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 13, 2020**

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.)**

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

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

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

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

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

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

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

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

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

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

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

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

Item 8.01 Other Events

On January 13, 2020, Amicus Therapeutics, Inc. ("the Company") issued a press release announcing its full-year 2020 strategic outlook and financial guidance. A copy of this press release is filed as Exhibit 99.1 hereto and is incorporated herein by reference. Additionally, the senior management of the Company will be using the presentation attached as Exhibit 99.2 to this Current Report, and incorporated herein by reference, in its meetings with investors and analysts at the 38th Annual J.P. Morgan Healthcare Conference.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits:

Exhibit No.	Description
99.1	January 13, 2020 Press Release
99.2	Presentation Materials - 38th Annual J.P. Morgan Healthcare Conference

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: January 13, 2020

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

Exhibit 99.1

Amicus Therapeutics Provides Full-Year 2020 Strategic Outlook and Financial Guidance

2020 Galafold Revenue Guidance of $250M-$260M

Focused on Pompe Phase 3 PROPEL study and manufacturing to support 2021 BLA and MAA

Continued Progress Across Industry Leading Rare Disease Gene Therapy Portfolio

Strong Balance Sheet with $450M+ Cash – Cash Runway Well into 2022

CRANBURY, NJ, January 13, 2020 – Amicus Therapeutics (Nasdaq: FOLD), a global, patient centric biotechnology company focused on discovering, developing and delivering novel medicines for rare diseases, today provided its full-year 2020 strategic outlook and financial guidance.

Over the course of 2019, Amicus substantially met or exceeded its five key strategic priorities:

- Nearly doubled annual revenue for Galafold with 1,000+ Fabry patients on Galafold by year end
- Completed enrollment in pivotal study in Pompe disease and reported additional Phase 2 data
- Reported additional two-year results from Phase 1/2 clinical study in CLN6 Batten disease and substantially completed enrollment in ongoing CLN-3 Batten disease Phase 1/2 study
- Established preclinical proof of concept for Fabry and Pompe gene therapies
- Maintained a strong financial position

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, “During 2019, Amicus continued our journey of becoming a leading global rare disease company. Following continued momentum and strong adoption across the globe for our Fabry precision medicine Galafold, we have exceeded our 2019 guidance. Patients are also now being treated in multiple Amicus clinical studies, including our Phase 1/2 and Phase 3 study for AT-GAA in Pompe disease, as well as Phase 1/2 studies of our investigational gene therapies for CLN3 and CLN6 Batten disease. Our first clinical data from our gene therapy pipeline suggested that our CLN6 gene therapy has the potential to stabilize progression in this devastating childhood disease. In addition, our preclinical gene therapy work with our partners at the University of Pennsylvania gives us tremendous promise in the ability to develop next generation gene therapies for the many of patients living with a rare disease. Amicus is in a stronger position than ever and remains focused on transforming the lives of people living with these rare, life-threatening conditions and creating significant value for our shareholders.”

Amicus is focused on the following five key strategic priorities in 2020:

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

Mr. Crowley will discuss Amicus' corporate objectives and key milestones in a presentation at the 38th Annual J.P. Morgan Healthcare Conference on Tuesday, January 14, 2020 at 8:30 a.m. PT (11:30 a.m. ET). A live webcast of the presentation can be accessed through the Investors section of the Amicus Therapeutics corporate web site at http://ir.amicusrx.com/events.cfm, and will be archived for 90 days.

Full-Year 2019 Financial Summary and 2020 Guidance

Amicus previously announced full-year 2019 revenue of approximately $181 million (preliminary and unaudited) from commercial sales of Galafold.

For the full-year 2020, the Company anticipates total Galafold revenue of $250 million to $260 million based on the average exchange rates for 2019. Growth in 2020 is expected to be driven by continued growth in EU markets, further success from launches in the U.S. and Japan, as well as reimbursement in additional markets. Non-GAAP operating expense guidance for the year is $410 million to $420 million, driven by continued investment in the global Galafold launch, AT-GAA clinical studies, and advancing our gene therapy pipeline.

Cash, cash equivalents, and marketable securities totaled over $450 million (preliminary and unaudited) at December 31, 2019. The current cash position is anticipated to fund ongoing operations well into 2022.

Program Highlights

Galafold (Migalastat) Oral Precision Medicine for Fabry Disease

Galafold is an oral precision medicine for the treatment of Fabry disease in adults who have amenable *GLA* variants and is approved in over 40 countries around the world, including the U.S., EU, Japan and others.

Global Galafold Updates:

- Full-year 2019 Galafold® revenue of ~$181 million exceeds guidance
- Recent marketing authorizations received in key countries, including Brazil, Columbia and Taiwan
- Registry and other Phase 4 supportive studies underway

AT-GAA for Pompe Disease

AT-GAA is an investigational therapy in Phase 3 development that consists of cipaglucosidase alfa (ATB200), a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose 6-phosphate (M6P), to enhance uptake into cells, co-administered with miglustat (AT2221), a pharmacological chaperone. Positive results from a global Phase 1/2 clinical study (ATB200-02) have shown consistent and durable responses across key measures of safety, functional outcomes and biomarkers in both ERT-switch and ERT-naïve Pompe patients following up to 24 months of treatment with AT-GAA.

Pompe Program Updates:

- Completed enrollment of 123 patients in Phase 3 PROPEL study in 4Q2019 – data expected 1H2021
- Promising Innovative Medicine (PIM) designation issued by MHRA with potential for early access for Pompe patients in United Kingdom based on Phase 1/2 results
- Biologics Manufacturing with WuXi Biologics on Track with PPQ Runs at Commercial Scale

Anticipated Pompe Program Milestones in 2020:

- Plans to apply for and initiate a Rolling Biologics License Application (BLA) for AT-GAA in 2020 with addition of full clinical results in 1H2021 to support full approval under Fast Track Designation
- Retrospective natural history study data in approximately 100 ERT-treated Pompe patients
- Additional supportive studies, including an open-label study in pediatric patients

Gene Therapy Portfolio
Amicus has established an industry leading gene therapy portfolio of potential therapies for people living with rare diseases, through a license with Nationwide Children's Hospital and an expanded collaboration with the University of Pennsylvania. Our pipeline includes gene therapy programs in rare, neurologic lysosomal disorders, specifically: CLN6, CLN3, CLN8 and CLN1 Batten disease, Pompe disease, Fabry disease, CDKL5 deficiency disorder, Niemann-Pick Type C, Mucopolysaccharidosis Type IIIB, as well as a next generation program in Mucopolysaccharidosis Type IIIA. The expanded collaboration with Penn also provides us with exclusive disease-specific access and the option rights to develop potentially disruptive new gene therapy platform technologies and programs for most lysosomal disorders and a broader portfolio of rare diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy, and select other muscular dystrophies.

