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

FORM 8-K

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

Date of Report (Date of earliest event reported): **November 9, 2021**

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

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 November 9, 2021, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the fiscal quarter ended September 30, 2021. A copy of this press release is attached hereto as Exhibit 99.1. The Company will host a conference call and webcast on November 9, 2021 to discuss its second 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 November 9, 2021
99.2	November 9, 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.

AMICUS THERAPEUTICS, INC.

Date: November 9, 2021

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

Amicus Therapeutics Announces Third Quarter 2021 Financial Results and Corporate Updates

3Q21 Total Galafold® Revenue of $79.5M – an 18% increase over 3Q20

Reiterating 2021 Revenue Guidance of $300M-$315M

AT-GAA BLA and NDA for Pompe Disease Accepted for Review by the U.S. FDA;
Marketing Authorization Applications Submitted to European Medicines Agency

On Track to Complete Planned Business Combination of Amicus Gene Therapy Business
with ARYA IV Resulting in the Launch of Caritas Therapeutics in Late 2021/Early 2022

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

PHILADELPHIA, PA, Nov. 9, 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 September 30, 2021.

"In the second half of 2021, we have furthered our mission for people living with devastating rare diseases through the commercial execution of Galafold and advancement of the global regulatory filings and launch preparations for AT-GAA," stated, John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "As previously announced, our teams are proceeding with the business combination of our leading gene therapy portfolio with ARYA IV to launch Caritas Therapeutics, a next-generation genetic medicines company. This transaction will serve patients and shareholders well by accelerating funding for our gene therapy pipeline, while simultaneously strengthening the financial profile of Amicus. We are immensely excited for what the future of science and biotechnology holds as we accelerate our commitment to extraordinary patient dedication."

Corporate Highlights

- **Global revenue for Galafold® (migalastat) in the third quarter of 2021 reached $79.5 million, representing a year-over-year increase of 18% from total revenue of $67.4 million in the third quarter of 2020.**

- **Long-term Galafold data published in the September 2021 Issue of *Molecular Genetics and Metabolism Reports* showing generally stable renal function in patients with an amenable variant during long-term treatment, up to 8.6 years, of Galafold, irrespective of treatment status, gender or phenotype.**

- **U.S. Food and Drug Administration (FDA) accepted for review the Biologics License Application (BLA) for cipaglucosidase alfa and the New Drug Application (NDA) for miglustat, the two components of AT-GAA.** The FDA has set a Prescription Drug User Fee Act (PDUFA) action date of May 29, 2022 for the NDA and July 29, 2022 for the BLA.

- **Marketing Authorization Applications (MAA) submitted to the European Medicines Agency (EMA) for AT-GAA in Pompe disease.** The filings are based on the evaluation of the effects of AT-GAA in people living with Pompe disease and its safety profile, which include data from the Phase 1/2 and Phase 3 PROPEL studies as well as data from the long-term open-label extension study.

- **Amicus and ARYA Sciences Acquisition Corp IV ("ARYA IV"), a special purpose acquisition company, announced their intent to launch a next-generation genetic medicine company, Caritas Therapeutics, Inc.** Through a definitive business combination agreement, the Amicus gene therapy business will be acquired by ARYA IV. The transaction will result in two independent publicly traded companies with attractive stand-alone investment profiles. Amicus will become the largest shareholder in Caritas with a ~36% ownership stake (assuming no redemptions by ARYA's shareholders) and retain co-development and commercialization rights to the Fabry and Pompe gene therapy programs, as well as negotiation rights on select future muscular dystrophy programs. The Form S-4 is now filed with the Securities and Exchange Commission and the launch of Caritas Therapeutics is expected in late 2021 or early 2022.

- **Cash position sufficient to achieve self-sustainability and profitability in 2023.** Following the transaction with ARYA IV, the previously announced ~$200 million private investment from leading biotechnology investors, and through careful management of expenses, the Company is on the path to achieve self-sustainability and profitability by 2023 as it executes on the global Galafold launch and AT-GAA global regulatory filings.

