UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 8-K

CURRENT REPORT PURSUANT
TO SECTION 13 OR 15(D) OF THE
SECURITIES EXCHANGE ACT OF 1934

Date of report (Date of earliest event reported): **February 9, 2018**

ABEONA THERAPEUTICS INC.
(Exact name of registrant as specified in its charter)

Delaware	**001-15771**	**83-0221517**
(State or other jurisdiction of incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)

3333 Lee Parkway, Suite 600
Dallas, TX 75219
(Address of principal executive offices) (Zip Code)

(214)-665-9495
(Registrant's telephone number, including area code)

N/A
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter). Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01. Regulation FD Disclosure
Item 8.01. Other Items

Presentation relating to Abeona Therapeutics Inc. The presentation is attached as Exhibit 99.1 and is incorporated herein.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits.

99.1	Presentation entitled "A Global Phase 1/2 Clinical Trial of Systemic Gene Transfer of scAAV9.U1a.hSGSH for MPSIIIA: Safety, Tolerability, and Efficacy"

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Abeona Therapeutics Inc.
(Registrant)

By: /s/ Stephen B. Thompson
Stephen B. Thompson
Vice President Finance
Chief Accounting Officer

Dated: February 9, 2018

EXHIBIT INDEX

Exhibit Number	
99.1	Presentation entitled "A Global Phase 1/2 Clinical Trial of Systemic Gene Transfer of scAAV9.U1a.hSGSH for MPSIIIA: Safety, Tolerability, and Efficacy"

Exhibit 99.1

A Global Phase 1/2 Clinical Trial of Systemic Gene Transfer of scAAV9.U1a.hSGSH for MPS IIIA: Safety, Tolerability, and Efficacy

Kevin M. Flanigan, MD
Center for Gene Therapy
Nationwide Children's Hospital
Columbus, OH

Complexo Hospitalario Universitario de Santiago de Compostela

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Women's and Children's Hospital
ADELAIDE

Disclosure Information

ASGCT 2017

Kevin Flanigan, MD

I have the following financial relationships to disclose:

Consultant for Sarepta Therapeutics; PTC Therapeutics; Audentes; Tivorsan; Marathon; Italafarmico.

Grant/Research support from CureDuchenne; Ben's Dream/The Sanfilippo Research Foundation; The Children's Medical Research Foundation; The Sanfilippo Children's Research Foundation; Cure Sanfilippo Foundation; the National Institutes of Health (NINDS and NIAMS)

Clinical trial site principal investigator Abeona Therapeutics; PTC Therapeutics; BioMarin; Akashi; NIH; Sarepta.

- and -

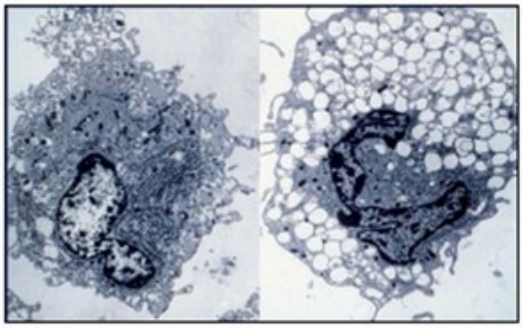
I will discuss the following off label use and/or investigational use in my presentation:

scAAV9.U1a.hSGSH for mucopolysaccharidosis type IIIA

Sanfilippo Syndrome (MPSIII)

Sanfilippo syndromes (MPS IIIA & IIIB) are inherited monogenic disorders that cause lysosomal enzyme deficiency

- Two most common forms, each categorized by a deficient enzyme:
  - MPS IIIA (*SGSH*)
  - MPS IIIB (*NAGLU*)
- Results in abnormal accumulation of glycosaminoglycans (GAGs) (sugars)
- Aggressive behavior, seizures, loss of speech/vision, inability to sleep
- *70% of children with MPS III do not reach age 18*



Normal cell

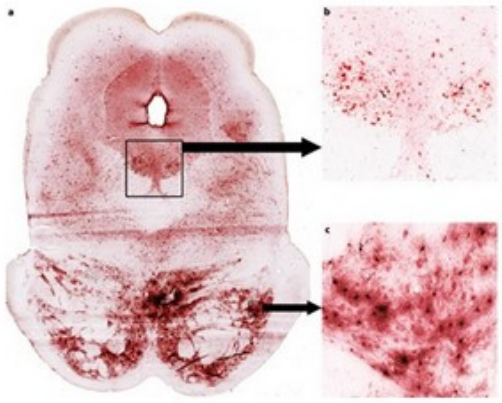
Cell with lysosome deficiency

No approved treatments available

- Incidence is estimated to be 1 in 70,000 births

Intravenous Administration of AAV9 as route and vector of choice for MPS gene therapy

- Systemic delivery of AAV9 demonstrated safety in Non-Human-Primates (n=8) for MPS IIIB up to 6 months
- AAV9 efficiently crosses the blood-brain-barrier
- Expressed in multiple CNS cell types
- Demonstrated CNS pathology reduction
- Systemic delivery (versus intracranial) has resulted in transduction of peripheral affected tissues (liver, spleen, etc.)

