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

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Canavan Research Illinois

Canavan Foundation
for the Prevention & Cure of Canavan Disease

Alex
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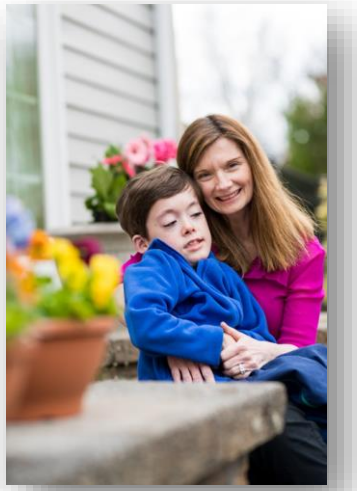
The Brain Charity



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canavan

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Natural History Study

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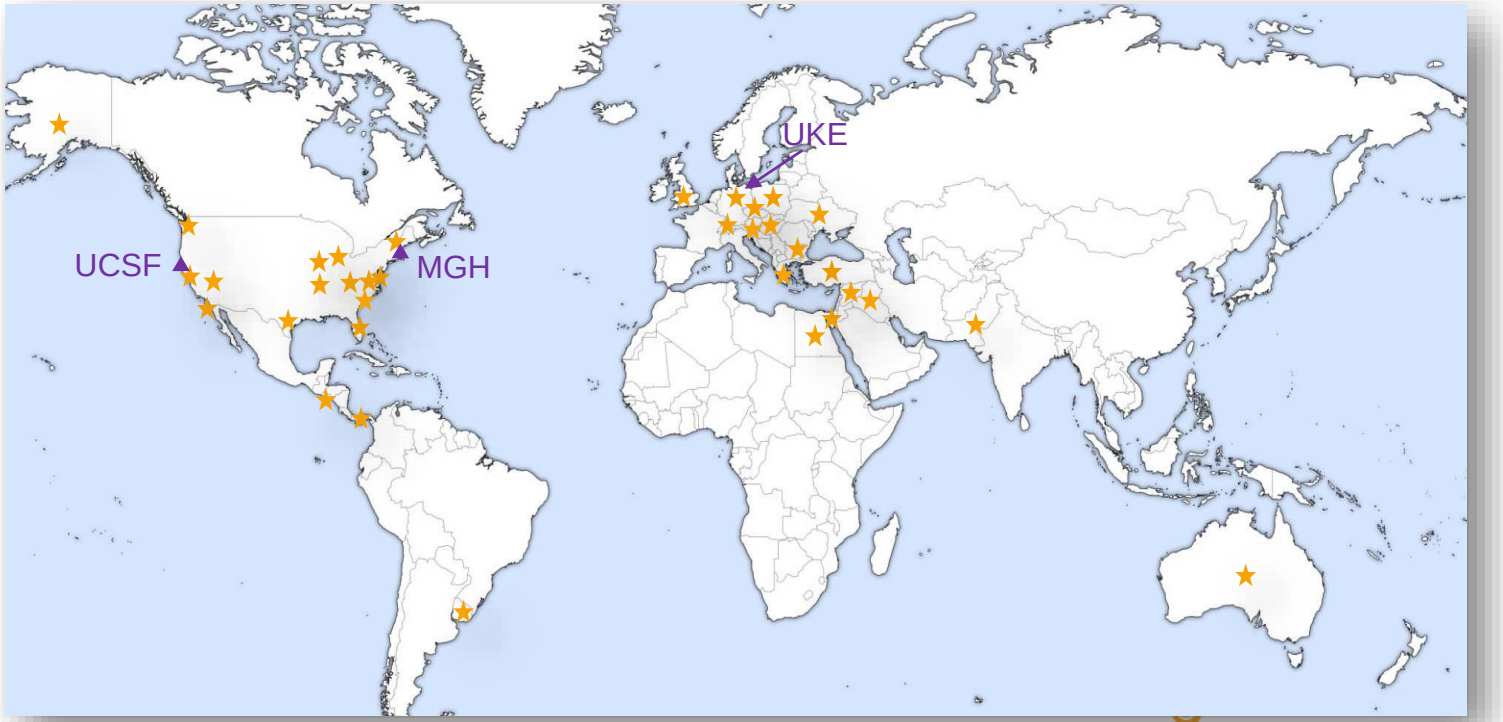
Study Updates

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

Age at Enrollment	Number of Participants
0-18 months	17
19-36 months	11
37-60 months	9
More than 60 months	22
Deceased	5



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Important Disclaimer

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

These data are insufficient to determine whether reductions in NAA levels will be associated with clinical benefit to patients. Early data from certain participants in the open-label trial are not necessarily indicative of future or final data in the clinical trial.

