

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): **September 20, 2018**

AMICUS THERAPEUTICS, INC.

(Exact Name of Registrant as Specified in Its Charter)

Delaware

(State or Other Jurisdiction of
Incorporation)

001-33497

(Commission File Number)

71-0869350

(IRS Employer Identification No.)

1 Cedar Brook Drive, Cranbury, NJ

(Address of Principal Executive Offices)

08512

(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:

- 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))

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

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 September 20, 2018, Amicus Therapeutics, Inc. (the "Company") will be holding a conference call and webcast using the presentation attached as Exhibit 99.1 to this Current Report. In addition, on September 20, 2018, the Company filed a press release announcing the acquisition of a gene therapy portfolio consisting of ten clinical and pre-clinical stage AAV programs in neurological lysosomal storage disorders and the closing of a five-year senior debt facility consisting of a \$150 million term loan. A copy of this press release is attached to this Current Report as Exhibit 99.2.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits:

<u>Exhibit No.</u>	<u>Description</u>
99.1	September 20, 2018 Conference Call Materials
99.2	Press Release dated September 20, 2018 titled "Amicus Therapeutics Acquires Gene Therapy Portfolio of Ten Clinical and Pre-Clinical Stage AAV Programs in Neurologic Lysosomal Storage Disorders"

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: September 20, 2018

By: /s/ Ellen S. Rosenberg
Ellen S. Rosenberg
General Counsel and Corporate Secretary



Amicus Establishes Gene Therapy Pipeline for Lysosomal Storage Disorders (LSDs)



Conference Call and Webcast
September 20, 2018

Safe Harbor

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to the acquisition of Celenex, preclinical and clinical development of our acquired product candidates, the timing and reporting of results from these preclinical studies and clinical trials, and financing plans 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 presentation may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, the benefits of this acquisition 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 product candidates as advisors. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. 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, 2017 as well as our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018 filed August 7, 2018 with the Securities and Exchange Commission. 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.

Amicus Establishes Gene Therapy Portfolio

License Through Nationwide Children's Hospital Combines Successful Amicus Development and Commercial Track Record in LSDs with Ten AAV Gene Therapy Programs for Rare Neurologic LSDs

Ground Breaking, Clinically Validated Science

Ten Gene Therapy Programs

Expertise and Relationships in Gene Therapy

Compelling Data in Three Lead Programs

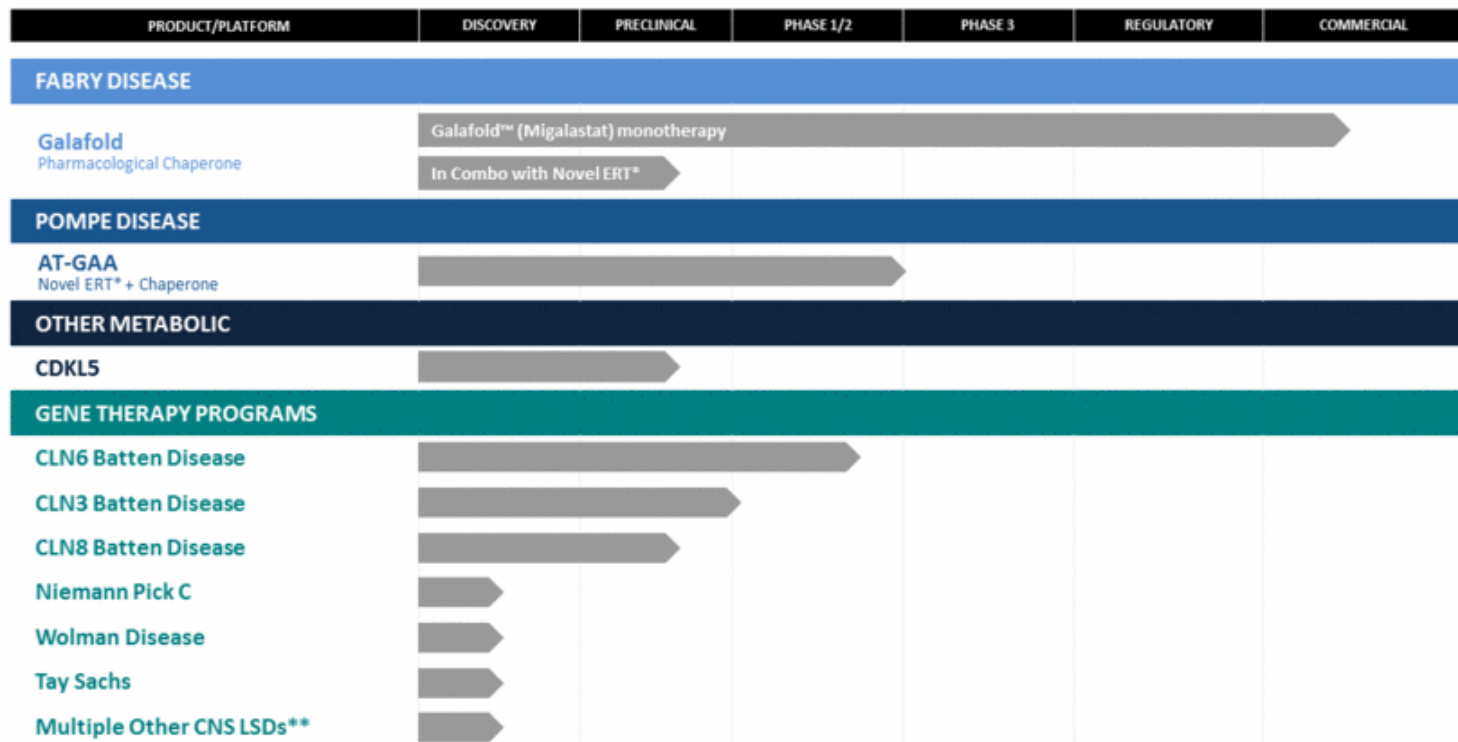
Leading Gene Therapy Portfolio in Neurologic Lysosomal Storage Disorders

"I firmly believe that Amicus is the optimal scientific and clinical partner to move these programs forward and I look forward to actively collaborating with the Amicus team on the development of these important potential therapies."

