

**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): **August 8, 2020**

**AMICUS THERAPEUTICS, INC.**

(Exact Name of Registrant as Specified in Its Charter)

**Delaware**  
(State or Other Jurisdiction of  
Incorporation)

Delaware  
**(State or Other Jurisdiction  
of Incorporation)**

001-33497  
**(Commission  
File Number)**

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

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

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

(Former Name or Former Address, if Changed Since Last Report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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

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

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (17 CFR §230.405) or Rule 12b-2 of the Securities Exchange Act of 1934 (17 CFR §240.12b-2). Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

**Item 2.02. Results of Operations and Financial Condition**

On August 10, 2020, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the fiscal quarter ended June 30, 2020. A copy of this press release is attached hereto as Exhibit 99.1. The Company will host a conference call and webcast on August 10, 2020 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 5.02. Departure of Directors or Certain Officers; Election of Directors; Appointment of Certain Officers; Compensatory Arrangements of Certain Officer.**

On August 8, 2020, Dr. Ted W. Love informed the Company that, after 8 years of service as a member of the Board of Directors (the "Board"), he will be retiring from the Board, effective immediately.

**Item 9.01 Financial Statements and Exhibits****(d) Exhibits:**

<b>Exhibit No.</b>	<b>Description</b>
<a href="#">99.1</a>	<a href="#">Press Release dated August 10, 2020</a>
<a href="#">99.2</a>	<a href="#">August 10, 2020 Conference Call Presentation Materials</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

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**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: August 10, 2020

By: /s/ Ellen S. Rosenberg

Name: Ellen S. Rosenberg

Title: Chief Legal Officer and Corporate Secretary

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**Amicus Therapeutics Announces Second Quarter 2020 Financial Results and Corporate Updates**

***Galafold 2Q2020 Revenue of \$62.4 Million, On-Track to Achieve 2020 Revenue Guidance of \$250M-\$260M***

***AT-GAA Phase 3 PROPEL Study Readout and Rolling BLA Submission to U.S. FDA on Schedule***

***Advancing Industry-Leading Rare Disease Gene Therapy Portfolio***

***Path to Profitability Achieved without the Need for Any Future Dilutive Financings***

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

**CRANBURY, NJ, Aug. 10, 2020** – Amicus Therapeutics (Nasdaq: FOLD), a global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel medicines for rare diseases, today announced financial results for the second quarter ended June 30, 2020. The Company also summarized recent program updates and reiterated its full-year 2020 guidance.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “During the second quarter, we made tremendous progress advancing our mission for patients and are on track to achieve our 2020 key strategic priorities, including our global Fabry launch, Pompe late-stage development program, and advancing our industry-leading gene therapy pipeline. Following our strategic financing in July, Amicus is firmly on a path to profitability without the need to access the equity markets. Through these efforts, we remain strongly positioned to achieve our vision of delivering groundbreaking new medicines and hopefully, one day, cures for people living with rare diseases.”

**Corporate Highlights**

- **Global revenue for Galafold® (migalastat) in the second quarter of 2020 was \$62.4M.** Second quarter revenue represented a year-over-year increase of 41% from total revenue of \$44.1 million in the second quarter of 2019. On a constant currency basis, second quarter 2020 total revenue was \$63.3 million, representing operational revenue growth measured at constant currency exchange rates of 43%, which was offset by a negative currency impact of \$1.0 million, or 2%.
- **Strong second quarter revenue represents the continued performance across the global business, including new patient starts from switch and naïve patients throughout the quarter in all major regions, including that hardest hit by COVID-19.** Performance driven largely by strong patient demand. Global compliance and adherence rates continue to exceed 90%.
- **1,000+ mutations added to the EU Galafold label.** This update includes a number of new mutations identified by creating and testing all possible single-base pair point mutations in the GLA gene. Including this list will help physicians assess treatment options in an expeditious manner. Amenable mutations continue to represent up to half of all people living with Fabry disease.
- **Global Phase 3 PROPEL clinical study of AT-GAA in late-onset Pompe disease (LOPD) remains on track for top line data in 1H21.** To date, 97%+ of the 2,810 planned infusions and assessments for the ongoing PROPEL study have been completed on schedule. The Company plans to initiate a rolling BLA for AT-GAA in the second half of 2020, completing final submission in the first half of 2021.
- **Expanded Access Program for infantile-onset Pompe patients underway.** Amicus has initiated an expanded access program for its investigational medicine AT-GAA for young children living with infantile-onset Pompe disease (IOPD).
- **Additional Phase 1/2 CLN6 data to be presented this year at the Child Neurology Society Annual Meeting in October.** Regulatory interactions are ongoing and the Company expects to provide feedback on the path forward in early 2021.