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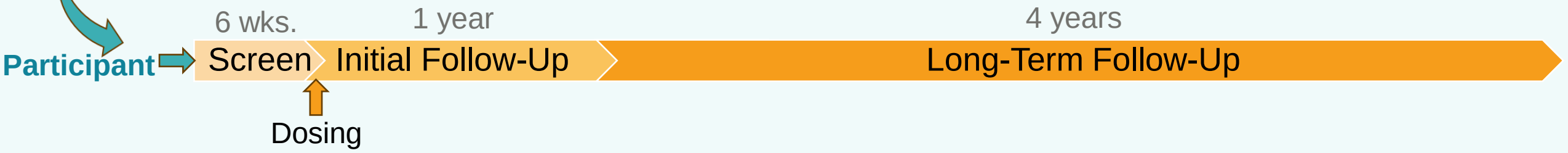
Study Overview and Status

Study investigators review potential participants for screening on an ongoing basis



Dose Finding Phase: Staggered enrollment and dosing

Dose Level 1

We are here

Dose Level 2

Required waiting period between each participant for formal safety review

Expansion Phase: Anticipated faster pace of enrollment and dosing

Expected first quarter 2025

Dose Selection and Expansion

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CANaspire Updates – Enrollment and Safety

Enrollment		
8 Participants Enrolled at ***Low Dose***	**1** Participant Enrolled at ***High Dose***	**9** Total Participants Enrolled

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
As of 29 Mar 2024, 12 serious adverse events (SAEs) have been reported in 6 participants; 1 SAE was considered possibly related to BBP-812
To date, the safety profile of BBP-812 has been consistent with other AAV9 gene therapies given IV