Gene Therapy Program Updates:

- In CLN6, received Orphan Designation in the U.S. and EU; Rare Pediatric Disease Designation granted
- In CLN3, received Orphan Designation in the U.S. and EU; Rare Pediatric Disease Designation granted

Anticipated Gene Therapy Pipeline Milestones in 2020:

- Dose additional patients in CLN6 Phase 1/2 study and plan to advance regulatory discussions to finalize clinical and regulatory path
- Initiate long-term follow-up of initial CLN6 patients in Phase 1/2 study in 1H2020 to obtain long-term safety and efficacy data
- Plan to advance regulatory discussions to finalize clinical and regulatory path in CLN3
- Report initial data on patients enrolled in CLN3 Phase 1/2 study
- Complete IND enabling toxicology work in Pompe disease and progress towards IND
- Additional preclinical data expected in multiple programs
- Disclose up to two additional IND candidates
- Manufacturing advancements across portfolio

About Galafold
Galafold® (migalastat) 123 mg capsules is an oral pharmacological chaperone of alpha-Galactosidase A (alpha-Gal A) for the treatment of Fabry disease in adults who have amenable *GLA* variants. In these patients, Galafold works by stabilizing the body's own dysfunctional enzyme so that it can clear the accumulation of disease substrate. Globally, Amicus Therapeutics estimates that approximately 35 to 50 percent of Fabry patients may have amenable *GLA* variants, though amenability rates within this range vary by geography. Galafold is approved in over 40 countries around the world, including the U.S., EU, 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 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, business development plans and the projected revenues, sales, expenses 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 or projections will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's revenue, sales, expenses and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans and strategies. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2018. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this press 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.

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FOLD–G

Exhibit 99.2

Amicus Therapeutics®

38th Annual J.P. Morgan Healthcare Conference

John F. Crowley, Chairman and Chief Executive Officer
January 14, 2020

Forward-Looking Statements

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, business development plans and the projected revenues, sales, expenses 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 or projections will be achieved. Any or all of the forward-looking statements in this presentation may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this presentation due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's revenue, sales, expenses and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans and strategies. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2018. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this presentation to reflect events or circumstances after the date hereof.

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A RARE COMPANY

A leading fully-integrated, global rare disease biotechnology company

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

EMPLOYEES in 27 Countries

~$450M+ Cash as of 12/31/19*

Two Clinical-Stage Gene Therapies

AT-GAA
Phase 3 in Pompe Disease

Gene Therapy PLATFORM
Protein Engineering & Glycobiology

GLOBAL COMMERCIAL ORGANIZATION

World Class BIOLOGICS Capabilities

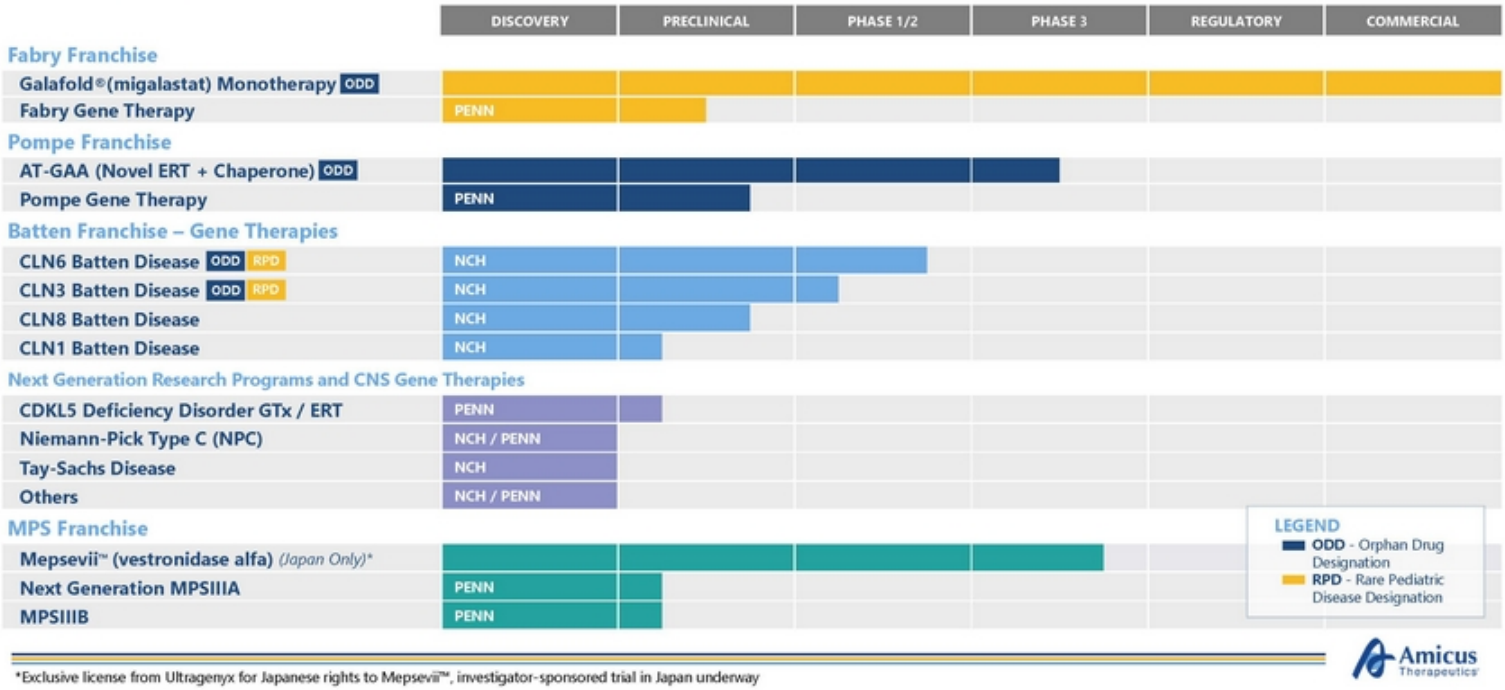
Robust R&D Engine
Nearly 50+ Lysosomal Disorders and More Prevalent Rare Diseases

*Preliminary and unaudited

A RARE OPPORTUNITY

A broad and patient-focused portfolio to drive value creation

A RARE PORTFOLIO



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

Amicus Therapeutics

2019 Key Strategic Priorities

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

2020 Key Strategic Priorities

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

Our Passion for Making a Difference Unites Us

Amicus is now poised to create significant shareholder value while advancing our mission for patients

Amicus Therapeutics

Key Takeaways

Recent successes across our science, clinical, regulatory and commercial efforts position us for the future

- Galafold Continues Strong Launch Performance & Cornerstone of Amicus Success
- AT-GAA for Pompe Advances Toward Approval as "Crown Jewel" of Amicus Portfolio
- Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future
- Strong Financial Outlook with Current Cash Well into 2022

Fabry Disease Overview

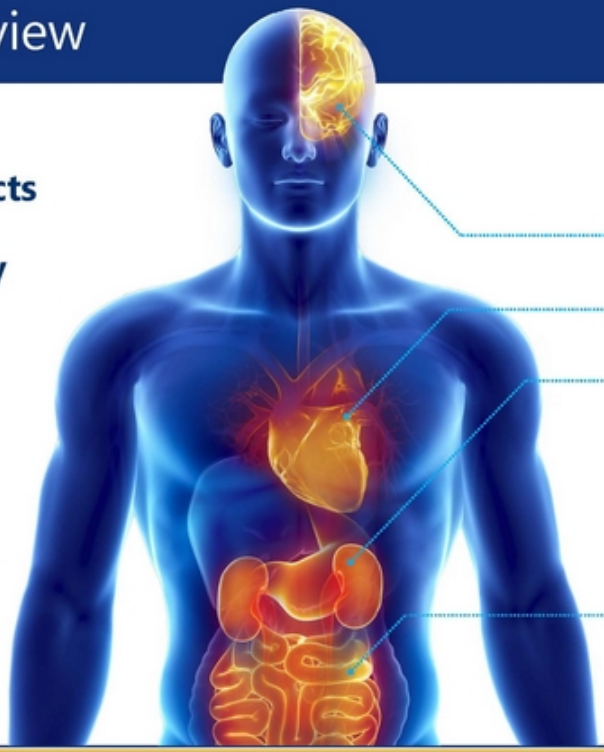
"We support the disease communities – and their families"
- Amicus Belief Statement

