

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

FORM 8-K

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

Date of Report (Date of earliest event reported): **May 14, 2020**

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

Delaware

(State or Other Jurisdiction of
Incorporation)

Delaware
(State or Other Jurisdiction
of Incorporation)

001-33497

(Commission
File Number)

71-0869350

(I.R.S. Employer
Identification No.)

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

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

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

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

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

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

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 May 14, 2020, Amicus Therapeutics, Inc. ("Amicus") released presentation materials it plans to use in meetings with investors and analysts. This presentation reflects, among other updates, Amicus and Nationwide Children's Hospital's ("NCH") agreement to amend the existing license agreement to remove rights to the CLN8 program which will revert to NCH for further development. A copy of this presentation is attached hereto as Exhibit 99.1 and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits**(d) Exhibits:**

Exhibit No.	Description
99.1	Corporate Presentation
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: May 14, 2020

By: /s/ Ellen S. Rosenberg

Name: Ellen S. Rosenberg

Title: Chief Legal Officer and Corporate Secretary



General Corporate & Gene Therapy Overview:

At the Forefront of the Human Genome Medicine Revolution



May 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, 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, 2019 and the Quarterly Report filed on Form 10-Q to be filed today. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

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. Full reconciliations of GAAP results to the comparable non-GAAP measures for the reported periods appear in the financial tables section of this presentation. 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.

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 2H2022

A RARE PORTFOLIO

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3	REGULATORY	COMMERCIAL
Fabry Franchise						
Galafold® (migalastat) Monotherapy ODD						
Fabry Gene Therapy	PENN					
Pompe Franchise						
AT-GAA (Novel ERT + Chaperone) ODD						
Pompe Gene Therapy	PENN					
Batten Franchise – Gene Therapies						
CLN6 Batten Disease ODD RPD						
CLN3 Batten Disease ODD RPD						
CLN1 Batten Disease						
Next Generation Research Programs and CNS Gene Therapies						
CDKL5 Deficiency Disorder GTX / ERT	PENN					
Niemann-Pick Type C (NPC)	NCH / PENN					
Others	NCH / PENN					
MPS Franchise						
Mepsevii™ (vestronidase alfa) <i>(Japan Only)*</i>						
Next Generation MPSIIIA	PENN					
MPSIIB	PENN					

LEGEND

- ODD** - Orphan Drug Designation
- RPD** - Rare Pediatric Disease Designation

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





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

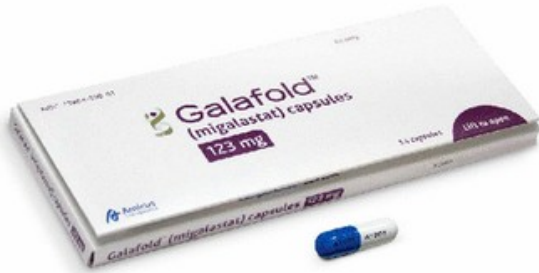
...taking a leadership role in
the treatment of Fabry disease

"We push ideas as far and as fast as possible"
- Amicus Belief Statement

Galafold Snapshot (as of March 31, 2020)

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



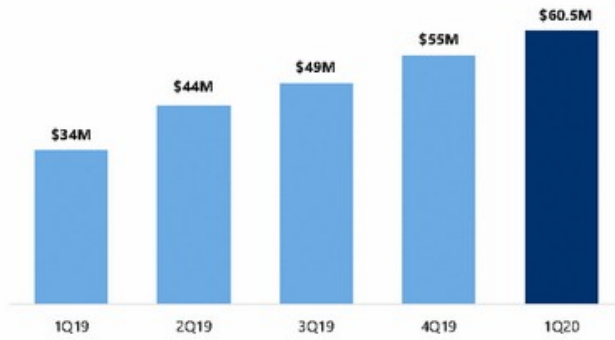
Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and a amenable mutation in the gene encoding alpha-galactosidase associated with Fabry Disease with residual activity. For more information on Galafold, including the full U.S. prescribing information, please visit www.amicustherapeutics.com/galafold. For more information on Amicus, including our pipeline of clinical and commercial products, our financial performance, and our strategy, please visit www.amicustherapeutics.com.



Galafold Quarterly Performance

Quarterly Growth Remains Steady with 1Q20 Revenue of \$60.5M, Growing 78% Year-over-Year

