufficient to Fund Operations into at least 2021

Gene Therapy Pipeline Provides 14 New Programs and Future Growth Platform

Conference Call and Webcast Today at 8:30am ET

CRANBURY, NJ, November 5, 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 third quarter ended September 30, 2018. The Company also summarized recent program updates, reiterated its full-year 2018 revenue guidance and reduced its net cash spend guidance for the year.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “The third quarter marked a major transformation for Amicus that brings us several steps closer toward our 2023 vision to treat at least 5,000 patients and to achieve \$1 billion in annual global revenue. In the last three months, we received our first U.S. drug approval for Galafold, expanded our pipeline to include 14 new gene therapy programs for rare metabolic diseases, and presented positive 18-month data for our differentiated Pompe treatment paradigm. With a strong revenue base and \$500M+ peak sales opportunity for Galafold, as well as \$564 million in cash, we have never been in a stronger position to advance our robust portfolio to drive significant value for shareholders and the patient communities that we serve.”

### Third Quarter 2018 Financial Results and Full-Year 2018 Financial Guidance

- Total revenue in the third quarter 2018 was \$20.6 million, a year-over-year increase of 89% from total revenue of \$10.9 million in the third quarter of 2017. Third quarter revenue was impacted by uneven ordering patterns over the summer months in Europe.
- Cash, cash equivalents, and marketable securities totaled \$564.4 million at September 30, 2018, compared to \$358.6 million at December 31, 2017.
- Total operating expenses for the third quarter 2018 were \$172.5 million compared to \$284.3 million in the third quarter 2017. The decrease is due primarily to a non-cash impairment charge incurred in 3Q17, partially offset by an upfront payment of \$100 million for the Celenex asset acquisition which was reflected in the third quarter 2018 as a research and development expense.
- Net cash spend was \$35.2 million for the third quarter 2018. Net loss was \$159.2 million, or \$0.84 per share, for the third quarter 2018 compared to a net loss of \$111.7 million, or \$0.69 per share, for the third quarter 2017. Total net operating expenses were \$172.5 million, which includes the \$100 million Celenex asset acquisition cost.

“We are very pleased with the momentum of the global Galafold launch,” said Bradley L. Campbell, President and Chief Operating Officer of Amicus Therapeutics. “We see continued strong uptake and growth, along with very high compliance and adherence to this new oral Fabry treatment option, in both patients who are ERT-experienced and an increasing number who are ERT-treatment naive in Europe. As

anticipated, quarter-over-quarter revenue reflect some uneven ordering patterns as well as a rising number of extended, 90-day prescriptions as we headed into the summer months, which have normalized since the start of the fourth quarter. These adoption trends may reflect the emerging Fabry treatment paradigm with a stable oral medication that can be taken during summer travels and on holidays. Japan is also off to a solid start, now with a double-digit number of patient prescriptions. Importantly, the first 12 weeks of the U.S. launch have significantly exceeded our expectations. With more than 100 individual prescriptions for Galafold in the U.S., we are seeing robust patient demand from a broad prescriber base of more than 40 Fabry physicians. Given this global momentum, we are confident in meeting the higher end of our full-year 2018 guidance and setting a solid foundation for 2019."

#### 2018 Financial Guidance

For the full-year 2018 the Company reiterated its total Galafold revenue guidance to \$80 to \$90 million. This reflects global revenue from all expected 2018 commercial markets. The Company is lowering its full-year 2018 net cash spend to \$190 to \$210 million from the previous range of \$220 to \$250 million. The current cash position, including Galafold revenues, is sufficient to fund ongoing operations into at least 2021. Potential future business development collaborations, pipeline expansion, and investment in manufacturing capabilities could impact the Company's future capital requirements.

