Amicus Therapeutics®

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

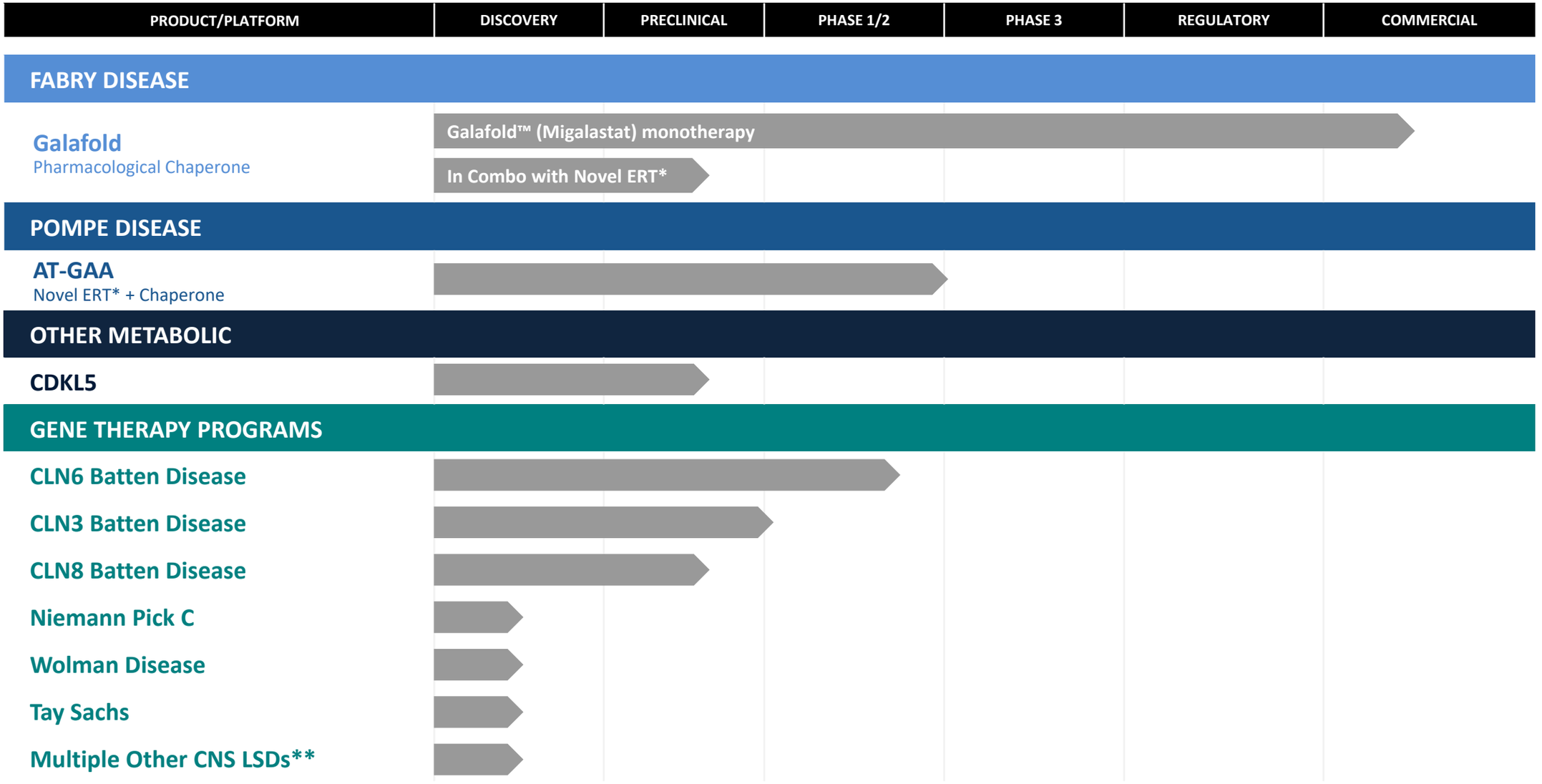
- **Ground Breaking, Clinically Validated Science**
- **Ten Gene Therapy Programs**
- **Expertise and Relationships in Gene Therapy**
- **Compelling Data in Three Lead Programs**
- **Leading Gene Therapy Portfolio in Neurologic Lysosomal Storage Disorders**