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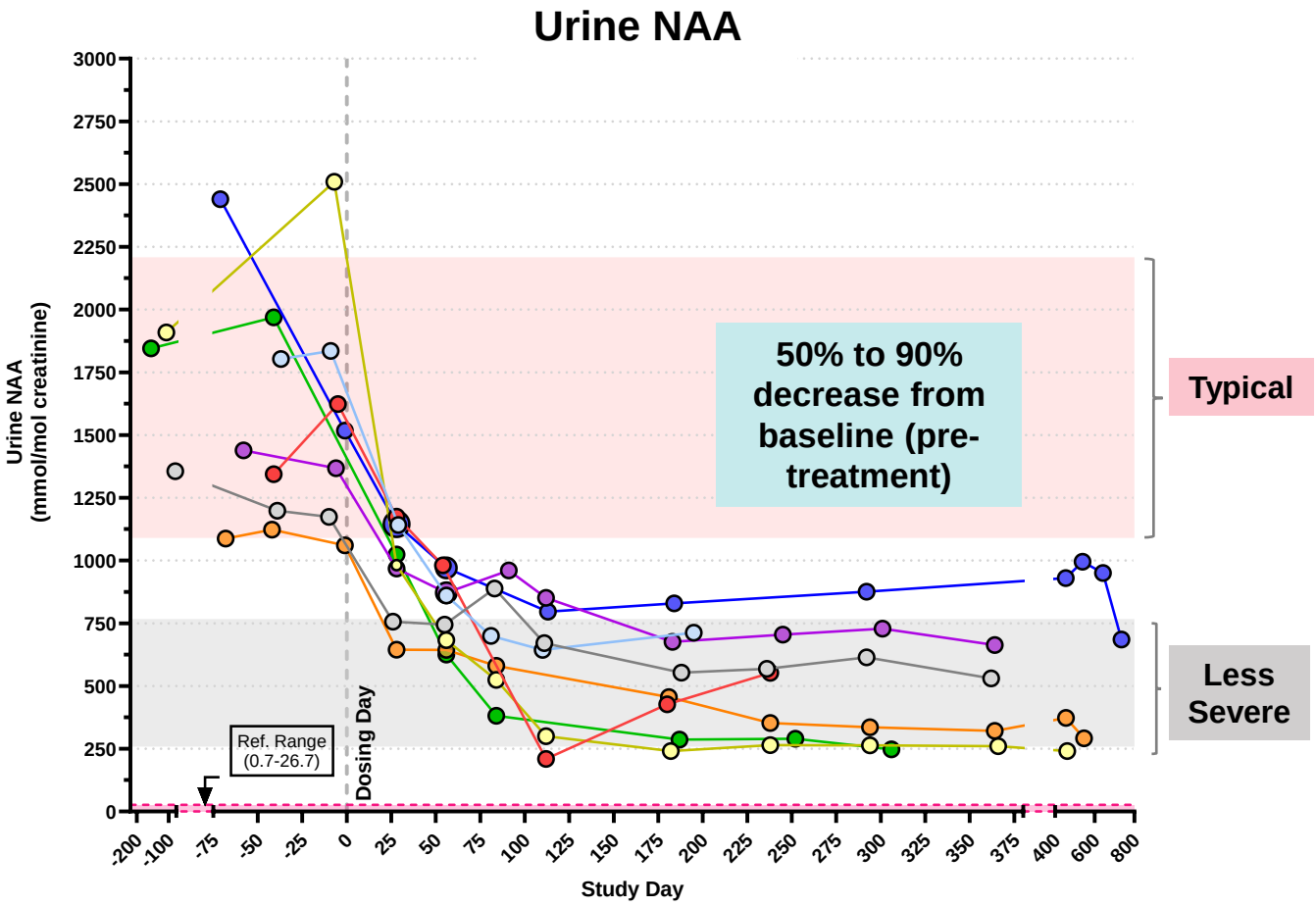
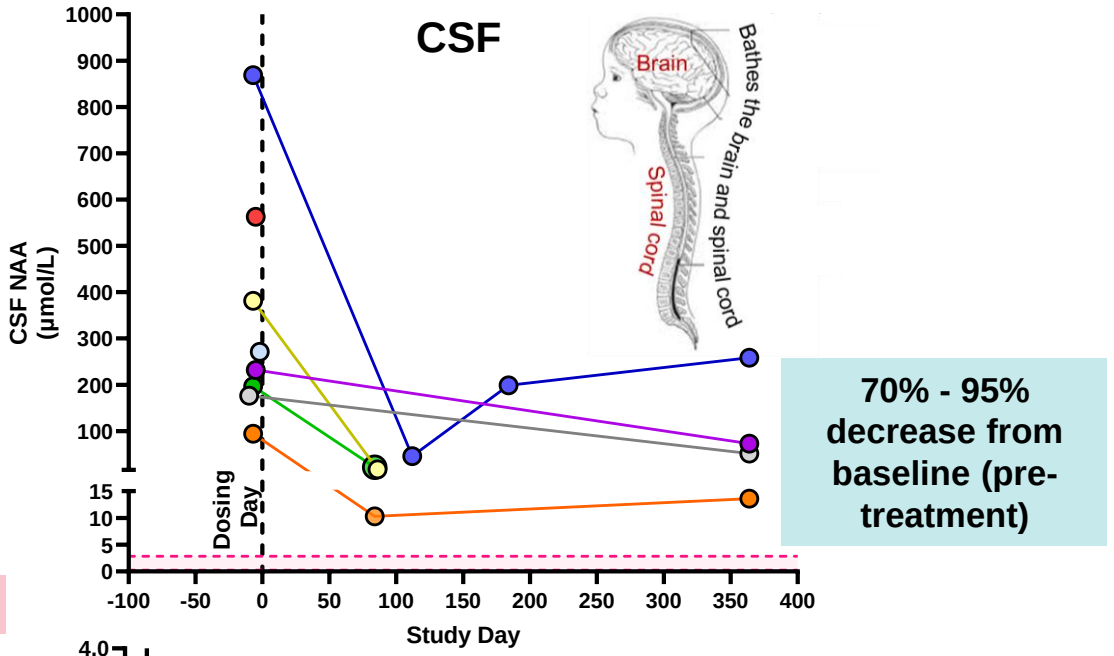
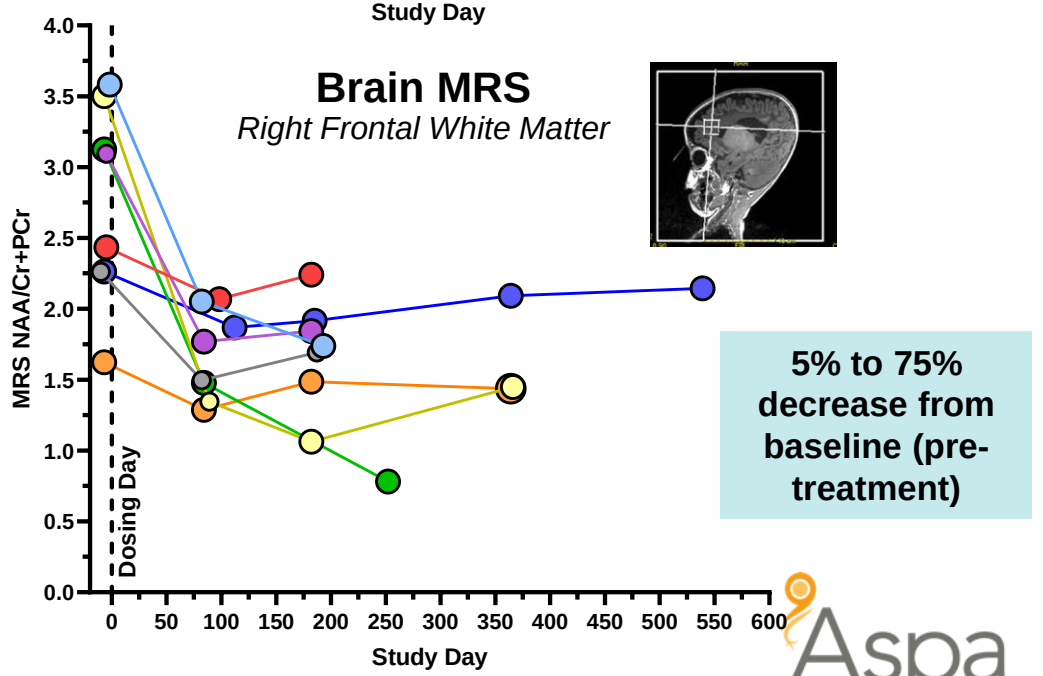
NAA Levels of Participants after Dosing With BBP-812