#### Program Highlights

##### Galafold (Migalastat) Oral Precision Medicine for Fabry Disease

Galafold is an oral precision medicine for Fabry disease approved in the EU and other geographies to treat Fabry disease in patients 16 years or older who have amenable genetic mutations. The U.S. FDA approved Galafold under Subpart H for the treatment of adult patients with a confirmed diagnosis of Fabry disease and an amenable genetic variant. For patients who are not suitable for treatment with Galafold on the basis of their genetic mutations, or variants, Amicus is advancing a next-generation gene therapy.

##### Global Galafold Updates:

- U.S. FDA approval on August 10, 2018
- U.S. launch tracking ahead of internal expectations with 103 new patient prescriptions, also known as patient referral forms (PRFs), as of October 31, 2018. Time to shipment is approximately 60 days, limiting 2018 revenue impact but providing a strong foundation for 2019.
- Launched in Australia on November 1 following formal listing on life saving drugs program
- Pricing and reimbursement secured in 22 countries
- Approvals secured in eight total geographies including Australia, Canada, EU, Israel, Japan, South Korea, Switzerland, and United States and pending in Taiwan

##### 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.

Positive results from a global Phase 1/2 clinical study (ATB200-02) have shown consistent and durable responses across key measures of safety, functional outcomes and biomarkers in both ERT-switch and ERT-naïve Pompe patients following up to 18 months of treatment with AT-GAA. The Company's strategy is to enhance the body of clinical data for AT-GAA in ongoing studies and the upcoming pivotal study (PROPEL, also referred to as ATB200-03) to deliver this potential new therapy to as many people living with Pompe disease as soon as possible.

##### Recent and Anticipated AT-GAA Program Milestones:

- ✓ Positive 18-month data from ATB200-02 Phase 1/2 clinical study at World Muscle Society
- ✓ 1,000L GMP material released for pivotal study.



- Initiation of PROPEL pivotal study to support full approval in U.S. and EU, and other geographies (4Q18)
- Completion of retrospective natural history study in approximately 100 ERT-treated Pompe patients (4Q18)
- Additional ATB200-02 study data from up to 10 additional ERT-switch patients in Cohort 4 (2019)
- Initiation of studies in additional patient populations, including pediatric patients (2019)
- Update on long-term manufacturing strategy

As part of the Company's long-term commitment to provide multiple solutions to address the significant unmet needs of the Pompe community, Amicus is also advancing a next-generation gene therapy as a potential cure for Pompe disease.

**Gene Therapy Portfolio: 14 New Programs for Rare Metabolic Diseases**

During the third quarter and early fourth quarter, Amicus expanded its pipeline [link [here](#)] to include 14 new gene therapy programs and future growth platform for rare metabolic diseases. The Company [acquired](#) 10 preclinical and clinical stage adeno associated virus (AAV) programs (intrathecal delivery) for neurologic lysosomal storage disorders (LSDs) currently in development at Nationwide Children's Hospital. In [collaboration](#) with the University of Pennsylvania, the Company is advancing four next-generation AAV gene therapies for Fabry disease, Pompe disease, CDKL5 deficiency disorder (CDD) and one additional undisclosed rare metabolic disorder.

Additional details are available in the third quarter 2018 results slide presentation at [www.amicusrx.com](http://www.amicusrx.com) in the Investors & Media section [link [here](#)].

**Gene Therapy Pipeline Highlights:**

- Batten Disease: Compelling proof-of-concept demonstrated in preclinical studies in CLN6, CLN3, and CLN8, as well as initial clinical safety and efficacy in a Phase 1/2 study in patients with CLN6.
- Pompe Disease: Early proof-of-principle for Amicus DNA constructs for an optimized gene therapy to address all aspects of Pompe disease including the central nervous system, heart, and muscles.
- Fabry Disease: Early proof-of-principle for Amicus DNA constructs for an optimized gene therapy to deliver stable, active enzyme to lysosomes.