Quarterly Galafold Sales

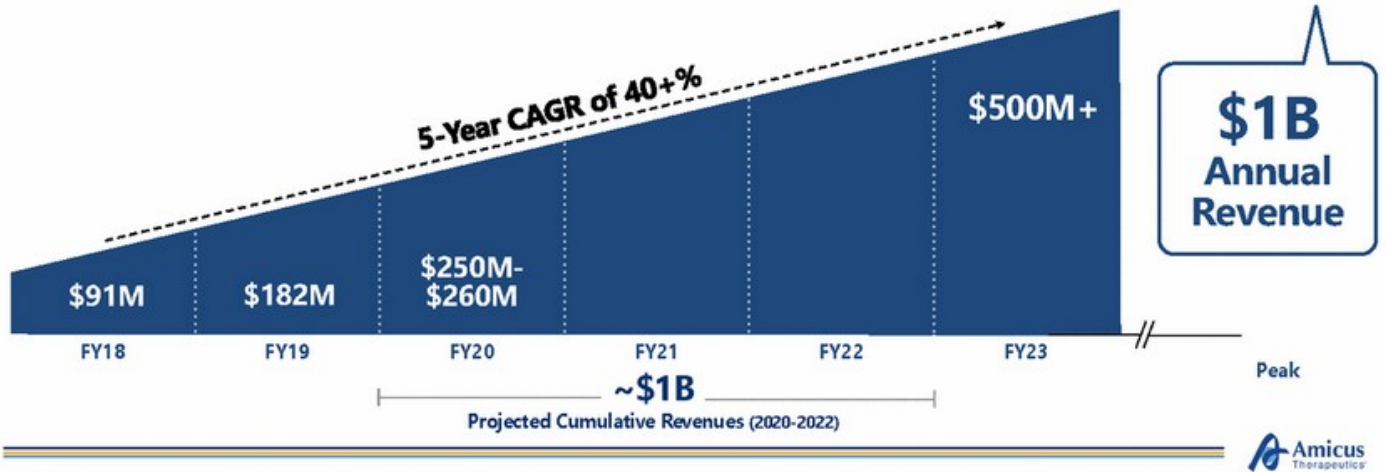


Year-over-Year Galafold Sales Growth



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



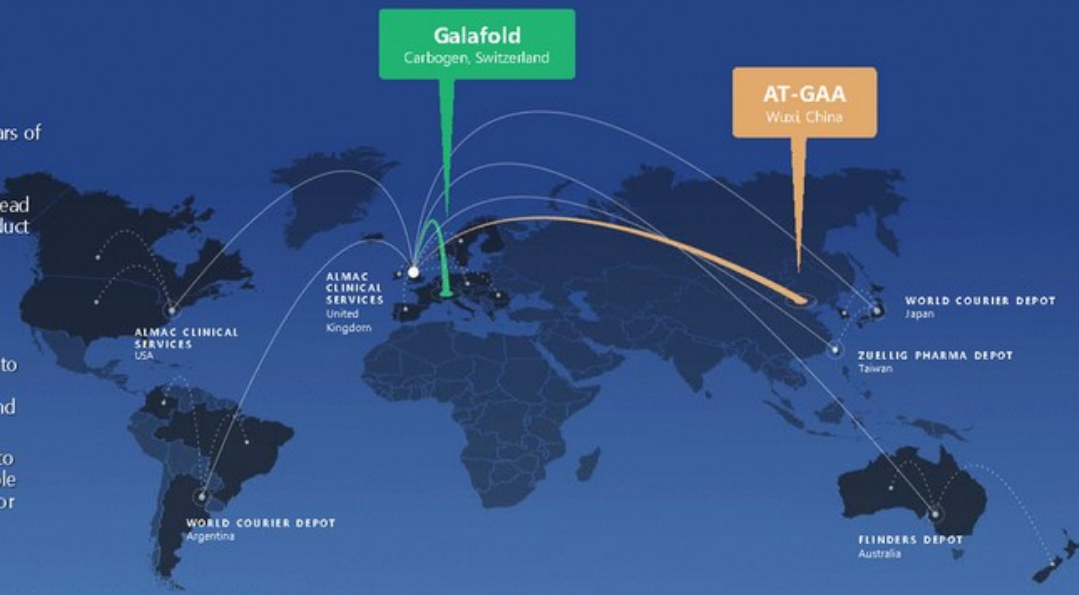
Global Supply Chain

Existing Supply Chain Strategy:

- **Galafold:** Hold multiple years of inventory in API and Drug Product
- **AT-GAA:** Built inventory ahead of time and move drug product to UK

Post COVID-19:

- **Galafold:** Push inventory into the supply chain as far as possible down to country and pharmacy level
- **AT-GAA:** Push inventory into supply chain as far as possible and coordinate site by site for delivery





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 WW¹; 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 sales²

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

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

ATB200
Investigational human recombinant GAA enzyme
IV infusion
Designed for enhanced targeting to muscle cells

AT2221
Investigational pharmacological chaperone
Orally administered
May function to stabilize ATB200 while in the blood

AT-GAA

The image features a large, light blue 3D molecular model of the AT-GAA protein structure. To the left, text describes ATB200 as an investigational human recombinant GAA enzyme administered via IV infusion, designed for enhanced targeting to muscle cells. To the right, text describes AT2221 as an investigational pharmacological chaperone administered orally, which may function to stabilize ATB200 while in the blood. A circular inset on the right shows a smaller view of the protein structure. The background is a dark green gradient with small white particles.

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

AT-GAA is the first ever second-generation product for any lysosomal disorder to earn FDA Breakthrough Therapy Designation (BTM)

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



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

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



BTM Features

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



BTM 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

Pompe Patient Experience in Phase 1/2 Clinical Study (ATB200-02)

Phase 1/2 results showed strong and durable effects in patients out to two years, leading to dramatic improvements in muscle strength and function, as well as significant improvements in key biomarkers of disease



6-Min Walk Test (m)	Cohort	Baseline (n=10)	Change at Month 6 (n=10 ^a) Mean (SD)	Change at Month 12 (n=10 ^b) Mean (SD)	Change at Month 24 (n=9 ^{b,c}) Mean (SD)
		Cohort 1 ERT-Switch Ambulatory	397.2 (96.8)	+23.9 (52.2)	+42.2 (46.5)
	Cohort	Baseline (n=5)	Change at Month 6 (n=5) Mean (SD)	Change at Month 12 (n=5) Mean (SD)	Change at Month 24 (n=5) Mean (SD)
	Cohort 3 ERT-Naive	399.5 (83.5)	+41.8 (29.4)	+63.1 (29.1)	+60.7 (36.5)

FVC (% Predicted)	Cohort	Baseline (n=9 ^d)	Change at Month 6 (n=9 ^e) Mean (SD)	Change at Month 12 (n=9 ^e) Mean (SD)	Change at Month 24 (n=8 ^{b,c}) Mean (SD)
		Cohort 1 ERT-Switch Ambulatory*	52.6 (14.7)	-1.2 (4.0)	-3.0 (6.0)
	Cohort	Baseline (n=5)	Change at Month 6 (n=5) Mean (SD)	Change at Month 12 (n=5) Mean (SD)	Change at Month 24 (n=5) Mean (SD)
	Cohort 3 ERT-Naive	53.4 (20.3)	+4.4 (5.6)	+4.6 (8.8)	+6.8 (6.8)