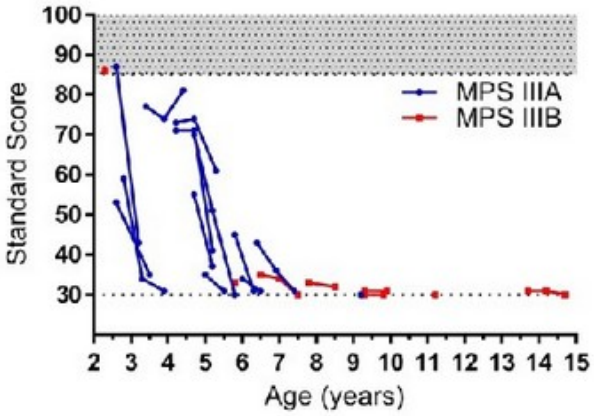


scAAV9-GFP expression in brain 3 weeks after intravenous injection of $1.0x10^{13}$ vgp/kg into 3 year old cynomolgous macaque

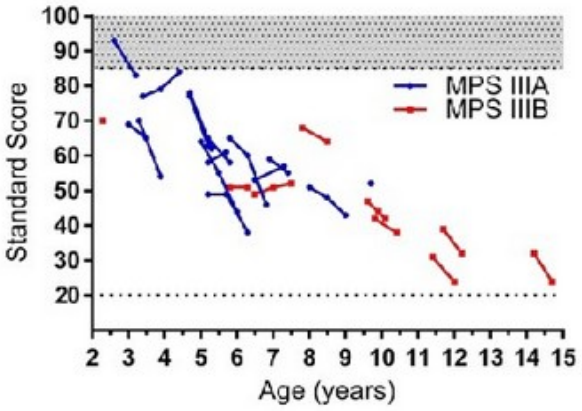
Ref: Bevan 2011 Mol Ther 19(11):1971-80
Murrey 2014 Hum Gen Ther Clin Dev 25:72-84.

Clinical Trial Readiness:
The NCH MPSIII Natural History Study

- **25 subjects: 15 MPS IIIA and 10 MPS IIIB**
- Assessments at 0, 6, and 12 months
- Established standards for biochemical (serum and CSF) studies and volumetric (MRI) relevant to the clinical trial
- Validated neurocognitive and behavior outcome measures for MPS III clinical trials



Leiter-3 Nonverbal-IQ Score over 6 months.
Normal range of unaffected children is shown shaded.



Vineland Scale - Behavior Assessments over 1 year
Normal range of unaffected children is shown shaded.

Truxal et al (2016) Mol Genet Metab, 119(3):239-248

Phase I/II gene transfer clinical trial of scAAV9.U1a.hSGSH for Mucopolysaccharidosis (MPS) IIIA

ClinicalTrials.gov: NCT02716246

- Phase 1/2 open-label, dose-escalation clinical trial
  - **Cohort 1**: 5 X 10^{12} vg/kg (n=3 subjects): ages 6.5, 7.0 and 5.4
  - **Cohort 2**: 1 X 10^{13} vg/kg (n=3 subjects): ages 2.9, 2.5 and 3.2
  - **Cohort 3**: 3 x 10^{13} vg/kg (4 patients enrolled): ages 5.2, 2.3, 4.9 and 8.0
    - will enroll 4-5 additional patients at three global sites: US, Spain and Australia

Primary Outcome	Determination of safety based on the development of unacceptable toxicity: defined as the occurrence of two or more unanticipated Grade III or higher treatment-related toxicity.
Secondary Outcomes	• Reduction in CSF and/or urinary HS and/or GAG • Increase in CSF and serum SGSH enzyme activity levels • Reduced liver and spleen volumes at 6 and/or 12 months after treatment, as measured by magnetic resonance imaging (MRI) • Improved adaptive functioning, or arrest of decline in adaptive functioning, as assessed by parent report using the Vineland Adaptive Behavior Scale • Improved cognitive ability or arrest of cognitive deterioration at 6 and/or 12 months after treatment, as measured by direct testing of the child using the Leiter International Performance Scale and the Mullen Scales of Early Learning