**Upcoming Gene Therapy Pipeline Milestones in 2018 and 2019:**

- First Patient in CLN3 Batten disease Phase 1/2 Study (4Q18)
- Completion of enrollment in CLN6 Batten disease Phase 1/2 study
- Preliminary data from CLN6 Batten disease Phase 1/2 study
- Enrollment of full initial cohort in CLN3 Batten disease Phase 1/2 study
- Preclinical data for next-generation gene therapies for Fabry, Pompe and CDD
- Preclinical work across additional neurologic LSDs

**Conference Call and Webcast**

Amicus Therapeutics will host a conference call and audio webcast today, November 5, 2018, at 8:30 a.m. ET to discuss the third 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: 2292105.

An audio webcast and slide presentation 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 November 5, 2018. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 2292105.

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### About Galafold

Galafold™ (migalastat) 123 mg capsules is an oral pharmacological chaperone of alpha-Galactosidase A (alpha-Gal A) for the treatment of Fabry disease in adults who have amenable GLA variants. In these patients, Galafold works by stabilizing the body's own dysfunctional enzyme so that it can clear the accumulation of disease substrate. Globally, Amicus Therapeutics estimates that approximately 35 to 50 percent of Fabry patients may have amenable GLA variants, though amenability rates within this range vary by geography. Galafold is approved in Australia, Canada, European Union, Israel, Japan, South Korea, Switzerland and the U.S.

### U. S. INDICATIONS AND USAGE

Galafold is indicated for the treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data.

This indication is approved under accelerated approval based on reduction in kidney interstitial capillary cell globotriaosylceramide (KIC GL-3) substrate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

### U.S. IMPORTANT SAFETY INFORMATION

#### ADVERSE REACTIONS

The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia.

#### USE IN SPECIFIC POPULATIONS

There is insufficient clinical data on Galafold use in pregnant women to inform a drug-associated risk for major birth defects and miscarriage. Advise women of the potential risk to a fetus.

It is not known if Galafold is present in human milk. Therefore, the developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for Galafold and any potential adverse effects on the breastfed child from Galafold or from the underlying maternal condition.

Galafold is not recommended for use in patients with severe renal impairment or end-stage renal disease requiring dialysis.

The safety and effectiveness of Galafold have not been established in pediatric patients.

To report Suspected Adverse Reactions, contact Amicus Therapeutics at 1-877-4AMICUS or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

For additional information about Galafold, including the full U.S. Prescribing Information, please visit <https://www.amicusrx.com/pi/Galafold.pdf>.

### 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.
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- Contraindications to Galafold include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on Galafold or switched to Galafold.
- OVERDOSE: General medical care is recommended in the case of Galafold overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received Galafold. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at [www.ema.europa.eu](http://www.ema.europa.eu).

#### About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-centric biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases. For more information please visit the company's website at [www.amicusrx.com](http://www.amicusrx.com), and follow us on [Twitter](#) and [LinkedIn](#).

#### 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 September 30, 2018 to be filed November 6, 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.

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CONTACTS:

Investors/Media:

Amicus Therapeutics

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Jennifer Paganelli

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(347) 658-8290

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TABLE 1

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue:				
Net product sales	\$ 20,596	\$ 10,874	\$ 58,601	\$ 22,201
Cost of goods sold	4,310	1,790	10,060	3,626
Gross Profit	<u>16,286</u>	<u>9,084</u>	<u>48,541</u>	<u>18,575</u>
Operating Expenses:				
Research and development	138,227	40,641	213,685	103,502
Selling, general and administrative	31,867	21,647	88,435	60,090
Changes in fair value of contingent consideration payable	1,300	(244,250)	2,700	(238,622)
Loss on impairment of assets	—	465,427	—	465,427
Depreciation	1,073	851	3,015	2,486
Total operating expenses	<u>172,467</u>	<u>284,316</u>	<u>307,835</u>	<u>392,883</u>
Loss from operations	<u>(156,181)</u>	<u>(275,232)</u>	<u>(259,294)</u>	<u>(374,308)</u>
Other income (expense):				
Interest income	2,721	1,190	7,371	2,702
Interest expense	(4,715)	(4,351)	(13,763)	(12,820)
Change in fair value of derivatives	—	—	(2,739)	163
Other (expense) income	(1,039)	2,044	(3,593)	4,891
Loss before income tax	<u>(159,214)</u>	<u>(276,349)</u>	<u>(272,018)</u>	<u>(379,372)</u>
Income tax benefit	51	164,683	1,104	164,578
Net loss attributable to common stockholders	<u>\$ (159,163)</u>	<u>\$ (111,666)</u>	<u>\$ (270,914)</u>	<u>\$ (214,794)</u>
Net loss attributable to common stockholders per common share — basic and diluted	<u>\$ (0.84)</u>	<u>\$ (0.69)</u>	<u>\$ (1.47)</u>	<u>\$ (1.44)</u>
Weighted-average common shares outstanding — basic and diluted	189,162,841	160,796,841	184,606,790	148,963,864

