Aspa Therapeutics' BBP-812 Gene Therapy Program for Canavan Disease: Updates

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23Apr2025

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Canavan Research Illinois

Canavan Foundation
for the Prevention & Cure of Canavan Disease

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Natural History Study (CVN-101)

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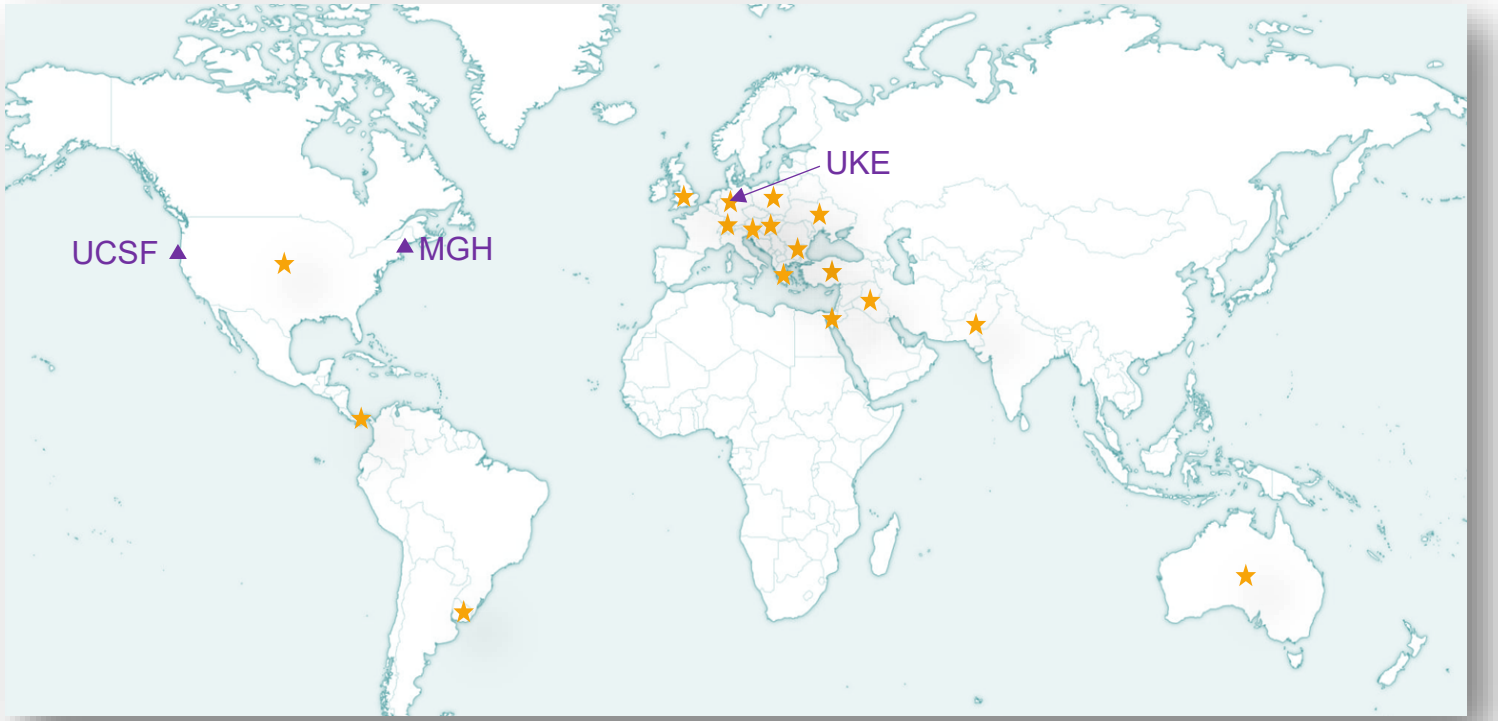
Natural History Study

Study Updates

- 67 participants enrolled from 17 countries
- Current sites: Mass. General Hospital (MGH), Boston, MA; University Clinic (UKE), Hamburg, Germany; University of California, San Francisco (UCSF) Oakland, CA



