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): **January 10, 2022**

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.)**

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 January 10, 2022, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing preliminary 2021 revenue and its 2022 strategic outlook, along with various business updates. A copy of the press release is filed as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

The information furnished pursuant to this Item 2.02, including Exhibit 99.1, 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 liabilities of that section, and shall not be 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 8.01 – Other Events

On January 10, 2022, the Company also published presentation materials which senior management will be using in its meetings with investors and analysts at the 40th Annual J.P. Morgan Healthcare Conference. A copy of these materials is filed as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits:

Exhibit No.	Description
99.1	Press Release dated January 10, 2022
99.2	Presentation Materials – 40th Annual J.P. Morgan Healthcare Conference
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.

AMICUS THERAPEUTICS, INC.

Date: January 10, 2022

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

Exhibit 99.1

Amicus Therapeutics Reports Preliminary 2021 Revenue and Provides 2022 Strategic Outlook and Revenue Guidance

Full-Year 2021 Galafold® Revenue of ~$306M, Representing 17% YoY Growth

Expect Double-Digit Growth (15-20%) of 2022 Galafold Revenue with $350M-$365M in Global Sales

U.S. and EU Regulatory Reviews Underway for AT-GAA in Pompe Disease

AT-GAA Global Launch Preparations Accelerating

Cash Flow and Balance Sheet Sufficient to Achieve Self-Sustainability and Profitability by 2023

PHILADELPHIA, PA, January 10, 2022 – Amicus Therapeutics (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on developing and commercializing novel medicines for rare diseases, today provided its preliminary and unaudited 2021 revenue, corporate updates, and full-year 2022 outlook and revenue guidance.

Corporate Highlights:

- **Global revenue for Galafold® (migalastat) in 2021 reached $306 million driven by strong new patient accruals and sustained patient adherence, representing a year-over-year increase of 17%.**
- **AT-GAA regulatory reviews are underway:** In the U.S., the Food and Drug Administration (FDA) accepted for review the Biologics License Application (BLA) for cipaglucosidase alfa and the New Drug Application (NDA) for miglustat, the two components of AT-GAA. The FDA has set a Prescription Drug User Fee Act (PDUFA) action date of May 29, 2022 for the NDA and July 29, 2022 for the BLA. In the EU, the Marketing Authorization Applications (MAA) were submitted and validated in the fourth quarter by the European Medicines Agency (EMA).
- **AT-GAA launch preparations are accelerating:** Development of global launch plans, targeted investments in additional personnel, and launch inventory are fully underway as company believes AT-GAA can rapidly become the new standard of care treatment regimen for people living with Pompe disease.
- **Pipeline of next generation genetic medicines to advance through both internal efforts and creation of R&D focused new company, Caritas Therapeutics.**
- **Cash Flow and Balance Sheet sufficient to achieve self-sustainability and profitability in 2023.** Through careful management of expenses, the Company is on the path to achieve self-sustainability and profitability in 2023 as it executes on the global Galafold expansion and prepares for AT-GAA global launch.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “In 2021, Amicus made great strides for people worldwide living with rare diseases through the broad execution of our annual strategic priorities. Despite the resurgence of COVID with Delta and Omicron variants, the Galafold business remains very strong, and we delivered on our full year revenue guidance and expect robust growth this year driven by strong adoption across the globe for our Fabry disease precision medicine. We are underway with the global regulatory reviews and launch preparations for AT-GAA in Pompe disease with high expectations that this novel medicine has the potential to become the new standard of care in Pompe disease treatment and the potential to address unmet needs for thousands of Pompe patients in the years ahead. We see further opportunity ahead to impact the lives of those living with rare disease through our genetic medicine business and capabilities. Together, Amicus is in a stronger position than ever and we remain steadfast on our mission of transforming the lives of people living with rare, life-threatening conditions and creating significant value for our shareholders.”

Bradley Campbell, President and Chief Operating Officer of Amicus Therapeutics, Inc., stated, “We are looking ahead to transforming Amicus into a leading global rare disease biotechnology company led by two innovative therapies that we believe meaningfully impact the lives of people living with Fabry and Pompe disease. This year we will be focused on continuing to bring Galafold to patients around the world and delivering on the anticipated approval and launch of AT-GAA.”

Amicus is focused on the following five key strategic priorities in 2022:

- Continued double-digit Galafold growth (15-20%) with revenue of $350M to $365M
- Secure FDA approval and positive CHMP opinion for AT-GAA
- Initiate successful, rapid launch in the U.S. for AT-GAA
- Advance best-in-class next generation genetic medicines and capabilities
- Maintain strong financial position on path to profitability

Mr. Crowley and Mr. Campbell will discuss the Amicus corporate objectives and key milestones in a presentation at the 40th Annual J.P. Morgan Healthcare Conference on Wednesday, January 12, 2022, at 3:45 p.m. ET. A live webcast of the presentation can be accessed through the Investors section of the Amicus Therapeutics corporate website at http://ir.amicusrx.com/events.cfm, and will be archived for 90 days.

Full-Year 2021 Revenue Summary and 2022 Revenue Guidance

Global revenue for Galafold in full-year 2021 was approximately $306 million, preliminary and unaudited, representing a year-over-year increase of 17% from total revenue of $260.9 million in 2020. Full-year revenue benefited from a positive currency impact of approximately $7 million. Fourth quarter Galafold revenue was approximately $84 million, preliminary and unaudited.

For the full-year 2022, the Company anticipates total Galafold revenue of $350 million to $365 million. Double-digit revenue growth (15-20%) in 2022 is expected to be driven by continued underlying demand from both switch and naïve patients, geographic expansion, the continued diagnosis of new Fabry patients and commercial execution across all major markets, including the U.S., EU, U.K., and Japan.

The current cash position is sufficient to achieve self-sustainability and profitability in 2023.

