Updated Results of Transpher B, a Multicenter, Single-Dose, Phase 1/2 Clinical Trial of ABO-101 Gene Therapy for Sanfilippo Syndrome Type B (Mucopolysaccharidosis IIIB)

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Study Sponsor Abeona Therapeutics

Disclosures

I have nothing to disclose

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Sanfilippo Syndrome (MPS III)

A group of four clinically indistinguishable lysosomal enzyme deficiencies that result in accumulation of the glycosaminoglycan (GAG) heparan sulfate (HS)

- Global incidence varies by regions and it is estimated 0.17-2.35 per 100,000 births*
- MPS IIIB is the second subtype in frequency and it is caused by a deficiency in N-Acetyl-Alpha-Glucosaminidase (NAGLU)

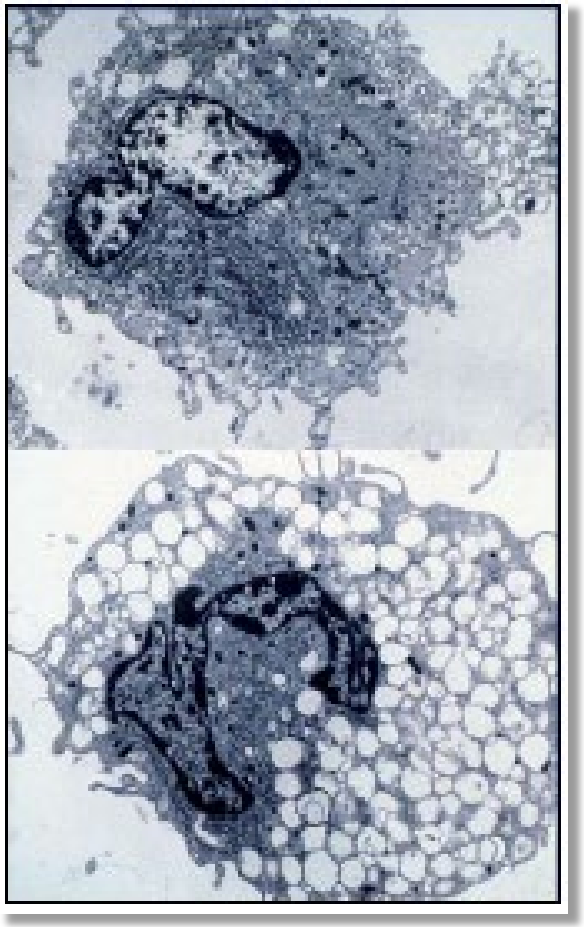
Disease manifest as early as 12-24 months involving:

- *Central nervous system features predominate (gray > white matter)*
  - Slowing and then regression of development, first speech/cognitive then gross motor
  - Impulsivity, hyperactivity, sleep disturbance, aggressive behavior, seizures
  - Relentless loss of skills progressing to dementia
- *Somatic features are milder than other MPS disorders*
  - Coarse facial features/hirsutism, frequent otitis media, airway compromise, Umbilical hernia, hepatosplenomegaly, mild dysostosis multiplex/short stature, heart valve thickening

No approved treatments available

- 70% of children with MPS III do not reach age 18 years of age

Normal cell



Cell with
lysosome deficiency

* Zelei et al. 2018. Orphanet Journal of Rare Diseases

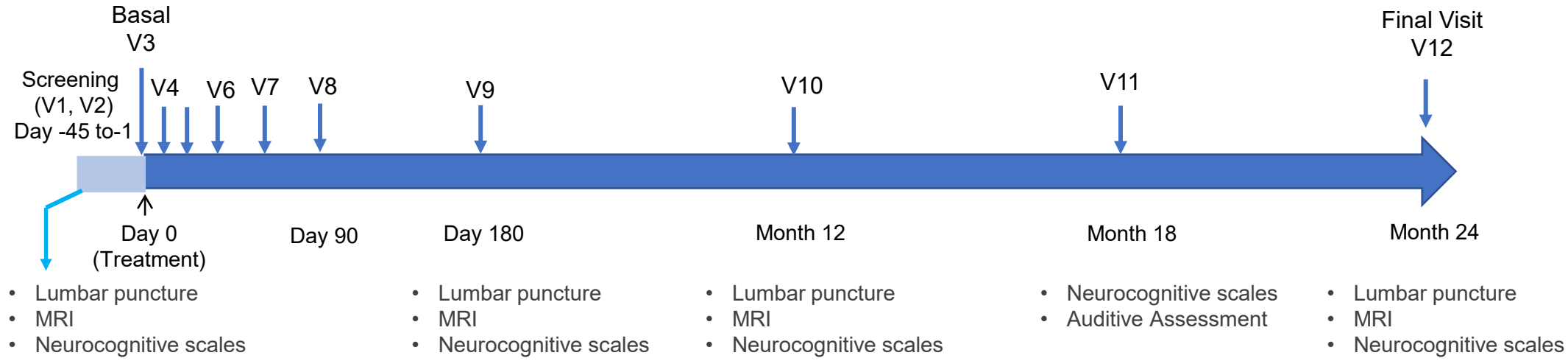
Transpher B Phase 1/2 Clinical Trial for MPS IIIB with rAAV9.CMV.hNAGLU