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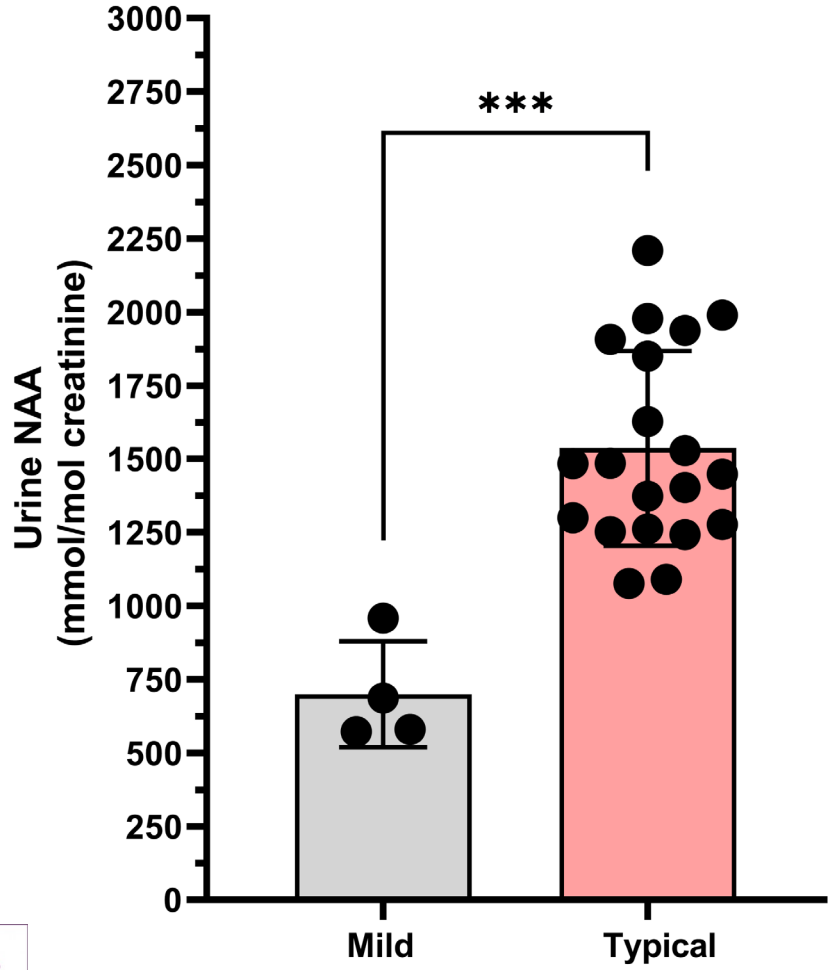
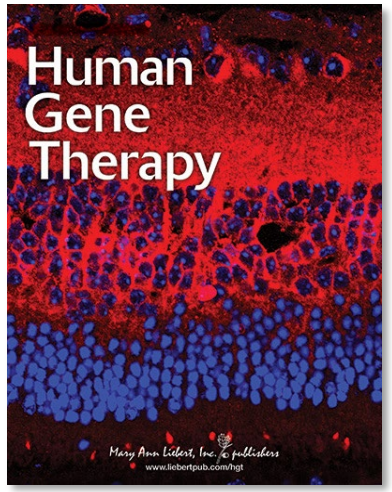
Aspa Published Data from Natural History Study





Urine *N*-Acetylaspartate Distinguishes Phenotypes in Canavan Disease

Amanda Nagy,[1] Florian Eichler,[1] Annette Bley,[2] Janna Bredow,[2] Alexander Fay,[3] Elise L. Townsend,[4] Beth Leiro,[5] Adam Shaywitz,[5] Genevieve Laforet,[5] Danielle Crippen-Harmon,[5] and Rachel Williams[5,*]

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Mild phenotype is defined as achieved the CDC milestone “pulls to stand/walks holding on”; Cr, creatinine; NAA, N-acetylaspartic acid.
Nagy A, et al, Urine N-Acetylaspartate Distinguishes Phenotypes in Canavan Disease. Hum Gene Ther. 2024, PMID: 39628365.

