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): **April 30, 2019**

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

Delaware	
(State or Other Jurisdiction of Incorporation)	
001-33497	**71-0869350**
(Commission File Number)	(IRS Employer Identification No.)
1 Cedar Brook Drive, Cranbury, NJ	**08512**
(Address of Principal Executive Offices)	(Zip Code)

Registrant's telephone number, including area code: **(609) 662-2000**

(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))

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

Emerging growth company o

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. o

Item 8.01. Other Events

On April 30, 2019, Amicus Therapeutics, Inc. (the "Company") issued a press release announcing initial positive preclinical Pompe disease gene therapy data at the American Society of Gene & Cell Therapy ("ASGCT") 22nd Annual Meeting in Washington D.C. A copy of this press release is attached hereto as Exhibit 99.1. In addition, the Company will be using the presentation attached as Exhibit 99.2 during its conference call and webcast. Both exhibits are incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits

Exhibits:

Exhibit No.	Description
99.1	Press release dated April 30, 2019
99.2	ASGCT Presentation Materials

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: April 30, 2019

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

Amicus Therapeutics Presents Positive Preclinical Pompe Disease Gene Therapy Data at American Society of Gene & Cell Therapy (ASGCT) 22nd Annual Meeting

Preclinical Studies Show Robust Uptake and Glycogen Reduction in Multiple Tissues, Including Brain and Spinal Cord

Initial Validation for Collaboration Combining Amicus-Engineered Transgenes with Penn's AAV Gene Therapy Technologies

Significant and Broad New Amicus Platform Technology with Potential to Engineer Lysosomal Proteins to Enhance Targeting

Ongoing Preclinical Studies to Confirm Clinical Candidate Selection in 2019

Conference Call Today at 8:30am

CRANBURY, NJ, April 30, 2019 – Amicus Therapeutics (Nasdaq: FOLD) today announced initial preclinical data from its investigational adeno-associated viral (AAV) gene therapy program for Pompe disease in mice. These data will be highlighted in a poster (Poster 82, Abstract 518) entitled "Development of a Novel Gene Therapy for Pompe Disease: Engineered Acid Alpha-Glucosidase Transgene for Improved Expression and Muscle Targeting," at the American Society of Gene & Cell Therapy (ASGCT) 22nd Annual Meeting in Washington, D.C. today at 5:00 p.m. ET.

Pompe disease is an inherited lysosomal storage disorder caused by deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA lead to accumulation of glycogen in cells, which results in the clinical manifestations of Pompe disease. Amicus, in collaboration with the Gene Therapy Program of the Perelman School of Medicine at the University of Pennsylvania (Penn), is developing a novel gene therapy for Pompe disease that combines the Amicus protein-engineering and glycobiology expertise with Penn's adeno associated virus (AAV) gene transfer technologies.

This initial preclinical study used a single high dose of AAV in GAA knockout mice with either natural unmodified hGAA ("natural hGAA") or an Amicus/Penn engineered hGAA transgene with a lysosomal-targeting cell receptor binding motif ("engineered hGAA"). The Amicus/Penn engineered hGAA is designed for optimal expression, secretion, and targeting which enables efficient cross-correction in target tissues (via the binding motif).

Preclinical Poster Highlights for Amicus/Penn AAV Gene Therapy for Pompe Disease:

- The Amicus/Penn hGAA AAV gene therapy demonstrated more uniform cellular uptake and lysosomal targeting compared to natural hGAA AAV gene therapy.
- The engineered hGAA AAV gene therapy demonstrated robust glycogen reduction in all key tissues in Pompe disease that were assessed.
- In the central nervous system (CNS), the engineered hGAA AAV gene therapy showed robust glycogen reduction in neuronal cells, suggesting this may be an effective way to address neuronal aspects of Pompe disease. Natural hGAA AAV gene therapy did not show glycogen reduction in neuronal cells.
- Initial findings validate the Amicus/Penn collaboration, as well as the potential of this platform to design constructs that enhance protein targeting across multiple lysosomal disorders.
- Additional preclinical studies to evaluate this engineered hGAA with various doses and routes of AAV administration are underway.
- The Pompe AAV gene therapy program builds upon the protein engineering and manufacturing expertise used to successfully develop AT-GAA, the Company's late-stage enzyme replacement therapy (ERT)-chaperone treatment paradigm.

