

**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): **March 2, 2020**

AMICUS THERAPEUTICS, INC.

(Exact Name of Registrant as Specified in Its Charter)

Delaware
(State or Other Jurisdiction of
Incorporation)

Delaware
(State or Other Jurisdiction
of Incorporation)

001-33497
(Commission
File Number)

71-0869350
(I.R.S. Employer
Identification No.)

1 Cedar Brook Drive, Cranbury, NJ 08512
(Address of Principal Executive Offices, and Zip Code)

609-662-2000
Registrant's Telephone Number, Including Area Code

(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))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock Par Value \$0.01	FOLD	NASDAQ

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (17 CFR §230.405) or Rule 12b-2 of the Securities Exchange Act of 1934 (17 CFR §240.12b-2). 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 March 2, 2020, Amicus Therapeutics, Inc. (the “Company”) issued a press release announcing its financial results for the fiscal year ended December 31, 2019. A copy of this press release is attached hereto as Exhibit 99.1. The Company will host a conference call and webcast on March 2, 2020 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:**

Exhibit No.	Description
99.1	Press release dated March 2, 2020
99.2	March 2, 2020 Conference Call Presentation Materials
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

Signature Page

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: March 2, 2020

AMICUS THERAPEUTICS, INC.

By: /s/ Ellen S. Rosenberg

Name: Ellen S. Rosenberg

Title: Chief Legal Officer and Corporate Secretary

Amicus Therapeutics Announces Full-Year 2019 Financial Results and 2020 Corporate Updates

2019 Galafold Revenue Nearly Doubled to \$182.2M

On Track to Achieve 2020 Revenue Guidance of \$250M-\$260M

Focused on Pompe Phase 3 PROPEL Study, Manufacturing to Support 2021 BLA and MAA, and Accelerating Expanded Access Program for Infantile-Onset Patients

Advancing Industry-Leading Rare Disease Gene Therapy Portfolio

Strong Balance Sheet with \$450M+ Cash – Cash Runway Well into 2022

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

CRANBURY, NJ, March 2, 2020 – [Amicus Therapeutics](#) (Nasdaq: FOLD), a global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel medicines for rare diseases, today announced financial results for the full-year ended December 31, 2019. The Company also summarized recent program updates and reiterated its full-year 2020 guidance.

Corporate Highlights for Full-Year 2019 and Year-to-Date 2020

- **Galafold® (migalastat), the first oral treatment option for people living with Fabry and who have an amenable variant, revenue grew from \$91.2 million in full-year 2018 to \$182.2 million in full-year 2019, exceeding the high end of the full-year 2019 guidance range of \$170 million to \$180 million. Over the course of 2019, Amicus received key marketing authorizations around the globe, including Argentina, Brazil, Colombia and Taiwan.**
- **Global Phase 3 PROPEL clinical trial of AT-GAA in late-onset Pompe disease exceeded enrollment and inventory build remains on track.** As [previously announced](#), 59 clinical sites enrolled 123 participants globally in the Phase 3 PROPEL study. Process performance qualification (PPQ) runs nearing successful completion with key strategic partner, WuXi Biologics, and will serve as the foundation for the Chemistry, Manufacturing, and Control (CMC) module for a biologics license application (BLA) submission.
- **The Company plans to apply for and initiate a rolling BLA for AT-GAA, completing final submission in the first half of 2021.**
- **Focus on an Expanded Access Program for infantile-onset Pompe patients.** Amicus intends to offer an expanded access program for infantile-onset patients.
- **Presented positive interim results in ongoing Phase 1/2 clinical study for CLN6 Batten disease.** Data on motor, language, seizure and vision sub scores suggest stabilization of these individual components in most patients, in particular those children treated at a younger age.
- **Amicus continues to carefully manage expenses and investments, while executing on the Galafold launch and advancing development programs.** The current cash position is expected to fund ongoing operations well into 2022.