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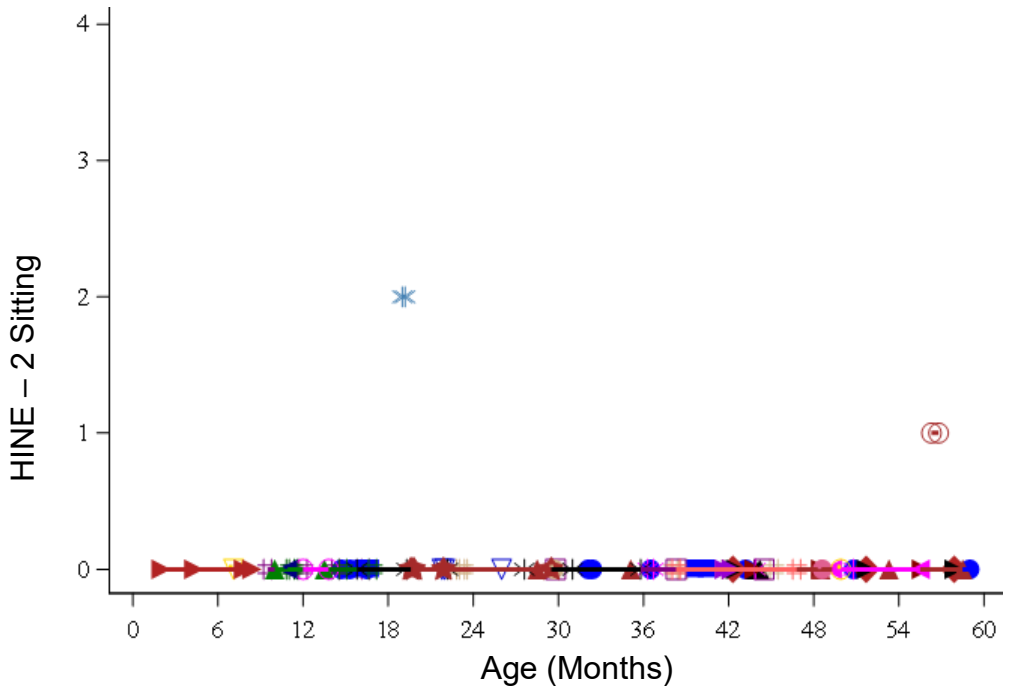
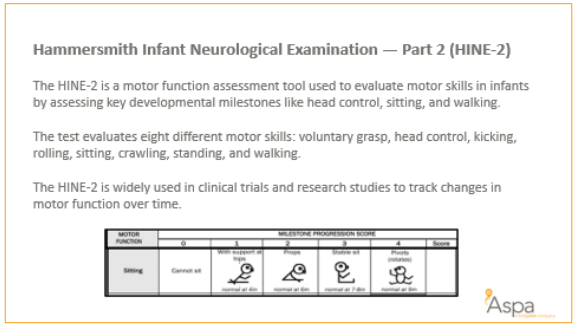
Key Learnings



- Thank you!
- Natural history data show little to no gains in motor function in individuals with Canavan between the age of 0-60 months
- This highlights the difficulty in gaining and maintaining skills in this population.



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Gene Therapy Clinical Trial (CVN-102)

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Important Disclaimer

The following slides discuss Aspa's investigational gene therapy BBP-812 which has not been approved by the FDA or other regulatory authorities.