Hung Do, PhD, Chief Science Officer of Amicus Therapeutics, stated, “These very important preclinical results validate our capabilities to develop engineered GAA proteins that can efficiently cross-correct target cells and tissues via a gene replacement therapy for Pompe disease. This approach may be applicable to other lysosomal disorders as we continue to combine our Amicus protein engineering expertise, together with Penn’s vector engineering expertise, to develop novel gene therapies.”

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, added, “Developing a potential cure for Pompe has been a personal and professional goal for many years. These data are profound and it is extremely rewarding to see these preclinical results that show our Amicus engineered GAA is optimized for uptake into target tissues and gets to the right cellular compartments, especially in the central nervous system. These data also provide preliminary and compelling evidence that the Amicus technology to design constructs that enhance protein targeting may be a significant platform for multiple lysosomal disorders. As these data exceed our expectations, our preclinical studies are progressing well ahead of schedule and we now expect to select a clinical candidate in 2019 to move forward into IND-enabling studies. Our mission has always been to develop potential best in class medicines and that is precisely what we are doing with both our novel, late-stage treatment paradigm AT-GAA as well as this preclinical gene therapy program.”

Amicus is currently developing AAV gene therapies in collaboration with the Gene Therapy Program of the Perelman School of Medicine at the University of Pennsylvania (Penn) for Pompe disease, Fabry disease, CDKL5 deficiency disorder (CDD) and one additional undisclosed rare metabolic disease. The agreement between Amicus and Penn is a Research, Collaboration and License Agreement, providing funding to Penn to advance the preclinical research programs in the Wilson Lab and to license certain technologies invented under the funded Research Collaboration.

“Amicus has differentiated itself by focusing on proteins and protein engineering, with a specific track record in the lysosomal disorders, which I believe are critically important to developing AAV gene therapies that can safely and effectively address these diseases,” said James M. Wilson, MD PhD, Professor of Medicine and Pediatrics at the Perelman School of Medicine at Penn. “These initial preclinical results are a significant step in highlighting our collaboration to rapidly advance gene therapies into the clinic for patients with urgent unmet needs in a disease like Pompe. These results demonstrate that the Amicus gene therapy that we have jointly developed has the potential to address both the neuromuscular as well as the motor neuron aspects of Pompe disease.”

Conference Call and Webcast on April 30, 2019 at 8:30 a.m. ET

Amicus Therapeutics will host a conference call and audio webcast today, April 30, 2019 at 8:30 a.m. ET to discuss the preclinical data presented at ASGCT. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international), conference ID: 1866796.

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 April 30, 2019. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 1866796.

About Pompe Disease

Pompe disease is an inherited lysosomal storage disorder caused by deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA leads to accumulation of glycogen in cells, which results in the clinical manifestations of Pompe disease. The disease can be debilitating, and is characterized by severe muscle weakness that worsens over time. Pompe disease ranges from a rapidly fatal infantile form with significant impacts to heart function to a more slowly progressive, late-onset form primarily affecting skeletal muscle. It is estimated that Pompe disease affects approximately 5,000 to 10,000 people worldwide.