Updates and Anticipated Milestones by Program

Galafold (migalastat) Oral Precision Medicine for Fabry Disease

- Sustain double-digit revenue growth in 2022 of $350 million to $365 million
- Continue geographic expansion
- Registry and other Phase 4 studies ongoing

AT-GAA for Pompe Disease

- U.S. Prescription Drug User Fee Act (PDUFA) action date of May 29, 2022 for the NDA and July 29, 2022 for the BLA
- EU Committee for Medicinal Products for Human Use (CHMP) opinion expected in late 2022
- Continue to broaden access through early access plans in the U.K., Germany, Japan, and other countries
- Ongoing supportive studies, including pediatric and extension studies

Gene Therapy Pipeline

- Advance IND-enabling studies, manufacturing activities, and regulatory activities for the Fabry disease gene therapy program towards an anticipated IND in 2023
- Progress preclinical studies, manufacturing activities, and regulatory activities for the Pompe disease gene therapy program
- Discontinue CLN6 Batten disease gene therapy program following review of long-term extension study data. It was recently determined that any initial stabilization of disease progression at the two-year time point was not maintained through the long-term extension study. Amicus plans to further analyze and share the Phase 1/2 data with key stakeholders in the CLN6 Batten disease community and work with the community to support continued research efforts to find better treatments and cures which are so desperately and urgently needed
- Advance CLN3 Batten disease program with the higher dose, different promoter, and intra-cisterna magna (ICM) route of delivery pending further Phase 1/2 clinical data and pre-clinical data expected in 2022. These data will inform timeline for commencement of any pivotal clinical study

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 m2). The safety and efficacy of Galafold in children less than 12 years of age have not yet been established. No data are available.
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.
- While taking Galafold, effective birth control should be used. It is not known whether Galafold is excreted in human milk.
- Contraindications to Galafold include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- Galafold 123 mg capsules are not for children (≥12 years) weighing less than 45 kg.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on Galafold or switched to Galafold.
- OVERDOSE: General medical care is recommended in the case of Galafold overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received Galafold. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

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

About Fabry Disease

Fabry disease is an inherited lysosomal disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A), which results from mutations in the GLA gene. The primary biological function of alpha-Gal A is to degrade specific lipids in lysosomes, including globotriaosylceramide (referred to here as GL-3 and also known as Gb3). Lipids that can be degraded by the action of alpha-Gal A are called "substrates" of the enzyme. Reduced or absent levels of alpha-Gal A activity lead to the accumulation of GL-3 in the affected tissues, including heart, kidneys, and skin. Accumulation of GL-3 and progressive deterioration of organ function is believed to lead to the morbidity and mortality of Fabry disease. The symptoms can be severe, differ from person to person, and begin at an early age.

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 us on Twitter and LinkedIn.

Forward Looking Statement

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product. In addition to the impact of the COVID-19 pandemic, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. Statements regarding corporate financial guidance and financial goals and the attainment of such goals. With respect to statements regarding projections of the Company's revenue and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2020 and the Quarterly Report filed on Form 10-Q for the quarter ended September 30, 2021. 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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Amicus Therapeutics®

40th Annual J.P. Morgan Healthcare Conference

At the Forefront of Therapies for Rare Diseases

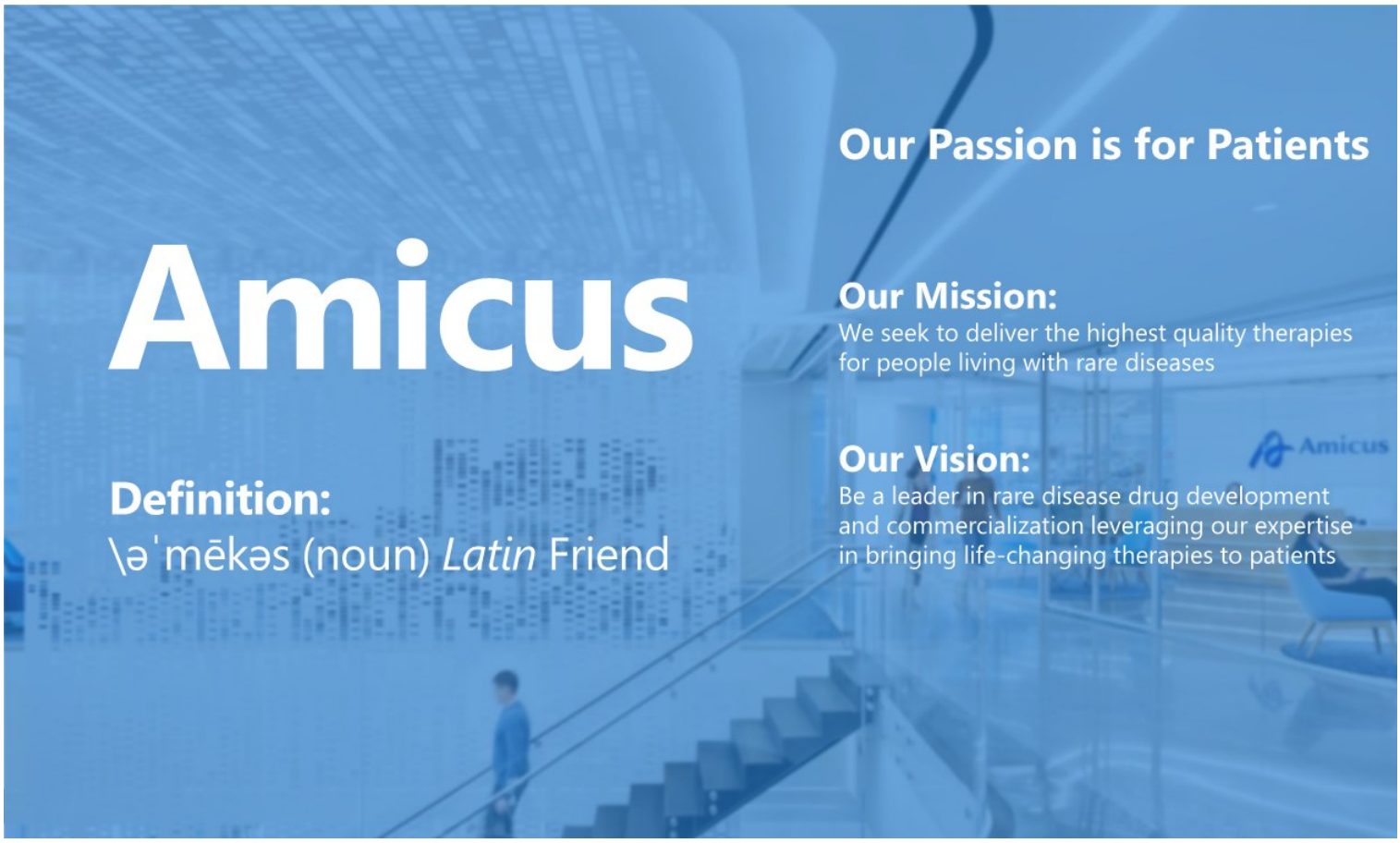
January 12, 2022

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 prod 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 candida commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements sho 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 affec 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 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 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 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 imp 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-pa 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 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, with 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 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 successful in commercializing Galafold in Europe, U.K., Japan, the U.S. and other geographies or our other product candidates if and when approved; the potential that preclinical and clini 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 produ 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 tr 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 regard 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, forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2020, and on Form 10-Q for the quarter ended Septem 30, 2021. 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 th 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 w supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financ 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 cert 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 th 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-GA 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 t 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 potentia unpredictable, impact on our future GAAP results.


