

**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): **February 28, 2019**



**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 February 28, 2019, Amicus Therapeutics, Inc. (the “Company”) issued a press release announcing its financial results for the fiscal year ended December 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 February 28, 2019 to discuss its full year results of operations. A copy of the conference call presentation materials is attached hereto as Exhibit 99.2. Both exhibits are incorporated herein by reference.

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 February 28, 2019</a>
<a href="#">99.2</a>	<a href="#">February 28, 2019 Conference Call Presentation Materials</a>

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: February 28, 2019

AMICUS THERAPEUTICS, INC.

By: /s/ Ellen S. Rosenberg

Name: Ellen S. Rosenberg

Title: General Counsel and Corporate Secretary

# Amicus Therapeutics Announces Full-Year 2018 Financial Results and 2019 Corporate Updates

*Continued Strong Galafold Launch Trends in Early 2019 - On Track to Achieve FY19 Revenue Guidance of \$160M-\$180M*

*FY18 Galafold Revenue of \$91.2M Reflects 650+ Fabry Patients Treated at end of 2018 - 1,000+ Patients Expected by end of 2019*

*Clinical Data Out to Month 24 and Breakthrough Therapy Designation in Late Onset Pompe Disease Continue to Support AT-GAA as Next Potential Standard of Care*

*Amicus Global Research and Gene Therapy Center of Excellence in Philadelphia to Further Strengthen Leadership in Gene Therapy for Rare Diseases*

*Strong Balance Sheet with \$500M+ Cash*

*Conference Call and Webcast Today at 8:30 a.m. ET*

CRANBURY, NJ, February 28, 2019 – [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 full year ended December 31, 2018. The Company also summarized recent program updates and reiterated its full-year 2019 guidance.

## Corporate Highlights for Full-Year 2018 and Year-to-Date 2019

- **More than doubled global revenue for Galafold (migalastat) in 2018.** As [previously announced](#), revenue grew from \$36.9 million in full-year 2017 to \$91.2 million in full-year 2018, exceeding the high end of the full-year 2018 guidance range of \$80 million to \$90 million.
- **Strong Launch Trends Continue in Early 2019.** On track to achieve full-year 2019 revenue guidance of \$160M-\$180M and 1,000+ patients on Galafold by year-end.
- **Updated AT-GAA clinical data in Pompe disease presented at WORLDSymposium™ in February 2019.** As [previously announced](#), consistent and durable responses continued across key measures of safety, functional outcomes and biomarkers for both ERT-naïve and ERT-switch patients treated with AT-GAA for up to 24 months in the ongoing Phase 1/2 clinical study.
- **AT-GAA received Breakthrough Therapy Designation (BTD) in late onset Pompe disease.** The [BTD](#) was based on clinical efficacy results from the ongoing ATB200-02 Phase 1/2 clinical study, and further strengthens the Company's conviction in the potential for AT-GAA to become the next standard of care.
- **Enrollment momentum in ongoing pivotal PROPEL study in Pompe disease and Phase 1/2 study in CLN3 Batten disease.** The PROPEL study is on track to achieve full enrollment by year-end 2019. The first patient remains in the CLN3 Batten disease study with no serious adverse events after more than two months following a single administration of AAV9-CLN3 gene therapy. Further patient dosing is expected in the coming months.
- **Robust gene therapy pipeline continues to advance toward important data milestones.** Additional two-year data from Phase 1/2 study in CLN6 Batten disease expected mid-year. Preclinical proof-of-concept for Fabry and Pompe gene therapies anticipated throughout 2019.
- **New Global Research and Gene Therapy Center of Excellence in Philadelphia.** This new headquarters for the global Amicus science organization and the gene therapy leadership team advances the Company's commitment to world-class science.
- **Strong financial position to continue executing the Galafold launch and advance development programs.** The current cash position of approximately \$504.2 million at December 31, 2018 is expected to fund ongoing operations into at least mid-2021.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, "Over the course of last year and into early 2019 we have made great strides in our continued evolution as a leading global rare disease biotechnology company. We are on track and well-capitalized to achieve all of our 2019 key strategic priorities including our global Fabry launch, Pompe late-stage development program, and gene therapy pipeline. With a very successful, now global, commercial precision medicine product in Fabry disease, a late stage program with BTD in late onset Pompe disease and 14 Gene Therapy programs for rare diseases in development, including two in the clinic, I believe that we are now, more than ever before, strongly positioned to achieve our vision. We endeavor to deliver groundbreaking new medicines and hopefully one day cures for people living with rare metabolic diseases and to be one of a handful of leading companies in the world in this field. Achieving this vision will create enormous value for people with these life-threatening conditions as well as our shareholders."