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 Statements

This press release contains "forward- looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to initial preclinical data from its investigational adeno-associated viral (AAV) gene therapy program for Pompe disease in mice and the potential implications of these data for the future advancement and development of a gene therapy for Pompe disease and other lysosomal disorders and development of potential platform technologies. Words such as, but not limited to, “look forward to,” “believe,” “expect,” “anticipate,” “estimate,” “intend,” "confidence," "encouraged," “potential,” “plan,” “targets,” “likely,” “may,” “will,” “would,” “should” and “could,” and similar expressions or words identify forward-looking statements. The forward looking statements included in this press release are based on management's current expectations and belief's which are subject to a number of risks, uncertainties and factors, including that the preliminary data based on a small patient sample and reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented, that the Company will be not able to demonstrate the safety and efficacy of AT-GAA, that later study results will not support further development, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize AT-GAA. In addition, all forward looking statements are subject to the other risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2018. As a consequence, actual results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these forward looking statements, which speak only of the date hereof. All forward looking statements are qualified in their entirety by this cautionary statement and we undertake no obligation to revise this press release to reflect events or circumstances after the date hereof.

CONTACTS:

Investors/Media:
Amicus Therapeutics
Sara Pellegrino, IRC
Vice President, Investor Relations & Corporate Communications
spellegrino@amicusrx.com

---

(609) 662-5044

Media:

Amicus Therapeutics
Marco Winkler
Director, Corporate Communications
mwinkler@amicusrx.com
(609) 662-2798

FOLD–G

Amicus Therapeutics®

Preclinical Pompe Gene Therapy Results at ASGCT

Conference Call & Webcast

April 30, 2019

Forward-Looking Statements

This presentation contains "forward- looking statements" within the meaning of the Private Securities Litigation Reform Act of 1 including statements relating to initial preclinical data from its investigational adeno-associated viral (AAV) gene therapy progr Pompe disease in mice and the potential implications of these data for the future advancement and development of a gene ther for Pompe disease and other lysosomal disorders and development of potential platform technologies. Words such as, but not li to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "confidence," "encouraged," "potential," "plan," "targets," " "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. The forward lo statements included in this press release are based on management's current expectations and belief's which are subject to a nu of risks, uncertainties and factors, including that the preliminary data based on a small patient sample and reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented, that the Comp will be not able to demonstrate the safety and efficacy of AT-GAA, that later study results will not support further developmen even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize AT-GAA. In addition, all forward looking statements are subject to the ot risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2018. As a consequence, results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these fo looking statements, which speak only of the date hereof.

Pompe Disease Overview

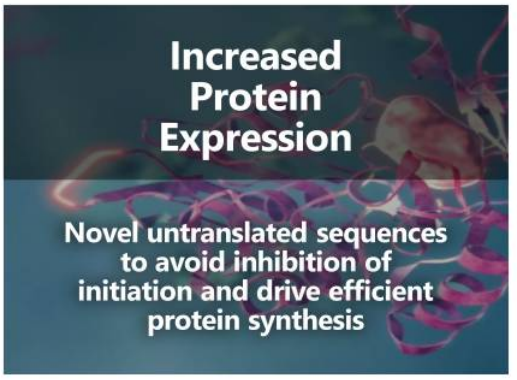
Pompe Disease is a Fatal Neuromuscular and Motorneuron Disorder that Affects a Broad Range of People

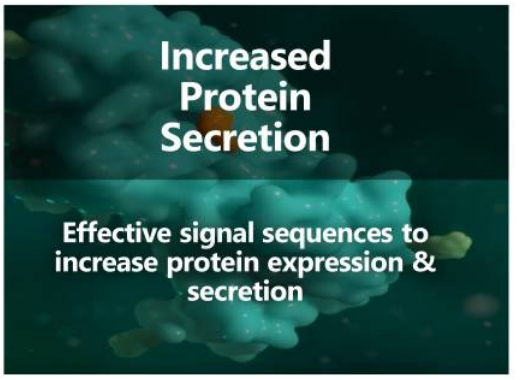
- 5,000 – 10,000+ patients diagnosed WW[1]; newborn screening suggests underdiagnosis
- Respiratory and cardiac failure are leading causes of morbidity and mortality
- Age of onset ranges from infancy to adulthood
- Deficiency of GAA leading to glycogen accumulation and cellular dysfunction
- Symptoms include muscle weakness, respiratory failure, and cardiomyopathy
- ~$900M+ Global Pomp ERT sales in FY18[2]