Third Quarter 2021 Financial Results

- Total revenue in the third quarter of 2021 was $79.5 million, a year-over-year increase of 18% from total revenue of $67.4 million in the third quarter of 2020. On a constant currency basis, third quarter 2021 total revenue was $78.5 million, representing operational revenue growth measured at constant currency exchange rates of 16.5%. Reported revenue was aided by a positive currency impact of $1.0 million, or 1.5%.
- Cash, cash equivalents, and marketable securities totaled $557.0 million at September 30, 2021, compared to $483.3 million at December 31, 2020.
- Total GAAP operating expenses of $110.2 million for the third quarter of 2021 decreased as compared to $111.8 million for the third quarter 2020, reflecting the timing of investments in our pipeline.
- Total non-GAAP operating expenses of $93.6 million for the third quarter of 2021 increase as compared to $92.4 million in the third quarter of 2020.[1]
- Net loss was reduced to $50.3 million, or $0.19 per share, compared to a net loss of $64.0 million, or $0.25 per share, for the third 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, UK 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]
- 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.

2021 Milestones by Program

Galafold (migalastat) Oral Precision Medicine for Fabry Disease

- Continue revenue growth in 2021
- EU label expanded to cover adolescent population
- Continue geographic expansion
- Registry and other Phase 4 studies

AT-GAA for Pompe Disease

- BLA and NDA submissions accepted for review in 3Q21; EU MAA submissions completed in 4Q21
- Ongoing supportive studies, including pediatric and extension studies

Gene Therapy Portfolio

- 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 Good Manufacturing Practice (GMP) clinical-grade material
- Advance manufacturing activities and regulatory discussions for the CLN6 Batten disease gene therapy program
- Continue to progress IND-enabling studies, manufacturing activities and regulatory discussions in both Fabry and Pompe 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, November 9, 2021, at 8:30 a.m. ET to discuss the third 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: 3686792.

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 November 9, 2021. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 3686792.

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 m2). The safety and efficacy of Galafold in children less than 12 years of age have not yet been established. No data are available.
- 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.
- Galafold 123 mg capsules are not for children (≥12 years) weighing less than 45 kg.
- 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 September 30, 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.

CONTACTS:

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TABLE 1

Amicus Therapeutics, Inc.

Consolidated Statements of Operations

(in thousands, except share and per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
Net product sales	$ 79,545	$ 67,437	$ 223,360	$ 190,315
Cost of goods sold	11,696	8,399	26,615	21,627
Gross profit	67,849	59,038	196,745	168,688
Operating expenses:				
Research and development	59,333	70,419	186,453	229,150
Selling, general, and administrative	46,107	37,850	135,109	112,722
Changes in fair value of contingent consideration payable	3,288	1,034	4,780	2,680
Depreciation and amortization	1,520	2,496	4,691	6,299
Total operating expenses	110,248	111,799	331,033	350,851
Loss from operations	(42,399)	(52,761)	(134,288)	(182,163)
Other income (expense):				
Interest income	108	518	323	2,898
Interest expense	(8,165)	(6,784)	(24,307)	(14,148)
Loss on extinguishment of debt	(257)	(7,276)	(257)	(7,276)
Other income (expense)	237	3,019	(2,729)	29
Loss before income tax	(50,476)	(63,284)	(161,258)	(200,660)
Income tax benefit (expense)	182	(727)	(5,925)	(4,791)
Net loss attributable to common stockholders	$ (50,294)	$ (64,011)	$ (167,183)	$ (205,451)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.19)	$ (0.25)	$ (0.63)	$ (0.80)
Weighted-average common shares outstanding — basic and diluted	267,464,637	259,161,799	266,085,788	258,091,170

TABLE 2

Amicus Therapeutics, Inc.

Consolidated Balance Sheets

(in thousands, except share and per share amounts)

	September 30, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 385,903	$ 163,240
Investments in marketable securities	171,057	320,029
Accounts receivable	51,427	46,923
Inventories	22,072	19,556
Prepaid expenses and other current assets	20,081	29,721
Total current assets	650,540	579,469
Operating lease right-of-use assets, net	21,270	23,296
Property and equipment, less accumulated depreciation of $18,789 and $14,487 at September 30, 2021 and December 31, 2020, respectively	41,991	43,863
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	22,077	19,095
Total Assets	$ 956,675	$ 886,520
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 24,474	$ 17,063
Accrued expenses and other current liabilities	72,453	96,841
Contingent consideration payable	17,000	8,900
Operating lease liabilities	7,175	6,872
Total current liabilities	121,102	129,676
Deferred reimbursements	7,406	7,406
Long-term debt	388,719	389,254
Contingent consideration payable	7,605	16,925
Deferred income taxes	4,896	4,896
Operating lease liabilities	43,495	45,604
Other non-current liabilities	6,823	6,379
Total liabilities	580,046	600,140
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 shares authorized, 278,585,092 and 262,063,461 shares issued and outstanding at September 30, 2021 and December 31, 2020, respectively	2,805	2,650
Additional paid-in capital	2,579,953	2,308,578
Accumulated other comprehensive income (loss):		
Foreign currency translation adjustment	6,617	8,412
Unrealized loss on available-for-sale securities	(184)	(185)
Warrants	83	12,387
Accumulated deficit	(2,212,645)	(2,045,462)
Total stockholders' equity	376,629	286,380
Total Liabilities and Stockholders' Equity	$ 956,675	$ 886,520