*- Kathrin Meyer, Ph.D. PI at Meyer Lab
Nationwide Children's Hospital and Assistant
Professor at The Ohio State University*

Pipeline

Developing Therapies for People Living with Rare Metabolic Diseases with a New Focus on Gene Therapy



*Enzyme replacement therapy ** CNS LSD: Central Nervous System Lysosomal Storage Disorder

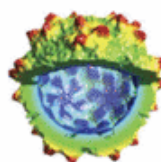
Validated Gene Therapy Platform

Portfolio is Based on a Validated Gene Therapy Approach Across Multiple CNS Diseases

- Clinically validated AAV gene therapy approach
 - Nationwide Children’s Hospital Center for Gene Therapy (NCH)
 - Intrathecal delivery with robust expression throughout the CNS
- Preclinical safety and efficacy studies replicated across multiple diseases at NCH
 - SMA
 - Rett Syndrome
 - ALS
 - CLN6
 - CLN3



Foust, Kaspar et al, 2009



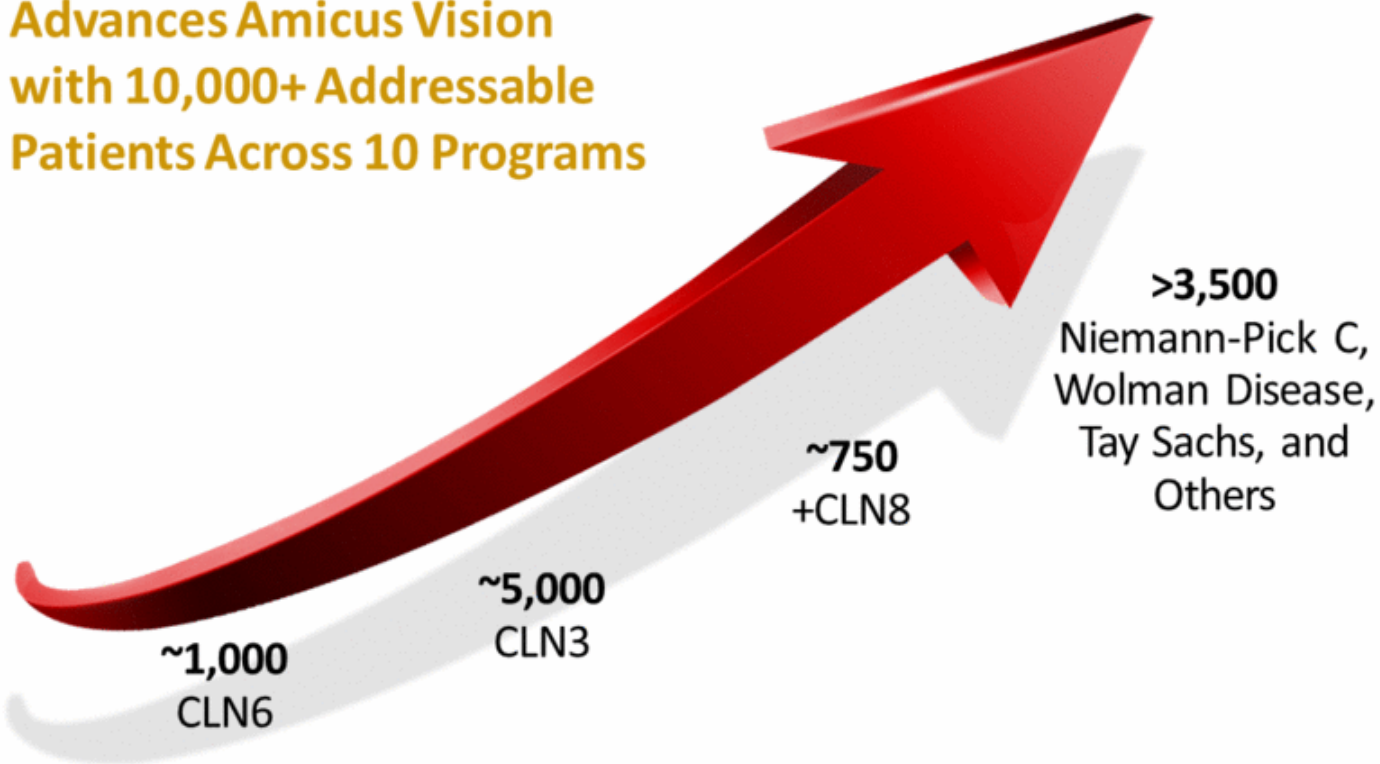
scAAV9-CLN6

AAV9-CLN6 Transgene

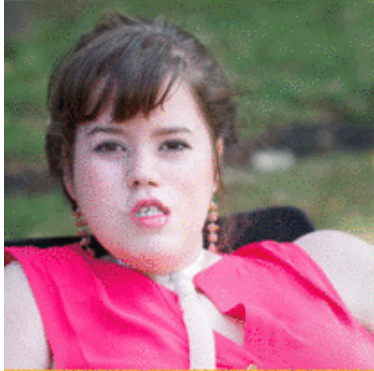
Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

