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): **December 6, 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.)

1330 Avenue of the Americas, 33rd Floor,
New York, NY 10019
(Address of principal executive offices) (Zip Code)

(646) 813-4712
(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.

On December 6, 2018, Abeona Therapeutics Inc. (the "Company") presented at its second R&D Day in New York, New York. A replay of the presentation will be available on the investor relations section of the Company's website. Copies of the presentation slides and of the press release dated December 6, 2018 relating to the presentation are furnished as Exhibits 99.1 and 99.2, respectively, to this Current Report on Form 8-K and are incorporated herein by reference.

The information furnished pursuant to this Current Report on Form 8-K, including Exhibits 99.1 and 99.2 hereto, shall not be considered "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be incorporated by reference into future filings by the Company under the Securities Act of 1933, as amended, or under the Exchange Act, unless the Company expressly sets forth in such future filings that such information is to be considered "filed" or incorporated by reference therein.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	Description
99.1	Abeona Therapeutics Inc. R&D Day presentation, dated December 6, 2018.
99.2	Press release, dated December 6, 2018.

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/ Neena Patil
Name: Neena Patil
Title: General Counsel and Secretary

Date: December 6, 2018

Exhibit 99.1


Abeona
THERAPEUTICS
2018 R&D Day
December 6, 2018
MONSIE
Living with RDEB

8:00 **Breakfast / Registration**

8:45 **"The Edge of Hope" and Opening Remarks**

9:00 **EB-101 Review: Pivotal Phase 3 Preparation** Jean Tang, M.D., Ph.D., Jay Bircher, Max Colao, João Siffert, M.D.

9:30 **Panel: *CMC and Regulatory: Challenges, Strategies, and Capabilities*** Kaye Spratt, Ph.D., Maritza McIntyre, Ph.D., Adam Davis, Ph.D., Jay Bircher

9:55 **Break & Coffee**

10:10 **Fireside Chat: *Gene Therapy 2019: What's Next?*** Barry Byrne, M.D., Ph.D., Tim Miller, Ph.D.

10:35 **MPS IIIA & B Clinical Update** João Siffert, M.D., Adam Davis, Ph.D., Kaye Spratt, Ph.D.

11:00 **Break & Coffee**

11:10 **Batten Disease: CLN1 & CLN3 Programs** Steven Gray, Ph.D., Tim Miller, Ph.D.

11:30 **Next Generation AAV Capsid Library (AIM)** Tim Miller, Ph.D.

11:50 **Panel: *Engineering AAV for Improved Infectivity, Tropism Onset of Expression*** Tim Miller, Ph.D., Steven Gray, Ph.D., Mitch Drumm, Ph.D.

12:15 **Closing Remarks** João Siffert, M.D.

12:30 **Lunch**


EB-101 for Recessive Dystrophic Epidermolysis Bullosa (RDEB)
NOEMI
Living with RDEB

EB-101: Breakthrough Therapy for RDEB

Jean Tang, M.D., Ph.D.
Clinical Overview and Phase 1/2 Trial Results

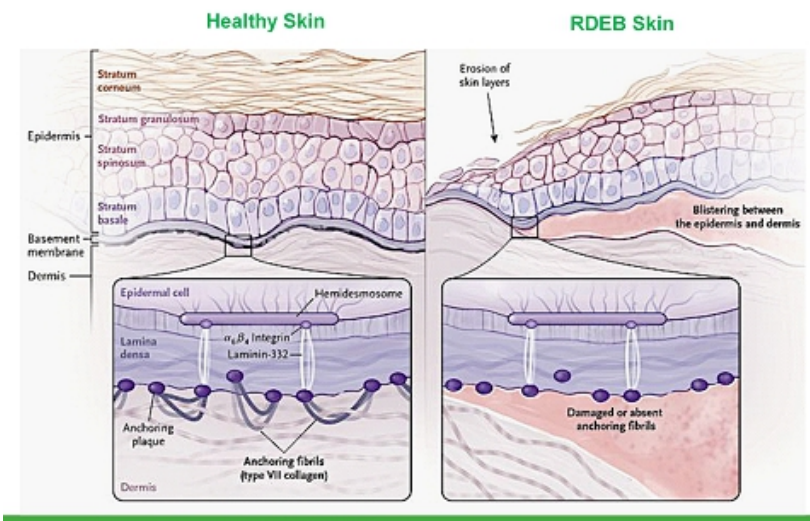
Jay Bircher
EB-101 Manufacturing and Quality Update

Max Colao
EB Commercial Assessment

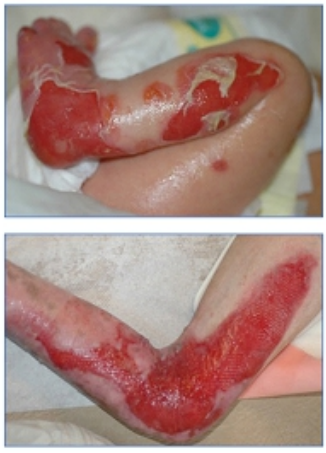
João Siffert, M.D.
Abeona Clinical Program Update

RDEB is among the most devastating forms of EB, caused by mutation of type VII collagen and breakdown of anchoring fibrils

