TAYSHA GENE THERAPIES

Bringing New Cures to Life

CLN7 Program

October 05, 2021 | 8:00 – 9:00 AM ET

Legal disclosure

FORWARD LOOKING STATEMENTS

This presentation contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this presentation, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “predict,” “project,” “target,” “potential,” “will,” “would,” “could,” “should,” “continue,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These forward-looking statements are subject to a number of risks, uncertainties and assumptions. Risks regarding our business are described in detail in our Securities and Exchange Commission filings, including in our Annual Report on Form 10-K for the year ended December 31, 2020, and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2021. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. The forward-looking statements contained in this presentation reflect our current views with respect to future events, and we assume no obligation to update any forward-looking statements except as required by applicable law.

This presentation includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties as well as our own estimates of potential market opportunities. All of the market data used in this prospectus involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such data. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. Our estimates of the potential market opportunities for our product candidates include several key assumptions based on our industry knowledge, industry publications, third-party research and other surveys, which may be based on a small sample size and may fail to accurately reflect market opportunities. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions.

Introduction

RA Session II

President, Founder & CEO

Agenda

Topic	Presenter
Introduction	RA Session II
Disease Overview	Angela Schulz, MD, PhD
Preclinical Pharmacology and Toxicology Data	Steven Gray, PhD
Clinical Development Strategy	Suyash Prasad, MBBS, MSc, MRCP, MRCPCH, FFPM
Closing Remarks	RA Session II

Speaker biographies

Angela Schulz, MD, PhD

Head of NCL Specialty Clinic, University Medical Center Hamburg-Eppendorf

- Specialist in pediatric and adolescent medicine, with expertise in palliative medicine and neuropediatrics
- Research is focused on neurodegenerative brain diseases, and is the PI for clinical study: Natural History and Longitudinal Clinical Assessments in NCLs/Batten Disease, International DEM-CHILD Database

Steven Gray, PhD

Associate Professor Department of Pediatrics at UTSW and Chief Scientific Advisor to Taysha

- Expertise in AAV gene therapy vector engineering, optimizing approaches to deliver a gene to the nervous system
- Research focus includes preclinical studies to apply AAV-based platform gene transfer technologies toward the development of treatments for neurological diseases such as Rett Syndrome, Giant Axonal Neuropathy (GAN), Tay-Sachs, Krabbe, AGU, and Batten Disease, and have expanded into human clinical studies to test a gene therapy approach for GAN and CLN7 Batten disease

Suyash Prasad, MBBS, MSc, MRCP, MRCPCH, FFPM

Chief Medical Officer and Head of Research and Development at Taysha

- Expertise in international drug development, including preclinical, Phase I-IV trials, regulatory filings, commercial application
- Former CMO of Audentes Therapeutics; led XLMTM AAV8 program from preclinical to initial positive clinical data
- Prior roles include Medical Affairs and Clinical Development at BioMarin, Genzyme Therapeutics, and Eli Lilly and Company
- UK board certified with postgraduate qualifications in Pediatrics, Internal Medicine, Pharmaceutical Development, and Translational Science

CLN7 program – A strategic fit

Patient Value

- Unmet medical need
- Close collaboration with Batten Hope, the leading CLN7 patient advocacy group

Strategic Value

- Strategic fit
  - AAV9 vector platform
  - Monogenic CNS disease
  - Intrathecal delivery
  - HEK293 manufacturing process
- Aligns with Company's core competencies
- Meaningful market opportunity (estimated 4,000 patients globally)

Scientific Value

- Encouraging preclinical data in relevant rodent models suggest that first-generation construct has the potential to reduce overall disease pathology, preserve motor function and prolong survival
- Close collaboration with Dr. Steven Gray, Associate Professor in the department of Pediatrics at UT Southwestern and Taysha's Chief Scientific Advisor

Portfolio Value

- Risk management with readthrough to other programs
- Pipeline diversification
- Additional shot on goal

CLN7 late infantile disease – A derisked target

- De-risked target with two patients dosed with first-generation construct; dosing of one additional patient expected by year-end
- Preliminary human proof-of-concept data from current Phase 1 clinical trial using first-generation construct expected by year-end 2021
- Next-generation construct expected to improve potency, safety profile, packaging efficiency, and manufacturability over first-generation construct
- Initiation of a planned pivotal study with next-generation construct expected in 2022 with reference to human proof-of-concept data generated from first-generation construct
- Potential market opportunity exceeds $3B

Taysha provides grant to Batten Hope

Batten Hope

- Grant from Taysha to support patient awareness, disease education, and newborn screening initiatives
- Batten Hope directly funded GMP clinical trial material for the currently open Phase 1 clinical proof-of-concept CLN7 trial
- Gina Hann, Batten Hope Founder, President and Treasurer
  - Founded Batten Hope because she believes that every parent given a rare disease diagnosis like her son Joseph, deserves the opportunity to work toward an outcome for their child
- Batten Hope initiated a fund in collaboration with Dallas Foundation to help serve the vision of gene therapy at UTSW, under the guidance of Dr. Berge Minassian, Chief of Pediatric Neurology and Chief Medical Advisor at Taysha

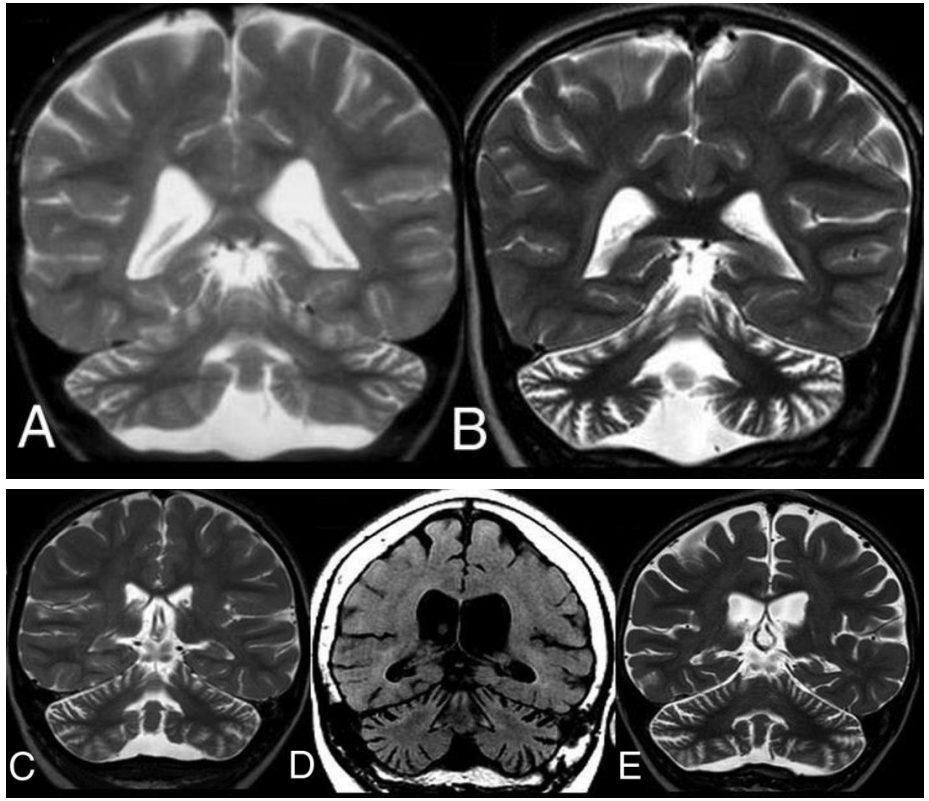
Disease Overview

Angela Schulz, MD, PhD

Head of NCL Specialty Clinic
University Medical Center Hamburg-Eppendorf

CLN7 disease is a severe neurodegenerative lysosomal storage disease

- Batten disease is the common name for rare, fatal, inherited disorders of the nervous system called neuronal ceroid lipofuscinoses (NCLs)
- CLN7 disease, a form of Batten disease, is caused by a mutation in the gene, Major Facilitator Superfamily Domain Containing 8 (*MFSD8*), resulting in a lysosomal storage disease (LSD)
- CLN7 presents as a “late infantile” or “variant late infantile” disease, with almost all patients experiencing symptom onset (>95% of reported cases) between ages 2-7 years
- Inherited autosomal recessive pattern - both copies of the *CLN7* gene variant (one from each parent, or carrier) must be present for diagnosis
- Survival rarely beyond teenage years
- Symptomatic intervention to treat seizures, dystonia, anxiety, sleep disorders, and spasms
- Estimated prevalence is 4,000 patients worldwide – numbers of diagnosed patients have significantly increased over the last 3 years with newly genetic testing techniques such as next generation sequencing panels