ABO-101	• Recombinant-AAV9 expressing a functional copy of NAGLU under the control of CMV promoter administered IV
Cohorts	• **Cohort 1**: 2 x 10^{13} vg/kg (n=2) • **Cohort 2**: 5 x 10^{13} vg/kg (n=5) • **Cohort 3**: 1 x 10^{14} vg/kg (n=4 up to 12)
Inclusion Criteria	• Birth - 2 yrs of age or older than 2 years with a Developmental Quotient (DQ) ≥ 60 (using the Bayley Scale) • Confirmed Diagnosis of MPS IIIB by genetic and enzymatic determinations
Primary Endpoint	• Age Equivalent Developmental score compared with Natural History Study data • Product safety
Secondary Endpoints	• Change from baseline in biomarkers after treatment • Change from baseline in Liver, spleen and brain volume by MRI • Neurocognitive function as measured by Mullen Scales of Early Learning or Bayley Scales of Infant and Toddler Development • Adaptive functioning, by Vineland Adaptive Behavior Scale (caregiver report) • Change from baseline in the Sanfilippo Behavior Rating Scale [Time Frame: Month 6, 12, 18, 24] • Change from baseline in Pediatric Quality of Life Inventory (PedsQL™) total score [Time Frame: Month 6, 12, 18, 24] • Change from baseline in parent quality of life, using the Parenting Stress Index, 4th Edition (PSI-4) short form [Time Frame: Month 12, 24]

For further detail: ClinicalTrials.gov: NCT03315182 **EudraCT Number:** EudraCT2014-001411-39

Clinical Trial Design and Schedule of Visits

Study Duration	24 months (followed by a long-term follow up study for additional 3 years)
Administration	Single intravenous administration in 15-45 minutes Hospital for 2 days Steroids for the first 2 months (1 mg/kg prednisone or prednisolone)
Comparator Group	Natural History Studies
Visit Schedule	Screening, basal, Days 7, 14, 30, 60, 90, 180, Months 12, 18 and 24



Enrollment Update

19 patients have been screened

- 4 patients have failed inclusion/exclusion criteria
- 11 patients have been treated (Cohort 1=2; Cohort 2=5; Cohort 3=4)
- 4 patients under screening

Two pairs of siblings have been enrolled and treated

- A 5.3 year old girl in Cohort 1 and her 4 months old sister in Cohort 2 (under a protocol waiver)
- A 3.7 year old male and his 1.75 year old sister in Cohort 2