Our Passion is for Patients
Amicus
Our Mission:
We seek to deliver the highest quality therapies for people living with rare diseases
Our Vision:
Be a leader in rare disease drug development and commercialization leveraging our expertise in bringing life-changing therapies to patients
Amicus
Definition:
\əˈmēkəs (noun) Latin Friend

A Rare Company

Patient Dedicated, Rare Disease Biotechnology Company with Sustained Double-Digit Revenue Growth, a Global Commercial Infrastructure, and Late-stage Development Capabilities

Galafold® (migalastat)

First Oral Precision Medicine for Fabry Disease

Gene Therapy PLATFORM

Protein Engineering & Glycobiology

World-Class CLINICAL DEVELOPME Capabilities

EMPLOYEES in 27 Countries

GLOBAL COMMERCIAL ORGANIZATION

AT-GAA

a Two-Component Therapy under **Regulatory Review** for **Pompe Disease**

$557M

Cash

as of 9/30/21

Robust R&D Engi

Nearly 50+ Lysosomal Disorders and More Prevale Rare Diseases

2021 Strategic Priorities Accomplished:
Setting the Stage for a Successful 2022

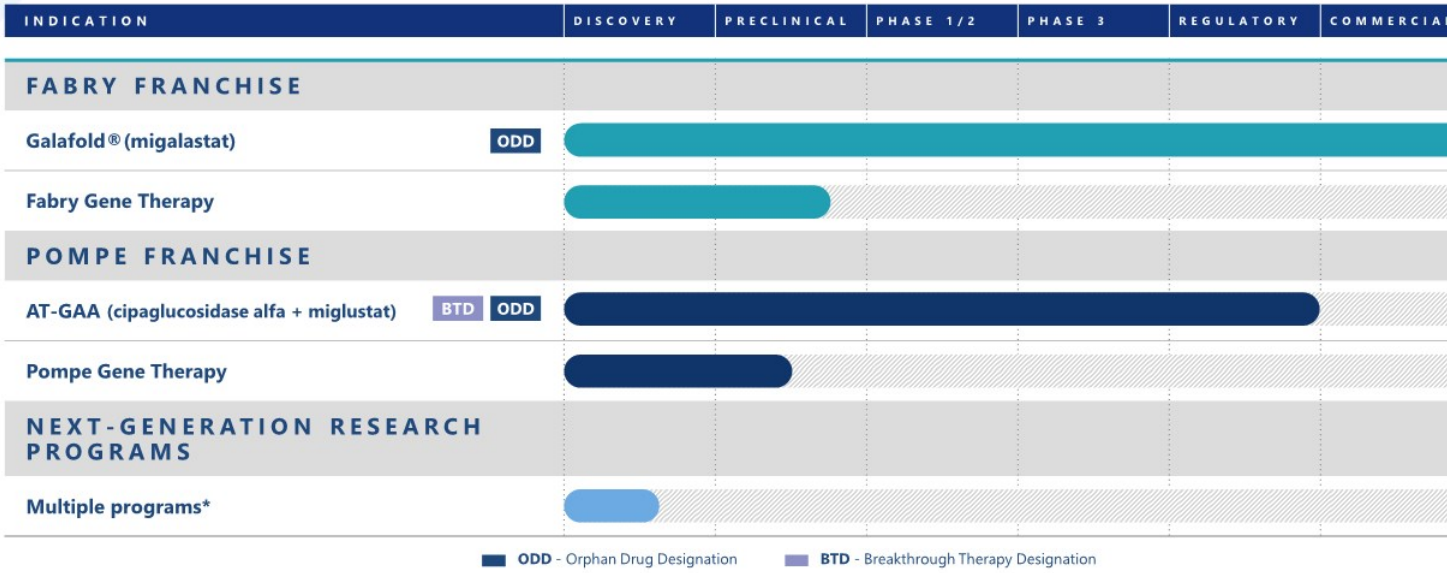
1. Achieve double-digit Galafold growth and revenue of $300M to $315M ✓
2. Report data from the AT-GAA Phase 3 PROPEL study and complete BLA and MAA filings for regulatory approvals ✓
3. Advance clinical studies, regulatory discussions, and scientific data across industry leading gene therapy pipeline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

2022 Strategic Priorities to Drive Value

1. Continued double-digit Galafold growth (15-20%) with revenue of $350M to $365M
2. Secure FDA approval and positive CHMP opinion for AT-GAA
3. Initiate successful, rapid launch in U.S. for AT-GAA
4. Advance best-in-class next-generation genetic medicines and capabilities
5. Maintain strong financial position on path to profitability

Amicus Pipeline

Streamlined Rare Disease Pipeline with Focus on Fabry and Pompe, including Shared Gene Thera Programs with Caritas, and Optionality to Future Programs



*Includes additional discovery programs and right of first negotiation to certain muscular dystrophy programs that are intended to be developed by Caritas

Positioned for Significant Value Growth

Focused on Execution and Driving Sustainable Double-Digit Revenue Growth on Path to Profitability

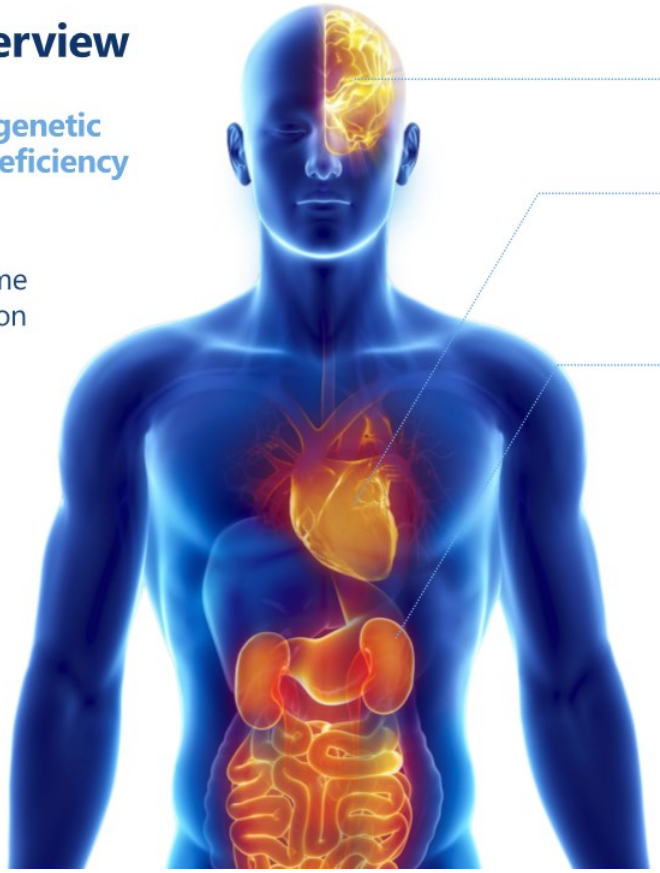
- Continue to bring Galafold to as many patients as possible, sustain double digit revenue growth
- Successful launch of AT-GAA for people living with Pompe disease
- Advance next-generation gene therapies in Fabry and Pompe diseases
- Fully leverage global capabilities and infrastructure as a leader in rare diseases
- Achieve self-sustainability and profitability in 2023