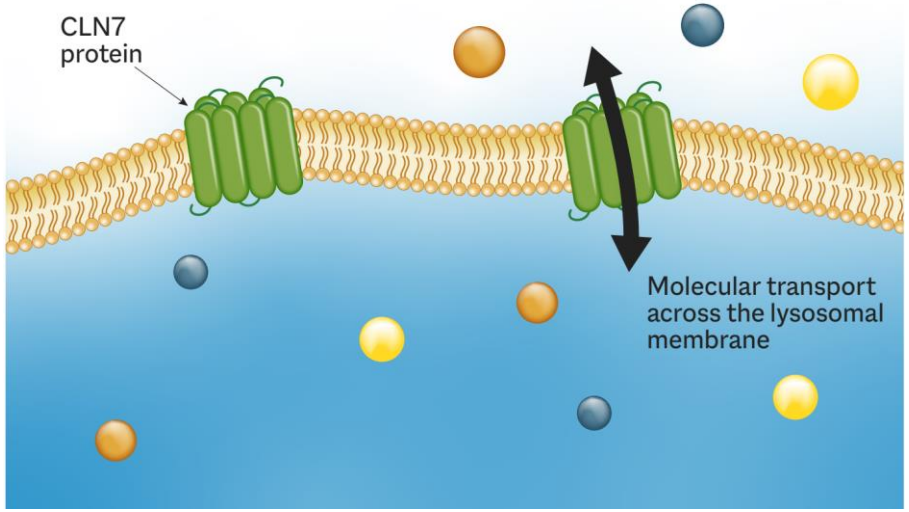


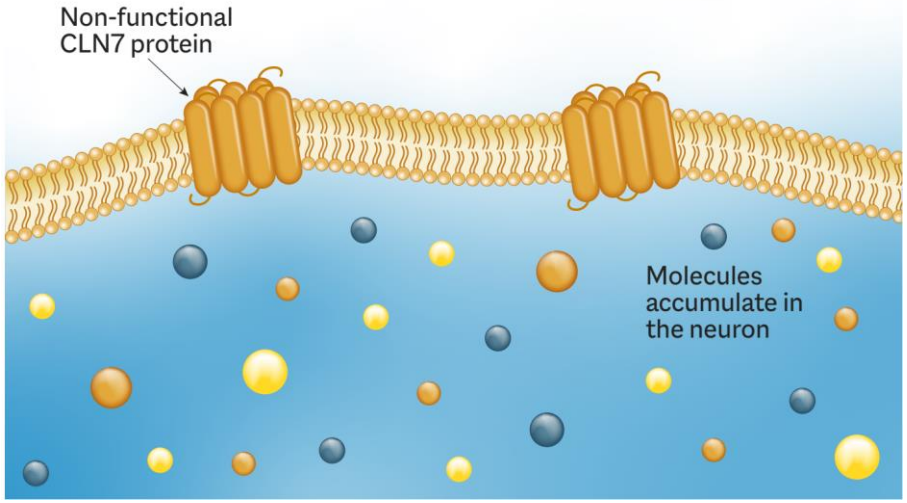
Coronal T2-weighted MR imaging of 4 different patients (*A*, *B*, *C*, and *E*) and coronal reformatted CT of 1 patient (*D*). Note predominant cerebellar-over-cerebral atrophy.

1. Anderson GW, Goebel HH, Simonati A. Human pathology in NCL. *Biochim Biophys Acta*. 2013;1832(11):1807-1826. doi:10.1016/j.bbadis.2012.11.014
2. Mink JW, Augustine EF, Adams HR, Marshall FJ, Kwon JM. Classification and natural history of the NCLs. *J Child Neurol*. 2013;28(9):1101-1105. doi:10.1177/0883073813494268
3. Biswas A, Krishnan P, Amirabadi A, Blaser S, Mercimek-Andrews S, Shroff M. Expanding the Neuroimaging Phenotype of NCLs. *AJNR*. 2020;41(10):1930-1936. doi:10.3174/ajnr.A6726
4. https://www.ninds.nih.gov/Disorders/Patient-Caregiver-Education/Fact-Sheets/Batten-Disease-Fact-Sheet

Molecular underpinnings of CLN7

- More than 45 different *CLN7* mutations have been characterized
- Disruption of the *MFSD8/CLN7* gene identified as the cause of the LINCL form of the NCLs
- Although the exact function of the protein is unknown, it is thought to transport molecules across the lysosomal membrane
- Dysfunction of the MFSD8 protein results in accumulation of lysosomal storage material or autofluorescent ceroid lipopigments in neuronal and peripheral tissues
- Accumulations can cause cell damage leading to cell death
- Individuals with CLN7 disease have gradual nerve cell loss in certain parts of the brain, which likely leads to the signs and symptoms of this condition





1. Connolly KJ, O'Hare MB, Mohammed A, et al. The neuronal ceroid lipofuscinosis protein Cln7 functions in the postsynaptic cell to regulate synapse development. *Sci Rep*. 2019;9(1):15592. Published 2019 Oct 30. doi:10.1038/s41598-019-51588-w
2. Kousi M, Siintola E, Dvorakova L, et al. Mutations in CLN7/MFSD8 are a common cause of variant late-infantile neuronal ceroid lipofuscinosis. *Brain*. 2009;132(Pt 3):810-819. doi:10.1093/brain/awn366

Case report of CLN7 patient – 4 years 10 month old boy

Non-consanguineous family

Motor Developmental Milestones	
Sitting	6 mo.
Crawling	7 mo.
Standing w/ no support	8 mo.
Walking w/ no support	10 mo.

Language Developmental Milestones	
First words	10 mo.
2-word sentences	36 mo. – Delayed

Clinical Presentation	
First seizure	3 yrs. 8 mo.
Motor language decline	3 yrs. 8 mo.

Diagnostics	
cMRI showing cerebral atrophy	3 yrs. 8 mo.

Genetics	4 yrs.*
Homozygous mutation in *CLN7* gene	
c.754+2T>A / c.754+2T>A (splice defect)	

*Four months after symptom onset

Case report of CLN7 patient

16 months

18 months

21 months

21 months

Case report of CLN7 patient

3 years 9 months

4 years

4 years 9 months

4 years 9 months

Batten disease natural history data

Ongoing natural history studies in the EU and US underway that encompass all NCLs, including CLN7:

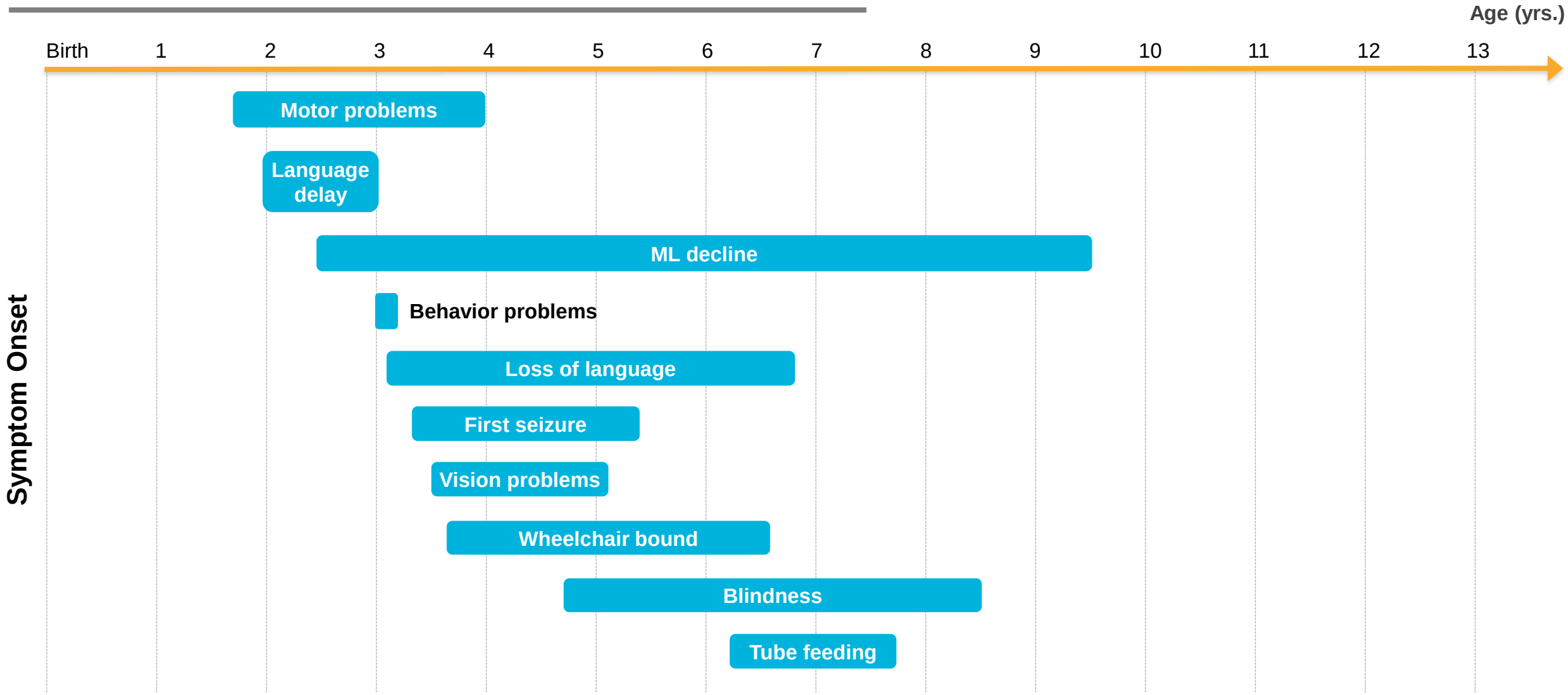
- Coordination of international DEM-CHILD Patient Database for all NCLs (data on >250 NCL patients)
  - To collect precise natural history data of all NCL types
  - To improve early diagnosis of NCLs
  - To optimize standard of care for patients
  - To establish evaluation tools for experimental therapies
  - To make these data available to third parties (scientists and industry) in a transparently regulated and time-effective manner
- Publication anticipated in the near future by UTSW

