



## Amicus Therapeutics Announces Third Quarter 2022 Financial Results and Corporate Updates

November 7, 2022

**Year-to-Date 2022 Reported Revenue Growth of 8% (16% at CER)**

**On-Track to Deliver Full-Year 2022 Double-Digit Revenue Growth of 15-20% at CER**

**Advancing Launch Preparation for AT-GAA in Pompe Disease**

**Updating Full-Year 2022 Non-GAAP Operating Expense Guidance from \$470M-\$485M to \$430M-\$440M**

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

PHILADELPHIA, Nov. 07, 2022 (GLOBE NEWSWIRE) -- [Amicus Therapeutics](#) (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on developing and commercializing novel medicines for rare diseases, today announced financial results for the quarter ended September 30, 2022.

Bradley Campbell, President and Chief Executive Officer of Amicus Therapeutics, Inc., stated, "We remain laser focused on advancing our key strategic priorities for the year. The strong commercial uptake of Galafold® globally is on track to deliver double-digit operational growth in 2022. In addition, we are making great progress towards gaining regulatory approvals of AT-GAA for people living with Pompe disease around the world, and are well underway with our launch readiness. Through our continued commercial success with Galafold, our highly anticipated approvals of AT-GAA in the months ahead, together with our careful expense management, we believe Amicus is well positioned to drive significant value for shareholders and further our mission to bring innovative new medicines to people living with rare diseases."

### Corporate Highlights

- **Global revenue in the third quarter of 2022 was \$81.7 million.** Third quarter revenue represented a year-over-year increase of 3% from total revenue of \$79.5 million in the third quarter of 2021. Third quarter operational revenue growth measured at constant exchange rates (CER)<sup>1</sup> was 14%.

(in thousands)	Three Months Ended September 30,		Year over Year % Growth		Nine Months Ended September 30,		Year over Year % Growth	
	2022	2021	As Reported	at CER <sup>1</sup>	2022	2021	As Reported	at CER <sup>1</sup>
Net Product Revenues	\$81,691	\$79,545	3%	14%	\$241,137	\$223,360	8%	16%

- **Updating full-year 2022 non-GAAP operating expense guidance to \$430 million to \$440 million from \$470 million to \$485 million,** driven by prudent expense management while maintaining AT-GAA manufacturing and pre-launch activities.
- **The Company is on track to achieve non-GAAP profitability<sup>2</sup> in the second half of 2023 as we continue to expand Galafold globally and anticipate the launch of AT-GAA in multiple geographies.** Based on the current operating plan, timing of AT-GAA approvals, and through careful management of expenses, the Company is on track to reach Non-GAAP profitability in the second half of 2023.
- **AT-GAA regulatory reviews progressing and pre-launch activities underway.** In the European Union, the regulatory review is on-track and the Company expects the Committee for Medicinal Products for Human Use (CHMP) opinion in December 2022. In October, the U.S. Food and Drug Administration (FDA) deferred action on the Biologics License Application (BLA) for cipaglucosidase alfa and the New Drug Application (NDA) for miglustat, the two components of AT-GAA due to the FDA's inability to complete the manufacturing facility inspection within the review period. The company is now actively engaged with the FDA on developing a plan and logistics for the pre-approval inspection.
- **Expanded access programs continue to meet the growing demand for AT-GAA across multiple countries.** In the U.K., under the Early Access to Medicines Scheme (EAMS) multiple physicians have requested access for multiple patients across each of the leading Pompe centers in the country. Additional expanded access programs are in place in the U.S., France, Germany, and Japan with multiple Pompe patients participating in each.
- **Amicus entered into an At-the-Market (ATM) equity distribution agreement with Goldman Sachs & Co. LLC (the "Sales Agent") on November 7, 2022, under which Amicus may, at its discretion and from time to time, sell shares of its common stock having an aggregate value of up to \$250 million through the Sales Agent (the "ATM Program").** Amicus expects to use the net proceeds, if any, from the ATM Program for general corporate purposes,

including for the support of the anticipated launch of AT-GAA, continued commercialization of Galafold, manufacturing capabilities, research and development, potential business development opportunities and other capital expenditures.

