

**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, 2019**



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:

<u>Title of each class</u>	<u>Trading Symbol(s)</u>	<u>Name of each exchange on which registered</u>
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 8, 2019, Amicus Therapeutics, Inc. (the “Company”) issued a press release announcing its financial results for the fiscal quarter ended June 30, 2019. A copy of this press release is attached hereto as Exhibit 99.1. The Company will host a conference call and webcast on August 8, 2019 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:

<u>Exhibit No.</u>	<u>Description</u>
99.1	Press Release dated August 8, 2019
99.2	August 8, 2019 Conference Call Presentation Materials

Signatures

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 8, 2019

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



Amicus Therapeutics Announces Second Quarter 2019 Financial Results and Corporate Updates

2Q19 Galafold® (migalastat) Revenue of \$44.1M Represents Highest Quarterly Growth Since Launch Driven by Continued Strong Global Uptake

Reiterating FY19 Revenue Guidance of \$160M-\$180M

Full Enrollment in Pompe Pivotal Study of AT-GAA on Track by YE19

Significant Progress Across Industry Leading Rare Disease Gene Therapy Portfolio

Strong Balance Sheet with \$575M+ Cash

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

CRANBURY, NJ, August 8, 2019 — Amicus Therapeutics (Nasdaq: FOLD), a global biotechnology company focused on discovering, developing and delivering novel medicines for rare metabolic diseases, today announced financial results for the second quarter ended June 30, 2019. The Company also summarized recent program updates and reiterated its full-year 2019 guidance.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “2019 has been a great period of growth and achievement at Amicus as we continue to build a leading global rare disease biotechnology company. We are on track to meet or exceed all of our key strategic priorities for the year - continued success for Galafold, tremendous momentum in our Phase 3 Pompe clinical study, positive interim Phase 1/2 clinical data for our first gene therapy for CLN6 Batten disease, and an industry leading rare disease gene therapy portfolio. We are in a very strong position to deliver on our mission for patients and shareholders.”

Corporate Highlights for 2Q19 and Early 3Q19

- **Global revenue for Fabry precision medicine Galafold in the second quarter of 2019 was \$44.1 million and continues to track toward full-year 2019 revenue guidance of \$160 million to \$180 million. Now expecting to exceed 1,000 patients on therapy well before year-end.** Second quarter revenue represented a year-over-year increase of 107% from total revenue of \$21.3 million in the second quarter of 2018, and a quarter over-quarter increase of 30% from total revenue of \$34.0 million in the first quarter of 2019. As of June 30, 2019, Galafold represented an estimated 24% of global market share of treated amenable patients. Global compliance and adherence rates continue to exceed 90%.
- **Enrollment momentum in ongoing pivotal PROPEL study in Pompe disease at participating sites in 29 countries.** This global study at the leading Pompe centers in the world is on track to achieve full enrollment of ~100 patients by year-end 2019. The study is now more than a majority enrolled. The Pompe patient and physician community around the world continues to show strong interest in AT-GAA, which has U.S. Breakthrough Therapy Designation, as the next potential standard of care.
- **Positive interim Phase 1/2 clinical data for gene therapy in CLN6 Batten disease.** The results for our AAV-CLN6 gene therapy showed a positive impact on motor and language function in seven of eight patients with available efficacy data (treated from 16 to 25 months), compared to a natural history dataset, as well as in comparisons within sibling pairs.
- **Strategic partnerships with Catalent Biologics and Thermo Fisher for clinical and commercial gene therapy manufacturing.** Brammer Bio, a part of Thermo Fisher, is assisting with clinical and commercial-scale capabilities for Amicus gene therapy programs in CLN6 and additional forms of Batten disease. Paragon Gene Therapy, a unit of Catalent Biologics, will support clinical manufacturing capabilities and capacity for multiple active preclinical lysosomal disorder programs in collaboration with Penn.
- **Major expansion of gene therapy collaboration with the Perelman School of Medicine at the University of Pennsylvania (Penn).** Amicus now has exclusive worldwide rights to Penn’s next generation gene therapy

technologies for a majority of lysosomal disorders and 12 additional rare diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy and select other muscular dystrophies.

