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

FORM 8-K

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

Date of Report (Date of earliest event reported): **May 8, 2019**

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

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

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:

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

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

Emerging growth company o

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. o

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, $0.01 Par Value	FOLD	NASDAQ

Item 2.02 Results of Operations and Financial Condition

On May 8, 2019, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the fiscal quarter ended March 31, 2019. A copy of this press release is attached hereto as Exhibit 99.1. The Company will host a conference call and webcast on May 8, 2019 to discuss its first quarter results of operations. A copy of the conference call presentation materials is attached hereto as Exhibit 99.2. Both exhibits are incorporated herein by reference.

In accordance with General Instruction B.2. of Form 8-K, the information in this Current Report on Form 8-K and the Exhibits shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits:

Exhibit No.	Description
99.1	Press Release dated May 8, 2019
99.2	May 8, 2019 Conference Call Presentation Materials

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: May 8, 2019

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

Amicus Therapeutics Announces First Quarter 2019 Financial Results and Corporate Updates

1Q19 Galafold® (migalastat) Strong Launch Trends Continue with Largest Quarterly Number of Net New Patients Added Globally (150+ Net New Galafold Patients in 1Q19)

1Q19 Galafold Revenue of $34.0M and Early 2Q19 Momentum on Key Launch Metrics

Maintaining FY19 Revenue Guidance of $160M-$180M and 1,000+ Fabry Patients on Galafold Globally

AT-GAA in Pompe Disease Continues to Advance in PROPEL Pivotal Study with Full Enrollment on Track by Year-End

Gene Therapy Focus Advances with Positive Preclinical Pompe Gene Therapy Data and Significant Progress Across All Gene Therapy Programs - On Track to Report Additional Phase 1/2 Clinical Data in CLN6 Batten Disease in Mid-2019 (Q3)

Strong Balance Sheet with $438.3M+ Cash

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

CRANBURY, NJ, May 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 first quarter ended March 31, 2019. The Company also summarized recent program updates and reiterated its full-year 2019 guidance.

Corporate Highlights for 1Q19 and Early 2Q19

- **Global revenue for Galafold (migalastat) in the first quarter of 2019 was $34.0M, in line with management expectations, and continues to track toward full-year 2019 revenue guidance of $160M-$180M and 1,000+ patients by year-end**. The first quarter revenue represented a year-over-year increase of 104% from total revenue of $16.7 million in the first quarter of 2018**.**

  Patient adoption and wide prescriber experience continues globally for Galafold. Highest number since launch of net new patients added in 1Q19 (150+) with higher than anticipated prescriptions in EU, Japan and US, and continued global compliance and adherence rates exceeding 90%. Over 200 patient referral forms (PRFs) written now by over 90 physicians in the United States, with continued strong reimbursement from payors, since the launch in August 2018.

- **New intellectual property issued for Galafold.** The new patent covers a method of treatment and provides additional protection through 2038.
- **Positive Initial Proof-of-Concept for Preclinical Pompe Gene Therapy Data.** Positive preclinical results showed robust uptake and glycogen reduction in multiple tissues, including brain and spinal cord. Additional preclinical studies underway to select a clinical candidate in 2019. This program is significantly ahead of schedule reflecting a strong collaboration with the University of Pennsylvania.
- **Enrollment momentum in ongoing pivotal PROPEL study in Pompe disease**. The PROPEL study is now underway at 24 participating sites and is on track to achieve full enrollment of ~100 patients by year-end 2019. Significant enthusiasm from Pompe patient and physician community continues.
- **Additional ERT-Switch Patients (Cohort 4) Fully Enrolled in Pompe Phase 1/2 Clinical Study**: Initial 6-month data in Cohort 4, and complete 24-month data in Cohorts 1-3, anticipated in 2H19.