## Full-Year 2018 Financial Results

- Total revenue in the full-year 2018 was \$91.2 million, an increase from total revenue of \$36.9 million in the full-year 2017.
- Cash, cash equivalents, and marketable securities totaled \$504.2 million at December 31, 2018 compared to \$358.6 million at December 31, 2017.
- Total operating expenses decreased to \$405.6 million for the full-year 2018 compared to \$472.7 million in the full-year 2017. Operating expenses reflecting increased investments in the Galafold launch, Pompe program, and gene therapy pipeline.
- Net cash spend was \$189.3 million for the full-year 2018, which was below full-year 2018 net cash spend guidance of \$200 million to \$225 million and reflects careful expense management.
- Net loss was \$349.0 million, or \$1.88 per share, compared to a net loss of \$284.0 million, or \$1.85 per share, for the full-year 2017.

## 2019 Key Strategic Priorities

- Nearly double again, annual revenue for Galafold (FY19 guidance of \$160M-\$180M in worldwide revenue) with 1,000+ Fabry patients on Galafold by year end.
- Complete enrollment in pivotal study in Pompe disease and report additional Phase 2 data.
- Report additional two-year results from Phase 1/2 clinical study in CLN6 Batten disease and complete enrollment in ongoing CLN3 Batten disease Phase 1/2 study.
- Establish preclinical proof of concept for Fabry and Pompe gene therapies.
- Maintain a strong financial position.

## 2019 Financial Guidance

Amicus recorded \$91.2 million in full-year 2018 revenue from commercial sales and reimbursed expanded access programs for Galafold. For the full-year 2019 the Company anticipates total Galafold revenue of \$160 million to \$180 million. Growth in 2018 was largely driven by EU and other countries outside the U.S. and Japan. Growth in 2019 is expected to be driven by continued growth in EU markets, further geographic expansion, and further success from the first full year of launch in the U.S. and Japan.

Cash, cash equivalents, and marketable securities totaled \$504.2 million at December 31, 2018. The Company expects to end 2019 with approximately \$300 million in cash on hand. The current cash position is anticipated to fund ongoing operations into at least mid- 2021.

## **Anticipated 2019 Milestones by Program**

Amicus [previously announced](#) full-year 2018 program updates as well as anticipated 2019 program milestones in early January 2019. All anticipated milestones remain on track as follows:

### **Galafold (migalastat) Oral Precision Medicine for Fabry Disease**

- On track to meet full-year 2019 revenue guidance range of \$160 million to \$180 million.

### **AT-GAA for Pompe Disease**

- Initial 6-month data in additional ERT-switch patients (Cohort 4) in Phase 1/2 ATB200-02 clinical study.
- Retrospective natural history study data in approximately 100 ERT-treated Pompe patients.
- Additional supportive studies, including an open-label study in pediatric patients.
- Full enrollment in Phase 3 PROPEL study.
- Advance agreed upon CMC requirements to support BLA.

### **Gene Therapy Pipeline:**

- Additional two-year data from CLN6 Batten disease Phase 1/2 study.
- Full enrollment of ongoing 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.

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## **Conference Call and Webcast**

Amicus Therapeutics will host a conference call and audio webcast today, February 28, 2019 at 8:30 a.m. ET to discuss the full year 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: 6983148.

A live 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 register on the website 15 minutes prior to the start of the call. A replay of the call will be available for seven days beginning at 11:30 a.m. ET on February 28, 2019. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 6983148.

## **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>.

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## **EU Important Safety Information**

Treatment with Galafold should be initiated and supervised by specialists experienced in the diagnosis and treatment of Fabry disease. Galafold is not recommended for use in patients with a nonamenable mutation.