Amicus Therapeutics®

Fabry Disease Overview

Fabry is a rare inherited genetic disorder caused by the deficiency of the GLA enzyme

- Deficiency of α-Gal A enzyme leading to GL-3 accumulation
- 1,000+ known mutations
- 13,000+ diagnosed WW (51% female/49% male[4])



Leading Causes of Death

TRANSIENT ISCHEMIC ATTACK (TIA) & STROKE[1]

HEART DISEASE[2]
- Irregular heartbeat (fast or slow)
- Heart attack or heart failure
- Enlarged heart

KIDNEY DISEASE[3]
- Protein in the urine
- Decreased kidney function
- Kidney failure

Life-Limiting Symptoms

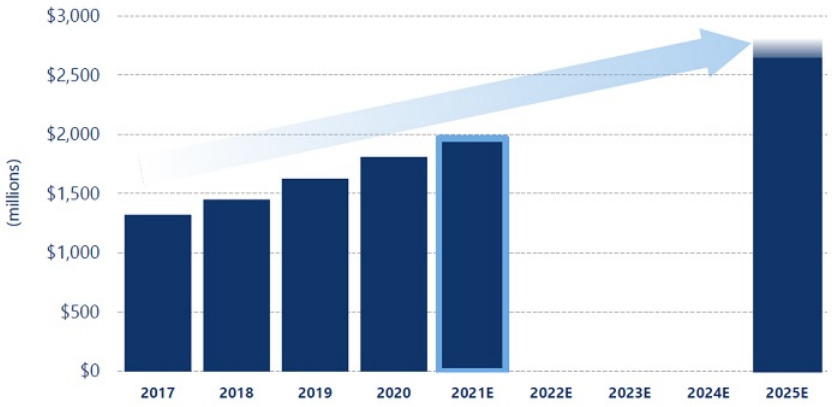
GASTROINTESTINAL[3]
- Nausea, vomiting, cramping, diarrhea
- Pain/bloating after eating, feeling full
- Constipation
- Difficulty managing weight

1. Desnick R, *et al.* Ann Intern Med. 2003
2. Yousef Z, *et al.* Eur Heart J. 2013
3. Germain D. Orphanet J Rare Dis. 2010
4. Fabry Registry 2011

Global Fabry Market

Global Fabry Disease Market Growth Continues to be Driven by Diagnosing New Patients in Addition to the Introduction of Galafold

Global Fabry market to exceed $1.9B in 2021 and tracking toward ~$2.6B by 2025[1]



- Fabry Disease is believed to be significantly underdiagnosed
  - Newborn screening studies suggest Fabry could be one of the more prevalent human genetic diseases (~1:1,000 to ~1:4,000)
- In 2021, Galafold was the fastest growing medicine for Fabry disease and the greates contributor to Fabry market growth
  - Introduction of Galafold has led to market expansion with 800+ naive patients diagno and treated for the first time

[1]Global market measured by reported sales of approved therapies for Fabry disease – 2025 sales projected using 8% CAGR

2021 Galafold Success

Building on Galafold's Success and Leveraging Leadership Position to Drive Continued Growth

Galafold is first and only approved oral treatment option with a unique mechanism of action for Fabry patients with amenable variants

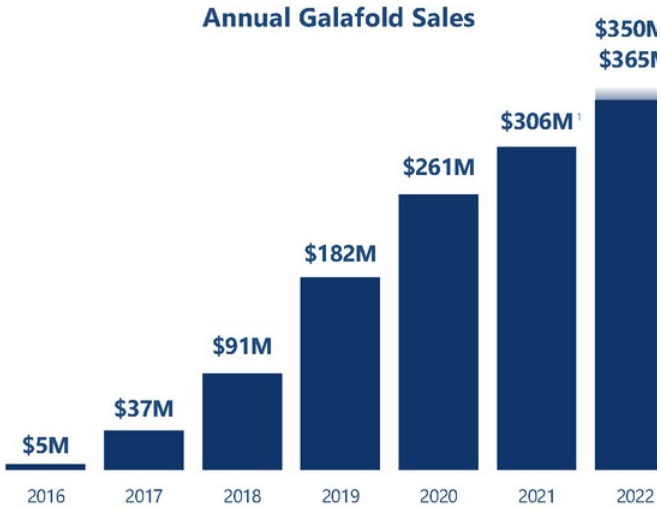
Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including the full 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.

[1]Preliminary and unaudited

Key Performance Indicators Lay the Groundwork for 2022

FY21 Reflects Continued Galafold Strength with 1,750+ Treated Patients as Rate of Net New Patients Accelerating into 2022

- Hybrid business model (virtual/in-person) surpassed pre-COVID physician interactions
- Achieved estimated 49%+ global share of treated amenable patients
- Multiple new markets opened in 2021
- Global mix of switch (~55%) and previously untreated patients (~45%)
- Compliance and adherence over 90%+
- Continue to support diagnostic initiatives to drive a shorter pathway to diagnosis
- Expect non-linear quarterly growth to continue



[1]Preliminary and unaudited

Galafold Growth Opportunity

$1B Annual Sales Opportunity at Peak

Sustained double-digit revenue growth

Grew Galafold sales by +17% in 2021

Near-term growth to $500M driven by:

