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 10, 2021**

AMICUS THERAPEUTICS, INC.
(Exact Name of Registrant as Specified in Its Charter)

Delaware
(State or Other Jurisdiction of Incorporation)

Delaware	001-33497	71-0869350
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(I.R.S. Employer Identification No.)**

3675 Market Street, Philadelphia PA 19104
(Address of Principal Executive Offices, and Zip Code)

215-921-7600
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 10, 2021, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the fiscal quarter ended March 31, 2021. A copy of this press release is attached hereto as Exhibit 99.1. The Company will host a conference call and webcast on May 10, 2021 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 10, 2021
99.2	May 10, 2021 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: May 10, 2021

AMICUS THERAPEUTICS, INC.
By: /s/ Ellen S. Rosenberg
Name: Ellen S. Rosenberg
Title: Chief Legal Officer and Corporate Secretary

Amicus Therapeutics Announces First Quarter 2021 Financial Results and Corporate Updates

Galafold® (migalastat) Performance Reflects Continued Strong Adoption in All Key Global Regions; On-Track to Achieve Revenue Guidance of $300M-$315M

1Q21 Total Galafold Revenue of $66.4M Driven by Continued Global Growth

Positive Pre-BLA Meeting Held with U.S. FDA for AT-GAA in Pompe Disease; Rolling BLA Submission On-Track for Completion in 2Q21 with Global Submissions Expected Throughout 2021

New Data from Pompe and Fabry Gene Therapy Programs to be Presented at American Society of Gene & Cell Therapy 24th Annual Meeting May 11th-14th

Continue to Strengthen Senior Management Team with Addition of Sébastien Martel as SVP, Strategy & Business Development

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

PHILADELPHIA, PA, May 10, 2021 – Amicus Therapeutics (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on discovering, developing and delivering novel medicines for rare diseases, today announced financial results for the quarter ended March 31, 2021.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, "Throughout the first quarter, we remained focused on furthering our vision for patients and on achieving our 2021 key strategic priorities, including the continued commercial growth of Galafold, progressing the regulatory and launch preparations for AT-GAA, and advancing our robust gene therapy pipeline. Through these efforts, we continued to fuel our mission to make a profound difference in the lives of patients."

"We are very pleased with the continued momentum of the Galafold uptake globally," said Bradley L. Campbell, President and Chief Operating Officer of Amicus Therapeutics, Inc. "In Q1, we saw an increase of patients on treatment and a continued high rate of compliance, despite quarter-over-quarter revenue being impacted by the typical uneven ordering patterns from Q4 to Q1 that were exacerbated by COVID. Importantly, the number of patients on Galafold at the end of the quarter were ahead of our internal estimates and the first weeks of Q2 continue to exceed expectations. Based on this momentum, and continuing to anticipate a second half recovery from COVID, we are confident in meeting our full-year 2021 guidance."

Corporate Highlights

- **Global revenue for Galafold® (migalastat) in the first quarter of 2021 was $66.4 million.** First quarter revenue represented a year-over-year increase of 9.8% from total revenue of $60.5 million in the first quarter of 2020. On a constant currency basis, first quarter total revenue was $63.0 million, representing operational revenue growth measured at constant currency exchange rates of 4.1%, which was benefited by a positive currency impact of $3.4 million, or 5.7%.

- **Galafold momentum continues to track ahead of internal expectations and remains on track to achieve the full year revenue guidance of $300M to $315M.**
  - First quarter revenue reflected increased patient demand offset by the timing of orders in ex-U.S. geographies, reauthorizations in the U.S., and irregular ordering patterns due to COVID, which was consistent with our expectation of non-linear quarter-to-quarter growth.
  - In Q1, the number of patients on Galafold were ahead of the Company's internal estimates as there was continued growth across the 30+ markets where Galafold is reimbursed.
  - The Company continues to expect the number of new patients starting on Galafold treatment in 2021 to be greater than in 2020.
  - Global compliance and adherence rates continue to exceed 90%.

- **On-track to complete the rolling BLA submission of AT-GAA in Pompe disease in the second quarter of this year** following positive written communication from a pre-BLA meeting with the U.S. FDA. Additional regulatory submissions in the European Union and in other geographies are expected throughout 2021.