Dosing and short-term monitoring

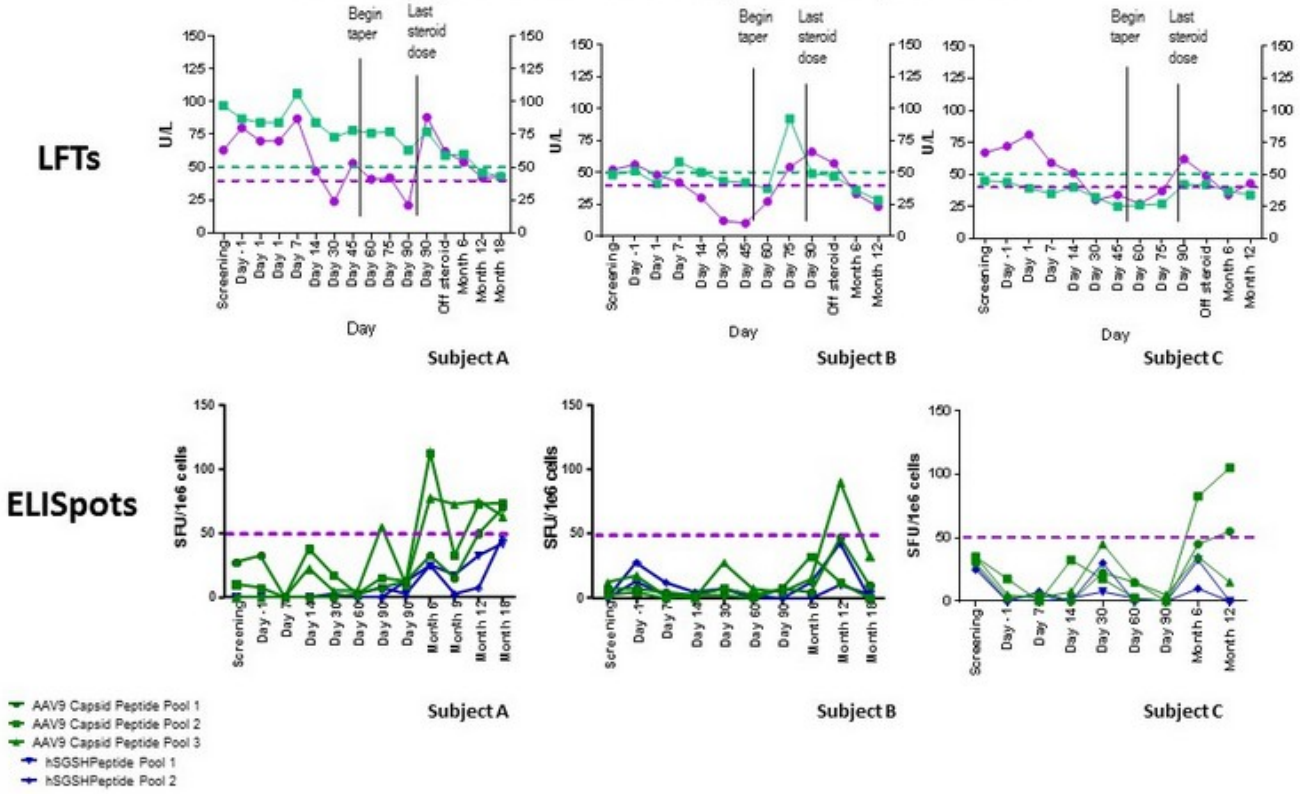
- **Day -1:** subjects receive first dose of oral prednisolone elixir at 1 mg/kg/day.
- **Day 0:** subjects receive gene transfer delivered under sedation within the Procedure Center over 15 minutes.
- **First 48 hours:** Subjects are monitored in the Pediatric Intensive Care Unit (PICU) for 24 hours, then discharged to the neurology service (hospital floor) for monitoring for another 24 hours prior to discharge
- Discharged on oral prednisolone elixir

No drug-related serious adverse events (SAE) through >3,100 days cumulative post-injection

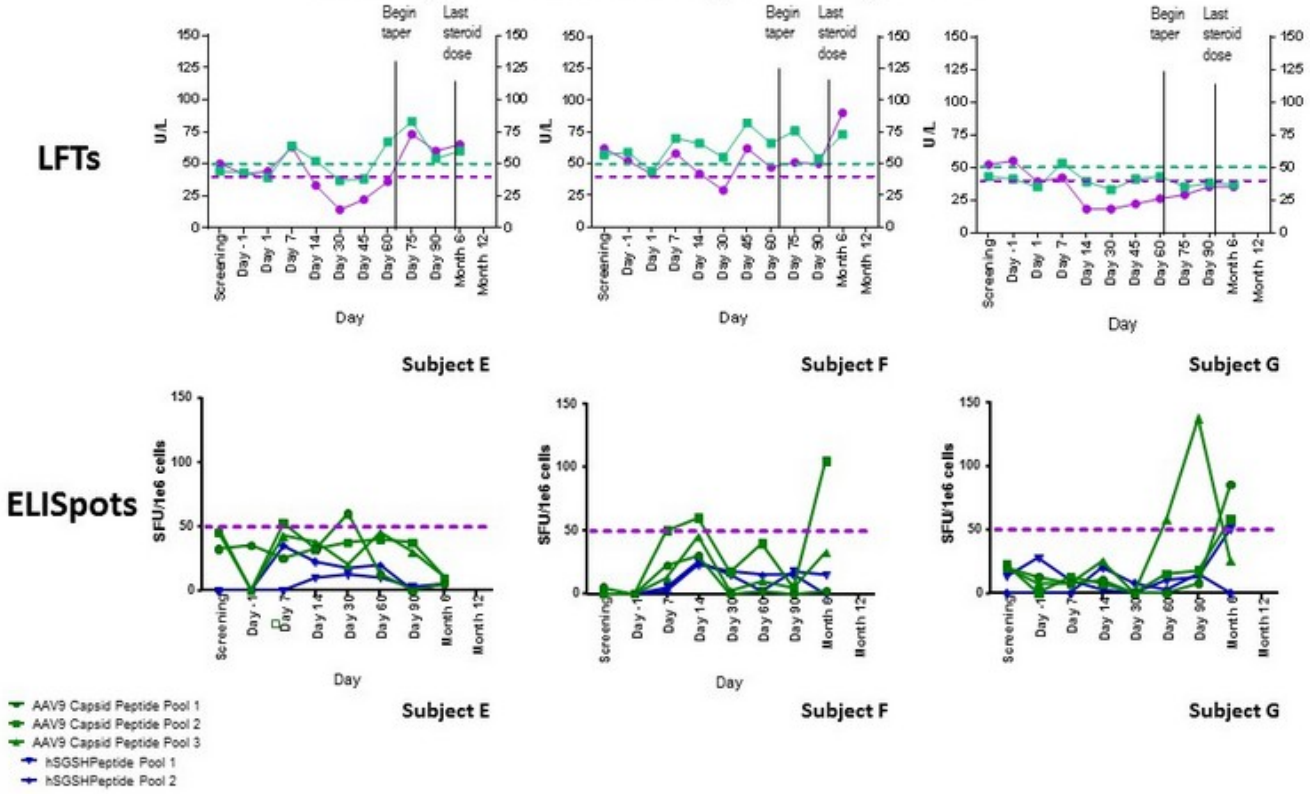
Most common *non-serious* adverse events (AE):