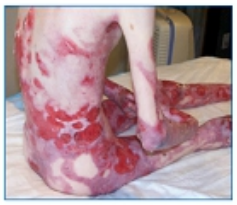
Type VII collagen (C7) gene is responsible for anchoring the epidermis to dermis



RDEB



Beyond chronic epidermal wounds, patients suffer from pain, itch and widespread complications impacting QoL and life expectancy



Dermatologic	• Widespread blistering and extensive dystrophic scarring • Born without patches of skin • Infections from open wounds or blisters • 75-90% lifetime risk of squamous cell carcinoma
Oral Cavity / GI	• Fusion of tongue to floor of month (ankyloglossia) • Diminution of oral cavity size (microstomia) • Esophageal mucous membrane blisters and erosion • Malnutrition and growth retardation due to lack of food / fluid intake
Orthopedic	• Fusion of fingers/toes resulting in "mitten" hands/feet (pseudosyndactyly) • Development of muscle contractures • Poor ambulatory function due to progressive blistering and fusion of toes
Urologic / Renal	• Urethral erosions, strictures, bladder dysfunction, glomerulonephritis
Ocular	• Corneal erosions leading to scarring and vision loss
Cardiac	• Dilated cardiomyopathy due to carnitine and selenium deficiencies
Patient QoL	• Stress and depression • Pain and itch at wound site

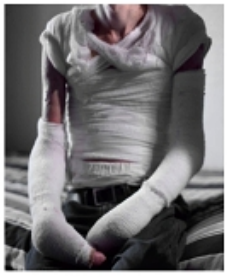
1. Sorg, L et al. 2015; 2. Fine, JD. 2010; 3. Intong, LRA et al. 2012.

No approved treatments – wound care is essential to promote healing & prevent fluid loss, but time-consuming, painful, & costly

Standard Blister and Wound Dressing

- Dressings typically consist of three layers
- Direct costs associated with RDEB wound care range from $20,000 to $240,000/year
- Water baths may aid with bandage removal; creams and gels prevent irritation and crusting

Secondary Treatments / Interventions



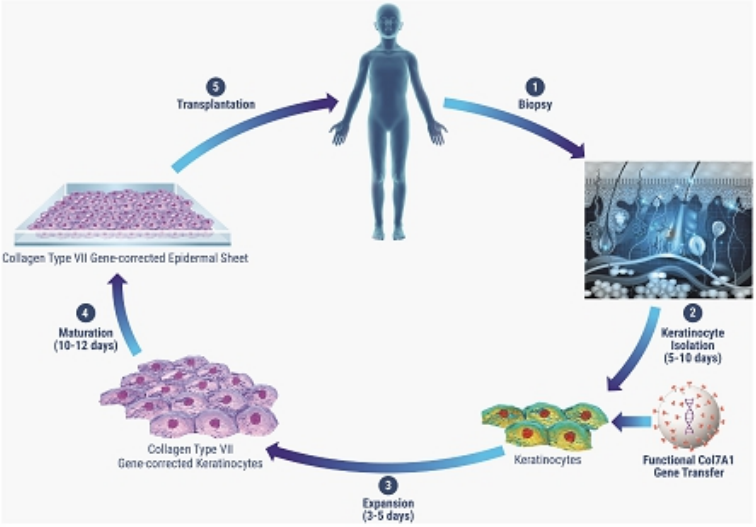
- Measures to reduce skin friction
- Measures to control body temperature to prevent fluid loss
- Proactive nutritional support, including enteral tube feeding
- Careful monitoring of chronic wounds; biopsy may be required

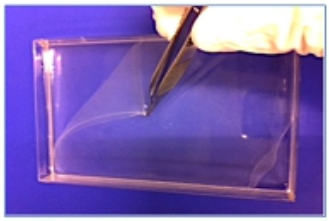
Implications of Poorly Treated Wounds

- Occurrence of strikethrough and exudate can lead to serious infections
- Severe RDEB patients have a 75-90% risk of developing squamous cell carcinoma (SCC)

1. International Consensus, Best Practice Guidelines for Skin and Wound Care in EB, 2012; 2. Stanford Basic Care Tips for EB: A Parents Guide; 3. Pfender, EG et al., "Dystrophic EB", 2006.