2020 Key Strategic Priorities

- Achieve \$250 million to \$260 million of global product revenue for Galafold
- Complete Pompe Phase 3 PROPEL study, enroll pediatric studies and advance manufacturing to support 2021 BLA and MAA

- Advance clinical development, manufacturing and regulatory discussions for CLN6 and CLN3 Batten programs
- Progress Pompe gene therapy towards IND and disclose up to two additional IND candidates
- Maintain strong financial position

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, "Amicus has 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 our 2020 key strategic priorities including our global Fabry launch, Pompe late-stage development program, and gene therapy pipeline. With a very successful, commercial product in Fabry disease, a late stage program with Breakthrough Therapy Designation in late onset Pompe disease and 14 gene therapy programs for rare diseases in development, including two in the clinic, we are now, strongly positioned to achieve our vision of delivering groundbreaking new medicines and hopefully one day cures for people living with rare metabolic diseases."

Full-Year 2019 Financial Results

- Total revenue in the full-year 2019 was \$182.2 million, an increase from total revenue of \$91.2 million in the full-year 2018.
- Cash, cash equivalents, and marketable securities totaled \$452.7 million at December 31, 2019, compared to \$504.2 million at December 31, 2018.
- Total GAAP operating expenses were \$464.3 million for the full-year 2019, compared to \$405.6 million in the full-year 2018. Operating expenses reflecting increased investments in the Galafold launch, Pompe program, and gene therapy pipeline.
- Total non-GAAP operating expenses of \$411.8 million for the full-year 2019 increased as compared to \$268.8 million for the full-year 2018, reflecting continued investments in the Galafold launch, Pompe program, and gene therapy pipeline. Non-GAAP operating expenses came in at the lower end of the guidance range of \$410 million to \$420 million. Full reconciliation of GAAP results to the Company's non-GAAP adjusted measures for all reporting periods appear in the tables to this press release.
- Net loss was \$356.4 million, or \$1.48 per share, compared to a net loss of \$349.0 million, or \$1.88 per share, for the full-year 2018.

2020 Financial Guidance

- For the full-year 2020, the Company anticipates total Galafold revenue of \$250 million to \$260 million based on the average exchange rates for 2019.
- Non-GAAP operating expense guidance for the full-year 2020 is \$410 million to \$420 million, driven by continued investment in the global Galafold launch, AT-GAA clinical studies, and advancing our gene therapy pipeline. A reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure is not available without unreasonable effort due to high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure.
- Cash, cash equivalents, and marketable securities totaled \$452.7 million at December 31, 2019. The current cash position is anticipated to fund ongoing operations well into 2022.

Anticipated 2020 Milestones by Program

Amicus [previously announced](#) 2020 program milestones in early January 2020. All anticipated milestones remain on track as follows:

Galafold (migalastat) Oral Precision Medicine for Fabry Disease

- On track to meet full-year 2020 revenue guidance range of \$250 million to \$260 million
- Registry and other Phase 4 supportive studies underway

AT-GAA for Pompe Disease

- Plans to apply for and initiate a Rolling Biologics License Application (BLA) for AT-GAA in 2020, with addition of full clinical results in 1H2021 to support full approval under Fast Track Designation
- Retrospective natural history study data in approximately 100 ERT-treated Pompe patients
- Additional supportive studies, including an open-label study in 12 to 18-year-old patients

Gene Therapy Portfolio

- Dose additional patients in CLN6 Phase 1/2 study and plan to advance regulatory discussions to finalize clinical and regulatory path
 - Initiate long-term follow-up of initial participants in the CLN6 Phase 1/2 study in 1H2020 to obtain long-term safety and efficacy data
 - Plan to advance regulatory discussions to finalize clinical and regulatory path in CLN3
 - Report initial data on patients enrolled in CLN3 Phase 1/2 study
 - Complete IND-enabling toxicology work in Pompe disease and progress towards IND
 - Additional preclinical data expected in multiple programs
 - Disclose up to two additional IND candidates
 - Manufacturing advancements across portfolio
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Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, March 2, 2020, at 8:30 a.m. ET to discuss the full-year 2019 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: 2782337.

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 March 2, 2020. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 2782337.

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 over 40 countries around the world, including the U.S., EU, U.K, Japan and others.

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 ($\geq 10\%$) 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.