- **Initial data from the Phase 1/2 CLN3 study expected in early 2021 based on changes in medical conference schedules.** Regulatory interactions are ongoing and concurrent with the data, the Company expects to provide feedback on the regulatory pathway.
- **Positive preclinical Pompe gene therapy data presented at American Society of Cell and Gene Therapy meeting in May.** Positive results showed the Amicus engineered hGAA having better targeting and clearance of glycogen storage in Pompe mice. Preliminary data in non-human primates suggested therapeutically relevant expression levels in target organs.
- **Cash position sufficient to achieve self-sustainability without the need for any future dilutive financings.** The previously announced debt facility places Amicus firmly on a path to profitability, while the Company continues to carefully manage expenses and investments, while executing on the Galafold launch and advancing development programs.

#### **2020 Key Strategic Priorities**

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

#### **Second Quarter 2020 Financial Results**

- Total revenue in the second quarter 2020 was \$62.4 million, a year-over-year increase of 41% from total revenue of \$44.1 million in the second quarter of 2019. On a constant currency basis, second quarter 2020 total revenue was \$63.3 million, representing operational revenue growth measured at constant currency exchange rates of 43%, which was offset by a negative currency impact of \$1.0 million, or 2%.
- Cash, cash equivalents, and marketable securities totaled \$309.6 million at June 30, 2020, compared to \$452.7 million at December 31, 2019.
- Total GAAP operating expenses of \$107.0 million for the second quarter 2020 decreased as compared to \$115.2 million for the second quarter 2019, reflecting decreased travel and third-party costs, offset by continued investments in our gene therapy pipeline.
- Total non-GAAP operating expenses of \$95.9 million for the second quarter of 2020 decreased as compared to \$103.6 million in the second quarter of 2019, reflecting decreased travel and third-party costs, offset by continued investments in our gene therapy pipeline.<sup>1</sup>
- Net loss was \$52.5 million, or \$0.20 per share, compared to a net loss of \$84.6 million, or \$0.36 per share, for the second quarter 2019.

<sup>1</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.

#### **2020 Financial Guidance**

- For the full-year 2020, the Company anticipates total Galafold revenue of \$250 million to \$260 million based on the average exchange rates for 2019.
- Non-GAAP operating expense guidance for the full-year 2020 is \$410 million to \$420 million, driven by continued investment in the global Galafold launch, AT-GAA clinical studies, and advancing our gene therapy pipeline.<sup>2</sup>
- Cash, cash equivalents, and marketable securities totaled \$309.6 million at June 30, 2020. Based on current operating models, the Company believes that the current cash position, along with the net proceeds from the 2020 Senior Secured Term Loan and expected revenues, is sufficient to fund the Company's operations and ongoing research programs through to profitability.

<sup>2</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.



## **Anticipated 2020 Milestones by Program**

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

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

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

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

### **Gene Therapy Portfolio**

- Report further safety and efficacy data in the CLN6 Batten disease Phase 1/2 study and advance regulatory discussions to finalize clinical and regulatory path
- Initial data from the CLN3 Batten disease Phase 1/2 study expected in early 2021 and advance regulatory discussions to finalize clinical and regulatory path
- Continue IND-enabling toxicology work in Pompe disease and progress towards IND
- Additional preclinical data expected across multiple programs with disclosure of up to two additional IND candidates
- Manufacturing advancements across portfolio

## **Conference Call and Webcast**

Amicus Therapeutics will host a conference call and audio webcast today, August 10, 2020 at 8:30 a.m. ET to discuss the second quarter 2020 financial results and corporate updates. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international), conference ID: 4949075.

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](http://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 August 10, 2020. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 4949075.

