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Positive Interim Clinical Data from Ongoing Phase 1/2 Study in CLN6 Batten Disease

August 1, 2019

Forward-Looking Statements

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials and the prospects and timing of the potential regulatory approval of our product candidates. In particular, this press release relates to interim data from an ongoing Phase 1/2 study to investigate intrathecal administration of AAV-CLN6 gene therapy. The inclusion of forward-looking statements arising from this interim data, ongoing study and natural history preliminary data 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, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: 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; and the potential that we will need additional funding to complete all of our studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. The interim data and Phase 1/2 study discussed herein is inherently preliminary and early in the study, derived from a limited patient set, and later trial results with this patient set or others may not be consistent with these preliminary 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, 2019 and Quarterly Report on Form 10-Q for the quarter ended March 31, 2019. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

5 Key Takeaways for AAV-CLN6 Gene Therapy

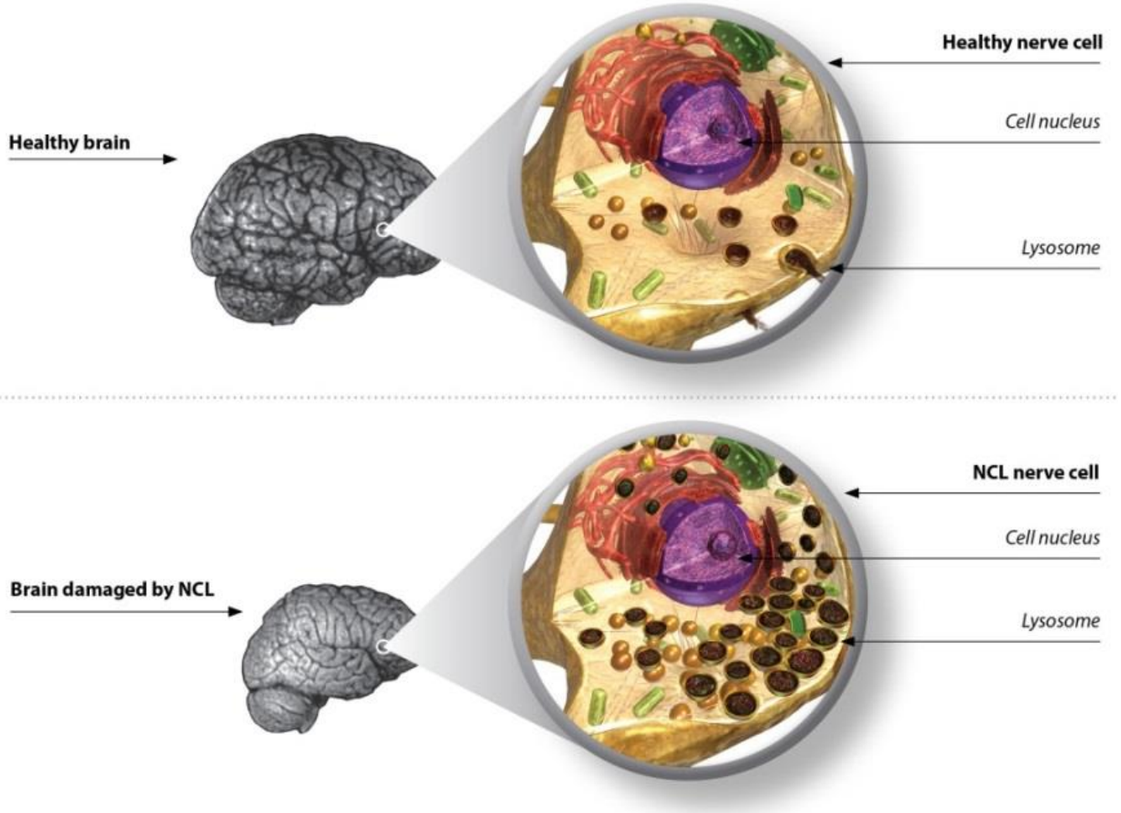
Interim Safety and Efficacy Data Demonstrate the Potential for AAV-CLN6 Gene Therapy to Stabilize Progression of a Devastating Disease

- **Meaningful impact on motor and language function** in children with a fatal neurologic disease that destroys brain function
- **Evidence of disease stabilization** in seven out of the eight children following AAV-CLN6 gene transfer
- **Natural history cohort shows progressive loss of language and motor function** in all untreated patients
- **Sibling comparisons (in-study and natural history) provide further support** for AAV-CLN6 gene therapy and early intervention
- **Favorable safety profile** with intrathecal administration of AAV in all study participants

