

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549**

FORM 8-K

**CURRENT REPORT PURSUANT TO
SECTION 13 OR 15(d) OF THE
SECURITIES EXCHANGE ACT OF 1934**

Date of Report (Date of earliest event reported): **May 7, 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 May 7, 2020, Amicus Therapeutics, Inc. (the “Company”) issued a press release announcing its financial results for the fiscal quarter ended March 31, 2020. A copy of this press release is attached hereto as Exhibit 99.1. The Company will host a conference call and webcast on May 7, 2020 to discuss its first quarter 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 May 7, 2020
99.2	May 7, 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.

AMICUS THERAPEUTICS, INC.

Date: May 7, 2020

By: /s/ Ellen S. Rosenberg

Name: Ellen S. Rosenberg

Title: Chief Legal Officer and Corporate Secretary



Amicus Therapeutics Announces First Quarter 2020 Financial Results and Corporate Updates

Galafold 1Q2020 Revenue of \$60.5 Million Reflects Continued Strong Adoption in All Key Global Regions, On Track to Achieve 2020 Revenue Guidance of \$250M-\$260M

AT-GAA in Late-Onset Pompe Disease Granted Rolling BLA Submission by U.S. FDA

CLN3 Batten Disease Gene Therapy Granted Fast Track Designation by U.S. FDA

Infantile-Onset Pompe Disease Expanded Access Program Underway

Cash Runway Now Well into 2H2022

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

CRANBURY, NJ, May 7, 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 first quarter ended March 31, 2020. The Company also summarized recent program updates and reiterated its full-year 2020 guidance.

Revenue

- **Global revenue for Galafold® (migalastat) in the first quarter of 2020 was \$60.5M and continues to track toward full-year 2020 revenue guidance of \$250 million to \$260 million.** First quarter revenue represented a year-over-year increase of 78% from total revenue of \$34.0 million in the first quarter of 2019. On a constant currency basis, first quarter 2020 total revenue was \$61.4 million, representing operational revenue growth measured at constant currency exchange rates of 81%, which was offset by a negative currency impact of \$0.9 million, or 3%.
- **First quarter revenue represents the continued performance across the global business, including new patient starts from switch and naïve patients throughout the quarter in all major regions, including those hardest hit by COVID-19.** Performance driven largely by strong patient demand with a minimal contribution from ordering patterns as healthcare organizations in some countries adjusted to the impact of the COVID-19 virus. Global compliance and adherence rates continue to exceed 90%.

Supply Chain

- **Global supply chain for Galafold maintains continuous supply during the COVID-19 pandemic.** With sufficient inventory on hand and product effectively moving down the supply chain into individual countries, Amicus continues to fulfill the needs of Fabry patients without interruption. The company expects to be able to continuously supply this oral precision medicine in all global geographies for any and all people living with Fabry disease who have an amenable variant throughout the current COVID-19 pandemic and beyond.
- **Manufacturing and supply of AT-GAA remain intact globally.** Amicus maintains focus on providing a continuous supply of AT-GAA to all patients and continues to coordinate drug delivery per a site-by-site basis in order to address the needs of each patient. The Company has a high degree of confidence that there is ample supply of AT-GAA to support all ongoing clinical studies in the Pompe program, including the Phase 3 PROPEL study, which continues to track towards completing on schedule.

Research and Development

- **Global Phase 3 PROPEL clinical study of AT-GAA in late-onset Pompe disease remains on track for top line data in 1H21.** To date, 97% out of the 2,250 planned infusions for the ongoing PROPEL study have been completed on schedule. Process performance qualification (PPQ) runs have been successfully completed for the drug substance 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 initiate a rolling BLA for AT-GAA in the second half of 2020, completing final submission in the first half of 2021.