- **Positive initial proof-of-concept for preclinical Pompe gene therapy data at American Society of Cell and Gene Therapy meeting.** Positive preclinical results showed robust uptake and glycogen reduction in multiple tissues, including brain and spinal cord, and were a key driver for the expanded Amicus-Penn collaboration. Additional Pompe preclinical studies and selection of a clinical candidate are on track in 2019.
- **Exclusive license for Japanese rights to Ultragenyx's (Nasdaq: RARE) Mepsevii™ (vestronidase alfa), an enzyme replacement therapy (ERT) for the lysosomal disorder Mucopolysaccharidosis type VII (MPS VII, Sly syndrome).** Amicus will leverage its existing infrastructure, relationships, and experience in clinical development, regulatory approvals, and commercialization in Japan. Mepsevii™ is currently approved for the treatment of children and adults with MPS VII in the U.S., EU, and Brazil. Amicus plans to use the existing Mepsevii data package, as well as data from an ongoing investigator-sponsored trial in Japan, to support a J-NDA submission.
- **Strong financial position to continue executing the Galafold launch, advance development programs and build leadership position in gene therapy science, development and manufacturing capabilities and capacity.** The current cash position of \$575.7 million at June 30, 2019, which includes \$189.0 million in net proceeds raised from our June 2019 public offering of common stock, is expected to fund ongoing operations into 2021.

2Q19 Financial Results

- Total revenue in the second quarter 2019 was \$44.1 million, a year-over-year increase of 107% from total revenue of \$21.3 million in the second quarter of 2018, and a quarter over-quarter increase of 30% from total revenue of \$34.0 million in the first quarter of 2019. This includes year-over-year operational revenue growth measured at constant currency exchange rates of 115%, offset by a negative currency impact of \$1.7M, or 8%.
- Cash, cash equivalents, and marketable securities totaled \$575.7 million at June 30, 2019, compared to \$504.2 million at December 31, 2018.
- Total operating expenses of \$115.2 million for the second quarter of 2019 increased as compared to \$65.1 million in the second quarter of 2018, reflecting continued investments in the Galafold launch, Pompe clinical study program, and our gene therapy pipeline.
- Net loss was \$84.6 million, or \$0.36 per share, compared to a net loss of \$61.8 million, or \$0.33 per share, for the second quarter 2018.

2019 Key Strategic Priorities

- Nearly double annual worldwide revenue for Galafold (FY19 guidance of \$160 million to \$180 million) with over 1,000 Fabry patients on Galafold by year end.
- Complete enrollment in pivotal Phase 3 PROPEL clinical study in Pompe disease and report additional Phase 2 data.
- Report additional two-year results from Phase 1/2 clinical study in CLN6 Batten disease and complete enrollment in ongoing CLN3 Batten disease Phase 1/2 study.
- Establish preclinical proof of concept for Fabry and Pompe gene therapies.
- Maintain a strong financial position.

2019 Financial Guidance

For the full-year 2019 the Company anticipates total Galafold revenue of \$160 million to \$180 million. Growth in 2019 is expected to be driven by continued growth in EU markets, further geographic expansion, and successful adoption in the first full year of launch in the U.S. and Japan. For the remainder of the year, consistent with Galafold adoption trends and ordering patterns seen in previous years, Amicus expects flat to moderate revenue growth in the third quarter and higher revenue growth in the fourth quarter of 2019.

Cash, cash equivalents, and marketable securities totaled \$575.7 million at June 30, 2019. The Company expects to end 2019 with approximately \$400 million in cash on hand. The current cash position is anticipated to fund ongoing operations into 2021.

Anticipated 2019 Milestones by Program

Galafold Oral Precision Medicine for Fabry Disease

- Continued growth in global market share of treated amenable patients.
- Geographic expansion following recent approval in Argentina
- On track to meet full-year 2019 revenue guidance range of \$160 million to \$180 million.
- Target of 1,000+ patients on Galafold before year-end.

AT-GAA for Pompe Disease

- Initial 6-month data in additional ERT-switch patients (Cohort 4) and full 24-month data from the first three cohorts in Phase 1/2 ATB200-02 clinical study at World Muscle Society, October 1-5, 2019 (Copenhagen, Denmark)
- Retrospective natural history study data in approximately 100 ERT-treated Pompe patients.
- Additional supportive studies, including an open-label study in pediatric patients.
- Full enrollment in Phase 3 PROPEL clinical study.
- Advance agreed upon CMC requirements to support BLA.