"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

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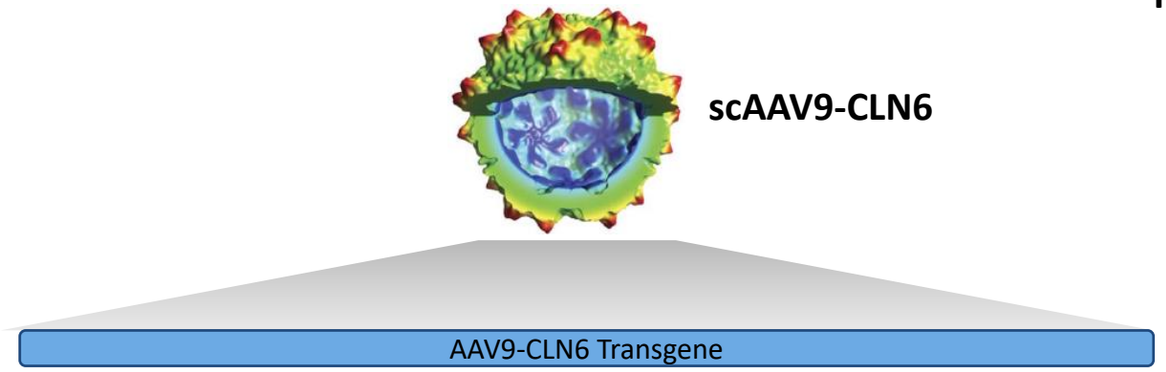
Validated Gene Therapy Platform

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

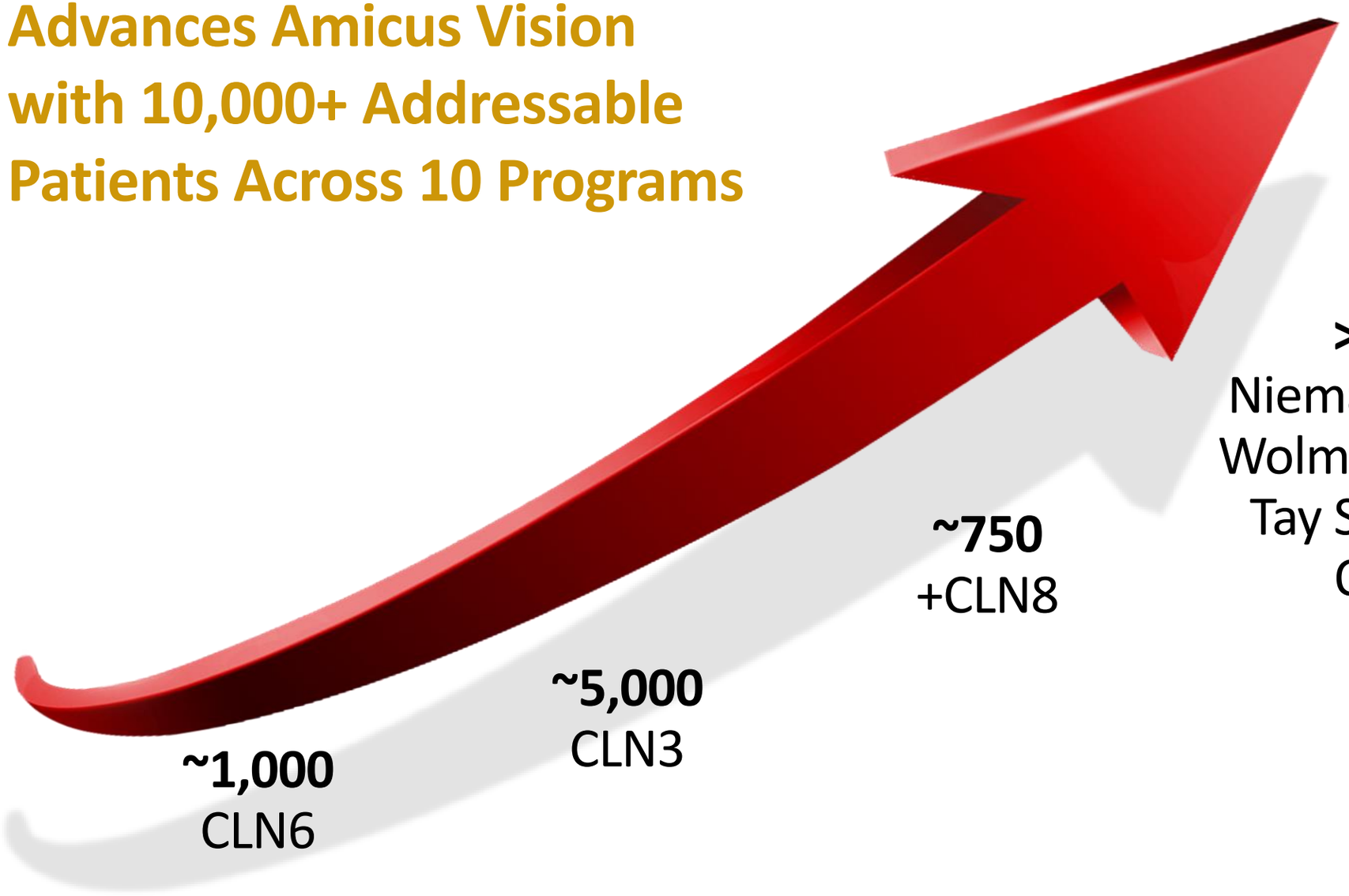


scAAV9-CLN6

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

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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