Safety Update

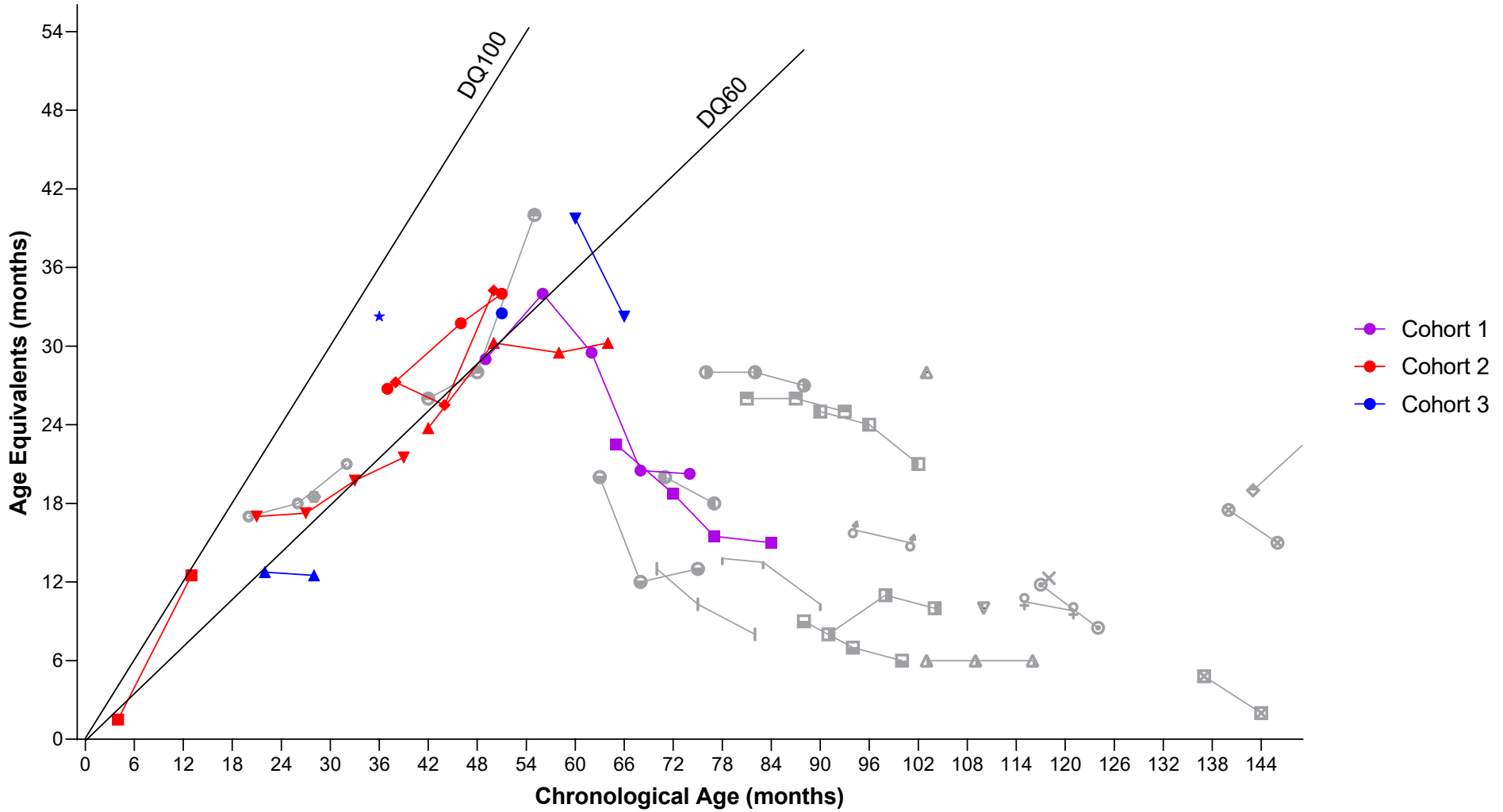
Mean follow up as of January 2021

- Cohort 1: 31 months
- Cohort 2: 17 months
- Cohort 3: 7 months

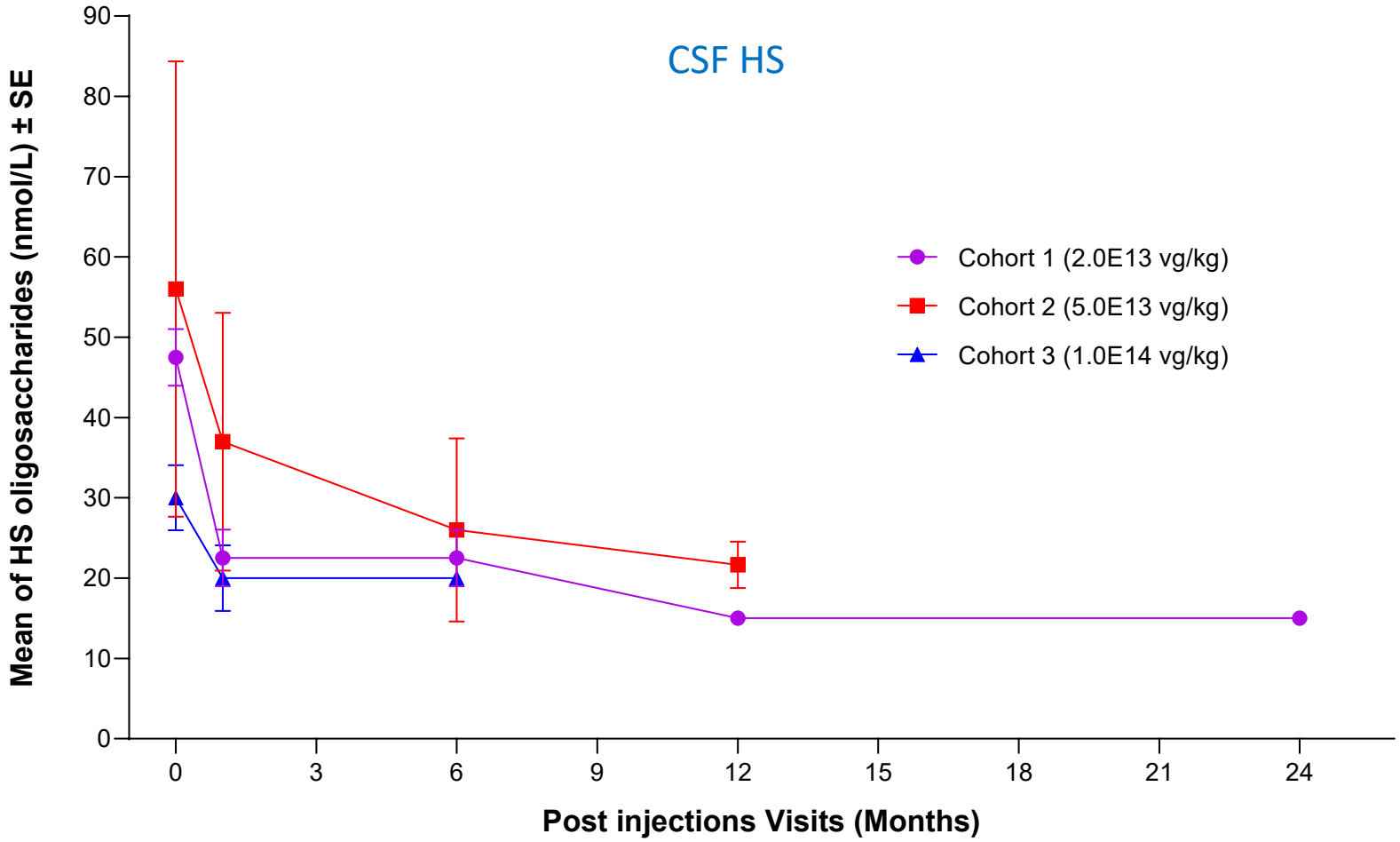
ABO-101 has been well tolerated

- No deaths
- No infusion-related adverse events
- One drug-related SAE: an episode of prolonged hospitalization after treatment for observation due to Grade 2 diarrhea and vomiting (from Day 2 to Day 5)
- Drug-related AEs include
  - Subclinical, transient ALT and AST elevations, without accompanying changes in bilirubin
  - Mild and transient decrease in WBC, absolute lymphocyte counts and platelets
  - Vomiting, diarrhea, fever, anorexia and/or asthenia have been observed in some children for 1 or 2 days soon after product administration. They are well tolerated and in general don't require treatment
  - ELISpot to AAV9 capsid peptide pools have been negative in all subjects at all timepoints, except in one subject in Cohort 1 and one in Cohort 2 (they were positive at Month 12 but negative again at Month 18)