- **Preclinical data from the Company's Fabry and Pompe gene therapy clinical candidates to be presented at the American Society of Gene & Cell Therapy 24th Annual Meeting on May 11th-14th.** As part of the research collaboration with the Gene Therapy Program of the Perelman School of Medicine at the University of Pennsylvania (Penn), new non-human primate data from the Fabry AAV gene therapy program will be presented. In addition, new data from our optimized gene therapy candidate in Pompe disease, which in preclinical models prevented the development of muscle fiber pathology in young Pompe mice and reversed pre-existing muscle fiber pathology in aged Pompe mice, will be presented. The data continue to validate the synergies of combining Amicus-engineered transgenes with Penn's AAV technologies to develop next-generation gene therapies.

- **Clinical Batten gene therapy programs continue to advance**. The Company continues to follow the first 13 CLN6 patients and the 4 CLN3 patients in their respective Phase 1/2 studies. Focus remains on progressing manufacturing, clinical, and regulatory activities to enable next clinical studies.

- **Cash position sufficient to achieve self-sustainability without the need for any future dilutive financings.** The Company continues to carefully manage expenses and investments, while executing on the Galafold launch, proceeding with AT-GAA global regulatory submissions and advancing development programs.

First Quarter 2021 Financial Results

- Total revenue in the first quarter 2021 was $66.4 million, a year-over-year increase of 9.8% from total revenue of $60.5 million in the first quarter of 2020. On a constant currency basis, first quarter 2021 total revenue was $63.0 million, representing operational revenue growth measured at constant currency exchange rates of 4.1%. Reported revenue was aided by a positive currency impact of $3.4 million, or 5.7%.
- Cash, cash equivalents, and marketable securities totaled $417.4 million at March 31, 2021, compared to $483.3 million at December 31, 2020.
- Total GAAP operating expenses of $112.9 million for the first quarter 2021 decreased as compared to $132.0 million for the first quarter 2020, reflecting the timing of investments in our pipeline.
- Total non-GAAP operating expenses of $90.5 million for the first quarter of 2021 decreased as compared to $116.7 million in the first quarter of 2020, reflecting the timing of investments in our pipeline.[1]
- Net loss was $65.7 million, or $0.25 per share, compared to a net loss of $88.9 million, or $0.35 per share, for the first quarter 2020.

[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.

2021 Financial Guidance

- For the full-year 2021, the Company anticipates total Galafold revenue of $300 million to $315 million. Double-digit revenue growth in 2021 is expected to be driven by continued operational growth and commercial execution across all major markets, including the U.S., EU, U.K. and Japan.
- Non-GAAP operating expense guidance for the full-year 2021 is $410 million to $420 million, driven by continued investment in the global Galafold launch, AT-GAA clinical studies and pre-launch activities, and advancing our gene therapy pipeline.[2]
- Cash, cash equivalents, and marketable securities totaled $417.4 million at March 31, 2021. Based on current operating models, the Company believes that the current cash position and expected future revenues are sufficient to fund the Company's operations and ongoing research programs through to self-sustainability.

[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 2021 Milestones by Program

Galafold (migalastat) Oral Precision Medicine for Fabry Disease
- Continue revenue growth in 2021
- Plan to expand EU label to cover adolescent population
- Continue geographic expansion

- Registry and other Phase 4 studies

AT-GAA for Pompe Disease
- Complete the BLA submission in 2Q21 and the EU MAA submission to be completed in 2H2021
- Ongoing supportive studies, including pediatric and extension studies

Gene Therapy Portfolio
- Advance manufacturing activities and regulatory discussions for the CLN6 Batten disease gene therapy program to enable dosing of additional patients with GMP clinical grade material
- Reported initial data from the CLN3 Batten disease gene therapy Phase 1/2 study; Advance manufacturing activities and regulatory discussions to enable dosing additional patients with GMP clinical grade material
- Continue to progress IND-enabling work in both Pompe and Fabry gene therapies
- Disclose additional preclinical data and potential IND candidate declarations across multiple preclinical programs
- Manufacturing advancements and updates across the portfolio

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, May 10, 2021 at 8:30 a.m. ET to discuss the first quarter 2021 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: 5767104.

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 10, 2021. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 5767104.