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

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

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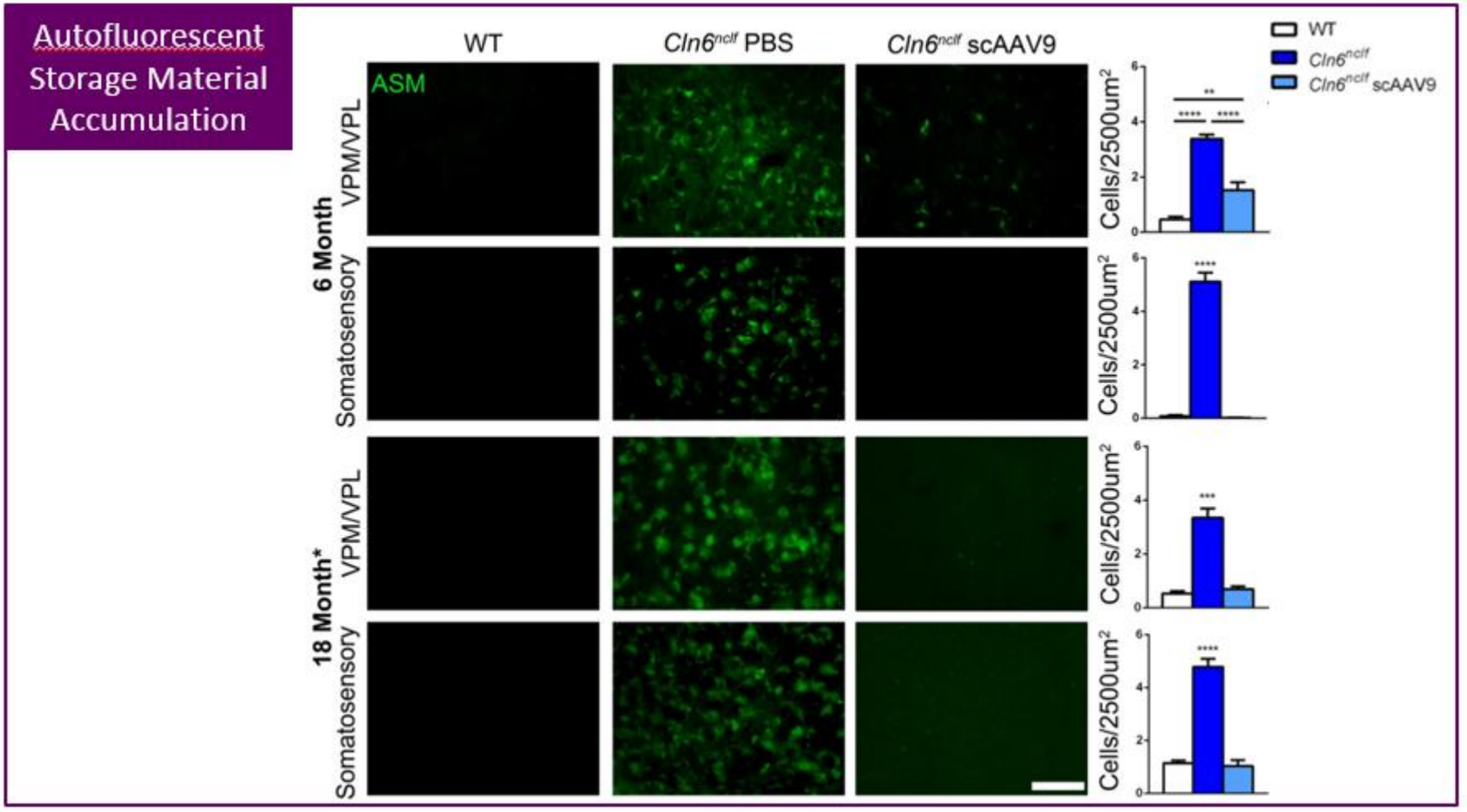
CLN6: Preclinical Mouse Data
Autoflourescent Storage Material