Cognitive Age Equivalent Data

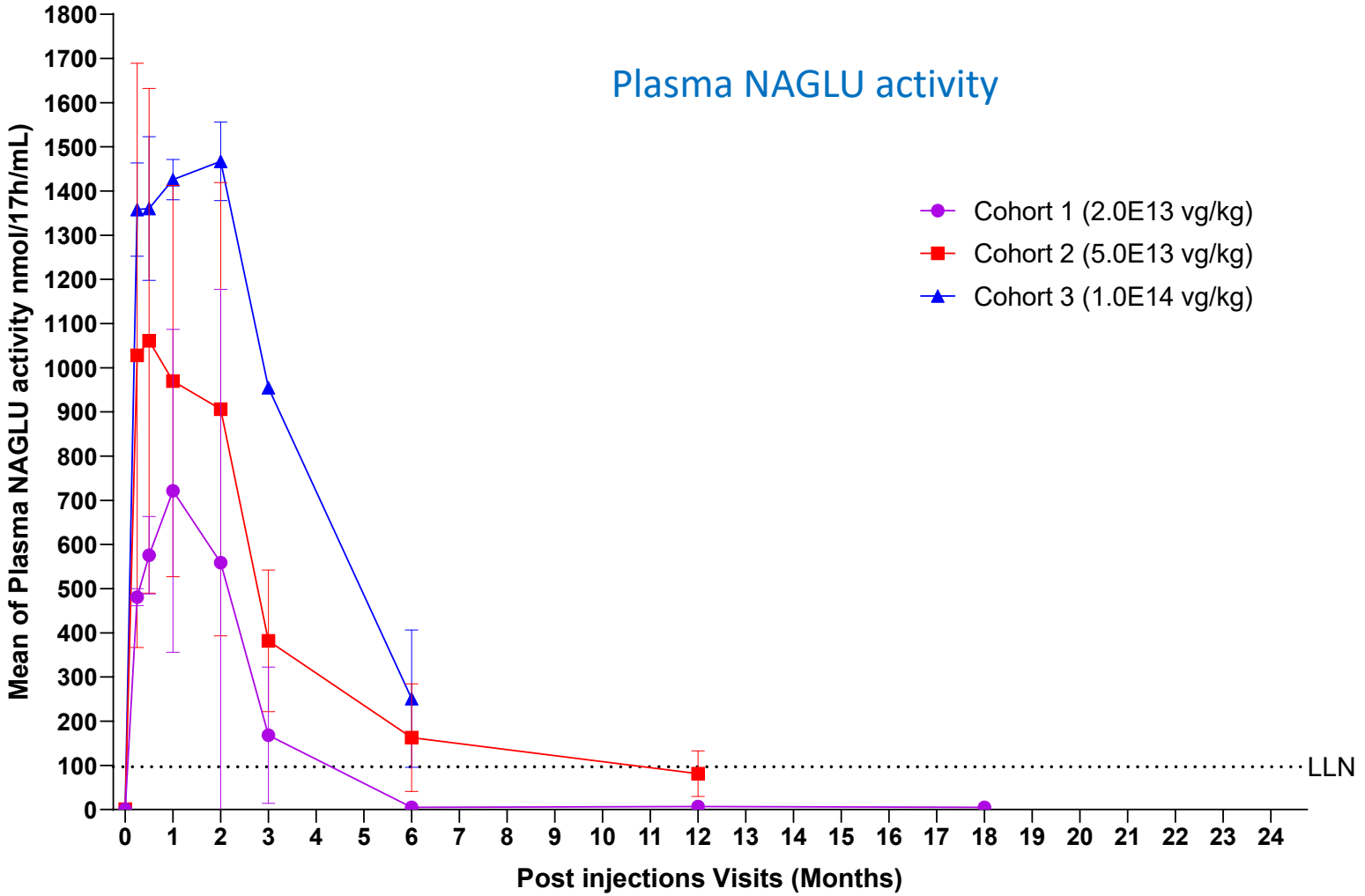


Rapid and Sustained Reduction in CSF Heparan Sulfate



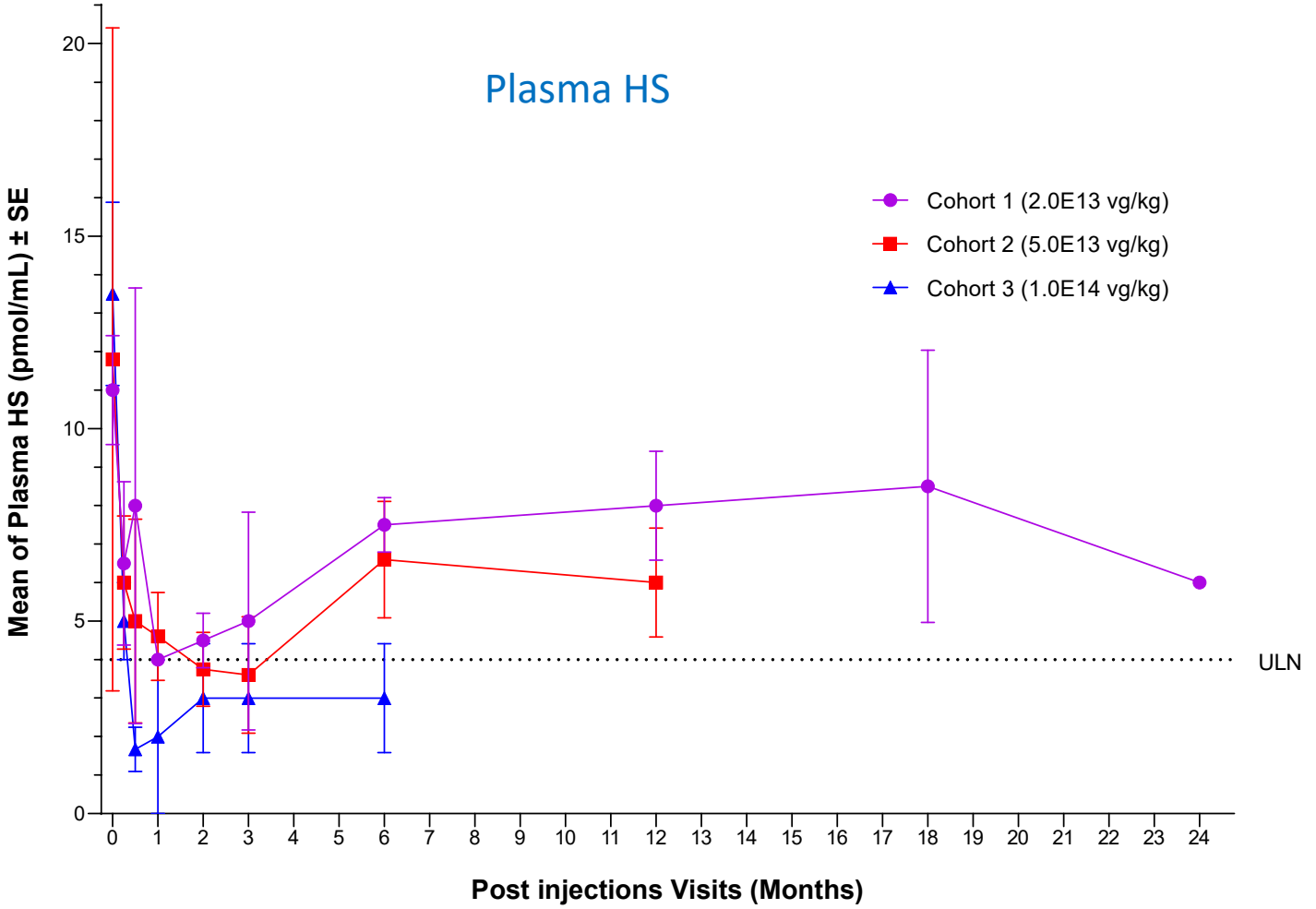
No Patients	Screening	Month 1	Month 6	Month 12	Month 24
Cohort 1	2	2	2	2	1
Cohort 2	5	5	5	3	
Cohort 3	4	4	2		