TABLE 2

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

	September 30, 2018	December 31, 2017
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 201,827	\$ 49,060
Investments in marketable securities	362,556	309,502
Accounts receivable	14,189	9,464
Inventories	6,311	4,623
Prepaid expenses and other current assets	16,151	19,316
<b>Total current assets</b>	<u>601,034</u>	<u>391,965</u>
Property and equipment, less accumulated depreciation of \$15,483 and \$12,515 at September 30, 2018 and December 31, 2017, respectively	10,659	9,062
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	6,099	5,200
<b>Total Assets</b>	<u>\$ 838,589</u>	<u>\$ 627,024</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	\$ 54,330	\$ 53,890
Deferred reimbursements	2,750	7,750
Contingent consideration payable	8,800	8,400
<b>Total current liabilities</b>	<u>65,880</u>	<u>70,040</u>
Deferred reimbursements	14,156	14,156
Convertible notes	172,186	164,167
Senior secured term loan	146,622	—
Contingent consideration payable	19,300	17,000
Deferred income taxes	6,465	6,465
Other non-current liabilities	3,029	2,346
<b>Total liabilities</b>	<u>427,638</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, 189,254,341 and 166,989,790 shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively	1,941	1,721
Additional paid-in capital	1,731,174	1,400,758
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	(875)	(1,659)
Unrealized gain on available-for-sale securities	(211)	(436)
Warrants	13,063	16,076
Accumulated deficit	(1,334,141)	(1,063,610)
<b>Total stockholders' equity</b>	<u>410,951</u>	<u>352,850</u>
<b>Total Liabilities and Stockholders' Equity</b>	<u>\$ 838,589</u>	<u>\$ 627,024</u>

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# 3Q18 Financial Results & Corporate Highlights

November 5, 2018





## Forward Looking Statements

*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 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 September 30, 2018 to be filed November 6, 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.*

# Amicus Today

 **Galafold™**  
(migalastat)

First Oral Precision  
Medicine for Fabry Disease



**500+**  
**EMPLOYEES**  
globally

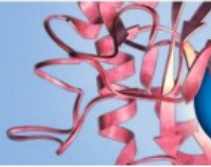


**PORTFOLIO**

of 15 programs for rare  
metabolic diseases

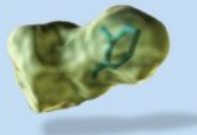
**BIOLOGICS**  
PLATFORM

Protein Engineering  
& Glycobiology



**AT-GAA\***

Investigational  
Therapy for  
Pompe Entering  
Phase 3



**~\$564M**  
Cash  
(9/30/18)

**Gene  
Therapy  
Platforms**

**GLOBAL  
FOOTPRINT**  
in 27 countries



Leading Expertise in  
**Lysosomal  
Storage  
Disorders**



\* AT-GAA, also known as ATB200/AT2221

# Corporate Highlights: 3Q18 and Early 4Q18

» **Well Capitalized to Advance Toward 2023 Vision: 5,000+ Patients & \$1B+ in Revenue**