Data from interim analysis 2

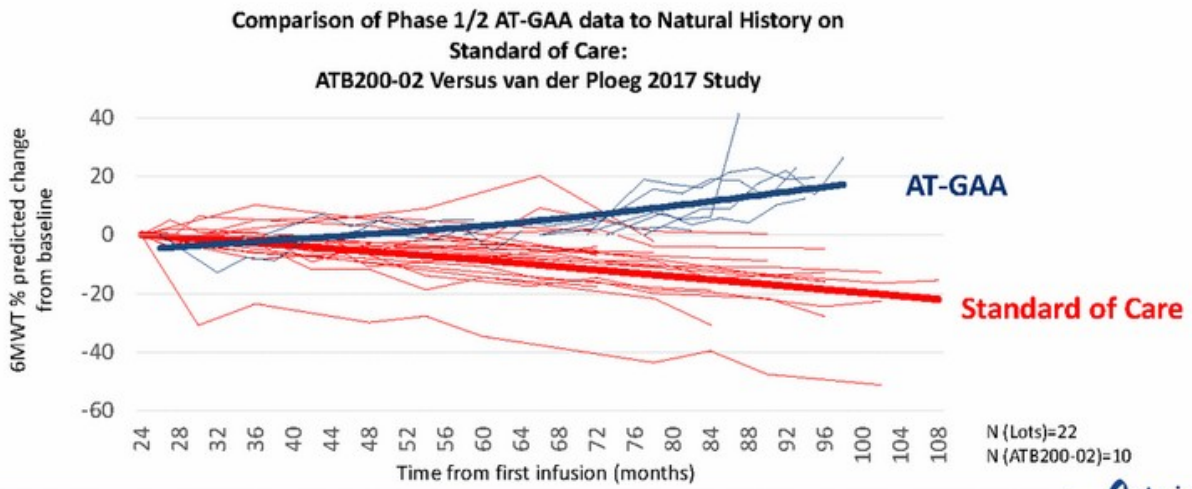
*One patient in Cohort 1 discontinued after 18 weeks due to burden of travel; baseline value is not shown for this patient. ^bOne patient in Cohort 1 discontinued from study before Month 24. ^cBaseline FVC not available for 1 patient in Cohort 1.



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



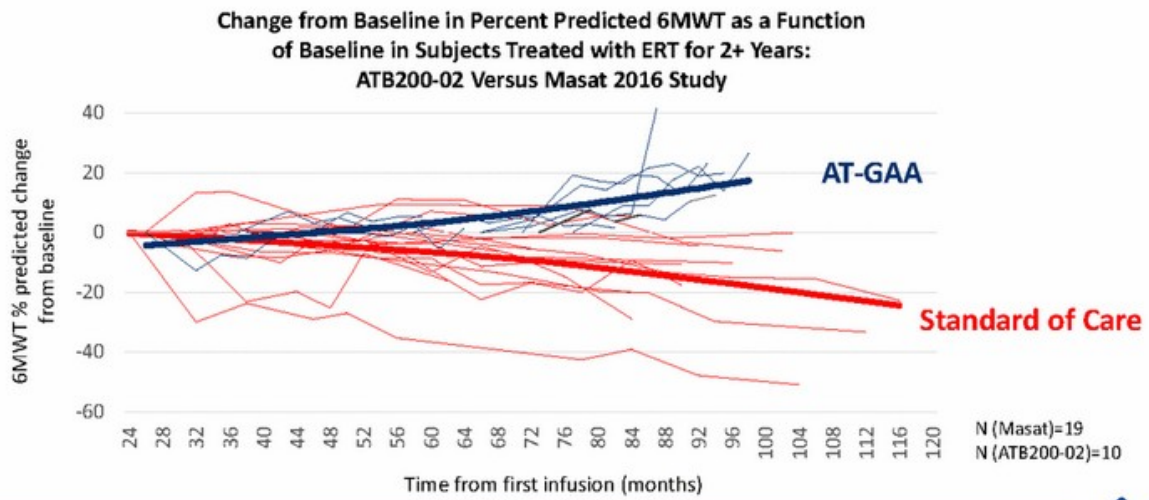
1. Data for AT-GAA represent time from first infusion of SCC ERT and change from baseline at the time of switching from SoC to AT-GAA
2. Source: ATB200-02 IAR7; Ans T. van der Ploeg et al. Poster presented at the 13th Annual WORLD Symposium™ 2017, February 13–17, 2017, San Diego, CA, USA



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

Masat 2016

Second natural history data set confirms the large treatment effect of AT-GAA



Source: ATB200-02 IAA7; Masat et al 2016; Nature Scientific Reports | 6:36182



PROPEL (ATB200-03) Study Design



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)

52-Week Primary Treatment Period (Double-Blind)

2:1 Randomization



Primary Endpoint: 6-Minute Walk Test at Week 52;
Multiple Secondary Endpoints

- 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



AT-GAA: Key Takeaways



AT-GAA for Pompe
Advances Toward
Approval as "Crown
Jewel" of Amicus
Portfolio

- PROPEL study timelines are on track with data expected 1H2021
 - To date, **97%** of the 2,250 planned infusions for the ongoing PROPEL study have been completed on schedule
- Breakthrough Therapy Designation and the Promising Innovative Medicine designation highlight unmet need in Pompe disease today
- U.S. FDA grants rolling BLA submission and company plans to initiate in 2H2020
- Expanded Access Program for infantile-onset Pompe patients underway
- Process performance qualification (PPQ) runs with our partners at WuXi have been successfully completed for the drug substance
- Peak revenue potential of \$1B-\$2B, with exclusivity well into 2030s