1. https://clinicaltrials.gov/ct2/show/NCT04613089

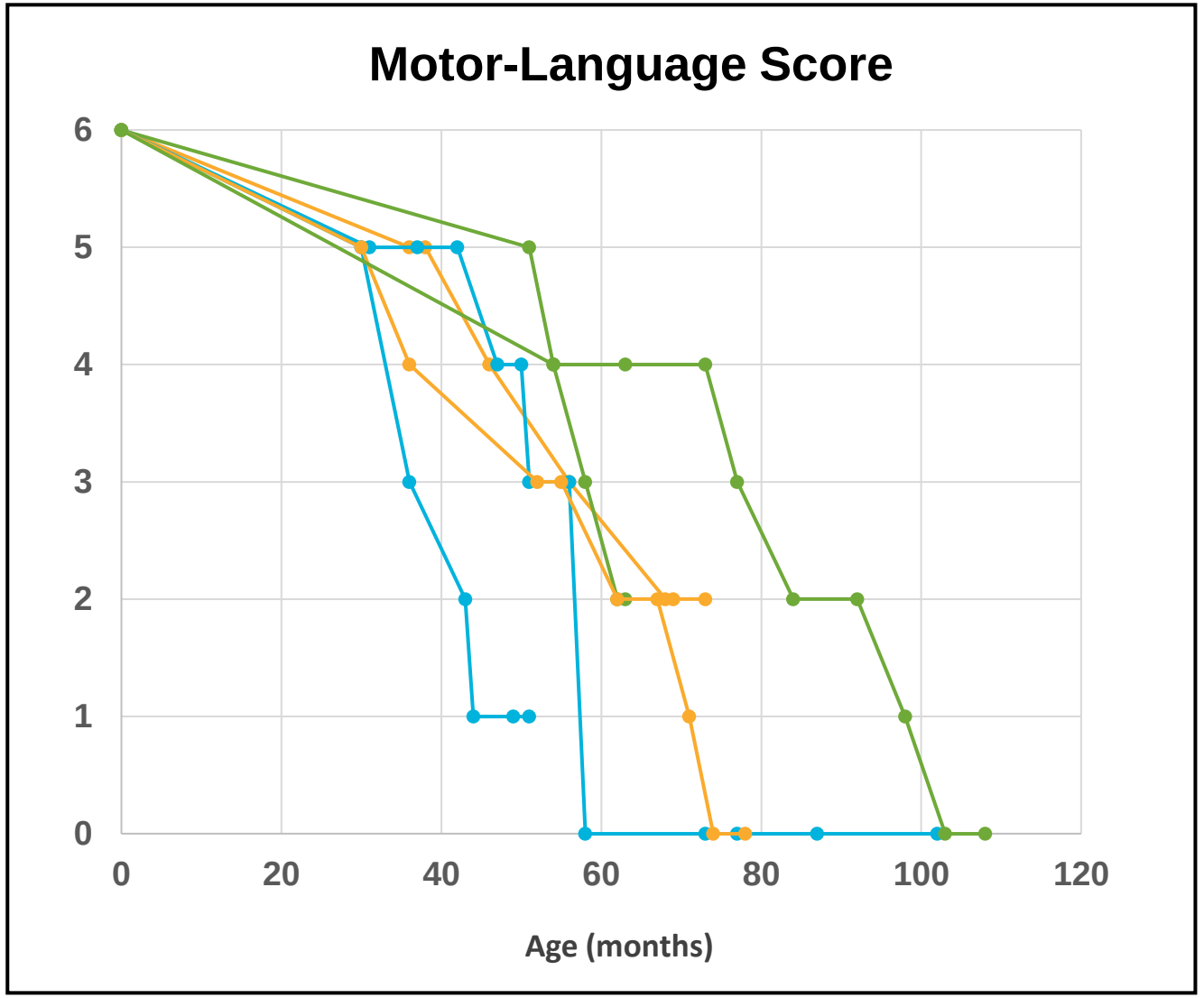
Natural history cohort of 12 patients (Hamburg)

Type of first symptom	Patient numbers	Patient age (mean / range)
Motor problems	9	3 yrs. 3 mo. (1 yr. 9 mo. – 4 yrs.)
Language delay	8	2 yrs. 5 mo. (2 yrs. – 3 yrs.)
Vision problems	3	4 yrs. 2 mo. (3 yrs. 6 mo. – 5 yrs.)
Behaviour problems	2	3 yrs. (3 yrs. – 3 yrs. 1 mo.)
Progression of symptoms	**Patient numbers (%)**	**Patient age (mean / range)**
First seizure	10 (100)	4 yrs. 5 mo. (3 yrs. 4 mo. – 5 yrs. 9 mo.)
Start of motor / language decline	10 (100)	4 yrs. 7 mo. (2 yrs. 6 mo. – 9 yrs. 6 mo.)
Wheelchair bound	6 (60)	5 yrs. 8 mo. (3 yrs. 8 mo. – 6 yrs. 5 mo.)
Complete loss of expressive language	4 (40)	5 yrs. 8 mo. (3 yrs. 2 mo. – 6 yrs. 8 mo.)
Completely blind	7 (70)	6 yrs. 2 mo. (4 yrs. 10 mo. – 8 yrs. 6 mo.)
Tube feeding	4 (40)	6 yrs. 9 mo. (6 yrs. 3 mo. – 7 yrs. 10 mo.)

CLN7 disease – Age ranges at first symptom onset



Hamburg LINCL Motor-Language Scale in CLN7 patients



Preclinical Pharmacology and Toxicology Data

Steven Gray, PhD

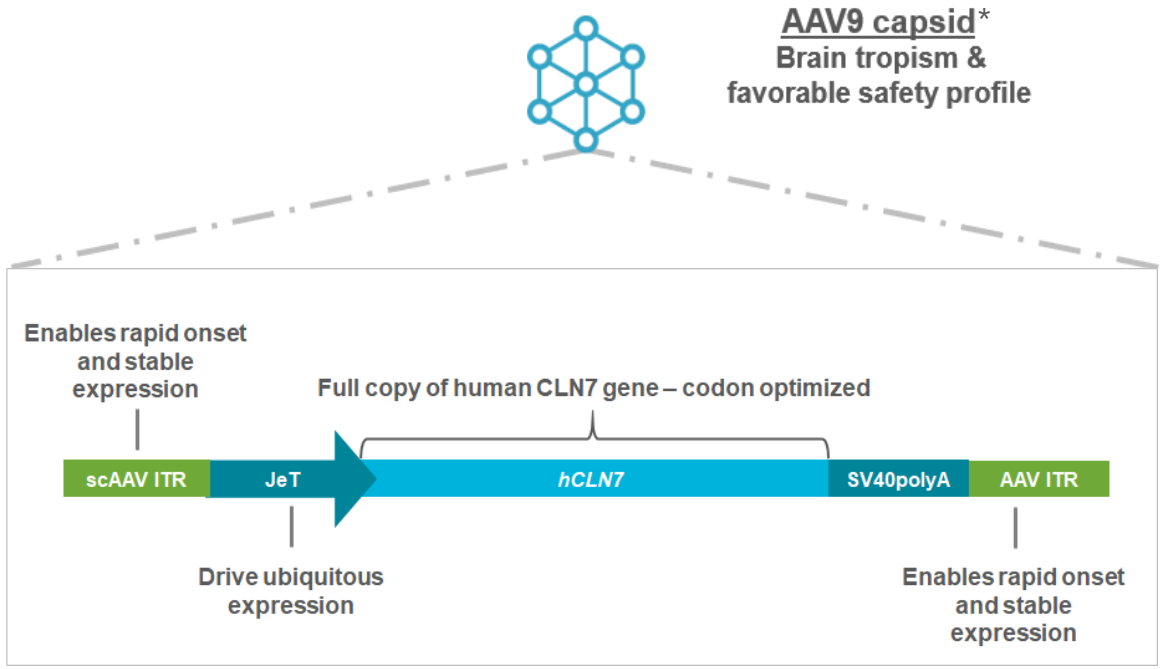
Associate Professor, Department of Pediatrics at UTSW
Chief Scientific Advisor, Taysha

Disclosure: Dr. Gray is an inventor on the design of the CLN7 vector and has the potential to receive royalty income related to this invention.