Fabry Disease Overview

Fabry Disease is a Fatal Genetic Disorder that Affects Multiple Organs and is Believed to be Significantly Underdiagnosed

Key Facts:

- α-Gal A enzyme deficiency leads to substrate (GL-3) accumulation
- >1,000 known mutations
- ~10K diagnosed WW (51% female/49% male[4])
- Newborn screening studies suggest 5-10 fold greater incidence (~1:1000 - 1:4000)



Leading Causes of Death:

- Transient Ischemic Attack (TIA) & Stroke[1]
- Heart Disease[2]
- Kidney Disease[3]

Life-Limiting Symptoms:

- Gastrointestinal[3]

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

Amicus Therapeutics

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, 2019)

Galafold is the cornerstone of Amicus' success. It is an orally delivered small molecule precision medicine with a unique mechanism of action for Fabry patients with amenable variants that replaces the need for intravenously delivered enzyme replacement therapy

One of the Most Successful Rare Disease Launches

~$181M
FY19 Galafold Revenue (preliminary unaudited)

$250-260M
FY20 Global Galafold Rev. Guidance

Geographic Expansion in 2019
Including Argentina, Brazil, Colombia, Taiwan

348
Amenable Variants in U.S. Label

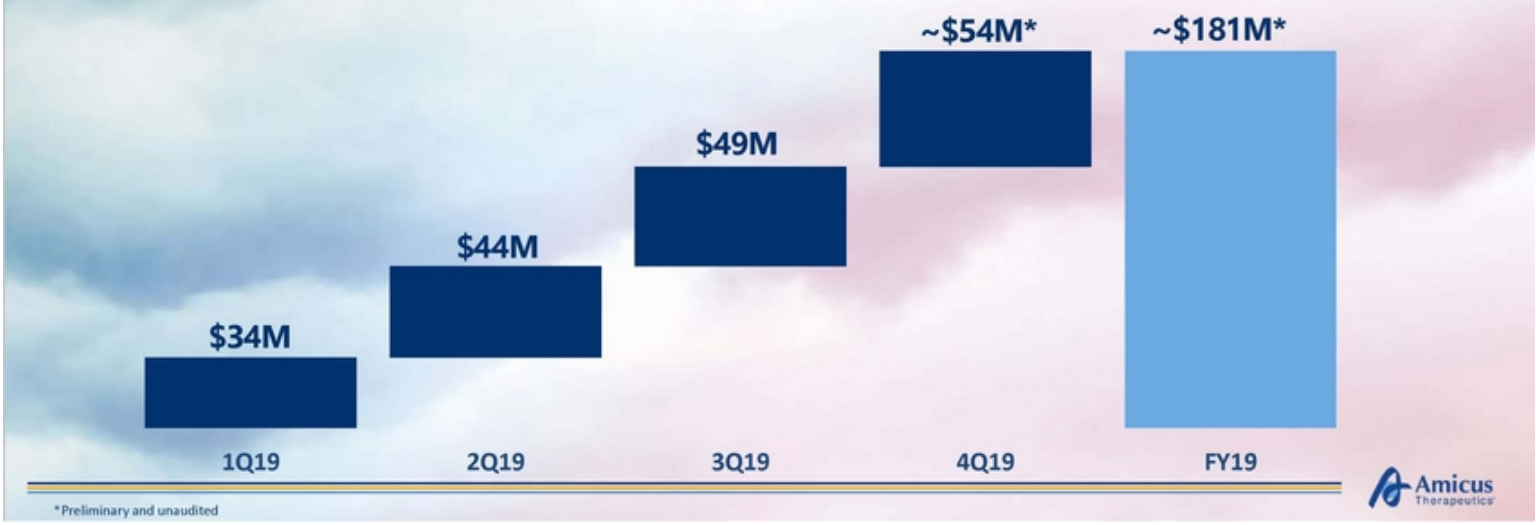
40+
Countries with Regulatory Approvals: Including U.S., EU, Japan, and Other Countries

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

Amicus Therapeutics

Galafold Success and FY19 Galafold Revenue Guidance

Strong full-year performance of ~$181M (preliminary/unaudited) revenue, exceeding guidance of $170-$180M



*Preliminary and unaudited

Amicus Therapeutics

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

Global commercial metrics continue to be very strong with >90% compliance and adherence, 30% global market share of treated amenable patients and continued broad market access

FY19 Strength Reflects Positive Momentum Across All Key Global Commercial Metrics and 1,000+ Treated Patients

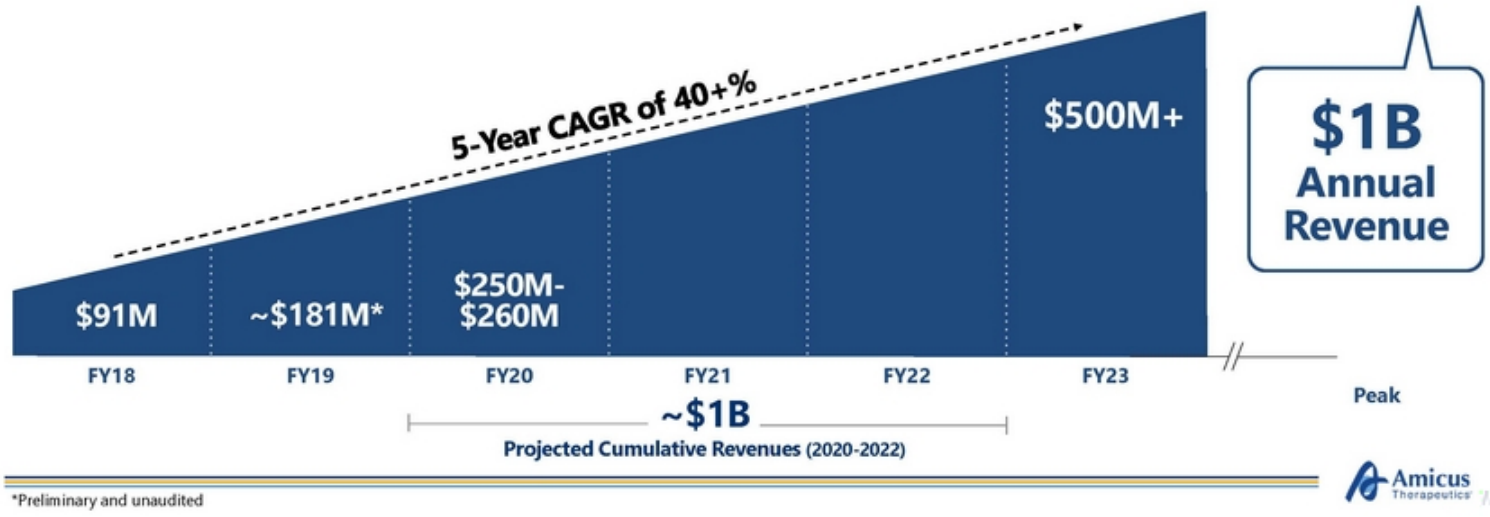
- **Global**: 30%+ estimated global market share of treated amenable patients (as of 9/30/19)*
- **U.S.**: Steady growth in adoption from 100+ prescribers and broad reimbursement coverage
- **EU:** Accelerated patient growth in new and established markets throughout 2019
- **APAC**: Continued strong contribution from Japan and Australia
- **LATAM**: New approvals in Brazil, Colombia and Argentina lay strong foundation for future growth
- **Demographics**: Global mix of switch (65%) and previously untreated patients (35%)

*Market share based on reported global Fabry sales for the calendar year ending 3Q19 and assumes a 35% amenability rate.