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 galactosidase alpha gene (*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 (≥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 m^2). 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, and revenue goals, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations and/or revenue 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 or to treatment sites. 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, UK, 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, commercialization 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 corporate financial guidance and financial goals and the attainment of such goals and 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, 2020 and Quarterly Report 10-Q for the quarter ended March 31, 2021, 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.

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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,	
	2021	**2020**
Net product sales	$ 66,402	$ 60,525
Cost of goods sold	6,539	6,552
Gross profit	59,863	53,973
Operating expenses:		
Research and development	64,117	89,120
Selling, general, and administrative	46,726	40,215
Changes in fair value of contingent consideration payable	471	931
Depreciation and amortization	1,604	1,764
Total operating expenses	112,918	132,030
Loss from operations	(53,055)	(78,057)
Other (expense) income:		
Interest income	165	1,515
Interest expense	(7,992)	(3,729)
Other expense	(3,200)	(8,316)
Loss before income tax	(64,082)	(88,587)
Income tax expense	(1,582)	(361)
Net loss attributable to common stockholders	$ (65,664)	$ (88,948)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.25)	$ (0.35)
Weighted-average common shares outstanding — basic and diluted	264,369,317	256,968,248

TABLE 2

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

	March 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 184,833	$ 163,240
Investments in marketable securities	232,596	320,029
Accounts receivable	44,931	46,923
Inventories	18,801	19,556
Prepaid expenses and other current assets	21,730	29,721
Total current assets	502,891	579,469
Operating lease right-of-use assets, less accumulated amortization of $7,499 and $7,574 at March 31, 2021 and December 31, 2020, respectively	22,363	23,296
Property and equipment, less accumulated depreciation of $15,961 and $14,487 at March 31, 2021 and December 31, 2020, respectively	43,445	43,863
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	20,538	19,095
Total Assets	$ 810,034	$ 886,520
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 16,110	$ 17,063
Accrued expenses and other current liabilities	57,178	96,841
Contingent consideration payable	19,600	8,900
Operating lease liabilities	6,764	6,872
Total current liabilities	99,652	129,676
Deferred reimbursements	7,406	7,406
Long-term debt	389,789	389,254
Contingent consideration payable	6,696	16,925
Deferred income taxes	4,896	4,896
Operating lease liabilities	44,431	45,604
Other non-current liabilities	6,268	6,379
Total liabilities	559,138	600,140
Commitments and contingencies		
Stockholders' equity		
Common stock, $0.01 par value, 500,000,000 shares authorized, 266,007,718 and 262,063,461 shares issued and outstanding at March 31, 2021 and December 31, 2020, respectively	2,680	2,650
Additional paid-in capital	2,350,507	2,308,578
Accumulated other comprehensive income (loss):		
Foreign currency translation adjustment	9,020	8,412
Unrealized loss on available-for-sale securities	(185)	(185)
Warrants	—	12,387
Accumulated deficit	(2,111,126)	(2,045,462)
Total stockholders' equity	250,896	286,380
Total Liabilities and Stockholders' Equity	$ 810,034	$ 886,520

TABLE 3

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

	Three Months Ended March 31,	
	2021	**2020**
Total operating expenses - as reported GAAP	**$ 112,918**	**$ 132,030**
Research and development:		
Share-based compensation	6,305	5,253
Selling, general and administrative:		
Share-based compensation	14,049	7,343
Changes in fair value of contingent consideration payable	471	931
Depreciation and amortization	1,604	1,764
Total operating expense adjustments to reported GAAP	22,429	15,291
Total operating expenses - as adjusted	$ 90,489	$ 116,739

Exhibit 99.2

Amicus Therapeutics®

1Q21 Financial Results Conference Call & Webcast

May 10, 2021

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 presentation may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, and revenue goals, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations and/or revenue 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 or to treatment sites. 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, UK, 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, commercialization 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 corporate financial guidance and financial goals and the attainment of such goals and 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, 2020 and on Form 10-Q for the quarter ended March 31, 2021, 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.