Addressable Patient Populations*

**Advances Amicus Vision
with 10,000+ Addressable
Patients Across 10 Programs**



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



Batten Disease

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

Batten Disease Overview

Batten Disease is a Group of Rare, Fatal, Lysosomal Storage Disorders of the Central Nervous System with High Unmet Need and Limited Treatment Options

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 typically begin in early and late childhood
- Most affected children do not survive into adulthood



Lead Program Status

The CLN6 and CLN3 Program are Clinical Stage; CLN8 has Definitive Preclinical Efficacy Data in a Mouse Model of Disease

PRECLINICAL MOUSE MODEL DATA

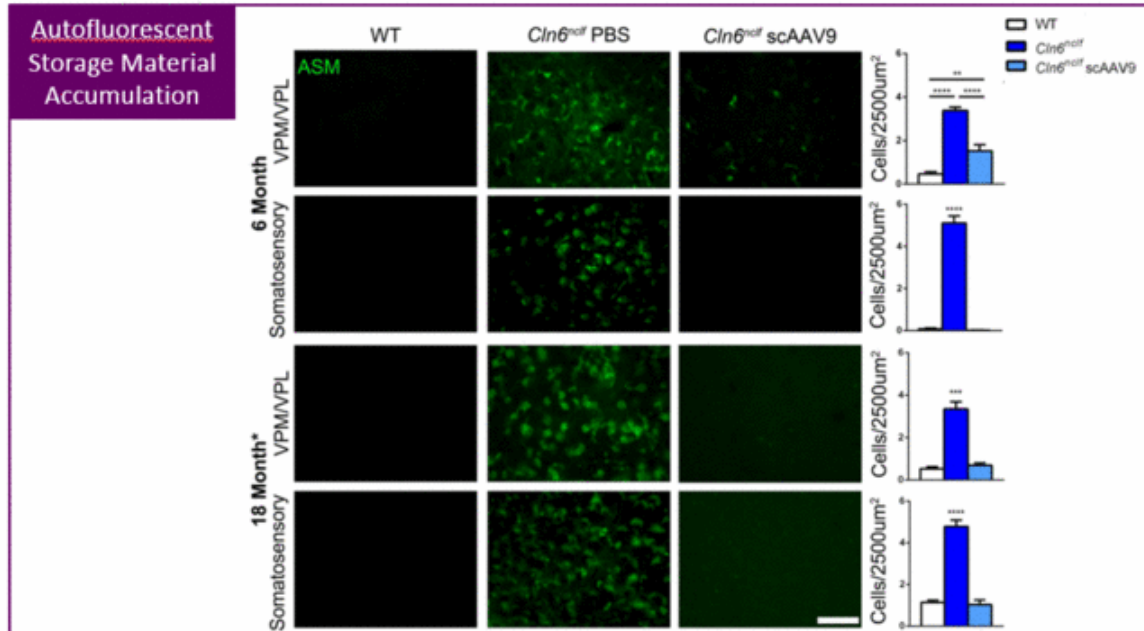
	Storage Material	Motor & Cognitive Function	Survival	Safety & Brain Expression in NHP	GMP Clinical Supply	IND Active	Preliminary Clinical Data
CLN6	✓	✓	✓	✓	✓	✓	✓
CLN3	✓	✓	N/A*	✓	✓	✓	Pending
CLN8	✓	✓	✓	Pending	Pending	Pending	Pending

*CLN3 mouse model does not have impaired survival

CLN6: Preclinical Mouse Data

Autofluorescent Storage Material

Single AAV9-CLN6 Administration Results in Reduction of Autofluorescent Substrate Material Throughout the Brain for up to 18 months



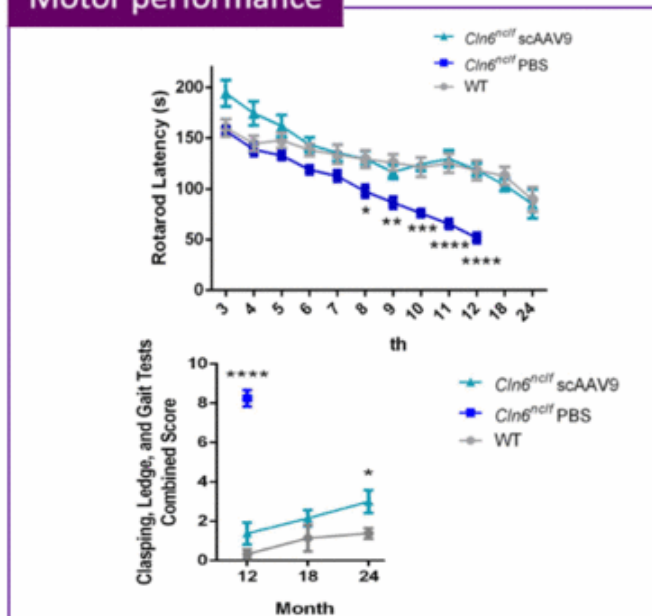
Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