Amicus Therapeutics

Galafold Growth Trajectory

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

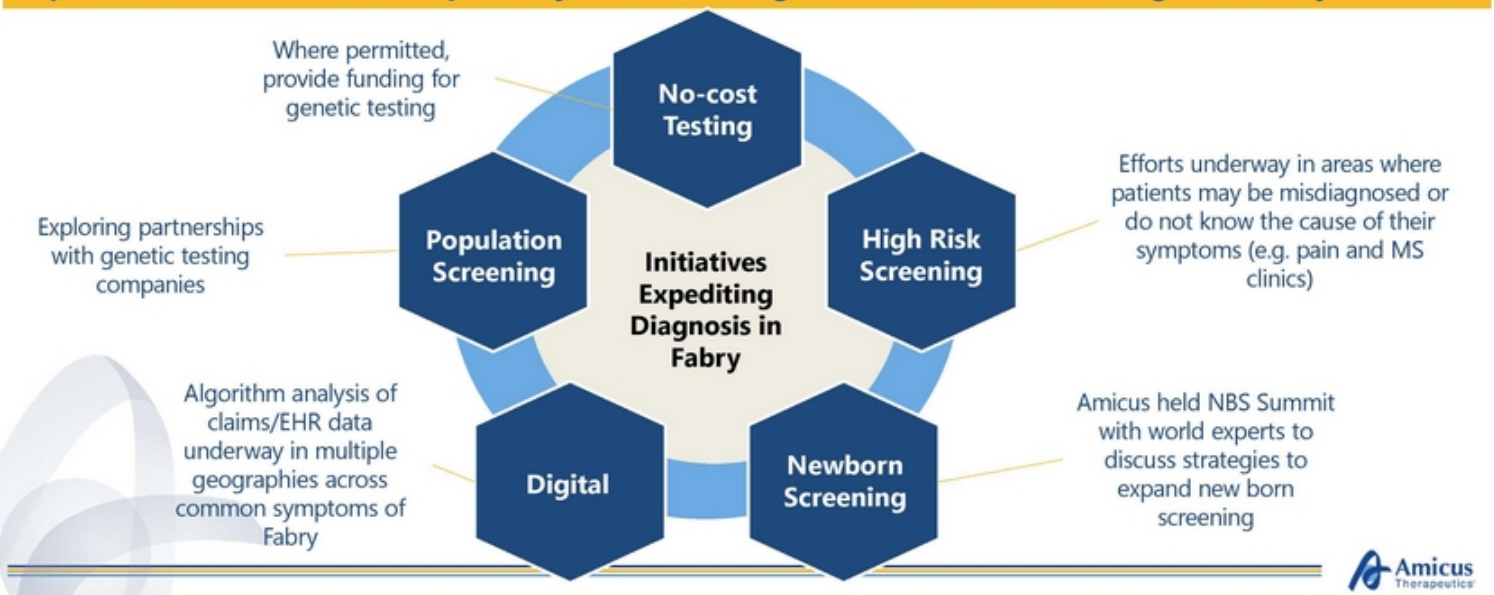


*Preliminary and unaudited

Amicus Therapeutics

Fabry Disease Diagnostic and Growth Drivers

Fabry disease is both underdiagnosed and misdiagnosed. Expanded screening initiatives have the potential to drive a shorter pathway to correct diagnosis for individuals living with Fabry disease



Amicus Therapeutics

Galafold Opportunity

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

*Preliminary and unaudited

Amicus Therapeutics

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

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

Pompe Disease Overview

Pompe disease is a severe and fatal muscular dystrophy and one of the most prevalent lysosomal disorders with very high unmet medical need

- 5,000 – 10,000+ patients diagnosed WW[1]; newborn screening suggests underdiagnosis
- Age of onset ranges from infancy to adulthood
- 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 glycogen accumulation and cellular dysfunction
- Symptoms include muscle weakness, respiratory failure, and cardiomyopathy
- ~$1B+ global Pompe ERT sales[2]

1. National Institute of Neurological Disorders and Stroke (NIH). 2. Based on calendar year ending September 30, 2019. Exchange rate as of 1/6/19. Source: Sanofi Press Releases

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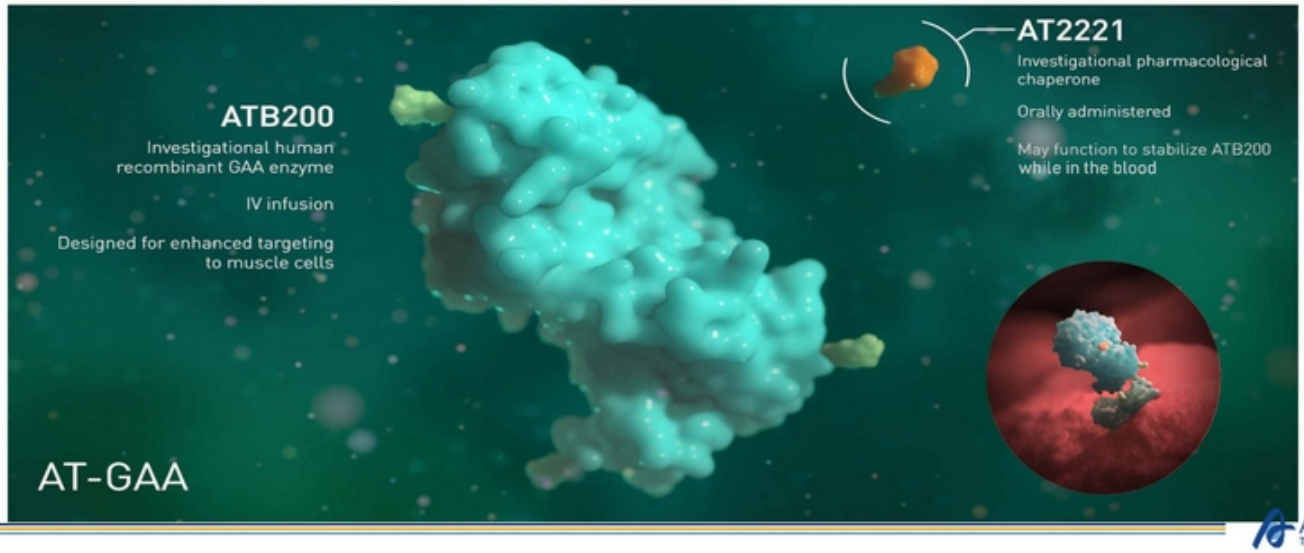
AT-GAA: Foundation in Protein Engineering