Non-GAAP Financial Measures

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. 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

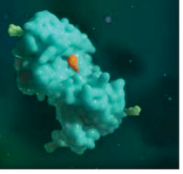
Galafold® (migalastat)
First Oral Precision Medicine for Fabry Disease

Gene Therapy PLATFORM
Protein Engineering & Glycobiology

World Class BIOLOGICS Capabilities

EMPLOYEES in 27 Countries

AT-GAA
Phase 3 in Pompe Disease



GLOBAL COMMERCIAL ORGANIZATION

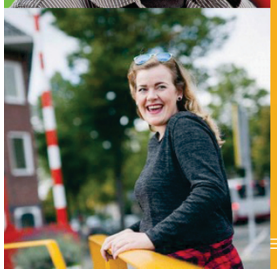
Robust R&D Engine
Nearly 50+ Lysosomal Disorders and More Prevalent Rare Diseases

$417.4M Cash as of 3/31/21

Two Clinical-Stage Gene Therapies

2021 Key Strategic Priorities

1. Achieve double-digit Galafold growth and revenue of $300M to $315M
2. Report data from the AT-GAA Phase 3 PROPEL study and complete BLA and MAA filings for regulatory approvals
3. Advance clinical studies, regulatory discussions and scientific data across industry leading gene therapy pipeline
4. Further manufacturing capabilities and capacity to build world-class technical operations to support all gene therapy programs
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, 2021)

Galafold 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 ERT

One of the Most Successful Rare Disease Launches

- **$66.4M** 1Q21 Galafold Revenue
- **1,384** Amenable Mutations Included in the EU Label
- **$300M-$315M** FY21 Global Galafold Revenue
- **Continued Expansion in 2021**
- **348** Amenable Variants in U.S. Label
- **40+** Countries with Regulatory Approvals: including U.S., EU, Japan, and other Countries

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.amicusrx.com/pi/Galafold.pdf. 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.

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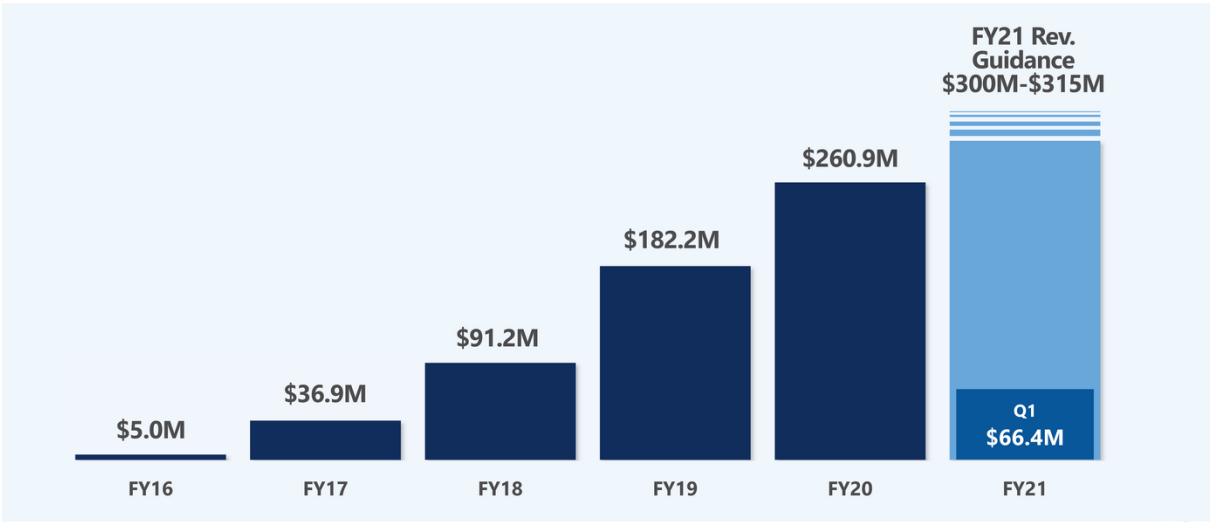
Galafold Success and FY21 Revenue Guidance

Galafold momentum remains on track to achieve full year 2021 revenue guidance



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Outlook for 2021

Continued double-digit Galafold revenue growth to $300M-$315M in 2021

- Global demand remains strong with continued growth anticipated in 2021 and beyond
- Number of patients on Galafold were ahead of the Company's internal expectations at end of Q1
- In 2021, project double-digit revenue growth with net new patient starts expected to be greater than in 2020
- COVID continues to impact time between patient identification and treatment initiation
- Expect higher patient adds and revenue growth in the second half of 2021 as COVID impact eases
- Continue to see >90% compliance and adherence rates globally