- **Additional data from Phase 1/2 CLN6 Batten disease study anticipated 3Q19.** A total of 12 patients have received a single administration of AAV9-CLN6 gene therapy (exposure ranging from ~5 to 39 months). The top-line data are expected to include two-year results in approximately half the patients in 3Q19 with more detail at a scientific congress later in 2019.
- **Low dose cohort fully enrolled in Phase 1/2 study in CLN3 Batten disease**. A total of three patients were dosed in the low dose cohort in the CLN3 Batten disease study with no serious adverse events to date following a single administration of AAV9-CLN3 gene therapy. The high dose cohort is expected to commence in the coming months.
- **Robust gene therapy pipeline continues to advance toward important data milestones**. Additional preclinical proof-of-concept data in Pompe, initial preclinical data in Fabry, and further advancement of neurologic lysosomal disorder preclinical programs, expected throughout 2019.
- **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 approximately $438.3 million at March 31, 2019 is expected to fund ongoing operations into at least mid-2021.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “During the first quarter and into the second quarter, we have made tremendous progress in executing across our five key strategic priorities for 2019. With the success of the global Galafold launch and long-term opportunity in Fabry, our leadership position in Pompe disease with a late-stage Phase 3 program and now positive preclinical data in gene therapy, and a rapidly advancing gene therapy pipeline that includes two clinical-stage programs in Batten disease, we are marching closer toward our vision to treat 5,000+ patients and achieve $1B+ in revenue by 2023. As we continue to build a leading global biotechnology company in rare metabolic diseases, specifically within the field of human genomic medicine, we remain more focused than ever on developing groundbreaking new medicines and hopefully one day cures, and delivering them to people living with these life-threatening conditions as quickly as possible.”

First Quarter 2019 Financial Results

- Total revenue in the first quarter 2019 was $34.0 million, a year-over-year increase of 104% from total revenue of $16.7 million in the first quarter of 2018. This includes year-over-year operational revenue growth measured at constant currency exchange rates of 114.9%, offset by negative currency impact of $1.8M, or 11.0%.
- Cash, cash equivalents, and marketable securities totaled $438.3 million at March 31, 2019, compared to $504.6 million at December 31, 2018.
- Total operating expenses of $111.3 million for the first quarter of 2019 increased as compared to $70.3 million in the first quarter of 2018, reflecting continued investments in the Galafold launch, Pompe program, and gene therapy pipeline.
- Net loss was $120.3 million, or $0.56 per share, compared to a net loss of $49.9 million, or $0.28 per share, for the first quarter 2018.

2019 Key Strategic Priorities

- Nearly double annual revenue for Galafold (FY19 guidance of $160M-$180M in worldwide revenue) with 1,000+ Fabry patients on Galafold by year end.
- Complete enrollment in pivotal 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. Consistent with Galafold adoption trends and ordering patterns seen in previous years, Amicus expects higher revenue growth in the second and fourth quarters of 2019.

Cash, cash equivalents, and marketable securities totaled $438.3 million at March 31, 2019. The Company expects to end 2019 with approximately $300 million in cash on hand. The current cash position is anticipated to fund ongoing operations into at least mid-2021.

Anticipated 2019 Milestones by Program

Galafold (migalastat) Oral Precision Medicine for Fabry Disease

- On track to meet full-year 2019 revenue guidance range of $160 million to $180 million.

AT-GAA for Pompe Disease

- Initial 6-month data in additional ERT-switch patients (Cohort 4) in Phase 1/2 ATB200-02 clinical study.
- 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 study.
- Advance agreed upon CMC requirements to support BLA.

Gene Therapy Pipeline:

- Additional two-year data from CLN6 Batten disease Phase 1/2 study.
- Full enrollment of ongoing CLN3 Batten disease Phase 1/2 study.
- Additional preclinical data including next-generation gene therapies for Fabry and Pompe
- Selection of Pompe AAV gene therapy clinical candidate to move into IND-enabling studies

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Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, May 8, 2019 at 8:30 a.m. ET to discuss the first 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: 8358807.

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 May 8, 2019. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 8358807.

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 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 (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia.

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

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

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

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

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

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

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

- Galafold is not intended for concomitant use with enzyme replacement therapy.
- Galafold is not recommended for use in patients with Fabry disease who have severe renal impairment (<30 mL/min/1.73 m^2). The safety and efficacy of Galafold in children 0–15 years of age have not yet been established.
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.
- While taking Galafold, effective birth control should be used. It is not known whether Galafold is excreted in human milk.
- Contraindications to Galafold include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on Galafold or switched to Galafold.
- OVERDOSE: General medical care is recommended in the case of Galafold overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received Galafold. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

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

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

Non-GAAP Financial Measures
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:
Amicus Therapeutics
Sara Pellegrino, IRC
Vice President, Investor Relations and Corporate Communications
spellegrino@amicusrx.com
(609) 662-5044

Media:

Amicus Therapeutics
Marco Winkler
Director, Corporate Communications
mwinkler@amicusrx.com
(609) 662-2798

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TABLE 1

Amicus Therapeutics, Inc.
Consolidated Statements of Operations
(Unaudited)
(in thousands, except share and per share amounts)

