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): **March 1, 2021**

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

Delaware
(State or Other Jurisdiction of
Incorporation)

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

3675 Market Street, Philadelphia, PA 19104
(Address of Principal Executive Offices, and Zip Code)

215-921-7600
Registrant's Telephone Number, Including Area Code

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

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

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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

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

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

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

Item 2.02 – Results of Operations and Financial Condition

On March 1, 2021, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing its financial results for the fiscal year ended December 31, 2020. A copy of this press release is attached hereto as Exhibit 99.1. The Company will host a conference call and webcast on March 1, 2021 to discuss its full year 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 March 1, 2021
99.2	March 1, 2021 Conference Call Presentation Materials
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

Signature Page

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

Date: March 1, 2021

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

Amicus Therapeutics Announces Full-Year 2020 Financial Results and Corporate Updates

2020 Total Galafold Revenue of $260.9M; Increased 43 Percent Year-on-Year

Continued Strong Global Growth of Galafold Expected in 2021 with Revenue of $300M-$315M

AT-GAA Rolling BLA Submission in Pompe Disease Planned for Completion in 2Q21 and Other Global Submissions Expected Throughout 2021

CLN6 Batten Disease Gene Therapy Granted Fast Track Designation by U.S. FDA

Cash Position Sufficient to Achieve Self-Sustainability

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

PHILADELPHIA, PA, Mar. 1, 2021 – Amicus Therapeutics (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on discovering, developing and delivering novel medicines for rare diseases, today announced financial results for the full year ended December 31, 2020.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, "During 2020, Amicus remained resilient on our journey to becoming a leading global rare disease biotechnology company. Despite the challenges of COVID, Amicus emerged from 2020 a better and stronger company organizationally, strategically, scientifically and financially. Galafold continues its path to becoming the worldwide standard of care for Fabry patients with amenable variants. And we are steadfast and passionate in our commitment to advancing AT-GAA to global regulatory submissions as fast as possible for the benefit of all people living with Pompe disease. The data from the Phase 3 PROPEL study we believe continue to show the overwhelmingly positive benefits of AT-GAA compared to the only approved medicine in this devastating disease. And the unmet need in Pompe is so great. We have the team, the resources and the programs that strongly position us 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 full year of 2020 was $260.9 million**. Full year revenue represented a year-over-year increase of 43% from total revenue of $182.2 million in the full year of 2019. On a constant currency basis, full year 2020 total revenue was $258.6 million, representing operational revenue growth measured at constant currency exchange rates of 42%, which was further benefited by a positive currency impact of $2.3 million, or 1%. Galafold performance was driven largely by strong patient demand. Global compliance and adherence rates continue to exceed 90%.

- **Results from the global Phase 3 PROPEL clinical study of AT-GAA in late-onset Pompe disease (LOPD) were presented at the 17th Annual WORLD*Symposium*™ 2021.** The Company plans to complete the BLA submission in the second quarter of this year and anticipates additional regulatory submissions in the European Union and in other geographies throughout 2021.

- **The U.S. Food and Drug Administration (FDA) granted Fast Track Designation to the CLN6 Batten disease gene therapy, AT-GTX-501.** The Fast Track program facilitates the development and accelerates the review of new drugs for serious conditions, which have the potential to address unmet medical needs. A drug development program with Fast Track designation is afforded greater access to the FDA for the purpose of expediting the drug's development, review and potential approval**.**

- **Initial clinical data from the Phase 1/2 CLN3 gene therapy study were presented at the 17th Annual WORLD*Symposium*™ 2021.** Results suggest early signs of disease stabilization and the potential to slow the neurological disease progression in children living with CLN3 Batten disease. Regulatory interactions are ongoing and the Company expects to start the next clinical study in the second half of this year.

- **Preclinical data from the Company's Fabry disease gene therapy clinical candidate, AT-GTX-701, presented at the 17th Annual WORLD*Symposium*™ 2021.** As part of the research collaboration with the Gene Therapy Program of the Perelman School of Medicine at the University of Pennsylvania (Penn), initial data from the Fabry AAV gene therapy with an engineered GLA transgene improved for stability demonstrated greater substrate reduction than wild type constructs across all tissues and doses. These findings further validate the synergies of combining Amicus-engineered transgenes with Penn's AAV technologies to develop next generation gene therapies.

- **Cash position sufficient to achieve self-sustainability without the need for any future dilutive financings.** The Company continues to carefully manage expenses and investments, while executing on the Galafold launch, proceeding with AT-GAA global regulatory submissions and advancing development programs.

Full Year 2020 Financial Results

- Total revenue in the full year 2020 was $260.9 million, a year-over-year increase of 43% from total revenue of $182.2 million in the full year of 2019. On a constant currency basis, full year 2020 total revenue was $258.6 million, representing operational revenue growth measured at constant currency exchange rates of 42%.