Gene Therapy Pipeline:

- Detailed data presentation of interim clinical results in CLN6 Batten disease at Amicus Analyst Day on October 10, 2019 (New York, NY) and in a poster at Child Neurology Society Annual Meeting, October 23-26, 2019 (Charlotte, NC).
- Advancement of clinical, regulatory and manufacturing activities for CLN6 Batten disease
- High dose cohort on track to commence in Phase 1/2 clinical study of CLN3 Batten disease with full enrollment by year end.
- Additional preclinical data including next-generation gene therapies for Fabry and Pompe.
- Continued advancement of preclinical gene therapy programs in CLN8 and CLN1 Batten disease.
- Selection of Pompe AAV gene therapy clinical candidate to move into IND-enabling studies.
- Selection of Amicus site for late process development and manufacturing facilities.

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, August 8, 2019 at 8:30 a.m. ET to discuss the second quarter 2019 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: 8871329.

A live audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate website at <http://ir.amicusrx.com/>, and will be archived for 30 days. 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 8, 2019. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 8871329.

About Galafold

Galafold® (migalastat) 123 mg capsules is an oral pharmacological chaperone of alpha-Galactosidase A (alpha-Gal A) for the treatment of Fabry disease in adults who have amenable *GLA* variants. In these patients, Galafold works by stabilizing the body's own dysfunctional enzyme so that it can clear the accumulation of disease substrate. Globally, Amicus Therapeutics estimates that approximately 35 to 50 percent of Fabry patients may have amenable *GLA* variants, though amenability rates within this range vary by geography. Galafold is approved in Argentina, Australia, Canada, European Union, Israel, Japan, South Korea, Switzerland and the U.S.

U. S. INDICATIONS AND USAGE

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

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

U.S. IMPORTANT SAFETY INFORMATION

ADVERSE REACTIONS

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

USE IN SPECIFIC POPULATIONS

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

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

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

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

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

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

EU Important Safety Information

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

- Galafold is not intended for concomitant use with enzyme replacement therapy.
- Galafold is not recommended for use in patients with Fabry disease who have severe renal impairment (< 30 mL/min/1.73 m²). The safety and efficacy of Galafold in children 0—15 years of age have not yet been established.
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.
- While taking Galafold, effective birth control should be used. It is not known whether Galafold is excreted in human milk.
- Contraindications to Galafold include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on Galafold or switched to Galafold.
- OVERDOSE: General medical care is recommended in the case of Galafold overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received Galafold. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

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

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-dedicated biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases. For more information please visit the company's website at www.amicusrx.com, and follow on Twitter and LinkedIn.

Non-GAAP Financial Measures

Operational revenue growth excluding the impact of foreign currency exchange rates is a non-GAAP financial measure and should not be considered a replacement for, and should be read together with, the most comparable GAAP financial measure.

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, 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. 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, 2018. 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/Media:

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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,	
	2019	2018	2019	2018
Revenue:				
Net product sales	\$ 44,130	\$ 21,309	\$ 78,176	\$ 38,005
Cost of goods sold	5,367	3,135	9,422	5,750
Gross profit	38,763	18,174	68,754	32,255
Operating expenses:				
Research and development	70,981	34,660	135,574	75,458
Selling, general, and administrative	42,578	29,172	86,881	56,568
Changes in fair value of contingent consideration payable	480	300	1,863	1,400
Depreciation and amortization	1,154	973	2,145	1,942
Total operating expenses	115,193	65,105	226,463	135,368
Loss from operations	(76,430)	(46,931)	(157,709)	(103,113)
Other income (expense):				
Interest income	2,599	2,913	5,238	4,650
Interest expense	(4,625)	(4,560)	(11,079)	(9,048)
Loss on exchange of convertible notes	(4,501)	—	(40,624)	—
Change in fair value of derivatives	—	(7,600)	—	(2,739)
Other income (expense)	(877)	(5,316)	209	(2,554)
Loss before income tax	(83,834)	(61,494)	(203,965)	(112,804)
Income tax (expense) benefit	(717)	(339)	(885)	1,053
Net loss attributable to common stockholders	\$ (84,551)	\$ (61,833)	\$ (204,850)	\$ (111,751)
Net loss attributable to common stockholders per common share — basic and diluted	\$ (0.36)	\$ (0.33)	\$ (0.91)	\$ (0.61)
Weighted-average common shares outstanding — basic and diluted	238,089,824	188,621,423	225,848,013	182,303,128