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

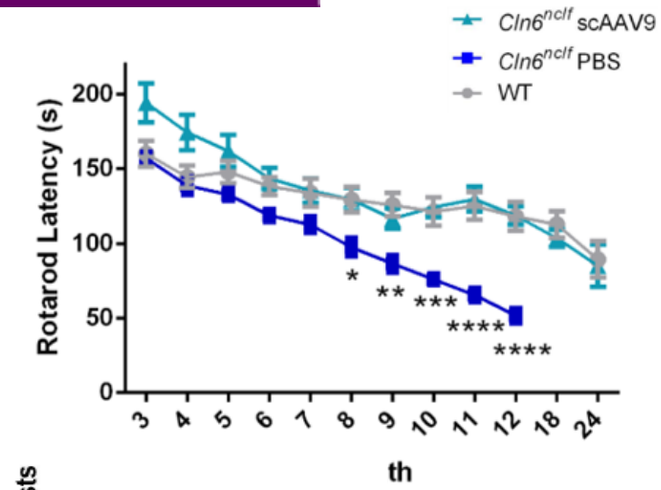


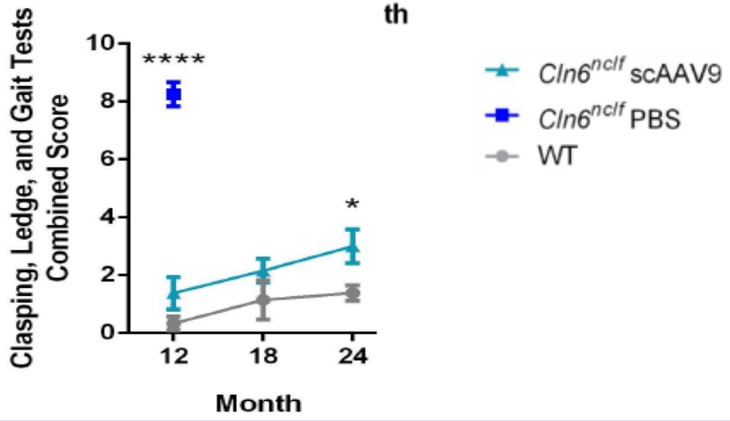
Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

CLN6: Preclinical Mouse Data
Motor Performance and Cognitive Behavior

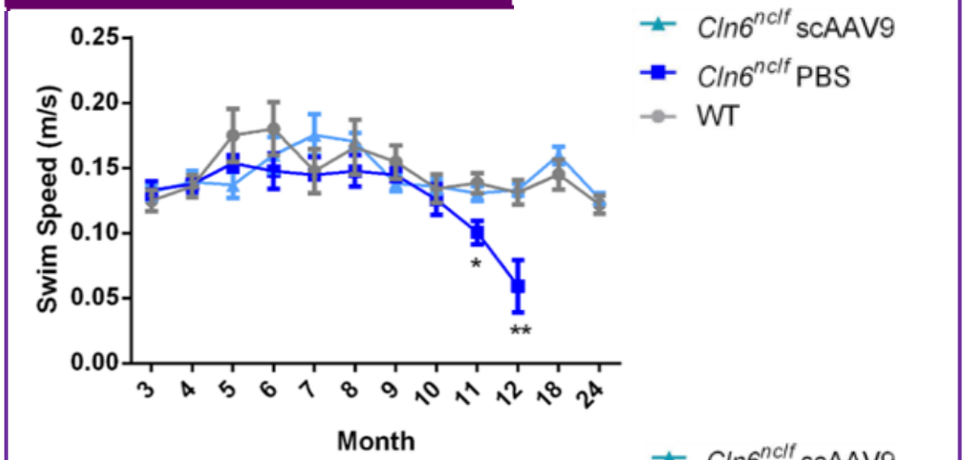
Single AAV9-CLN6 Administration Improves Motor Performance & Cognitive Behavior Out to Month 24

