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 28, 2019**

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:

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

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 1.01. Entry into a Material Definitive Agreement.

On May 28, 2019, Amicus Therapeutics, Inc. ("Amicus" or the "Company") entered into an Amended and Restated Research, Collaboration and License Agreement (the "Agreement") with The Trustees of the University of Pennsylvania ("Penn") pursuant to which Amicus and the Wilson Laboratory at Penn will continue to collaborate with respect to the pre-clinical research and development of next generation parvovirus gene therapy products for the treatment of Pompe disease, Fabry disease and CDKL5 deficiency and will initiate pre-clinical research and development of next generation parvovirus gene therapy products for the treatment of Niemann Pick Type C, Mucopolysaccharidosis Type IIIA and Mucopolysaccharidosis Type IIIB (collectively, the "Indications").

Under the Agreement, Amicus also has the right to initiate additional programs for pre-clinical research and development of next generation parvovirus gene therapy products for the treatment of certain lysosomal storage diseases and other rare metabolic disorders specified in the Agreement.

As under the Research, Collaboration and License Agreement we entered into with Penn on October 8, 2018 ("Original Agreement"), under the Agreement, Penn granted the Company exclusive, worldwide licenses (with the right to sublicense) under certain patent rights arising out of the research programs or covering an Amicus-selected parvovirus gene therapy product developed at Penn and non-exclusive, worldwide licenses (with the right to sublicense) under certain patent rights pertaining to manufacturing, background patent rights and know-how, in each case, to make, have made, use, sell, offer for sale and import licensed products for the Indications.

As under the Original Agreement, the pre-clinical research and development activities of Penn under the Agreement will be overseen by James M. Wilson, M.D., Ph.D. and will be conducted by the Wilson Laboratory at Penn in accordance with a mutually-agreed research plan for a specified period of time and the Company will fund such research and development activities in accordance with a mutually-agreed budget. As under the Original Agreement, the Company will be responsible for clinical development and commercialization of the licensed products for the Indications and is obligated to make certain milestone and royalty payments with respect to licensed products for each Indication.

In addition, the Wilson Laboratory will conduct a discovery research program in accordance with a mutually-agreed research plan for five years beginning on the date of the Agreement. The Company will provide $10 million each year during such five year period to fund such discovery research program. Pursuant to the Agreement, in connection with the Company's funding of the discovery research program, the Company is eligible to receive a low double-digit percentage share of revenue Penn receives from third-party licensees of certain patent rights generated in the discovery research program.

Following the effective date of the Agreement, the Company paid to Penn an option fee in the low single-digit millions to select the Niemann Pick Type C, Mucopolysaccharidosis Type IIIB and next generation Mucopolysaccharidosis Type IIIA Indications. Following the selection of each additional Indication, the Company will be obligated to pay Penn an option fee in the low single-digit millions.

If Amicus terminates the Agreement during the term of the pre-clinical research and development activities or the discovery research program, other than for Penn's uncured breach or bankruptcy, the Company will be obligated to pay Penn any portion of the initial budget for such pre-clinical research and development activities or discovery research program, as applicable, that has not yet been paid.

The foregoing description of the Agreement does not purport to be complete and is subject to, and qualified in its entirety by, the full text of such agreement. The Company intends to seek confidential treatment for certain portions of the Agreement and expects to file a copy of the Agreement as an exhibit to the Company's Quarterly Report on Form 10-Q for the quarter ending June 30, 2019.

Item 8.01. Other Events

On May 29, 2019, the Company issued a press release announcing a major expansion of its gene therapy collaboration with the University of Pennsylvania. A copy of this press release is filed as Exhibit 99.1 to this Current Report on Form 8-K. In addition, the Company will be using the presentation attached as Exhibit 99.2 to this Current Report on Form 8-K during its conference call and live audio webcast. Both exhibits are incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits:

Exhibit No.	Description
99.1	Press Release dated May 29, 2019 titled, "Amicus Therapeutics and the University of Pennsylvania Announce Major Expansion of Gene Therapy Collaboration."
99.2	May 29, 2019 Presentation