CLN6: Preclinical Mouse Data

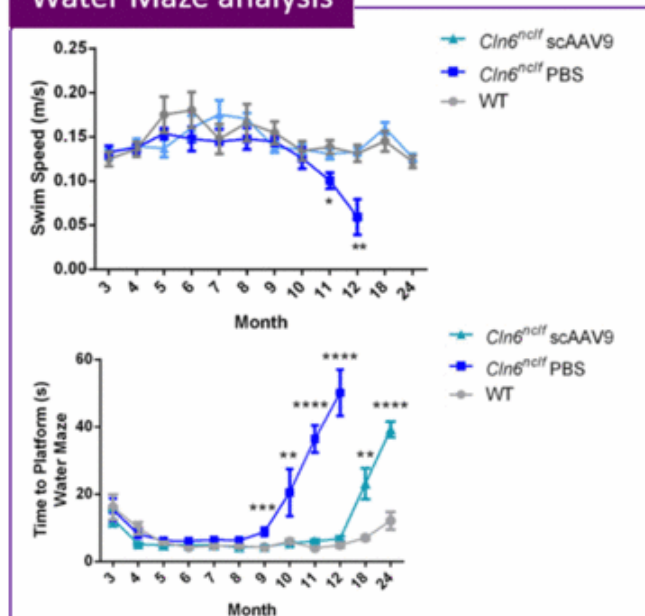
Motor Performance and Cognitive Behavior

Single AAV9-CLN6 Administration Improves Motor Performance & Cognitive Behavior Out to Month 24

Motor performance



Water Maze analysis

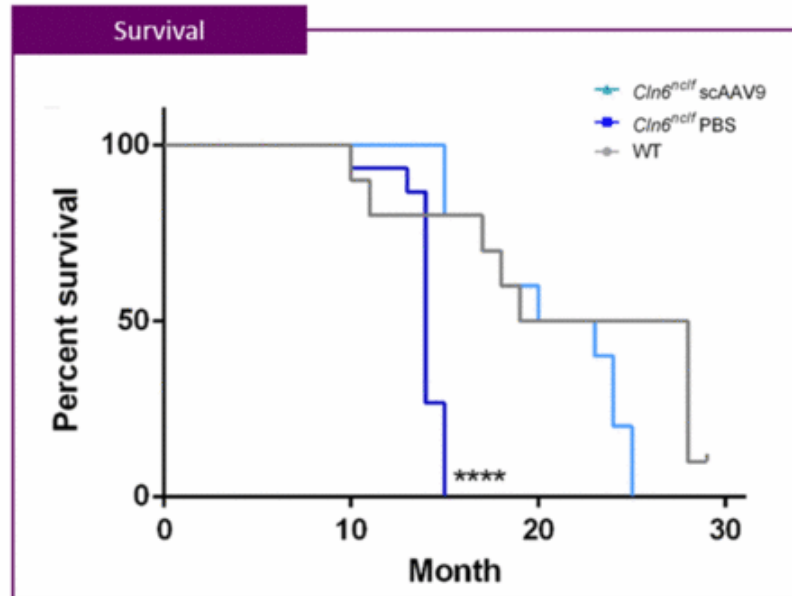


Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy; Data on file

CLN6: Preclinical Mouse Data

Survival

Single AAV9-CLN6 Administration Significantly Extends Median Survival

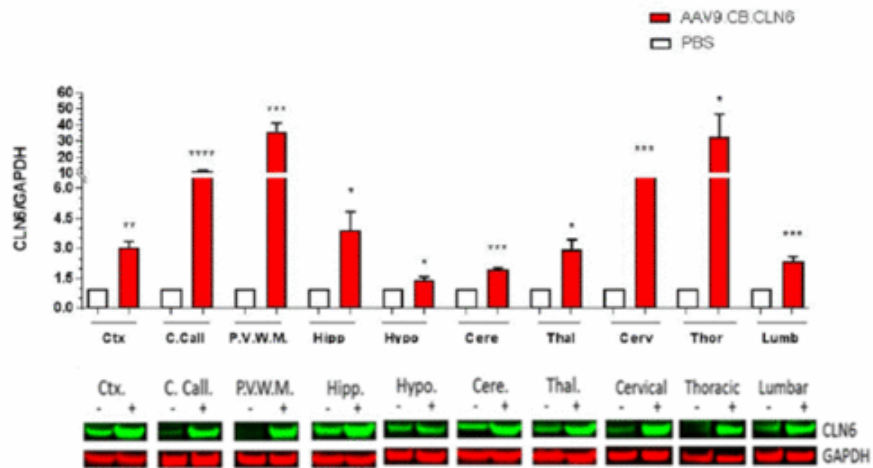


Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

CLN6 Expression in NHP Safety Study

Demonstrated Safety and Meaningful Transduction and CLN6 Expression Demonstrated Throughout the Brain in NHPs

Western Blot on various brain regions of AAV9-CLN6 injected juvenile NHPs



Source: Meyer 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, From mouse to human—Translating intrathecal gene therapy for NCLs; Data on file