- Galafold is not intended for concomitant use with enzyme replacement therapy.
- Galafold is not recommended for use in patients with Fabry disease who have severe renal impairment (<30 mL/min/1.73 m<sup>2</sup>). The safety and efficacy of Galafold in children 0–15 years of age have not yet been established.
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.
- While taking Galafold, effective birth control should be used. It is not known whether Galafold is excreted in human milk.
- Contraindications to Galafold include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- 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-dedicated 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 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, Japan, the US 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, 2018 filed today. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

**CONTACTS:****Investors/Media:**

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**TABLE 1**

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

	Years Ended December 31,		
	2018	2017	2016
<b>Revenue:</b>			
Net product sales	\$ 91,245	\$ 36,930	\$ 4,958
Cost of goods sold	14,404	6,236	833
Gross profit	76,841	30,694	4,125
Operating expenses:			
Research and development	270,902	149,310	104,793
Selling, general and administrative	127,200	88,671	71,151
Changes in fair value of contingent consideration payable	3,300	(234,322 )	6,760
Loss on impairment of assets	—	465,427	—
Restructuring charges	—	—	69
Depreciation	4,216	3,593	3,242
Total operating expenses	405,618	472,679	186,015
Loss from operations	(328,777 )	(441,985 )	(181,890 )
Other income (expenses):			
Interest income	10,461	4,096	1,602
Interest expense	(22,402 )	(17,240 )	(5,398 )
Change in fair value of derivatives	(2,739 )	—	—
Loss on extinguishment of debt	—	—	(13,302 )
Other income (expense)	(5,632 )	6,008	(4,793 )
Loss before income tax	(349,089 )	(449,121 )	(203,781 )
Income tax benefit	94	165,119	3,739
<b>Net loss attributable to common stockholders</b>	<b>\$ (348,995 )</b>	<b>\$ (284,002 )</b>	<b>\$ (200,042 )</b>
Net loss attributable to common stockholders per common share — basic and diluted	\$ (1.88 )	\$ (1.85 )	\$ (1.49 )
Weighted-average common shares outstanding — basic and diluted	185,790,021	153,355,144	134,401,588

**TABLE 2**

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

	December 31,	
	2018	2017
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 79,749	\$ 49,060
Investments in marketable securities	424,403	309,502
Accounts receivable	21,962	9,464
Inventories	8,390	4,623
Prepaid expenses and other current assets	16,592	19,316
<b>Total current assets</b>	<b>551,096</b>	<b>391,965</b>
Property and equipment, less accumulated depreciation of \$15,671 and \$12,515 at December 31, 2018 and 2017, respectively	11,375	9,062
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	6,683	5,200
<b>Total Assets</b>	<b>\$ 789,951</b>	<b>\$ 627,024</b>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	\$ 80,625	\$ 53,890
Deferred reimbursements	5,500	7,750
Contingent consideration payable	—	8,400
<b>Total current liabilities</b>	<b>86,125</b>	<b>70,040</b>
Deferred reimbursements	10,156	14,156
Convertible notes	175,006	164,167
Senior secured term loan	146,734	—
Contingent consideration payable	19,700	17,000
Deferred income taxes	6,465	6,465
Other non-current liabilities	2,853	2,346
<b>Total Liabilities</b>	<b>447,039</b>	<b>274,174</b>
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$.01 par value, 500,000,000 shares authorized, 189,383,924 shares issued and outstanding at December 31, 2018 Common stock, \$.01 par value, 250,000,000 shares authorized, 166,989,790 shares issued and outstanding at December 31, 2017	1,942	1,721
Additional paid-in capital	1,740,061	1,400,758
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	495	(1,659 )
Unrealized loss on available-for securities	(427 )	(436 )
Warrants	13,063	16,076
Accumulated deficit	(1,412,222 )	(1,063,610 )
<b>Total stockholders' equity</b>	<b>342,912</b>	<b>352,850</b>
<b>Total Liabilities and Stockholders' Equity</b>	<b>\$ 789,951</b>	<b>\$ 627,024</b>



# FY18 Financial Results Conference Call & Webcast

February 28, 2019



## 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, Japan, the US 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, 2018 filed today. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.*

# Rare Disease Day – February 28, 2019

Rare Disease Day 2019 is “Bridging Health and Social Care”



# Amicus Highlights

## GALAFOLD'S EXTRAORDINARY LAUNCH SUCCESS

- 650+ Patients and \$91.2M Global Sales in FY18
- FY19 Guidance of \$160M-\$180M
- \$500M Potential Sales by 2023
- \$1B+ Addressable Market Opportunity by 2028