	Three Months Ended March 31,	
	2019	**2018**
Revenue:		
Net product sales	$ 34,046	$ 16,696
Cost of goods sold	4,055	2,615
Gross profit	29,991	14,081
Operating expenses:		
Research and development	64,593	40,798
Selling, general and administrative	44,303	27,396
Changes in fair value of contingent consideration payable	1,383	1,100
Depreciation and amortization	991	969
Total operating expenses	111,270	70,263
Loss from operations	(81,279)	(56,182)
Other income (expense):		
Interest income	2,639	1,737
Interest expense	(6,454)	(4,488)
Loss on exchange of convertible notes	(36,123)	—
Change in fair value of derivatives	—	4,861
Other income	1,086	2,764
Loss before income tax	(120,131)	(51,308)
Income tax (expense) benefit	(168)	1,392
Net loss attributable to common stockholders	$ (120,299)	$ (49,916)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.56)	$ (0.28)
Weighted-average common shares outstanding — basic and diluted	213,519,287	175,977,700

TABLE 2

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

	March 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 96,349	$ 79,749
Investments in marketable securities	341,978	424,403
Accounts receivable	22,960	21,962
Inventories	8,167	8,390
Prepaid expenses and other current assets	13,592	16,592
Total current assets	483,046	551,096
Operating and finance lease right-of-use assets, less accumulated amortization of $2,111 and $0 at March 31, 2019 and December 31, 2018, respectively	36,308	—
Property and equipment, less accumulated depreciation of $15,794 and $15,671 at March 31, 2019 and December 31, 2018, respectively	13,286	11,375
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	11,265	6,683
Total Assets	$ 764,702	$ 789,951
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	$ 60,596	$ 80,625
Deferred reimbursements	2,750	5,500
Operating and finance lease liabilities	2,523	—
Total current liabilities	65,869	86,125
Deferred reimbursements	11,406	10,156
Convertible notes	22,052	175,006
Senior secured term loan	146,766	146,734
Contingent consideration payable	20,767	19,700
Deferred income taxes	6,465	6,465
Operating and finance lease liabilities	36,100	—
Other non-current liabilities	3,609	2,853
Total liabilities	313,034	447,039
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 shares authorized, 230,180,714 and 189,383,924 shares issued and outstanding at March 31, 2019 and December 31, 2018, respectively	2,347	1,942
Additional paid-in capital	1,970,607	1,740,061
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	(1,309)	495
Unrealized gain (loss) on available-for-sale securities	157	(427)
Warrants	12,387	13,063
Accumulated deficit	(1,532,521)	(1,412,222)
Total stockholders' equity	451,668	342,912
Total Liabilities and Stockholders' Equity	$ 764,702	$ 789,951

Amicus Therapeutics

1Q19 Financial Results Conference Call & Webcast

May 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, th prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plan financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be reg 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 ou wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with resp 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 risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate 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 tha may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or our other product candidates if and whe 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 add funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be pred of future results. With respect to statements regarding projections of the Company's revenue and cash position, actual results may differ based market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject t 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 the forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautio statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

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

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 Statemer

Galafold Snapshot (as of March 31, 2019)

One of the Most Successful Rare Disease Launches

$34.0M
1Q19 Galafold Revenue

$160-180M
FY19 Global Galafold Rev. Guidance

Geographic Expansion in 2019

Galafold™ (migalastat)