EB-101: Ex-Vivo Autologous Gene-Corrected Cell Therapy

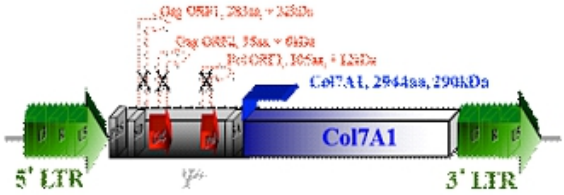


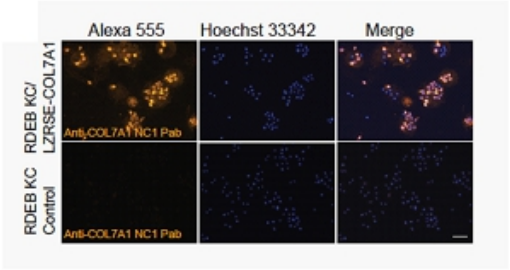


EB-101: 26-days post growth

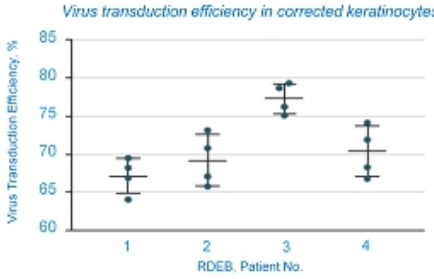
EB-101: ready for patients

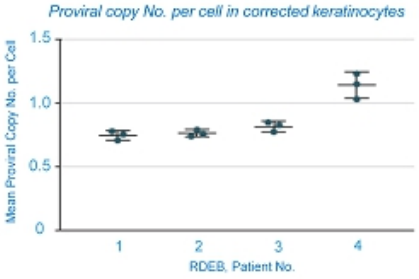
EB-101: Retroviral Vector LZRSE with 9 kB COL7A1 Gene





Virus Transduction Efficiency and Mean Proviral Copy Number per Cell in Corrected Keratinocytes in 4 Patients With RDEB





128-patient Natural History study supports clinical design

Multi-year enrollment complete:

- 1,436 wounds (1,041 recurrent wounds/395 chronic open wounds)
- 100% of patients reported chronic open wounds (no healing> 3m) or recurrent wounds
- 38% of chronic open wounds were large (≥40 cm^2) and 39% remained unhealed for ≥7 yrs
- 53% of recurrent wounds were present for ≥7 years
- Lack of efficacy for wound healing in RDEB patients treated with allograft products
  - 13 patients with a total of 15 chronic wounds treated with Apligraf® and Dermagraft® allograft
  - Only 7% (1/15 treated wounds) remained healed after 3 months
  - 0/15 treated wounds remained healed after 6 months

EB-101: Phase 1/2 Trial Results

Primary Endpoint: Safety

- Wound healing percent versus baseline at 3, 6 and 12 months

Secondary Endpoints

- Duration of Type VII collagen (C7) expression
- Presence of anchoring fibrils
- Patient Reported Outcomes: Pain, Itch and Durability
- Long-term (5 year) follow-up protocol ongoing

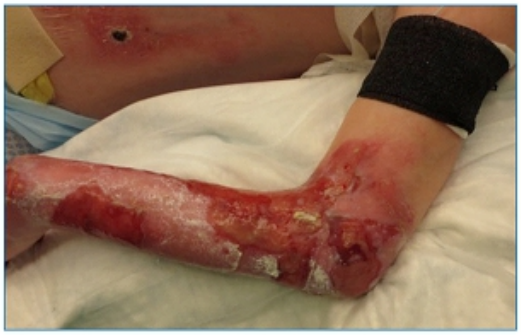
ClinicalTrials.gov

EB-101: Phase 1/2 Trial Patient Characteristics

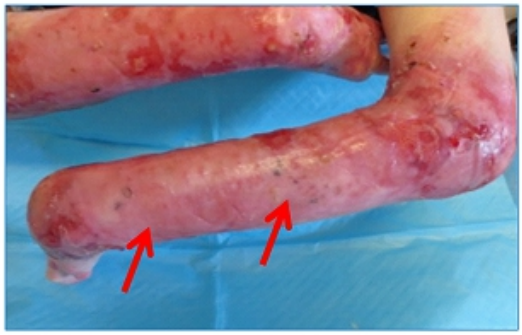
Patient #	1	2	3	4	5	6	7
Age	23	19	32	18	32	32	45
Sex	M	M	M	M	F	M	F
COL7A1 Mutation 1 (Location)	90delC (exon 2)	90delC (exon 2)	6527dupC (exon 80)	8053 C>T (exon 109)	4172dupC (exon 36)	8440 C>T (exon 114)	6176A>G
COL7A1 Mutation 2 (Location)	5048_5051 dup4 (GAAA) (exon 54)	5048_5051 dup4 (GAAA) (exon 54)	7485+5 G>A (intron 98)	7929+ (11_26) del16 (intron 106)	4182_4188dup7 (exon 36)	8440 C>T (exon 114)	6501G>A
C7 expression by IF	Undetectable NC1 and NC2	Undetectable NC1 and NC2	Trace NC1; Undetectable NC2	Undetectable NC1 and NC2	Undetectable NC1 and NC2	Undetectable NC1 and NC2	Reduced NC1 and NC2
C7 Expression by Western	NC1+	NC1+	NC1+	NC1+	NC1+	NC1+	NC1+
EM	No mature AF; sub-LD split	No mature AF; sub-LD split	No mature AF; sub-LD split	No mature AF; sub-LD split	Rudimentary AFs, low LH24	Poorly formed AFs	Rudimentary AFs, low LH24
Circulating C7-antibodies	Negative	Negative	Negative	Negative*	Negative	Negative	Negative
Wounded surface area	8%	10-15%	4%	25-30%	10%	20%	5%
History of SCC	No	No	No	No	Yes	No	No
Previous allograft	No	No	No	No	No	Yes	No

Primary endpoint (safety): No systemic viral infection, immune reaction, or squamous cell carcinoma