- Irritability (n=6), attributed to prednisolone therapy
- Vomiting (n=5), judged unrelated/unlikely related
- Upper respiratory infection (n=5), judged unrelated/unlikely related
- ALT elevations (n=4), resolved (n=3) and resolving/recovering (n=1), judged possibly/probably related
- Cushingoid facial fullness (n=4), attributed to prednisolone therapy
- Thrombocytopenia (n=4), judged unlikely/possibly related
  - All platelet changes resolved by Day 30 (n=3), except one, still ongoing

Cohort 1 Serum transaminases and ELISpot remain stable through 12 months post-injection

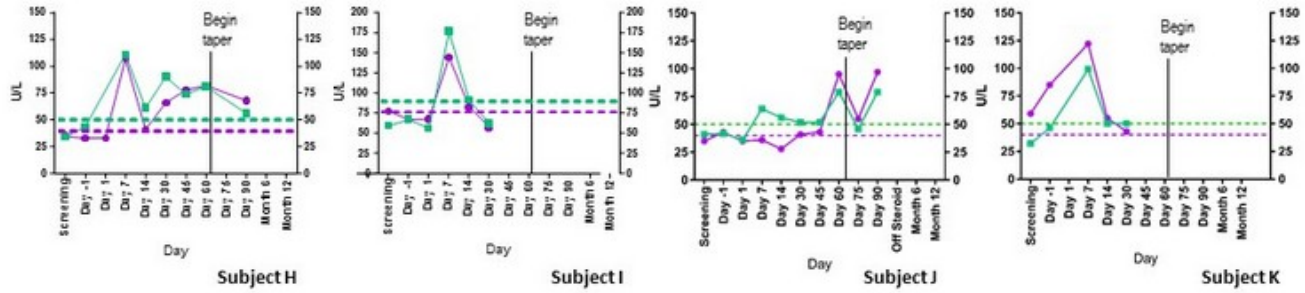


Cohort 2 Serum transaminases and ELISpot remain stable through 6 months post-injection

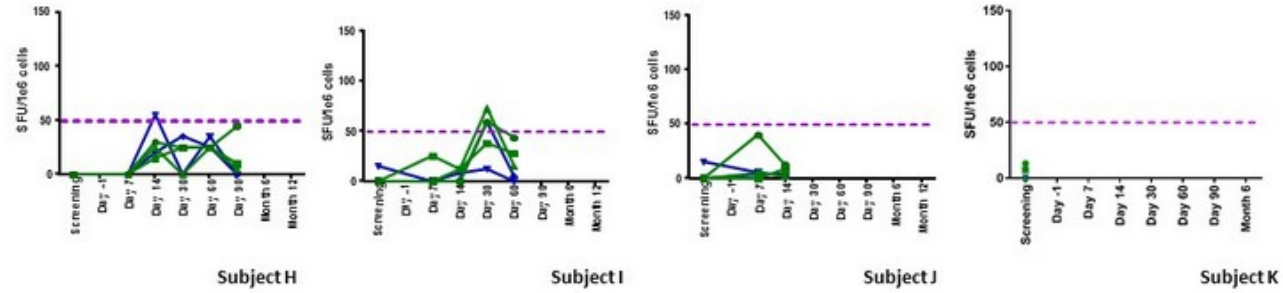


Cohort 3 Serum transaminases and ELISpot remain stable through Day 60 post-injection