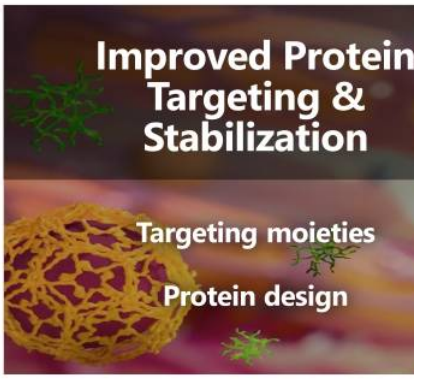
1. National Institute of Neurological Disorders and Stroke (NIH). 2. Sanofi Press Release & 10-K

Amicus Protein Engineering Expertise & Technologies for Gene Therap

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

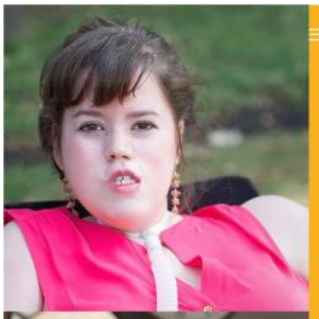






GENE THERAPY PROGRAM

Penn

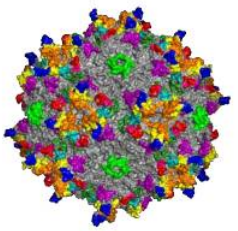


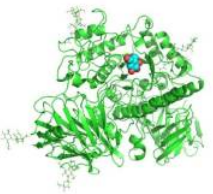
Development of a Novel Gene Therapy for Pompe Disease: Engineered Acid Alpha-Glucosidase Transgene for Improved Expression and Muscle Targeting

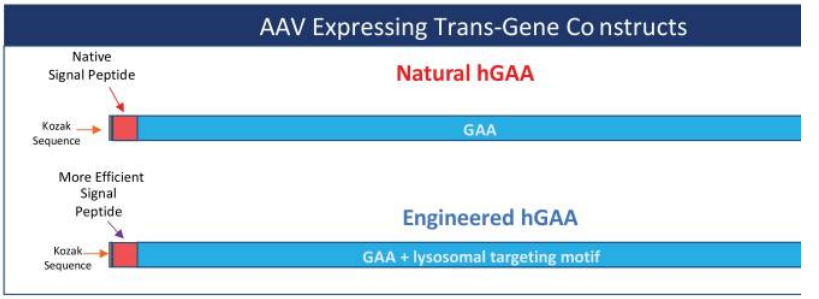
American Society of Gene & Cell Therapy (ASGCT) 22nd Annual Meetin
April 30, 201

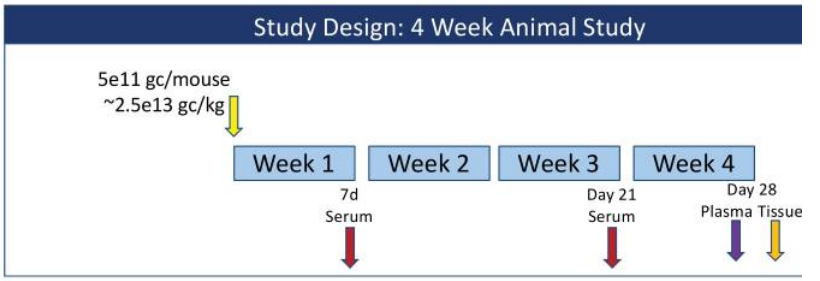
AAV Gene Therapy Initial High-Dose Preclinical POC Study