TABLE 2

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

	June 30, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	\$ 220,578	\$ 79,749
Investments in marketable securities	355,078	424,403
Accounts receivable	28,709	21,962
Inventories	10,395	8,390
Prepaid expenses and other current assets	20,116	16,592
Total current assets	634,876	551,096
Operating lease right-of-use assets, less accumulated amortization of \$2,641 and \$0 at June 30, 2019 and December 31, 2018, respectively	35,052	—
Property and equipment, less accumulated depreciation of \$16,890 and \$15,671 at June 30, 2019 and December 31, 2018, respectively	15,273	11,375
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	12,035	6,683
Total Assets	\$ 918,033	\$ 789,951
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	\$ 84,119	\$ 80,625
Deferred reimbursements	2,750	5,500
Operating lease liabilities	2,678	—
Total current liabilities	89,547	86,125
Deferred reimbursements	11,406	10,156
Convertible notes	2,070	175,006
Senior secured term loan	146,994	146,734
Contingent consideration payable	21,247	19,700
Deferred income taxes	6,465	6,465
Operating lease liabilities	36,259	—
Other non-current liabilities	3,987	2,853
Total liabilities	317,975	447,039
Commitments and contingencies		
Stockholders' equity:		
Common stock, \$0.01 par value, 500,000,000 shares authorized, 254,513,522 and 189,383,924 shares issued and outstanding at June 30, 2019 and December 31, 2018, respectively	2,589	1,942
Additional paid-in capital	2,201,447	1,740,061
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	352	495
Unrealized gain (loss) on available-for-sale securities	355	(427)
Warrants	12,387	13,063
Accumulated deficit	(1,617,072)	(1,412,222)
Total stockholders' equity	600,058	342,912
Total Liabilities and Stockholders' Equity	\$ 918,033	\$ 789,951



2Q19 Financial Results Conference Call & Webcast

August 8, 2019



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, 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. 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, 2018 and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2019. 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.

2Q19 and Early 3Q19 Highlights

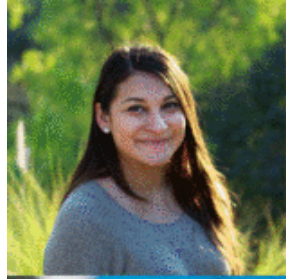
2Q19 Galafold Revenue of \$44.1M - Continued Global Strength for Successful Rare Disease Launch

AT-GAA PROPEL Pivotal Study Now Majority Enrolled

Positive 2-Year Interim Clinical Results in CLN6 Batten Disease - Potential to Halt Progression of Fatal Neurologic Disease

Expanded Penn Collaboration Provides Industry's Largest Rare Disease Gene Therapy Pipeline

Strengthened Balance Sheet with \$575M+ Cash at 6/30/19



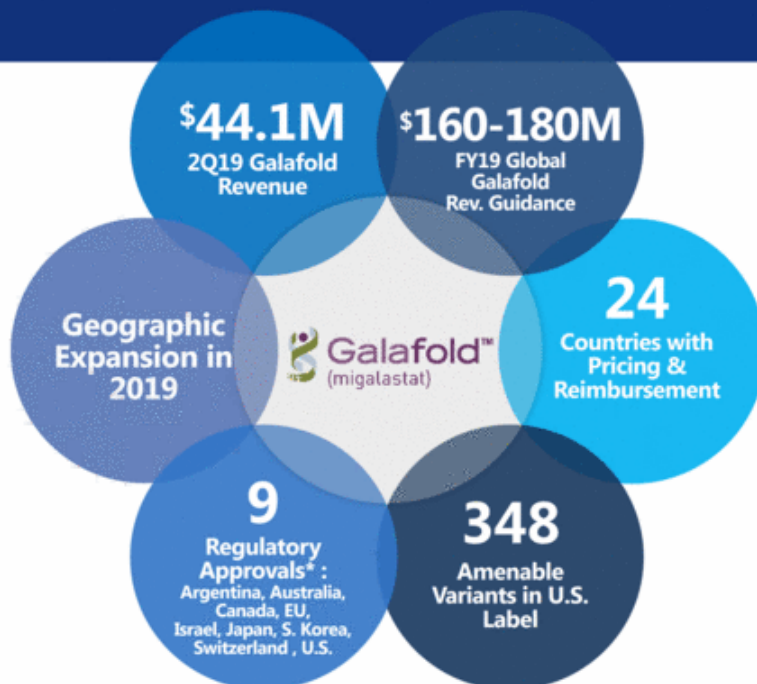
Galafold[®] (migalastat) Global Launch...