- **Galafold U.S. intellectual property estate further strengthened following the issuance of 19 new patents this year.** Galafold is protected by orphan drug regulatory exclusivities and a broad U.S. intellectual property portfolio of 46 orange book listed patents, including 5 composition of matter patents, and 30 of which provide protection through at least 2038.
- **Amicus intends to file infringement suits against Abbreviated New Drug Application (ANDA) filers.** Paragraph IV Certification Notice Letters were received from Teva Pharmaceuticals USA, Inc., Aurobindo Pharma Limited, and Lupin Limited in connection with ANDAs filed with the U.S. Food and Drug Administration (FDA) requesting approval to market generic migalastat. Amicus intends to file its lawsuits later today and intends to vigorously enforce its intellectual property rights.

### **Third Quarter 2022 Financial Results**

- Total revenue in the third quarter 2022 was \$81.7 million, a year-over-year increase of 3% from total revenue of \$79.5 million in the third quarter of 2021. On a constant currency basis, third quarter 2022 total revenue growth was 14%. Reported quarterly revenue was offset by a negative currency impact of \$8.6 million, or 11%.
- Cash, cash equivalents, and marketable securities totaled \$354.7 million on September 30, 2022, compared to \$482.5 million at December 31, 2021.
- Total GAAP operating expenses of \$102.1 million for the third quarter 2022 decreased 7% as compared to \$110.2 million for the third quarter 2021.
- Total non-GAAP operating expenses of \$85.5 million for the third quarter of 2022 decreased 9% as compared to \$93.6 million in the third quarter of 2021, reflecting the reprioritization of the gene therapy portfolio.<sup>3</sup>
- Net loss was \$33.3 million, or \$0.12 per share, compared to a net loss of \$50.3 million, or \$0.19 per share, for the third quarter 2021, demonstrating that the Company is progressing on the path towards non-GAAP profitability.

### **2022 Financial Guidance**

- For the full-year 2022, the Company anticipates total Galafold revenue growth between 15 and 20% at CER<sup>1</sup> driven by continued underlying demand from both switch and treatment-naïve patients, geographic expansion, the continued diagnosis of new Fabry patients and commercial execution across all major markets, including the U.S., EU, U.K., and Japan. Applying average October 2022 exchange rates, the negative currency impact on full-year 2022 Galafold reported sales would be approximately 9%.
- Amicus is updating its non-GAAP operating expense guidance for the full-year 2022 from \$470 million to \$485 million to \$430 million to \$440 million, driven by prudent expense management offset by continued investment in the global Galafold launch, AT-GAA clinical studies and pre-launch activities, in addition to certain non-recurring costs for manufacturing to support the global launch of AT-GAA and committed obligations for the gene therapy portfolio. In 2023, Amicus expects non-GAAP operating expense at a level below 2021.<sup>4</sup>

### **Anticipated 2022 Milestones by Program**

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

- Sustain double-digit revenue growth in 2022 of 15-20% at CER<sup>1</sup>
- Continue geographic expansion
- Registry and other Phase 4 studies ongoing

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

- FDA deferred action on the AT-GAA filing until a manufacturing site inspection can be conducted. Per the Agency's guidance, a Type A meeting has been requested to develop plans and logistics for the pre-approval inspection
- EU Committee for Medicinal Products for Human Use (CHMP) opinion expected in December 2022
- Continue to broaden expanded access programs in the U.S., U.K., Germany, France, Japan, and other countries
- Ongoing supportive studies, including pediatric and extension studies

<sup>1</sup> In order to illustrate underlying performance, Amicus discusses its results in terms of constant exchange rate (CER) growth. This represents growth calculated as if the exchange rates had remained unchanged from those used in the comparative period. Full-year 2022 Galafold revenue guidance utilizes the average actual exchange rates for 2021.