Continued penetration into existing markets

Expansion into new geographies

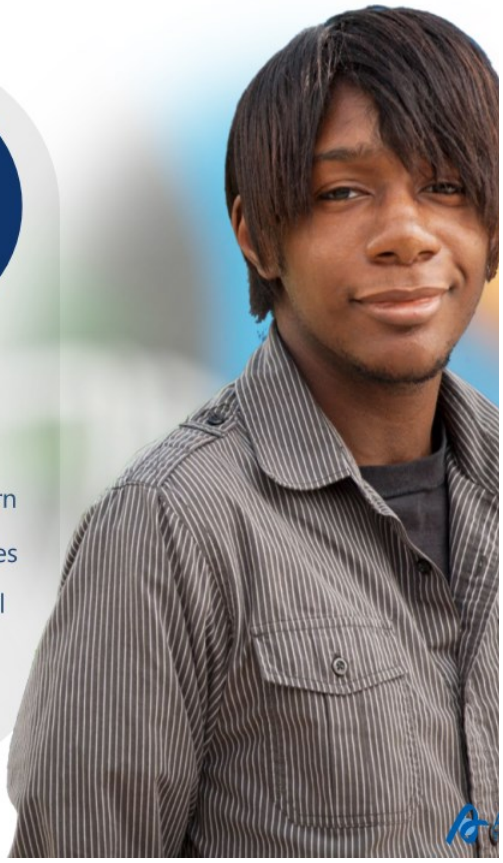
Broadening of labels

Long-term growth towards peak sales potential driven by:

Penetration of diagnosed untreated population

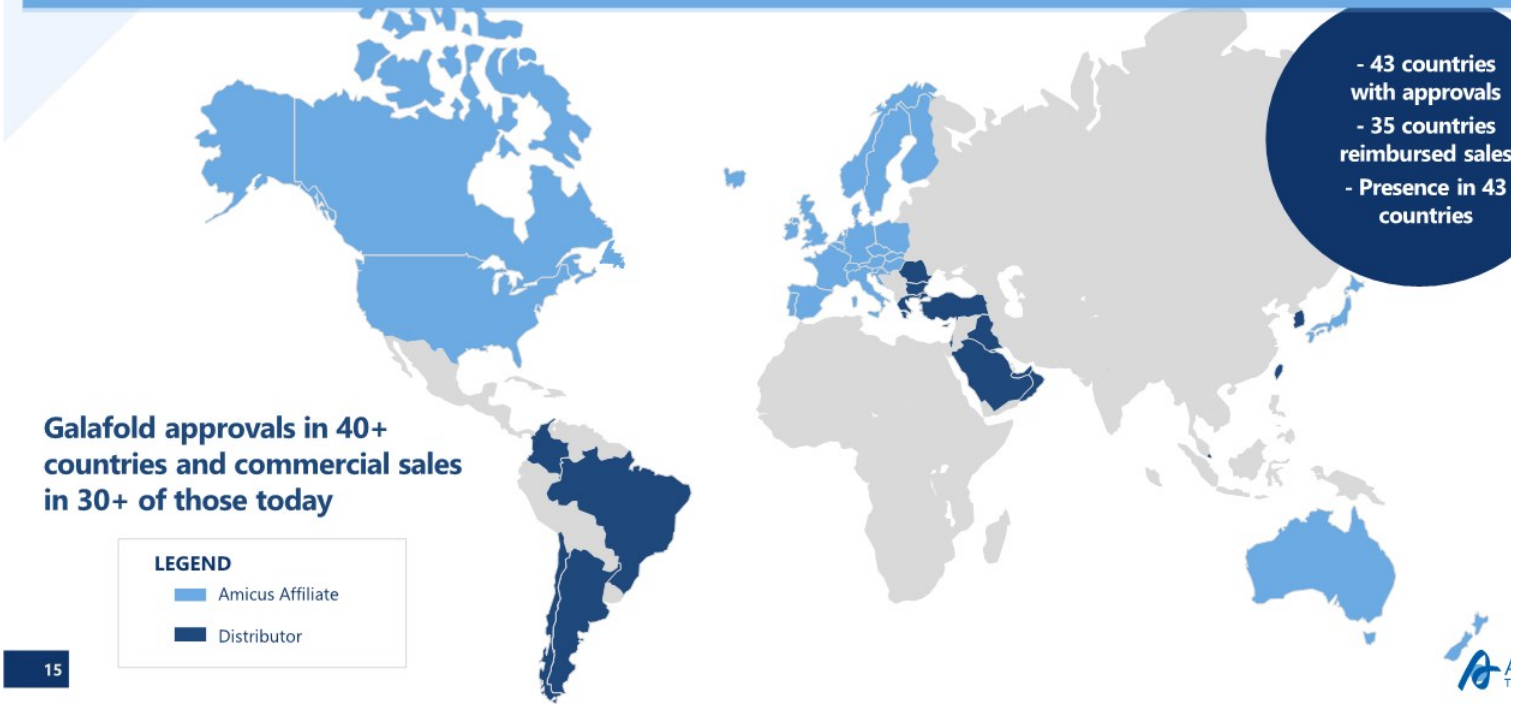
Increase in newborn screening and diagnostic initiatives

Strong intellectual property rights



Experienced Global Commercial Team

Global Commercial Infrastructure Highly Leverageable to Ensure Strong Global Launch of AT-GAA



Amicus Therapeutics®

AT-GAA
(cipaglucosidase alfa + miglustat)

... potential to establish a new standard of care
for people living with Pompe disease

Pompe Disease Overview

Pompe is a Severe and Fatal Neuromuscular Disease Caused by the Deficiency of Lysosomal Enzyme GA

5,000 – 10,000+ patients diagnosed WW[1]; newborn screening suggests significant underdiagnosis

Age of onset ranges from infancy to adulthood

Majority of patients on current standard of care decline after ~2 years

Respiratory and cardiac failure are leading causes of morbidity and mortality

Deficiency of GAA leading to lysosomal glycogen accumulation and cellular dysfunction

Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

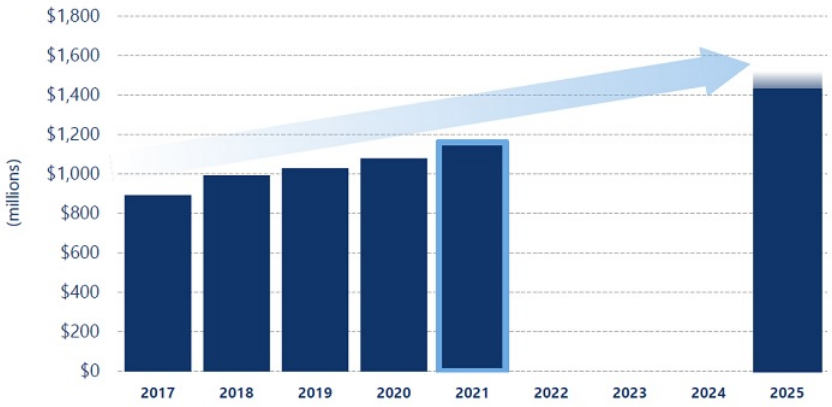
~$1.1B+ global Pompe ERT sales[2]