- **Expanded Access Program for infantile-onset Pompe patients underway.** Amicus has initiated an expanded access program for its investigational medicine AT-GAA for young children living with infantile-onset Pompe disease (IOPD).
- **Plan to advance regulatory discussions to finalize clinical and regulatory path in both CLN6 and CLN3 this year.** Additional data in the CLN6 Phase 1/2 study, as well as, initial CLN3 Phase 1/2 data expected later this year.
- **The U.S. Food and Drug Administration (FDA) has granted Fast Track Designation to the CLN3 Batten disease gene therapy, AT-GTX-502, for the treatment of pediatric patients less than 18 years of age.** The Fast Track program facilitates the development and accelerates the review of new drugs for serious conditions, which have the potential to address unmet medical needs. A drug development program with Fast Track designation is afforded greater access to the FDA for the purpose of expediting the drug's development, review and potential approval.
- **Additional preclinical gene therapy data expected across multiple programs in industry leading gene therapy portfolio.** As previously announced, one oral presentation and two posters accepted at the American Society of Gene & Cell Therapy 23rd Annual Meeting being held virtually on May 12–15.

Financial Strength

- **Cash runway now extended to well into 2H2022.** Amicus continues to carefully manage expenses and investments, while executing on the Galafold launch and advancing development programs. Extension of the cash runway will be achieved through continued careful expense management, prioritization of very early stage research programs and more measured capital expenditures.
- **First quarter non-GAAP operating expense on track for full-year guidance.** First quarter operating expenses driven by the timing of charges related to payments of the process performance qualification runs of AT-GAA and initiation of the tech transfer to our gene therapy contract manufacturing partners. Non-GAAP operating expense to decline throughout 2020 on path to achieving the 2020 non-GAAP operating expense guidance of \$410 million to \$420 million.

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 Investigational New Drug (IND) application 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, “During the first quarter, we have made tremendous progress advancing our mission for patients. Following success in the first quarter, we are on track and well-capitalized to achieve our 2020 key strategic priorities including our global Fabry launch, Pompe late-stage development program, and advancing our industry-leading gene therapy pipeline. In addition, we have taken steps to prioritize our spend and now see our cash runway lasting well into the second half of 2022. Through these efforts, we remain strongly positioned to achieve our vision of delivering groundbreaking new medicines and hopefully, one day, cures for people living with rare diseases.”



First Quarter 2020 Financial Results

- Total revenue in the first quarter 2020 was \$60.5 million, a year-over-year increase of 78% from total revenue of \$34.0 million in the first quarter of 2019. On a constant currency basis, first quarter 2020 total revenue was \$61.4 million, representing operational revenue growth measured at constant currency exchange rates of 81%, which was offset by a negative currency impact of \$0.9 million, or 3%.
- Cash, cash equivalents, and marketable securities totaled \$338.9 million at March 31, 2020, compared to \$452.7 million at December 31, 2019.
- Total GAAP operating expenses of \$132.0 million for the first quarter 2020 increased as compared to \$111.3 million for the first quarter 2019, reflecting continued investments in the Galafold launch, Pompe program, and gene therapy pipeline.
- Total non-GAAP operating expenses of \$116.7 million for the first quarter of 2020 increased as compared to \$96.2 million in the first quarter of 2019, reflecting continued investments in the Galafold launch, Pompe clinical study program, and gene therapy pipeline. First quarter non-GAAP operating expense on track for full-year guidance. Non-GAAP operating expense to decline throughout 2020 on path to achieving the 2020 expense guidance of \$410 million to \$420 million.¹
- Net loss was \$88.9 million, or \$0.35 per share, compared to a net loss of \$120.3 million, or \$0.56 per share, for the first quarter 2019.

¹ 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.

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.²
- Cash, cash equivalents, and marketable securities totaled \$338.9 million at March 31, 2020. The current cash position is anticipated to fund ongoing operations now well into 2H2022.

² 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.