Dose-Dependent Increase in Plasma NAGLU Activity



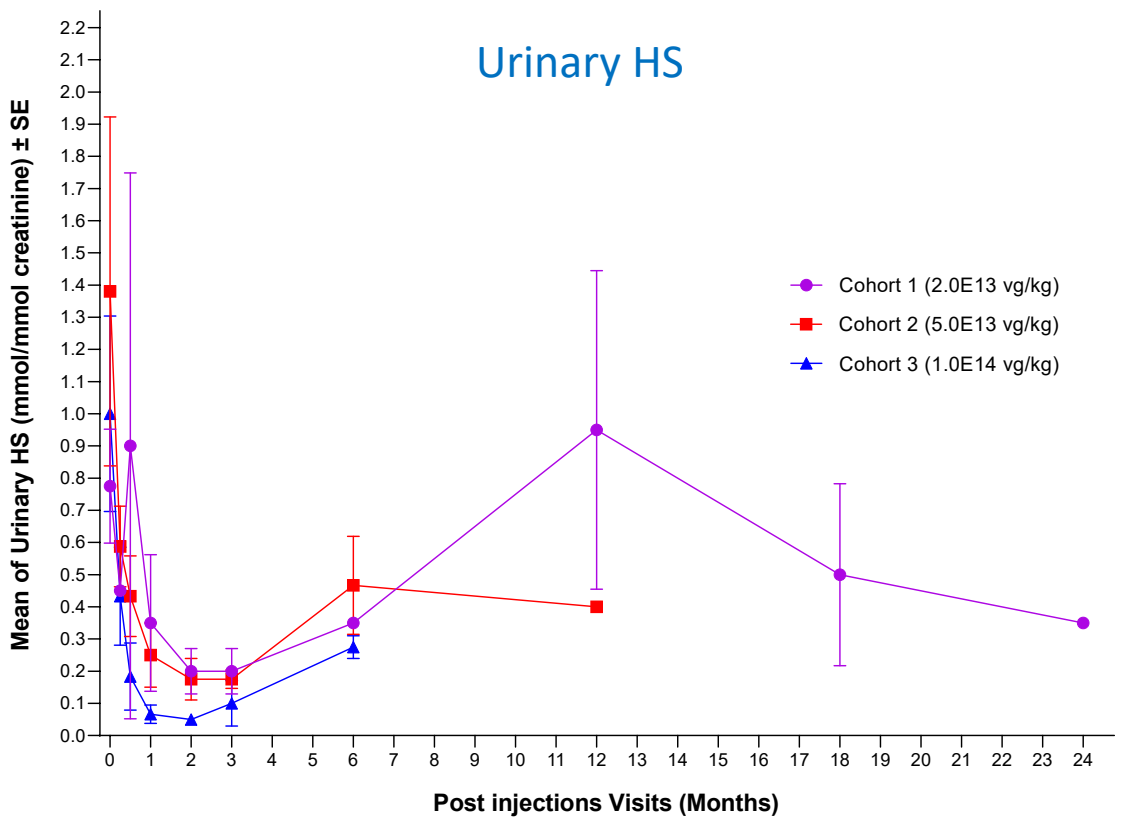
No Patients	Screening	Day 7	Day 14	Month 1	Month 2	Month 3	Month 6	Month 12	Month 18	Month 24
Cohort 1	2	2	2	2	2	2	2	2	2	
Cohort 2	5	3	3	5	5	5	4	4		
Cohort 3	4	4	4	4	3	1	2			