Signatures

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 29, 2019

By: /s/ Ellen S. Rosenberg

Name: Ellen S. Rosenberg

Title: Chief Legal Officer and Corporate Secretary

Amicus Therapeutics and the University of Pennsylvania Announce Major Expansion of Gene Therapy Collaboration

Exclusive Disease-Specific Worldwide Rights to Penn's Next Generation Gene Therapy Technologies from the Wilson Lab for the Majority of Lysosomal Disorders

Current Collaboration Extended to Include Three New Indications: Niemann-Pick Type C (NPC), Mucopolysaccharidosis Type IIIA (MPS IIIA) and Mucopolysaccharidosis Type IIIB (MPS IIIB)

New Research Programs also Encompass 12 Additional Rare Diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy and Select Other Muscular Dystrophies

Robust Amicus R&D Engine Created through Combination of Amicus Expertise in Protein Engineering with Penn's Vector Technology, Translational Science, Manufacturing and Immunology Capabilities

Amicus Now Positioned with Industry Leading Gene Therapy Pipeline in Rare Diseases

Conference Call Today at 8:30am

CRANBURY, NJ and Philadelphia, PA, May 29, 2019 – Amicus Therapeutics (Nasdaq: FOLD) and the Perelman School of Medicine at the University of Pennsylvania (Penn) today announced a major expansion to their collaboration with rights to pursue collaborative research and development of novel gene therapies for lysosomal disorders (LDs) and 12 additional rare diseases. The collaboration has been expanded from three to six programs for rare genetic diseases and now includes: Pompe disease, Fabry disease, CDKL5 deficiency disorder (CDD), Niemann-Pick Type C (NPC), Mucopolysaccharidosis Type IIIB (MPS IIIB), as well as a next generation program in Mucopolysaccharidosis Type IIIA (MPS IIIA), both also known as part of Sanfillipo Syndrome. In addition to these three new programs, a discovery research agreement provides Amicus with exclusive disease-specific access to rights to collaborate with Penn's Gene Therapy Program (GTP) to develop potentially disruptive new gene therapy platform technologies and programs for the majority of lysosomal disorders and 12 additional rare diseases.

John F. Crowley, Chairman and Chief Executive Officer of Amicus stated, "The extension of our collaboration with Penn is a bold step forward in our commitment to create potential cures that may alleviate an enormous amount of suffering for countless numbers of people in the world living with rare diseases, many of them children. Together with Penn we are now able to focus on additional lysosomal disorders, as well as several more prevalent rare diseases for which we can apply our understanding of underlying disease biology in rare metabolic disease, Amicus' protein-engineering and development expertise and the world renowned capabilities of Dr. Jim Wilson's laboratory to develop novel gene therapy candidates. With a globally approved precision medicine product for Fabry, a late-stage biologic product with breakthrough therapy designation for Pompe, and now the industry's largest rare disease gene therapy pipeline, Amicus is well-positioned to become a leading global biotechnology company at the forefront of human genomic medicine."

Building off the initial success of the ongoing Amicus-Penn collaboration, including compelling initial preclinical proof-of-concept data in Pompe disease, this expanded relationship will continue to combine Amicus' protein engineering and glycobiology expertise with Penn's gene transfer technologies to develop novel gene therapies designed for optimal cellular uptake, targeting, dosing, safety and manufacturability.

“This agreement is a significant step forward in creating a world class industry-academia gene therapy partnership in rare diseases,” said James M. Wilson, MD PhD, Professor of Medicine and Pediatrics at the Perelman School of Medicine. “We have already seen highly encouraging preclinical results and proof-of-concept in Pompe disease through our existing collaboration and are excited by what we can further achieve together. We are looking forward to expanding the relationship further for additional preclinical programs and committing to the research required to further advance the technology platforms at Penn. We have seen the first results of our combined capabilities and platforms and I believe that we can further expand and accelerate our efforts to rapidly develop gene therapies for many more patients with unmet needs.”