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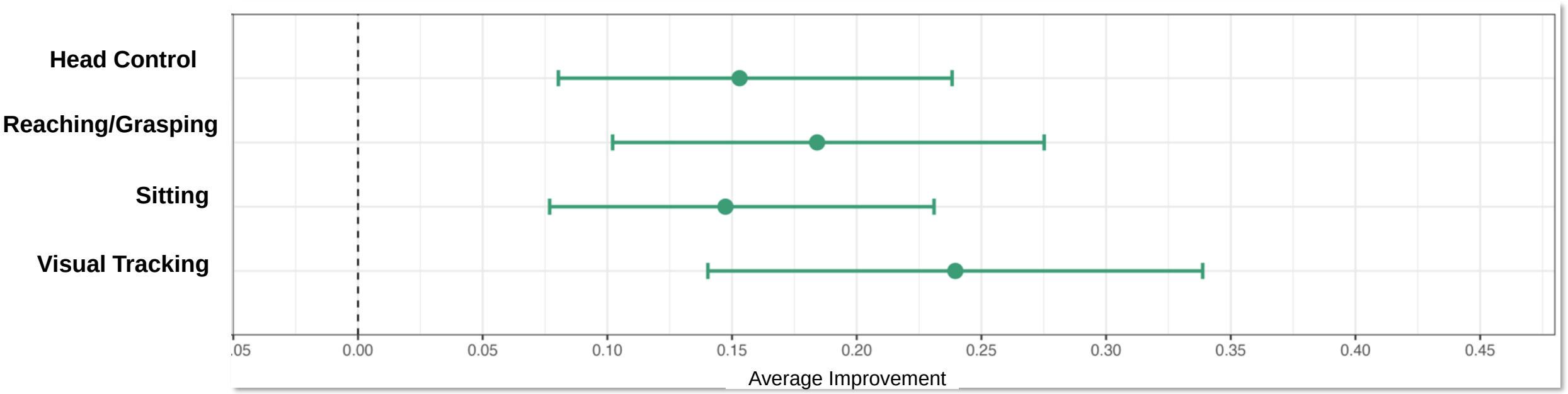
Proportion of 8 Participants Improving in 4 Key Abilities After Low-dose BBP-812 Compared to Natural History



Domain Items:
Head Control = HINE-2 (Goal = non-zero score)
Reaching/Grasping = HINE-2 (Goal = non-zero score)
Sitting = GMFM-88 Item #23 (Goal = non-zero score)
Visual Tracking = CDC Milestone (Goal = Yes)

Other Individual-Participant Changes (not representative of all participants):
- Supported and unsupported standing
- Walking (with and without a mobility aid)
- Counting and communication

*The physical effects and functional impact of this investigational gene therapy remain unknown.

More Understanding to Come

- Although all nine participants had rapid and sustained decreases in NAA after dosing, levels were still many times higher than normal

- Despite trending evidence that lower NAA levels are related to less severe Canavan disease, that does not necessarily mean that lowering NAA levels will make existing severe Canavan disease milder

- It is not clear whether restoring ASPA activity and lowering NAA levels will lead to clinical improvement in children with Canavan disease

Thank you!

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clinicaltrials.gov/study/NCT04126005

CANaspire

clinicaltrials.gov/study/NCT04998396

Aspa Clinical Studies

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2023 Presentation

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