TABLE 3

Amicus Therapeutics, Inc.

Reconciliation of Non-GAAP Financial Measures

(in thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
Total operating expenses - as reported GAAP	**$ 110,248**	**$ 111,799**	**$ 331,033**	**$ 350,851**
Research and development:				
Share-based compensation	3,775	8,626	13,232	17,241
Selling, general and administrative:				
Share-based compensation	8,066	7,282	30,699	19,671
Changes in fair value of contingent consideration payable	3,288	1,034	4,780	2,680
Depreciation and amortization	1,520	2,496	4,691	6,299
Total operating expense adjustments to reported GAAP	16,649	19,438	53,402	45,891
Total operating expenses - as adjusted	$ 93,599	$ 92,361	$ 277,631	$ 304,960

Exhibit 99.2

Amicus Therapeutics®

3Q21 Financial Results Conference Call & Webcast

November 9, 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 September 30, 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

Amicus has built 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
Registration in Pompe Disease

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

$557M
Cash
as of 9/30/21

Two Clinical-Stage Gene Therapies

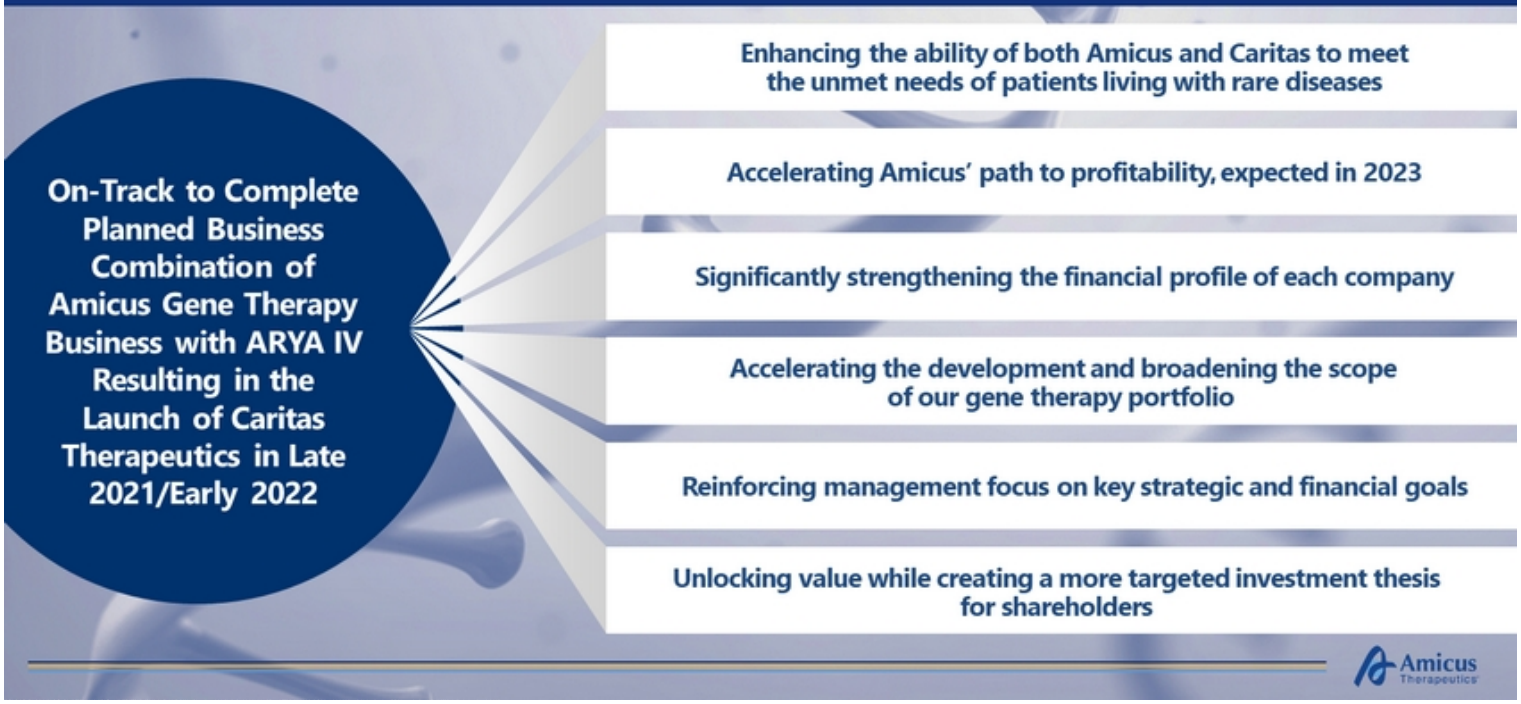
GLOBAL COMMERCIAL ORGANIZATION

Amicus Therapeutics

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

Caritas Strategic Rationale



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 September 30, 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

- $79.5M +18% YoY — 3Q21 Galafold Revenue
- $300M-$315M — FY21 Global Galafold Revenue
- 348 — Amenable Variants in U.S. Label
- 40+ — Countries with Regulatory Approvals: Including U.S., EU, U.K., Japan and Other Countries
- Continued Expansion in 2021
- 1,384 — Amenable Mutations Included in the EU Label

Amicus Therapeutics

Galafold Success and FY21 Revenue Guidance

Galafold momentum remains on track to deliver within full year 2021 revenue guidance

Galafold Long-Term Treatment Publication