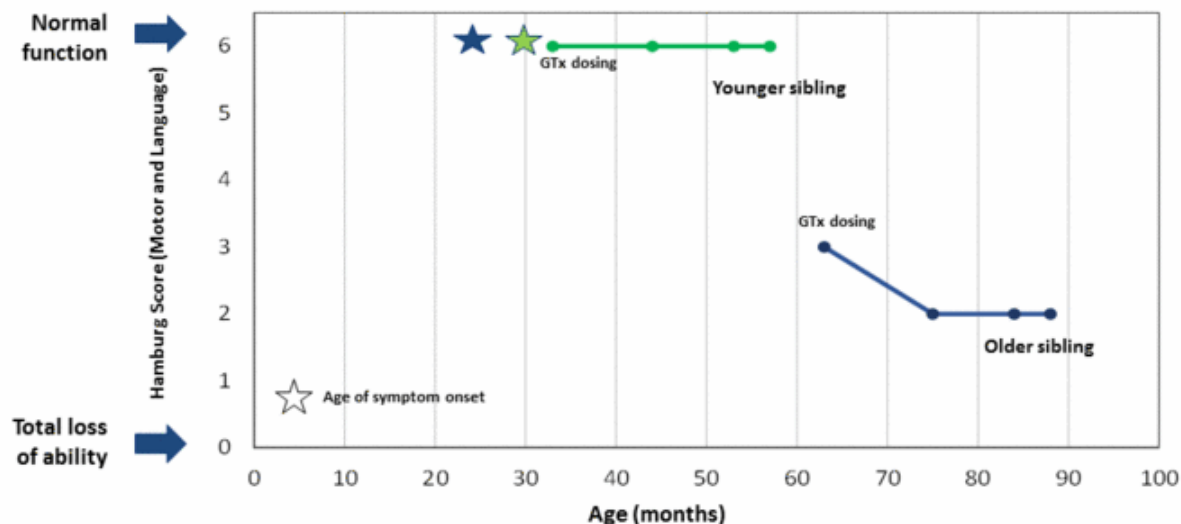
CLN6: Clinical Data Summary

Encouraging Safety and Efficacy Data from an Ongoing Single-arm Phase 1/2 study

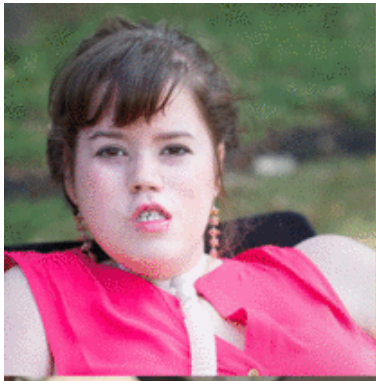
- Single-arm study with all patients receiving gene therapy (n=12)
 - Single intrathecal administration of 1.5×10^{13} vg scAAV9-CLN6
- Ten patients currently treated; additional patients in screening
 - Average follow-up duration: 12 months (range 1-24 months)
- Generally well-tolerated
 - Majority of adverse events were mild
 - Data Safety Monitoring Board (DSMB) has permitted study to proceed and enroll additional patients
- Encouraging preliminary efficacy data
- Additional data to be presented in 2019

Efficacy Data: Matched Sibling Case Report

Encouraging Interim Efficacy Data in First Two Patients Treated with Gene Therapy with Two Years of Follow-up



- Two siblings (same genotype) treated with gene therapy at ages 2.8 and 5.3 years, respectively
- Two years post treatment, Hamburg motor and language scores indicate no disease progression in the younger sibling
- Disease progression in older sibling has shown evidence of stabilization

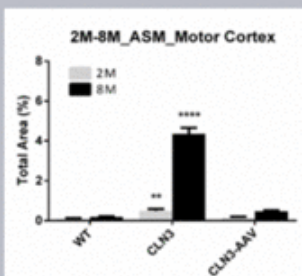


CLN3

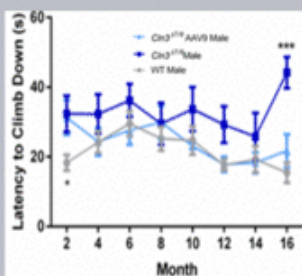
Preclinical Data Overview

CLN3: Preclinical Summary

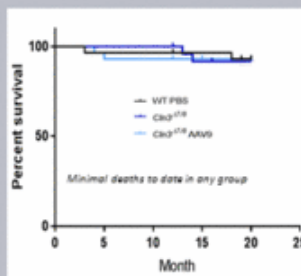
AAV9-CLN3 Administration Resulted in Storage Material Reduction and Motor/Cognitive Function Improvement in Mouse Model of Disease and Widespread Expression in the Brain of NHPs



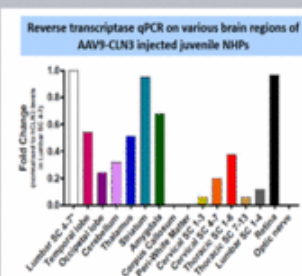
Reduction of storage material in mouse model



Improvement of motor function and cognitive behavior in mouse model



Comparable survival in mouse model



Widespread gene expression in brain of NHPs

Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy; Meyer 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, From mouse to human –Translating intrathecal gene therapy for NCLs;