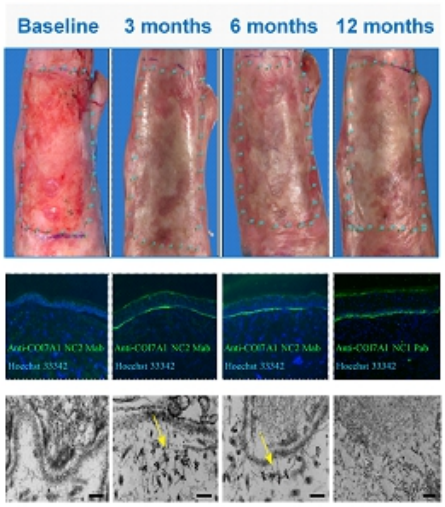
Chronic wounds >5 yr



Healed wounds (6 months)



Gene therapy skin grafts restore collagen 7 that forms functional anchoring fibrils



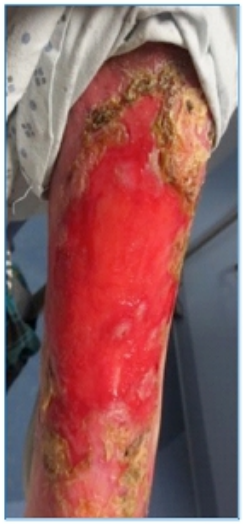
Site	Location	Descript	Estimated duration	3m	6m	12m
A	R lateral hand	Erosion	3-5 yrs			
B	R medial hand	Scar tissue	3-5 yrs			
C	L ventral foot	Erosion and scar	3-5 yrs			
D	L hand	Scar tissue	3-5 yrs			
E	R foot	Erosion and scar	3-5 yrs			
Z	L ventral foot	Induced wound	New			

Safety Parameters	3m	6m	12m
Immunogenicity	-	-	IgA,Ig M
RCR	-	-	-
SCC	-	-	-

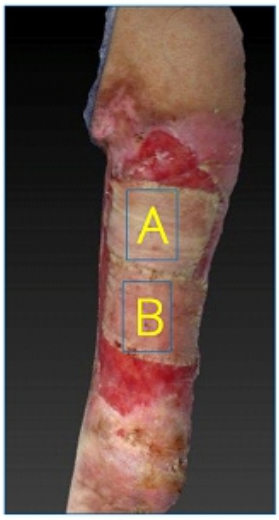
≥75% healed
50-70% healed
< 50% healed

Patient 5: Wound healing (left arm, chronic open wound (10+yr)