- AAV Transgene:
  - Natural-hGAA (AAV.hGAA nat)
  - Engineered-hGAA (AAV.hGAA eng)
- Dose\Route:
  - 5e11 gc/mouse (~2.5e13 gc/kg)
  - Tail Vein IV
- Animal Model:
  - Pompe Model *Gaa* -/- B6:129-GAAtm1Rabn/J (aka 6neo)
  - Wild-type *Gaa* +/+ (Pompe model litter-mates)
- Gender:
  - Male
  - Female
- Age: 4-6 weeks at AAV dosing

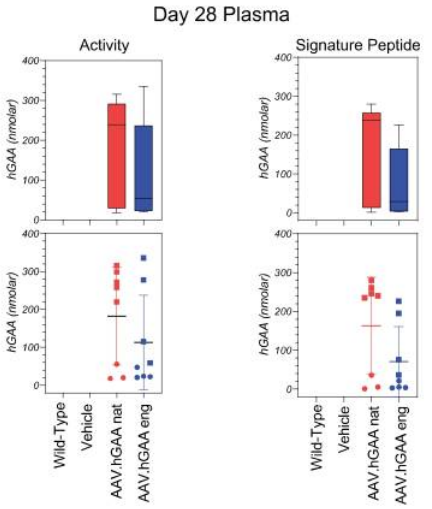




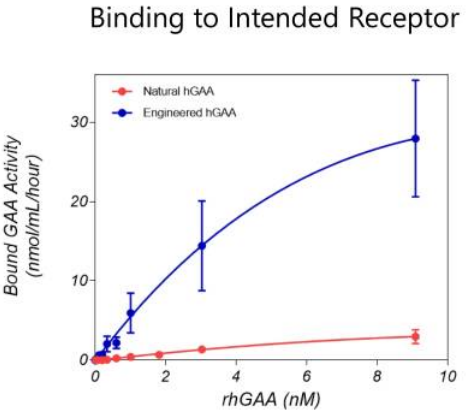




GAA Plasma Activity, Concentration and Cell-Surface Receptor Bindi



- High levels of engineered and natural hGAA were measured in plasma at day 28



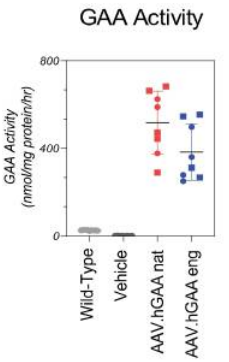
Day 28 Plasma Samples used to evaluate receptor binding

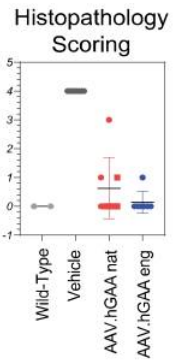
- Engineered hGAA was able to efficiently bind the intended receptor to enable cellular uptake

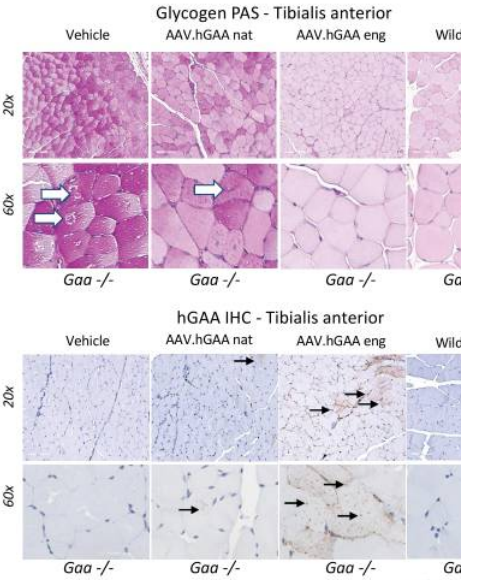
Tibialis Anterior – 28 days after AAV Gene Therapy

Engineered hGAA was more Efficient at Cross-Correction as Indicated by Greater Cellular Uptake, Uniform Glycogen Reduction ar Pathology Correction