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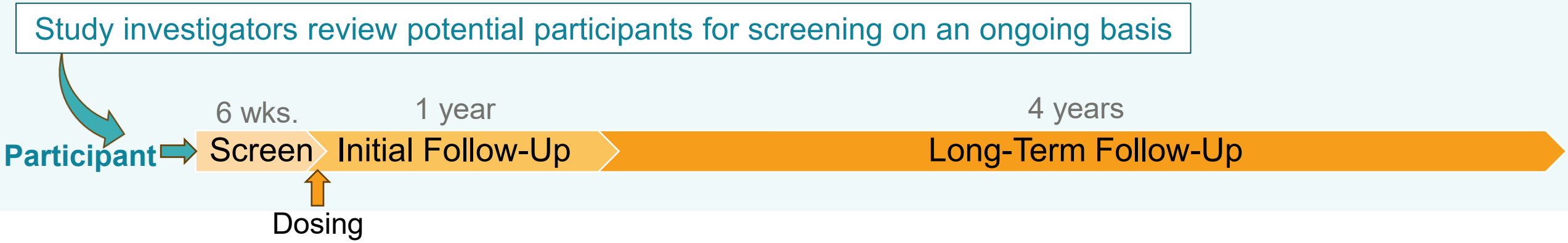
Individual Experience

Study investigators review potential participants for screening on an ongoing basis



Study Overview

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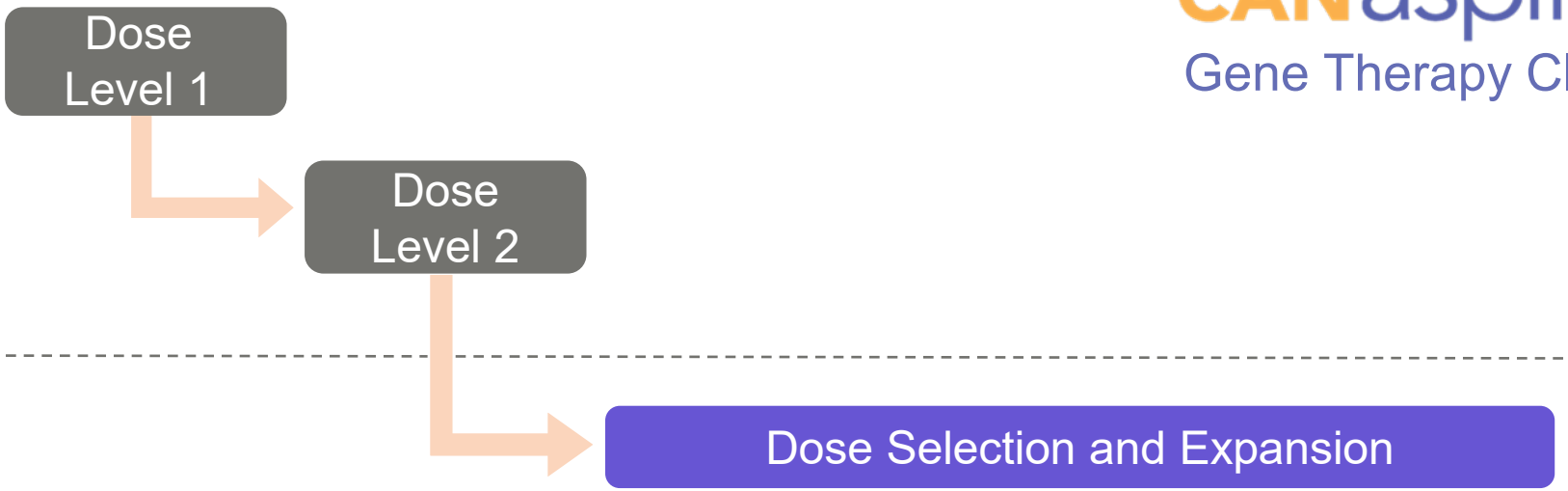
Gene Therapy Clinical Trial



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Gene Therapy Clinical Trial

Updates – Enrollment and Safety

Dosed Participants		
8 Participants at ***Low Dose***	**6** Participants at ***High Dose***	**14** Total Participants

Safety
Intravenous (IV) infusions were well-tolerated with no infusion reactions
All participants experienced at least 1 adverse event (AE); most were mild or moderate in severity and considered unlikely or not related to BBP-812
18 serious adverse events (SAEs) have been reported in 9 participants; 1 SAE was considered possibly related and 2 SAEs were related to BBP-812
To date, the safety profile of BBP-812 has been generally consistent with other AAV9 gene therapies given IV