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



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

AT-GAA is the first ever second-generation product for <u>any</u> lysosomal disorder to earn FDA Breakthrough Therapy Designation (BTD)

Plans to apply for and initiate a rolling BLA submission for AT-GAA in LOPD in 2020

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

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

BTD Features

- Intensive guidance on an efficient drug development program
- Organizational commitment involving senior agency staff
- All Fast Track program features including rolling submission

BTD Criteria

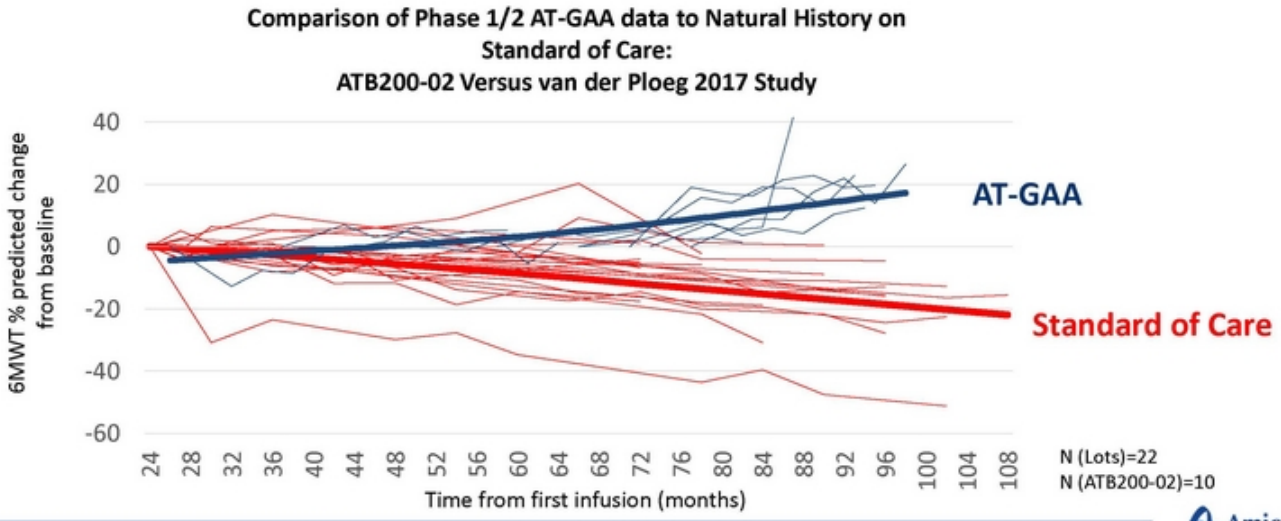
- Intended to treat a serious or life-threatening disease or condition
- Preliminary clinical evidence indicates drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints

Amicus Therapeutics

6MWT Natural History: Phase 1/2 AT-GAA Data vs. Medical Literature

van der Ploeg 2017

Improvement in percentage predicted 6MWD seen in all patients who switched from alglucosidase alfa to AT-GAA



1. Data for AT-GAA represent time from first infusion of SOC ERT and change from baseline at the time of switching from SoC to AT-GAA
2. Source: ATB200-02 IA#7; Ans T. van der Ploeg et al. Poster presented at the 13th Annual WORLD Symposium™ 2017, February 13–17, 2017, San Diego, CA, USA

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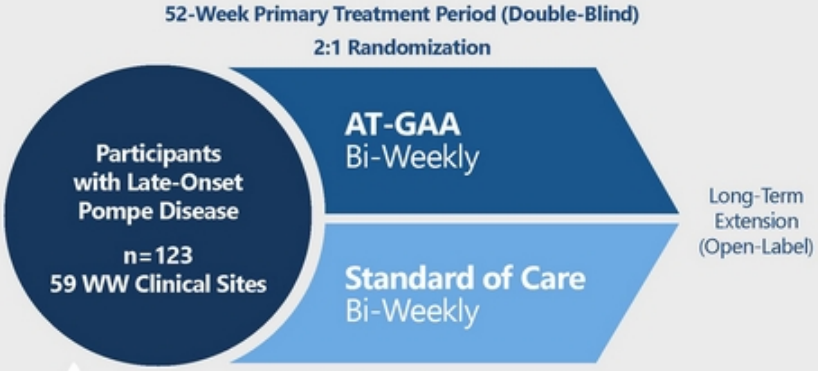
PROPEL (ATB200-03) Study Design

PROPEL

Phase 3 exceeded enrollment with data expected in 1H2021. The study is highly powered for success and supports a broad label, with FDA and EMA agreement on study design and primary endpoint (6MWT)



- **PROPEL pivotal study over-enrolled with data expected in 1H2021**
- **Study includes ERT-Switch and ERT-Naïve Patients**
- **FDA and EMA agreed upon primary endpoint of 6MWD, an integrated measure of disease progression that evaluates both cardiopulmonary and musculoskeletal systems**

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Pompe Biologics Manufacturing

Amicus and partner WuXi Biologics have successfully produced AT-GAA at 1,000L commercial scale, demonstrating unique capabilities in Amicus biologics process science, manufacturing and quality control

- Manufacturing PPQ runs at WuXi biologics are underway
- Agreements on biocomparability between 250L and 1,000L scale with key regulators (FDA, BfARM)
- All PROPEL participants treated with drug manufactured at 1,000L
- Current bioreactor capacity to supply global population

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AT-GAA: Key Takeaways

- PROPEL pivotal study exceeded enrollment with data expected 1H2021
- Breakthrough Therapy Designation and the Promising Innovative Medicine designation highlight unmet need in Pompe disease today
- Plan to submit and initiate rolling submission of Biologics License Application in 2020
- Manufacturing PPQ runs at WuXi biologics on track
- Peak revenue potential of $1B-$2B, with exclusivity well into 2030s