“Penn Medicine has put Philadelphia on the map as the global epicenter of gene therapy research and development, and under the leadership and vision of Jim Wilson, our expanded agreement with Amicus is an exciting milestone for a field which is in the midst of transformative breakthroughs,” said J. Larry Jameson, MD, PhD, Executive Vice President of the University of Pennsylvania for the Health System and Dean of the Perelman School of Medicine. “We are thrilled to be part of this collaboration, which will help to bolster our city’s growing reputation as a magnet for talent and an engine for gene therapy innovation.”

Extended Research, Collaboration and License Agreement for Six Rare Metabolic Diseases
Penn’s vector technology is designed to improve targeting, tropism, safety, immunogenicity, and gene delivery, while Amicus’ protein engineering capabilities may optimize protein expression, secretion, targeting and uptake of the target protein. The agreement between Amicus and Penn is a Research, Collaboration and License Agreement, with funding provided to Penn to advance the preclinical research programs in the Wilson Lab and to license certain technologies invented under the funded Research Collaboration. The initial collaboration program focused on developing innovative new gene therapies for Pompe disease, Fabry disease, CDD and one additional undisclosed rare metabolic disorder. With the extension of the agreement, NPC and MPS IIIB, as well as a next-generation program for MPS IIIA, have now been added as three additional collaboration programs.

New Next Generation Research Program: R&D Engine for LDs and Additional Rare Diseases
Under a new five-year next-generation research agreement, Penn will conduct discovery research to develop potentially disruptive new gene therapy technologies. Amicus will continue to advance its own research and technology platforms to combine with Penn’s technologies, which can be used in the collaborative research programs for the disease indications. Terms of the agreement include a $10 million annual investment from Amicus, each year for five years, into GTP’s discovery research program with the ability to extend. Amicus will receive exclusive disease-specific rights to collaborate with GTP to research and develop products for many lysosomal disorders. The Amicus rights for additional collaborative research programs also include additional rare diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy and select other muscular dystrophies. For many of these indications, there is potential to apply protein-engineering and targeting motifs to enable cross-correction with next-generation gene therapy technology.

Conference Call and Webcast on May 29, 2019 at 8:30 a.m. ET
Amicus Therapeutics will host a conference call and audio webcast today, May 29, 2019 at 8:30 a.m. ET to discuss the expanded collaboration. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international), conference ID: 1695255.

A live audio webcast and accompanying slide deck can also be accessed via the Investors section of the Amicus Therapeutics corporate website at http://ir.amicusrx.com/, and will be archived for 30 days. Web participants are encouraged to register on the website 15 minutes prior to the start of the call. A replay of the call will be available for seven days beginning at 11:30 a.m. ET on May 29, 2019. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 1695255.

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.

About the Perelman School of Medicine at the University of Pennsylvania

Penn Medicine is one of the world's leading academic medical centers, dedicated to the related missions of medical education, biomedical research, and excellence in patient care. Penn Medicine consists of the *Raymond and Ruth Perelman School of Medicine at the University of Pennsylvania* (founded in 1765 as the nation's first medical school) and the *University of Pennsylvania Health System*, which together form a $7.8 billion enterprise. The Perelman School of Medicine has been ranked among the top medical schools in the United States for more than 20 years, according to U.S. News & World Report's survey of research-oriented medical schools. The School is consistently among the nation's top recipients of funding from the National Institutes of Health, with $425 million awarded in the 2018 fiscal year. The University of Pennsylvania Health System's patient care facilities include: the Hospital of the University of Pennsylvania and Penn Presbyterian Medical Center—which are recognized as one of the nation's top "Honor Roll" hospitals by U.S. News & World Report—Chester County Hospital; Lancaster General Health; Penn Medicine Princeton Health; and Pennsylvania Hospital, the nation's first hospital, founded in 1751. Additional facilities and enterprises include Good Shepherd Penn Partners, Penn Home Care and Hospice Services, Lancaster Behavioral Health Hospital, and Princeton House Behavioral Health, among others. Penn Medicine is powered by a talented and dedicated workforce of more than 40,000 people. The organization also has alliances with top community health systems across both Southeastern Pennsylvania and Southern New Jersey, creating more options for patients no matter where they live. Penn Medicine is committed to improving lives and health through a variety of community-based programs and activities. In fiscal year 2018, Penn Medicine provided more than $525 million to benefit our community.