  Reported revenue was aided by a positive currency impact of $2.3 million, or 1%.

- Cash, cash equivalents, and marketable securities totaled $483.3 million at December 31, 2020, compared to $452.7 million at December 31, 2019.

- Total GAAP operating expenses of $476.8 million for the full year 2020 increased as compared to $464.3 million for the full year 2019, reflecting continued investments in our pipeline offset by decreased travel and third-party costs.

- Total non-GAAP operating expenses of $415.7 million for the full year of 2020 increased as compared to $411.8 million in the full year of 2019, reflecting continued investments in our pipeline offset by decreased travel and third-party costs.[1]

- Net loss was $276.9 million, or $1.07 per share, compared to a net loss of $356.4 million, or $1.48 per share, for the full year 2019.

[1] Full reconciliation of GAAP results to the Company's non-GAAP adjusted measures for all reporting periods appear in the tables to this press release.

2021 Financial Guidance

- For the full-year 2021, the Company anticipates total Galafold revenue of at least $300-$315 million. Double-digit revenue growth in 2021 is expected to be driven by continued operational growth and commercial execution across all major markets, including the U.S., EU, U.K. and Japan.

- Non-GAAP operating expense guidance for the full-year 2021 is $410 million to $420 million, driven by continued investment in the global Galafold launch, AT-GAA clinical studies and pre-launch activities, and advancing our gene therapy pipeline.[2]

- Cash, cash equivalents, and marketable securities totaled $483.3 million at December 31, 2020. Based on current operating models, the Company believes that the current cash position, which includes the net proceeds from the 2020 Senior Secured Term Loan, and expected future revenues are sufficient to fund the Company's operations and ongoing research programs through to self-sustainability.

[2] A reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure is not available without unreasonable effort due to high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure.

Anticipated 2021 Milestones by Program

Galafold (migalastat) Oral Precision Medicine for Fabry Disease

- Continued revenue growth in 2021

- Plans to expand EU label to cover adolescent population

- Continued geographic expansion

- Registry and other Phase 4 studies

AT-GAA for Pompe Disease

- Complete the BLA submission in 1H2021 and the EU MAA submission to be completed in 2H2021
- Ongoing supportive studies, including pediatric and extension studies

Gene Therapy Portfolio

- Advance manufacturing and regulatory discussions for the CLN6 Batten disease gene therapy program and begin dosing additional patients with GMP grade material
- Report initial data from the CLN3 Batten disease Phase 1/2 study, advance manufacturing and regulatory discussions, and submit protocol for next clinical study to the IND
- Continue to progress IND-enabling work in both Pompe and Fabry gene therapy
- Disclose additional preclinical data and potential IND candidate declarations across multiple preclinical programs
- Manufacturing advancements and updates across the portfolio

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, March 1, 2021 at 8:30 a.m. ET to discuss the full year 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: 6959755.

A live audio webcast and related presentation materials can also be accessed via the Investors section of the Amicus Therapeutics corporate website at ir.amicusrx.com. Web participants are encouraged to register on the website 15 minutes prior to the start of the call. A replay of the call will be available for seven days beginning at 11:30 a.m. ET on March 1, 2021. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 6959755.

About Galafold

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

U.S. INDICATIONS AND USAGE

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

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

U.S. IMPORTANT SAFETY INFORMATION

ADVERSE REACTIONS

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

USE IN SPECIFIC POPULATIONS

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

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

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

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

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

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

EU Important Safety Information

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

- Galafold is not intended for concomitant use with enzyme replacement therapy.
- Galafold is not recommended for use in patients with Fabry disease who have severe renal impairment (<30 mL/min/1.73 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

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

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, and revenue goals, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations and/or revenue from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product or to treatment sites. In addition to the impact of the COVID-19 pandemic, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, UK, Japan, the US and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies, commercialization and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding corporate financial guidance and financial goals and the attainment of such goals and statements regarding projections of the Company's revenue and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2020 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.

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TABLE 1

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

	Years Ended December 31,		
	2020	**2019**	**2018**
Net product sales	$ 260,886	$ 182,237	$ 91,245
Cost of goods sold	31,044	21,963	14,404
Gross profit	229,842	160,274	76,841
Operating expenses:			
Research and development	308,443	286,378	270,902
Selling, general, and administrative	156,407	169,861	127,200
Changes in fair value of contingent consideration payable	3,144	3,297	3,300
Depreciation and amortization	8,846	4,775	4,216
Total operating expenses	476,840	464,311	405,618
Loss from operations	(246,998)	(304,037)	(328,777)
Other (expense) income:			
Interest income	3,226	10,249	10,461
Interest expense	(22,425)	(18,872)	(22,402)
Loss on exchange of convertible notes	—	(40,624)	—
Loss on extinguishment of debt	(7,276)	—	—
Change in fair value of derivatives	—	—	(2,739)
Other expense	(781)	(2,626)	(5,632)
Loss before income tax	(274,254)	(355,910)	(349,089)
Income tax (expense) benefit	(2,598)	(478)	94
Net loss attributable to common stockholders	$ (276,852)	$ (356,388)	$ (348,995)
Net loss attributable to common stockholders per common share — basic and diluted	$ (1.07)	$ (1.48)	$ (1.88)
Weighted-average common shares outstanding — basic and diluted	258,867,380	240,421,001	185,790,021