CLN3: Clinical Status

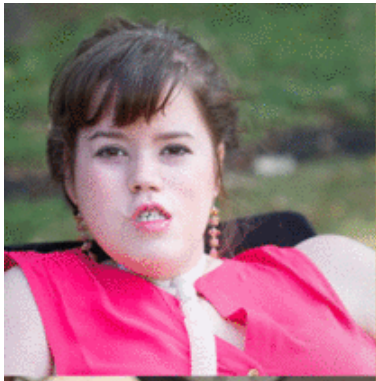
Active IND

GMP clinical supply available

Study initiated at NCH

FPI anticipated in coming months

CLN3
1st to Clinic

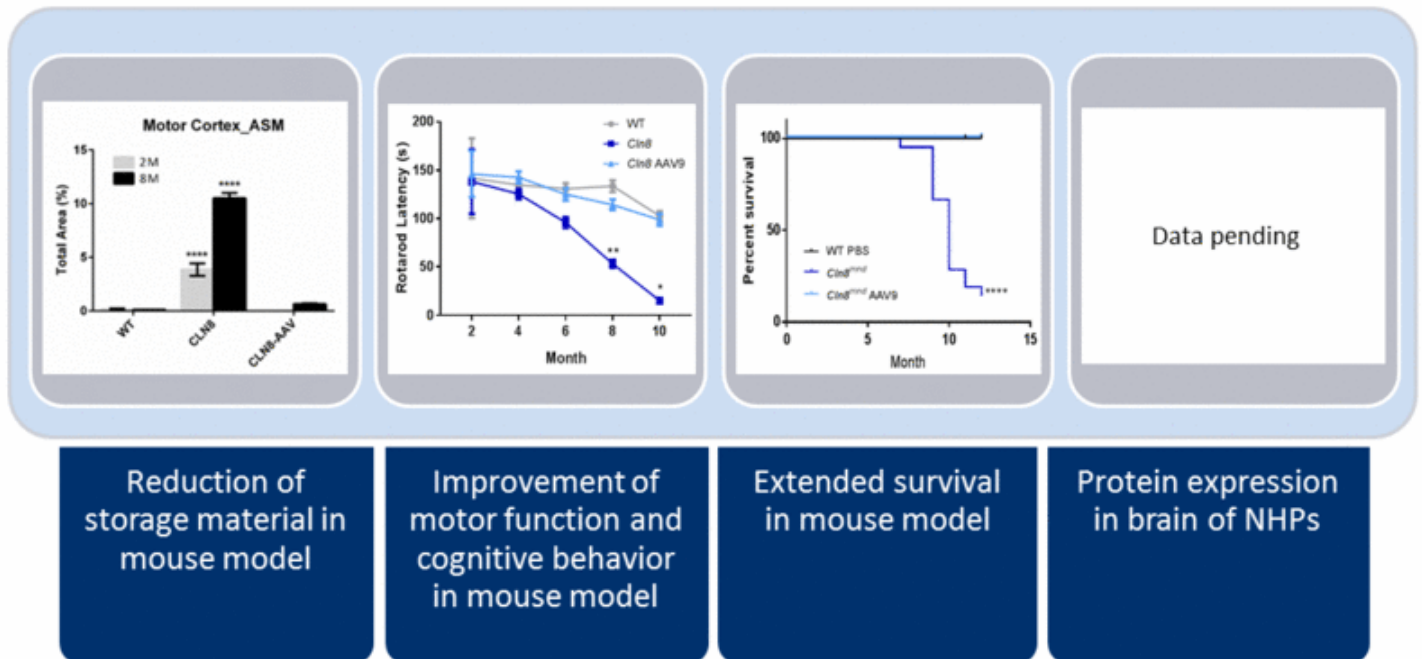


CLN8

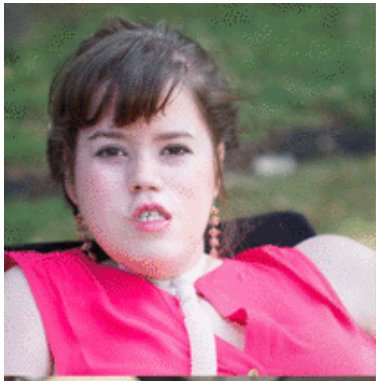
Preclinical Data Overview

CLN8: Preclinical Summary

AAV9-CLN8 Administration Resulted in Storage Material Reduction, Motor/Cognitive Function Improvement and Extended Survival in Mouse Model of Disease