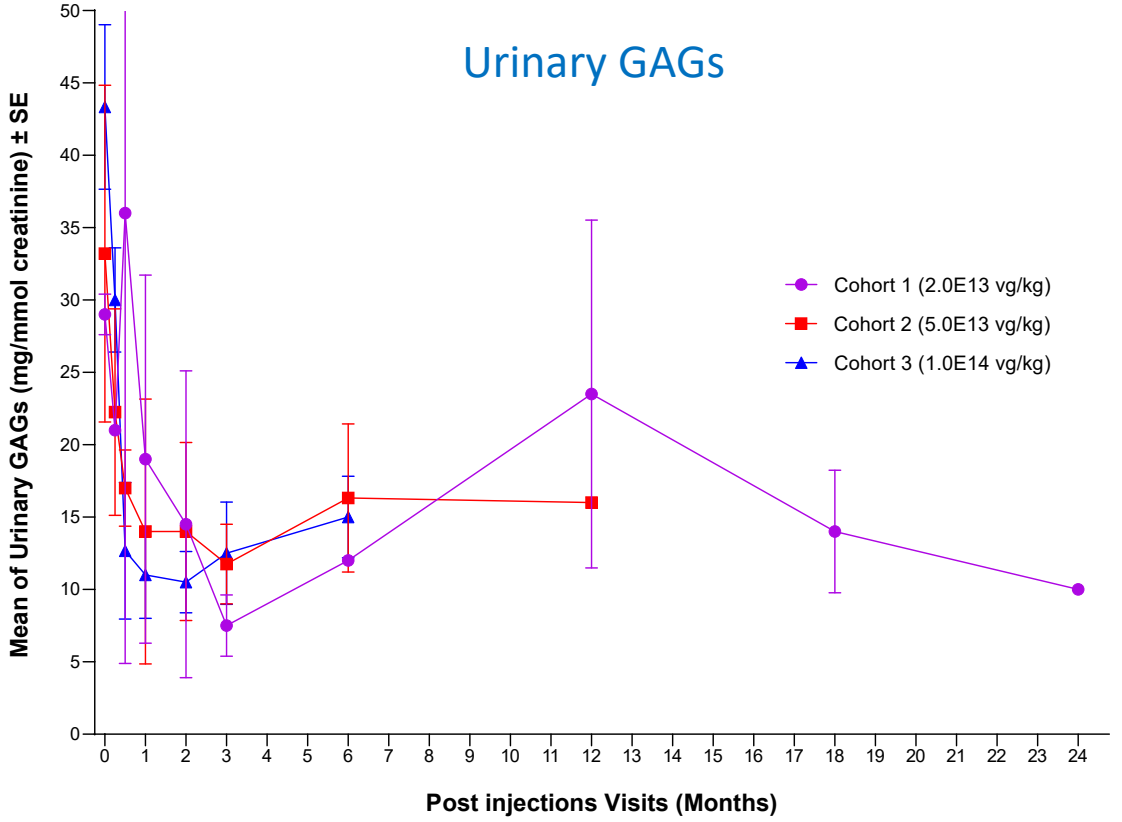
Dose-Dependent Reduction of Plasma Heparan Sulfate



No Patients	Screening	Day 7	Day 14	Month 1	Month 2	Month 3	Month 6	Month 12	Month 18	Month 24
Cohort 1	2	2	2	1	2	2	2	2	2	1
Cohort 2	5	3	3	5	4	5	5	2		
Cohort 3	4	3	3	4	2	2	2			