TABLE 2

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

	December 31,	
	2020	**2019**
Assets		
Current assets:		
Cash and cash equivalents	$ 163,240	$ 142,837
Investments in marketable securities	320,029	309,903
Accounts receivable	46,923	33,284
Inventories	19,556	14,041
Prepaid expenses and other current assets	29,721	20,008
Total current assets	579,469	520,073
Operating lease right-of-use assets, less accumulated amortization of $7,574 and $5,342 at December 31, 2020 and December 31, 2019, respectively	23,296	33,315
Property and equipment, less accumulated depreciation of $14,487 and $17,604 at December 31, 2020 and December 31, 2019, respectively	43,863	47,705
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	19,095	28,317
Total Assets	$ 886,520	$ 850,207
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 17,063	$ 21,722
Accrued expenses and other current liabilities	96,841	99,901
Contingent consideration payable	8,900	—
Operating lease liabilities	6,872	7,189
Total current liabilities	129,676	128,812
Deferred reimbursements	7,406	8,906
Long-term debt	389,254	149,505
Contingent consideration payable	16,925	22,681
Deferred income taxes	4,896	5,051
Operating lease liabilities	45,604	53,531
Other non-current liabilities	6,379	5,296
Total liabilities	600,140	373,782
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 shares authorized, 262,063,461 and 255,417,869 shares issued and outstanding at December 31, 2020 and December 31, 2019, respectively	2,650	2,598
Additional paid-in capital	2,308,578	2,227,225
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	8,412	2,785
Unrealized (loss) gain on available-for securities	(185)	40
Warrants	12,387	12,387
Accumulated deficit	(2,045,462)	(1,768,610)
Total stockholders' equity	286,380	476,425
Total Liabilities and Stockholders' Equity	$ 886,520	$ 850,207

TABLE 3

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

	December 31		
	2020	**2019**	**2018**
Total operating expenses - as reported GAAP	**$ 476,840**	**$ 464,311**	**$ 405,618**
Research and development:			
Share-based compensation	20,817	17,575	11,740
Asset acquisition related expenses for in-process R&D	-	-	100,000
Selling, general and administrative:			
Share-based compensation	28,334	26,855	17,520
Changes in fair value of contingent consideration payable	3,144	3,297	3,300
Depreciation and amortization	8,846	4,775	4,216
Total operating expense adjustments to reported GAAP	61,141	52,502	136,776
Total operating expenses - as adjusted	**$ 415,699**	**$ 411,809**	**$ 268,842**

Exhibit 99.2

Amicus Therapeutics®

Full Year 2020 Financial Results Conference Call & Webcast

March 1, 2021

Forward-Looking Statements

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

Non-GAAP Financial Measures

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

Rare Disease Day® 2021
Remembering Rossella

A RARE COMPANY

A leading fully integrated, global rare disease biotechnology company

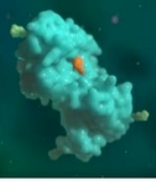
Galafold® (migalastat)
First Oral Precision Medicine for Fabry Disease

Gene Therapy PLATFORM
Protein Engineering & Glycobiology

World Class BIOLOGICS Capabilities

EMPLOYEES in 27 Countries

AT-GAA
Phase 3 in Pompe Disease



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

$483.3M Cash as of 12/31/20

Two Clinical-Stage Gene Therapies

GLOBAL COMMERCIAL ORGANIZATION

2021 Key Strategic Priorities

1. Achieve double-digit Galafold growth and revenue $300M to $315M
2. Report data from the AT-GAA Phase 3 PROPEL study and complete BLA and MAA filings for regulatory approvals
3. Advance clinical studies, regulatory discussions and scientific data across industry leading gene therapy pipeline
4. Further manufacturing capabilities and capacity to build world-class technical operations to support all gene therapy programs
5. Maintain strong financial position

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 December 31, 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 ERT

One of the Most Successful Rare Disease Launches

- $260.9M FY20 Galafold Revenue
- $300M-$315M FY21 Global Galafold Revenue
- 1,384 Amenable Mutations Included in the EU Label
- 348 Amenable Variants in U.S. Label
- Continued Expansion in 2021
- 40+ Countries with Regulatory Approvals: including U.S., EU, Japan, and other Countries