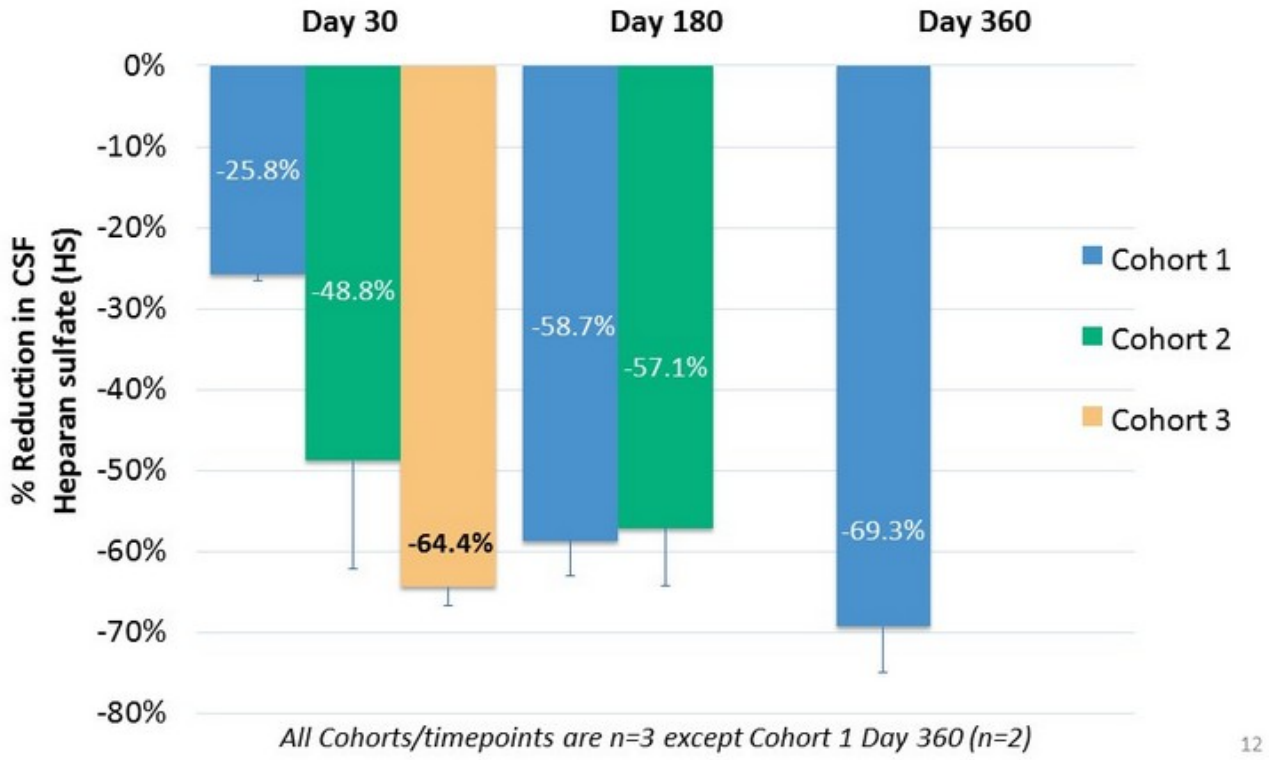
LFTs



ELISpots

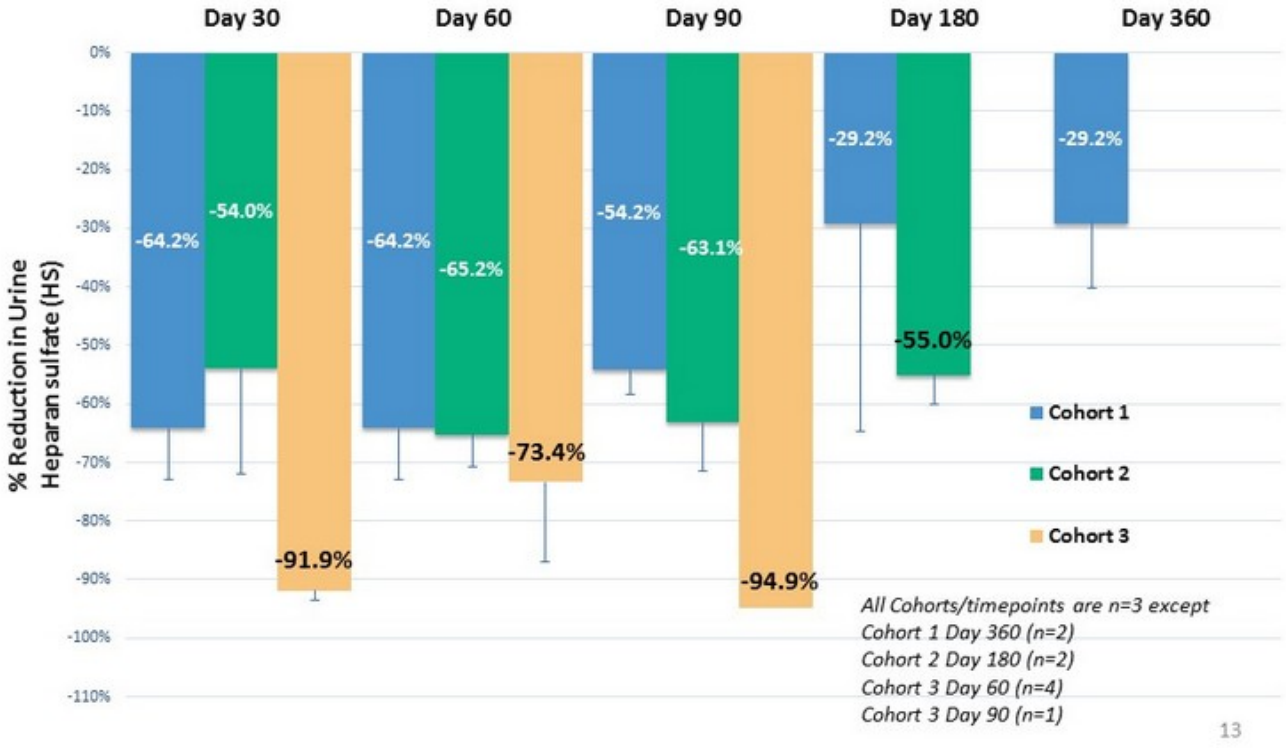


Durable, Time and Dose-dependent Reduction in CSF Heparan Sulfate

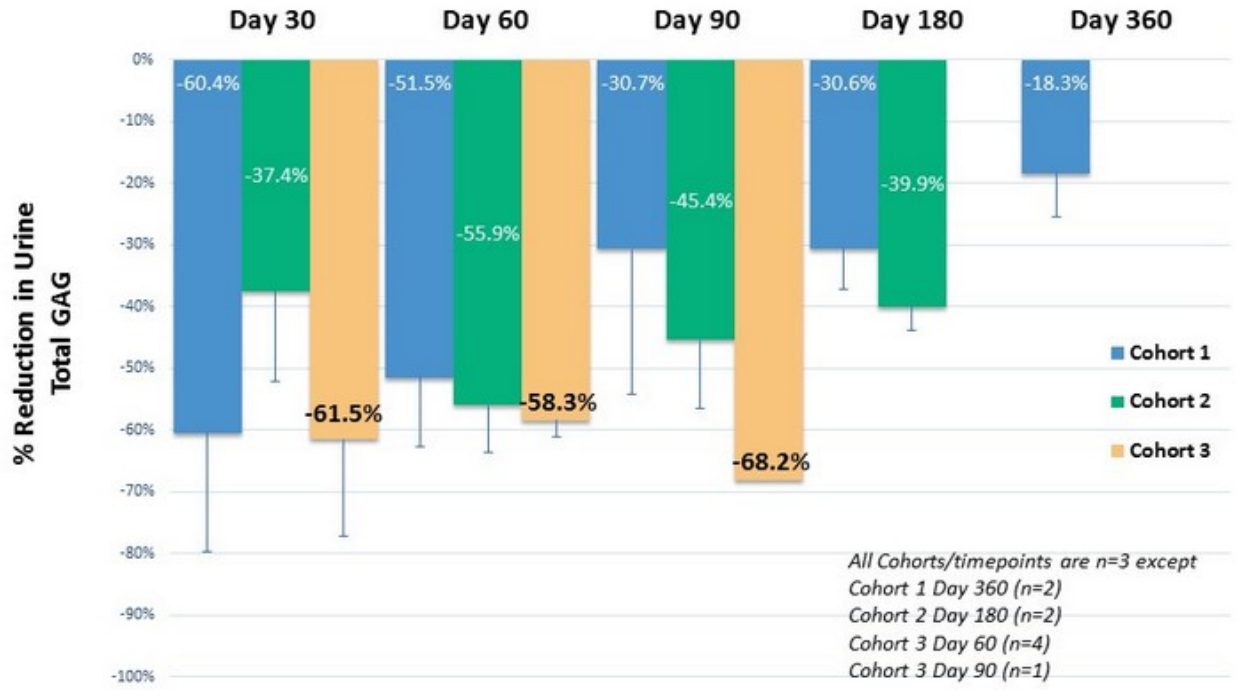


All Cohorts/timepoints are n=3 except Cohort 1 Day 360 (n=2)

Durable, Time and Dose-dependent Reduction in Urine Heparan Sulfate