- GAA activity in the tibialis anterior was ~15 – 20 fold higher than wild-type levels for both engineered hGAA and natural hGAA
- PAS staining showed more uniform and complete glycogen reduction and clearance of autophagic vacuoles for engineered hGAA
- PAS staining showed incomplete glycogen reduction and less clearance of autophagic vacuoles for natural hGAA (white arrows)
- IHC illustrated greater cellular uptake (black arrows) of engineered hGAA compared to natural hGAA
- Similar results seen in other muscle groups (see Appendix)





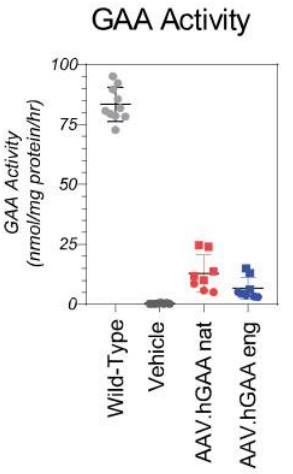


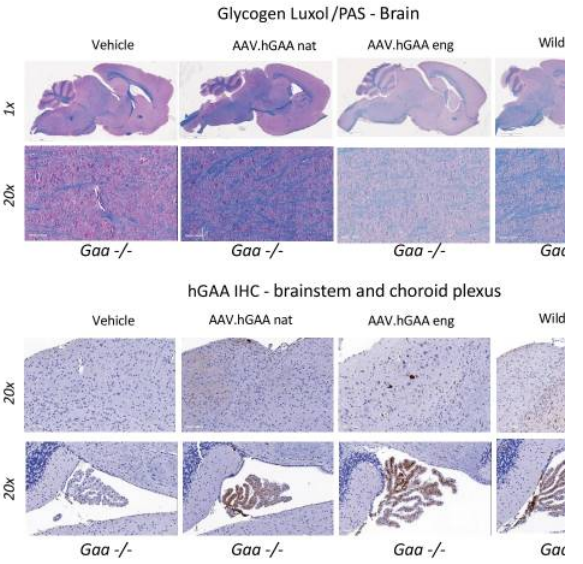
Periodic acid–Schiff (PAS) is a staining method used to detect polysaccharides such as glycogen in tissues

Brain– 28 days after AAV Gene Therapy

Engineered hGAA was able to Cross-Correct the Brain at Low Levels Due to Efficient Cellular Uptake while Natural hGAA was not a Cross-Correct at Similar Expression Levels

- GAA activity in the brain was ~5-fold lower than wild-type levels for both engineered hGAA and natural hGAA
- Both engineered hGAA and natural hGAA likely produced by the choroid plexus and secreted into the cerebrospinal fluid
- Glycogen close to wild-type levels for engineered hGAA, even though activity was only 20% of wild-type levels
- Little/no glycogen clearance with natural hGAA