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

E.U. and U.K. Important Safety Information

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

- Galafold is not intended for concomitant use with enzyme replacement therapy.
- Galafold is not recommended for use in patients with Fabry disease who have severe renal impairment (<30 mL/min/1.73 m²). The safety and efficacy of Galafold in children 0–15 years of age have not yet been established.
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.
- While taking Galafold, effective birth control should be used. It is not known whether Galafold is excreted in human milk.
- Contraindications to Galafold include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on Galafold or switched to Galafold.
- OVERDOSE: General medical care is recommended in the case of Galafold overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received Galafold. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

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

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. 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, and follow on [Twitter](#) and [LinkedIn](#).

Non-GAAP Financial Measures

In addition to financial information prepared in accordance with U.S. GAAP, this press release also contains adjusted financial measures that we believe provide investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. Full reconciliations of GAAP results to the comparable non-GAAP measures for the reported periods appear in the financial tables section of this press release. When we provide our expectation for non-GAAP operating expenses on a forward-looking basis, a reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure generally is not available without unreasonable effort due to potentially high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure in the relevant future period, such as unusual gains or losses. The variability of the excluded items may have a significant, and potentially unpredictable, impact on our future GAAP results.

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, 2019 to be 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:

Andrew Faughnan
Director, Investor Relations
afaughnan@amicusrx.com
(609) 662-3809

Media:
Christopher Byrne
Executive Director, Corporate Communications
cbyrne@amicusrx.com
(609) 662-2798

FOLD—G
TABLE 1

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

	Years Ended December 31,		
	2019	2018	2017
Net product sales	\$ 182,237	\$ 91,245	\$ 36,930
Cost of goods sold	21,963	14,404	6,236
Gross profit	160,274	76,841	30,694
Operating expenses:			
Research and development	286,378	270,902	149,310
Selling, general, and administrative	169,861	127,200	88,671
Changes in fair value of contingent consideration payable	3,297	3,300	(234,322)
Loss on impairment of assets	—	—	465,427
Depreciation and amortization	4,775	4,216	3,593
Total operating expenses	464,311	405,618	472,679
Loss from operations	(304,037)	(328,777)	(441,985)
Other income (expenses):			
Interest income	10,249	10,461	4,096
Interest expense	(18,872)	(22,402)	(17,240)
Loss on exchange of convertible notes	(40,624)	—	—
Change in fair value of derivatives	—	(2,739)	—
Other (expense) income	(2,626)	(5,632)	6,008
Loss before income tax	(355,910)	(349,089)	(449,121)
Income tax (expense) benefit	(478)	94	165,119
Net loss attributable to common stockholders	\$ (356,388)	\$ (348,995)	\$ (284,002)
Net loss attributable to common stockholders per common share — basic and diluted	\$ (1.48)	\$ (1.88)	\$ (1.85)
Weighted-average common shares outstanding — basic and diluted	240,421,001	185,790,021	153,355,144

TABLE 2

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

	December 31,	
	2019	2018
Assets		
Current assets:		
Cash and cash equivalents	\$ 142,837	\$ 79,749
Investments in marketable securities	309,903	424,403
Accounts receivable	33,284	21,962
Inventories	14,041	8,390
Prepaid expenses and other current assets	20,008	16,592
Total current assets	<u>520,073</u>	<u>551,096</u>
Operating lease right-of-use assets, less accumulated amortization of \$5,342 and \$0 at December 31, 2019 and December 31, 2018, respectively	33,315	—
Property and equipment, less accumulated depreciation of \$17,604 and \$15,671 at December 31, 2019 and December 31, 2018, respectively	47,705	11,375
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	28,317	6,683
Total Assets	<u>\$ 850,207</u>	<u>\$ 789,951</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	\$ 120,373	\$ 80,625
Deferred reimbursements	1,250	5,500
Operating lease liabilities	7,189	—
Total current liabilities	<u>128,812</u>	<u>86,125</u>
Deferred reimbursements	8,906	10,156
Convertible notes	2,131	175,006
Senior secured term loan	147,374	146,734
Contingent consideration payable	22,681	19,700
Deferred income taxes	5,051	6,465
Operating lease liabilities	53,531	—
Other non-current liabilities	5,296	2,853
Total Liabilities	<u>373,782</u>	<u>447,039</u>
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$.01 par value, 500,000,000 shares authorized, 255,417,869 and 189,383,924 shares issued and outstanding at December 31, 2019 and December 31, 2018, respectively	2,598	1,942
Additional paid-in capital	2,227,225	1,740,061
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	2,785	495
Unrealized gain (loss) on available-for securities	40	(427)
Warrants	12,387	13,063
Accumulated deficit	(1,768,610)	(1,412,222)
Total stockholders' equity	<u>476,425</u>	<u>342,912</u>
Total Liabilities and Stockholders' Equity	<u>\$ 850,207</u>	<u>\$ 789,951</u>