CLN7 is a neurodegenerative disease caused by autosomal recessive mutations in the *MFSD8* gene[1]

- MFSD8 is a 518 amino acid transmembrane protein thought to be a lysosomal protein[1]
- *MFSD8* mutations are associated with a uniform late-infantile manifestation, suggesting mutations cause a complete loss of gene function[2]
  - One exception of a mutation causing a juvenile onset form of the disease has been described[3]
  - Specific compound heterozygous alleles are associated with non-syndromic localized maculopathy in adults[4]
- Disease onset typically occurs at 2-7 years of age
  - Presenting symptoms include developmental regression and seizures[2]
- Disease progression is rapid with further mental and motor regression and profound speech impairment[2]
- Mean age of death is 11.5 years of age (range 6.5-18 years) [2]
- First-generation construct in clinical development with next-generation construct to be used in planned pivotal study

* First-generation construct



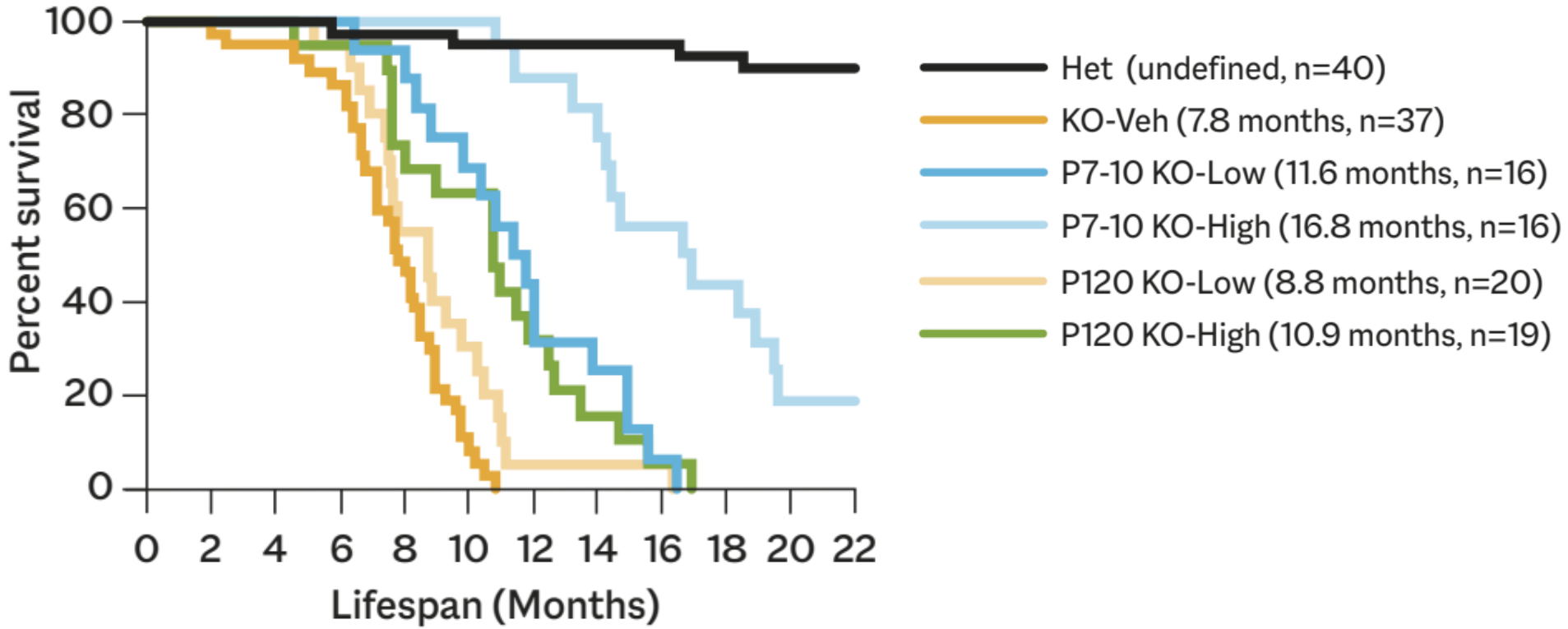
- Aims to deliver a full-length copy of hCLN7 using the AAV9 capsid
- Engineered for rapid and sustained gene expression

1. Siintola et al. Am J Hum Genet 2007, 2. Kousi et al. Hum Mutation 2012, 3. Kousi et al. Brain 2009,, 4. Khan et al. Inve Opthal Vis Sci. 2017

First-generation CLN7 construct – Key preclinical studies to date

#	Study Scope (ID)	Model System	Route of Administration & Dose (vg/animal)	Major Findings
1	In vitro PoC Assay (NSR-004.2-PHAR-1)	Patient fibroblasts (CLN7 -/-)	Titers tested were 1×10^{3}, 1×10^{4}, 1×10^{5}, and 5×10^{5}	• Mutations in CLN7 reduce lysosomal function and restoring the expression of wild-type CLN7 in patient fibroblast lines with AAV vectors can restore function in living cultures
2	PoC In vivo efficacy study (NSR-004.2-PHAR-2) (NSR-004.2-PHAR-3)	CLN71d mice	IT KO Low: 1.25×10^{11} IT KO High: 5×10^{11}	• Accumulation of SCMAS and astrogliosis are major hallmarks of the underlying disease pathology, and CLN7 gene transfer into the mouse model reduces this pathology • Survival was greatly improved in a dose and age of treatment dependent manner, evident that high dose at earlier age provides the largest benefit for survival and quality of life • Behavioral rescue in Rotarod performance, a measure of motor function, was also observed in treated mice up to 9 months following injection, providing evidence for durable therapeutic benefit beyond survival
3	Non-GLP immunotoxicity study (NSR-004.2-TOXI-1)	First-generation construct in CLN71d mice	IT KO Low: 1.25×10^{11} IT KO High: 5×10^{11}	• An Interferon γ (INFγ) response is unlikely to confound the preclinical safety or efficacy studies conducted for first-generation construct • The codon-optimized human CLN7 transgene has a low risk of generating novel immune epitopes that would stimulate an INFγ response
4	Dose-ranging non-GLP chronic toxicology study (NSR-004.2-TOXI-2)	Juvenile WT CH57BL/6J mice	IT 8WK: 9.5×10^{11} IT 6WK: • 4.47×10^{11} • 1.48×10^{11} • 4.47×10^{10}	• First-generation construct vector does not affect weight or body condition over a 1-year period of longitudinal monitoring • Histopathology did not find any signs of toxicity due to first-generation construct at 1-year post-injection • No significant difference of survival rates between male or female mice • IT doses up to 9.5×10^{11} vg/mouse are safe and well tolerated in WT mice. Highest dose injected in the mice is a 3.8-fold higher titer than the highest dose proposed in humans, and twice the volume proposed in humans. Thus, the maximum tolerated dose in mice up to one-year post-injection provides a wide safety margin above what is proposed in humans
5	Dose-ranging GLP toxicology study (NSR-004.2-TOXI-3)	Sprague Dawley juvenile rats	IT Low: 5×10^{11} IT Mid: 2×10^{12} IT High: 6×10^{12}	• Following out to 91 days post-injection, found no significant test article-related effects on study parameters, with the exception of increased thymus weights in males at high dose, suggesting first-generation construct was overall well tolerated • No test article-related clinical observations, body weight, food consumption, or accelerating Rotarod values, therefore, the NOAEL was 6×10^{12} vg/animal

CLN7 knockout mice treated with first-generation construct had improved survival rates

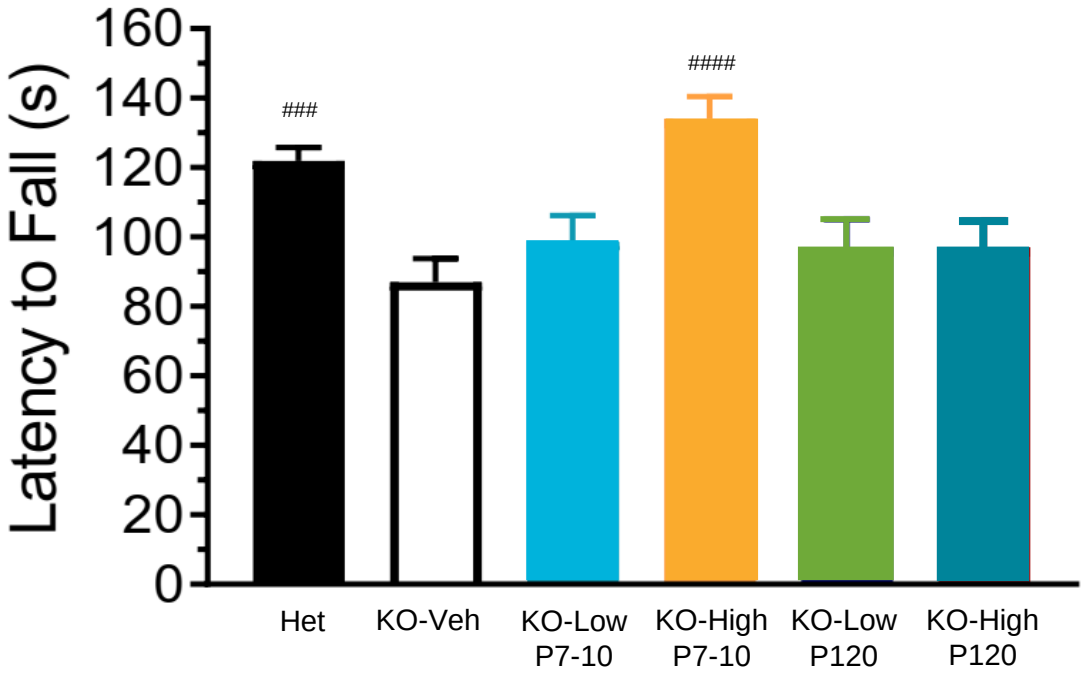


- No behavioral deficits observed between Het control animals and KO-Veh or KO-first-generation construct dosed animals at 2 months or 4 months of age
- Survival of KO-Veh animals drastically decreased between 6 and 9 months of age
- High-dose/early treatment group showed the greatest survival benefit (doubled lifespan), and low-dose/late treatment groups showed moderately increased survival