CLN6 Batten Disease Overview

CLN6 Batten Disease is a Fatal Neurologic Disease that Rapidly Robs Children of Their Ability to Walk, Speak, Think, See, and Often Ends in Death During Childhood

- Mutation in CLN6 gene leads to lysosomal dysfunction
- Usually presents at 2-3 years of age after typical childhood development
- Rapidly robs children of their ability to walk, speak, think, and see
- No approved therapy and urgent need for treatment
- Early intervention is critical
- Estimated population is ~1,000 globally



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

Hamburg Motor & Language Scale

Following Symptom Onset, Natural History Indicates Rapid Degradation of Motor and Language Ability, on the Hamburg Scale, with Each Point Decline Representing Significant Impairment

Hamburg Motor & Language Scale

Motor Function	Language Function
3 Normal	3 Normal
2 Clumsy, falls	2 Abnormal
1 Non-walking	1 Minimal
0 Immobile	0 Unintelligible or no vocalization

In each domain, the rating is structured so that a score of:

3 = normal condition

2 = slight or just noticeable abnormality

1 = severe abnormality

0 = complete loss of function

Wyrwich et al. 2018. Journal of Inborn Errors of Metabolism and Screening

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Clinical Study Design

Safety and Efficacy of a Single Administration of Intrathecally Delivered AAV-CLN6 Gene Therapy Evaluated for a Number of Key Parameters Including Hamburg Motor + Language Score

Key Eligibility Criteria

- Diagnosis of CLN6 determined by genotyping
- Hamburg motor and language score ≥3
- Age ≥1 year

Efficacy Evaluations

- Hamburg scale
- UBDRS
- Additional measures include: Cognitive and Language Ability, Vision, QOL, Ophthalmologic Assessments, Brain MRI

Baseline Characteristics (n=8)

Study Represents a Cross Section of CLN6 Batten Disease with Interim Efficacy Results Now Available for 8 Patients Who Have up to 2 Years of Follow up After Treatment

Patient	Gender	Age at Enrollment (months)	Exposure Duration (months)*	Hamburg Motor + Language at Baseline	Time Between Baseline and Last Measure (Months)
1	F	63	39	3	25
2	F	30	38	6	23
3	M	36	36	5	24
4	M	66	28	4	24
5	F	79	27	3	24
6	M	56	26	5	24
7	M	19	20	5	19
8	M	61	17	4	16

*Calculated to June 1, 2019. Presented data include all sibling pairs with ≥1 year data and non-sibling pts with ≥2 year data

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Clinical Safety Summary (Interim Data) (n=12)