## **About Galafold**

Galafold<sup>®</sup> (migalastat) 123 mg capsules is an oral pharmacological chaperone of alpha-Galactosidase A (alpha-Gal A) for the treatment of Fabry disease in adults who have amenable *GLA* variants. In these patients, Galafold works by stabilizing the body's own dysfunctional enzyme so that it can clear the accumulation of disease substrate. Globally, Amicus Therapeutics estimates that approximately 35 to 50 percent of Fabry patients may have amenable *GLA* variants, though amenability rates within this range vary by geography. Galafold is approved in over 40 countries around the world, including the U.S., EU, U.K., Japan and others.

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

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

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

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

### **ADVERSE REACTIONS**

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



## USE IN SPECIFIC POPULATIONS

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

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

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

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

To report Suspected Adverse Reactions, contact Amicus Therapeutics at 1-877-4AMICUS or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](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 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](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 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](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 and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product. In addition to the impact of the COVID-19 pandemic, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. Statements regarding corporate financial guidance and financial goals and the attainment of such goals. With respect to statements regarding projections of the Company's revenue and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2019, the Quarterly Report filed on Form 10-Q for the quarter ended March 31, 2020, and the Quarterly Report filed on Form 10-Q to be filed today. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

## CONTACTS:

### Investors:

Amicus Therapeutics  
Andrew Faughnan  
Director, Investor Relations  
[afaughnan@amicusrx.com](mailto:afaughnan@amicusrx.com)  
(609) 662-3809

### Media:

Amicus Therapeutics  
Diana Moore  
Head of Global Corporate Communications  
[dmoore@amicusrx.com](mailto:dmoore@amicusrx.com)  
(609) 662-5079

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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 June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Net product sales	\$ 62,353	\$ 44,130	\$ 122,878	\$ 78,176
Cost of goods sold	6,676	5,367	13,228	9,422
Gross profit	55,677	38,763	109,650	68,754
Operating expenses:				
Research and development	69,611	70,981	158,731	135,574
Selling, general, and administrative	34,657	42,578	74,872	86,881
Changes in fair value of contingent consideration payable	715	480	1,646	1,863
Depreciation and amortization	2,039	1,154	3,803	2,145
Total operating expenses	107,022	115,193	239,052	226,463
Loss from operations	(51,345)	(76,430)	(129,402)	(157,709)
Other income (expense):				
Interest income	865	2,599	2,380	5,238
Interest expense	(3,635)	(4,625)	(7,364)	(11,079)
Loss on exchange of convertible notes	—	(4,501)	—	(40,624)
Other income (expense)	5,326	(877)	(2,990)	209
Loss before income tax	(48,789)	(83,834)	(137,376)	(203,965)
Income tax expense	(3,703)	(717)	(4,064)	(885)
<b>Net loss attributable to common stockholders</b>	<b>\$ (52,492)</b>	<b>\$ (84,551)</b>	<b>\$ (141,440)</b>	<b>\$ (204,850)</b>
Net loss attributable to common stockholders per common share — basic and diluted	\$ (0.20)	\$ (0.36)	\$ (0.55)	\$ (0.91)
Weighted-average common shares outstanding — basic and diluted	257,973,329	238,089,824	257,548,623	225,848,013