Forward Looking Statement

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to the collaboration with the University of Pennsylvania, and the development of potential gene therapy product candidates. 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, the benefits of this collaboration may never be realized, 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 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; the potential that we will need additional funding to complete all of our studies and manufacturing and the potential that certain individuals may not continue to support the development of product candidates. . 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 as well as our Quarterly Report on Form 10-Q for the quarter ended March 31, 2019. 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.

Penn's Financial Disclaimer - As an inventor of technology licensed or optioned to Amicus pursuant to this Gene Therapy Collaboration, Dr. Wilson and Penn may receive additional financial benefits under the license in the future.

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FOLD–G

Amicus Therapeutics

Amicus Therapeutics Expands Gene Therapy Collaboration with University of Pennsylvania

May 29, 2019

Forward-Looking Statements

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 rela preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical tr prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be re 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 o wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with re statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potentia progress, timing, and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this release du risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indica the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potent regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or our other product candidates if an approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issu potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need ad funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be pr of future results. With respect to statements regarding projections of the Company's revenue and cash position, actual results may differ b market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2018 and Quarterly report on Form 10-Q for the quarte March 31, 2019. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date he forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update th release to reflect events or circumstances after the date hereof.

Amicus Gene Therapy Expansion

Transforms Amicus into a True Platform Gene Therapy and Protein Engineering Company at t Forefront of Human Genomic Medicine with Industry Leading Gene Therapy Pipeline in Rare Dis

- Partnership Combines Amicus' Protein Engineering Platform and Glycobiology Expertise with Penn's Gene Transfer Technologies
- Accelerates Efforts to Develop Novel Gene Therapies for Optimal Cellular Uptake, Targeting, Dosing, Safety and Manufacturability
- Exclusive Disease-Specific Worldwide Rights to Penn's Next Generation Gene Therapy Technologies from the Wilson Lab for the Majority of Lysosomal Disorders
- Fulfills Amicus Mission to Develop and Deliver Medicines as Quickly as Possible for Many More Thousands of People Living with Rare Diseases
- Establishes Industry Leading Rare Disease Gene Therapy Portfolio and Creates an R&D Engine within the Lysosomal Disorders while Applying Scientific Expertise to Broader Rare Disease Indications

"This agreement is a significant ste forward in creating a world class industry-academia gene therapy partnership in rare diseases."

- Jim

"The extension of our collaboration Penn is a bold step forward in our commitment to create potential cur that may alleviate an enormous am of suffering for countless numbers people in the world living with rare diseases, many of them children."

- John C

Expanded Amicus-Penn Collaboration Terms

World Class Industry-Academia Partnership in Rare Diseases

- Exclusive Disease-Specific Worldwide Rights to Penn's Next Generation Gene Therapy Technologies from the Wilson Lab for th Majority of Lysosomal Disorders
- Current Collaboration Extended to Include Three New Indications: Niemann-Pick Type C (NPC) Mucopolysaccharidosis Type IIIA (MPS IIIA), and Mucopolysaccharidosis Type IIIB (MPS IIIB)
- New Research Programs also Encompass 12 Additional Rare Diseases, including Rett Syndrome, Angelman Syndrome, Myotor Dystrophy and Select Other Muscular Dystrophies
- Robust R&D Engine Created through Combination of Amicus Expertise in Protein Engineering with Penn's Vector Technology, Translational Science, Manufacturing and Immunology Capabilities
- Amicus to Invest $10M / Year for 5 Years for Research to Improve Safety, Efficacy and Manufacturability of Next Generation Vec with Option to Extend
- Amicus to Contribute Protein Engineering Platform Technology and Capabilities to Collaboration in Lieu of Any Additional Upfro Payments to Penn
- Amicus to Assume Development Costs for NPC, Next Generation MPS IIIA, and MPS IIIB, to begin immediately*