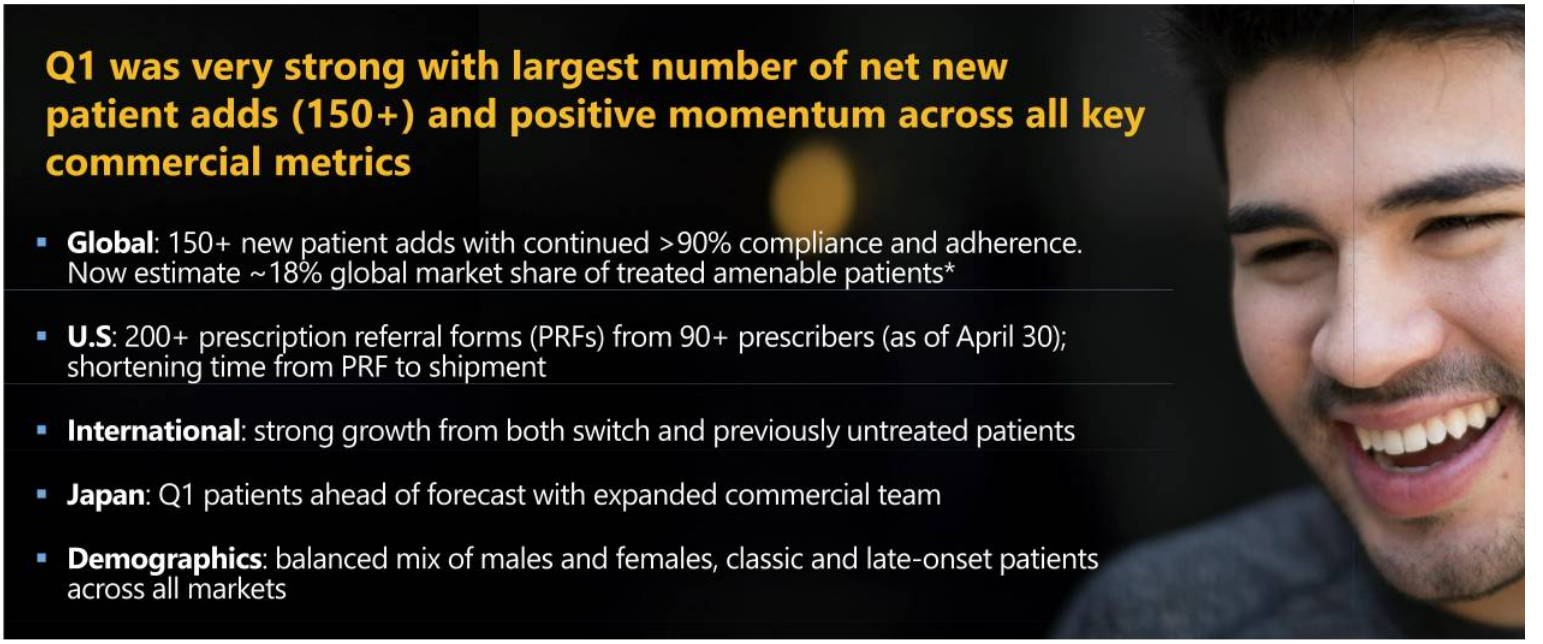
24
Countries with Pricing & Reimbursemen

8
Regulatory Approvals:
Australia, Canada, EU, Israel, Japan, S. Korea, Switzerland , U.S.

348
Amenable Variants in U.S. Label

Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit https://www.amicusrx.com/pi/Galafold.pdf. For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at www.ema.europa.eu.

Galafold Global Launch Momentum (as of March 31, 2019)



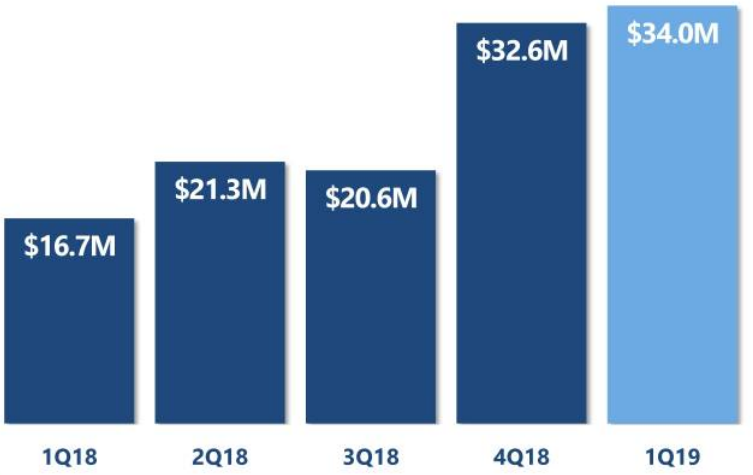
Q1 was very strong with largest number of net new patient adds (150+) and positive momentum across all key commercial metrics

- **Global**: 150+ new patient adds with continued >90% compliance and adherence. Now estimate ~18% global market share of treated amenable patients*
- **U.S**: 200+ prescription referral forms (PRFs) from 90+ prescribers (as of April 30); shortening time from PRF to shipment
- **International**: strong growth from both switch and previously untreated patients
- **Japan**: Q1 patients ahead of forecast with expanded commercial team
- **Demographics**: balanced mix of males and females, classic and late-onset patients across all markets