Durable, Time and Dose-dependent Reduction in Urine Total GAG

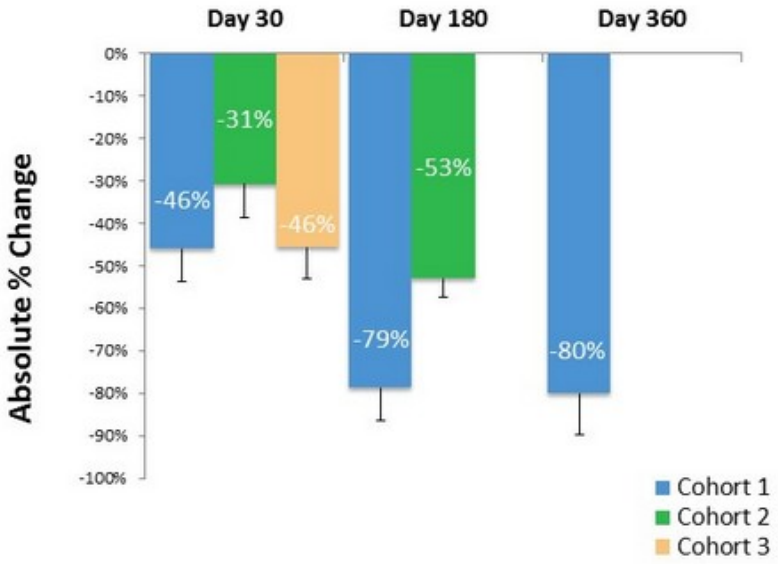


Durable, Time and Dose-dependent Reduction in Liver Volume by MRI

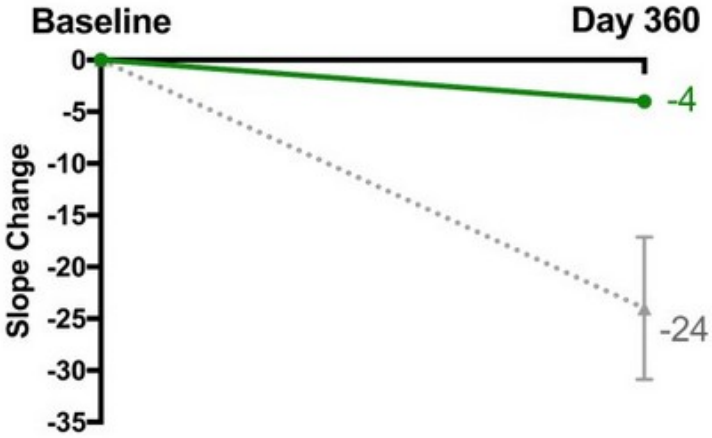
Cohort 1	% Normal Liver Volume		
	Subject A	Subject B	Subject C
Screening	225%	202%	225%
Day 30	183%	174%	157%
Day 180	147%	150%	119%
Day 360	147%	149%	116%