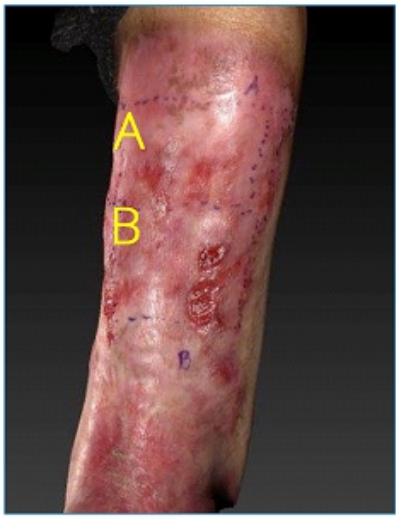
Baseline



Grafting

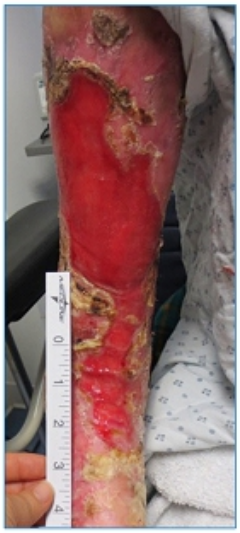


9 months

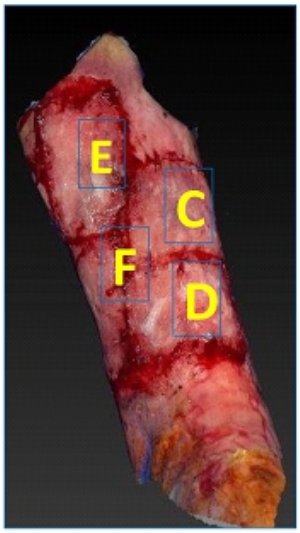


Patient 5: Wound healing seen for 9-12 months

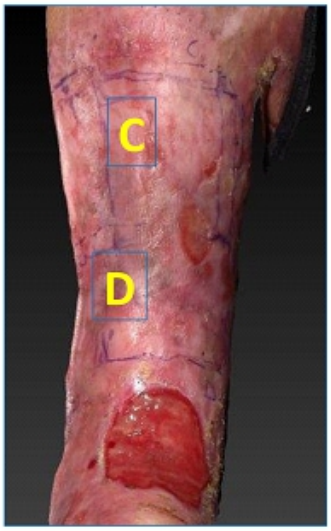
Baseline



Grafting



9 months



EB-101: Phase 1/2a results demonstrate durable efficacy

Patient	Site	Location	Description at Baseline	Wound Age	1 month[2]	3 months	6 months	12 months	2 years	3 years	4 years	5 years
1	A	R distal forearm	Erosion	>5 yrs								
	B	L forearm	Erosion	>5 yrs								
	C	R proximal forearm	Erosion	>5 yrs								
	D	R shoulder	Inflamed erosion	>5 yrs								
	E	L arm	New blister	1 wk								
	Z	R arm	Induced wound	New								
2	A	Central chest	Erosion	>5 yrs								
	B	L shoulder	Erosion and scar	>5 yrs								
	C	R forearm	Erosion and scar	3-5 yrs								
	D	R posterior shoulder	Inflamed erosion	>5 yrs								
	E	Lower back	Erosion	>5 yrs								
	Z	R upper chest	Induced wound	New								
3	A	R lateral hand	Erosion	3-5 yrs								
	B	R medial hand	Scar tissue	3-5 yrs								
	C	L ventral foot	Erosion and scar	3-5 yrs								
	D	L hand	Scar tissue	3-5 yrs								
	E	R foot	Erosion and scar	3-5 yrs								
	Z	L ventral foot	Induced wound	New							Unable to assess	
4	A	L distal forearm	Inflamed erosion	>5 yrs								
	B	L medial forearm	Inflamed erosion	>5 yrs								
	C	L proximal forearm	Inflamed erosion	>5 yrs								
	D	R lateral forearm	Inflamed erosion	>5 yrs								
	E	R distal forearm	Inflamed erosion	>5 yrs								
	Z	R medial forearm	Induced wound	New								
5	A	L upper arm	Erosion	16 yrs								
	B	L upper arm	Erosion	16 yrs								
	C	R upper arm	Erosion	16 yrs								
	D	R upper arm	Erosion	16 yrs								
	E	R upper arm	Erosion	16 yrs								
	F	R upper arm	Erosion	16 yrs								
6	A	R back axilla (lateral), upper row	Erosion	20 yrs								
	B	R back axilla (middle), upper row	Erosion	20 yrs								
	C	R back axilla (medial) upper row	Erosion	20 yrs								
	D	R back axilla (lateral) lower row	Erosion	20 yrs								
	E	R back axilla (middle), lower row	Erosion	20 yrs								
	F	R back axilla (medial) lower row	Erosion	20 yrs								
7	A	R back corner	Erosion	20 yrs								
	B	R lateral outer leg	Erosion	20 yrs								
	C	R Back central	Erosion	20 yrs								
	D	R Back medial	Erosion	20 yrs								
	E	R foot front anterior	Erosion	20 yrs								
	F	Back upper corner	Erosion	20 yrs								

RDEB Wound Healing
>75%
>50%-75%
<50%

EB-101 significantly improved patient-reported outcomes

	Pre-Grafting	3 Month	6 Month	9 Month	12 Month
Presence of PAIN at wound site (% reported yes)	58%	0%	17%	20%	0%
ITCH at wound site (% reported yes)	67%	5%	17%	50%	25%
Lack of durability at wound site (% reported yes)	90%	0%	0%	0%	0%
Ease of blistering at wound site (% reported yes)	83%	0%	0%	0%	0%

Summary of Phase 1/2 Trial Results

- Focus on large, difficult to treat chronic wounds
- No product-related serious adverse events observed to date
- Significant and durable wound healing, with up to 5 years of follow-up
- Continuous type VII collagen expression >2 years post treatment
- No detection of replication competent retrovirus (RCR) up to 4 years
- Phase 3 study warranted

Manufacturing

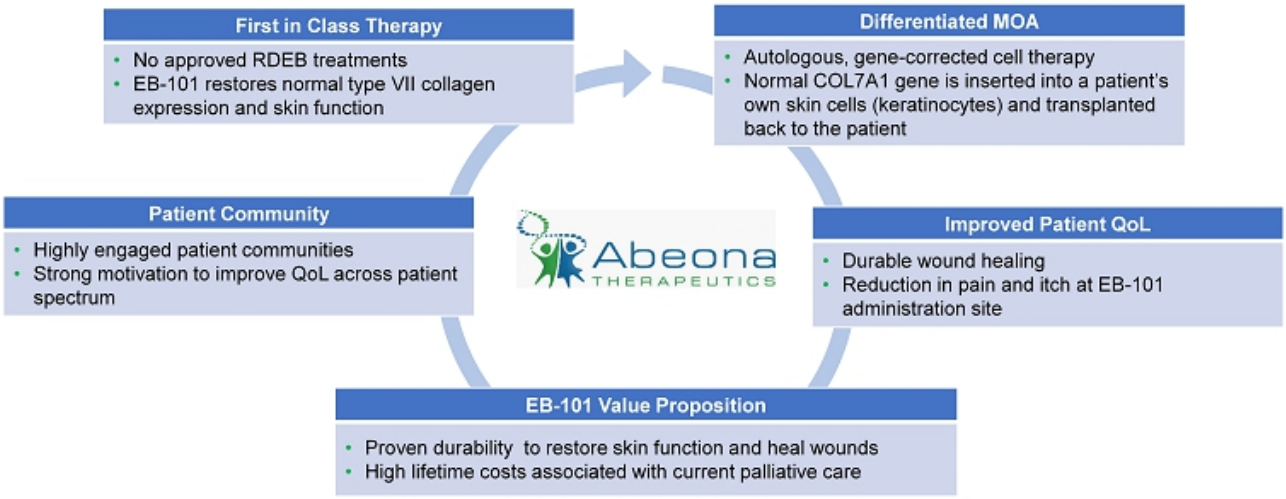
The Elisa Linton Center for Rare Disease Therapies