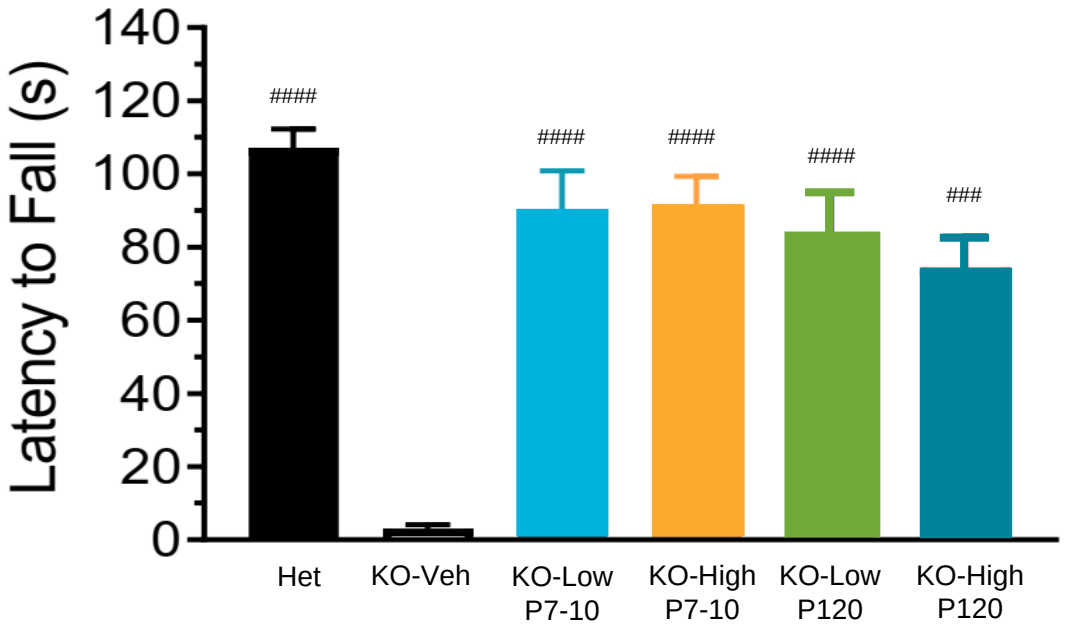
Low dose: 1.25×10^{11} vg/animal
High dose: 5×10^{11} vg/animal

CLN7 knockout mice treated with first-generation construct had sustained preservation of motor function on rotarod

6 Month Data*



9 Month Data*



* Rotarod: Maximum

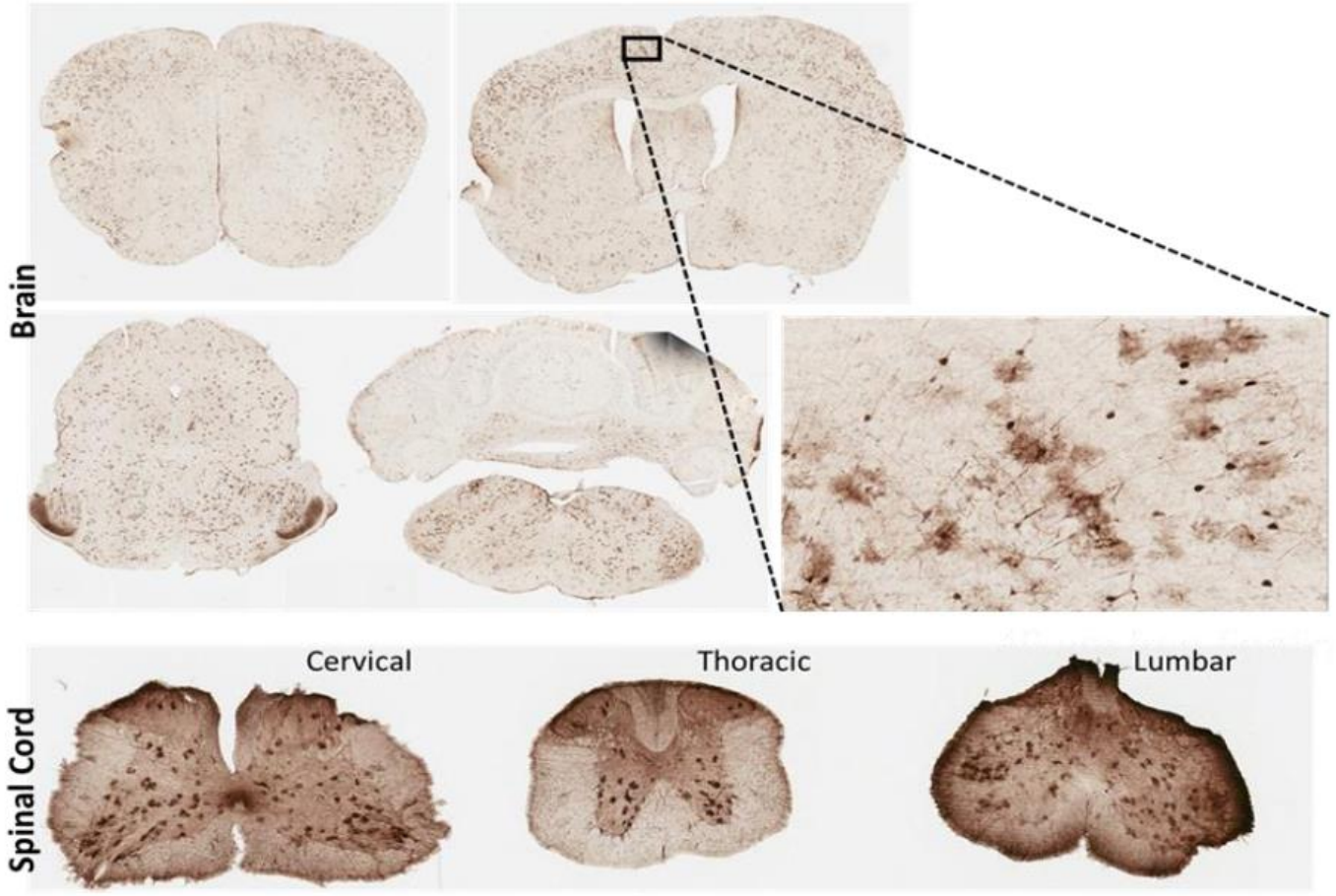
###p<0.001, ####p<0.0001 compared to KO-Veh

Low dose: 1.25 ×10^{11} vg/animal

High dose: 5 ×10^{11} vg/animal

Widespread GFP expression throughout the CNS and peripheral tissues with first-generation construct