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

Galafold Quarterly Performance

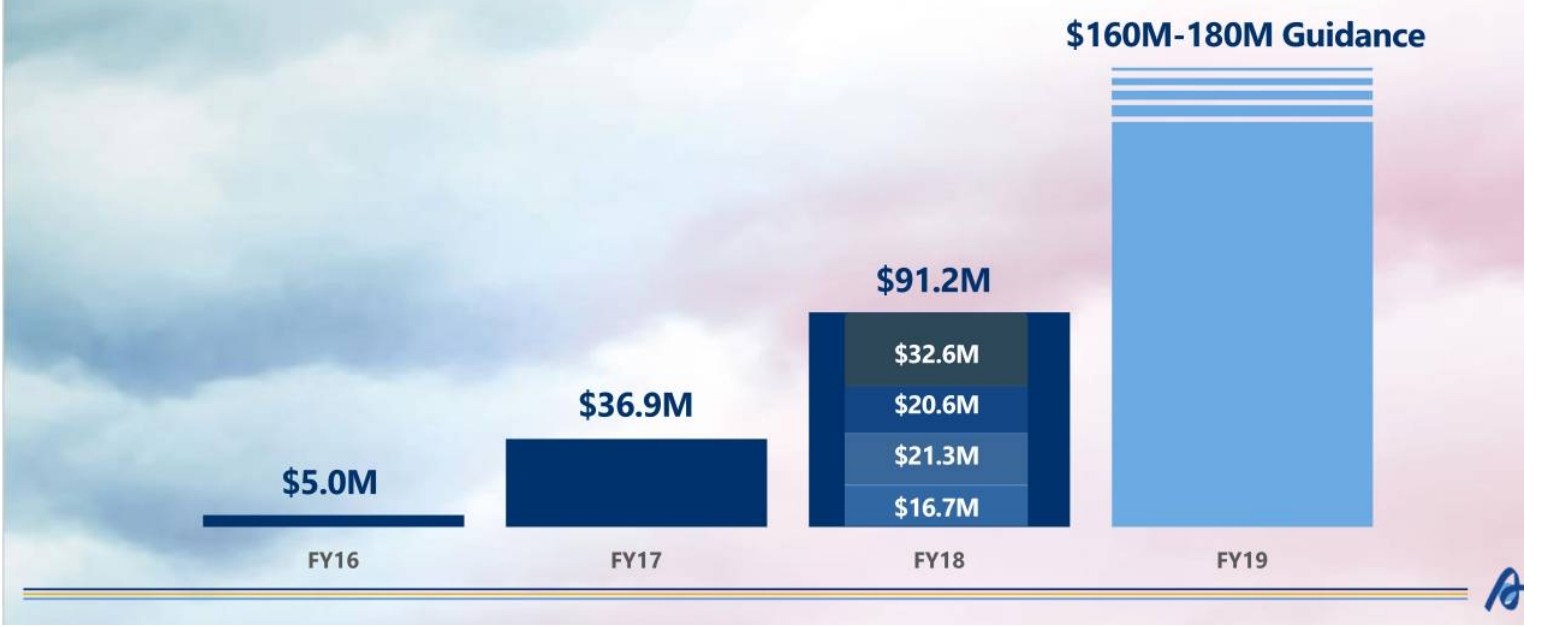
1Q19 Revenue of $34.0M Grew 104% Year-over-Year Reflecting Continued Stro
Growth in Global Adoption of First Fabry Oral Precision Medicine



- 1Q19 in-line with management expectations
- Consistent with Galafold adoption trends and ordering patterns in previous years, quarter to quarter growth will not be linear
- Strong start to 2Q19 ahead of management expectations
- Higher revenue growth expected in 2Q19 and 4Q19

Galafold Success and FY19 Galafold Revenue Guidance

On Track to Nearly DOUBLE Revenue Again and Serve 1,000+ Patients in 2019

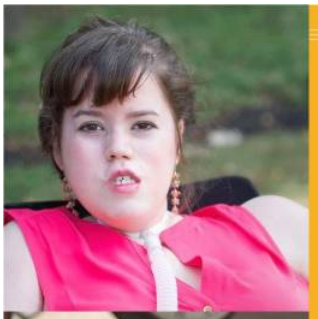


Total Amenable Patient Population ("TAPP")