<sup>2</sup> Based on projections of Amicus non-GAAP Net Income under current operating plans, which includes successful AT-GAA regulatory approvals and continued Galafold growth. We define non-GAAP Net Income as GAAP Net Income excluding the impact of share-based compensation expense, changes in fair value of contingent

consideration, loss on impairment of assets, depreciation and amortization, acquisition related income (expense), loss on extinguishment of debt, loss on impairment of assets, restructuring charges and income taxes.

<sup>3</sup> 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.

<sup>4</sup> 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.

### **Conference Call and Webcast**

Amicus Therapeutics will host a conference call and audio webcast today, November 7, 2022 at 8:30 a.m. ET to discuss the third quarter 2022 financial results and corporate updates. Participants and investors interested in accessing the call by phone will need to register using the [online registration form](#). After registering, all phone participants will receive a dial-in number along with a personal PIN number to access the event.

A live audio webcast and related presentation materials can also be accessed via the Investors section of the Amicus Therapeutics corporate website at [i.amicusrx.com](http://i.amicusrx.com). Web participants are encouraged to register on the website 15 minutes prior to the start of the call. An archived webcast and accompanying slides will be available on the Company's website shortly after the conclusion of the live event.

### **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 more than 40 countries around the world, including the U.S., EU, U.K., and Japan.

### **U.S. INDICATIONS AND USAGE**

Galafold is indicated for the treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (*GLA*) variant based on *in vitro* assay data.

This indication is approved under accelerated approval based on reduction in kidney interstitial capillary cell globotriaosylceramide (KIC GL-3) substrate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

### **U.S. IMPORTANT SAFETY INFORMATION**

#### **ADVERSE REACTIONS**

The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia.

#### **USE IN SPECIFIC POPULATIONS**

There is insufficient clinical data on Galafold use in pregnant women to inform a drug-associated risk for major birth defects and miscarriage. Advise women of the potential risk to a fetus.

It is not known if Galafold is present in human milk. Therefore, the developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for Galafold and any potential adverse effects on the breastfed child from Galafold or from the underlying maternal condition.

Galafold is not recommended for use in patients with severe renal impairment or end-stage renal disease requiring dialysis.

The safety and effectiveness of Galafold have not been established in pediatric patients.

To report Suspected Adverse Reactions, contact Amicus Therapeutics at 1-877-4AMICUS or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

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

### **EU Important Safety Information**

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

- Galafold is not intended for concomitant use with enzyme replacement therapy.
- Galafold is not recommended for use in patients with Fabry disease who have severe renal impairment (<30 mL/min/1.73 m<sup>2</sup>). The safety and efficacy of Galafold in children 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](http://www.ema.europa.eu).

### **About Amicus Therapeutics**

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare 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 diseases. For more information please visit the company's website at [www.amicusrx.com](http://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, expenses, cash position, and future profitability for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product. In addition to the impact of the COVID-19 pandemic, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies, manufacturing and launch preparations. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's revenue, expenses, cash position, and future profitability, 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, 2021 and Form 10-Q for the quarter ended September 30, 2022, that was 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 September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Net product sales	\$ 81,691	\$ 79,545	\$ 241,137	\$ 223,360
Cost of goods sold	13,436	11,696	29,215	26,615
Gross profit	68,255	67,849	211,922	196,745
Operating expenses:				
Research and development	52,970	59,333	212,806	186,453
Selling, general, and administrative	47,272	46,107	158,767	135,109
Changes in fair value of contingent consideration payable	567	3,288	(506)	4,780
Loss on impairment of assets	—	—	6,616	—
Depreciation and amortization	1,286	1,520	4,031	4,691
Total operating expenses	102,095	110,248	381,714	331,033
Loss from operations	(33,840)	(42,399)	(169,792)	(134,288)
Other income (expense):				
Interest income	563	108	1,052	323
Interest expense	(9,620)	(8,165)	(26,024)	(24,307)
Loss on extinguishment of debt	—	(257)	—	(257)
Other income (expense)	13,634	237	22,804	(2,729)
Loss before income tax	(29,263)	(50,476)	(171,960)	(161,258)
Income tax (expense) benefit	(4,023)	182	(8,743)	(5,925)
<b>Net loss attributable to common stockholders</b>	<b>\$ (33,286)</b>	<b>\$ (50,294)</b>	<b>\$ (180,703)</b>	<b>\$ (167,183)</b>
Net loss attributable to common stockholders per common share — basic and diluted	\$ (0.12)	\$ (0.19)	\$ (0.63)	\$ (0.63)
Weighted-average common shares outstanding — basic and diluted	289,223,709	267,464,637	288,841,092	266,085,788