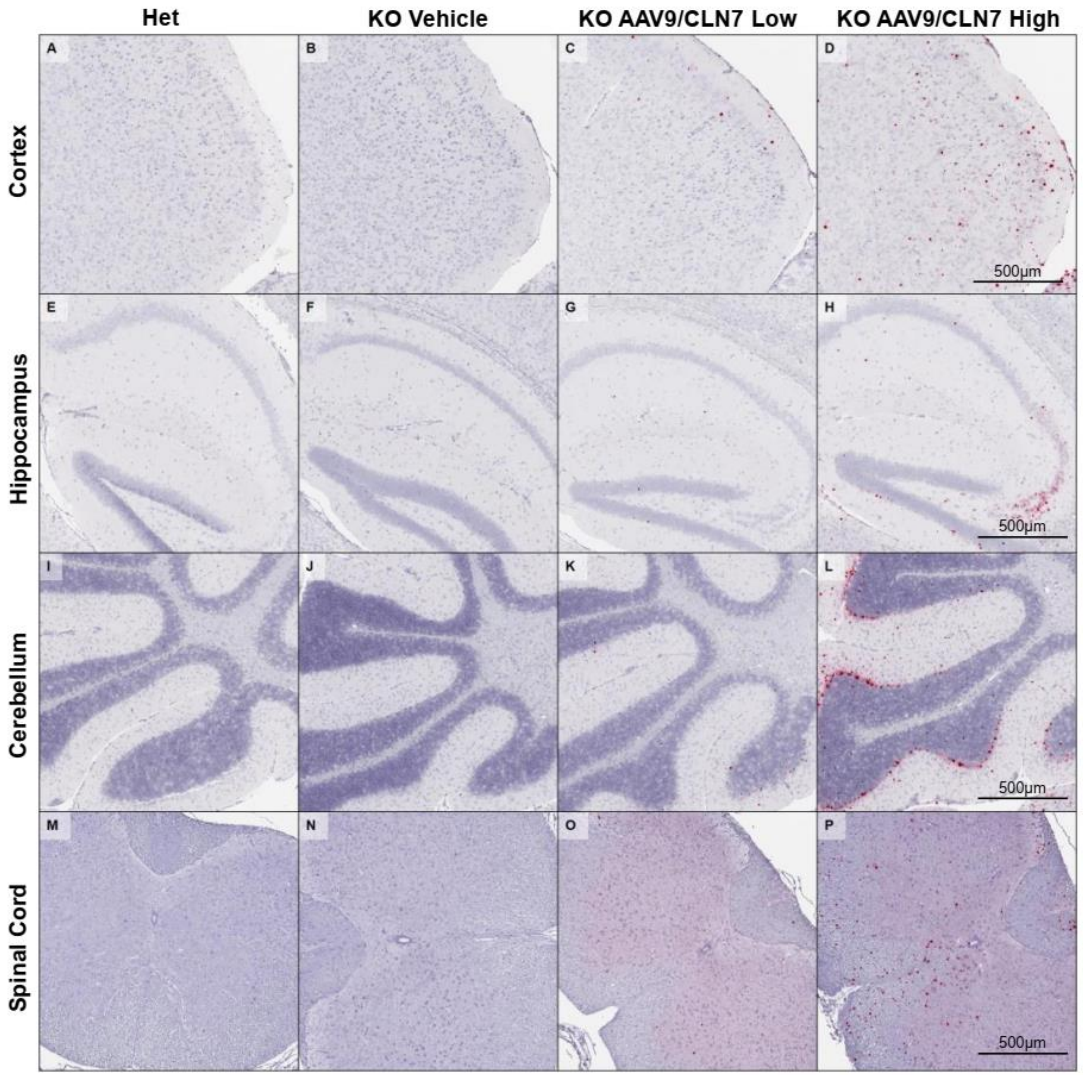
- A high-dose IT AAV9 study was conducted in eight-week-old wild type mice, using a self-complementary AAV9/CBh-GFP reporter vector at a dose of $4.15x10^{11}$ vg per mouse
- This vector construct is identical to the first-generation construct, except that the GFP expression cassette is inserted in place of the CLN7 expression cassette
- The dose is 1.8-fold higher than the high IT dose of the first-generation construct vector that was tested. This resulted in widespread GFP expression throughout the CNS and peripheral tissues
- At 4 weeks post-injection, immunohistochemistry of the brain and spinal cord was conducted to visualize the spatial distribution of GFP expression (n=4)
  - GFP expression is indicated by brown staining



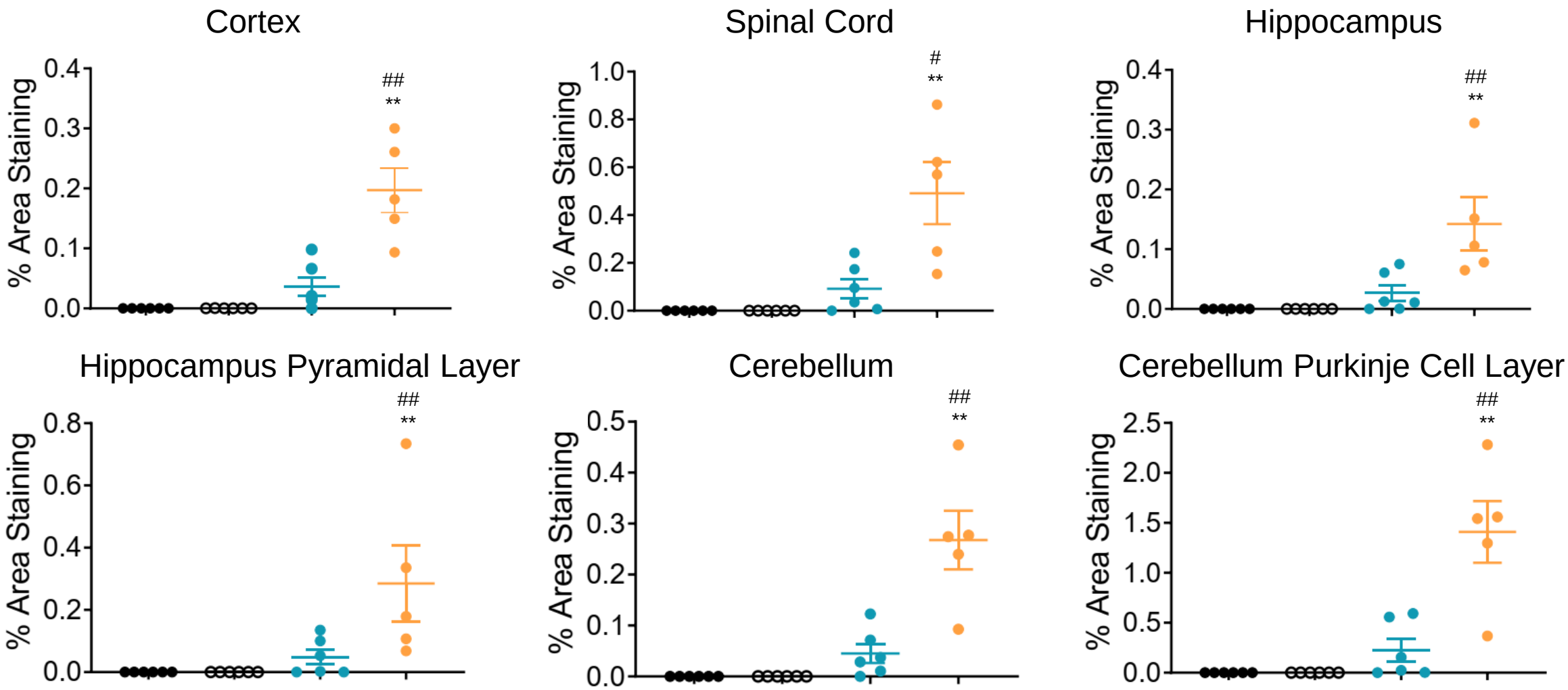
Bailey RM, Rozenberg A, and Gray SJ. Brain Research, 2020

mRNA staining in CLN7 knockout mice demonstrated target tissue expression

- RNAscope staining was used in a semi-quantitative analysis of hCLN7 mRNA expression in the brain and spinal cord of first-generation construct-dosed CLN71d KO mice
- CLN71d mice treated with first-generation construct at PND 7-10 also had dose-dependent detectable levels of hCLN7 mRNA in all tissues and brain regions assessed up to 4.5 months post-injection
- Positive hCLN7 mRNA staining is observable as a deep red color
- These results provide evidence for target tissue expression in animal models of CLN7 disease, and further highlight the need of a high dose to achieve desirable CLN7 expression levels across the CNS
- Results are consistent with previous published studies using a GFP reporter