Estimate based on 35% - 50% amenability

$1B+ Addressable Market Opportunity by 2028

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

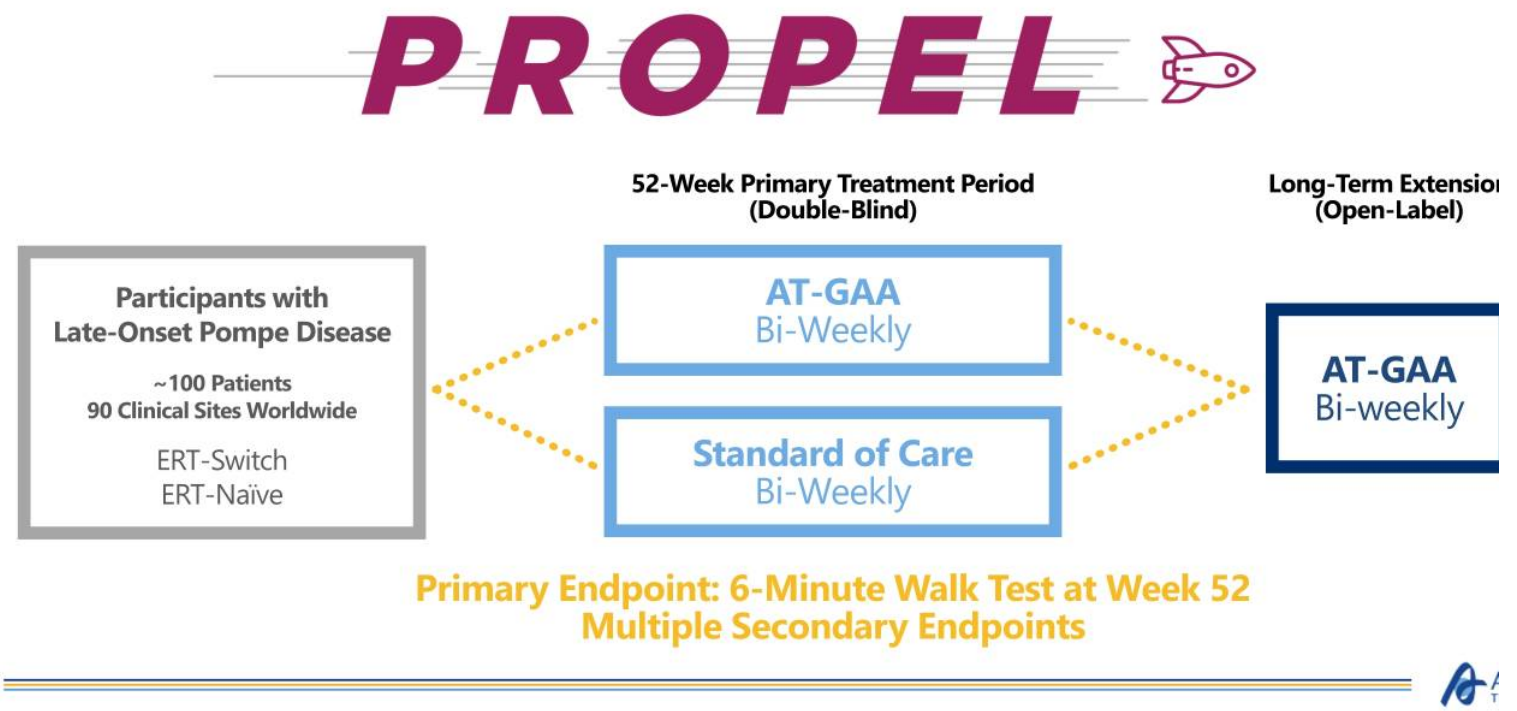


AT-GAA Novel ERT for Pompe Disease

"We encourage and embrace constant innovation

- Amicus Belief Statemer

PROPEL (ATB200-03) Study Design



AT-GAA: Breakthrough Therapy Designation

U.S. FDA Granted BTD to AT-GAA in Late-Onset Pompe Disease (LOPD)

BTD Criteria

- Intended to treat a serious or life threatening disease or condition
- Preliminary clinical evidence indicates drug may demonstrate substantial improvement over existing therapies on one o more clinically significant endpoints

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

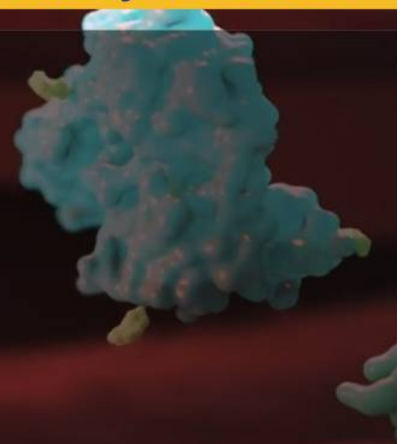
- Improvements in 6-minute walk distance
- Comparison to natural history of treated patients

BTD Features

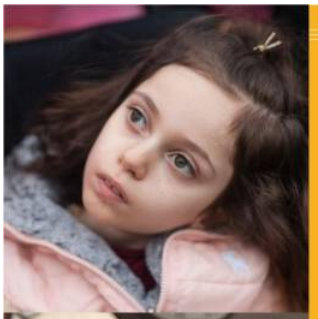
- Intensive guidance on an efficient drug development program
- Organizational commitment involving senior managers
- All Fast Track program features
- Potential Rolling BLA
- Potential for Priority Review