TABLE 3

Amicus Therapeutics, Inc.
Reconciliation of Non-GAAP Financial Measures
(in thousands)

	Years Ended December 31,		
	2019	2018	2017
Total operating expenses - as reported GAAP	\$ 464,311	\$ 405,618	\$472,679
Research and development:			
Share-based compensation	17,575	11,740	10,328
Research and development asset acquisition expense	—	100,000	—
Selling, general and administrative:			
Share-based compensation	26,855	17,520	12,773
Loss on impairment of assets	—	—	465,427
Changes in fair value of contingent consideration payable	3,297	3,300	(234,322)
Depreciation and amortization	4,775	4,216	3,593
Total operating expense adjustments to reported GAAP	52,502	136,776	257,799
Total operating expenses - as adjusted	\$ 411,809	\$ 268,842	\$ 214,880



2019 Financial Results Conference Call & Webcast

March 2, 2020

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 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 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 based on 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 our 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 our 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 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. In addition to financial information prepared in accordance with U.S. GAAP, this presentation also contains adjusted financial measures that we believe provide investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. With respect to statements regarding projections of the Company's revenue and cash position, actual results may differ from those 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, 2019 to be 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.

In addition to financial information prepared in accordance with U.S. GAAP, this presentation also contains adjusted financial measures that we believe provide investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. Full reconciliations of GAAP results to the comparable non-GAAP measures for the reported periods appear in the financial tables section of this presentation. When we provide a non-GAAP expectation for non-GAAP operating expenses on a forward-looking basis, a reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure is not available without unreasonable effort due to potentially high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure in the relevant period, such as unusual gains or losses. The variability of the excluded items may have a significant, and potentially unpredictable, impact on our future GAAP results.



A RARE COMPANY

A leading fully-integrated, global rare disease biotechnology company



First Oral Precision
Medicine for Fabry Disease



Gene Therapy
PLATFORM
Protein Engineering
& Glycobiology



World Class
BIOLOGIC
Capabilities



EMPLOYEES
in 27 Countries

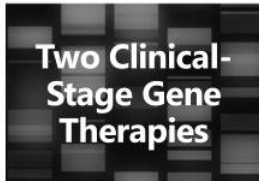


AT-GAA
Phase 3 in
Pompe Disease



\$450M+
Cash
as of 12/31/19

**Two Clinical-
Stage Gene
Therapies**



**GLOBAL
COMMERCIAL
ORGANIZATION**



**Robust R&D
Engine**

Nearly 50+ Lysosomal
Disorders and More
Prevalent Rare Disease



Key Takeaways

Recent successes across our science, clinical, regulatory and commercial efforts position us for the future



Galafold Continues Strong Launch Performance & Cornerstone of Amicus Success



AT-GAA for Pompe Advances Toward Approval as "Crown Jewel" of Amicus Portfolio



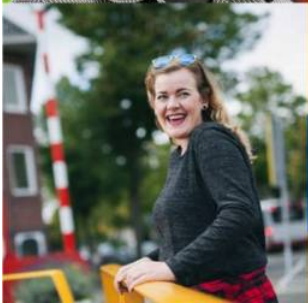
Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future



Strong Financial Outlook with Current Cash Well into 2025

2020 Key Strategic Priorities

- 1** **Achieve global product revenue for Galafold of \$250M-\$260M**
- 2** **Complete Pompe Phase 3 PROPEL study, enroll pediatric studies and advance manufacturing to support 2021 BLA and MAA**
- 3** **Advance clinical development, manufacturing and regulatory discussions for CLN6 and CLN3 Batten programs**
- 4** **Progress Pompe gene therapy towards IND and disclose up to two additional IND candidates**
- 5** **Maintain strong financial position**



Galafold[®] (migalastat) Global Launch...