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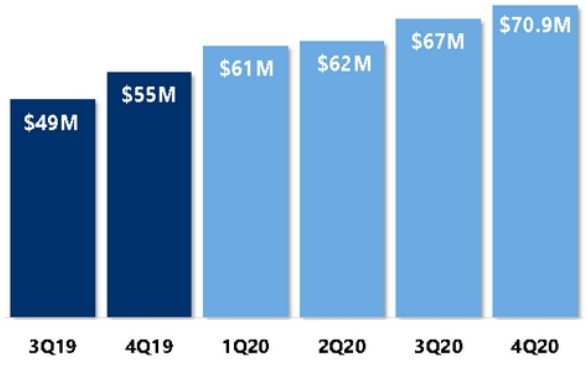
2020 Galafold Success

Growth remains strong with Q4 revenue of $70.9M and FY2020 revenue of $260.9M



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Galafold Global Commercial Momentum

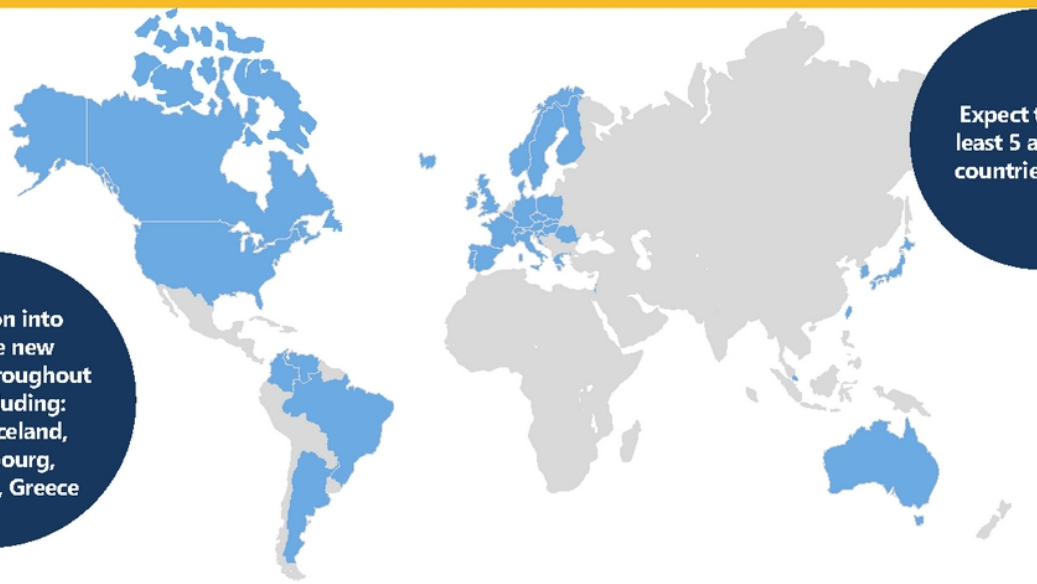
Strong global demand supported by high compliance and adherence rates laying foundation for continued growth anticipated in 2021

FY20 Strength Reflects Continued Strength with 1,400+ Treated Patients

- Strong patient demand and several new launch countries anticipated in 2021
- Global mix of switch (60%) and previously untreated patients (40%)
- Continued growing prescriber base in the U.S. to nearly 200 physicians
- Continue to support diagnostic initiatives to drive a shorter pathway to diagnosis

Galafold Geographic Growth

Regulatory approval in 40+ countries and commercial sales in 30+ of those today



Expansion into multiple new markets throughout 2020 including: Poland, Iceland, Luxembourg, Argentina, Greece

Expect to add at least 5 additional countries in 2021

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Outlook for 2021

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

- 2020 execution lays a solid foundation and global demand remains strong with continued growth anticipated in 2021 and beyond
- New Galafold patient additions slowed in Q4 due to COVID reemergence and resulting in increased lag time between patient identification and treatment initiation
- In 2021, project double-digit revenue growth with net new patient starts expected to be greater than in 2020
- Expect higher patient adds and revenue growth in the second half of 2021 as COVID impact eases
- Continue to see >90% compliance and adherence rates globally