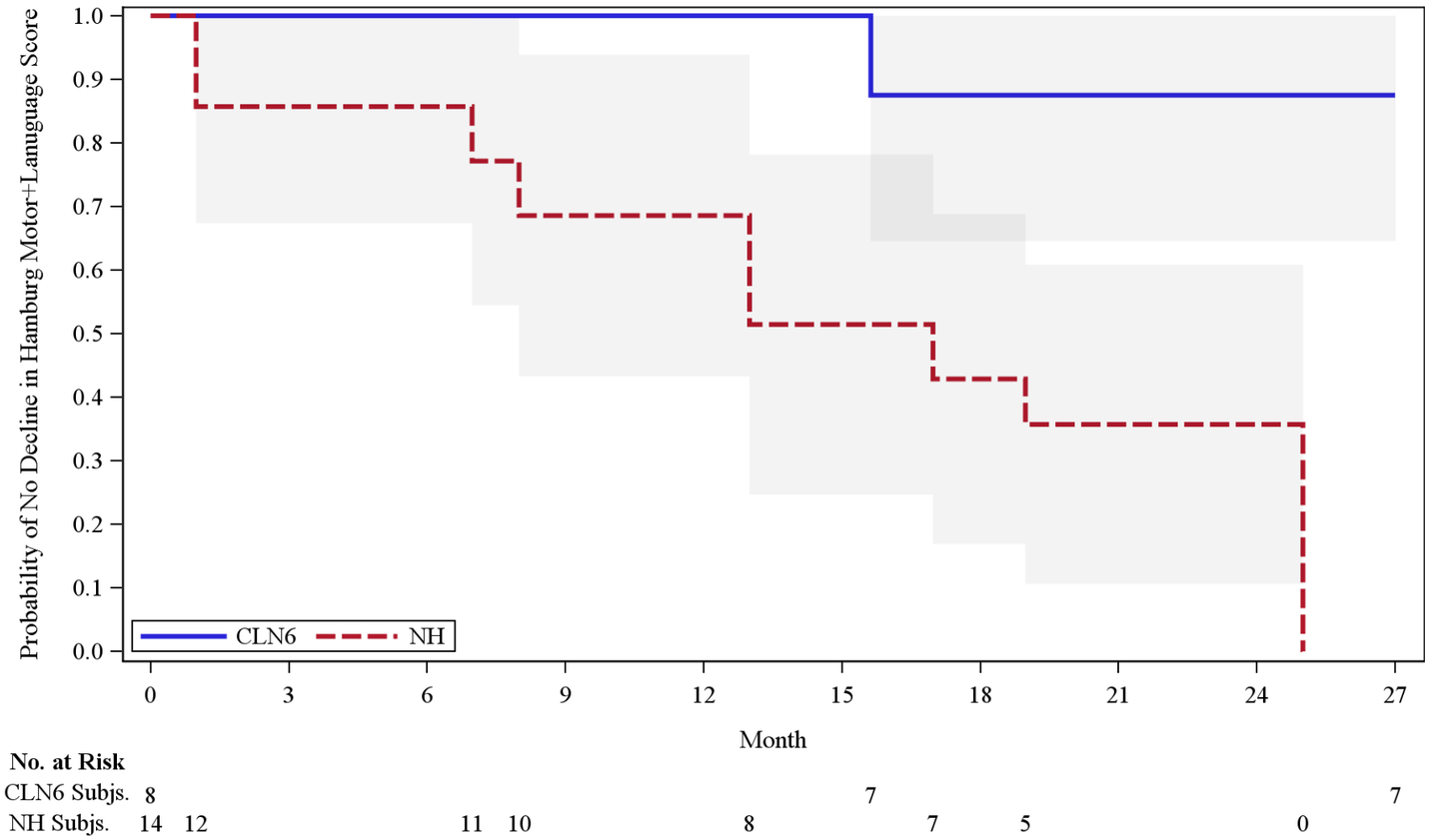
Data from an Ongoing 24 Month Single-arm Phase 1/2 Study Indicate Single Intrathecal AAV-CLN6 Administration is Generally Well Tolerated

- 12 patients with duration since gene transfer of 6 to 39 months
- Adverse events (n=130 events reported)
  - Majority of adverse events (AEs) were mild and unrelated to treatment
  - Nine Grade 3 (severe) AEs (all considered SAEs) reported in 4 patients
  - Three of 9 SAEs considered possibly related to treatment;
    - Related events included vomiting (2) and epigastric pain (1)
    - Recovery in all 3 cases
  - No Grade 4 (life-threatening) or Grade 5 (death) AEs reported
- No pattern of adverse events related to AAV9 or CLN6 immunogenicity

*Calculated from June 2019

Time to Unreversed 2-Point Decline in Hamburg Motor and Language
(Comparison to Interim Natural History Data - Unmatched)

Time to 2-Point Decline Analysis Suggests Clear Effect of Treatment and Separation of Treated Patients Compared to Natural History; All 14 Natural History Subjects had at Least a 2 Point Decline



Treated Patients (N=8)

CLN6 Natural History Patients (N=14)

Confidence bands are calculated using the survival probability estimates and its standard error