TABLE 2

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

	June 30, 2020	December 31, 2019
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 164,573	\$ 142,837
Investments in marketable securities	145,017	309,903
Accounts receivable	43,040	33,284
Inventories	12,979	14,041
Prepaid expenses and other current assets	18,275	20,008
<b>Total current assets</b>	<b>383,884</b>	<b>520,073</b>
Operating lease right-of-use assets, less accumulated amortization of \$6,219 and \$5,342 at June 30, 2020 and December 31, 2019, respectively	23,949	33,315
Property and equipment, less accumulated depreciation of \$21,194 and \$17,604 at June 30, 2020 and December 31, 2019, respectively	46,945	47,705
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	25,876	28,317
<b>Total Assets</b>	<b>\$ 701,451</b>	<b>\$ 850,207</b>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 14,306	\$ 21,722
Accrued expenses and other current liabilities	85,478	99,901
Operating lease liabilities	8,516	7,189
<b>Total current liabilities</b>	<b>108,300</b>	<b>128,812</b>
Deferred reimbursements	8,906	8,906
Convertible notes	2,203	2,131
Senior secured term loan	147,834	147,374
Contingent consideration payable	20,027	22,681
Deferred income taxes	5,051	5,051
Operating lease liabilities	43,666	53,531
Other non-current liabilities	4,511	5,296
<b>Total liabilities</b>	<b>340,498</b>	<b>373,782</b>
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$0.01 par value, 500,000,000 shares authorized, 258,223,842 and 255,417,869 shares issued and outstanding at June 30, 2020 and December 31, 2019, respectively	2,614	2,598
Additional paid-in capital	2,250,849	2,227,225
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	4,865	2,785
Unrealized gain on available-for-sale securities	288	40
Warrants	12,387	12,387
Accumulated deficit	(1,910,050)	(1,768,610)
<b>Total stockholders' equity</b>	<b>360,953</b>	<b>476,425</b>
<b>Total Liabilities and Stockholders' Equity</b>	<b>\$ 701,451</b>	<b>\$ 850,207</b>



TABLE 3

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

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2020</u>	<u>2019</u>	<u>2020</u>	<u>2019</u>
<b>Total operating expenses - as reported GAAP</b>	<b>\$ 107,022</b>	<b>\$ 115,193</b>	<b>\$ 239,052</b>	<b>\$ 226,463</b>
<b>Research and development:</b>				
Share-based compensation	3,362	3,952	8,615	8,984
<b>Selling, general and administrative:</b>				
Share-based compensation	5,046	5,983	12,389	13,695
<b>Changes in fair value of contingent consideration payable</b>	<b>715</b>	<b>480</b>	<b>1,646</b>	<b>1,863</b>
<b>Depreciation and amortization</b>	<b>2,039</b>	<b>1,154</b>	<b>3,803</b>	<b>2,145</b>
<b>Total operating expense adjustments to reported GAAP</b>	<b>11,162</b>	<b>11,569</b>	<b>26,453</b>	<b>26,687</b>
<b>Total operating expenses - as adjusted</b>	<b>\$ 95,860</b>	<b>\$ 103,624</b>	<b>\$ 212,599</b>	<b>\$ 199,776</b>



# 2Q20 Financial Results Conference Call & Webcast

August 10, 2020



# Forward-Looking Statements

*This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product. In addition to the impact of the COVID-19 pandemic, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. Statements regarding corporate financial guidance and financial goals and the attainment of such goals. With respect to statements regarding projections of the Company's revenue and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2019, , the Quarterly Report filed on Form 10-Q for the quarter ended March 31, 2020, and the Quarterly Report filed on Form 10-Q to be filed today. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.*

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

# A RARE COMPANY

A leading fully integrated, global rare disease biotechnology company

 **Galafold<sup>®</sup>**  
(migalastat)

First Oral Precision  
Medicine for Fabry Disease



**Gene Therapy**  
PLATFORM  
Protein Engineering  
& Glycobiology



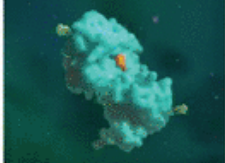
World Class  
**BIOLOGICS**  
Capabilities