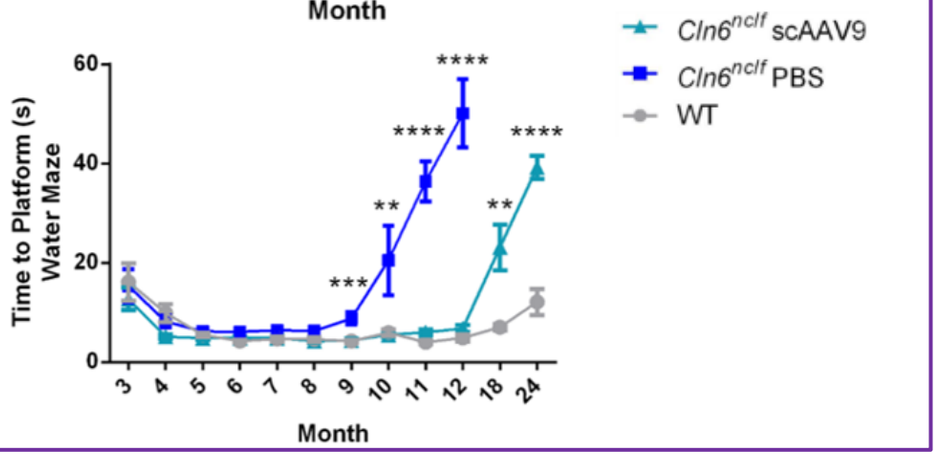
Motor performance





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

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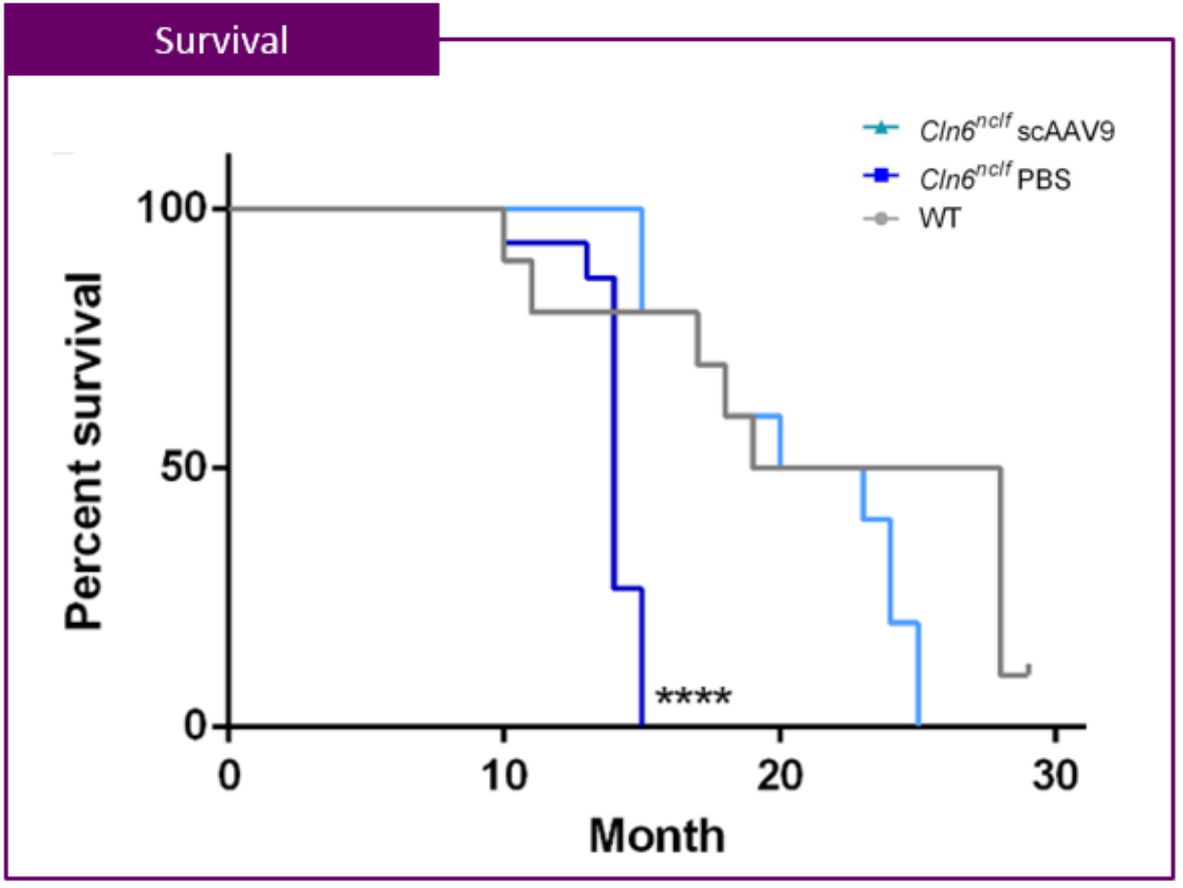
CLN6: Preclinical Mouse Data
Survival