**TABLE 2**

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

	September 30, 2022	December 31, 2021
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 277,592	\$ 245,197
Investments in marketable securities	77,108	237,299
Accounts receivable	52,303	52,672
Inventories	13,272	26,818
Prepaid expenses and other current assets	38,264	34,848
Total current assets	458,539	596,834
Operating lease right-of-use assets, net	29,871	20,586
Property and equipment, less accumulated depreciation of \$23,337 and \$19,882 at September 30, 2022 and December 31, 2021, respectively	32,449	42,496
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	17,872	24,427
<b>Total Assets</b>	<b>\$ 759,528</b>	<b>\$ 905,140</b>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 12,046	\$ 21,513
Accrued expenses and other current liabilities	125,235	98,153
Contingent consideration payable	19,833	18,900
Operating lease liabilities	7,536	7,409
Total current liabilities	164,650	145,975
Long-term debt	391,319	389,357

Operating lease liabilities	52,012	43,363
Deferred reimbursements	5,906	5,906
Deferred income taxes	4,930	4,930
Other non-current liabilities	8,146	8,240
<b>Total liabilities</b>	<b>626,963</b>	<b>597,771</b>
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$0.01 par value, 500,000,000 shares authorized, 280,887,136 and 278,912,800 shares issued and outstanding at September 30, 2022 and December 31, 2021, respectively	2,813	2,808
Additional paid-in capital	2,645,372	2,595,419
Accumulated other comprehensive (loss) gain:		
Foreign currency translation adjustment	(38,724)	5,251
Unrealized loss on available-for-sale securities	(354)	(270)
Warrants	83	83
Accumulated deficit	(2,476,625)	(2,295,922)
<b>Total stockholders' equity</b>	<b>132,565</b>	<b>307,369</b>
<b>Total Liabilities and Stockholders' Equity</b>	<b>\$ 759,528</b>	<b>\$ 905,140</b>

**TABLE 3**

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

	<b>Three Months Ended September 30,</b>		<b>Nine Months Ended September 30,</b>	
	<b>2022</b>	<b>2021</b>	<b>2022</b>	<b>2021</b>
<b>Total operating expenses - as reported GAAP</b>	<b>\$ 102,095</b>	<b>\$ 110,248</b>	<b>\$ 381,714</b>	<b>\$ 331,033</b>
<b>Research and development:</b>				
Share-based compensation	5,428	3,775	19,172	13,232
<b>Selling, general and administrative:</b>				
Share-based compensation	9,344	8,066	38,714	30,699
<b>Loss on impairment of assets</b>	<b>—</b>	<b>—</b>	<b>6,616</b>	<b>—</b>
<b>Changes in fair value of contingent consideration payable</b>	<b>567</b>	<b>3,288</b>	<b>(506)</b>	<b>4,780</b>
<b>Depreciation and amortization</b>	<b>1,286</b>	<b>1,520</b>	<b>4,031</b>	<b>4,691</b>
<b>Total operating expense adjustments to reported GAAP</b>	<b>16,625</b>	<b>16,649</b>	<b>68,027</b>	<b>53,402</b>
<b>Total operating expenses - as adjusted</b>	<b>\$ 85,470</b>	<b>\$ 93,599</b>	<b>\$ 313,687</b>	<b>\$ 277,631</b>