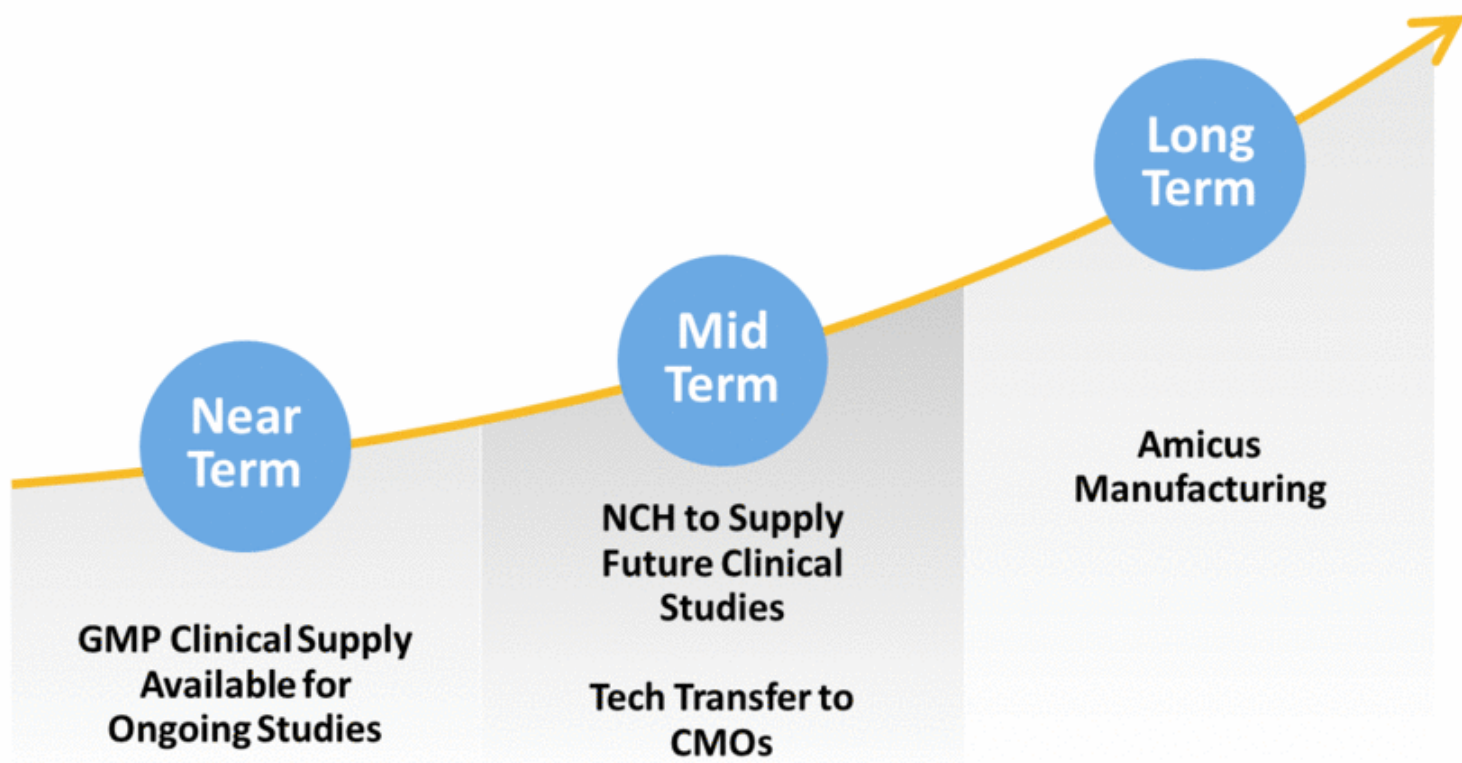


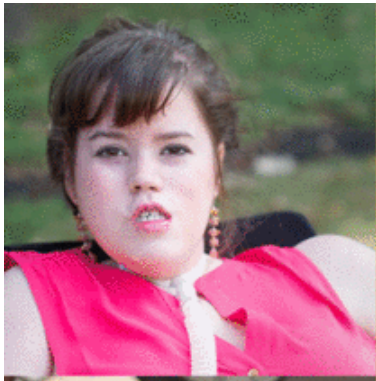
Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy; Meyer 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, From mouse to human –Translating intrathecal gene therapy for NCLs;



Manufacturing

Manufacturing: Proven Track Record at NCH





Deal Summary & Closing Remarks

Upcoming Program Milestones

Anticipating Multiple Program Milestones throughout 2018 & 2019

First Patient in CLN3 Phase 1/2 Study

Complete Enrollment in CLN6 Phase 1/2 Study

Preliminary Phase 1/2 Data in CLN6

Complete Enrollment in Initial Cohort in CLN3 Phase 1/2 Study

Preclinical Proof of Concept in Other Programs

Transaction Terms & Financing Overview

Acquisition to Drive Value as We Build the Leading Gene Therapy Pipeline in Metabolic Rare Diseases

CONSIDERATION*

- \$100M upfront cash payment
- Up to \$15M in development milestones and \$262M in BLA/MAA submission and approval milestones
- No more than \$75M owed over next 4 years
- Up to \$75M in tiered sales milestones (Tiers: \$500M/\$750M)

PHARMAKON ADVISORS FINANCING

- Acquisition and several years of related development costs financed through \$150M debt facility
- 5 year term; 4 years interest only; No equity dilution

TIMING

- Deal closed

*Lead programs only: CLN6, CLN3, and CLN8

Amicus Vision: Delivering for Patients and Shareholders

To build a top-tier, fully integrated, global biotechnology company whose medicines treat 5,000+ patients with \$1B+ in worldwide sales revenue by 2023



>350 Patients* | \$36.9M Global Sales



5,000 Patients* | \$1B Global Sales



*Clinical & commercial, all figures approximate



Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement





Amicus Therapeutics Acquires Gene Therapy Portfolio of Ten Clinical and Pre-Clinical Stage AAV Programs in Neurologic Lysosomal Storage Disorders

License with Nationwide Children's Hospital (NCH) through the Acquisition of Celenex (NCH Spinout)

Establishes Amicus as Leading Gene Therapy Company in Neurologic Lysosomal Storage Disorders (LSD) with Potential to Transform the Lives of 10,000+ Children with Fatal Genetic Diseases

Includes Clinical Stage Programs in CLN6 and CLN3 Batten Disease, a Preclinical Program in CLN8 Batten Disease and Additional LSD Programs in Niemann Pick C, Wolman Disease, Tay Sachs and Other Disorders

Leverages Amicus Therapeutics Expertise in Global Development and Delivery of Novel Therapies for Rare Metabolic Disorders with Nationwide Children's AAV Gene Therapy Technologies

Balance Sheet Strengthened with \$150 Million in Non-Dilutive Debt Financing

Conference Call and Webcast Scheduled for Today, September 20, 2018 at 8:00am ET

CRANBURY, NJ, September 20, 2018 — Amicus Therapeutics (Nasdaq: FOLD) today announced the signing of a definitive agreement in which Amicus Therapeutics will receive worldwide development and commercial rights for ten gene therapy programs developed at The Center for Gene Therapy at The Research Institute at Nationwide Children's Hospital and The Ohio State University. The ten programs are licensed to Amicus from Nationwide Children's Hospital through the acquisition of Celenex, a private, clinical stage gene therapy company. The lead programs in CLN6, CLN3, and CLN8 Batten disease are potential first-to-market curative therapies for these rare, devastating diseases. Batten disease, also known as Neuronal Ceroid Lipofuscinosis (NCL), is a family of rare disorders that can be life-threatening and debilitating, with high unmet medical need. More detail about these programs will be presented on this morning's conference call. The slides for this conference call are available on our website.