Single AAV9-CLN6 Administration Significantly Extends Median Survival

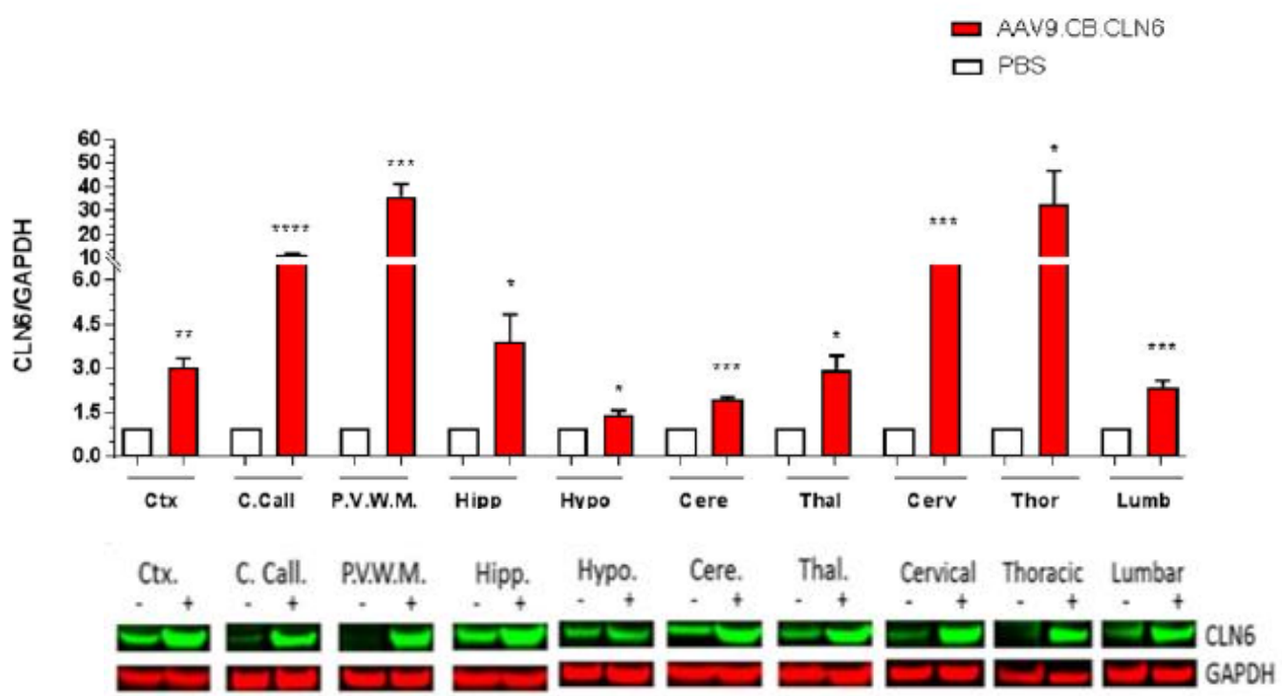


Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

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

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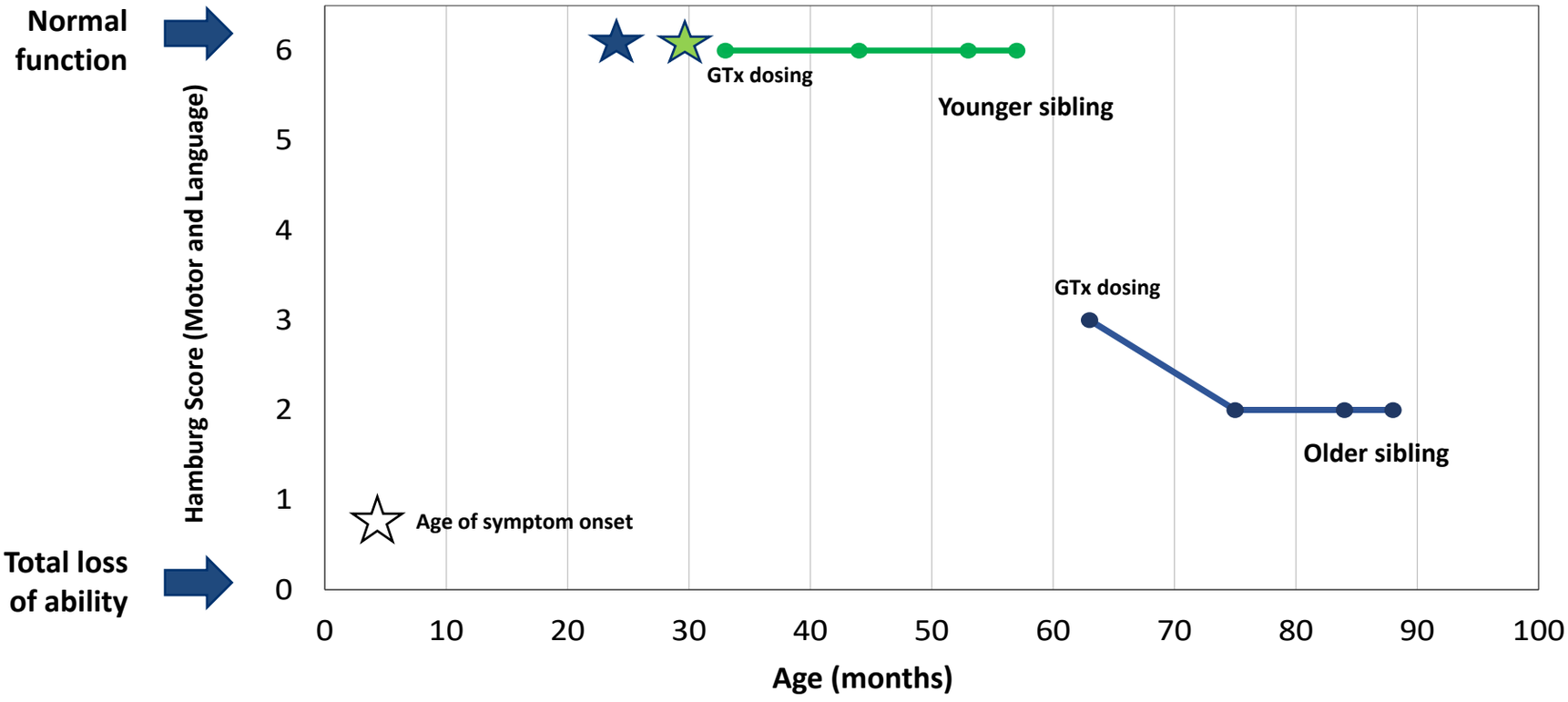
CLN6: Clinical Data Summary