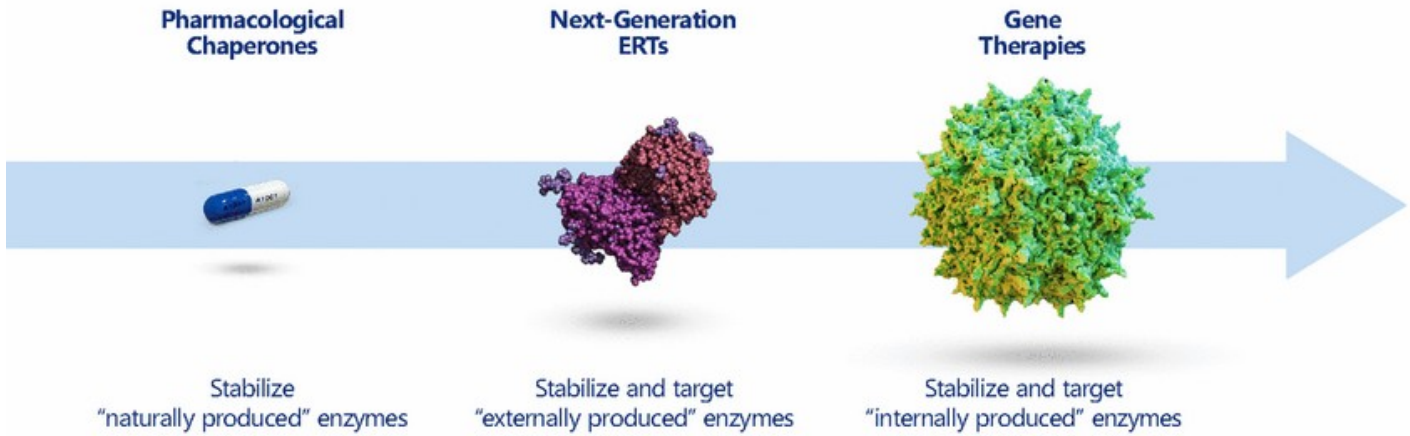


Next Generation Gene Therapy Platform

"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

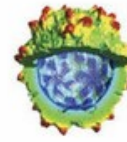


Validated Gene Therapy Platform for CNS

The Amicus Batten programs leverage AAV technologies and platforms utilized in the neuromuscular space at Nationwide Children's Hospital/Sanford and have robust preclinical and now clinical proof of concept

Clinically validated AAV gene therapy approach at NCH and Sanford

- Nationwide Children's Hospital Center for Gene Therapy (NCH)
- Intrathecal delivery with robust expression throughout CNS



Preclinical safety and efficacy studies replicated across multiple diseases at NCH

- SMA, CLN6, CLN3, CLN8

Amicus applying platform to multiple types of Batten disease and other Neurologic LSDs

- Two clinical programs in CLN6 and CLN3 Batten disease show initial safety in 15 patients; promising efficacy results in first 8 patients in CLN6
- Active preclinical program in CLN1 Batten disease with other neurologic LSDs in earlier preclinical development



Fouit, Kaspar et al, 2009

Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

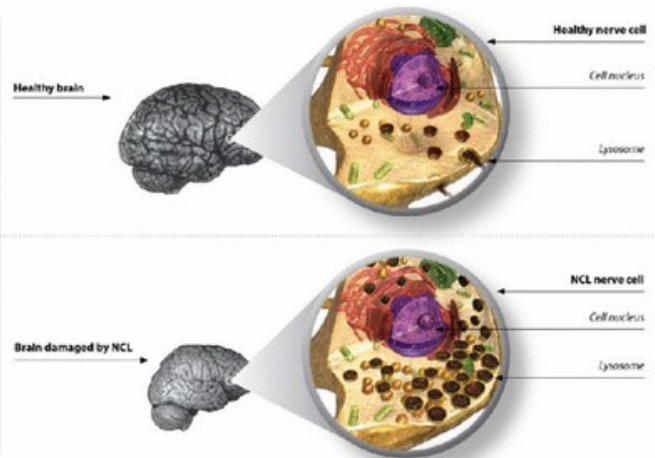


Batten Disease Overview

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

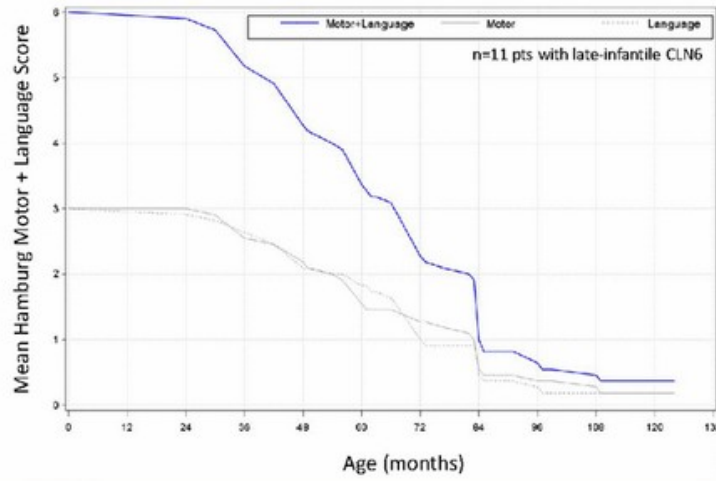
Disease Overview

- 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



CLN6 Natural History

CLN6 natural history shows a progressive decline of approximately one point per year in the Hamburg score from age two onwards with similar decline in motor and language