**EMPLOYEES**  
in 27 Countries



**AT-GAA**  
Phase 3 in  
Pompe Disease



**\$530M+**  
Cash  
as of 7/31/20

**Two Clinical-  
Stage Gene  
Therapies**

**GLOBAL  
COMMERCIAL  
ORGANIZATION**

**Robust R&D  
Engine**

Nearly 50+ Lysosomal  
Disorders and More  
Prevalent Rare Diseases



## 2020 Key Strategic Priorities

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



# Galafold<sup>®</sup> (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 June 30, 2020)

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 enzyme replacement therapy

### One of the Most Successful Rare Disease Launches



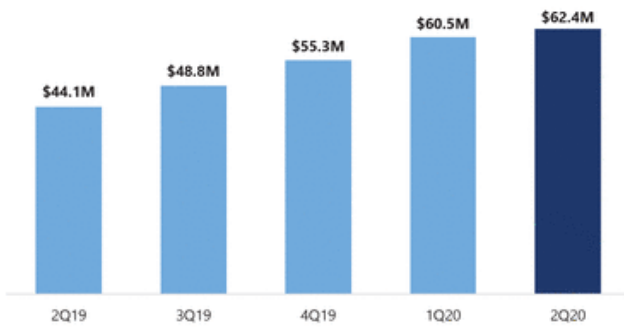
Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (50%) were headache, muscle aches, sinus tract infection, nausea and diarrhea. For additional information about Galafold, including the full U.S. Prescribing Information, please visit <http://www.amicustherapeutics.com/galafold>. For further important safety information for Galafold, including dosing and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European leaflet for Galafold available from the UK website at <http://www.amicustherapeutics.com>.



# Galafold Quarterly Performance

Growth remains steady with Q2 revenue of \$62.4M, or \$63.3M on a constant currency basis

Quarterly Galafold Sales

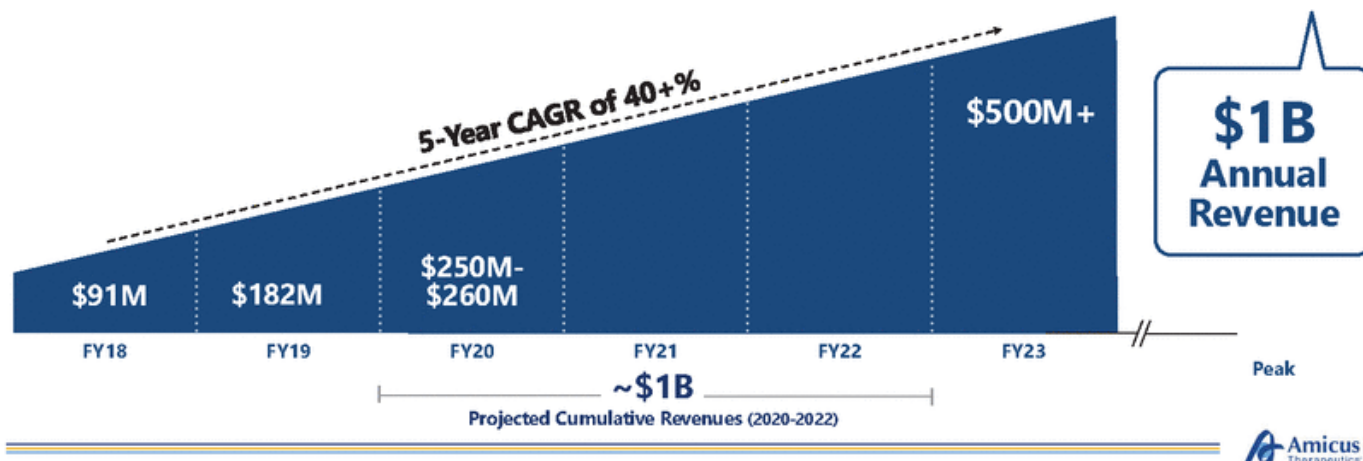


Year-over-Year Galafold Sales Growth



# Galafold Growth Trajectory

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





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

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

## AT-GAA: Foundation in Protein Engineering

Amicus scientists specializing in protein engineering and glycobiology created a uniquely glycosylated and highly phosphorylated ERT (AT-GAA) that significantly enhances targeting to key muscles affected in patients

**ATB200**  
Investigational human recombinant GAA enzyme  
• IV infusion  
Designed for enhanced targeting to muscle cells

**AT2221**  
Investigational enzyme stabilizer  
Orally administered

**AT-GAA**

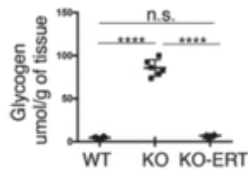
The image shows a large, light blue 3D molecular model of the AT-GAA enzyme. Two smaller orange and yellow structures are shown: one is ATB200, which is attached to the main enzyme structure, and the other is AT2221, which is shown separately. A circular inset on the right shows a smaller view of the enzyme structure.