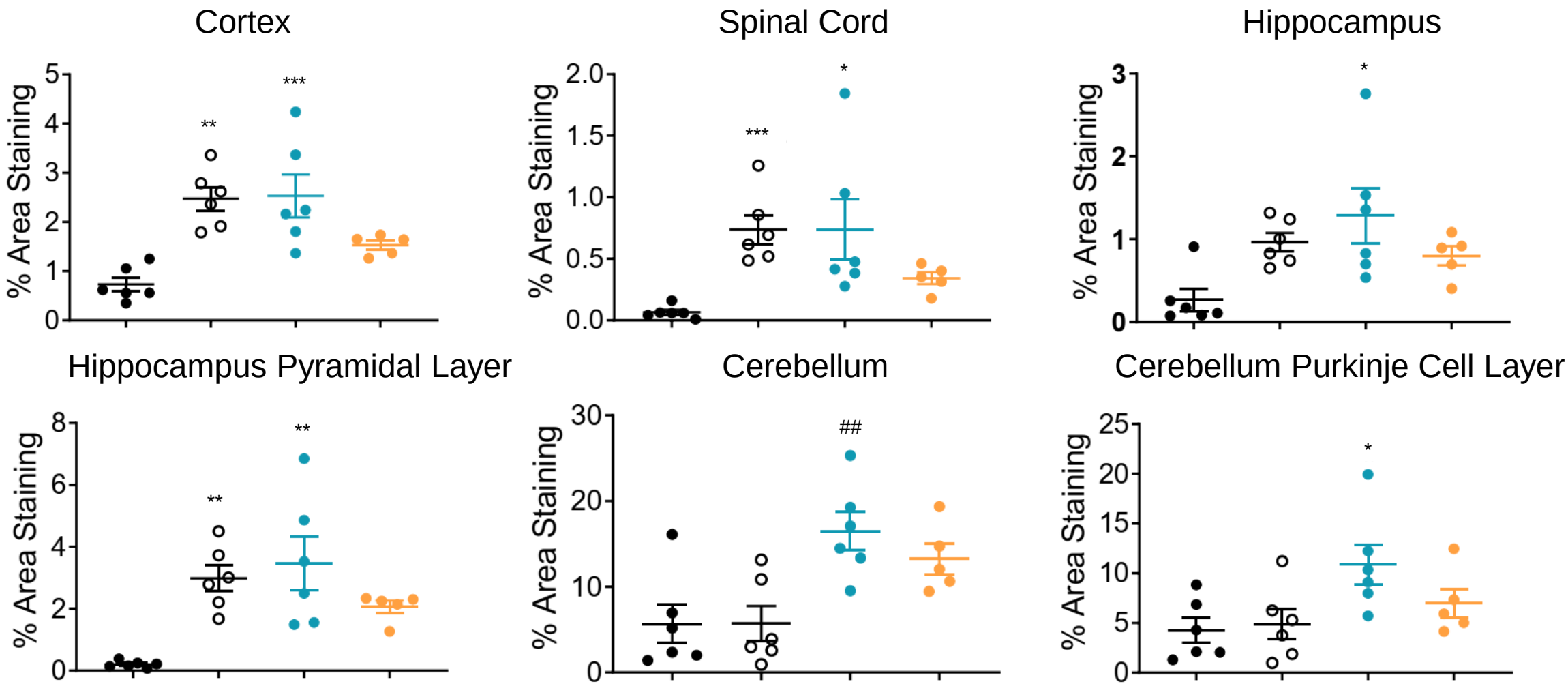


Percent area staining positive for hCLN7 mRNA by tissue region from first-generation construct



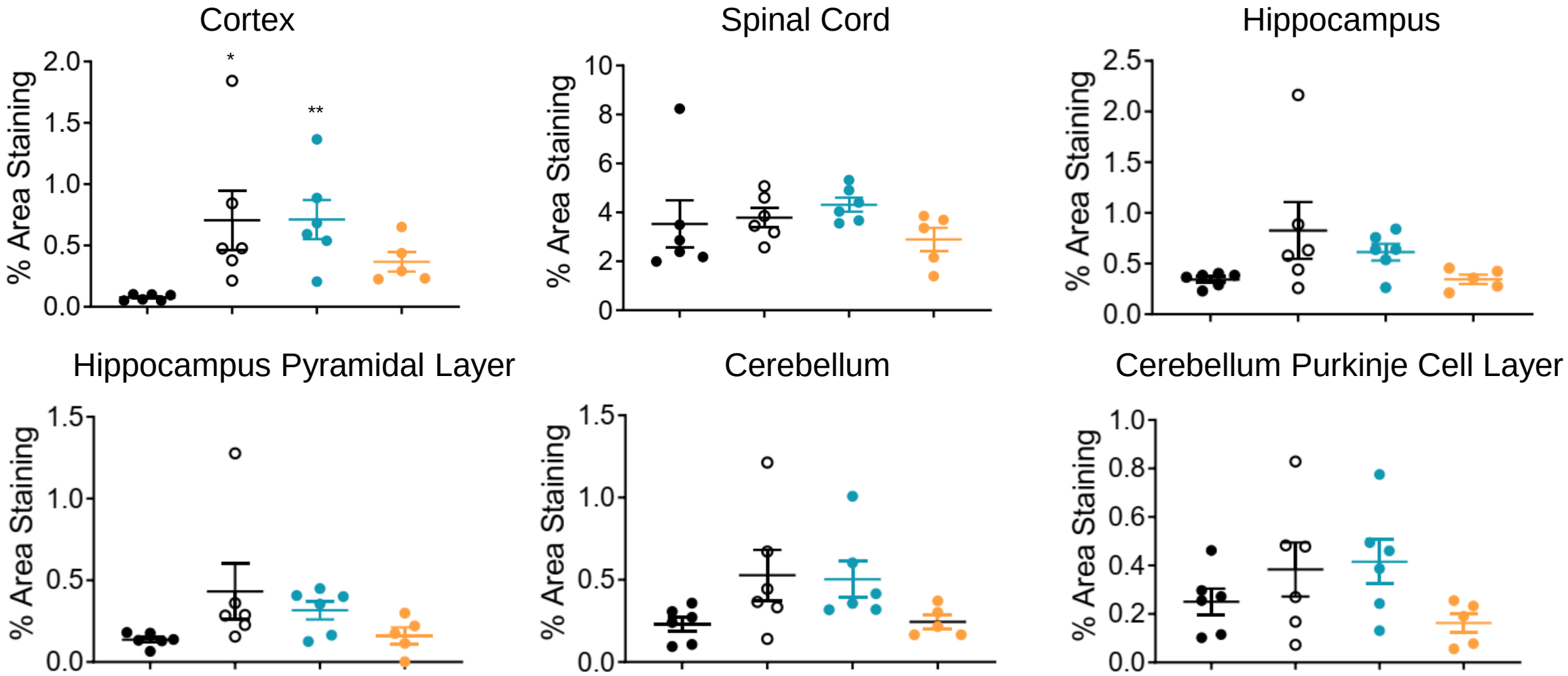
**p<0.01 compared to Het
##p<0.01, #p<0.1 compared to KO-Veh

Dose-responsive reduction in SCMAS (lysosome storage material) across the CNS with first-generation construct



*p<0.1, **p<0.01, ***p<0.001 compared to Het
##p<0.01 compared to KO-Veh

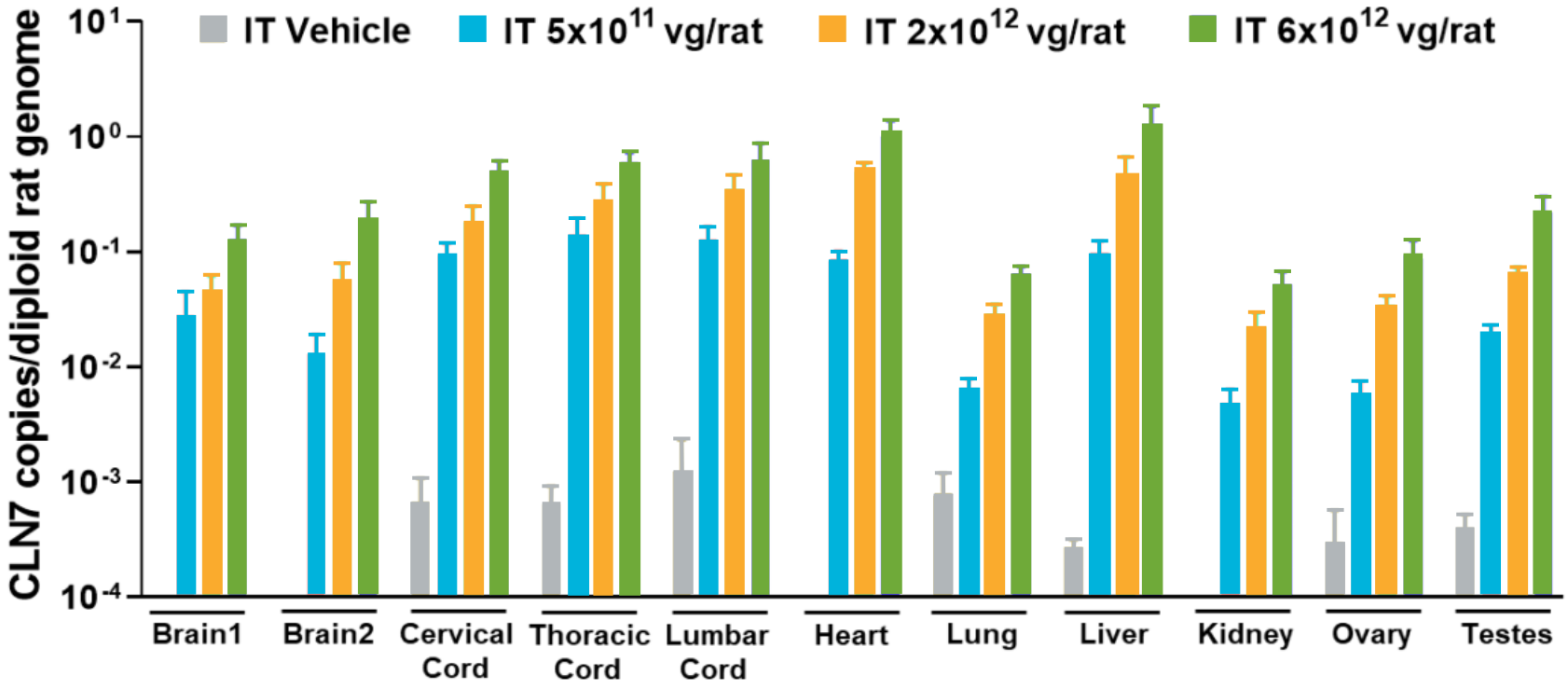
Dose-responsive reduction of GFAP with first-generation construct demonstrated reduced inflammation across the CNS