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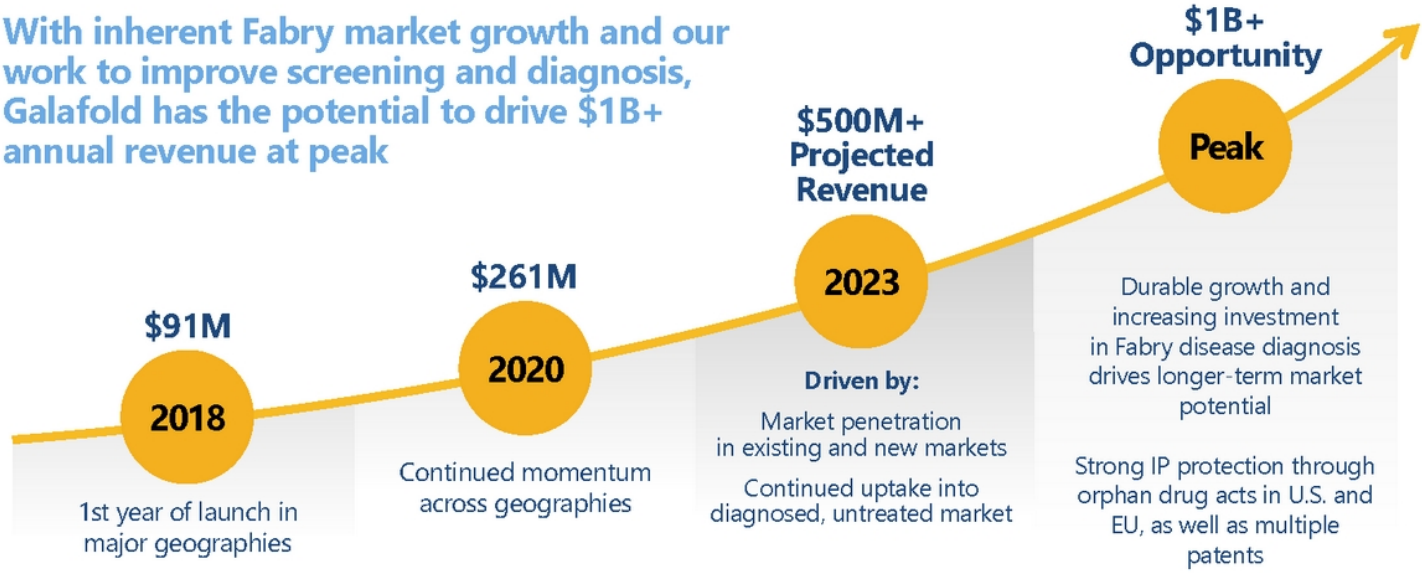
Galafold Opportunity

With inherent Fabry market growth and our work to improve screening and diagnosis, Galafold has the potential to drive $1B+ annual revenue at peak



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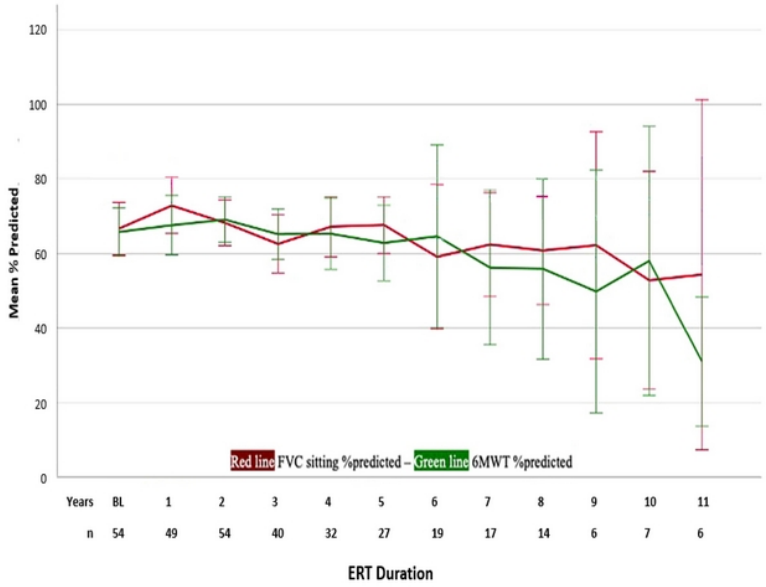
AT-GAA: Next Potential Standard of Care for Pompe Disease

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

Unmet Need in Pompe Disease Today

Natural history studies and publications in Pompe disease continue to highlight the unmet need and the continued decline on key measures of disease



"There is an initial positive effect on the most important outcome measures, however, a more limited long-term benefit of stabilization of the clinical course under ERT for many patients over a long period. Though, according to our data, this long-term therapeutic efficacy is weakest for the lung capacity and consecutively the need of additional ventilatory support over time. As respiratory insufficiency is the most frequent cause of death in Pompe disease, it is important to further improve this organ function in particular. The fast approval of novel therapeutic options is a great unmet need for Pompe patients."