1. National Institute of Neurological Disorders and Stroke (NIH)
2. Based on 12 months ended September 30, 2021. Source: Sanofi Press Release

Global Pompe Market

Global Pompe Disease Market Growth Continues to be Driven by the Diagnosis of New Patients
Only One Approved Therapy on the Market up until 2021

Global Pompe Market to exceed $1.1B in 2021 and tracking toward $1.5B+ by 2025[1]



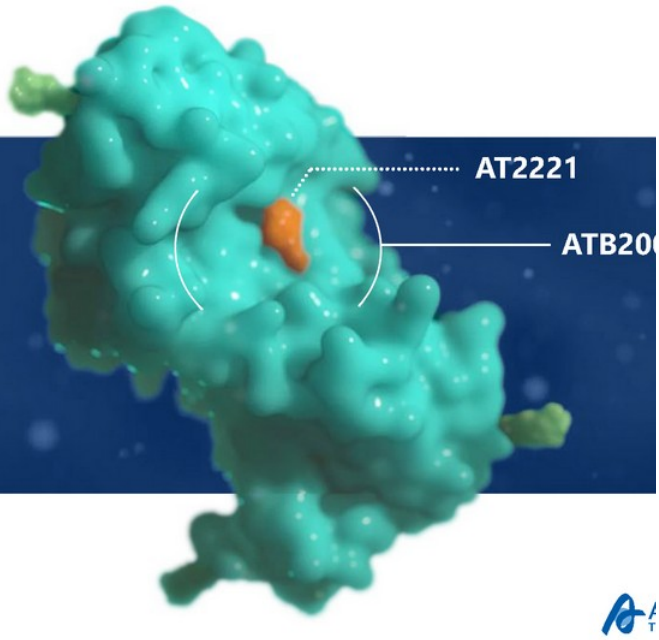
- Pompe Disease believed to be significantly underdiagnosed
  - Newborn screening studies suggests Pompe to be more prevalent than medical literature suggest (~1:10,000 to ~1:30,000)
  - Newborn screening already occurring in 27 U.S. states with 9 additional U.S. states pursuing NBS implementation for Pompe disease

[1]Global market measured by reported sales of approved therapies for Pompe disease – 2025 sales projected using 8% CAGR

AT-GAA: An Innovative Approach to Pompe Disease

Our Scientists Created a Uniquely Glycosylated and Highly Phosphorylated ERT (ATB200) that Significantly Enhances Targeting to Key Affected Muscles

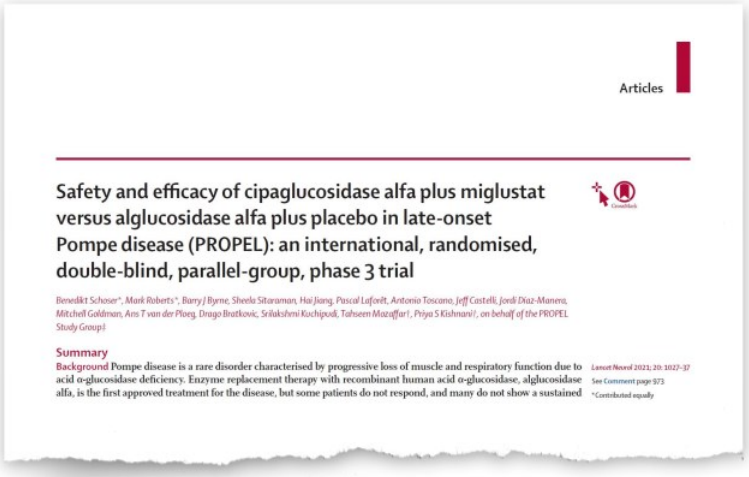
- AT-GAA is a two-component therapy combining ATB200, an ERT, with AT2221, an orally administered enzyme stabilizer
- Consists of a naturally occurring cell line that can be properly processed within the lysosome to its mature form which is required to optimally break down glycogen[1]



[1]Selvan et al. 2021, J Biol Chem 2021 Jan-Jun;296:100769
ERT: Enzyme Replacement Therapy

Phase 3 PROPEL Study

Clinically Meaningful Outcomes from Phase 3 PROPEL Study Provide the Basis for Global Regulatory Submissions of AT-GAA



Articles

Safety and efficacy of cipaglucosidase alfa plus miglustat versus alglucosidase alfa plus placebo in late-onset Pompe disease (PROPEL): an international, randomised, double-blind, parallel-group, phase 3 trial

Benedikt Schoser*, Mark Roberts*, Barry J Byrne, Sheela Sitaraman, Hai Jiang, Pascal Laforêt, Antonio Toscano, Jeff Castelli, Jordi Díaz-Manera, Mitchell Goldman, Ans T van der Ploeg, Drago Bratkovic, Srilakshmi Kuchipudi, Tahseen Mozaffar†, Priya S Kishnani†, on behalf of the PROPEL Study Group‡

Summary

Background Pompe disease is a rare disorder characterised by progressive loss of muscle and respiratory function due to acid α-glucosidase deficiency. Enzyme replacement therapy with recombinant human acid α-glucosidase, alglucosidase alfa, is the first approved treatment for the disease, but some patients do not respond, and many do not show a sustained

Lancet Neurol 2021; 20: 1027–37
See Comment page 973
*Contributed equally

- Peer-reviewed results from PROPEL suggest that treatment with AT-GAA provided clinically meaningful improvements over standard of care, including ERT-experienced patients with high unmet need
- The authors deemed AT-GAA to provide a differentiated mechanism of action and potential alternative treatment option for people living with late-onset Pompe Disease

Schoser et al. 2021, The Lancet Neurology, Volume 20, Issue12, P1027-1037

Phase 3 PROPEL Study Results

Endpoints Across Motor Function, Pulmonary Function, Muscle Strength, Pros, and Biomarkers Favored AT-GAA over Alglucosidase Alfa