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

Source: Data on file

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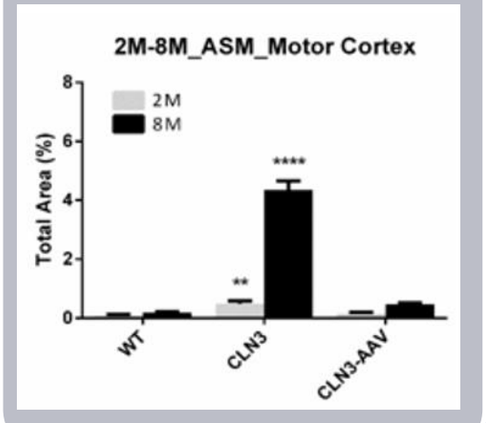
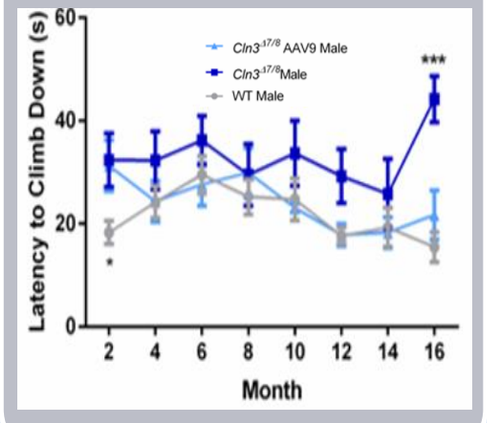
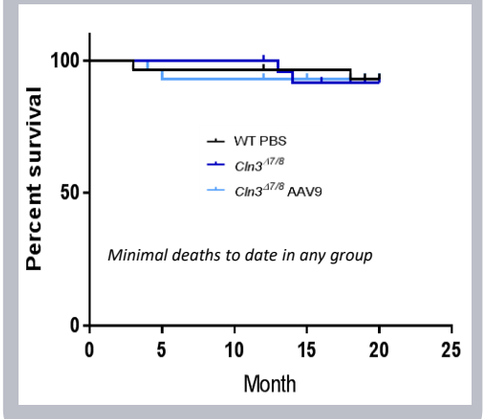
CLN3
Preclinical Data Overview