Modified from Goodschmidt et. al., 2021, *STIG study: real-world data of long-term outcomes of adults with Pompe disease under enzyme replacement therapy with alglucosidase alfa*

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PROPEL Topline Results:
Overall Population (n=122)

PROPEL

6MWD showed greater improvement with AT-GAA versus alglucosidase alfa but did not demonstrate statistical superiority; FVC demonstrated clinically significant improvement with AT-GAA over alglucosidase alfa

6MWD (m)

Treatment	Baseline	CFBL at Week 52	Difference	P-Value
AT-GAA (n=85)	357.9 (111.8)	+20.8 (4.6)	+13.6 (8.3)	p=0.072
Alglucosidase alfa (n=37)	351.0 (121.3)	+7.2 (6.6)		

FVC (% predicted)

Treatment	Baseline	CFBL at Week 52	Difference	P-Value
AT-GAA (n=85)	70.7 (19.6)	-0.9 (0.7)	+3.0 (1.2)	**p=0.023**
Alglucosidase alfa (n=37)	69.7 (21.5)	-4.0 (0.8)		

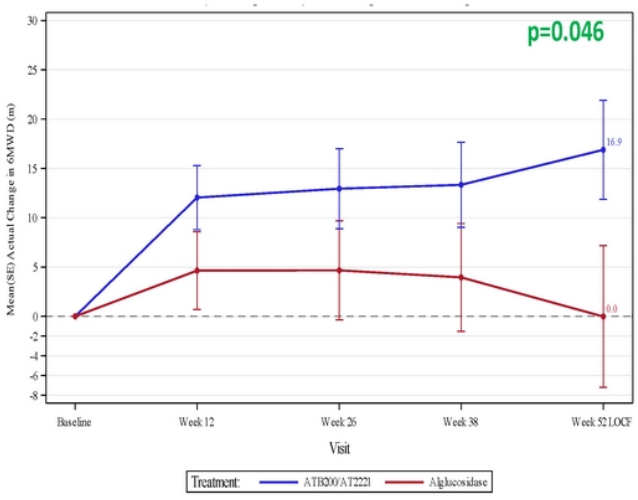
NOTES: Baseline is Mean (STDEV); CFBL is Mean LOCF (SE); P-values are nominal 2-sided; FVC data normally distributed and p–values are from ANCOVA.
Results exclude one clinically implausible patient who used an investigational anabolic steroid ostarine (selective androgen receptor modulator) just prior to study start.
6MWD data not normally distributed and 6MWD p-value is for non-parametric ANCOVA; 6MWD parametric MMRM p-value was p=0.097.

PROPEL Topline Results
ERT Experienced Population (n=95)

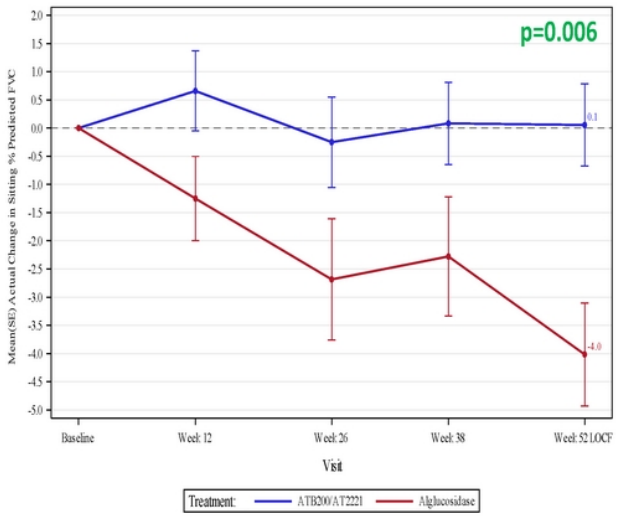
PROPEL

ERT experienced patients treated with AT-GAA demonstrated improvements over time in 6MWD and stabilization over time in FVC versus alglucosidase alfa

6MWD (m): Change from baseline (n=65, n=30)



FVC (% predicted): Change from baseline (n=65, n=30)



NOTE: Baseline is Mean (STDEV); CFBL is Mean (SE); P-values are nominal 2-sided; FVC data normally distributed and p–values are from ANCOVA
6MWD data not normally distributed and 6MWD p-value is for non-parametric ANCOVA; 6MWD parametric MMRM p-value was p=0.078

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Primary, Key Secondary and Biomarker Endpoint Heat Map

All Patients & ERT Experienced Patients

Endpoints across motor function, pulmonary function, muscle strength, PROs and biomarkers favored AT-GAA over alglucosidase alfa in both the overall and ERT experienced populations

Overall Population

	Alglucosidase alfa	AT-GAA
Motor Function		6MWD
		GSGC*
Pulmonary Function		FVC*
Muscle Strength		Lower MMT
PROs		PROMIS-Physical
		PROMIS-Fatigue
Biomarker		Hex4*
		CK*