## Preclinical Findings: Differentiated Impact on Pathogenic Cascade

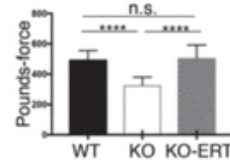
Meena et. al. 2020 (Independent 5-month AT-GAA study in GAA knockout mice)

**AT-GAA completely reversed glycogen accumulation and significantly improved a cascade of secondary abnormalities including autophagy, cell signaling and muscle proteostasis**

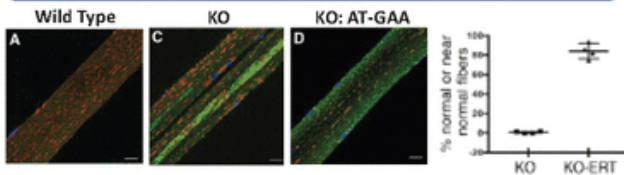
### Reversal of Glycogen Accumulation



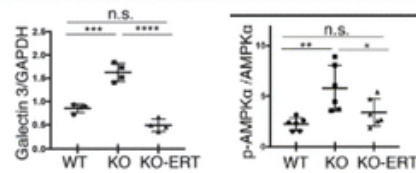
### Improved Grip Strength



### Reduced Autophagy



### Improved Markers of Lysosomal Damage and Cell Signaling



## AT-GAA: Key Takeaways



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

- PROPEL study timelines are on track with data expected 1H2021
  - To date, **97%+** of the 2,810 planned infusions and assessments for the ongoing PROPEL study have been completed on schedule
- Breakthrough Therapy Designation and the Promising Innovative Medicine designation highlight unmet need in Pompe disease today
- U.S. FDA grants rolling BLA submission and company on track to initiate in 2H2020
- Expanded Access Program for infantile-onset Pompe patients underway
- Process performance qualification (PPQ) runs with our partners at WuXi have been successfully completed for the drug substance
- Peak revenue potential of \$1B-\$2B, with exclusivity well into 2030s



# Next Generation Gene Therapy Platform

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



# A RARE PORTFOLIO

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3	REGULATORY	COMMERCIAL
<b>Fabry Franchise</b>						
Galafold® (migalastat) Monotherapy <b>ODD</b>						
Fabry Gene Therapy	PENN					
<b>Pompe Franchise</b>						
AT-GAA (Novel ERT + Chaperone) <b>ODD</b>						
Pompe Gene Therapy	PENN					
<b>Batten Franchise – Gene Therapies</b>						
CLN6 Batten Disease <b>ODD</b> <b>RPD</b>	NCH					
CLN3 Batten Disease <b>ODD</b> <b>RPD</b>	NCH					
CLN1 Batten Disease	NCH					
<b>Next Generation Research Programs and CNS Gene Therapies</b>						
CDKL5 Deficiency Disorder GTX / ERT	PENN					
Others	NCH / PENN					
<b>MPS Franchise</b>						
Mepsevii™ (vestronidase alfa) <i>(Japan Only)*</i>						
Next Generation MPSIIIA	PENN					
MPSIIIB	PENN					