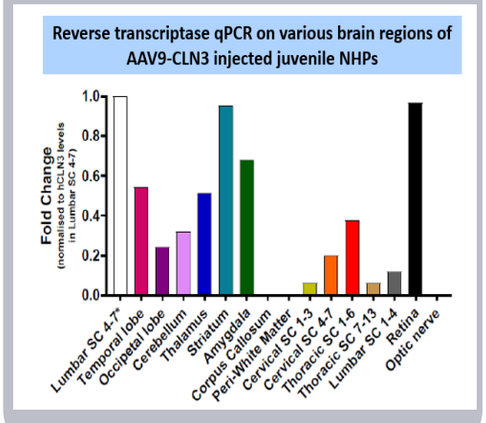
CLN3: Preclinical Summary

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

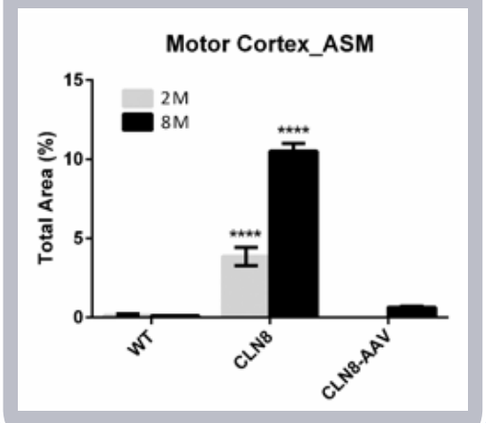
CLN3

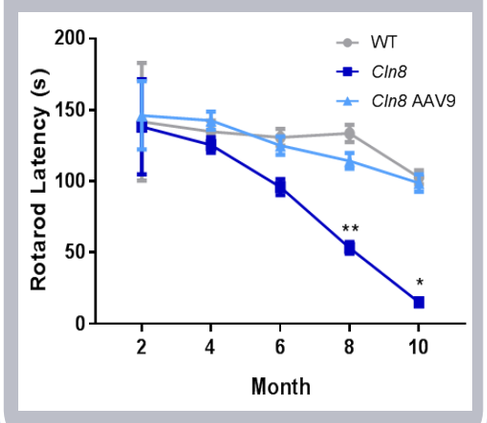
1st to Clinic

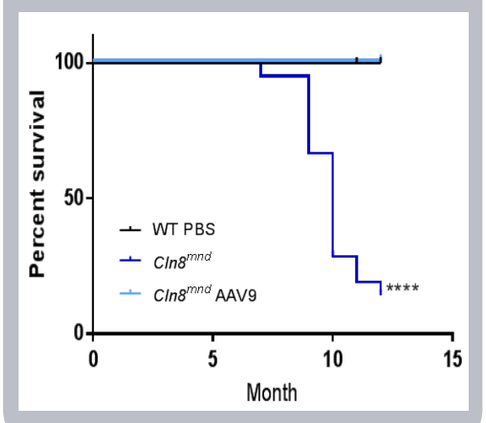
CLN8
Preclinical Data Overview

CLN8: Preclinical Summary

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







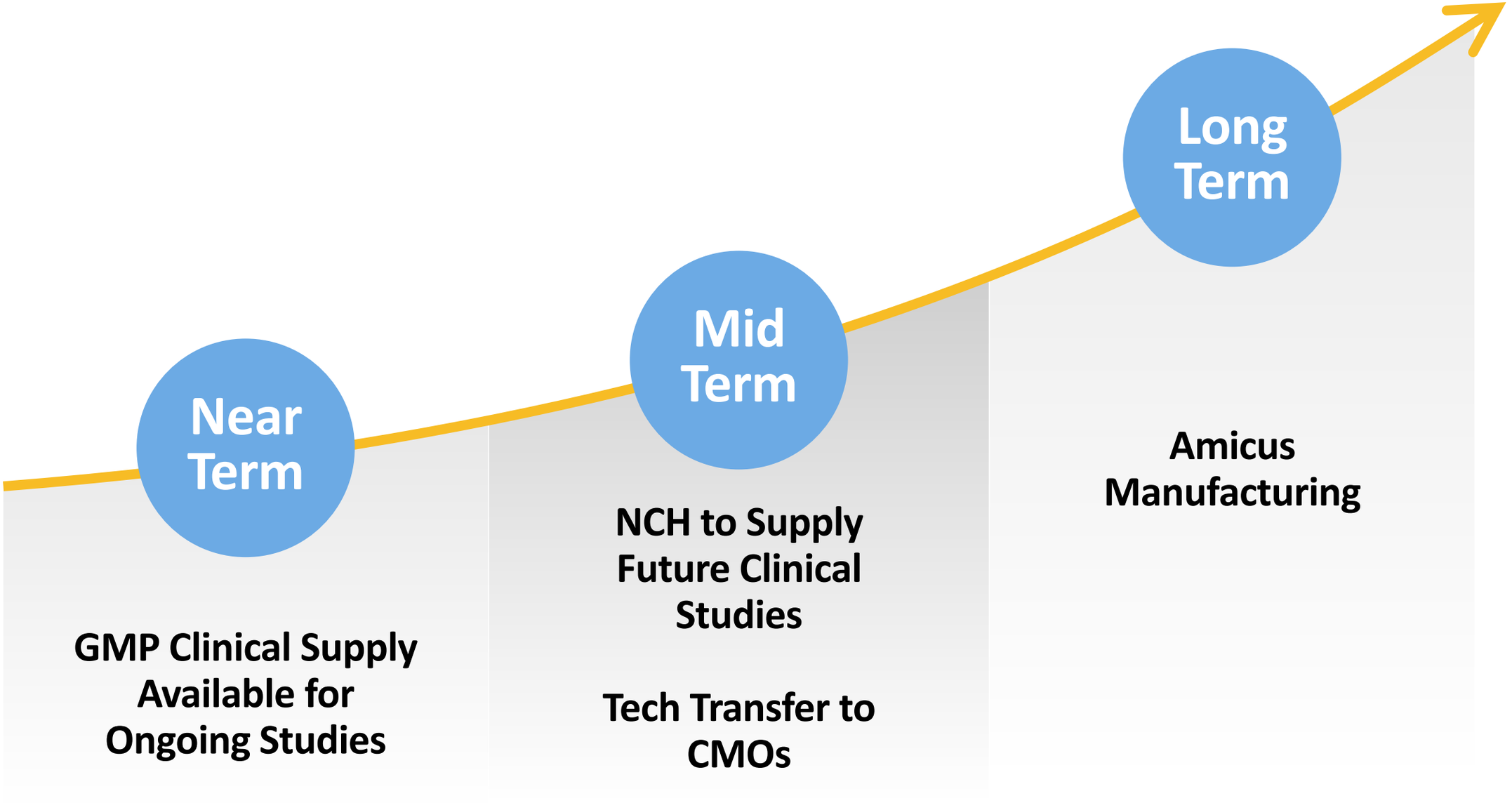
Data pending

Reduction of storage material in mouse model	Improvement of motor function and cognitive behavior in mouse model	Extended survival in mouse model	Protein 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;

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

YE17 → **2023**

*Clinical & commercial, all figures approximate

Amicus Therapeutics®

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement

Amicus Therapeutics®