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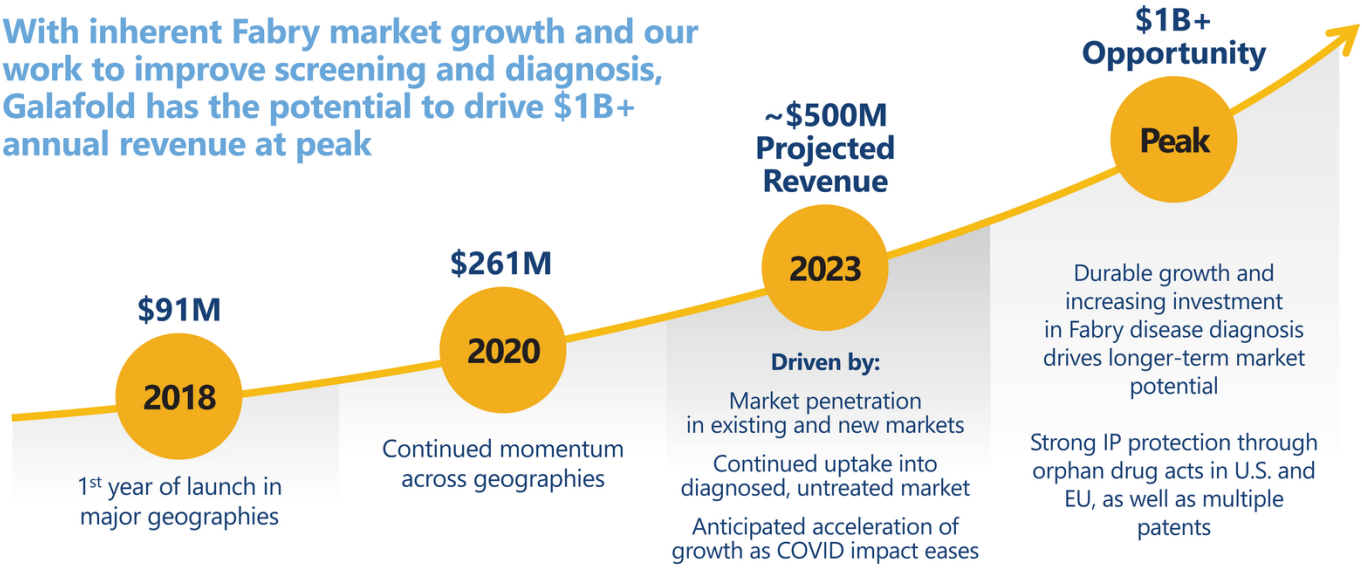
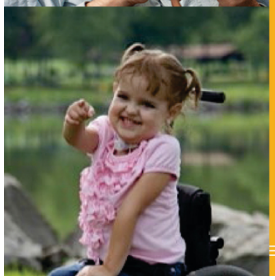
Galafold Opportunity

With inherent Fabry market growth and our work to improve screening and diagnosis, Galafold has the potential to drive $1B+ annual revenue at peak





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

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

Pompe Disease Overview

Pompe disease is a severe and fatal neuromuscular disease and one of the most prevalent lysosomal disorders with very high unmet medical need

- 5,000 – 10,000+ patients diagnosed WW[1]; newborn screening suggests underdiagnosis
- Age of onset ranges from infancy to adulthood
- Patients on current standard of care decline after ~2 years
- Respiratory and cardiac failure are leading causes of morbidity and mortality
- Deficiency of GAA leading to glycogen accumulation and cellular dysfunction
- Symptoms include muscle weakness, respiratory failure and cardiomyopathy
- ~$1B+ global Pompe ERT sales[2]

1. National Institute of Neurological Disorders and Stroke (NIH). 2. Based on calendar year ending December 31, 2020. Source: Sanofi Press Releases

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Phase 3 PROPEL Study

Primary, Key Secondary and Biomarker Endpoint Heat Map

Endpoints across motor function, pulmonary function, muscle strength, PROs and biomarkers favored AT-GAA over alglucosidase alfa in both the overall and ERT experienced populations

Overall Population

	Alglucosidase alfa	AT-GAA
Motor Function		6MWD
		GSGC*
Pulmonary Function		FVC*
Muscle Strength		Lower MMT
PROs		PROMIS-Physical
		PROMIS-Fatigue
Biomarker		Hex4*
		CK*