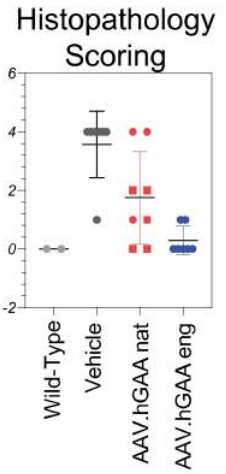


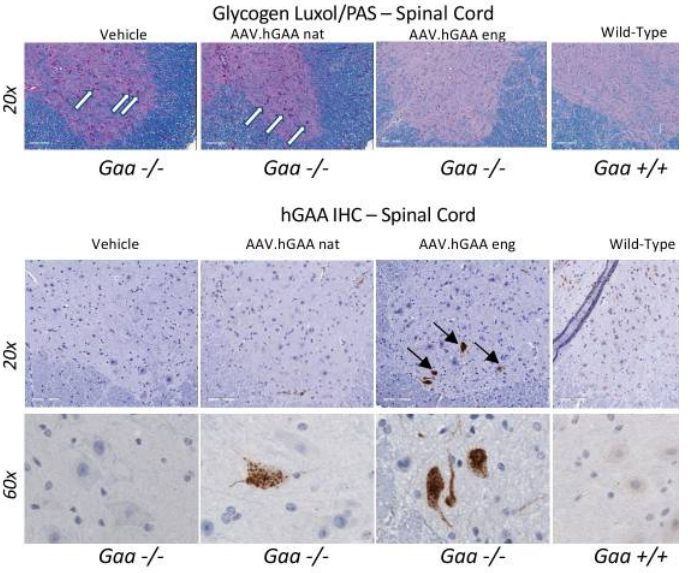


Spinal Cord– 28 days after AAV Gene Therapy

Engineered hGAA was able to Reduce Glycogen Efficiently in the Spinal Cord while Little Glycogen Reduction was Observed for N hGAA

- PAS staining showed that glycogen levels were close to wild-type in ventral horn, including motor neurons, for engineered hGAA
- PAS staining showed that glycogen levels were closer to vehicle in ventral horn, including motor neurons, for natural hGAA (white arrows)
- IHC demonstrated a stronger signal in motor neurons from animals receiving engineered hGAA compared with natural hGAA (black arrows)





Conclusions

- AAV gene therapy expressing the engineered hGAA led to more uniform cellular uptake, glycogen reduction, and resolved cellular dysfunction via efficient cross-correction.

- AAV gene therapy expressing the engineered hGAA demonstrated efficient cross-correction with resolution of cellular pathology in the CNS at low expression levels. At similar expression levels, nat hGAA did not demonstrate such effects.

Program Status and Anticipated Milestones

Initial Findings Validate Amicus/Penn Collaboration Combining Amicus-Engineered Transgenes with Penn's AAV G Therapy Technologies

- Pompe AAV gene therapy program builds upon protein engineering and manufacturing expertise u to successfully develop AT-GAA
- Additional preclinical studies underway (various doses and routes of AAV administration)
- Selection of clinical candidate in 2019 to move into IND-enabling studies
- Platform potential to design constructs that enhance protein targeting across multiple lysosomal disorders

Thank You

"Our passion for making a difference unites us"

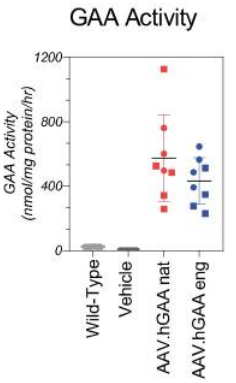
-Amicus Belief Statement

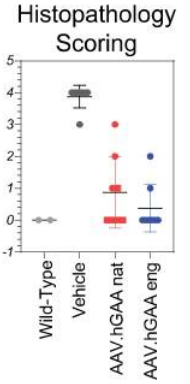
Appendix

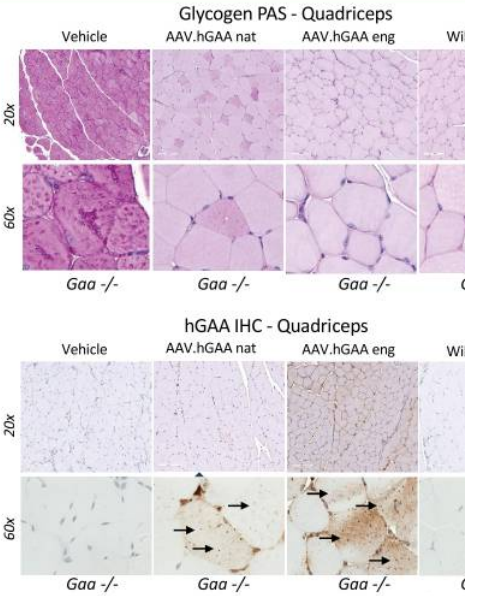
Quadriceps – 28 days after AAV Gene Therapy

Engineered hGAA was more efficient at cross-correction, indicated by greater cellular uptake, lysosomal targeting, and unifor glycogen reduction.