*Sufficient cash into 2021 including additional spend required under the Amicus-Penn collaboration

Expansion of Amicus and Penn Gene Therapy Collaboration

Amicus Global Research and Gene Therapy Center of Excellence in Philadelp

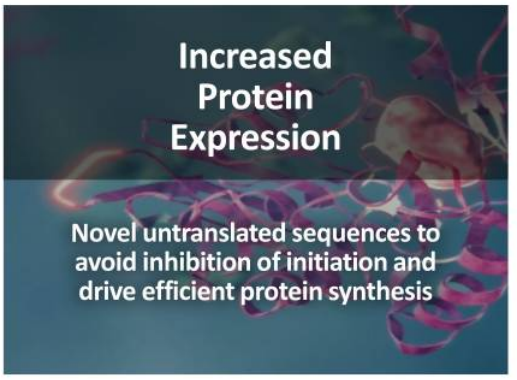
Strengthens Amicus Capabilities as a Leading Global Rare Disease Biotechnology Company in Burgeoning Hub for Medical Breakthroughs

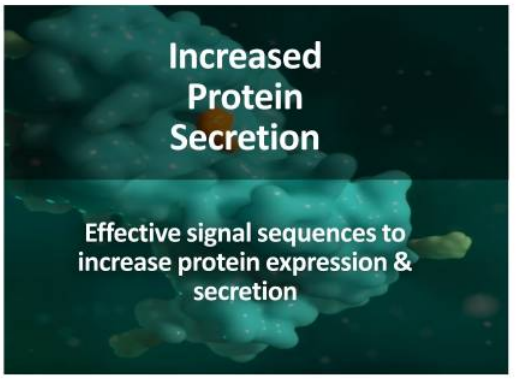
- New 75,000 sqft state-of-the-art facility
- Located in uCity Square Adjacent to Penn Campus
- Global Amicus science organization and gene therapy leadership team headquarters
- Co-led by Jeff Castelli, PhD, Chief Portfolio Officer and Head of Gene Therapy and Hung Do, PhD, Chief Science Officer
- Initial group occupying temporary space
- Permanent space to eventually hold ~200 employees

Artist rendering

Amicus Protein Engineering Expertise & Technologies for Gene Therap

Collaboration to Enable Greater Protein Expression and Delivery at Lower Gene Therapy Dos





GENE THERAPY PROGRAM

Penn

MPS IIIB Disease Overview

Fatal Lysosomal Disorder with Progressive Clinical Manifestations in Central Nervous System (CNS) and Spinal Cord

- Mutation of alpha-N-acetylglucosaminidase (NAGLU) leads to accumulation of glycoaminoglycans (GAGs) causing lysosomal dysfunction and neuroinflammation
- Signs and symptoms typically begin in early childhood
  - Initial behavioral disorders and intellectual deterioration, and sleep disorders
  - Loss of motor milestones and communication problems at age ~10 years
  - Seizures often occur after age 10
- Death occurs in the second decade in most cases
- Estimated to occur in 1 in 200,000 births

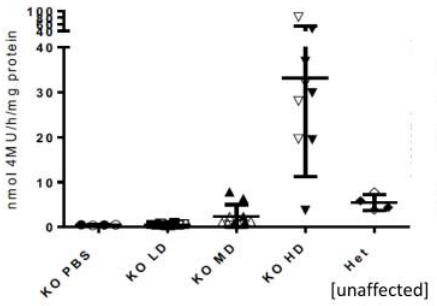
*Sources: www.orpha.net, NORD

MPS IIIB Program Summary

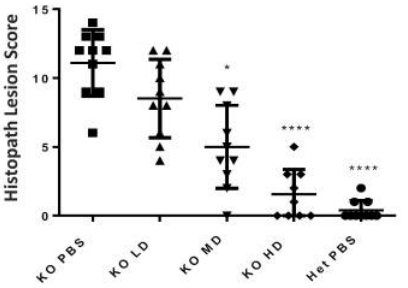
MPS IIIB program will Assess Whether Existing AAV-NAGLU Candidate can be Fur Improved to Enhance Cellular Uptake and Cross-Correction of Affected Cells