» **Current Cash Position is Sufficient to Fund Operations into at least 2021**

» **Galafold: International Growth and Strong U.S. Launch Momentum**

- U.S. launch exceeding expectations following August 2018 approval; now reimbursed in 22 countries
- 3Q18 revenue of \$20.6M – on track to meet \$80M-90M FY18 guidance range
- \$500M+ peak revenue potential; \$1B+ cumulative revenue from 2019E-2023E to drive R&D engine

» **AT-GAA: Positive 18-month Data Presented World Muscle Society (October 2018)**

- Highly differentiated ERT with potential to be the future standard of care
- On track to initiate pivotal study by YE18
- \$1B+ peak revenue potential

» **NEW Gene Therapy Portfolio for 14 Rare Metabolic Diseases**

- Industry leading Batten disease portfolio: Two clinical stage programs (CLN6 and CLN3); One preclinical (CLN8)
- Preclinical AAV (intrathecal) gene therapy programs for 7 additional neurologic LSDs
- Next-generation preclinical gene therapies for Fabry, Pompe, CDKL5 and one other indication
- \$1B+ peak revenue potential

# Robust Rare Disease Portfolio



Advancing one of the most robust rare disease portfolios in biotechnology





# Galafold<sup>®</sup> (Migalastat) Precision Medicine for Fabry Disease



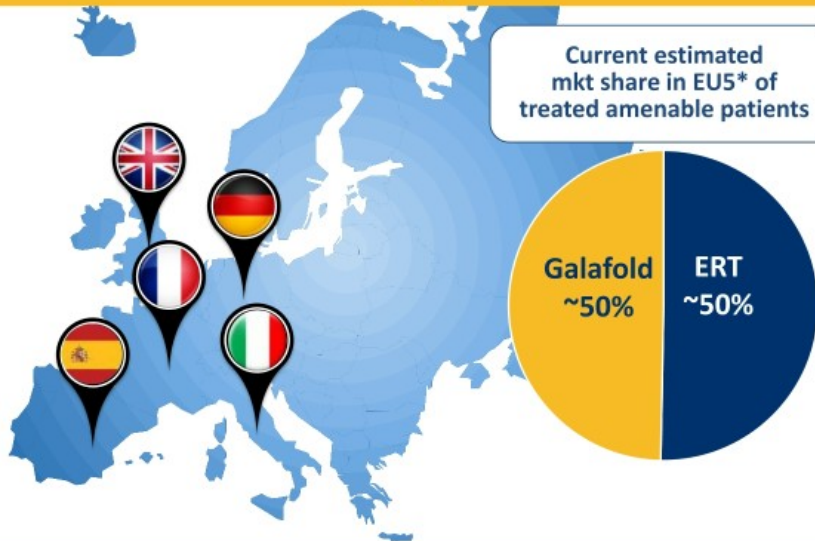
# Galafold Snapshot (as of November 5, 2018)

## FIRST Oral Precision Medicine for Fabry Disease Patients with Amenable Variants



# International Update (as of October 31, 2018)

## Continuing to Execute on Our Strategy with High Compliance and Adherence Among 500+ International Patients on Galafold



### MARKET DYNAMICS

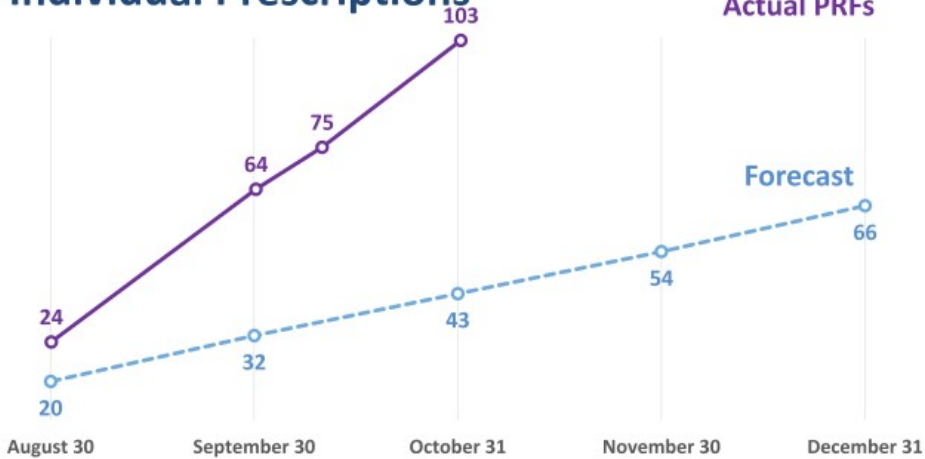
- Continued strong uptake and growth in ERT-switch patients; increasing number of previously untreated patients
- Very high rates of adherence and compliance (>90%)
- Balanced mix of males and females, classic and late-onset patients
- Oral ROA allows for new ordering patterns
- Continued high interest from physician community
- 145 HCPs attended inaugural Amicus *Fabry Connections* meeting in Madrid, Spain