- GAA activity in the quadriceps was ~20 fold higher than wild-type levels for both engineered hGAA and natural hGAA
- Glycogen reduction was more uniform for engineered hGAA by PAS staining
- IHC illustrated greater cellular uptake (solid arrows) of engineered hGAA compared to natural hGAA





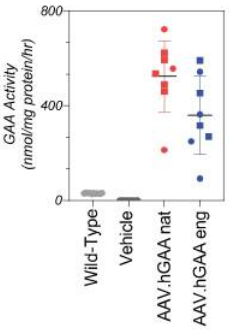


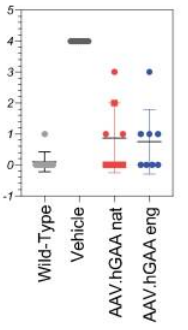
Periodic acid–Schiff (PAS) is a staining method used to detect polysaccharides such as glycogen in tissues

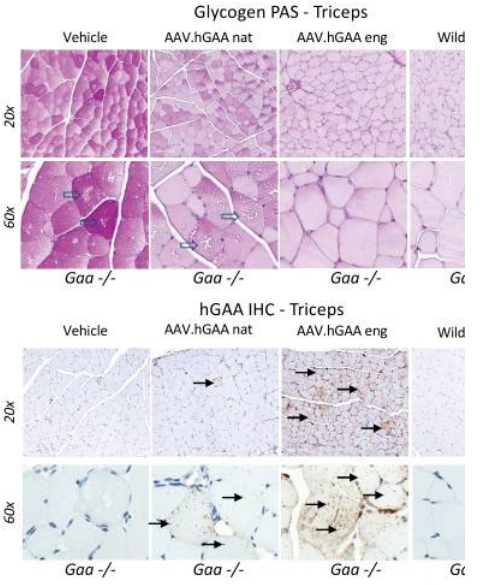
Triceps – 28 days after AAV Gene Therapy

Engineered hGAA was more efficient at cross-correction, indicated by greater cellular uptake, lysosomal targeting, and unifor glycogen reduction.

- GAA activity in the triceps was ~10 – 15 fold higher than wild-type levels for both engineered hGAA and natural hGAA
- PAS staining showed more complete and uniform glycogen reduction and clearance of autophagic vacuoles for engineered hGAA
- PAS staining showed incomplete glycogen reduction and less clearance of autophagic vacuoles for natural hGAA (open arrows)
- IHC illustrated greater cellular uptake (solid arrows) of engineered hGAA compared to natural hGAA







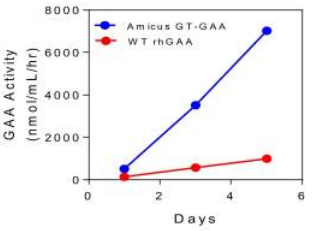
Periodic acid–Schiff (PAS) is a staining method used to detect polysaccharides such as glycogen in tissues

Early Proof of Principle for Optimized Gene Therapy

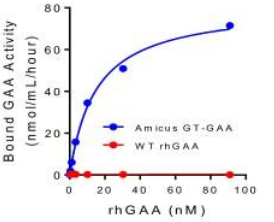
Amicus DNA Constructs Enable Optimized Gene Therapy in Pompe and Fabry

Pompe

Secreted GAA in Media

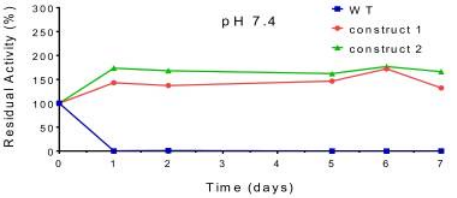


GAA Binding to Intended Receptor



Fabry

Alpha-Gal Activity: pH 7.4



Alpha-Gal Activity: pH 4.6