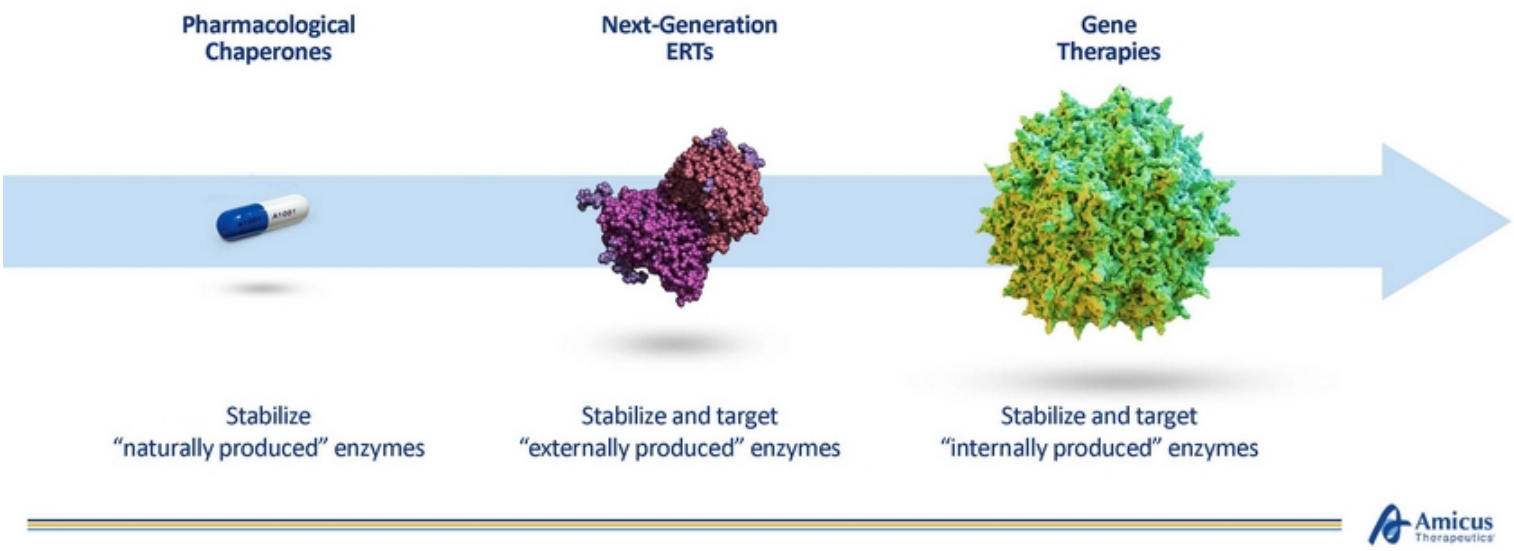
Amicus Therapeutics

Next Generation Gene Therapy Platform

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

A Natural Evolution: Chaperones to Optimized ERT to Gene Therapy

Amicus' expansion into gene therapy is built upon years of experience in developing genetic medicines designed to deliver deficient proteins to target cells and organelles

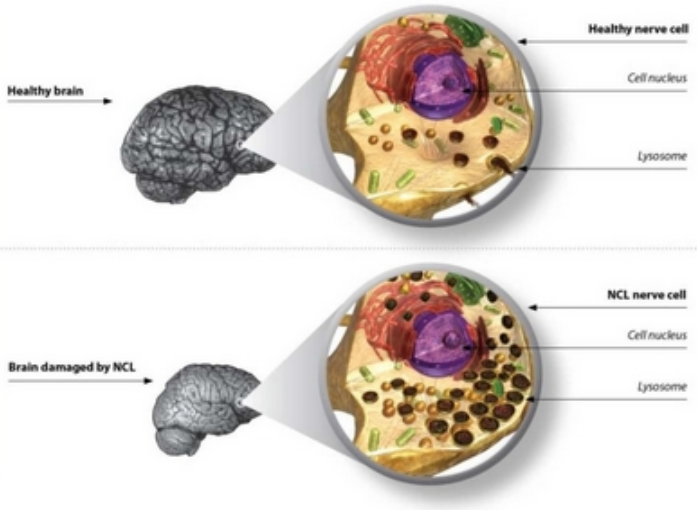


Batten Disease Overview

Batten disease is a devastating early childhood disease that is 100% fatal in children

Disease Overview

- A group of disorders known as neuronal ceroid lipofuscinoses (NCLs), collectively referred to as Batten disease
- Mutation in one of 13 different CLN genes leads to lysosomal dysfunction
- Signs and symptoms include loss of speech, ambulation, vision and cognition



Source: Batten Disease Fact Sheet, NINDS. Publication date June 2018.

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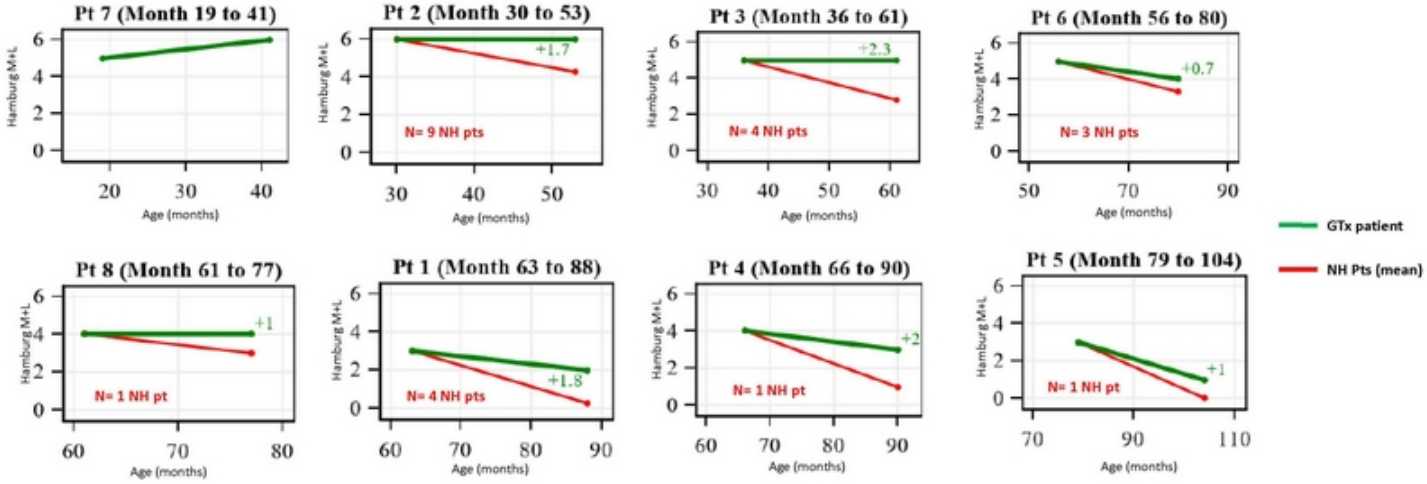
CLN6 Clinical Efficacy Data: Natural History Matched Comparisons

Analysis of treated patients demonstrates improvement compared to natural history patients matched for age and baseline Hamburg M+L score*



*Matched for age and exact baseline Hamburg score. No current match (for age and exact M+L score at baseline) for youngest patient (pt. 7)

Source: Data on file

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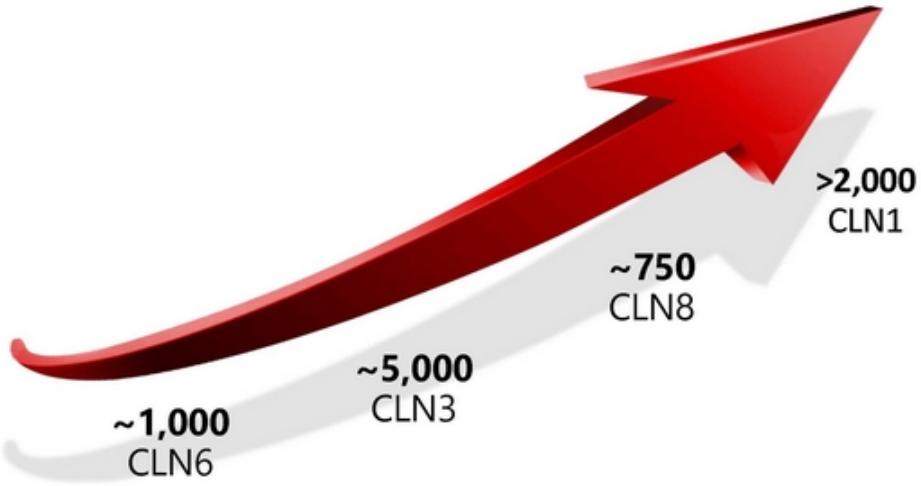
Batten Disease Franchise