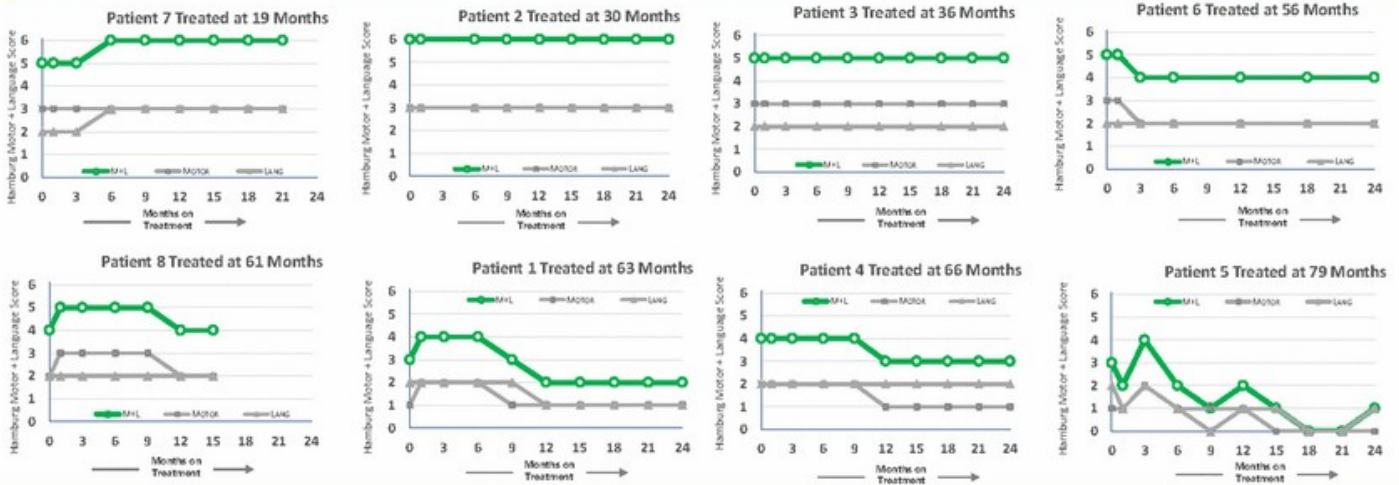


Source: Data on file: Ongoing Natural History study conducted by Nationwide Children's Hospital and Dr. Emily de los Reyes



Clinical Efficacy: Combined and Individual Hamburg Scores (n=8)

CLN6 gene therapy halts or substantially slows progression of disease with a positive impact on motor and language function in 7 out of 8 patients



Separate Motor and Language Scores are Consistent with the Respective Combined Score.

Source: Data on file

CLN6 Clinical Efficacy Data: Sibling Comparisons (Natural History)

Treated patients demonstrated stabilization relative to untreated siblings in the natural history data set who experienced substantial declines in motor and language ability

Treated AAV-CLN6 Patients vs Natural History Sibling with CLN6 (Hamburg Score: Motor + Language over time)

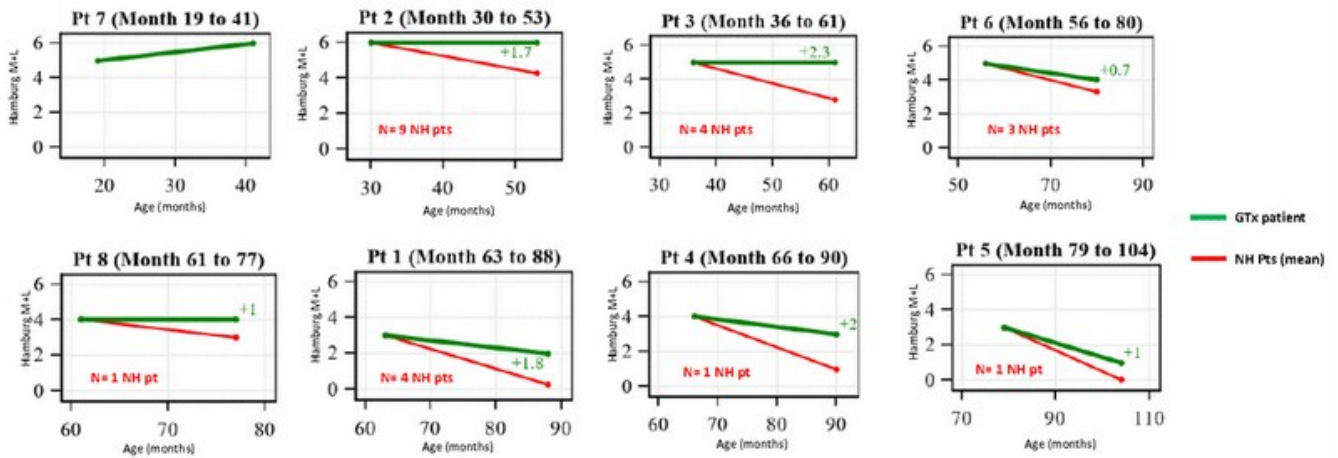


Source: Data on file



CLN6 Clinical Efficacy Data: Natural History Matched Comparisons

New analysis of treated patients demonstrate 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)

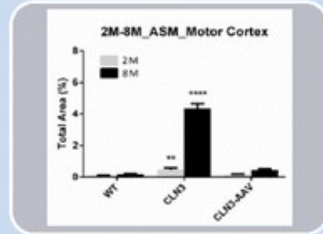
Source: Data on file



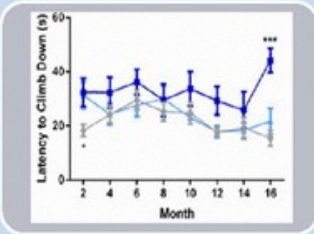
CLN3 Batten Disease: Preclinical and Clinical Summary

Amicus' second clinical stage gene therapy in CLN3 Batten disease has successfully completed dosing in three children in Cohort 1 (low dose) with dosing of additional Cohort 2 (high dose) patients in 2H 2019

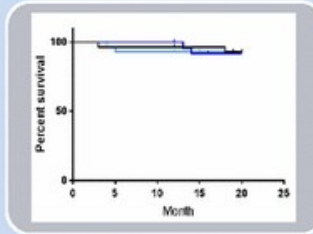
Preclinical Data in KO Mice



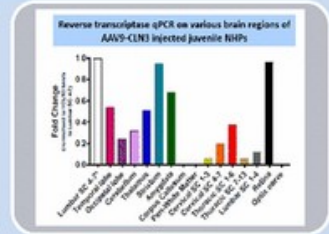
Reduction of storage material in mouse model