ERT Experienced Population

	Alglucosidase alfa	AT-GAA
Motor Function		6MWD*
		GSGC*
Pulmonary Function		FVC*
Muscle Strength		Lower MMT
PROs		PROMIS-Physical
		PROMIS-Fatigue
Biomarker		Hex4*
		CK*

Note: * Nominal P-value <0.05; based on LOCF means

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AT-GAA: Next Steps

- Rolling BLA submission expected to complete in 2Q
- Other key regulatory submissions for approval throughout 2021 including MAA in Europe
- Potential for early approval under EAMS framework with Priority Innovative Medicines Designation in U.K.
- 150+ patients worldwide now being treated with AT-GAA including adults, adolescents and infants
- Pediatric study for Pompe patients aged 12 to <18 with late-onset Pompe disease ongoing
- Clinical study for Pompe patients with infantile-onset disease expected to begin this year
- Expanded access program for Pompe infantile patients and adult-onset patients open and has enrolled multiple patients with Pompe. Further expanded access for all Pompe patients being considered.

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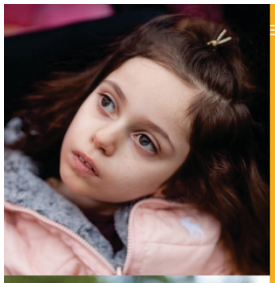
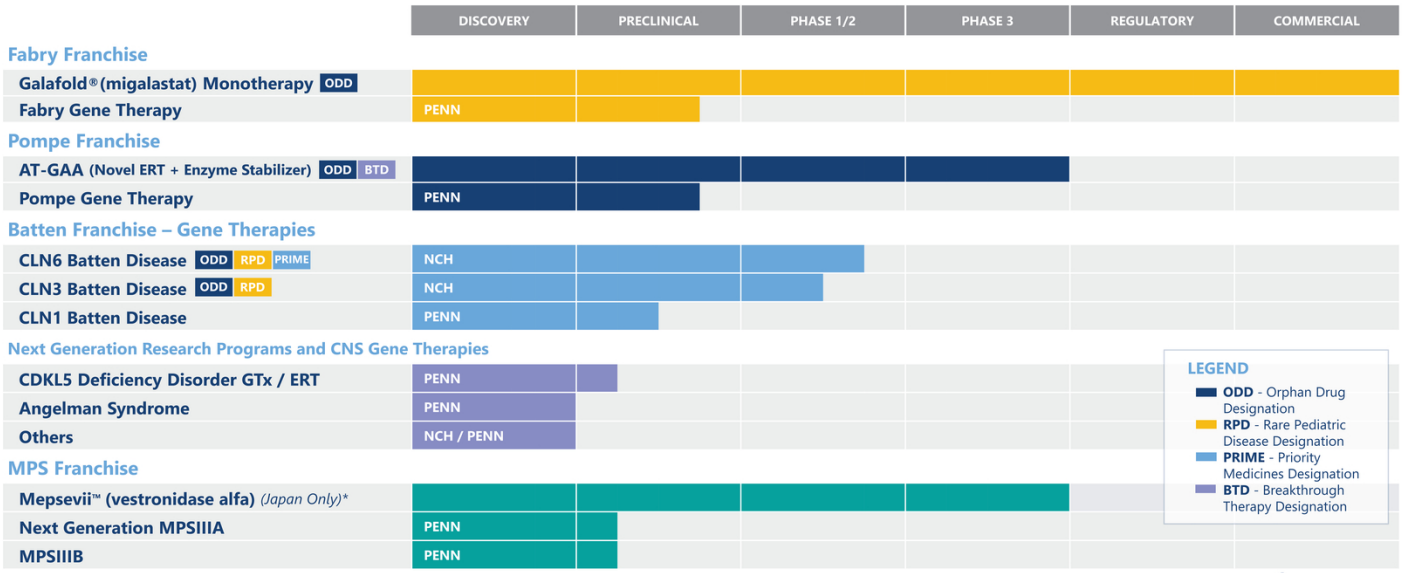