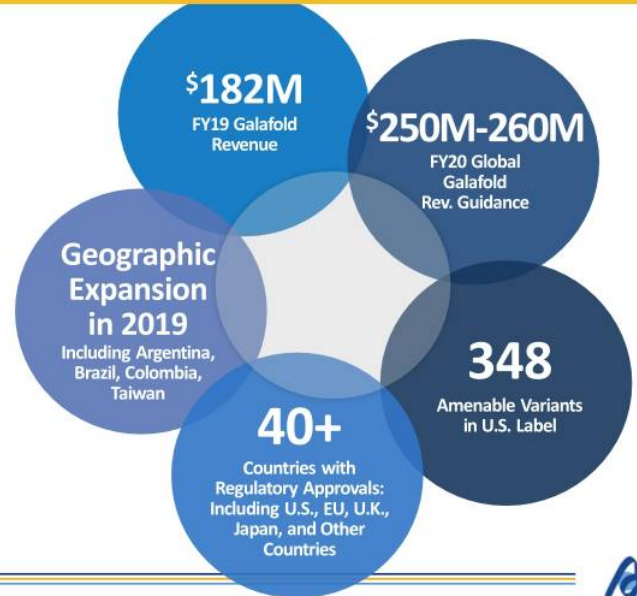
**...taking a leadership role in the
treatment of Fabry disease**

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

Galafold Snapshot (as of December 31, 2019)

Galafold is the cornerstone of Amicus' success. It is an orally delivered small molecule precision medicine with a unique mechanism of action for Fabry patients with amenable variants that replaces the need for intravenously delivered enzyme replacement therapy

One of the Most Successful Rare Disease Launches



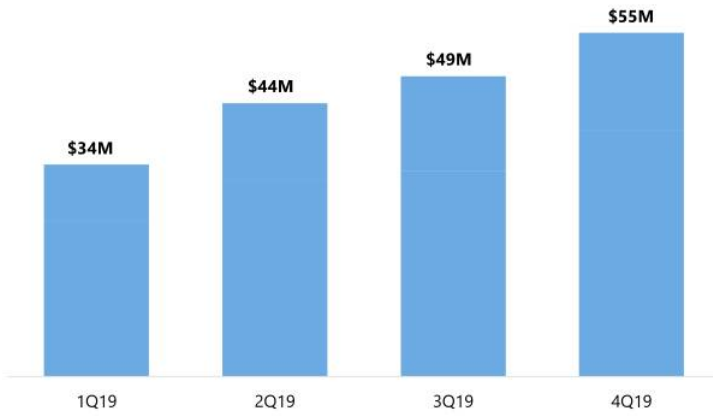
Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including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



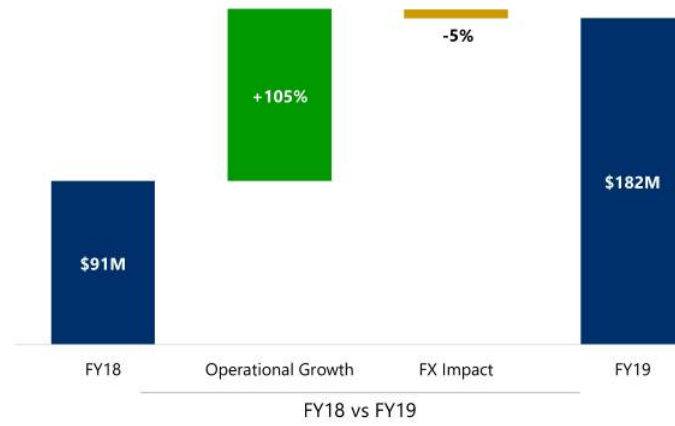
2019 Galafold Success

Strong full-year revenue performance of \$182M, exceeding guidance of \$170M-\$180M