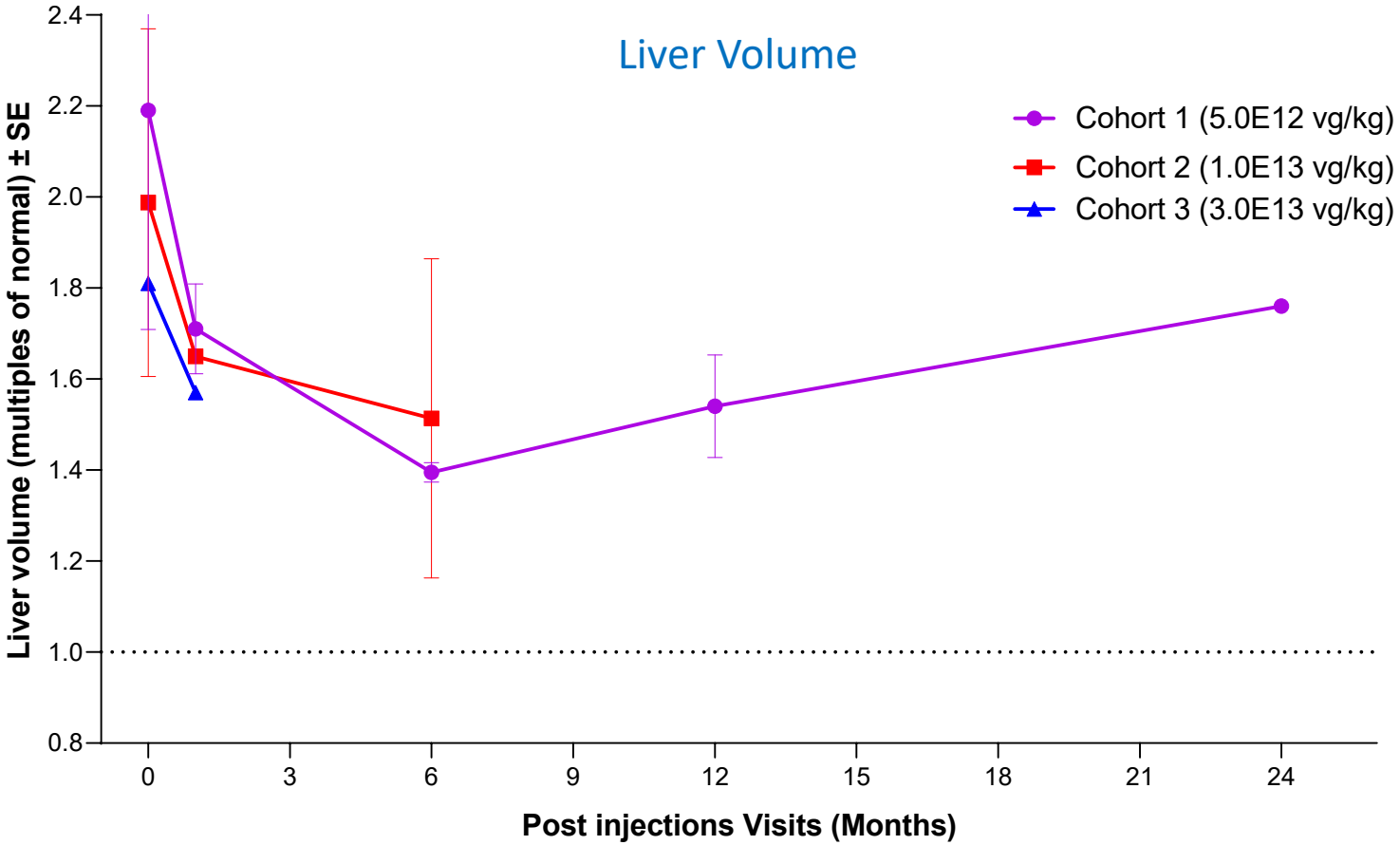
Dose-Dependent Reduction of Urinary GAGs and Heparan Sulfate





No Patients	Screening	Day 7	Day 14	Month 1	Month 2	Month 3	Month 6	Month 12	Month 18	Month 24
Cohort 1	2	1	2	2	2	2	1	2	2	1
Cohort 2	5	4	3	3	4	4	3	1		
Cohort 3	3	3	3	3	2	2	2			

Reduction of Liver Volume



No Patients	Screening	Month 1	Month 6	Month 12	Month 24
Cohort 1	2	2	2	1	1
Cohort 2	4	1	3		
Cohort 3	2	1			

Summary: Phase 1/2 Study Data (N=11) with ABO-101 (rAAV9.CMV.hNAGLU)

Well-tolerated with no infusion related or early acute reactions and no clinically significant AEs or laboratory abnormalities

- Follow-up: cohort 1 (n=2; 24 to 37 months); cohort 2 (n=5; 14 to 21 months); cohort 3 (n=1; 3 to 12 months)

Treatment with ABO-101 is associated with a dose dependent and sustained improvement in CNS and systemic biomarkers indicating a potent biologic effect post treatment

- Decreased CSF HS levels sustained up to 24 months
- Dose-dependent normalization of plasma NAGLU activity up to Month 6 in Cohort 3
- Dose-dependent reduction of plasma and urine Heparan Sulfate and Urinary GAGs
- Reduction in liver volume

Cognitive evaluation requires a longer follow-up to in children treated in Cohorts 2 and 3

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When your child needs a hospital, everything matters.℠

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