Published in the September 2021 Issue of *Molecular Genetics and Metabolism Reports* showing generally stable renal function during long-term treatment

Key Findings[1,2]

- Migalastat-treated patients had generally stable renal function for up to 8.6 years
- Migalastat stabilized eGFR in ERT-naive and ERT-experienced males and females compared to historical untreated controls
- Migalastat generally stabilized eGFR in ERT-naïve male patients with the classic phenotype

Molecular Genetics and Metabolism Reports 28 (2021) 100706

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Molecular Genetics and Metabolism Reports

journal homepage: www.elsevier.com/locate/ymgmr

Long-term follow-up of renal function in patients treated with migalastat for Fabry disease

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[i] Department of Nephrology, Royal Melbourne Hospital, University of Melbourne, Parkville, Victoria, Australia

1. Study limitations: Post hoc design, small sample sizes in some subgroups, lack of statistical comparisons with untreated or ERT-treated historical cohorts, assessment of renal function using eGFR, and the heterogeneity of statistical methods used to estimate eGFR slopes in the literature limited direct comparisons.
2. The study includes data that are not in the FDA-approved Prescribing Information (PI) for Galafold and the clinical relevance of the changes in eGFR in the treatment of Fabry disease has not been established.

Amicus Therapeutics

Outlook for 2021

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

- Global demand remains strong with 3Q21 revenue growth rate of 18% and continued growth anticipated in 2021 and beyond
- In 2021, project double-digit revenue growth with net new patient starts expected to be greater than in 2020
- Expanded EU label following the European Commission approval for use in adolescents
- COVID continues to impact time between patient identification and treatment initiation
- Expect higher patient adds and revenue growth in 2H2021
- Continue to see >90% compliance and adherence rates globally