...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 June 30, 2019)

One of the Most Successful Rare Disease Launches



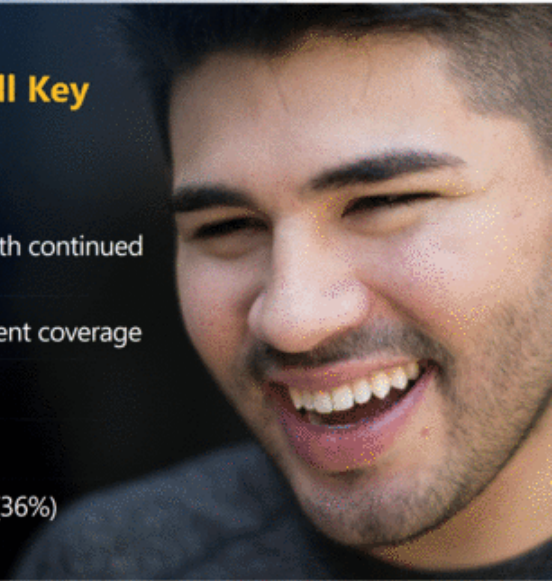
Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (123 mg) were headache, myalgia, upper respiratory tract infection, nausea and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit <http://www.amicus.com/Galafold>. For further important safety information for Galafold, including pregnancy 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.

*As of July 31, 2019

Galafold Global Launch Momentum (as of June 30, 2019)

2Q19 Strength Reflects Positive Momentum Across All Key Global Commercial Metrics

- **Global:** ~24% estimated global market share of treated amenable patients* with continued >90% compliance and adherence
- **U.S:** Steady growth in adoption from 100+ prescribers and broad reimbursement coverage
- **International:** Growing contribution from previously untreated patients
- **Japan:** on track to deliver full year objectives
- **Demographics:** global mix of switch (64%) and previously untreated patients (36%)



*Market share based on reported 2018 global Fabry sales and assumes a 35% amenability rate.

Galafold Quarterly Performance

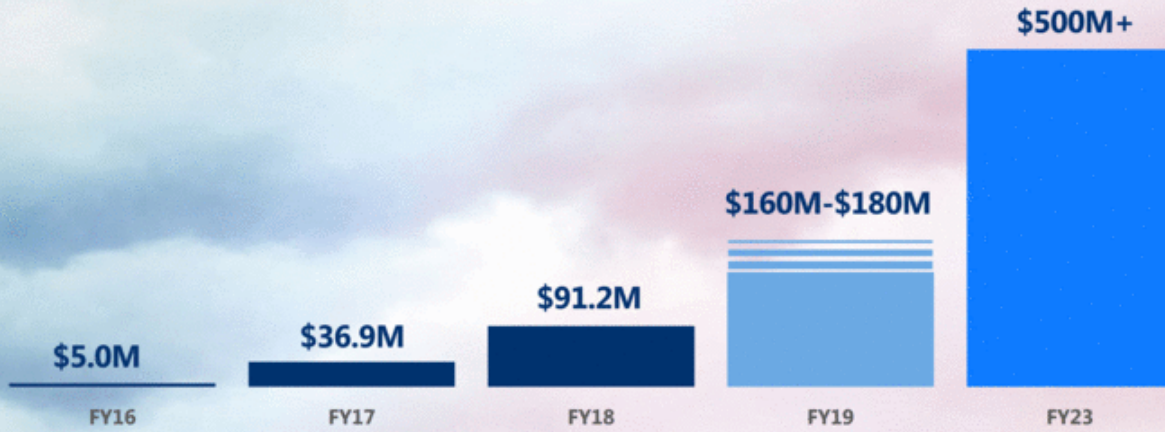
2Q19 Revenue of \$44.1M Grew 107% Year-over-Year Reflecting Continued Strong Growth in Global Adoption of First Fabry Oral Precision Medicine



- Consistent with Galafold adoption trends and ordering patterns in previous years, quarter to quarter growth will not be linear
- Higher revenue growth in 2Q19 as expected; +115% YoY constant currency revenue growth, offset by negative currency impact (-\$1.7M, or -8%)