Improvement of motor function and cognitive behavior in mouse model



Comparable survival in mouse model

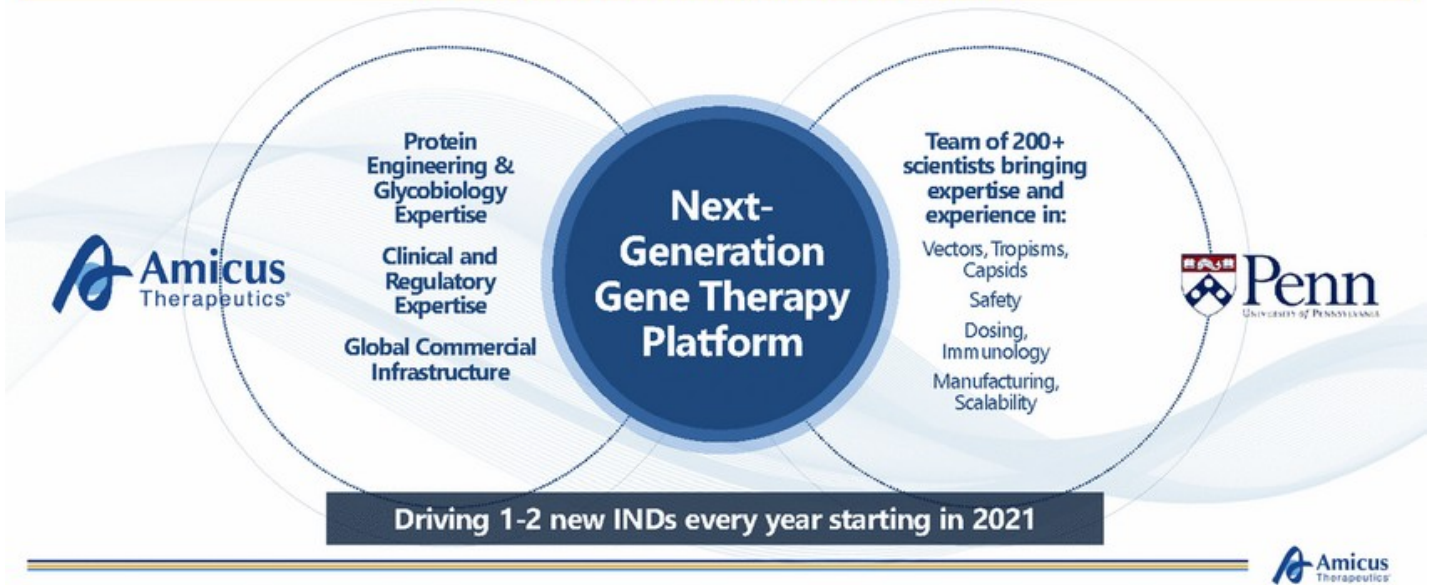


Widespread gene expression in brain of NHPs

Source: Data on file

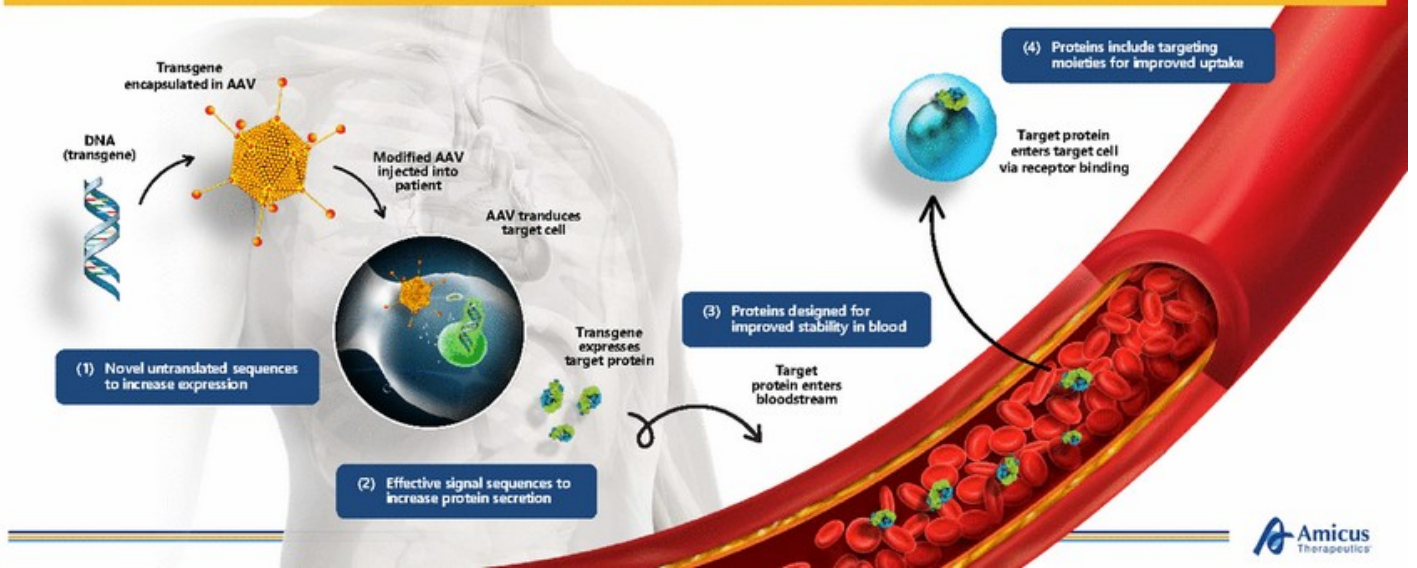
Combines Amicus and Penn Expertise Across Lysosomal and Rare Diseases