As of December 2024

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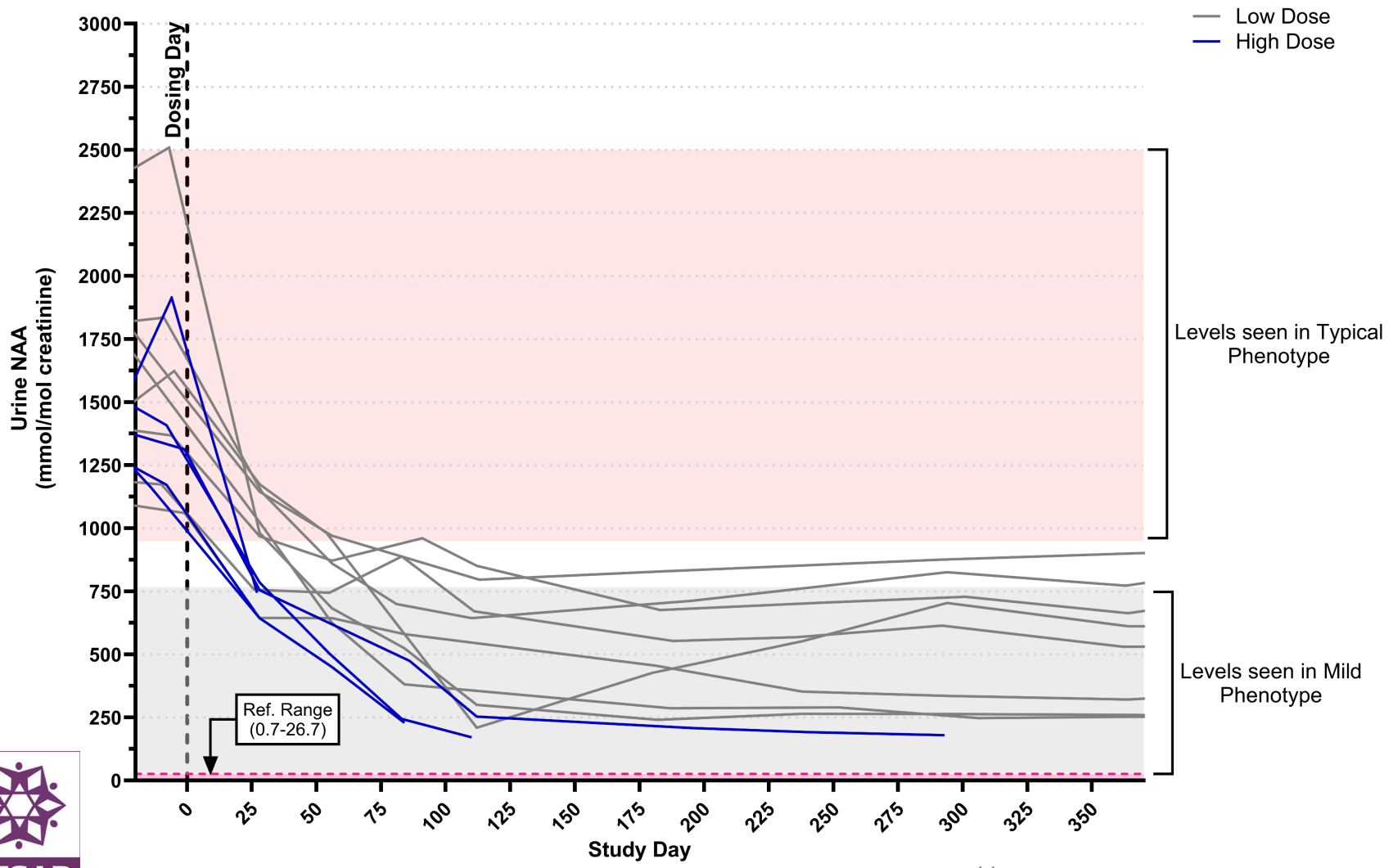
Emerging dose-dependent reductions in urine NAA



- Post-dose urine NAA levels drop to range seen in milder clinical presentation (phenotypes)
- High dose urine NAA levels are beginning to separate from low dose levels.