AAV-NAGLU Candidate (Unmodified WT): KO Mouse*

Brain NAGLU Activity

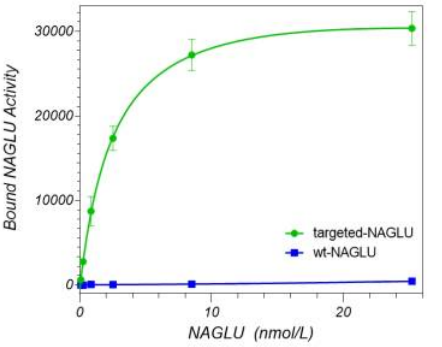


Brain Histopath Score



- AAV-NAGLU candidate (unmodified); 3 single ICV doses (LD, MD, HD)
- 4-mo old KO mice (n=10); Sacrificed 90-days post injection

NAGLU Binding to Intended Recepto

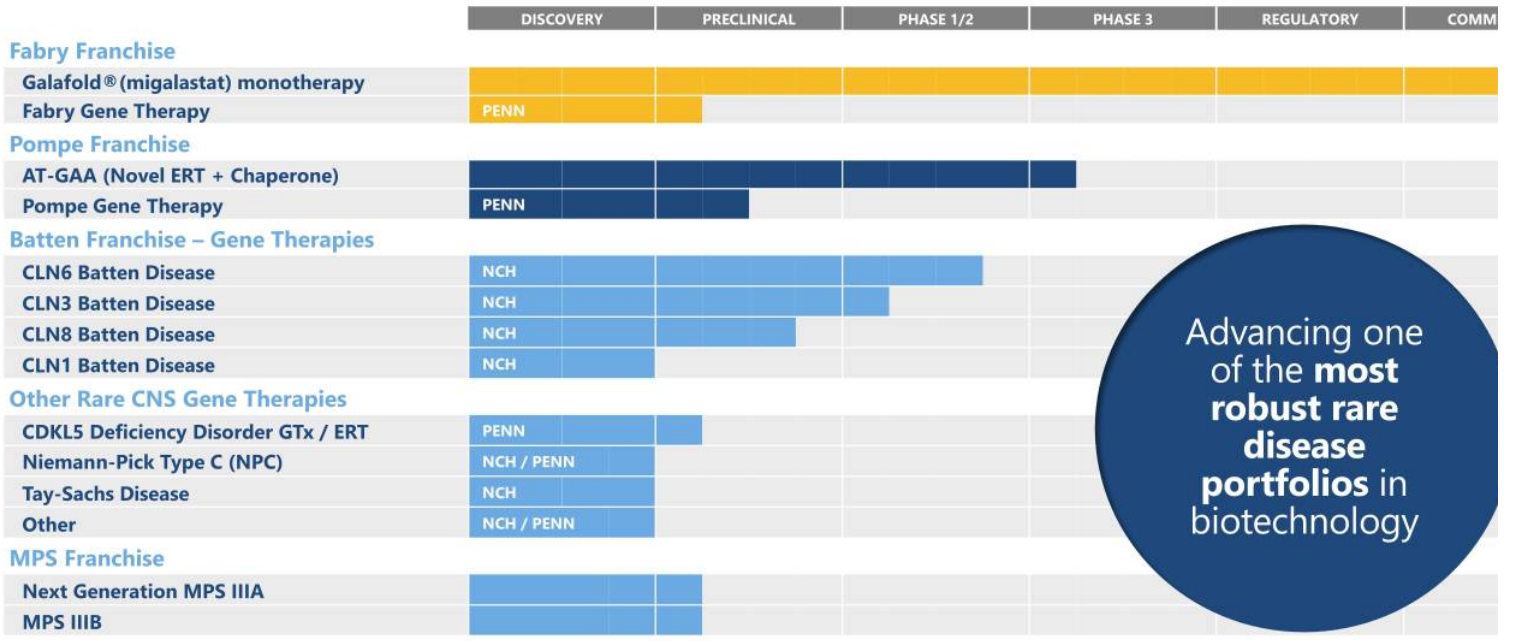


- Potential to engineer NAGLU transgene for improved receptor binding and cellular uptake

*Penn Data **Amicus Data

Expansion of Amicus and Penn Gene Therapy Collaboration

A RARE PORTFOLIO.