Facilities/Production – Elisa Linton Center

- Upfront investment to establish GMP and preparation for commercial production in Cleveland strengthens ability to execute product launch
- Established GMP manufacturing of EB-101 gene-corrected cell therapy
  - Cell Processing Facility:
    - Commercially viable GMP suites
    - Dedicated to the production of EB-101
    - Capacity for Commercial launch and scalable
- Abeona Cleveland to produce both clinical and commercial material


Commercialization
MONSIE
Living with RDEB

EB-101: Transformative treatment for patients suffering from RDEB



EB-101 Summary and Next Steps

Successful Phase 1/2

- Favorable safety profile with no product-related SAEs to date
- Significant and durable wound healing, with up to 5 years of follow-up

Established GMP manufacturing capability at Abeona

- Manufacture both clinical and commercial product in Cleveland
- Scalable capacity to support commercial launch

VITAL: Phase 3 Trial

- Regulatory CMC review 1H19
- First patient expected to enroll mid-2019

VITAL: A Pivotal Phase 3 Trial Study of EB-101 for the Treatment of Recessive Dystrophic Epidermolysis Bullosa (RDEB)

- Multicenter, n=10-15 evaluable pts (≥35 grafts), randomized (up to 6 wounds >20 cm^2 wounds unhealed for at least 6 months per patient) v. intra-patient controls (≥1 per patient)
- Primary endpoint is >50% healing at 3 months (by Canfield quantitation)
- Secondary endpoints:
  - Investigator global assessment (IGA)
  - PRO-Change in PAIN from baseline
  - PRO-Change in ITCH for baseline
  - Exploratory endpoint: C7 expression in 1/6 grafts on each patient

CMC and Regulatory Landscape for Gene Therapy: Challenges, Capabilities & Strategies

Maritza McIntyre, Ph.D.

Gene Therapy Landscape: CMC Opportunities and Challenges

- The gene therapy field has advanced to offer the potential for effective cures or significant improvements in the lives of patients with rare diseases and cancer
- Early, robust efficacy is often demonstrated during Phase 1 trials of gene therapy products for rare diseases
  - Opportunity to expedite clinical development- RMAT, streamlined pivotal trial design, novel endpoints, accelerated approval
- Expedited clinical development challenges incremental approach to CMC development
  - Pivotal trial study should include data from subjects treated with material comparable to product intended for commercialization
  - Full compliance with 211 GMP requirements required for approval
    - Process validation- including PPQ, facility, equipment, utility validation data submitted
    - Pre-approval inspection
    - Stability data to support expiration dating (validated methods)

Gene Therapy Landscape: CMC Opportunities and Challenges

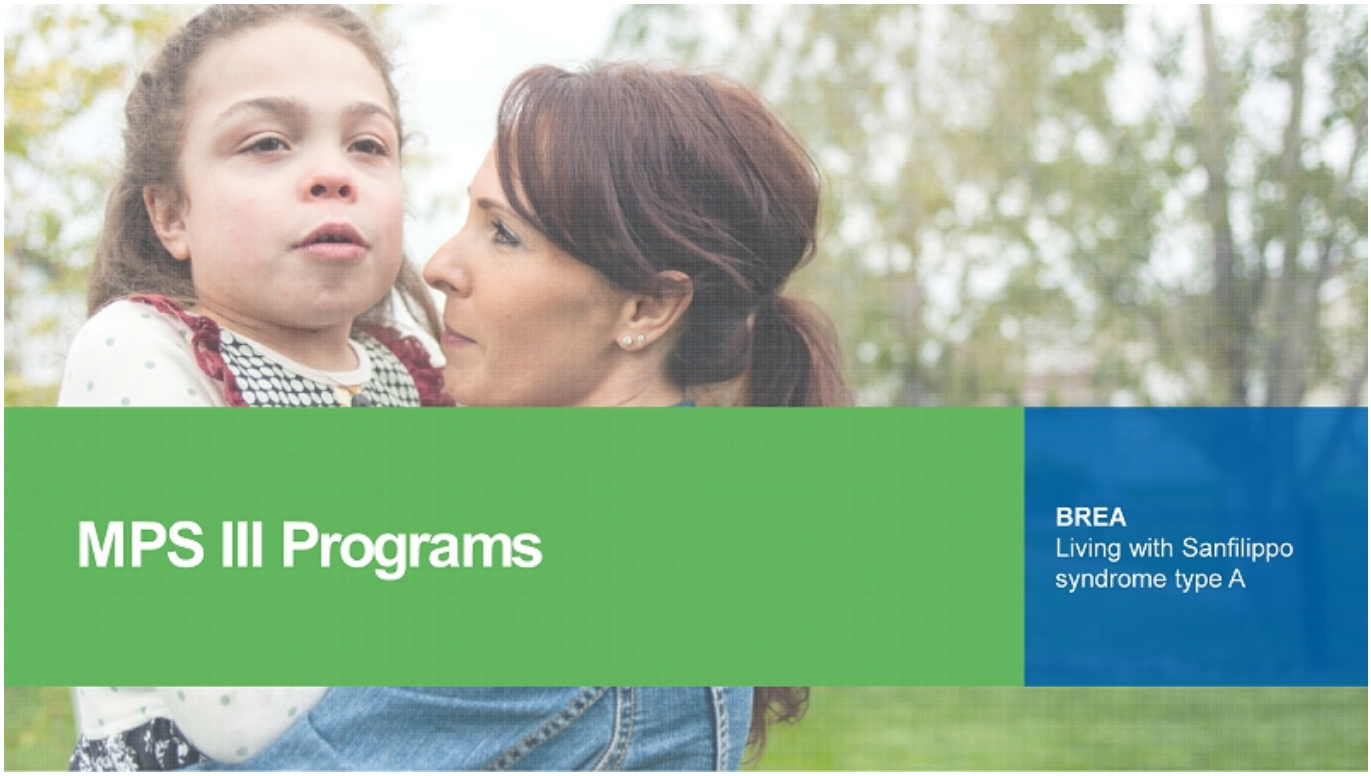
- Majority of translational/early clinical gene therapy research conducted by academic researchers and/or startups spun out of academia
  - Lack of regulatory and product development expertise to map out lifecycle requirements
  - Lack of process/analytical development expertise to develop methods, collect data during preclinical/early clinical phases
  - Lack of funding
- Manufacturing changes are inevitable
  - Process changes and scale-up to support commercial manufacture
  - Change in CMO or transition to in-house manufacture
  - Full GMP compliance
- Comparability demonstration may be complicated by:
  - Limited production data to set acceptance criteria for comparability
  - No/unqualified characterization methods-especially potency to demonstrate product has same quality and safety profile
  - No retained toxicology or Phase 1 lots for head to head testing
  - No stability data to support head to head testing of retains