Galafold Success and FY19 Galafold Revenue Guidance

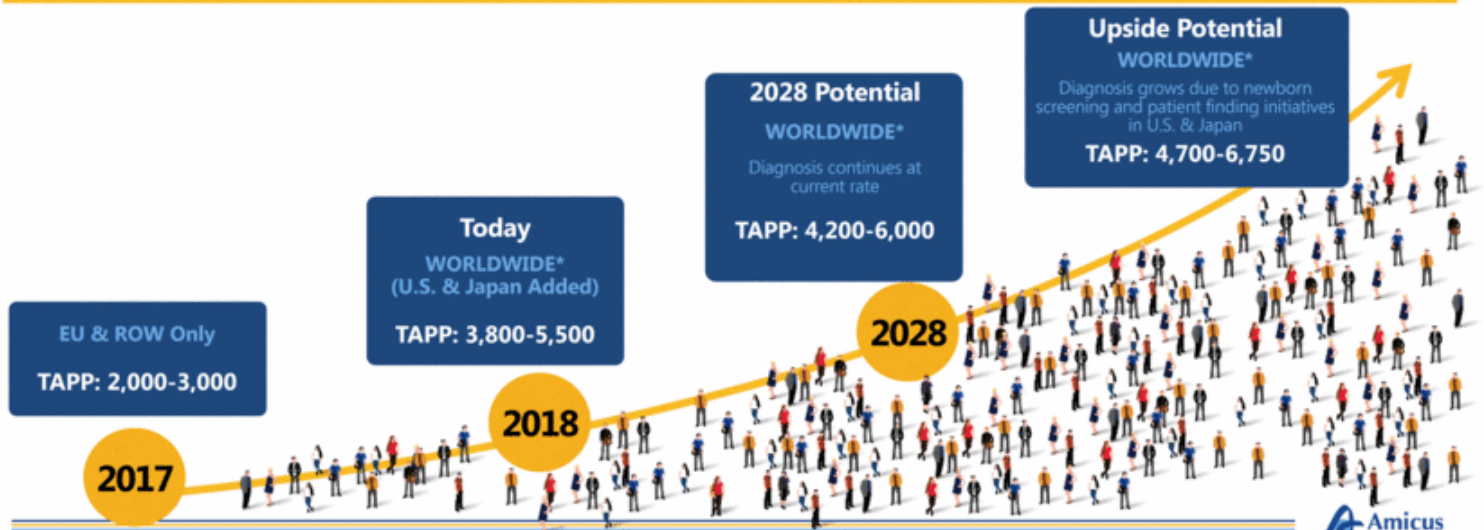
Galafold Sales in 2019 Represent the First Full Year of Launch in the Major Geographies in the World and will be between \$160-\$180M, with more than 1,000 Fabry Patients on Therapy by the End of 2019. Galafold Sales of \$500M+ are Expected by 2023



Total Amenable Patient Population ("TAPP")

Estimate based on 35% - 50% amenability

Fabry Disease is Increasingly Believed to be One of the Most Prevalent Human Genetic Diseases. The Total Amenable Patient Population ("TAPP") has the Potential to Make Galafold a \$1B+ Annual Product



*WORLDWIDE includes total amenable patient population in all Fabry ERT commercial markets today including an estimated effect of newborn screening on adult diagnostic rate.



AT-GAA Novel ERT for Pompe Disease

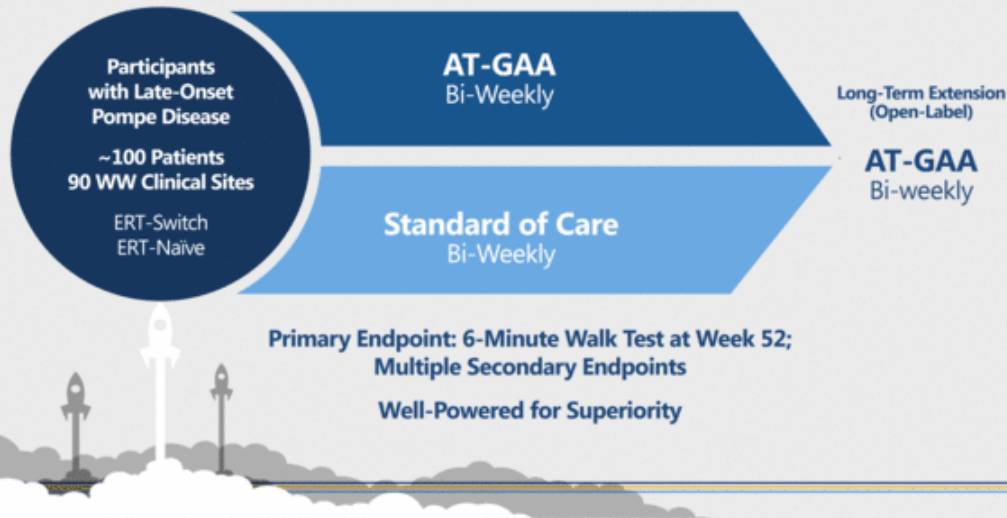
"We encourage and embrace constant innovation"
- Amicus Belief Statement