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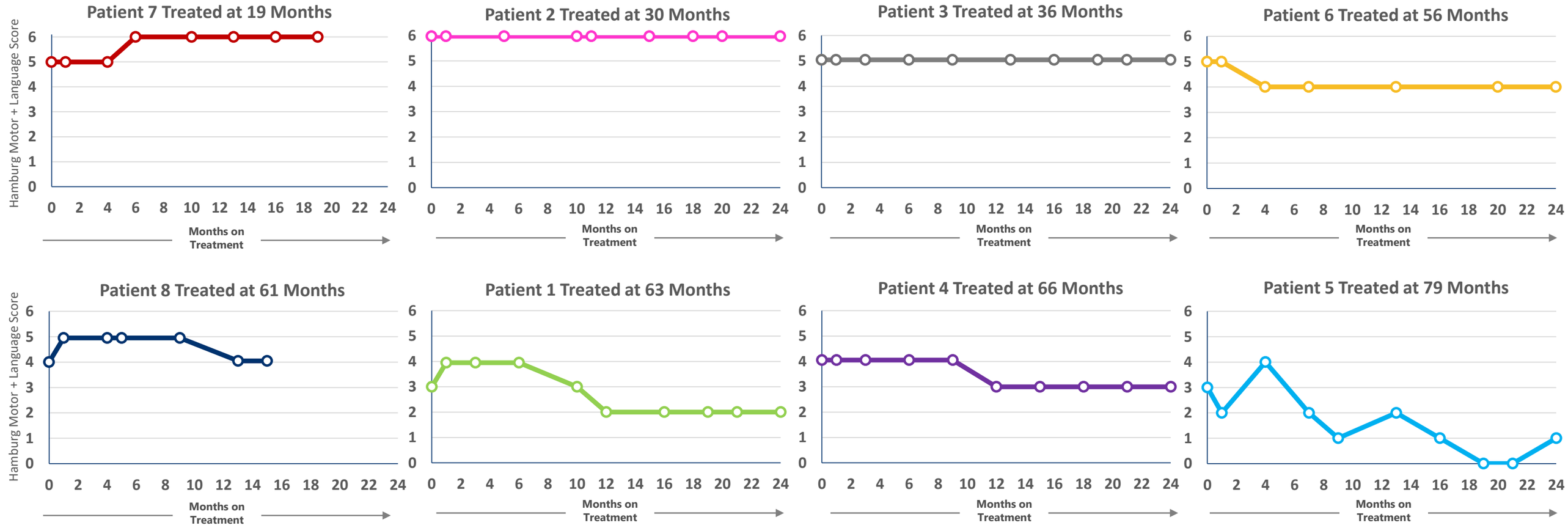
Clinical Efficacy: Hamburg M+L Score (n=8)

Efficacy Data Show a Positive Impact on Motor and Language Function

7 of 8 Patients Maintained Hamburg Score or had an Initial Change (+1 to -1 Points) Followed by Stabilization