Advancing one of the **most robust rare disease portfolios** in biotechnology

Rare Disease Operational Excellence

**Proven Capabilities Position Amicus at the Forefront of Human Genomic Medicin
Develop and Deliver the Highest Quality Therapies and Cures for Persons Living w
Rare Diseases**

5 Key Pillars of Value

Innovation/ Science

Global development

Global commercial

Manufacturing

Partnerships

Anticipated Milestones: 2019

Well-Positioned to Create Significant Value for Patients and Shareholders in 20

Galafold: Fabry Disease

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

AT-GAA: Pompe Disease

- ✓ Additional Phase 1/2 data (21 and 24 months)
- ✓ Breakthrough therapy designation (BTD) in LOPD
- ✓ Phase 1/2 study fully enrolled (Cohorts 1-4)
- PROPEL pivotal study enrollment (n=100)
- Additional Phase 1/2 data (Cohort 4)
- Natural history study data
- Additional supportive studies
- Advance CMC requirements to support BLA

Gene Therapy Programs

- Ongoing CLN3 Batten disease Ph 1/2 study enrollment
- Additional 2-year data from CLN Batten disease Phase 1/2 study
- Additional preclinical data inclu next-generation gene therapies Fabry and Pompe
- Selection of Pompe gene thera clinical candidate to move into enabling studies

Expansion of Amicus and Penn Gene Therapy Collaboration

A RARE VISION. Impacting Lives

>350 Patients* | ~$36M Global Sales

YE17

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

YE18

5,000 Patients* | $1B Global Sa

2023

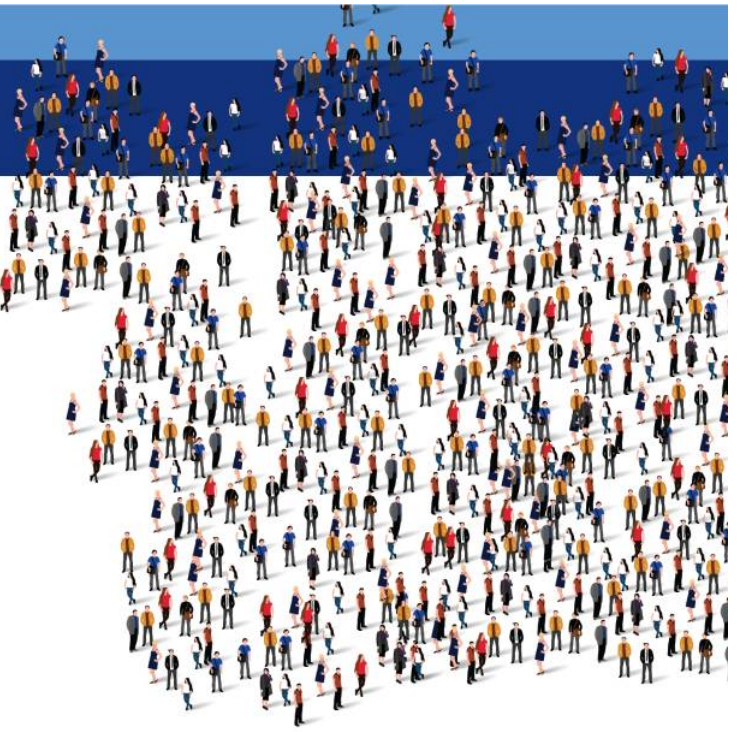
*Clinical & commercial, all figures approximate [1]Preliminary unaudited

Expansion of Amicus and Penn Gene Therapy Collaboration

A RARE VISION.

A Leader in the Human Genome Medicine Revolution

5,000 Patients* $1B Global Sales



MORE Patients More Indications

2023

LONGER-TERM VISION

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

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement

Amic
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