PROPEL (ATB200-03) Study Design



AT-GAA is the First Ever Second-Generation Product for Any Lysosomal Disorder to Earn Breakthrough Therapy Designation ("BTD") from the FDA

52-Week Primary Treatment Period (Double-Blind)
2:1 Randomization



BTD Criteria

- Preliminary clinical evidence indicates drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints

AT-GAA BTD Based on Ph 1/2 Clinical Efficacy

BTD Features

- Intensive guidance on an efficient drug development program
- Organizational commitment involving senior managers
- All Fast Track program features

PROPEL (ATB200-03) Study Global Enrollment **PROPEL**

Sites in 29 Countries are Now Active to Ensure Broad Access and Experience with AT-GAA



AT-GAA: 2019 Objectives

Advance AT-GAA for as Many Patients Worldwide as Quickly as Possible

- ✓ **Additional Phase 1/2 Data (up to 24 Months)**
- ✓ **Breakthrough Therapy Designation**
- **Enroll PROPEL study (n=100)**
- **Present additional Phase 1/2 data at World Muscle Society (Oct. 1-5)**
- **Report natural history study data**
- **Initiate pediatric study**
- **Advance agreed upon CMC requirements to support BLA**

Mepsevii™ for Mucopolysaccharidosis Type VII (MPS VII, Sly syndrome)

Licensed Exclusive Japanese Rights to Mepsevii to Leverage Existing Infrastructure, Relationships and Experience In Clinical Development, Regulatory Approvals and Commercialization within the Lysosomal Disorders Community in Japan

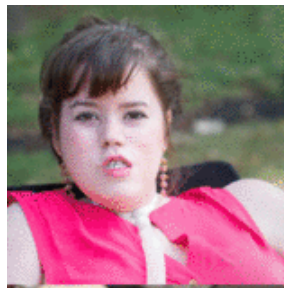
Mepsevii™
(vestronidase alfa-vjvk)
injection, for intravenous use

Amicus and Ultragenyx
are Aligned on Strategy
to Advance Mepsevii for
People in Japan with
MPS VII

Amicus Licensed Exclusive Japanese Rights to
Mepsevii (vestronidase alfa) from Ultragenyx

Mepsevii is currently approved for the treatment of children and
adults with MPS VII in U.S., EU and Brazil.

Existing Mepsevii studies and investigator-initiated
study in Japan to support future J-NDA submission



Gene Therapy Pipeline

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

A RARE PORTFOLIO.

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3	REGULATORY	COMMERCIAL
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Fabry Franchise

Galafold®(migalastat) monotherapy						
Fabry Gene Therapy	PENN					

Pompe Franchise

AT-GAA (Novel ERT + Chaperone)						
Pompe Gene Therapy	PENN					

Batten Franchise – Gene Therapies

CLN6 Batten Disease	NCH					
CLN3 Batten Disease	NCH					
CLN8 Batten Disease	NCH					
CLN1 Batten Disease	NCH					

Next Generation Research Programs and CNS Gene Therapies

CDKL5 Deficiency Disorder GTx / ERT	PENN					
Niemann-Pick Type C (NPC)	NCH / PENN					
Tay-Sachs Disease	NCH					
Other	NCH / PENN					

MPS Franchise - Gene Therapies

Next Generation MPSIIIA	PENN					
MPSIIIB	PENN					

In less than a year, Amicus assembled the largest portfolio of gene therapy programs for rare diseases in the entire industry

Amicus-Penn Gene Therapy Collaboration

New Indications and Next Generation Research Program to Harness Combination of Amicus-Penn Technologies



GENE
THERAPY
PROGRAM



Worldwide Rights to Penn's Next Generation Gene Therapy Technologies for the Majority of Lysosomal Disorders

Six Active Preclinical Programs: Fabry, Pompe, CDKL5 Deficiency Disorder (CDD), Niemann-Pick Type C, Next Generation MPS IIIA, and MPS IIIB

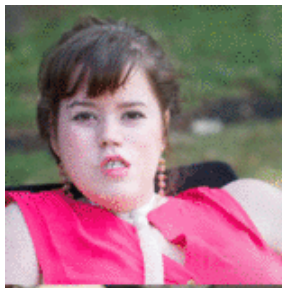
12 Additional Rare Diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy and Select Other Muscular Dystrophies