Amicus Therapeutics

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

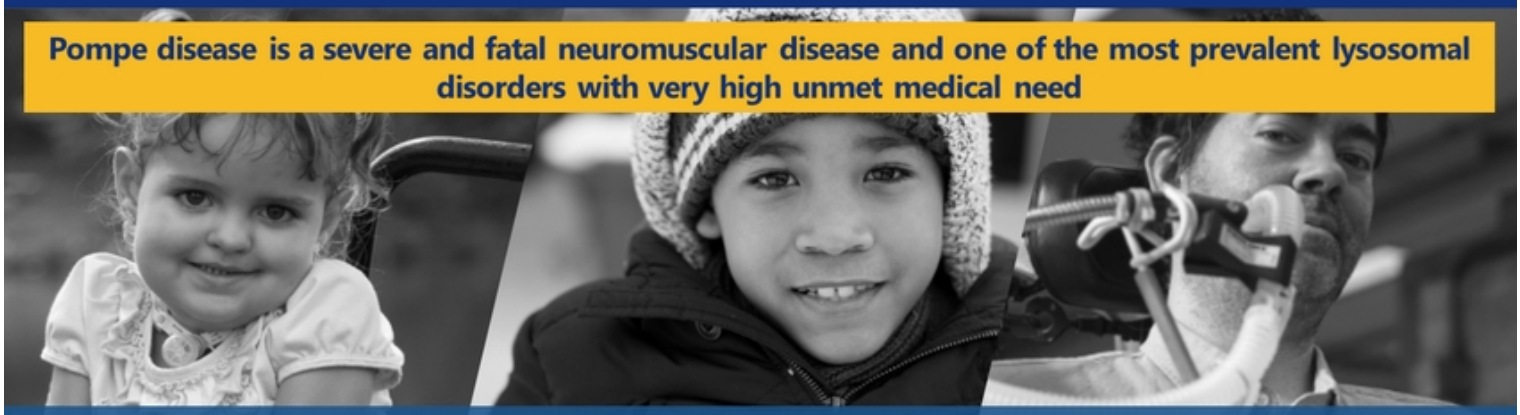
Amicus Therapeutics

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
- ~$1.1B+ global Pompe ERT sales[2]

1. National Institute of Neurological Disorders and Stroke (NIH). 2. Based on year ending June 30, 2021. Source: Sanofi Press Releases

Amicus Therapeutics

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

		Overall population				ERT-experienced			
	Endpoints	Cipaglucosidase alfa/miglustat n=85		Alglucosidase alfa/placebo n=37		Cipaglucosidase alfa/miglustat n=65		Alglucosidase alfa/placebo n=30	
		Baseline, mean	CFBL at week 52, mean (SE)	Baseline, mean	CFBL at week 52, mean (SE)	Baseline, mean	CFBL at week 52, mean (SE)	Baseline, mean	CFBL at week 52, mean (SE)
Motor function	6MWD, m	357.9	20.8 (4.6)	351.0	7.2 (6.6)	346.9	16.9 (5.0)	334.6	0.0 (7.2)
	GSGC total score	14.5	-0.5 (0.3)	14.5	0.8 (0.3)	15.6	-0.5 (0.3)	15.5	0.6 (0.4)
	10-meter walk, s	9.7	-0.5 (0.6)	9.6	1.9 (1.0)	10.4	-0.6 (0.9)	10.2	2.5 (1.2)
	4-stair climb, s	14.1	-8.5 (7.9)	8.2	0.3 (1.0)	17.3	-11.1 (10.5)	9.3	0.6 (1.2)
	Gower's maneuver, s	10.8	-0.3 (0.7)	19.8	-2.2 (1.4)	11.5	-0.4 (0.8)	23.9	-2.6 (1.9)
	Rising from chair, s	13.6	-10.2 (9.7)	4.5	-0.5 (0.7)	17.6	-13.7 (13.0)	5.2	-0.4 (0.9)
Pulmonary function	FVC, % predicted	70.7	-0.9 (0.7)	69.7	-4.0 (0.8)	67.9	0.1 (0.7)	67.5	-4.0 (0.9)
	MIP, % predicted	61.8	2.1 (2.1)	59.9	-2.7 (2.8)	61.3	1.0 (2.5)	55.0	-1.7 (1.5)
	MEP, % predicted	70.7	0.6 (2.4)	65.1	-1.6 (2.1)	70.7	-2.7 (2.7)	62.2	-3.9 (1.8)
Muscle strength	Lower MMT score	28.0	1.6 (0.4)	27.7	0.9 (0.4)	26.4	1.6 (0.5)	26.1	0.9 (0.5)
	Upper MMT score	34.3	1.5 (0.4)	34.7	0.7 (0.6)	33.7	1.8 (0.4)	34.2	0.4 (0.7)
	Total MMT score	62.3	3.1 (0.7)	62.4	1.4 (0.8)	60.1	3.4 (0.9)	60.3	1.1 (0.9)
PROs	PROMIS®-Physical Function	66.9	1.9 (0.8)	68.0	0.2 (1.8)	64.4	1.8 (0.9)	66.9	-1.0 (2.0)
	PROMIS®-Fatigue	22.3	-2.0 (0.6)	21.1	-1.7 (1.1)	22.0	-1.9 (0.7)	20.4	-0.3 (1.0)
Biomarkers	Urine Hex4, mmol/mol	4.6	-1.9 (0.3)	6.9	1.2 (0.7)	4.6	-1.7 (0.3)	7.2	1.9 (0.8)
	Serum CK, U/L	447.0	-130.5 (25.1)	527.8	60.2 (26.2)	441.8	-118.0 (28.4)	492.3	79.6 (26.9)

Based on LOCF means

Treatment group favored | Nominal statistical significance ($P<0.05$)

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

Source: Presented at the *16th International Congress on Neuromuscular Diseases (ICNMD)* May 2021.