\*Market share assumptions based on estimated number of treated amenable patients in EU5 as of October 2018

## Key U.S. Launch Metric – Individual Prescriptions (Patient Referral Forms)

**103 Individual Prescriptions (10/31/18) Significantly Exceeds Internal Forecast and Provides Strong Foundation for 2019**

### Individual Prescriptions

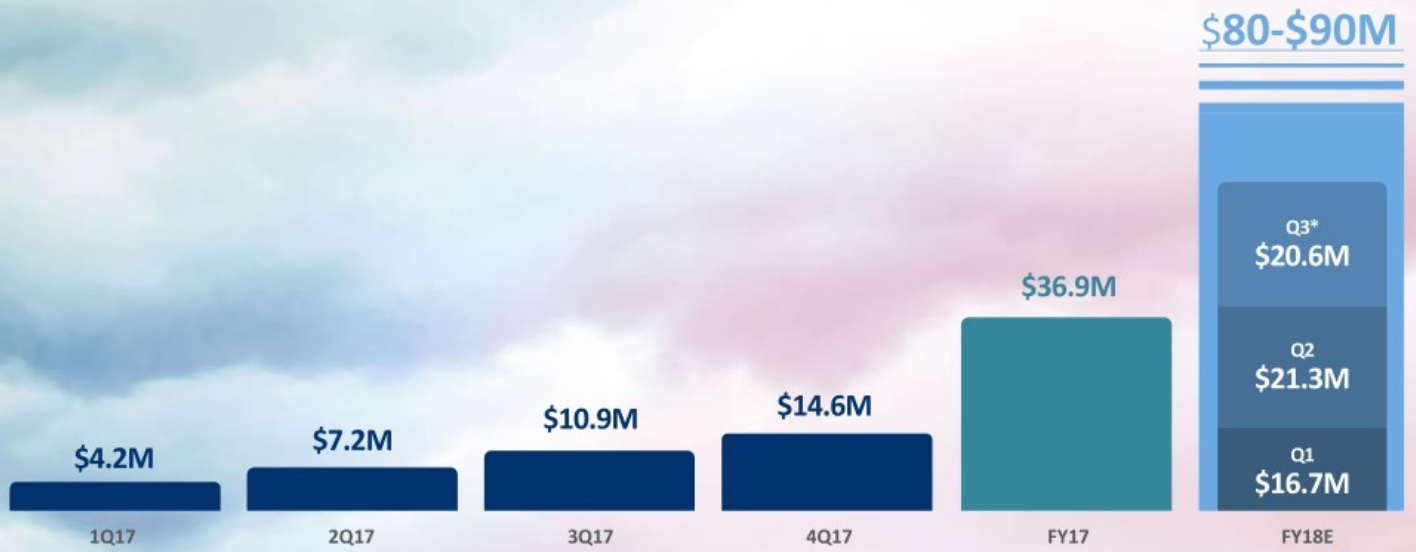


### Market Dynamics

- Strong patient and physician demand
- High conversion of study patients
- Growing prescriber base of 40+ physicians
- Patient demographics in line with launch strategy
- ~60 day average PRF to shipment limits FY18 impact
- Solid foundation for 2019