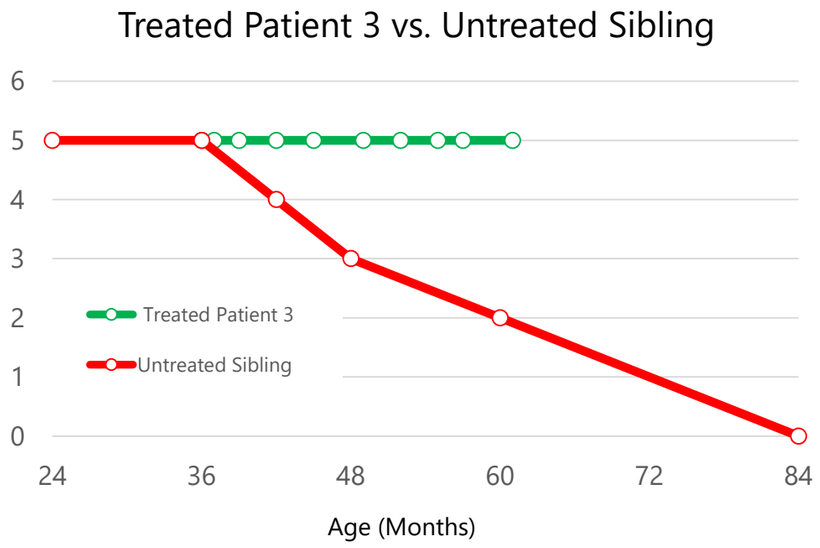


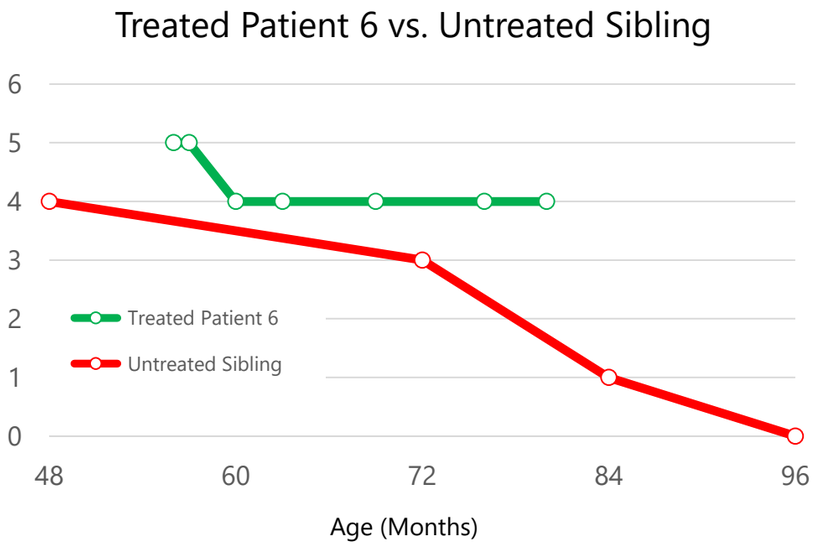
Natural history data suggest a 2-3 point decline in Hamburg Motor and Language over 24 months post symptom onset

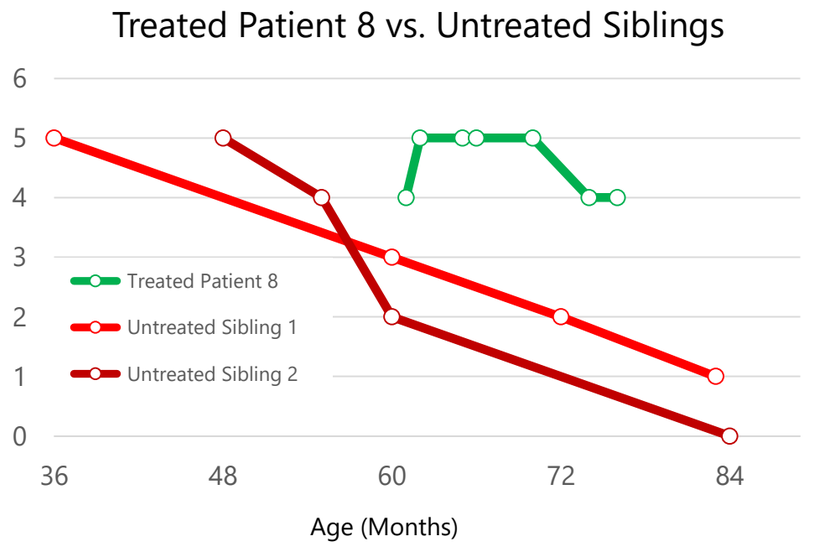
CLN6 Clinical Efficacy Data: Sibling Comparisons (Natural History)

Treated Patients Demonstrated Stabilization Relative to Untreated Siblings in the Natural History Data Set Who Experienced Substantial Declines in Motor and Language Ability

Treated AAV-CLN6 Patients vs Natural History Sibling with CLN6 (Hamburg Score: Motor + Language over time)