ERT Experienced Population

	Alglucosidase alfa	AT-GAA
Motor Function		6MWD*
		GSGC*
Pulmonary Function		FVC*
Muscle Strength		Lower MMT
PROs		PROMIS-Physical
		PROMIS-Fatigue
Biomarker		Hex4*
		CK*

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

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Post Hoc Non-Inferiority Analyses

Post hoc non-inferiority analyses of 6MWD and FVC are highly statistically significant

Parameter	Non-Inferiority Margin	1-sided p-value
6MWD	15 meters	0.0004
	10 meters	0.0026
	5 meters	0.0127
	3 meters	0.0224
% predicted FVC	1.1%	0.0008

NOTE1: 6MWD results are based on MMRM analysis using observed cases and FV results are based on ANCOVA using LOCF.
NOTE2: As Lumizyme did not show statistical significance versus placebo for 6MWD, the NI margins were not statistically justified.
Results exclude one clinically implausible patient who used an investigational anabolic steroid ostarine (selective androgen receptor modulator) just prior to study start.

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AT-GAA: Key Takeaways

- Rolling BLA submission expected to complete in Q2
- Other key regulatory submissions for approval throughout 2021 including MAA in Europe
- Potential for early approval under EAMS framework with Priority Innovative Medicines Designation in UK
- 150+ patients worldwide now being treated with AT-GAA including adults, adolescents and infants
- Pediatric study for Pompe patients aged 12 to <18 with late-onset Pompe disease ongoing
- Clinical study for Pompe patients with infantile onset disease expected to begin this year
- Expanded access program for Pompe infantile patients and adult-onset patients open and has enrolled multiple patients with Pompe. Further expanded access for all Pompe patients being considered.

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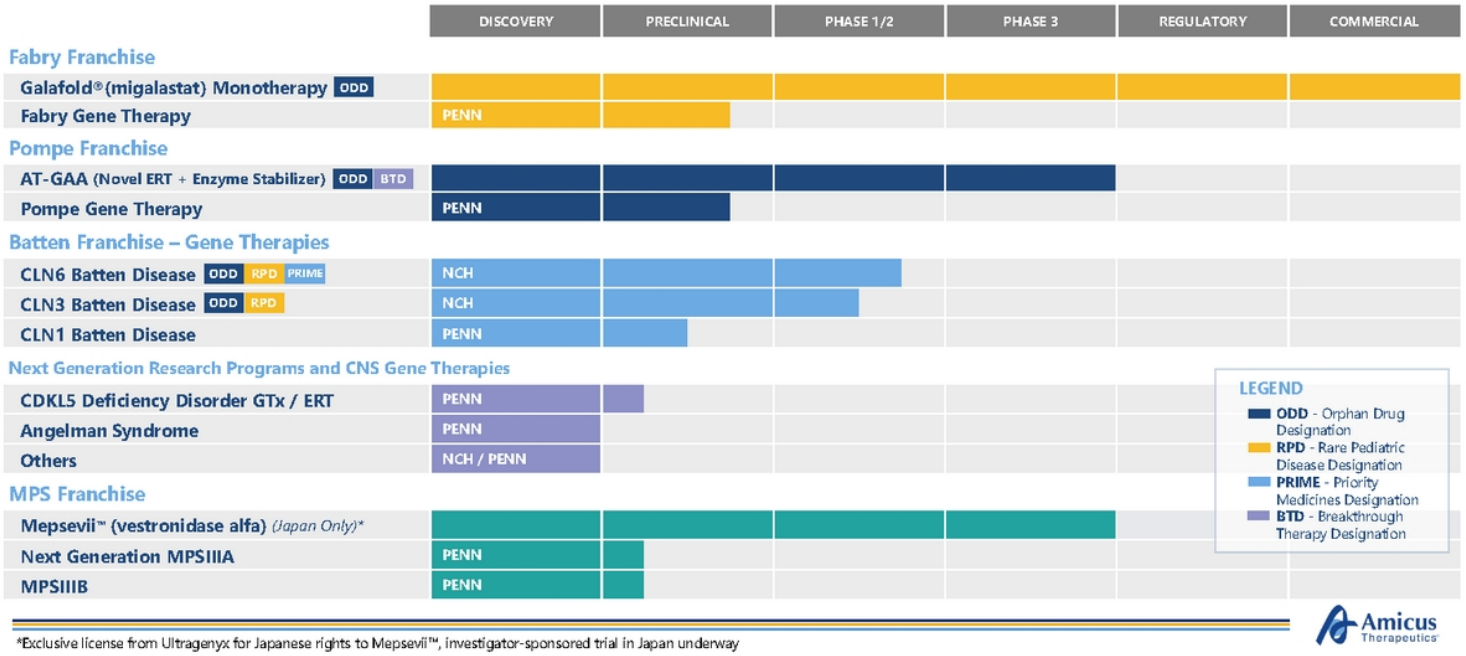
Next Generation Gene Therapy Platform