An R&D platform with rights to 50+ diseases



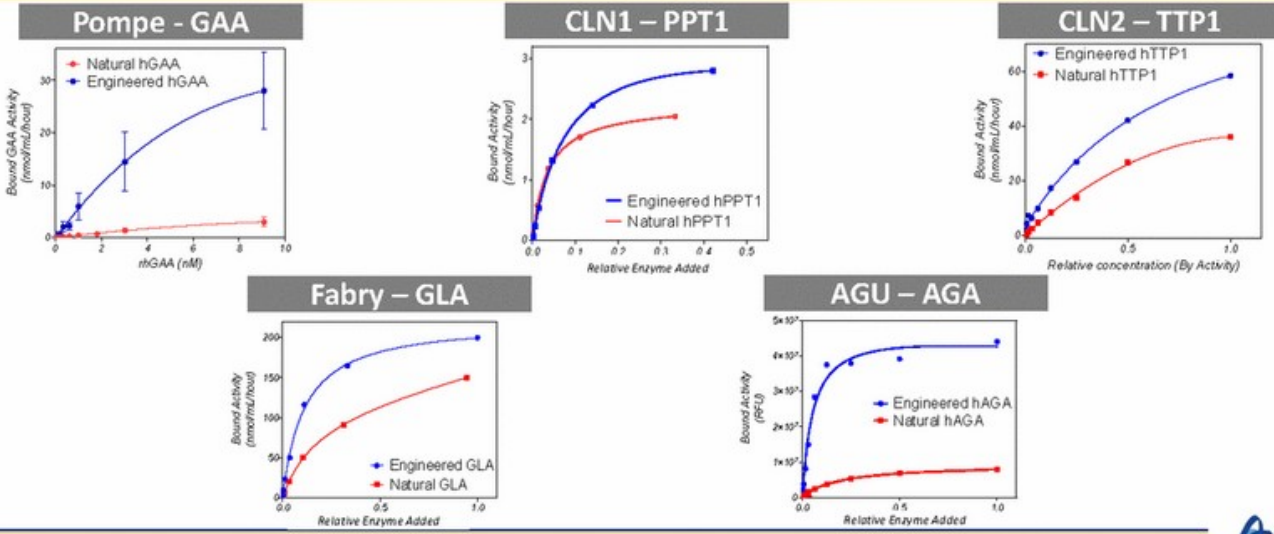
Amicus Approach: Engineered Transgenes for Optimal Cross-Correction

Amicus' unique technologies for protein engineering in Gene Therapy represent a new major platform technology and a groundbreaking advancement in the field



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

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

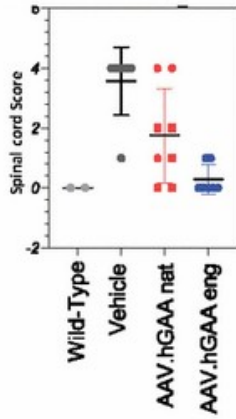


Source: Data on file

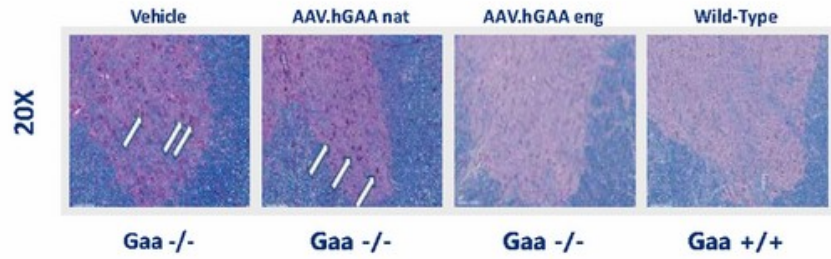
Initial Preclinical Pompe Gene Therapy Results: CNS

Only the AAV with the Amicus engineered hGAA transgene was able to significantly impact cell pathology and glycogen reduction in the CNS

Spinal Cord: Histopath



Spinal Cord: Glycogen PAS

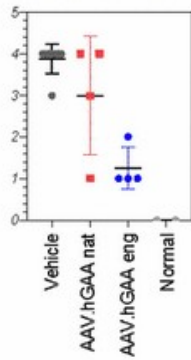


▪ Similar results observed in brain

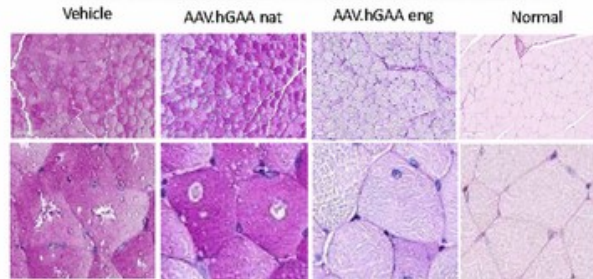
New Pompe Gene Therapy Low Dose Preclinical Data

Results from the low dose (2.5e12 gc/kg) study of engineered AAV-hGAA also showed improved cell pathology and glycogen reduction of the engineered construct versus natural GAA

Quadriceps: Histopath Scoring



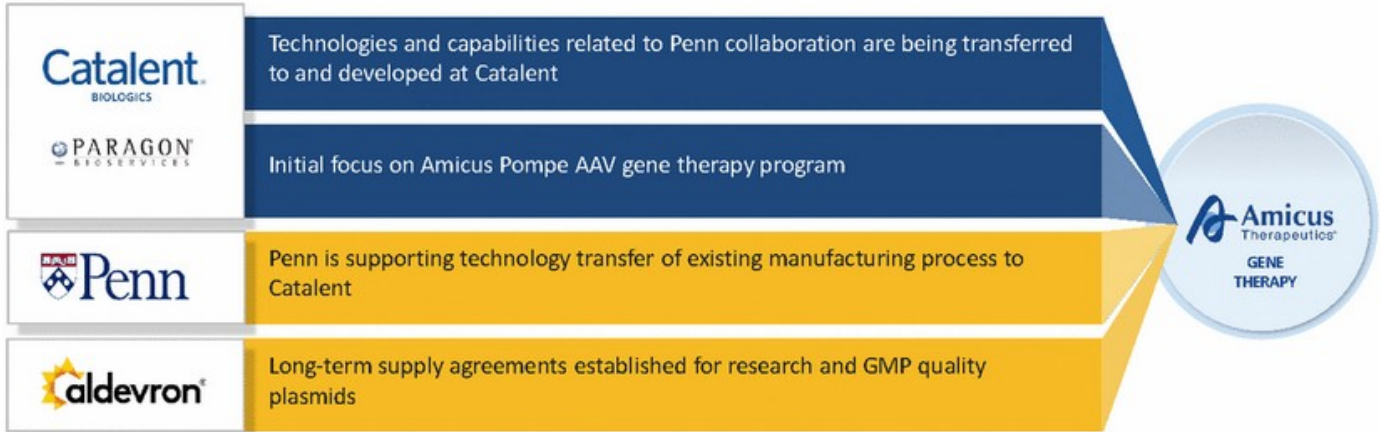
Quadriceps: Glycogen PAS



▪ Similar design as high dose preclinical study

GTx Manufacturing Strategy for Initial Penn Programs at Catalent

Catalent to leverage Penn's AAV manufacturing expertise and Amicus experience in complex biologics manufacturing and quality control as a competitive advantage.