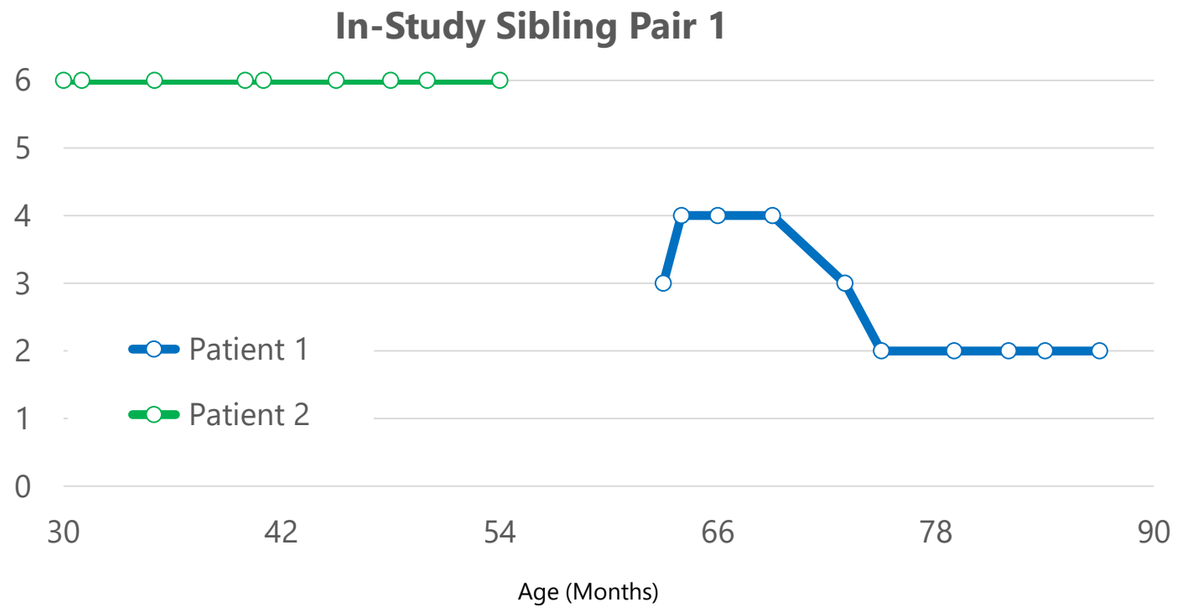


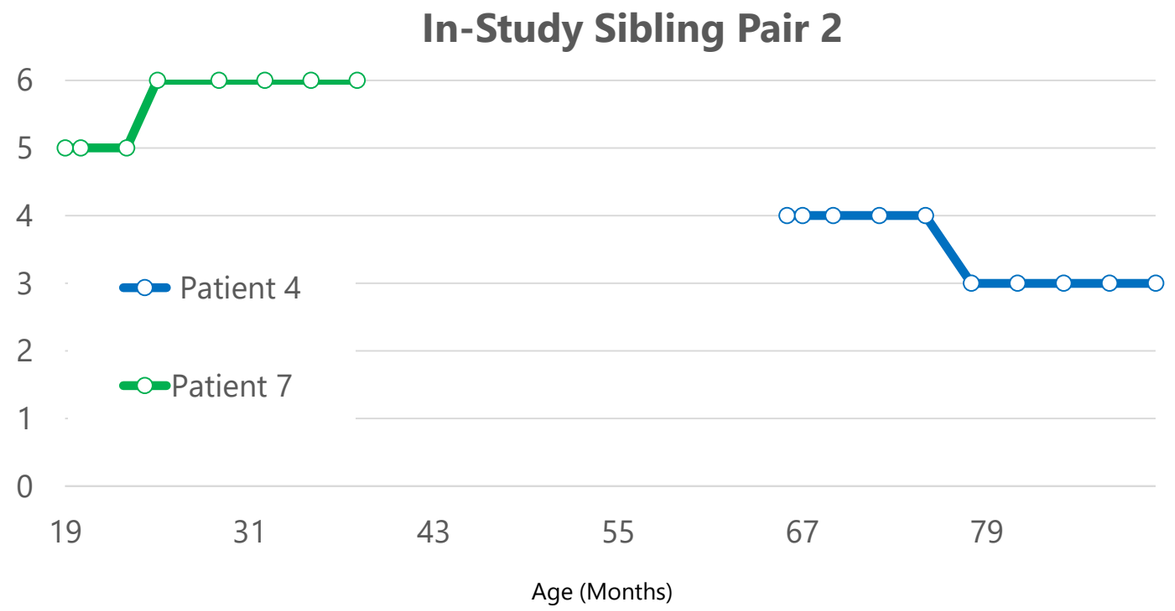


CLN6 Clinical Efficacy Data: Sibling Comparisons (In-Study)

For In-study Pairs, Younger Siblings Demonstrated Increase or No Change in Score Compared to Older Siblings Who had an Initial Change Followed by Stabilization

Treated In-Study Sibling Comparison (Hamburg Score: Motor + Language over time)





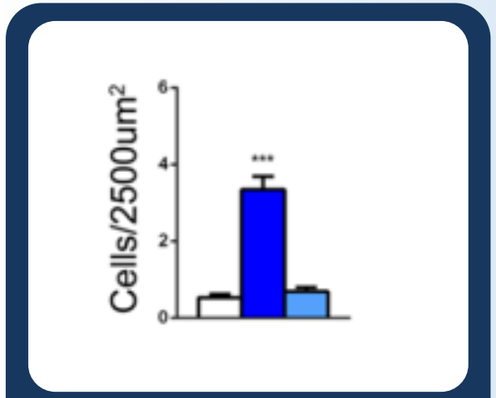
CLN6: Clinical Data Summary

Interim Safety and Efficacy Data Suggest that AAV-CLN6 Gene Therapy has the Potential to Stabilize Progression of a Devastating Disease that Destroys Brain Function and Kills Children