"The in-licensing and acquisition of these gene therapy programs provides an extraordinary opportunity to transform the lives of thousands of children living with some of the most devastating forms of lysosomal storage disorders, for which there are virtually no treatment options today," said John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics. "The groundbreaking work of Drs. Brian Kaspar and Kathrin Meyer at Nationwide Children's Hospital, along with collaborator, Arthur Burghes, Ph.D., professor at The Ohio State University, on these programs has led to remarkably strong and consistent pre-clinical results and now, in CLN6 Batten disease, encouraging early results in children. This is science and biotechnology at its best. And it has at its core the love, drive and passion of two remarkable parents, Gordon and Kristen Gray, who moved heaven and earth to partner with these researchers to advance these potentially life-saving medicines for their daughters and now for many thousands more. I am honored that they and their research team have chosen to entrust these ten programs to the passionate team of scientists and entrepreneurs at Amicus. I cannot think of a better foundation for Amicus' entry into gene therapies."

All acquired programs leverage intrathecal delivery, using the same AAV vector approach utilized successfully in clinical trials across other rare CNS indications, such as SMA. This approach and technology are considered to be a clinically validated gene delivery platform for diseases of the central nervous system (CNS). Brian Kaspar, Ph.D., co-founder of Celenex, and Kathrin Meyer, Ph.D., a Principal Investigator at Nationwide Children's Hospital Center for Gene Therapy, will continue to support these programs as scientific advisors to Amicus Therapeutics.

"The preclinical proof-of-concept we have seen to date in CLN6, CLN3, and CLN8 further support the applicability of the AAV vector we developed at Nationwide Children's in genetic disease of the CNS," said Kathrin Meyer, Ph.D. "I firmly believe that Amicus is the optimal scientific and clinical partner to advance these programs and look forward to actively collaborating with the Amicus team on the development of these important potential therapies and getting them to as many children as quickly as possible. They truly have the potential to transform lives."

Deal Terms

Under the terms of the agreement, Amicus will pay \$100 million in an upfront cash payment to acquire all of these assets.

Celenex shareholders are also eligible for up to \$15 million in development milestones and \$262 million in BLA/MAA submission and approval milestones across multiple programs. Amicus expects to pay no more than \$75 million over the next 4 years in these milestones. No royalties are owed to Celenex for any of these programs. Celenex shareholders may also be eligible for up to \$75 million in tiered sales (\$500 million/\$750 million) milestone payments. The acquisition and several years of related development costs for all of these programs will be financed through a new \$150 million debt facility provided by BioPharma Credit PLC, an investment fund managed by Pharmakon Advisors, L.P.

Skadden, Arps, Slate, Meagher & Flom LLP acted as legal counsel to Amicus Therapeutics on the transaction. RBC Capital Markets acted as exclusive financial advisor and Fenwick & West LLP acted as exclusive legal counsel to Celenex on the transaction. The transaction was approved by the Board of Directors of both companies and closed immediately.

Debt Facility

Today, Amicus Therapeutics also announced it has closed a five-year, senior credit facility with BioPharma Credit. The new credit facility consists of a \$150 million non-dilutive term loan, which requires interest-only payments through 2022 and matures in 2023. Interest will accrue at a floating rate of LIBOR plus 7.5%, subject to a floor and ceiling on the rate. There are no warrants or any equity conversion features associated with the loan. The proceeds from this financing will be used to support the cost of the acquisition and several years of related development costs.

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, September 20, 2018, at 8:00 a.m. ET to discuss the new gene therapy pipeline. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international), conference ID:

An audio webcast 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 go to the website 15 minutes prior to the start of the call to register, download, and install any necessary software. A telephonic replay of the call will be available for seven days beginning at 11:30 a.m. ET on September 20, 2018. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); conference ID: 7084639.

About Batten Disease

Batten disease is the common name for a broad class of rare, fatal, inherited disorders of the nervous system also known as neuronal ceroid lipofuscinoses, or NCLs. In these diseases, a defect in a specific gene triggers a cascade of problems that interferes with a cell's ability to recycle certain molecules. The disease has several forms with similar features and symptoms but vary in severity and age of onset. Each form is caused by a mutation in a different gene. Most forms of Batten disease/NCLs usually begin during childhood. The first symptom is usually progressive vision loss in previously healthy children followed by personality changes, behavioral problems and slow learning. Seizures commonly appear within 2-4 years of vision loss. Seizures and psychosis can appear at any time during the course of disease. Patients typically experience progressive loss of motor functions and eventually, those affected become wheelchair-bound, are bedridden, and die prematurely.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a global, patient-centric 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.

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 acquisition of Celenex, preclinical and clinical development of our acquired product candidates, the timing and reporting of results from these preclinical studies and clinical trials, and financing plans 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, the benefits of this acquisition 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 product candidates as advisors. Further, the results of

earlier preclinical studies and/or clinical trials may not be predictive of future results. 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, 2017 as well as our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018 filed August 7, 2018 with the Securities and Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this presentation to reflect events or circumstances after the date hereof.

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FOLD—G