Gene Therapy: Updates & Key Takeaways



Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future

- CLN6 Phase 1/2 interim data show profound impact with potential to become first ever approved gene therapy for fatal brain disease in children
- Additional patients to be dosed in Phase 1/2 study of CLN3 in 2021 with commercial supply
- Orphan drug designations granted in U.S. and EU for intrathecal AAV gene therapies for CLN6 and CLN3 Batten disease; CLN3 granted Fast Track designation by U.S. FDA
- Pompe gene therapy clinical candidate declared to move into IND-enabling studies
- Penn Collaboration is R&D engine, with rights to 50+ diseases
- 7 preclinical gene therapies in development



Financial & Operational Strategy

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

Financial Outlook: Key Takeaways

- Cash runway now well into 2H2022
 - Achieved through continued careful expense management, prioritization of very early stage research programs and more measured capital expenditures
- Non-GAAP quarterly operating expense expected to decline throughout 2020
- Company 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
- Only modest additional capital required in the outer years to extend runway into profitability with multiple non-equity sources available as/when needed



Financial Summary & Guidance

Strong Balance Sheet with \$338M+ Cash – Cash Runway Well into 2H2022

FINANCIAL POSITION

Cash	~\$338M+
Cash Runway¹	Well Into 2H2022
Debt^{1,2}	\$152.8M

CAPITALIZATION

Shares Outstanding (as of 3/31/2020)	257,449,995
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FINANCIAL GUIDANCE

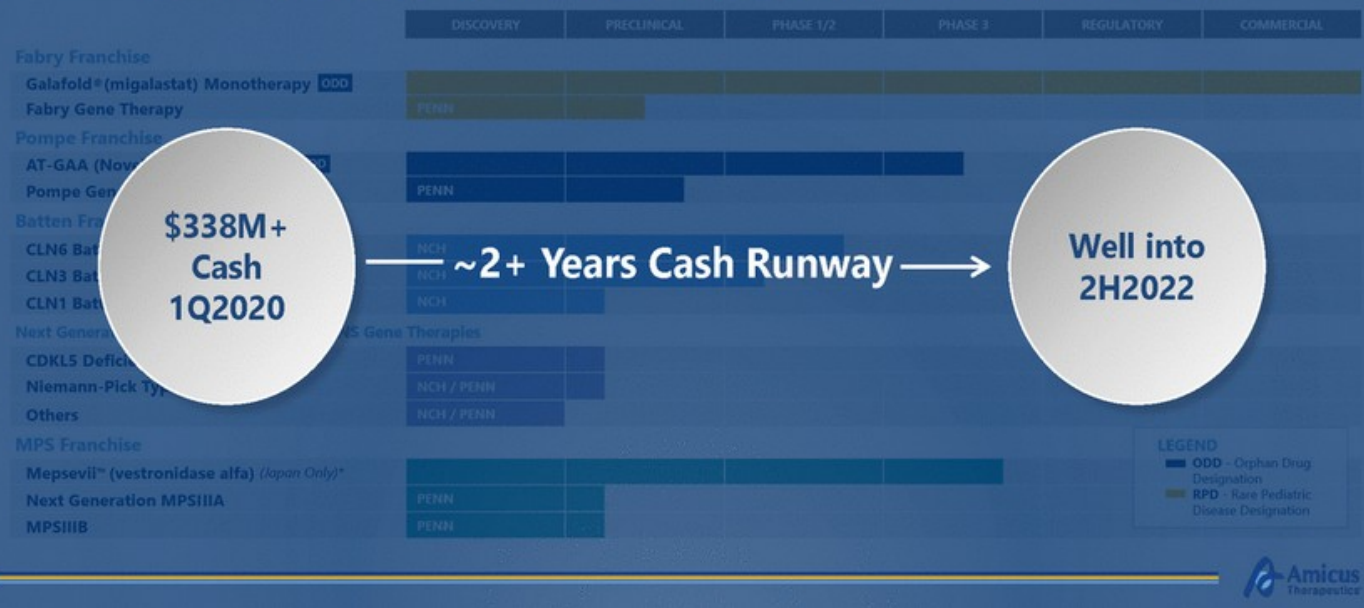
FY20 Galafold Revenue Guidance	\$250M-\$260M
FY20 Non-GAAP Operating Expense Guidance	\$410M-\$420M

¹Based on existing operating plan ²Includes \$2.8 million of convertible debt and \$150 million of straight debt



Cash Runway Now to Well into 2H2022 (~2+ years)

Fully funded through major milestones in portfolio and continued global growth



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

Our Passion for Making a Difference Unites Us

Amicus is now at a major inflection point and positioned to create significant shareholder value ahead while advancing our mission for patients.



Appendix



Non-GAAP Reconciliation

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

	Three Months Ended	
	March 31,	
	2020	2019
Total operating expenses - as reported GAAP	\$ 132,030	\$ 111,270
Research and development:		
Share-based compensation	5,253	5,032
Selling, general and administrative:		
Share-based compensation	7,343	7,712
Changes in fair value of contingent consideration payable	931	1,383
Depreciation and amortization	1,764	991
Total operating expense adjustments to reported GAAP	15,291	15,118
Total operating expenses - as adjusted	\$ 116,739	\$ 96,152