Anticipated 2020 Milestones by Program

Galafold (migalastat) Oral Precision Medicine for Fabry Disease

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

AT-GAA for Pompe Disease

- Plans to initiate a Rolling Biologics License Application (BLA) for AT-GAA in 2020, with addition of complete clinical results for PROPEL in 1H2021 to support full approval
- Retrospective natural history study data in approximately 100 enzyme replacement therapy treated Pompe patients
- Additional supportive studies, including an open-label study in 12- to <18-year-old patients

Gene Therapy Portfolio

- Report further safety and efficacy data in the CLN6 Batten disease Phase 1/2 study and advance regulatory discussions to finalize clinical and regulatory path
- Report initial data from the CLN3 Batten disease Phase 1/2 study and advance regulatory discussions to finalize clinical and regulatory path
- Continue IND-enabling toxicology work in Pompe disease and progress towards IND
- Additional preclinical data expected across multiple programs with disclosure of up to two additional IND candidates
- Manufacturing advancements across portfolio



Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, May 7, 2020 at 8:30 a.m. ET to discuss the first quarter 2020 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: 1287896.

A live audio webcast and related presentation materials can also be accessed via the Investors section of the Amicus Therapeutics corporate website at ir.amicusrx.com. 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 May 7, 2020. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 1287896.

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, 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>.

EU Important Safety Information

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

- Galafold is not intended for concomitant use with enzyme replacement therapy.
- Galafold is not recommended for use in patients with Fabry disease who have severe renal impairment (<30 mL/min/1.73 m²). The safety and efficacy of Galafold in children 0–15 years of age have not yet been established.
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.



- While taking Galafold, effective birth control should be used. It is not known whether Galafold is excreted in human milk.
- Contraindications to Galafold include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on Galafold or switched to Galafold.
- OVERDOSE: General medical care is recommended in the case of Galafold overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received Galafold. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

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

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. 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, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product. In addition to the impact of the COVID-19 pandemic, 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. Statements regarding corporate financial guidance and financial goals and the attainment of such goals. 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 and the Quarterly Report filed on Form 10-Q 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:

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Director, Investor Relations
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(609) 662-3809

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TABLE 1

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

	Three Months Ended March 31,	
	2020	2019
Net product sales	\$ 60,525	\$ 34,046
Cost of goods sold	6,552	4,055
Gross profit	53,973	29,991
Operating expenses:		
Research and development	89,120	64,593
Selling, general, and administrative	40,215	44,303
Changes in fair value of contingent consideration payable	931	1,383
Depreciation and amortization	1,764	991
Total operating expenses	132,030	111,270
Loss from operations	(78,057)	(81,279)
Other income (expense):		
Interest income	1,515	2,639
Interest expense	(3,729)	(6,454)
Loss on exchange of convertible notes	—	(36,123)
Other (expense) income	(8,316)	1,086
Loss before income tax	(88,587)	(120,131)
Income tax expense	(361)	(168)
Net loss attributable to common stockholders	\$ (88,948)	\$ (120,299)
Net loss attributable to common stockholders per common share — basic and diluted	\$ (0.35)	\$ (0.56)
Weighted-average common shares outstanding — basic and diluted	256,968,248	213,519,287