CLN6 results validate the broad potential of the intrathecal AAV platform to build a valuable and significant franchise to save thousands of children suffering from multiple types of Batten diseases with potential for $1B+ in recurring peak revenue



*Estimated addressable U.S., EU, Japan, and other major, reimbursable markets based on published incidence and prevalence

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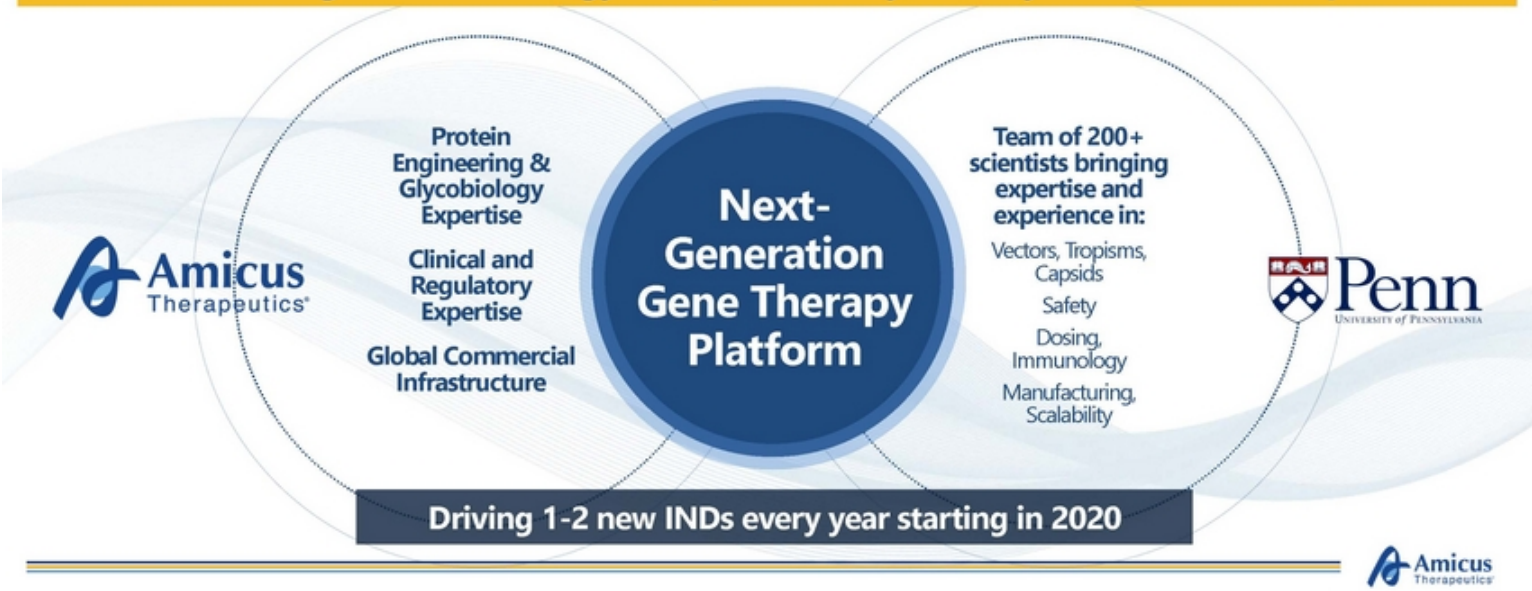
Combines Amicus and Penn Expertise Across Lysosomal and Rare Diseases

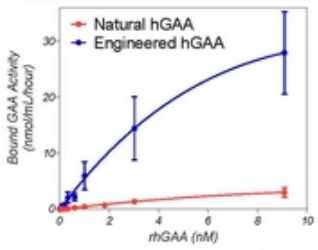
Combines Amicus expertise in protein engineering with Penn expertise in AAV vectors, manufacturing and immunology to improve safety, efficacy and speed development



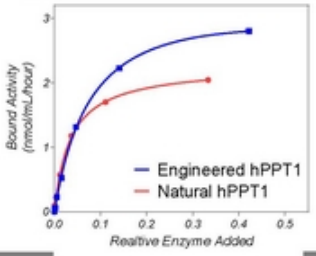
Protein Engineering Platform Has Potential To Be Broadly Applicable to Gene Therapies For Majority of LDs

Amicus has repeatedly validated the protein engineering platform approach in multiple indications to design transgenes with improved cellular uptake

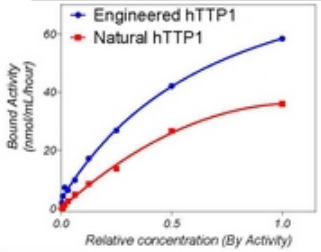
Pompe - GAA



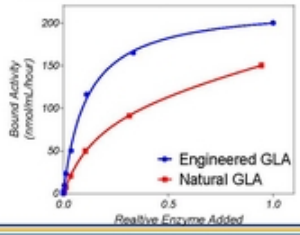
CLN1 – PPT1



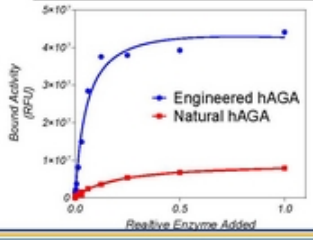
CLN2 – TTP1



Fabry – GLA



AGU – AGA



Source: Data on file

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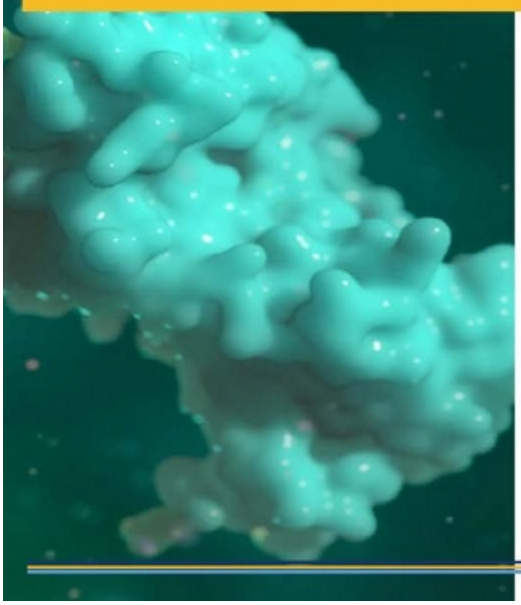
Pompe Gene Therapy Summary and Next Steps

Initial Pompe preclinical gene therapy data demonstrate differentiated profile and clear pathway toward the clinic



- Clinical candidate selected
- Toxicology batch manufacturing and GMP tech transfer to Paragon underway
- IND enabling toxicology work in progress
- Potential to enter clinic in 1H2021