*p<0.1, **p<0.01 compared to Het

Safety and biodistribution of first-generation construct in wild type rats

- IT delivery of first-generation construct resulted in dose dependent increase of CLN7 vector DNA across the CNS (brain and spinal cord) and peripheral organs (heart, lung, liver, kidney, ovary, and testes)
- Biodistribution patterns matched expected results based on previous published and unpublished studies
- Overall, first-generation construct was well-tolerated across all dose levels and time points



Clinical Development Strategy

Suyash Prasad, MBBS, MSc, MRCP, MRCPCH, FFPM

Chief Medical Officer and Head of R&D

Translating nonclinical data into human setting

- Disrupting the *CLN7*/*MFSD8* gene in mice by targeted deletion of exon 2 generating a novel knockout (KO) mouse model for CLN7 disease recapitulates key features of human CLN7 disease pathology[1]
  - The ultrastructure of the storage material in neurons of CLN7 KO mice resembled the storage material found in brains of CLN7 patients
  - MRI revealed brain atrophy in the olfactory bulb, cerebellum and cerebral cortex of CLN7 KO mice at the end stage of the disease. CLN7 KO mice recapitulated key neuropathological features of human CLN7 disease, in which neuroinflammation and neurodegeneration in the cerebellar and cerebral cortex were observed
- In CLN71 mice, there was age and dose-dependent rescue of multiple disease phenotypes, providing strong evidence for benefit of the first-generation construct for CLN7 disease[2]
- Dose selection of 5.0 $\times 10^{14}$ total vg for the first patient followed by 1.0 $\times 10^{15}$ total vg for subsequent patients in the human clinical trial is based on the minimum effective dose (MED) in the mouse model and the NOAEL from the toxicology study

1. Brandenstein L, Schweizer M, Sedlacik J, Fiehler J, Storch S. Lysosomal dysfunction and impaired autophagy in a novel mouse model deficient for the lysosomal membrane protein Cln7. *Hum Mol Genet*. 2016;25(4):777-791. doi:10.1093/hmg/ddv615
2. *In vivo Efficacy: AAV9/CLN7 Ameliorates Behavioral Deficits and Improves Survival in CLN71d Mouse Model of Batten Disease (NSR-004.2-PHAR-3)*

Current Phase 1 open-label, clinical proof-of-concept CLN7 trial with first-generation construct

Goals and Targets of Trial

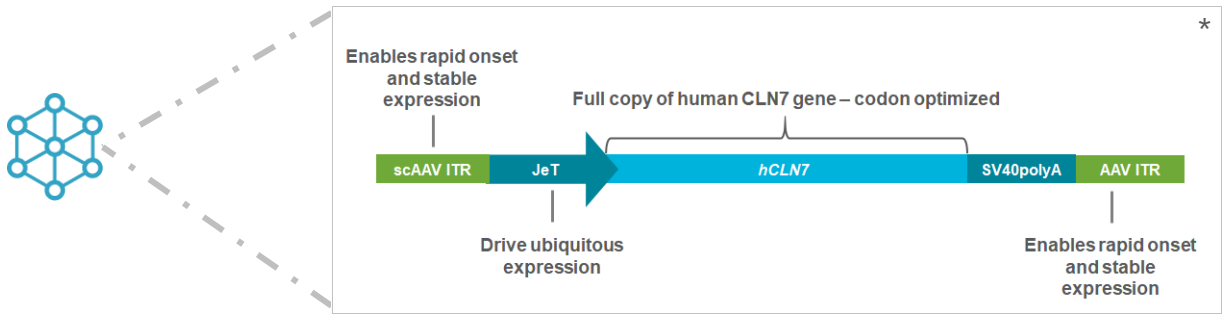
Goals

- **Primary** – Safety and tolerability by incidence and severity of treatment related SAEs
- **Secondary** – Efficacy: motor, cognition, and intelligence assessments

Target Recruitment

- 4 subjects 1-18 years of age, may expand number of patients based on DSMB discussions
- First patient has received initial dose of 5.0 x 10^{14} total vg
- Second patient has received higher dose of 1.0 x 10^{15} total vg
- Subsequent participants to receive higher dose of 1.0 x 10^{15} total vg

Product Details and Dose Cohorts



* First-generation construct

Dose Cohorts

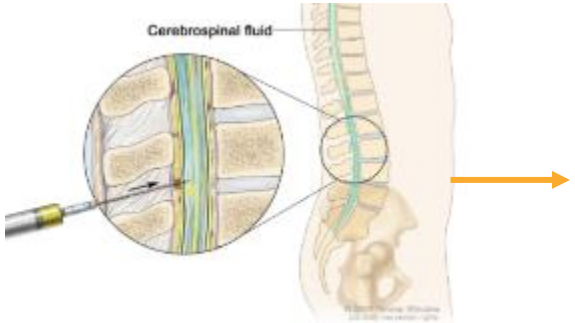
- 5.0 x 10^{14} total vg (n=1)
- 1.0 x 10^{15} total vg (n=3)

Doses are for participants ≥4 years of age and will be scaled down for younger participants as appropriate

Route and Method of Administration

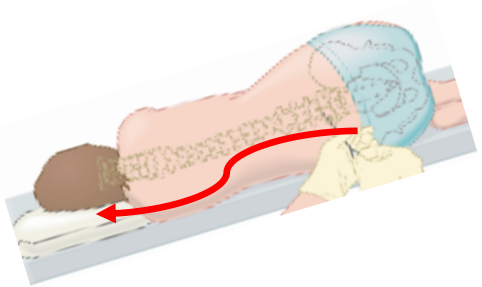
Administration

- Lumbar Intrathecal Infusion (IT)
- Amount and rate: 1 mL/min for total of 10-12 mL
- Immunosuppression regimen of prednisolone and sirolimus



Technique to Improve Transduction

- Trendelenburg position (15-30º)
- During infusion & 1 hour post infusion



Efficacy outcome measures

Disease burden assessments
- Clinical Global Impression Scale (CGI)
- Seizure diary
- Swallow function

Motor function assessments
- Timed Walk tests (2-Minute or 6-Minute Walk Test)
- Pediatric balance scale
- Gross Motor Function Measure (GMFM)

Neuropsychological/Developmental
- Mullen Scales of Early Learning
- Vineland Adaptive Behavior Scales (Vineland-3)

Imaging and neurophysiology
- Brain MRI
- Standard awake 60-minutes electroencephalogram (EEG)

Ophthalmologic assessments
- Eye examination
  - Visual acuity (VA)
  - Electroretinography (ERG)
  - Optical Coherence Tomography (OCT)

Quality of Life and Disease Burden measures
- Quality of Life Inventory-Disability (QI-Disability)
- Infant/Toddler Quality of Life Questionnaire (ITQOL)
- Healthcare resource utilization

Bridging from first-generation to next-generation construct for planned pivotal studies

- Next-generation construct design anticipated by year-end 2021
- Next-generation design enables opportunity to improve potency and potentially deliver lower doses
- Next-generation construct anticipated to improve safety profile over first-generation construct
- Significant improvements to packaging efficiency and manufacturability resulting in higher yields and lower cost of goods sold
- Commercial-grade GMP material for next-generation construct available in 2022
- Initiation of planned pivotal clinical trial with next-generation construct in 2022 with reference to clinical data from first-generation construct
- Oral presentation of preclinical CLN7 data from the first-generation construct at the 17th Annual International Congress on Neuronal Ceroid Lipofuscinosis (NCL) on October 8, 2021
- Poster on design rationale and discussion of outcome measures for ongoing Phase 1 clinical trial for the first-generation construct to be presented at the 17th Annual International Congress on NCL

Q & A

Closing Remarks

RA Session II

President, Founder & CEO

Thank you to our partners!

UT Southwestern Medical Center | Research Labs

Steven Gray Lab

BATTENHOPE

MILA'S MIRACLE FOUNDATION TO STOP BATTEN

Focused on achieving anticipated near-term milestones in 2021 and building long-term value

GAN clinical program update, including 3.5 x 10^{14} total vg cohort
GM2 gangliosidosis preliminary biomarker data in 2H 2021
CLN1 program to dose first patient in 2021 under open IND
Preliminary CLN7 data for first-generation construct by YE 2021

5 open IND/CTAs expected by the end of 2021, including Rett syndrome

Major demolition and detailed design of internal cGMP facility completed

5 additional programs currently in IND-enabling studies
TSHA-106 Angelman Investor Day October 2021
Numerous value generating catalysts over the next 18 months

TAYSHA GENE THERAPIES

Thank you