# Galafold Success and FY18 Galafold Revenue Guidance

On Track to Achieve Higher End of FY2018 Revenue Guidance of \$80-\$90M



\*QoQ revenue reflects new ordering patterns



# Financial Summary



## 3Q18 Select Financial Results

### 3Q18 Revenue of \$20.6M Primarily from International Galafold Sales

<i>(in thousands, except per share data)</i>	<b>Sept. 30, 2018</b>	<b>Sept. 30, 2017</b>
Product revenue	20,596	10,874
Cost of goods sold	4,310	1,790
R&D expense	138,227*	40,641
SG&A expense	31,867	21,647
Changes in fair value of contingent consideration	1,300	(244,250)
Loss on impairment of assets	-	465,427
Loss from operations	(156,181)	(275,232)
Income tax benefit	51	164,683
Net loss	(159,163)	(111,666)
Net loss per share	(0.84)	(0.69)

\*Inclusive of upfront payment of \$100 million for the Celenex asset acquisition

# Financial Summary & Guidance

**Strong Balance Sheet with \$564M Cash at 9/30/18 - Cash Runway into at Least 2021**

FINANCIAL POSITION	September 30, 2018
<b>Cash</b>	\$564M
<b>Debt</b>	\$319M
<b>Cash Runway<sup>1</sup></b>	Into at least 2021
CAPITALIZATION	
<b>Shares Outstanding<sup>2</sup></b>	189,254,341
FINANCIAL GUIDANCE	
<b>FY18 Net Cash Spend Guidance</b>	\$190M-\$210M
<b>Galafold Revenue Guidance</b>	\$80-\$90M

<sup>1</sup>Based on existing operating plan. <sup>2</sup>Includes shares from the February 2018 equity offering



# AT-GAA Novel ERT for Pompe Disease

## AT-GAA 18-Month Clinical Data Summary (ATB200-02 Study)

### Consistent and Durable Responses Across Key Measures of Safety, Functional Outcomes and Biomarkers in both ERT-Switch and ERT-Naïve Pompe Patients out to Month 18

- 6-minute walk test (6MWT) showed continued benefit in ERT-naïve and ERT-switch patients
- Timed motor function tests generally consistent with 6MWT results in both ambulatory cohorts
- Muscle strength increased in all cohorts, including nonambulatory ERT-switch patients
- Pulmonary function
  - Forced vital capacity (FVC), maximal inspiratory pressure (MIP), and maximal expiratory pressure (MEP) generally increased in ERT-naive patients
  - FVC, MIP, and MEP were generally stable in ERT-switch patients
- Fatigue severity scale
  - Improvement in fatigue score was observed in all cohorts
- Biomarkers and safety
  - Creatine kinase (CK) and urine hexose tetrasaccharide (Hex4) levels decreased in all cohorts
  - AT-GAA (ATB200/AT2221) was generally well tolerated
  - Adverse Events Generally Mild and Transient
- Very low rates of IARs (<1%) after 890+ total infusions across all cohorts

## Key Activities in 2018

### Significant Progress in Clinical, Regulatory, and GMP Manufacturing Activities in 2018

#### Year-to-Date Progress

##### CLINICAL

- Addt'l. Phase 1/2 ATB200-02 extension data presented at *WORLDSymposium*
- Addt'l. patients in Phase 1/2 ATB200-02 clinical study
- Initiation of retrospective natural history of ERT-treated patients
- 18-month data from ATB200-02 clinical study (4Q18)
- Initiation of larger registration-directed study
- Completion of a retrospective natural history study (4Q18)

##### REGULATORY

- EMA: Received Scientific Advice Working Party Guidance
- U.S. FDA type C meeting and U.S. update

##### MANUFACTURING

- Final FDA agreement on comparability between 1,000L and 250L GMP scale
- German regulatory authorities (BfArM) agreement on strategy to demonstrate comparability between 1,000L and 250L GMP scale
- Release for clinic of 1,000L GMP commercial scale material
- Announce plan for long-term commercial manufacturing





# Gene Therapy Pipeline

# Leading Gene Therapy Portfolio in Lysosomal Storage Disorders

License Through Nationwide Children’s Hospital and Collaboration with Penn  
Combine with Successful Amicus Development and Commercial Track Record in LSDs

- Ground-Breaking, Clinically Validated Science
- 14 Gene Therapy Programs
- Expertise and Relationships in Gene Therapy
- Compelling Data in Three Lead Batten Disease Programs; Earlier-Stage Fabry and Pompe Programs
- Leading Gene Therapy Portfolio in Lysosomal Storage Disorders

## Amicus Gene Therapy Portfolio

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3
CLN6 Batten Disease	NCH			
CLN3 Batten Disease	NCH			
CLN8 Batten Disease	NCH			
Fabry Gene Therapy	PENN			
Pompe Gene Therapy	PENN			
Neimann-Pick C	NCH			
Wolman Disease	NCH			
Tay-Sachs	NCH			
Multiple Other CNS LSDs	NCH			
CDKL5 Gene Therapy / ERT	PENN			
Other	PENN			



# Platform Proof-of-Concept for Lead Batten Disease Programs

**CLN6 and CLN3 Programs are Clinical Stage; CLN8 has Definitive Preclinical Efficacy Data in a Mouse Model of Disease – All Following Single AAV Intrathecal Administration**

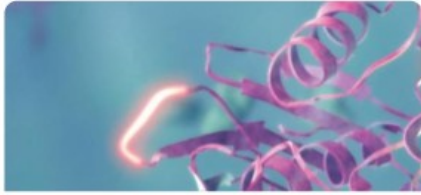
## PRECLINICAL MOUSE MODEL DATA

	Storage Material & Glial Activation	Motor & Cognitive Function	Survival	Safety & Brain Expression in NHP	GMP Clinical Supply	IND Active	Preliminary Clinical Data
<b>CLN6</b>	✓	✓	✓	✓	✓	✓	✓
<b>CLN3</b>	✓	✓	N/A*	✓	✓	✓	Pending
<b>CLN8</b>	✓	✓	✓	Pending	Pending	Pending	Pending

\*CLN3 mouse model does not have impaired survival

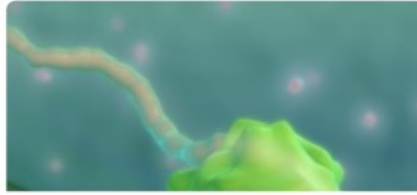
# Amicus Protein Engineering Expertise & Technologies for Gene Therapy

Collaboration with Penn to Enable Greater Protein Expression and Delivery at Lower Gene Therapy Doses for Fabry, Pompe, CDKL5 Deficiency Disorder and 1 Additional Indication



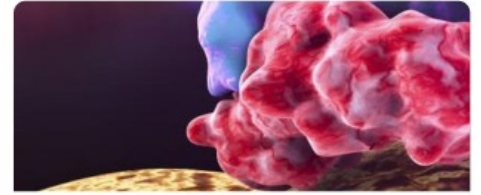
## Increased Protein Expression

Novel untranslated sequences to avoid inhibition of initiation and drive efficient protein synthesis



## Increased Protein Secretion

Effective signal sequences to increase protein expression & secretion



## Improved Protein Targeting and Stabilization

Targeting moieties  
Protein design



# Closing Remarks

John F. Crowley

## 2018 Key Strategic Priorities

On Track to Achieve All FIVE Key Strategic 2018 Priorities Outlined in January

- 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



# Amicus Vision: Delivering for Patients and Shareholders

To build a top-tier, fully integrated, global biotechnology company whose medicines treat 5,000+ patients with \$1B+ in worldwide sales revenue by 2023



>350 Patients\* | \$36.9M Global Sales

YE17



5,000 Patients\* | \$1B Global Sales

2023

\*Clinical & commercial, all figures approximate



Thank You