Research Program to Improve Safety, Efficacy and Manufacturability of Next Generation Vectors (\$10M / Year for 5 Years; Option to Extend)

5 Key Takeaways for AAV-CLN6 Gene Therapy

Interim Safety and Efficacy Data Demonstrate the Potential for AAV-CLN6 Gene Therapy to Stabilize Progression of a Devastating Disease

- **Meaningful impact on motor and language function** in children with a fatal neurologic disease that destroys brain function
- **Evidence of disease stabilization** in seven out of the eight children following AAV-CLN6 gene transfer
- **Natural history cohort shows progressive loss of language and motor function** in all untreated patients
- **Sibling comparisons (in-study and natural history) provide further support** for AAV-CLN6 gene therapy and early intervention
- **Favorable safety profile** with intrathecal administration of AAV in all study participants



Financial Summary

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

2Q19 Select Financial Results

2Q19 Revenue of \$44.1M Primarily from International Galafold Sales

<i>(in thousands, except per share data)</i>	June 30, 2019	June 30, 2018
Product revenue	44,130	21,309
Cost of goods sold	5,367	3,135
R&D expense	70,981	34,660
SG&A expense	42,578	29,172
Changes in fair value of contingent consideration	480	300
Loss from operations	(76,430)	(46,931)
Income tax expense	(717)	(339)
Net loss	(84,551)	(61,833)
Net loss per share	(0.36)	(0.33)

Financial Summary and Guidance

Strong Balance Sheet with \$575M+ Cash at 6/30/19 - Cash Runway into 2021

FINANCIAL POSITION

Cash	\$575.7M
Cash Runway¹	Into 2021
Debt²	\$152.8M

CAPITALIZATION

Shares Outstanding³	254,513,522
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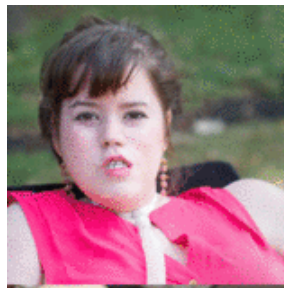
FINANCIAL GUIDANCE

FY19 Galafold Revenue Guidance	\$160M-\$180M
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¹Based on existing operating plan including proceeds from June 2019 equity offering to invest in manufacturing

²Includes \$2.8 million of convertible debt and \$150 million of straight debt

³Includes shares from June 2019 equity offering



Summary & Milestones

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

2019 Key Strategic Priorities

- 1 **Nearly double annual revenue for Galafold® (guidance \$160M-\$180M)**
- 2 **Complete enrollment in AT-GAA Pivotal Study (PROPEL) and report additional Phase 1/2 data**
- 3 **Report additional 2-year clinical results in CLN6-Batten disease and complete enrollment in ongoing CLN3-Batten disease Phase 1/2 study**
- 4 **Establish preclinical proof of concept for Fabry and Pompe gene therapies**
- 5 **Maintain strong financial position**

Anticipated Milestones

Well-Positioned to Create Significant Value for Patients and Shareholders

Galafold: Fabry Disease

- FY19 revenue guidance \$160M-\$180M
- Growth in existing markets
- Expansion into new markets
- Diagnostic initiatives

AT-GAA: Pompe Disease

- ✓ Additional Phase 1/2 data (21 and 24 months)
- ✓ Breakthrough therapy designation (BTD) in LOPD
- ✓ Phase 1/2 study fully enrolled (Cohorts 1-4)
 - PROPEL pivotal study enrollment (n=100)
 - Additional Phase 1/2 data (Cohort 4)
 - Natural history study data
 - Additional supportive studies
 - Advance CMC requirements to support BLA

Gene Therapy Programs

- ✓ Positive initial preclinical data for Pompe gene therapy
- ✓ Selection of CMO partners
- ✓ Complete enrollment in low-dose cohort in CLN3 Batten disease Phase 1/2 study
- ✓ Additional 2-year clinical data in CLN6 Batten disease
 - Advance CLN6 Batten disease clinical, manufacturing and regulatory strategy
 - Complete enrollment in high-dose cohort in CLN3 Batten disease Phase 1/2 study
 - Selection of Amicus late process development and manufacturing facilities
 - Additional preclinical data including next-generation gene therapies for Fabry and Pompe
 - Selection of Pompe AAV gene therapy IND candidate

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement