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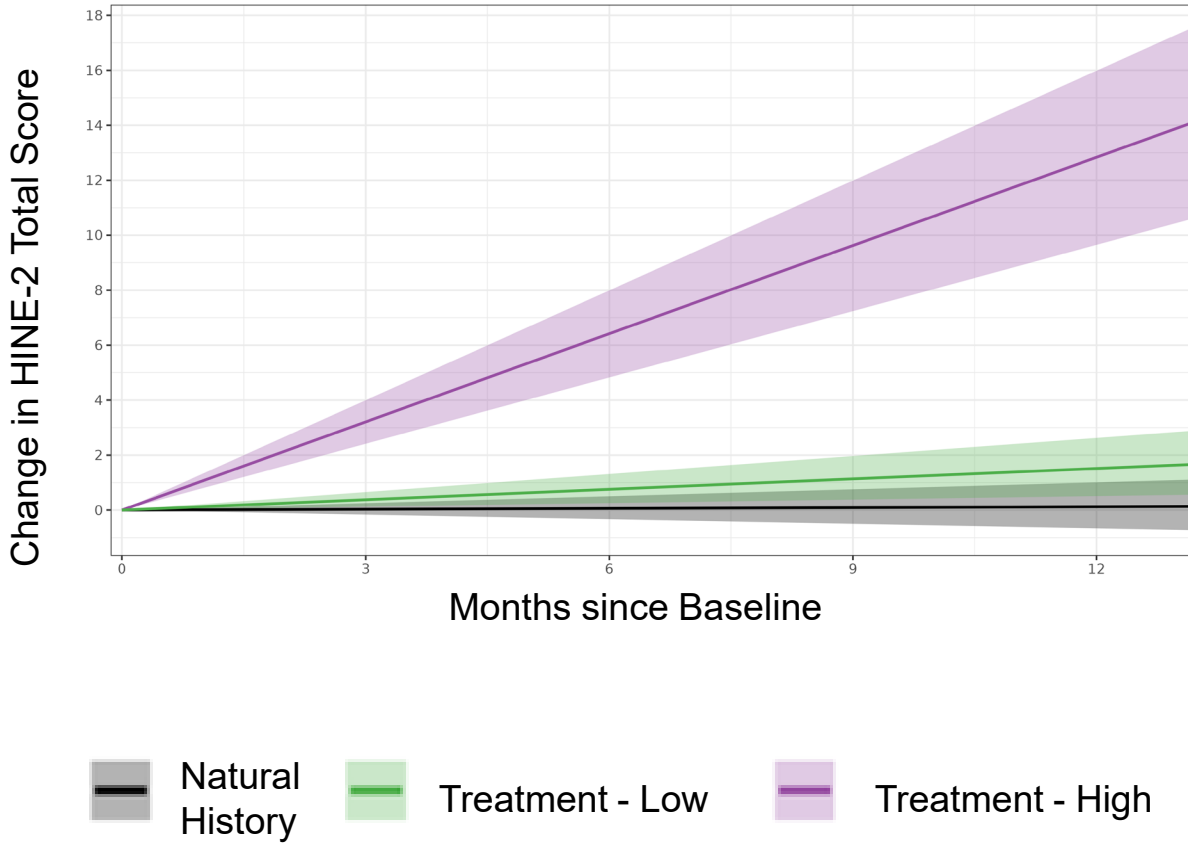
Preliminary Data Show That Children Dosed With BBP-812 Have Improved Motor Function Over Time Compared to Natural History (HINE-2)



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More Understanding to Come

- While these early results are encouraging, more participants will need to receive the investigational gene therapy and longer duration data are needed in order to fully understand the safety and efficacy of BBP-812.
- The results shown may not be typical for all participants.
- We will continue to explore the correlation between reduction in NAA and clinical improvement, an update the community as the trial progresses.

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Thank you!

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clinicaltrials.gov/study/NCT04126005

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clinicaltrials.gov/study/NCT04998396

Aspa Clinical Studies

treatcanavan.com

Human Gene Therapy

Urine NAA Publication

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2024 Presentation
MAT-US--162

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