TABLE 2

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

	March 31, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	\$ 123,231	\$ 142,837
Investments in marketable securities	215,642	309,903
Accounts receivable	40,555	33,284
Inventories	12,831	14,041
Prepaid expenses and other current assets	15,690	20,008
Total current assets	407,949	520,073
Operating lease right-of-use assets, less accumulated amortization of \$6,260 and \$5,342 at March 31, 2020 and December 31, 2019, respectively	32,501	33,315
Property and equipment, less accumulated depreciation of \$19,260 and \$17,604 at March 31, 2020 and December 31, 2019, respectively	47,688	47,705
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	29,414	28,317
Total Assets	\$ 738,349	\$ 850,207
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 16,239	\$ 21,722
Accrued expenses and other current liabilities	66,722	98,651
Deferred reimbursements	1,250	1,250
Operating lease liabilities	7,503	7,189
Total current liabilities	91,714	128,812
Deferred reimbursements	8,906	8,906
Convertible notes	2,167	2,131
Senior secured term loan	147,569	147,374
Contingent consideration payable	23,612	22,681
Deferred income taxes	5,051	5,051
Operating lease liabilities	52,522	53,531
Other non-current liabilities	4,214	5,296
Total liabilities	335,755	373,782
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$0.01 par value, 500,000,000 shares authorized, 257,449,955 and 255,417,869 shares issued and outstanding at March 31, 2020 and December 31, 2019, respectively	2,607	2,598
Additional paid-in capital	2,238,346	2,227,225
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	6,981	2,785
Unrealized (loss) gain on available-for-sale securities	(169)	40
Warrants	12,387	12,387
Accumulated deficit	(1,857,558)	(1,768,610)
Total stockholders' equity	402,594	476,425
Total Liabilities and Stockholders' Equity	\$ 738,349	\$ 850,207



TABLE 3

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

	Three Months Ended March 31,	
	2020	2019
Total operating expenses - as reported GAAP	\$ 132,030	\$ 111,270
Research and development:		
Share-based compensation	5,253	5,032
Selling, general and administrative:		
Share-based compensation	7,343	7,712
Changes in fair value of contingent consideration payable	931	1,383
Depreciation and amortization	1,764	991
Total operating expense adjustments to reported GAAP	15,291	15,118
Total operating expenses - as adjusted	\$ 116,739	\$ 96,152



1Q20 Financial Results Conference Call & Webcast

May 7, 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 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, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product. In addition to the impact of the COVID-19 pandemic, 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. Statements regarding corporate financial guidance and financial goals and the attainment of such goals. 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 and the Quarterly Report filed on Form 10-Q 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 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.

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
BIOLOGICS
Capabilities



**EMPLOYEES
in 27 Countries**



AT-GAA
Phase 3 in
Pompe Disease



\$338M+
Cash
as of 3/31/20

**Two Clinical-
Stage Gene
Therapies**

**GLOBAL
COMMERCIAL
ORGANIZATION**

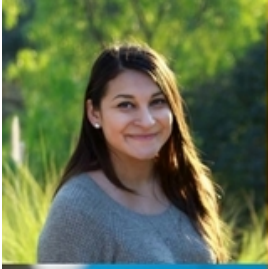
**Robust R&D
Engine**

Nearly 50+ Lysosomal
Disorders and More
Prevalent Rare Diseases

A graphic consisting of a grid of horizontal bars of varying lengths, representing different R&D capabilities or projects.

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...

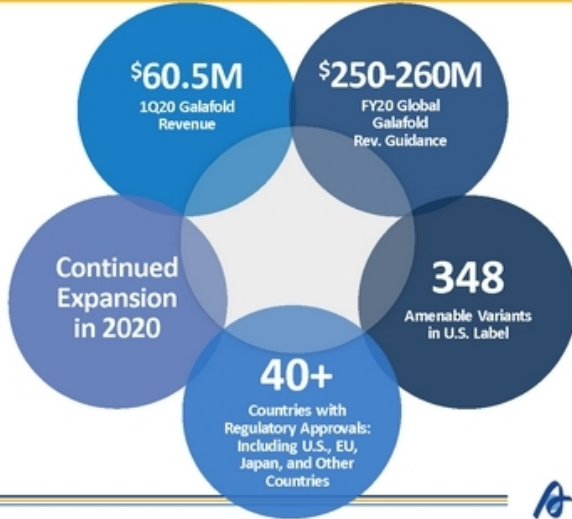
...taking a leadership role in the
treatment of Fabry disease

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

Galafold Snapshot (as of March 31, 2020)