Next Generation Gene Therapy Platform

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

A RARE PORTFOLIO



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

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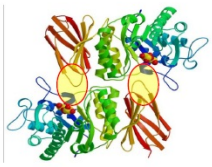
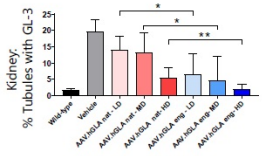
Amicus Protein Engineering Expertise & Technologies for Gene Therapy

Differentiated gene therapy approach for greater potency and optimized cross correction through transgene engineering for stability and targeting

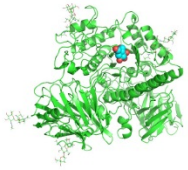
Fabry Gene Therapy

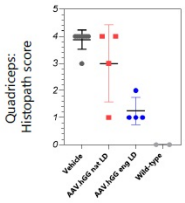




- Proprietary AAV capsid
- Pantropic capsid and ubiquitous promoter
- Engineered hGLA transgene at dimer interface designed for improved stability and optimized cross correction
- Preclinical data demonstrate robust substrate reduction across all Fabry disease relevant tissues, including first evidence of dorsal root ganglia storage reduction

Pompe Gene Therapy





- Proprietary AAV capsid
- Pantropic capsid and ubiquitous promoter
- Engineered hGAA transgene with cell receptor binding motif designed for improved uptake and optimized cross correction
- Preclinical data demonstrate robust glycogen reduction in all key Pompe disease tissues, including reduction in neurons of central nervous system

Note: Data from studies in KO GLA and GAA mice

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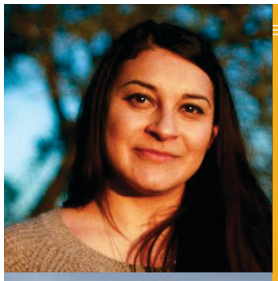

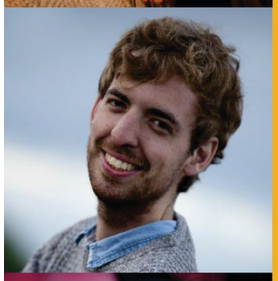

Financial Summary

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

1Q2021 Select Financial Results

1Q2021 revenue of $66.4M primarily from global Galafold sales

(in thousands, except per share data)	Mar. 31, 2021	Mar. 31, 2020
Product Revenue	$66,402	$60,525
Cost of Goods Sold	6,539	6,552
R&D Expense	64,117	89,120
SG&A Expense	46,726	40,215
Changes in Fair Value of Contingent Consideration	471	931
Depreciation and Amortization	1,604	1,764
Loss from Operations	(53,055)	(78,057)
Income Tax Expense	(1,582)	(361)
Net Loss	(65,664)	(88,948)
Net Loss Per Share	(0.25)	(0.35)

Financial Outlook: Key Takeaways

- Reaffirming full-year Galafold revenue guidance of $300 million to $315 million
- Non-GAAP operating expense guidance for 2021 is expected to remain flat at $410 million to $420 million
  - Driven by disciplined expense management and continued investment in the global Galafold launch, AT-GAA clinical studies and pre-launch activities and advancing our gene therapy pipeline
- Current cash position is sufficient to achieve self-sustainability without the need for any future dilutive financing

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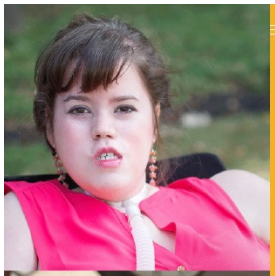

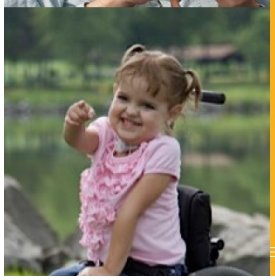

Closing Remarks

"We believe in our future to build long-term value for our stakeholders"
- Amicus Belief Statement

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement

Amicus Therapeutics®

Appendix

Reconciliation

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

	Three Months Ended March 31,	
	2021	**2020**
Total operating expenses - as reported GAAP	**$ 112,918**	**$ 132,030**
Research and development:		
Share-based compensation	6,305	5,253
Selling, general and administrative:		
Share-based compensation	14,049	7,343
Changes in fair value of contingent consideration payable	471	931
Depreciation and amortization	1,604	1,764
Total operating expense adjustments to reported GAAP	22,429	15,291
Total operating expenses - as adjusted	**$ 90,489**	**$ 116,739**