Quarterly Galafold Sales

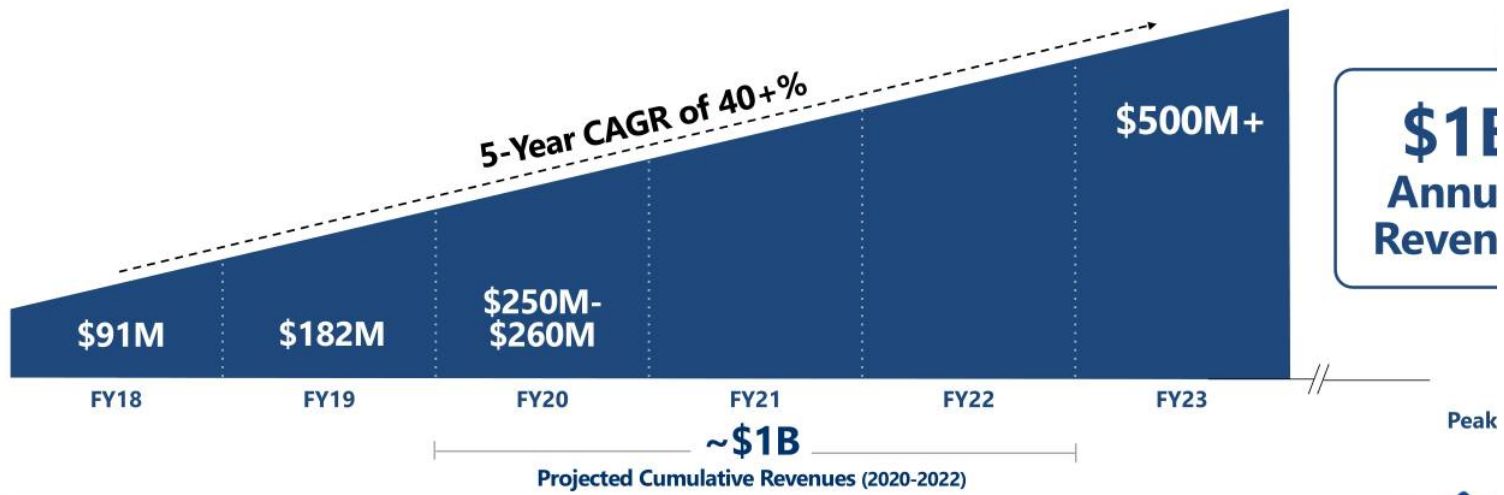


Year-over-Year Galafold Sales Growth



Galafold Growth Trajectory

Galafold is on track to generate \$1B+ in projected cumulative revenues from 2020-2022 and is on an anticipated path to \$500M+ in annual sales in 2023 and \$1B+ annual sales at peak





AT-GAA: Next Potential Standard of Care for Pompe Disease

“We encourage and embrace constant innovation
- Amicus Belief Statement

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

AT-GAA is the first ever second-generation product for any lysosomal disorder to earn FDA Breakthrough Therapy Designation (BTM)

Plans to apply for and initiate a rolling BLA submission for AT-GAA in LOPD in 2020



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 agency staff
- All Fast Track program features including rolling submission



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: Key Takeaways



AT-GAA for Pompe
Advances Toward
Approval as "Crown
Jewel" of Amicus
Portfolio

- PROPEL pivotal study exceeded enrollment with data expected 1H2021
- Breakthrough Therapy Designation and the Promising Innovative Medicine designation highlight unmet need in Pompe disease today
- Plan to submit and initiate rolling submission of Biologics License Application in 2020
- Manufacturing PPQ runs at WuXi biologics on track
- Peak revenue potential of \$1B-\$2B, with exclusivity well into 2030s



Financial Summary

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

2019 Select Financial Results

2019 revenue of \$182M from global Galafold sales

<i>(in thousands, except per share data)</i>	Dec. 31, 2019	Dec. 31, 2018
Product Revenue	\$182,237	\$91,245
Cost of Goods Sold	21,963	14,404
R&D Expense*	286,378	270,902
SG&A Expense	169,861	127,200
Changes in Fair Value of Contingent Consideration	3,297	3,300
Depreciation and Amortization	4,775	4,216
Loss from Operations	(304,037)	(328,777)
Income Tax (Expense) Benefit	(478)	94
Net Loss	(356,388)	(348,995)
Net Loss Per Share	(1.48)	(1.88)

*Inclusive of the 2018 upfront payment of \$100 million for the Celenex asst acquisition.