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



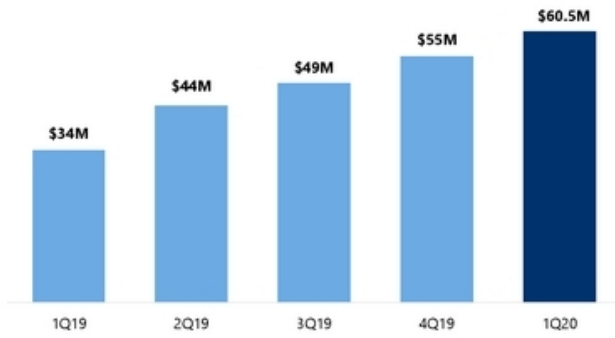
Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (20%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold including the full U.S. prescribing information, please visit <https://www.amicus-tx.com/galafold-us>. For further important safety information for Galafold, including contraindications, 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>.



Galafold Quarterly Performance

Quarterly Growth Remains Steady with 1Q20 Revenue of \$60.5M, Growing 78% Year-over-Year

Quarterly Galafold Sales

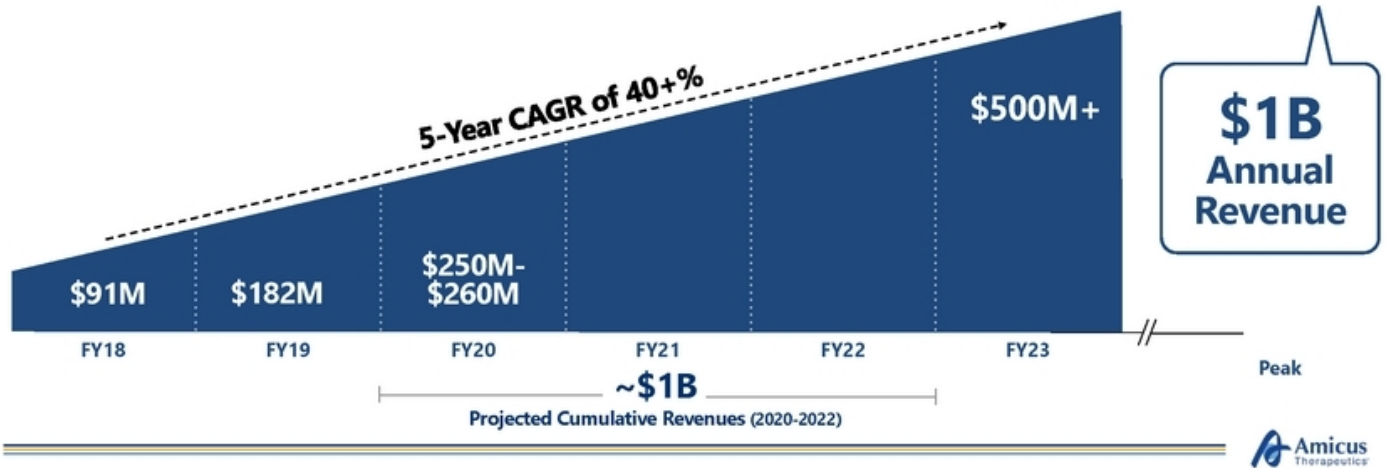


Year-over-Year Galafold Sales Growth



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



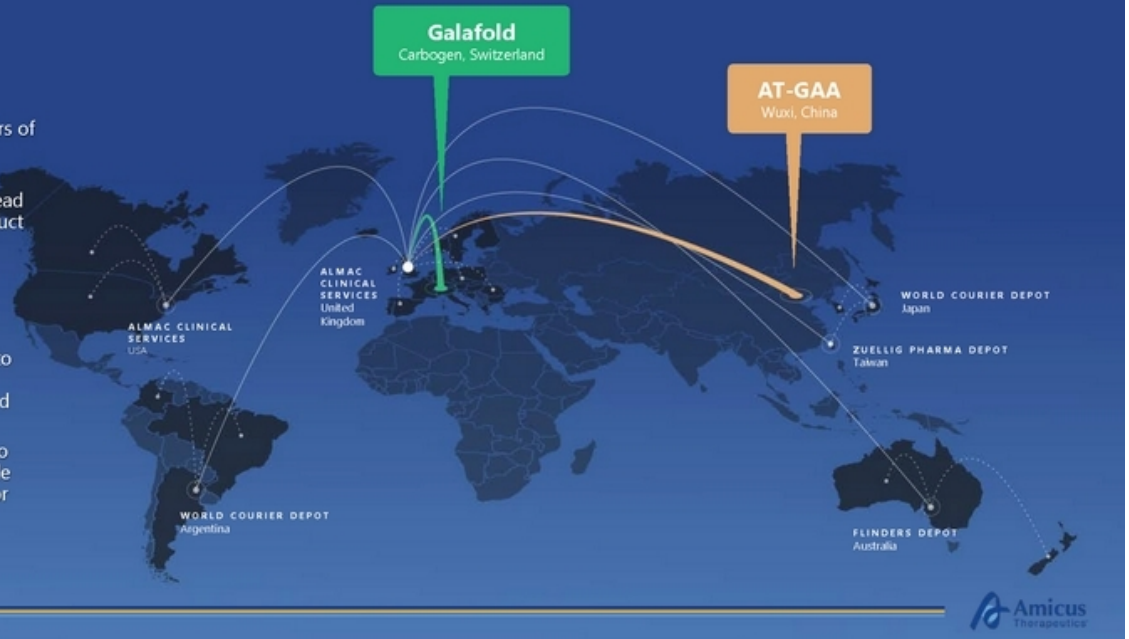
Global Supply Chain

Existing Supply Chain Strategy:

- **Galafold:** Hold multiple years of inventory in API and Drug Product
- **AT-GAA:** Built inventory ahead of time and move drug product to UK

Post COVID-19:

- **Galafold:** Push inventory into the supply chain as far as possible down to country and pharmacy level
- **AT-GAA:** Push inventory into supply chain as far as possible and coordinate site by site for delivery





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

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

AT-GAA: Key Takeaways



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

- PROPEL study timelines are on track with data expected 1H2021