Cohort 2	% Normal Liver Volume		
	Subject D	Subject E	Subject F
Screening	211%	194%	183%
Day 30	171%	157%	168%
Day 180	151%	140%	138%

Cohort 3	% Normal Liver Volume		
	Subject G	Subject H	Subject I
Screening	234%	151%	198%
Day 30	158%	134%	154%



Cohort 1: Evidence for Neurocognitive Stabilization (Leiter) Compared to Natural History Study at 1 Year Follow-Up



Cohort 1* ***Ages (5.4-7.0)***

NHS C1 Age Match

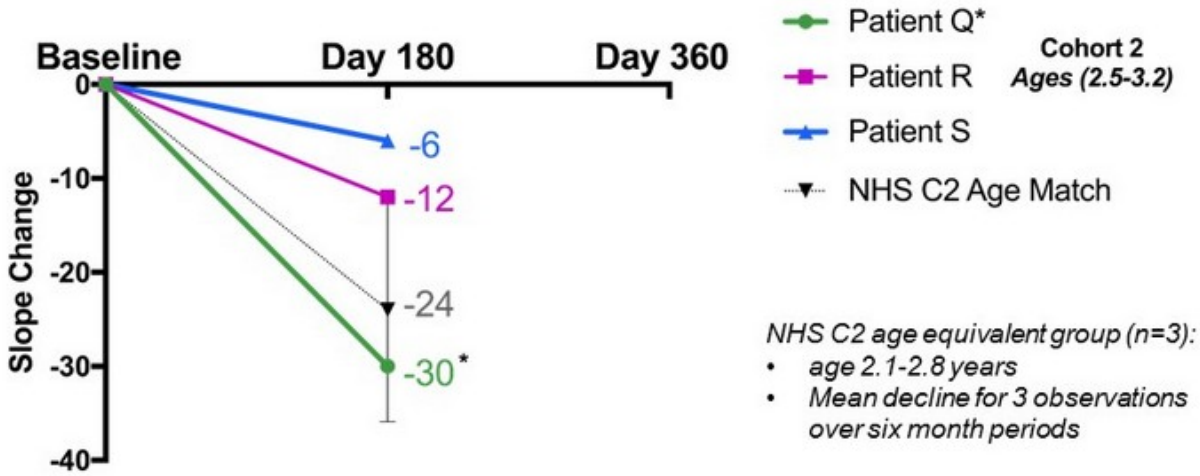
NHS C1 age equivalent group (n=8):

- *age 4.2 -6.4 years*
- *Mean decline for 12 observations over six month periods*

**Cohort 1 (n=2):*

- *1 subject was unable to be assigned a valid score at baseline and was assigned the test's floor value*
- *1 subject was unable receive a valid scored at either baseline or month 12 and was excluded from the analysis*

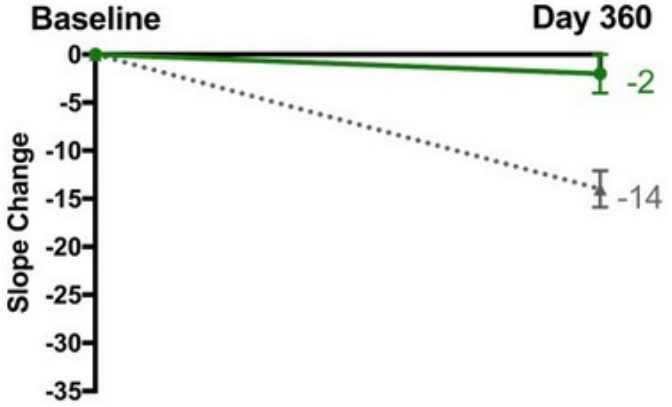
Cohort 2: Neurocognitive Stabilization (Leiter) in 2 of 3 Subjects Compared to Natural History Study at 6-months Follow-Up



NHS C2 age equivalent group (n=3):

- *age 2.1-2.8 years*
- *Mean decline for 3 observations over six month periods*

Cohort 1: Stabilization (Vineland) of Adaptive Behavior Compared to Natural History Study at 1 Year Follow-Up



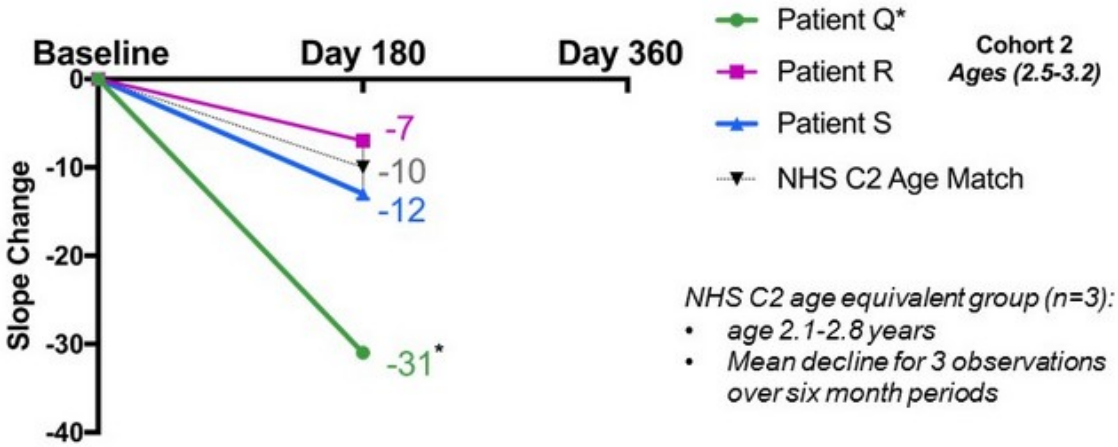
Cohort 1 (n=3) ***Ages (5.4-7.0)***

NHS - C1 Age Match

NHS C1 age equivalent group (n=9):