		Overall population				ERT-experienced			
	Endpoints	Cipaglucosidase alfa/miglustat n=85		Alglucosidase alfa/placebo n=37		Cipaglucosidase alfa/miglustat n=65		Alglucosidase alfa/placebo n=30	
		Baseline, mean	CFBL at week 52, mean (SE)	Baseline, mean	CFBL at week 52, mean (SE)	Baseline, mean	CFBL at week 52, mean (SE)	Baseline, mean	CFBL at week 52, mean (SE)
Motor function	6MWD, m	357.9	20.8 (4.6)	351.0	7.2 (6.6)	346.9	16.9 (5.0)	334.6	0.0 (7.2)
	GSGC total score	14.5	-0.5 (0.3)	14.5	0.8 (0.3)	15.6	-0.5 (0.3)	15.5	0.6 (0.4)
	10-meter walk, s	9.7	-0.5 (0.6)	9.6	1.9 (1.0)	10.4	-0.6 (0.9)	10.2	2.5 (1.2)
	4-stair climb, s	14.1	-8.5 (7.9)	8.2	0.3 (1.0)	17.3	-11.1 (10.5)	9.3	0.6 (1.2)
	Gower's maneuver, s	10.8	-0.3 (0.7)	19.8	-2.2 (1.4)	11.5	-0.4 (0.8)	23.9	-2.6 (1.9)
	Rising from chair, s	13.6	-10.2 (9.7)	4.5	-0.5 (0.7)	17.6	-13.7 (13.0)	5.2	-0.4 (0.9)
Pulmonary function	FVC, % predicted	70.7	-0.9 (0.7)	69.7	-4.0 (0.8)	67.9	0.1 (0.7)	67.5	-4.0 (0.9)
	MIP, % predicted	61.8	2.1 (2.1)	59.9	-2.7 (2.8)	61.3	1.0 (2.5)	55.0	-1.7 (1.5)
	MEP, % predicted	70.7	0.6 (2.4)	65.1	-1.6 (2.1)	70.7	-2.7 (2.7)	62.2	-3.9 (1.8)
Muscle strength	Lower MMT score	28.0	1.6 (0.4)	27.7	0.9 (0.4)	26.4	1.6 (0.5)	26.1	0.9 (0.5)
	Upper MMT score	34.3	1.5 (0.4)	34.7	0.7 (0.6)	33.7	1.8 (0.4)	34.2	0.4 (0.7)
	Total MMT score	62.3	3.1 (0.7)	62.4	1.4 (0.8)	60.1	3.4 (0.9)	60.3	1.1 (0.9)
PROs	PROMIS®-Physical Function	66.9	1.9 (0.8)	68.0	0.2 (1.8)	64.4	1.8 (0.9)	66.9	-1.0 (2.0)
	PROMIS®-Fatigue	22.3	-2.0 (0.6)	21.1	-1.7 (1.1)	22.0	-1.9 (0.7)	20.4	-0.3 (1.0)
Biomarkers	Urine Hex4, mmol/mol	4.6	-1.9 (0.3)	6.9	1.2 (0.7)	4.6	-1.7 (0.3)	7.2	1.9 (0.8)
	Serum CK, U/L	447.0	-130.5 (25.1)	527.8	60.2 (26.2)	441.8	-118.0 (28.4)	492.3	79.6 (26.9)

Based on LOCF means

Treatment group favored | Nominal statistical significance ($P<0.05$)

AT-GAA: Key Takeaways

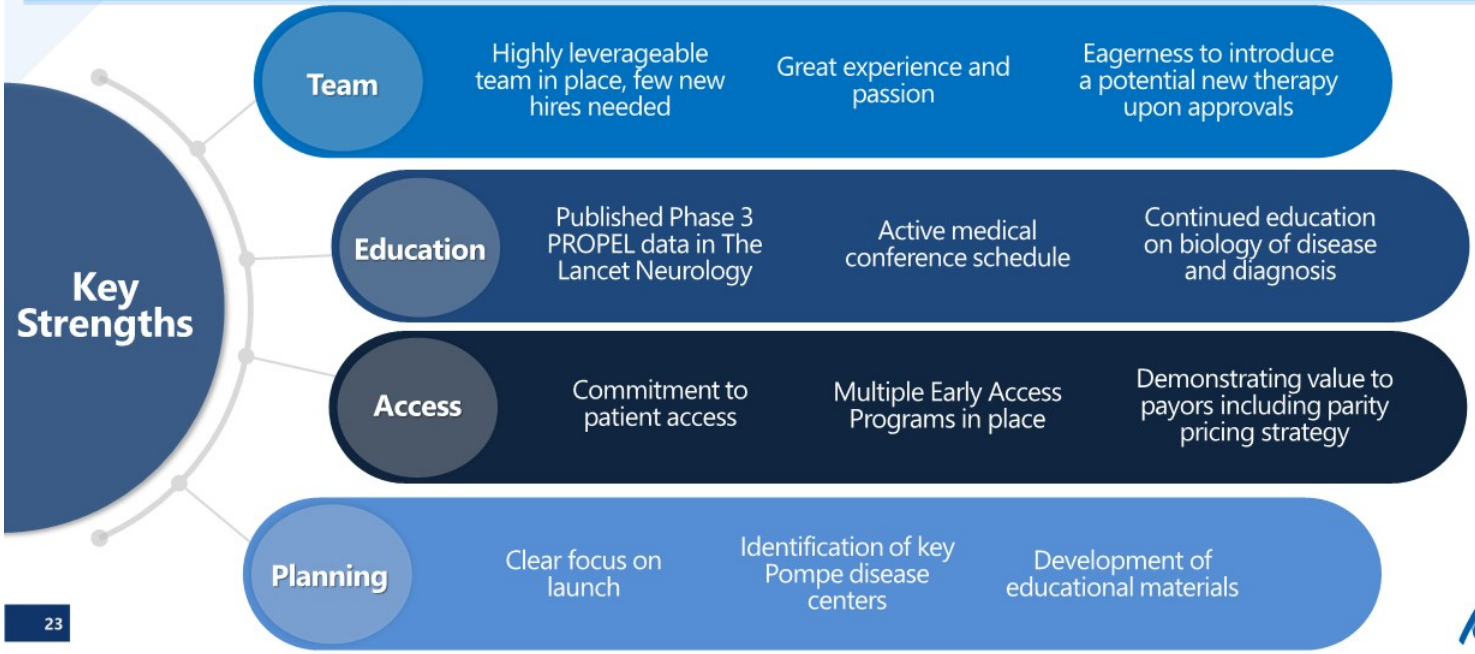
Focused on Advancing AT-GAA to as Many Patients as Possible through Global Regulatory Pathways and Early Access Schemes

- Regulatory status update:
  - U.S. PDUFA date mid 2022[1]
  - CHMP opinion late 2022
  - Planning for additional regulatory submissions
- Multiple early access mechanisms in place, including U.K., Germany, Japan, and others
- 150+ people living with Pompe disease are on AT-GAA today across our clinical extension studies and early access programs
- Ongoing supportive studies:
  - Late-Onset Pompe Disease (LOPD) in children and adolescents aged 0 to <18
  - Infantile-Onset Pompe Disease (IOPD)