 - To date, **97%** of the 2,250 planned infusions for the ongoing PROPEL study have been completed on schedule
- Breakthrough Therapy Designation and the Promising Innovative Medicine designation highlight unmet need in Pompe disease today
- U.S. FDA grants rolling BLA submission and company plans to initiate in 2H2020
- Expanded Access Program for infantile-onset Pompe patients underway
- Process performance qualification (PPQ) runs with our partners at WuXi have been successfully completed for the drug substance
- Peak revenue potential of \$1B-\$2B, with exclusivity well into 2030s



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	COMMERCIAL
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 Drug Designation
- RPD** - Rare Pediatric Disease Designation

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



Gene Therapy: Updates & Key Takeaways



Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future

- CLN6 Phase 1/2 interim data show profound impact with potential to become first ever approved gene therapy for fatal brain disease in children
- Additional patients to be dosed in Phase 1/2 study of CLN3 in 2021 with commercial supply
- Orphan drug designations granted in U.S. and EU for intrathecal AAV gene therapies for CLN6 and CLN3 Batten disease; CLN3 granted Fast Track designation by U.S. FDA
- Pompe gene therapy clinical candidate declared to move into IND-enabling studies
- Penn Collaboration is R&D engine, with rights to 50+ diseases
- 8 preclinical gene therapies in development