- *age 4.2 -6.4 years*
- *Mean decline for 12 observations over six month periods*

Cohort 2: Adaptive Behavior (Vineland)
Compared to Natural History Study at 6-months Follow-Up



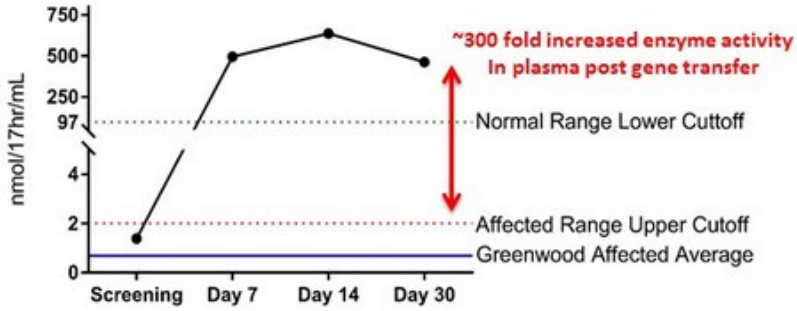
NHS C2 age equivalent group (n=3):

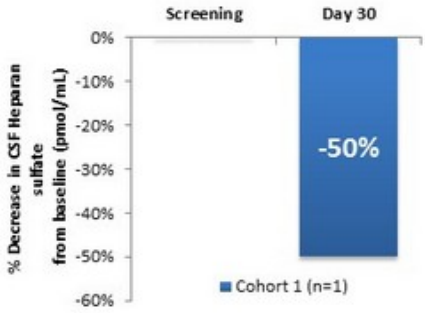
- *age 2.1-2.8 years*
- *Mean decline for 3 observations over six month periods*

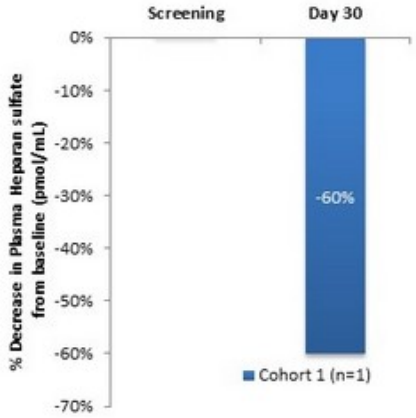
Systemic delivery of scAAV9.U1a.hSGSH for MPSIIIA is well tolerated and demonstrates a biological effect

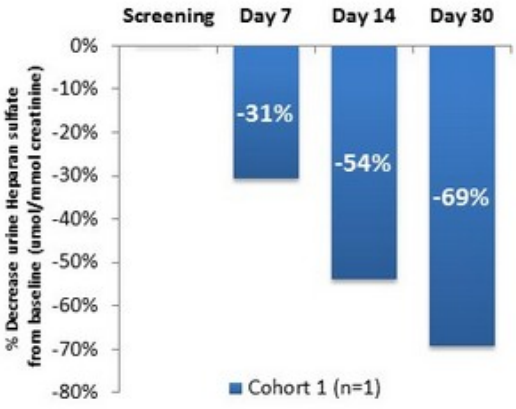
- Well-tolerated following systemic delivery in 10 subjects through > 3,100 follow-up days
- Evidence of efficacy, including:
  - Decreased CSF GAGs (HS fragments) at 30, 180 and 360 days
  - Diminished liver volumes at 30, 180 and 360 days
  - Decreased urinary GAGs (HS fragments and Total GAGs) at 30, 180 and 360 days
  - Preliminary evidence for stabilization or improvement of some cognitive functions at 6 months after treatment
    - 2 of 3 subjects in Cohort 1 improved neurocognitive scores at 1 year compared to NHS
    - 2 of 3 subjects in Cohort 2 improved neurocognitive scores at 6 months compared to NHS
- Dosing in Cohorts 2 and 3 demonstrate time- and dose-dependent biological effect

Further demonstration of Systemic AAV9 Safety and Efficacy in Phase 1/2 Clinical Trial for MPS IIIB









Acknowledgments

We gratefully thank all of the MPS patient and family community for participation in and support of our studies.

- **Nick Smith, MD, Ph.D – PI – Adelaide**
- **Maria Couce, MD – PI – Santiago de Compostella**
- **Maria Castro, MD – Sub-Investigator, Santiago de Compostella**
- **Kristen Truxal, MD – Co-Investigator, NCH**
- **Kim McBride, MD– Co-Investigator, NCH**
- **Maria Fuller, Ph.D – Director, SA Pathology**

- Doug McCarty, PhD
- Haiyan Fu, PhD
- Tabatha Simmons, PhD
- Shawn Aylward, MD
- Kelly McNally, PhD
- Krista Kunkler
- Federica Rinaldi, PhD

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Complexo Hospitalario Universitario de Santiago de Compostela

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