Gene Therapy Landscape: CMC Opportunities and Challenges

- Paradigm shifts in gene therapy CMC
  - Draft FDA Guidance on Gene Therapy for Rare Diseases (July 2018) recommends advancing CMC development to have more elements in place prior to first-in-human studies
  - Increased funding for early process and analytical method development is required
- Growing pharma involvement and expertise gained by established gene therapy companies will result in more seamless product development from early to commercial stage
  - They have the expertise and funds
  - May raise the bar for the whole field in terms of regulator's expectations
- Development of platform processes and analytical methods, standards will facilitate advancement from early to commercial gene therapy product development
- The filed would benefit from cooperation amongst all players in the field in a non-competitive space
  - FDA Workshop: Quantitation of AAV Based Gene Therapy Products-December 7, 2018 https://www.fda.gov/BiologicsBloodVaccines/NewsEvents/WorkshopsMeetingsConferences/ucm618876.htm
  - Standards Coordination Body- https://www.standardscoordinatingbody.org/

Fireside Chat: Gene Therapy in 2019 – What's Next?

Barry Byrne, M.D., Ph.D.
Tim Miller, Ph.D.



MPS III Programs

BREA
Living with Sanfilippo syndrome type A

Sanfilippo Syndrome (MPS IIIA and IIIB)

Inherited monogenic disorders causing lysosomal enzyme deficiency

- Two most common forms categorized by deficient enzymes:
  - MPS IIIA (*SGSH*), MPS IIIB (*NAGLU*)
- Abnormal accumulation of glycosaminoglycans (GAGs; heparan sulfate (HS))
- Loss of speech/vision, cognitive decline, behavioral abnormalities, seizures, sleep disturbances
- 70% of children with MPS III do not reach age 18 years

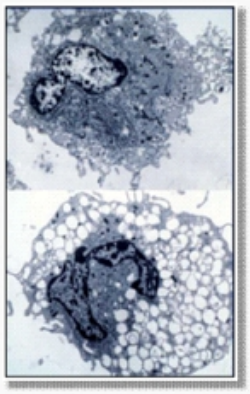
No approved treatments available

Estimated incidence of 1 in 70,000 births

Two ongoing global clinical trials

- ABO-102 (AAV-SGSH) for MPS IIIA: USA, EU, Australia clinical sites
- ABO-101 (AAV-NAGLU) for MPS IIIB: USA and EU clinical sites

Normal cell



Cell with lysosome dysfunction & accumulation of enzyme substrate

Natural History: Comparator control arm to assess treatment effects

Prospective, one-year Natural History study of MPS IIIA and IIIB

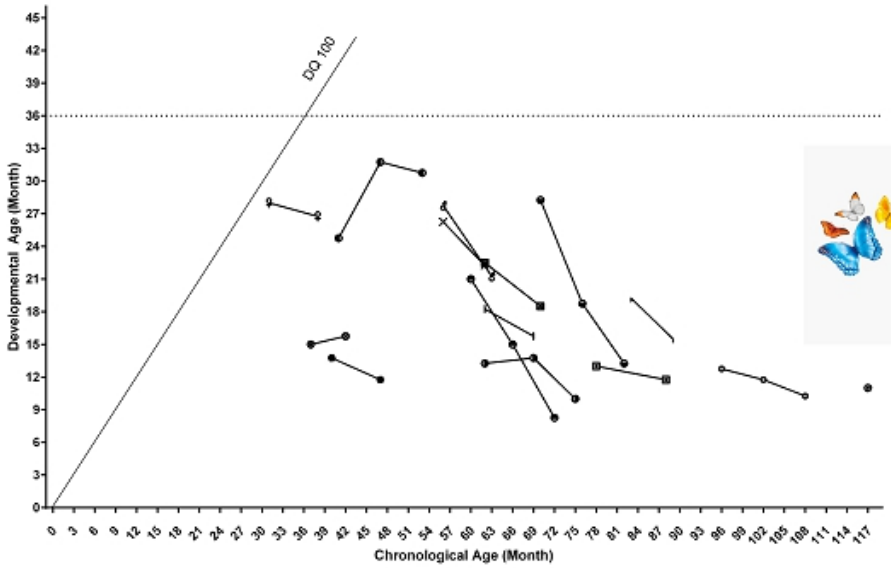
Enrollment complete: 25 subjects, 15 MPS IIIA & 10 MPS IIIB