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

A RARE PORTFOLIO



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

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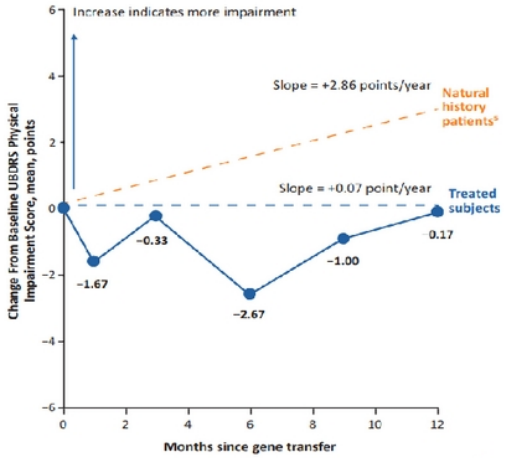
CLN3 Batten Disease Gene Therapy
AT-GTX-502

Early clinical data suggest early signs of disease stabilization compared to natural history; plan to submit IND for next clinical study in 2H2021

- Batten disease is a group of disorders known as neuronal ceroid lipofuscinoses (NCLs)
- Mutation in one of 13 different CLN genes leads to neuronal lysosomal dysfunction
- CLN3 Batten disease is one of the most common neurodegenerative disorders affecting children leading to blindness, motor impairment, learning difficulties, epilepsy and, ultimately, premature death
- UBDRS-physical is the key efficacy endpoint for this study in CLN3 Batten assessing gross and fine motor function, vision and speech

Low-Dose Cohort (n=3) Compared with Patients in the Natural History Study (n=82)



UBDRS=Unified Batten Disease Rating Scale. *Scheduled month 12 in-person UBDRS assessment was impacted by the COVID-19 pandemic.

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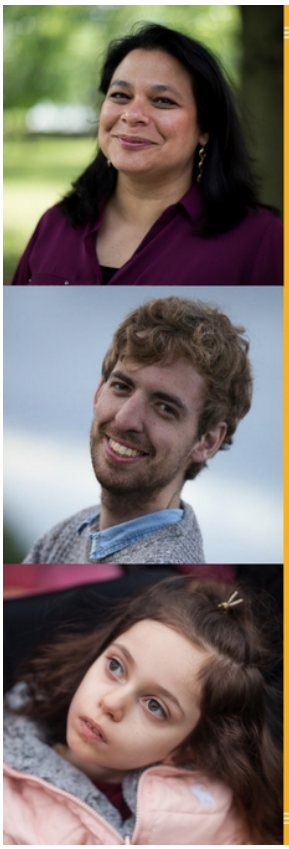

Financial Summary

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

2020 Select Financial Results

2020 revenue of $260.9M from global Galafold sales

(in thousands, except per share data)	Dec. 31, 2020	Dec. 31, 2019
Product Revenue	$260,886	$182,237
Cost of Goods Sold	31,044	21,963
R&D Expense	308,443	286,378
SG&A Expense	156,407	169,861
Changes in Fair Value of Contingent Consideration	3,144	3,297
Depreciation and Amortization	8,846	4,775
Loss from Operations	(246,998)	(304,037)
Loss on Extinguishment of Debt	(7,276)	-
Income Tax (Expense) Benefit	(2,598)	(478)
Net Loss	(276,852)	(356,388)
Net Loss Per Share	(1.07)	(1.48)

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Financial Outlook: Key Takeaways

- Galafold revenue in 2020 was $260.9 million, exceeding the Company's guidance
- 2020 Non-GAAP Operating Expenses of $415.7M in line with guidance of $410 million to $420 million
- Non-GAAP operating expense guidance for 2021 is expected to remain flat at $410 million to $420 million
  - Driven by disciplined expense management and continued investment in the global Galafold launch, AT-GAA clinical studies and advancing our gene therapy pipeline
- Current cash position is sufficient to achieve self-sustainability without the need for future dilutive financing

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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

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Appendix

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Reconciliation

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

	December 31		
	2020	2019	2018
Total operating expenses - as reported GAAP	**$ 476,840**	**$ 464,311**	**$ 405,618**
Research and development:			
Share-based compensation	20,817	17,575	11,740
Asset acquisition related expenses for in-process R&D	-	-	100,000
Selling, general and administrative:			
Share-based compensation	28,334	26,855	17,520
Changes in fair value of contingent consideration payable	3,144	3,297	3,300
Depreciation and amortization	8,846	4,775	4,216
Total operating expense adjustments to reported GAAP	61,141	52,502	136,776
Total operating expenses - as adjusted	**$ 415,699**	**$ 411,809**	**$ 268,842**