**LEGEND**

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

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

## Gene Therapy: Updates & Key Takeaways

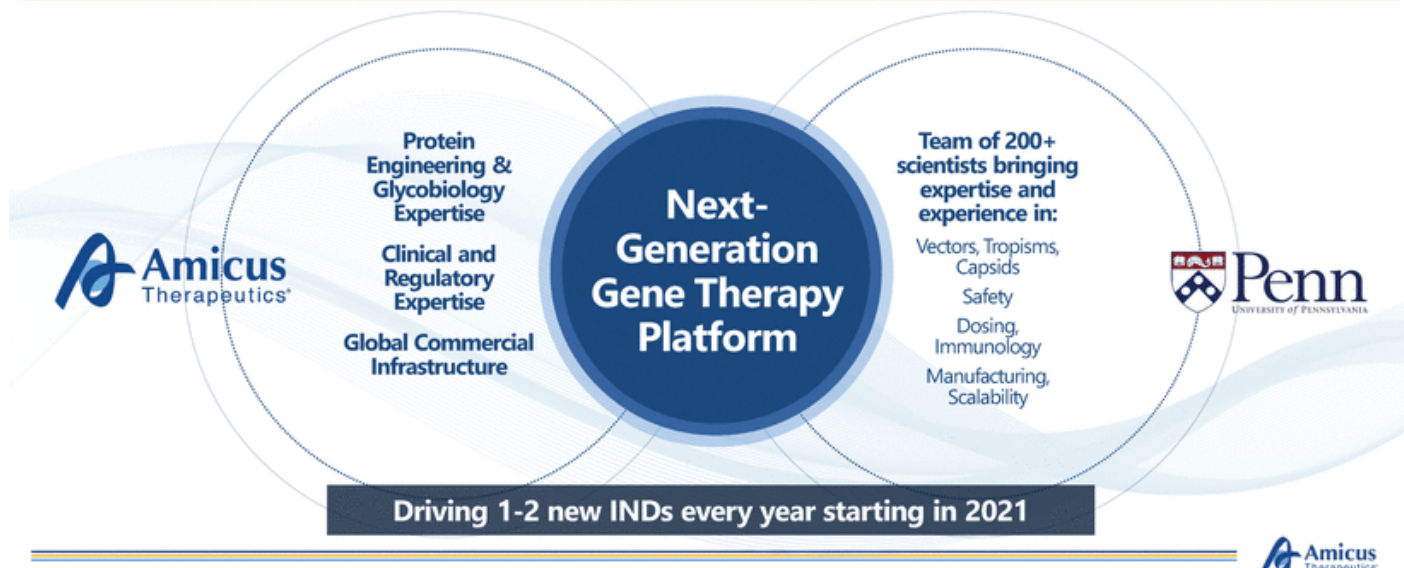


Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future

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

## Combines Amicus and Penn Expertise Across Lysosomal and Rare Diseases

An R&D platform with rights to 50+ diseases





# Financial Summary

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

## 2Q2020 Select Financial Results

**2Q2020 revenue of \$62.4M primarily from global Galafold sales**

<i>(in thousands, except per share data)</i>	<b>Jun. 30, 2020</b>	<b>Jun. 30, 2019</b>
<b>Product Revenue</b>	62,353	44,130
<b>Cost of Goods Sold</b>	6,676	5,367
<b>R&amp;D Expense</b>	69,611	70,981
<b>SG&amp;A Expense</b>	34,657	42,578
<b>Changes in Fair Value of Contingent Consideration</b>	715	480
<b>Depreciation and Amortization</b>	2,039	1,154
<b>Loss from Operations</b>	(51,345)	(76,430)
<b>Income Tax Expense</b>	(3,703)	(717)
<b>Net Loss</b>	(52,492)	(84,551)
<b>Net Loss Per Share</b>	(0.20)	(0.36)

## Financial Outlook: Key Takeaways



- Proceeds from recent debt facility places Amicus firmly on a path to profitability
  - Achieved through continued careful expense management, prioritization of very early stage research programs and more measured capital expenditures
- Current cash position of \$530M+ as of July 31<sup>st</sup>
- Company fully funded through major milestones in portfolio and continued global growth
- Cumulative Galafold projected revenue of \$1B+ in 2020-2022 offsets significant majority of company spend/investments
- Reaffirming full year Galafold revenue guidance of \$250 million to \$260 million and non-GAAP operating expense guidance of \$410 million to \$420 million



# Closing Remarks

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

Thank You

*"Our passion for making a difference unites us"*

*-Amicus Belief Statement*





# Appendix



# Reconciliation

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
<b>Total operating expenses - as reported GAAP</b>	<b>\$ 107,022</b>	<b>\$ 115,193</b>	<b>\$ 239,052</b>	<b>\$ 226,463</b>
<b>Research and development:</b>				
Share-based compensation	3,362	3,952	8,615	8,984
<b>Selling, general and administrative:</b>				
Share-based compensation	5,046	5,983	12,389	13,695
Changes in fair value of contingent consideration payable	715	480	1,646	1,863
Depreciation and amortization	2,039	1,154	3,803	2,145
<b>Total operating expense adjustments to reported GAAP</b>	<b>11,162</b>	<b>11,569</b>	<b>26,453</b>	<b>26,687</b>
<b>Total operating expenses - as adjusted</b>	<b>\$ 95,860</b>	<b>\$ 103,624</b>	<b>\$ 212,599</b>	<b>\$ 199,776</b>