Amicus Therapeutics

AT-GAA: Highlights

- U.S. FDA accepted for review the BLA and NDA for AT-GAA
- MAA for AT-GAA submitted with the EMA
- Granted positive scientific opinion through the Early Access to Medicines Scheme (EAMS) by the United Kingdom's MHRA
- 150+ patients worldwide now being treated with AT-GAA, including adults, adolescents and infants
- Pediatric study for adolescents up to 17 years with late-onset Pompe disease ongoing
- Clinical study for Pompe patients with infantile-onset disease expected to begin this year
- Expanded access program for infantile- and adult-onset patients open and has enrolled multiple patients with Pompe. Further expanded access for all Pompe patients being considered.

Amicus Therapeutics

Next-Generation Gene Therapy Platform

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

Harnessing the Power of Genetic Medicine

The mission of Caritas is to transform the lives of children and adults living with rare genetic diseases through advanced protein engineering and innovative vector technologies

CARITAS THERAPEUTICS

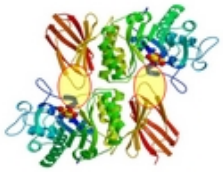
1. Diversified portfolio of clinical and preclinical rare disease development programs with pivotal study start in CLN3, three INDs, and two IND candidates targeted through end of 2023
2. Proprietary platform technologies and protein engineering capabilities enabling innovative, clinically differentiated gene therapies
3. Exclusive relationship with Penn to develop gene therapies for rare genetic disorders
4. Fully designed, ready-to-build, state-of-the-art clinical manufacturing facility with commercial expansion capabilities
5. Attractive risk and cost-sharing partnership with Amicus provides access to an established orphan disease commercial platform
6. Experienced public company leadership coupled with fully built out gene therapy discovery, research and development team

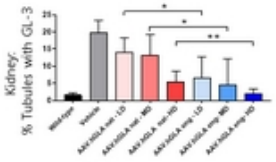
Amicus Therapeutics

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

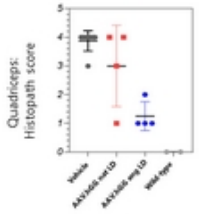
Fabry Gene Therapy





- 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
- IND expected in 2H2022

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 relevant tissues, including reduction in neurons of central nervous system
- IND-enabling work underway

Amicus and Caritas to co-develop the Fabry and Pompe gene therapies

Note: Data from studies in KO GLA and GAA mice

Amicus Therapeutics

Financial Summary

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

3Q21 Select Financial Results

3Q21 revenue of $79.5M and growth rate of 18% primarily from global Galafold sales

(in thousands, except per share data)	Sep. 30, 2021	Sep. 30, 2020
Product Revenue	$79,545	$67,437
Cost of Goods Sold	11,696	8,399
R&D Expense	59,333	70,419
SG&A Expense	46,107	37,850
Changes in Fair Value of Contingent Consideration	3,288	1,034
Depreciation and Amortization	1,520	2,496
Loss from Operations	(42,399)	(52,761)
Income Tax Benefit (Expense)	182	(727)
Net Loss	(50,294)	(64,011)
Net Loss Per Share	(0.19)	(0.25)

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
- Balance sheet further strengthened with a ~$200 million private investment from leading biotechnology investors
- Current cash position is sufficient to achieve self-sustainability and profitability by 2023

Amicus Therapeutics

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

Appendix

Amicus Therapeutics

Reconciliation

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Total operating expenses - as reported GAAP	**$ 110,248**	**$ 111,799**	**$ 331,033**	**$ 350,851**
Research and development:				
Share-based compensation	3,775	8,626	13,232	17,241
Selling, general and administrative:				
Share-based compensation	8,066	7,282	30,699	19,671
Changes in fair value of contingent consideration payable	3,288	1,034	4,780	2,680
Depreciation and amortization	1,520	2,496	4,691	6,299
Total operating expense adjustments to reported GAAP	16,649	19,438	53,402	45,891
Total operating expenses - as adjusted	$ 93,599	$ 92,361	$ 277,631	$ 304,960