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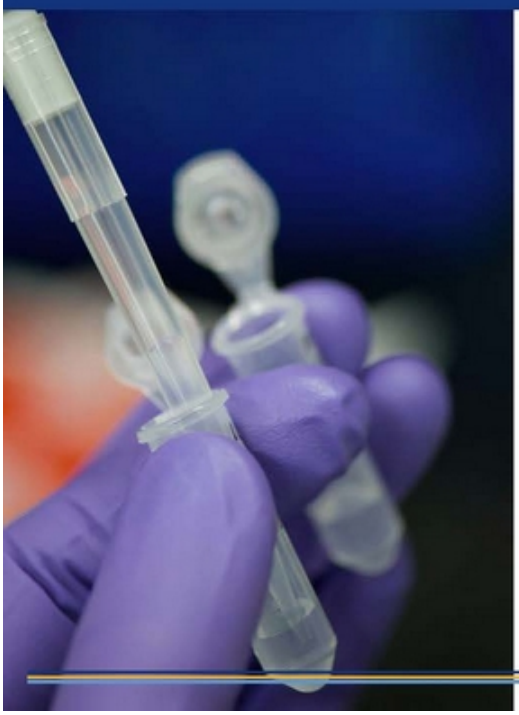
Gene Therapy: Key Takeaways



- CLN6 data show profound impact as compared to natural history matched for age and baseline. Potential to become first ever approved gene therapy for fatal brain disease in children
- AAV intrathecal platform increasingly gives confidence in CLN3 program (largest cause of childhood neurodegeneration, 5,000+ children)
- Penn Collaboration R&D engine, with rights to 50+ diseases and access to world class gene therapy technology and expertise
- 2 clinical and 8 preclinical gene therapies in development and one clinical candidate now generated (Pompe)

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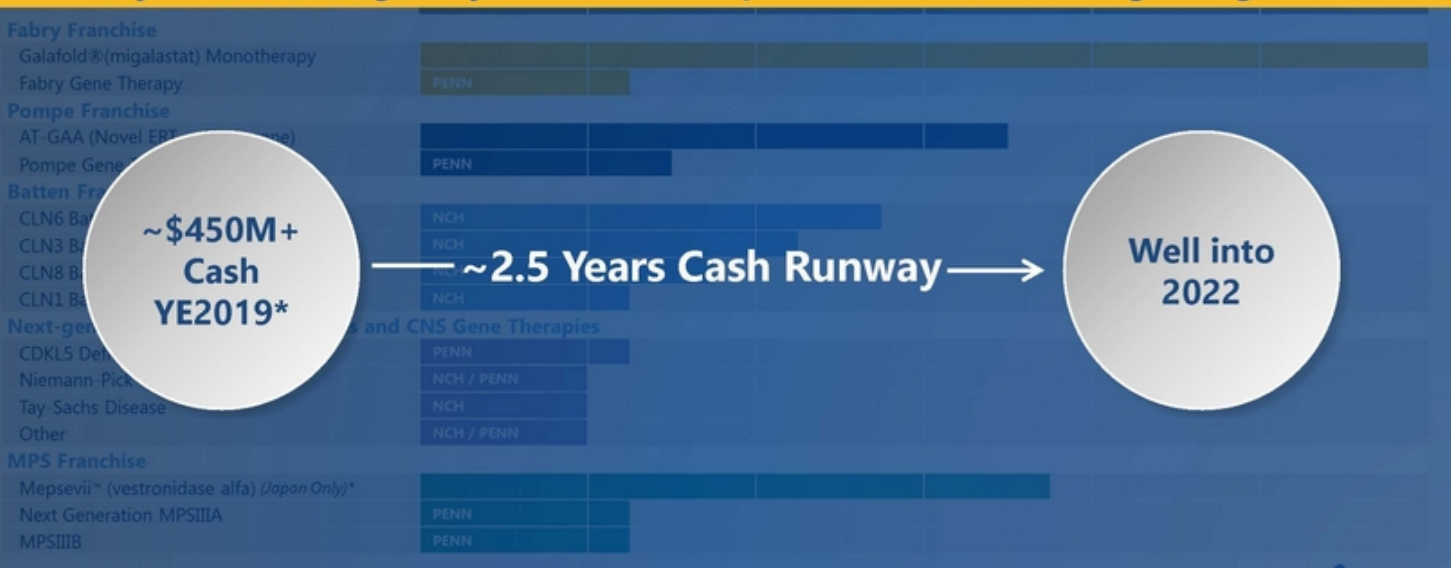
Financial Summary & Milestones

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

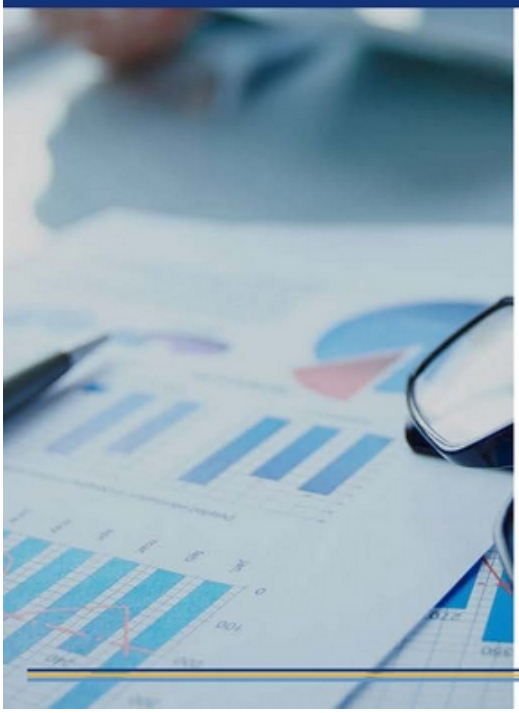
Cash Runway Now to Well into 2022 (~2.5 years)

Fully funded through major milestones in portfolio and continued global growth



*Preliminary and unaudited

Financial Outlook: Key Takeaways



- Company now fully funded through major milestones in portfolio and continued global growth
- Cumulative Galafold projected revenue of $1B+ in 2020-2022 offsets significant majority of company spend/investments
- Extended cash flow runway through OpEx savings, CapEx phasing, program prioritization and increased Galafold revenue projections
- No material business development planned or needed in next several years
- Only modest additional capital required in the outer years to extend runway into profitability with multiple non-equity sources available as/when needed

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At Major Inflection Point: Path to Profitability

Clear strategy to build our business, advance our portfolio and achieve profitability with the following key priorities:

- Grow Galafold
- Advance AT-GAA to pivotal data, global approvals and launch
- Progress CLN6, CLN3 and Pompe gene therapies into and through the clinic
- Generate 1-2 gene therapy INDs every year starting in 2021
- Discover and develop next generation protein engineering and gene therapy technologies with Penn

Only modest additional capital required in outer years to extend runway into profitability with multiple non-equity sources available as/when needed

Thank You

Appendix

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Financial Summary & Guidance

Strong Balance Sheet with ~$450M+ Cash – Cash Runway Well into 2022

FINANCIAL POSITION	
Cash[1]	~$450M+
Cash Runway[2]	Well Into 2022
Debt[1,3]	$152.8M
CAPITALIZATION	
Shares Outstanding (as of 12/31/2019)	255,417,869
FINANCIAL GUIDANCE	
FY20 Galafold Revenue Guidance	$250M-$260M
FY20 Non-GAAP Operating Expense Guidance	$410M-$420M

[1] Preliminary and unaudited [2] Based on existing operating plan [3] Includes $2.8 million of convertible debt and $150 million of straight debt

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