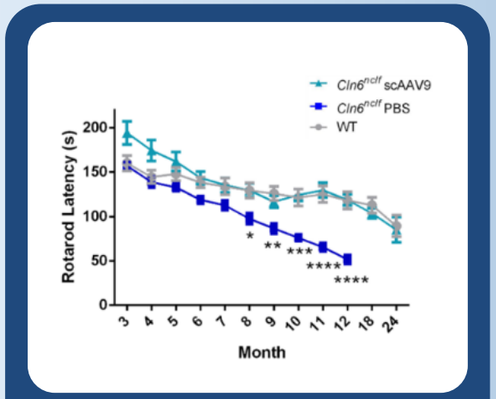
- 12 patients received AAV-CLN6 to date
- Treatment was generally well-tolerated
- Interim efficacy data (n=8 pts)
  - Stabilization of disease, in contrast to untreated siblings who experienced rapid decline in their motor and language ability
  - Younger patients show increase in score or stabilization
  - Majority of older patients show initial change followed by stabilization

Broad Platform Potential

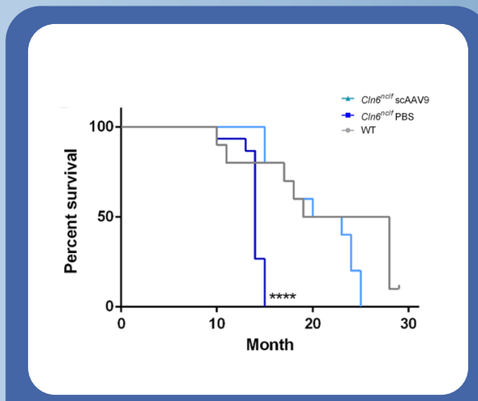
These Results Also Highlight the Translatability of Results from Mice to NHPs to Humans and Validate the Broad Potential of the Amicus Intrathecal AAV Platform Including CLN3 which is Now in the Clinic



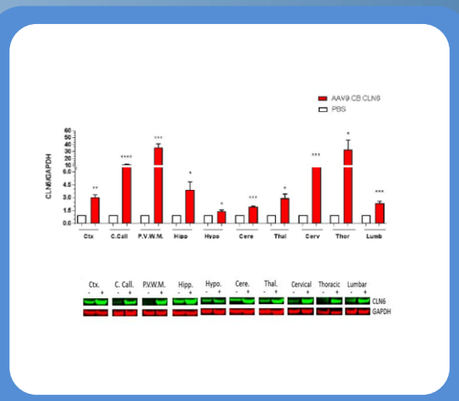
Prevention of storage material accumulation in mouse model



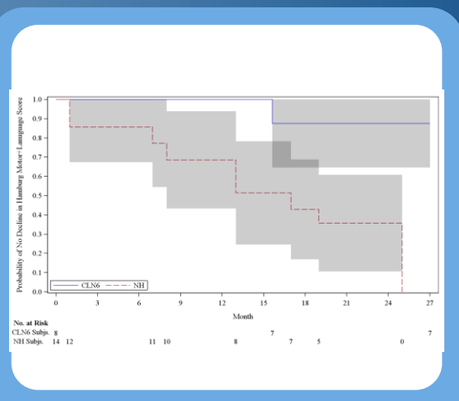
Improvement of motor function and cognitive behavior in mouse model



Improved survival in mouse model



Widespread gene expression in brain of NHPs



Initial efficacy data in humans

*Cain, *et al.*, **Mol Ther.** 2019 Jul 10 - https://www.ncbi.nlm.nih.gov/pubmed/31331814

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Phase 1/2 Data Summary

With these Initial Data we Look Forward to Advancing our Development, Manufacturing and Regulatory Strategy for our Batten Disease Programs

Next Steps

- Presentation of additional data in the eight initial patients at the Amicus Analyst Day in 4Q19 and in a poster at the Child Neurology Society Annual Meeting, October 23-26, 2019 (Charlotte, NC).
- Collection and presentation of additional natural history data in CLN6 Batten disease
- Dosing of additional patients
- Advance regulatory discussions
- Manufacturing of additional AAV-CLN6 gene therapy underway at Thermo Fisher (Brammer Bio)
- Continued advancement of AAV gene therapy programs in CLN3, CLN8 and CLN1 Batten disease.

Thank You

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