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**
- ✓ **Full enrollment of Phase 1/2 Study (Cohorts 1-4)**
- • **Full enrollment of PROPEL study (n=100)**
- • **Present additional Phase 1/2 data (Cohort 4)**
- • **Report natural history study data**
- • **Initiate supportive pediatric study**
- • **Advance agreed upon CMC requirements to support BLA**



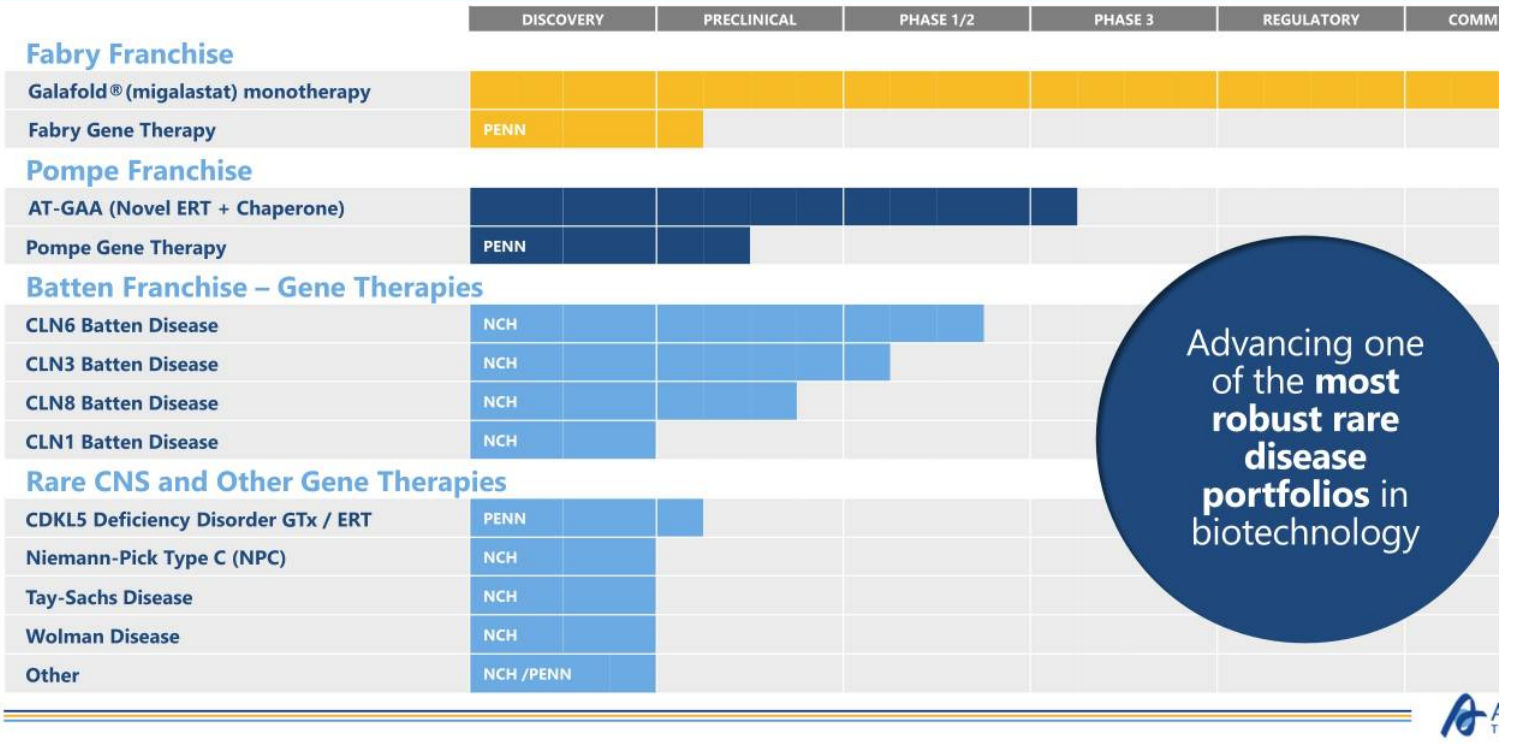
Gene Therapy Pipeline

"We have a duty to obsolete our own technologies

- Amicus Belief Statemer

Amicus Pipeline

A RARE PORTFOLIO.