Cash Runway Now to Well into 2022 (~2+ years)

Fully funded through major milestones in portfolio and continued global growth

**\$450M+
Cash
YE2019**

~2+ Years Cash Runway

**Well into
2022**



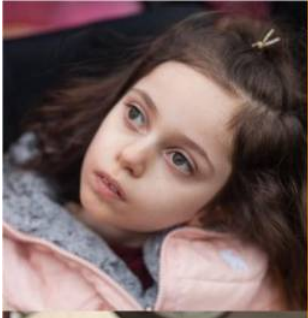
*Exclusive license from Ultragenyx for Japanese rights to Mepsevii[®], investigator-sponsored trial in Japan underway



Financial Outlook: Key Takeaways



- Company fully funded through major milestones in portfolio ; continued global growth
- Cumulative Galafold projected revenue of \$1B+ in 2020-2022 offsets significant majority of company spend/investments
- Extended cash flow runway through OpEx savings, CapEx phasing, program prioritization and increased Galafold revenue projections
- No material business development planned or needed in next several years
- Only modest additional capital required in the outer years to extend runway into profitability with multiple non-equity sources available as/when needed



Next Generation Gene Therapy Platform

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

A RARE PORTFOLIO

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3	REGULATORY	COMM
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Fabry Franchise

Galafold® (migalastat) Monotherapy ODD						
Fabry Gene Therapy	PENN					

Pompe Franchise

AT-GAA (Novel ERT + Chaperone) ODD						
Pompe Gene Therapy	PENN					

Batten Franchise – Gene Therapies

CLN6 Batten Disease ODD RPD	NCH					
CLN3 Batten Disease ODD RPD	NCH					
CLN8 Batten Disease	NCH					
CLN1 Batten Disease	NCH					

Next Generation Research Programs and CNS Gene Therapies

CDKL5 Deficiency Disorder GTx / ERT	PENN					
Niemann-Pick Type C (NPC)	NCH / PENN					
Others	NCH / PENN					

MPS Franchise

Mepsevii™ (vestronidase alfa) <i>(Japan Only)*</i>						
Next Generation MPSIIIA	PENN					
MPSIIIB	PENN					

LEGEND

- **ODD** - Orphan Designation
- **RPD** - Rare Pediatric Disease Designation

*Exclusive license from Ultragenyx for Japanese rights to Mepsevii™, investigator-sponsored trial in Japan underway



Combines Amicus and Penn Expertise Across Lysosomal and Rare Diseases

An R&D platform with rights to 50+ diseases, including 8 active preclinical programs



Gene Therapy: Updates & Key Takeaways



Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future

- CLN6 Phase 1/2 interim data shows profound impact with potential to become first ever approved gene therapy for fat brain disease in children
- Plan to report initial data for patients enrolled in CLN3 Phase study in 2H'20
- Orphan drug designations granted in U.S. and EU for intrathal AAV gene therapies for CLN6 and CLN3 Batten disease.
- Pompe gene therapy moving into IND-enabling studies
- Penn Collaboration is R&D engine, with rights to 50+ diseases
- 8 preclinical gene therapies in development



Closing Remarks

“We are business led and science driven
- Amicus Belief Statement

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement



Appendix



Non-GAAP Reconciliation

Amicus Therapeutics, Inc.
Reconciliation of Non-GAAP Financial Measures
(in thousands)

	Years Ended December 31,		
	2019	2018	2017
Total operating expenses - as reported GAAP	\$ 464,311	\$ 405,618	\$ 472,679
Research and development:			
Share-based compensation	17,575	11,740	10,328
Research and development asset acquisition expense	—	100,000	—
Selling, general and administrative:			
Share-based compensation	26,855	17,520	12,773
Loss on impairment of assets	—	—	465,427
Changes in fair value of contingent consideration payable	3,297	3,300	(234,322)
Depreciation and amortization	4,775	4,216	3,593
Total operating expense adjustments to reported GAAP	52,502	136,776	257,799
Total operating expenses - as adjusted	\$ 411,809	\$ 268,842	\$ 214,880