Combines Amicus and Penn Expertise Across Lysosomal and Rare Diseases

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





Financial Summary

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

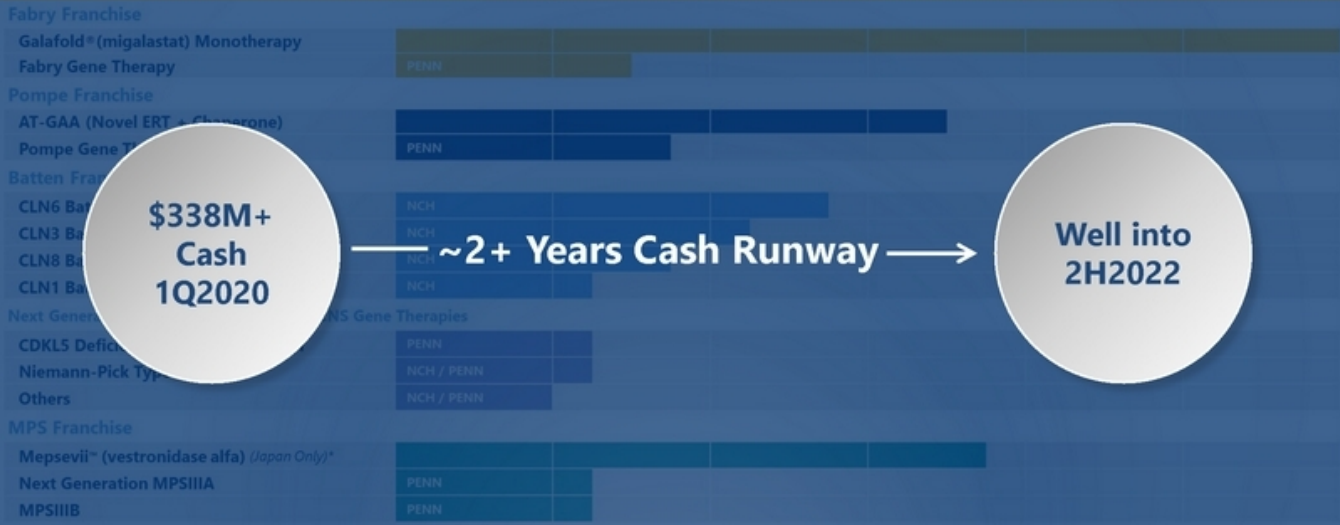
1Q2020 Select Financial Results

1Q2020 Revenue of \$60.5M Primarily from Global Galafold Sales

<i>(in thousands, except per share data)</i>	Mar. 31, 2020	Mar. 31, 2019
Product Revenue	60,525	34,046
Cost of Goods Sold	6,552	4,055
R&D Expense	89,120	64,593
SG&A Expense	40,215	44,303
Changes in Fair Value of Contingent Consideration	931	1,383
Depreciation and Amortization	1,764	991
Loss from Operations	(78,057)	(81,279)
Income Tax Expense	(361)	(168)
Net Loss	(88,948)	(120,299)
Net Loss Per Share	(0.35)	(0.56)

Cash Runway Now to Well into 2H2022 (~2+ years)

Fully funded through major milestones in portfolio and continued global growth



Financial Outlook: Key Takeaways

- Cash runway now well into 2H2022
 - Achieved through continued careful expense management, prioritization of very early stage research programs and more measured capital expenditures
- Non-GAAP quarterly operating expense expected to decline throughout 2020
- Company fully funded through major milestones in portfolio and continued global growth
- Cumulative Galafold projected revenue of \$1B+ in 2020-2022 offsets significant majority of company spend/investments
- Only modest additional capital required in the outer years to extend runway into profitability with multiple non-equity sources available as/when needed



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



Reconciliation

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

	Three Months Ended	
	March 31,	
	2020	2019
Total operating expenses - as reported GAAP	\$ 132,030	\$ 111,270
Research and development:		
Share-based compensation	5,253	5,032
Selling, general and administrative:		
Share-based compensation	7,343	7,712
Changes in fair value of contingent consideration payable	931	1,383
Depreciation and amortization	1,764	991
Total operating expense adjustments to reported GAAP	15,291	15,118
Total operating expenses - as adjusted	\$ 116,739	\$ 96,152