[1]FDA PDUFA date of May 29, 2022 for miglustat NDA and July 29, 2022 for cipaglucosidase alfa BLA

Launch Preparations

Experienced and Passionate Rare Disease Medical and Commercial Organization Poised for Second Successful Launch



Amicus Therapeutics®

Financial Summary

... maintaining a strong financial outlook

Transforming for the Future

Transforming Amicus into a Premier Development and Commercialization Company and Building Upon our Leadership in Fabry and Pompe Diseases

- We remain a global, patient-dedicated biotechnology company developing and delive novel high-quality medicines for people living with rare diseases
- Focus will be to grow our leadership position across our two lead indications of Fabry disease and Pompe disease:
  - Continue investing in the global commercialization of Galafold in Fabry disease through continue geographic and label expansion, as well as support for diagnostic initiatives
  - Focus on securing global approvals and executing the anticipated global launch of AT-GAA in Pompe disease
  - Prepare for co-development of gene therapy programs with Caritas in Fabry and Pompe diseases
- Positioning Amicus as a partner of choice for development and commercialization of rare disease therapies

Financial Outlook and Path to Profitability

Clear Strategy to Build our Business, Advance our Portfolio, and Achieve Profitability

Drive Revenue Growth

$306M[1] full-year 2021 revenue

2022 Galafold revenue guidance of **$350M-$365M**

Secure Approvals of AT-GAA

Galafold and AT-GAA expected to drive strong double-digit growth long term

Deliver on Financial Goals

Focused on prudent expense management

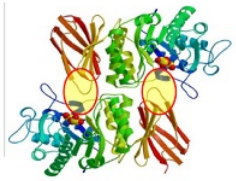
Achieve self-sustainability and profitability in 2023

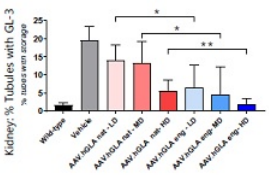
[1]Preliminary and unaudited

Expanding Our Leadership Position in Fabry and Pompe

Differentiated Gene Therapy Approach for Greater Potency and Optimized Cross Correction through Transgene Engineering for Stability and Targeting

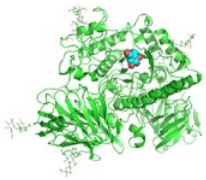
Fabry Gene Therapy

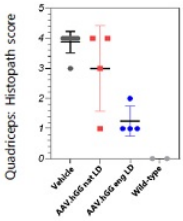




- Proprietary AAV capsid
- Pantropic capsid and ubiquitous promoter
- Engineered hGLA transgene at dimer interface designed for improved stability and optimized cross correction
- Preclinical data demonstrate robust substrate reduction across all Fabry disease relevant tissues, including first evidence of dorsal root ganglia storage reduction
- IND expected in 2023

Pompe Gene Therapy

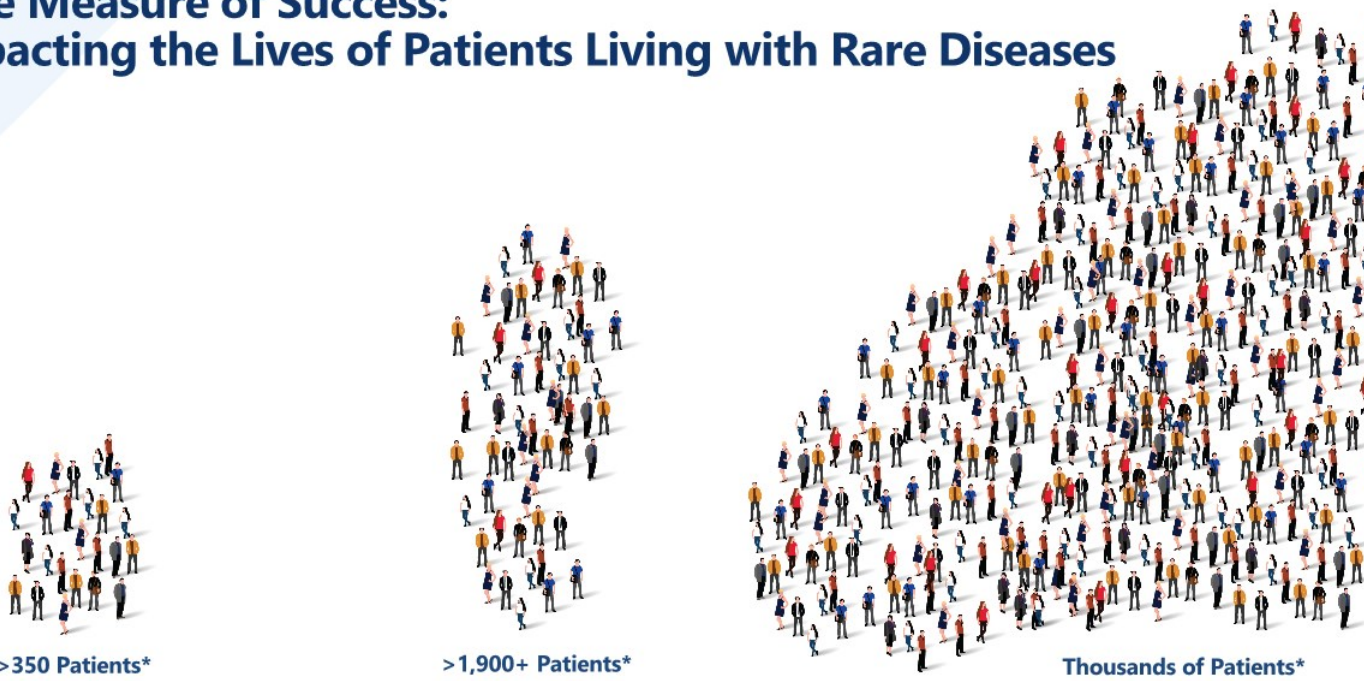




- Proprietary AAV capsid
- Pantropic capsid and ubiquitous promo
- Engineered hGAA transgene with cell receptor binding motif designed for improved uptake and optimized cross correction
- Preclinical data demonstrate robust glycogen reduction in all key Pompe disease relevant tissues, including reduction in neurons of central nervous system
- Preclinical and manufacturing work underway

Amicus and Caritas to co-develop the Fabry and Pompe gene therapies

True Measure of Success:
Impacting the Lives of Patients Living with Rare Diseases



>350 Patients*

>1,900+ Patients*

Thousands of Patients*

YE17 → YE21 → 2023+

*Clinical & commercial, all figures approximate

Amicus Therapeutics®

Thank You