Leading Lysosomal Disorder Gene Therapy Portfolio

Multiple Platforms Provide 14 Gene Therapy Programs and R&D Engine for Future Gro

Gene Therapy Program Updates

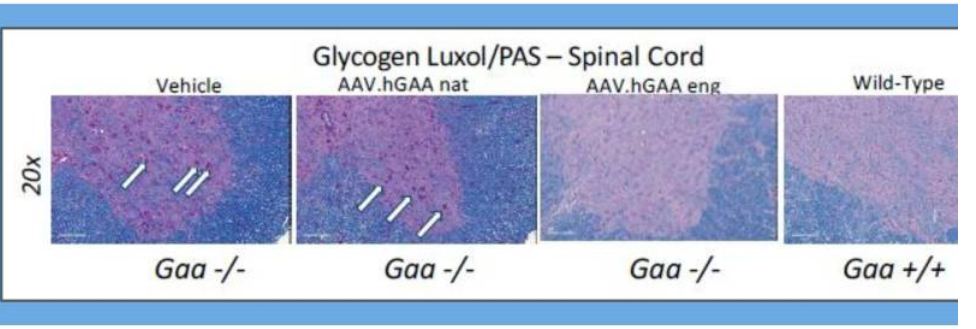
- Positive initial preclinical data for Pompe gene therapy
- CLN3 Batten Disease Phase 1/2 Study Enrolling; Low Dose Cohort Complete (n=3)
- Additional 2-Year CLN6 Batten Disease Phase 1/2 Data on Track for 3Q19
- Additional preclinical studies in progress including CLN8, CLN1, Pompe and Fabry
- R&D Engines for Future Growth

Preclinical Pompe Gene Therapy Results Presented at ASGCT

Initial Findings Validate Amicus/Penn Collaboration Combining Amicus-Engineered Transgenes with Penn's AAV G Therapy Technologies

Key findings:

- Improved cellular uptake and glycogen reduction observed with engineered AAV-hGAA
- Robust glycogen reduction in CNS observed only with engineered AAV-hGAA



Program Status and Anticipated Milestones

- Builds upon protein engineering and manufacturing expertise used to successfully dev AT-GAA
- Additional preclinical studies underway (various doses and routes of AAV administratio
- Selection of clinical candidate in 2019 to move into IND-enabling studies
- Platform potential to design constructs that enhance protein targeting across multiple lysosomal disorders

Milestones

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

Anticipated Milestones: 2019

Well-Positioned to Create Significant Value for Patients and Shareholders in 201

Galafold: Fabry Disease

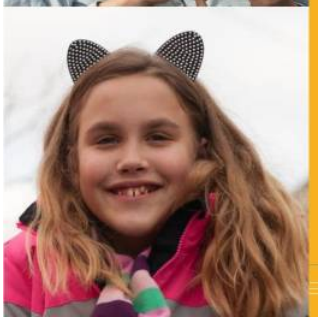
- FY19 revenue guidance $160M-$180M
- Growth in existing markets
- Expansion into new markets
- Diagnostic initiatives

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 Program

- Ongoing CLN3 Batten disease Phase 1/2 study enrollment
- Additional 2-year data from C Batten disease Phase 1/2 stud
- Additional preclinical data including next-generation ge therapies for Fabry and Pomp
- Selection of Pompe AAV ge therapy clinical candidate to move into IND-enabling stu



Financial Summary

Quarter Ended March 31, 2019

"Maximizing Value for Shareholder
is the Foundation for Our Future Successes
- Amicus Belief Statemer

1Q19 Select Financial Results

1Q19 Revenue was $34.0M (+104% vs. 1Q18). Includes YoY Operational Revenue Growth Measure Constant Currency Exchange Rates of 114.9%, Offset by Negative Currency Impact of $1.8M, or 11

(in thousands, except per share data)	Mar. 31, 2019	Mar. 31, 2018
Product revenue	34,046	16,696
Cost of goods sold	4,055	2,615
R&D expense	64,593	40,798
SG&A expense	44,303	27,396
Changes in fair value of contingent consideration	1,383	1,100
Loss from operations	(81,279)	(56,182)
Income tax (expense) benefit	(168)	1,392
Net loss	(120,299)	(49,916)
Net loss per share	(0.56)	(0.28)

Financial Summary and Guidance

Strong Balance Sheet with $435M+ Cash at 3/31/19- Cash Runway into 2021

FINANCIAL POSITION	March 31, 2019
Cash	~$438M
Cash Runway	Into at least mid-2021
CAPITALIZATION	
Shares Outstanding	230,180,714
FINANCIAL GUIDANCE	
Projected YE 2019 Cash Balance	~$300M
Galafold Revenue Guidance	$160M-$180M

A RARE VISION. Impacting Lives

>350 Patients* | ~$36M Global Sales

YE17

>700 Patients* | ~$91M[1] Global Sales

YE18

5,000 Patients* | $1B Global Sa

2023

*Clinical & commercial, all figures approximate [1]Preliminary unaudited

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement

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