## AT-GAA IN POMPE: POTENTIAL TO BECOME STANDARD OF CARE

- Continued Strength of Clinical Data
- Granted Breakthrough Therapy Designation
- Multiple Data Expected Throughout 2019
- 100+ Pompe Patients on AT-GAA by YE19
- \$1B-\$2B+ Market Opportunity

## LEADING GENE THERAPY PORTFOLIO IN RARE METABOLIC DISEASES

- Pipeline of 14 Gene Therapies
- 2 Clinical Stage Programs
- Established Global Research and Gene Therapy Center of Excellence in Philadelphia
- \$1B+ Peak Recurring Market Opportunity

## FINANCIAL STRENGTH

- \$500M+ Cash at 12/31/18 (runway into mid-2021)
- Growing Contribution from Galafold Revenues

## 2023 VISION

- 5,000+ Lives Transformed
- \$1B+ in Revenue
- Leading Global Rare Disease Biotech

## 2019 Key Strategic Priorities

- 1 **Nearly double annual revenue for Galafold (guidance \$160M-\$180M)**
- 2 **Complete enrollment in AT-GAA Pivotal Study (PROPEL) and report additional Phase 1/2 data**
- 3 **Report additional 2-year clinical results in CLN6-Batten disease and complete enrollment in ongoing CLN3-Batten disease Phase 1/2 study**
- 4 **Establish preclinical proof of concept for Fabry and Pompe gene therapies**
- 5 **Maintain strong financial position**



# Galafold<sup>®</sup> (migalastat) Global Launch...

**...taking a leadership role in the  
treatment of Fabry disease**

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

# Galafold Snapshot (as of December 31, 2018)

## One of the Most Successful Rare Disease Launches

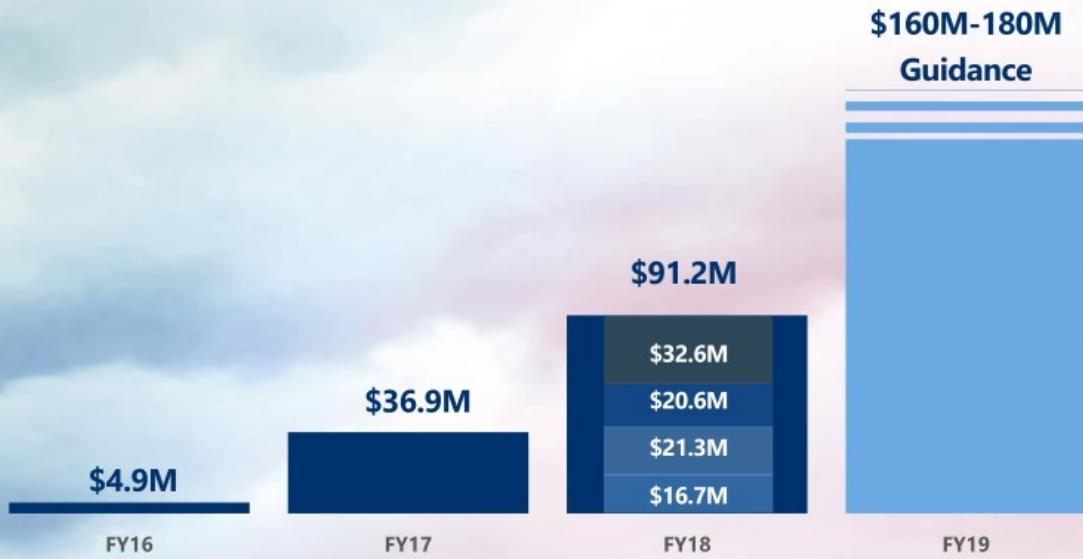


Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and dizziness. For additional information about Galafold, including the full U.S. Prescribing Information, please visit <https://www.amicus-therapeutics.com/Galafold>. 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).



# Galafold Success and FY18 Galafold Revenue Guidance

**On Track to Nearly DOUBLE Revenue Again and Serve 1,000+ Patients in 2019**



# Total Amenable Patient Population ("TAPP")

Estimate based on 35% - 50% amenability

## \$1B+ Addressable Market Opportunity by 2028



\*WORLDWIDE includes total amenable patient population in all Fabry ERT commercial markets today Estimated effect of newborn screening on adult diagnostic rate.





# AT-GAA Novel ERT for Pompe Disease

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

# AT-GAA: Breakthrough Therapy Designation

## U.S. FDA Granted BTM to AT-GAA in Late-Onset Pompe Disease (LOPD)

### BTM Criteria

- Intended to treat a serious or life threatening disease or condition
- Preliminary clinical evidence indicates drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints

### AT-GAA BTM Based on Ph 1/2 Clinical Efficacy

- Improvements in 6-minute walk distance
- Comparison to natural history of treated patients

### BTM Features

- Intensive guidance on an efficient drug development program
- Organizational commitment involving senior managers
- All Fast Track program features
- Potential Rolling BLA
- Potential for Priority Review

# AT-GAA: 2019 Objectives

## Advance AT-GAA for as Many Patients Worldwide as Quickly as Possible

- ✓ **Additional Phase 1/2 Data (up to 24 Months)**
- ✓ **Breakthrough Therapy Designation**
- **Enroll PROPEL study (n=100)**
- **Present additional Phase 1/2 data (Cohort 4)**
- **Report natural history study data**
- **Initiate supportive studies**
- **Advance agreed upon CMC requirements to support BLA**



# Gene Therapy Pipeline

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

# A RARE PORTFOLIO.

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3	REGULATORY	COMMERCIAL
<b>Fabry Franchise</b>						
Galafold® (migalastat) monotherapy						
Fabry Gene Therapy	PENN					
<b>Pompe Franchise</b>						
AT-GAA (Novel ERT + Chaperone)						
Pompe Gene Therapy	PENN					
<b>Batten Franchise – Gene Therapies</b>						
CLN6 Batten Disease	NCH					
CLN3 Batten Disease	NCH					
CLN8 Batten Disease	NCH					
CLN1 Batten Disease	NCH					
<b>Rare CNS and Other Gene Therapies</b>						
CDKL5 Deficiency Disorder GTx / ERT	PENN					
Niemann-Pick Type C (NPC)	NCH					
Tay-Sachs Disease	NCH					
Wolman Disease	NCH					
Other	NCH / PENN					

Advancing one of the **most robust rare disease portfolios** in biotechnology

# Leading LSD Gene Therapy Portfolio

**Multiple Platforms Provide 14 Gene Therapy Programs and R&D Engine for Future Growth**



GENE THERAPY PROGRAM



## Gene Therapy Anticipated Milestones

2-Year CLN6 Batten Disease Phase 1/2 Data (Mid-2019)

CLN3 Batten Disease Phase 1/2 Study Enrolling

CLN8 and CLN1 Batten Disease Preclinical Studies Underway

Fabry and Pompe Preclinical Studies in Progress

Engines for Future Growth

## Amicus Global Research and Gene Therapy Center of Excellence in Philadelphia

**Strengthens Amicus Capabilities as a Leading Global Rare Disease Biotechnology Company in Burgeoning Hub for Medical Breakthroughs**



- » New 75,000 sqft state-of-the-art facility
- » Located in uCity Square Adjacent to Penn Campus
- » Global Amicus science organization and gene therapy leadership team headquarters
- » Co-led by Jeff Castelli, PhD, Chief Portfolio Officer and Head of Gene Therapy and Hung Do, PhD, CSO
- » Initial group occupying temporary space
- » Permanent space to eventually hold ~200 employees

Artist rendering



# Milestones

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



# Anticipated Milestones: 2019

## Well-Positioned to Create Significant Value for Shareholders and Patients in 2019

### Galafold: Fabry Disease

- FY19 revenue guidance \$160M-\$180M
- Growth in existing markets
- Expansion into new markets
- Diagnostic initiatives

### AT-GAA: Pompe Disease

- ✓ Additional Phase 1/2 data (21 and 24 months)
- ✓ Breakthrough therapy designation (BTD) in LOPD
- PROPEL pivotal study enrollment (n=100)
- Additional Phase 1/2 data (Cohort 4)
- Natural history study data
- Additional supportive studies
- Advance CMC requirements to support BLA

### Gene Therapy Programs

- Ongoing CLN3 Batten disease Phase 1/2 study enrollment
- Additional 2-year data from CLN6 Batten disease Phase 1/2 study
- Preclinical proof of concept for Fabry, Pompe and CDD
- Preclinical work across additional neurologic LSDs



# A Rare Company

**Commercial Organizational Strength**

*"We are business led and science driven"*  
- Amicus Belief Statement

# Global Footprint

## Global operations in place across the largest rare disease markets

- » **150+** employees supporting our global Galafold business
  - » ~100 in Commercial
  - » ~50 in Medical Affairs
- » **11** countries with offices, including US, EU5, Japan
- » **20** countries with direct presence (Amicus personnel)
- » **24** markets with reimbursement
- » **27** markets with Amicus presence (direct or distributor)



# Experienced Passionate Commercial Team

Proven global capabilities driving Galafold's extraordinary launch success

Experience in  
Rare Disease  
Drug Launch

Diverse  
Background  
and  
Experiences

Passion for  
Patients



Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (20%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including the EU/US Prescribing Information, please visit <https://www.galafold.com/galafold>. 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 <https://www.ema.europa.eu>.

# An Innovative Product

## Novel Mechanism, Precision Medicine, Commercial Execution

### Product

- Differentiated product profile – first oral treatment option
- Rapid determination of amenability (website)
- Driving science through Publications, FollowMe Registry and Post Marketing Studies



### Execution

- Clear launch focus on priority patient segments
- Efficient outreach to key Fabry disease centers
- Strong education efforts on importance of genotype



Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit <https://www.amicus.com/Galafold.pdf>. For further important safety information for Galafold, including dosology 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](https://www.ema.europa.eu).

## Market Access

“Our medicines must be fairly priced and broadly accessible.”

- Amicus Founding Belief

- Proven patient-centric strategy of ensuring fair price and broad accessibility
- Rapid, successful reimbursement in global markets
- Amicus assist™ patient support program
- Amicus PROMISE to limit price increases to consumer inflation
- Amicus “pledge” to reinvest until there’s a cure

Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit <https://www.galafold.com/USPI>. For further important safety information for Galafold, including pathology 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 <https://www.ema.europa.eu/en/medicines/humans/EPAR/galafold/galafold.htm>.



# Financial Summary

Full Year Ended December 31, 2019

*“Maximizing Value for Shareholders  
is the Foundation for Our Future Successes”*  
- Amicus Belief Statement

# FY18 Select Financial Results

## FY18 Revenue of \$91.2M Primarily from International Galafold Sales

<i>(in thousands, except per share data)</i>	<b>Dec. 31, 2018</b>	<b>Dec. 31, 2017</b>
<b>Product revenue</b>	91,245	36,930
<b>Cost of goods sold</b>	14,404	6,236
<b>R&amp;D expense</b>	270,902*	149,310
<b>SG&amp;A expense</b>	127,200	88,671
<b>Changes in fair value of contingent consideration</b>	3,300	(234,322)
<b>Loss on impairment of assets</b>	-	465,427
<b>Loss from operations</b>	(328,777)	(441,985)
<b>Income tax benefit</b>	94	165,119
<b>Net loss</b>	(348,995)	(284,002)
<b>Net loss per share</b>	(1.88)	(1.85)

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



# Financial Summary and Guidance

**Strong Balance Sheet with \$500M+ Cash at 12/31/18 - Cash Runway into 2021**

FINANCIAL POSITION		December 31, 2018
<b>Cash</b>		~\$504M
<b>Cash Runway</b>		Into at least mid-2021
CAPITALIZATION		
<b>Shares Outstanding</b>		189,383,924
FINANCIAL GUIDANCE		
<b>Projected YE 2019 Cash Balance</b>		~\$300M
<b>Galafold Revenue Guidance</b>		\$160M-\$180M

# A RARE VISION. Impacting Lives



>350 Patients\* | ~\$36M Global Sales

YE17



>700 Patients\* | ~\$91M<sup>1</sup> Global Sales

YE18



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

2023

\*Clinical & commercial, all figures approximate <sup>1</sup>Preliminary unaudited

# Appendix



# Pompe Patient Experience in Phase 1/2 Clinical Study (ATB200-02)

**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**

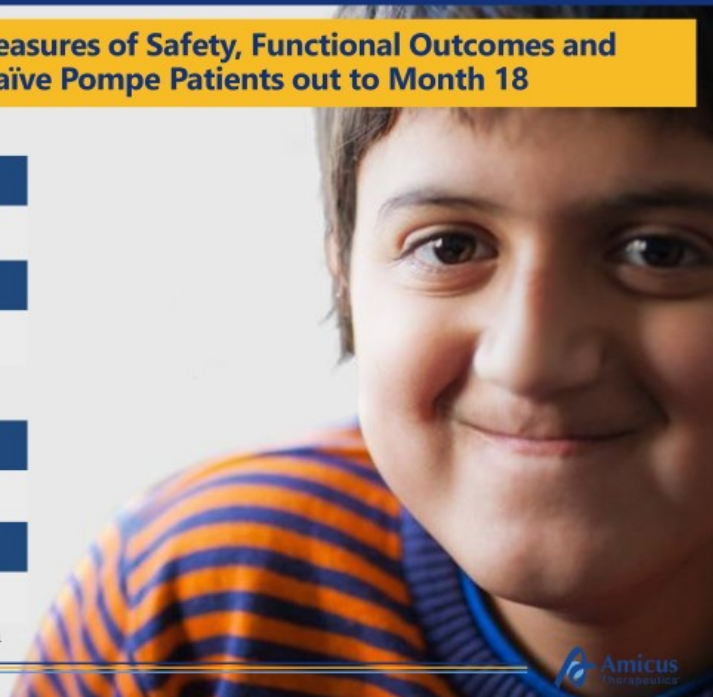
## 6-Minute Walk Test (m)

Cohort	Baseline (n=10)	Change at Month 24 <sup>a,b</sup> (n=8) Mean (SD)
Cohort 1 ERT-Switch Ambulatory	<b>397.2</b> (96.8)	<b>+53.6</b> (36.4)
Cohort	Baseline (n=5)	Change at Month 21 (n=5) Mean (SD)
Cohort 3 ERT-Naïve	<b>399.5</b> (83.5)	<b>+54.8</b> (34.7)

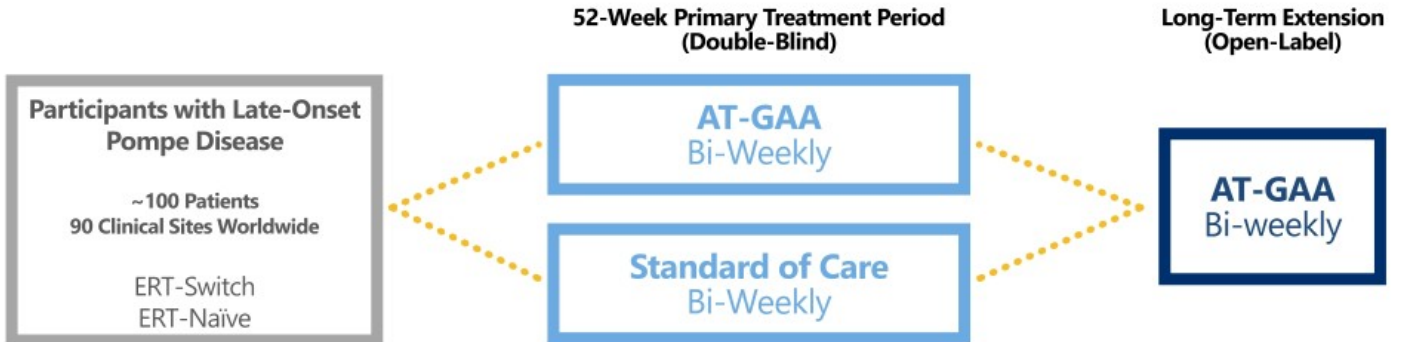
## FVC (% Predicted)

Cohort	Baseline (n=9*)	Change at Month 24 <sup>a,b,c</sup> (n=7) Mean (SD)
Cohort 1 ERT-Switch Ambulatory*	<b>52.6</b> (14.7)	<b>-0.6</b> (2.8)
Cohort	Baseline (n=5)	Change at Month 21 (n=5) Mean (SD)
Cohort 3 ERT-Naïve	<b>53.4</b> (20.3)	<b>+6.1</b> (9.7)

\*One patient in Cohort 1 discontinued from study (withdrew consent) before Month 24. <sup>a</sup>At the time of this interim analysis, 1 patient in Cohort 1 had not reached Month 24. <sup>b</sup>Baseline FVC missing for 1 patient in Cohort 1



# PROPEL (ATB200-03) Study Design



**Primary Endpoint: 6-Minute Walk Test at Week 52**  
**Multiple Secondary Endpoints**

Thank You

*"Our passion for making a difference unites us"*

*-Amicus Belief Statement*