- Study visits: Baseline, 6 and 12 months
- Neurocognitive and behavioral rating assessments
  - Timed functional motor tests
  - Standard laboratory assessments
  - Serum/leukocyte NAGLU or SGSH activity
  - Quality of life (PedsQL)
- Urinary GAG levels
- Brain, liver and spleen MRI (including DTI and 1H spectroscopy)*
- CSF for standard chemistries/cell counts and NAGLU or SGSH activity*

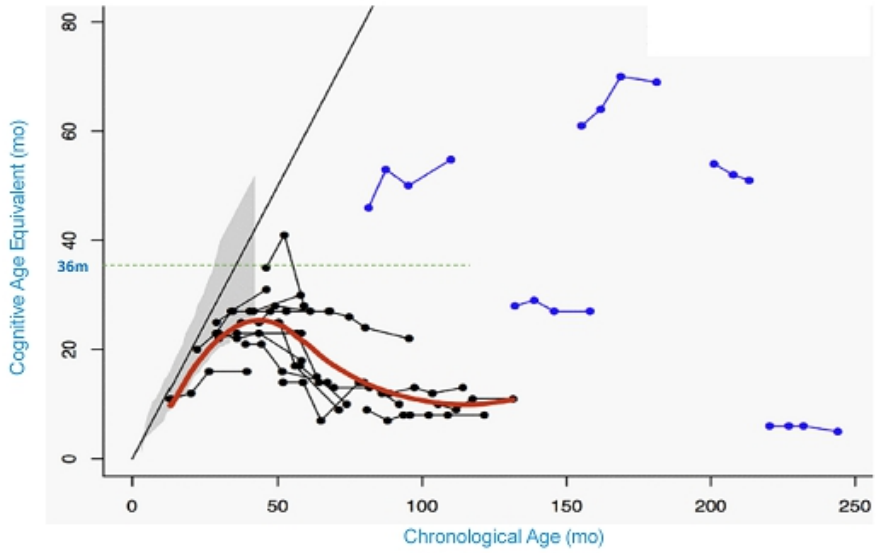
*only at baseline and 12 months

Truxal, K.V. et al. "A Prospective One-Year Natural History Study Of Mucopolysaccharidosis Types IIIA and IIIB: Implications For Clinical Trial Design". Molecular Genetics and Metabolism 119.3 (2016): 239-248.

Mullen Developmental Age: Nationwide Children's Hospital Natural History Study



MPS IIIA Natural History: Cognitive Assessments (N=25)

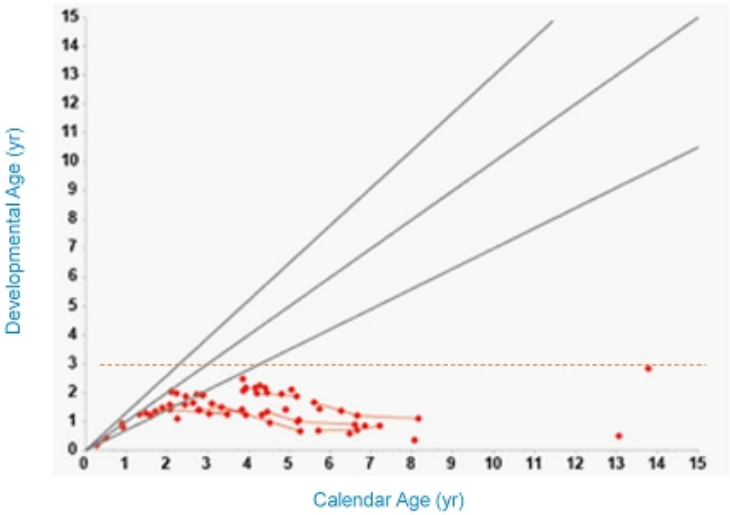


Rapid Progressors
Slow Progressors

UNIVERSITY OF MINNESOTA

Shapiro et al. 2016

MPS IIIA Natural History: Cognitive Developmental Age (N=46)



University of Pittsburgh- Buhrman et al. 2013


ABT-001 Clinical Trial Update

A Phase 1/2 Clinical Trial (AAV9-SGSH) for MPS IIIA

2-Year, Open-label, dose-escalation clinical trial

Intravenous Dosing

- **Cohort 1**: 5 x 10^{12} vg/kg (n=3)
- **Cohort 2**: 1 x 10^{13} vg/kg (n=3)
- **Cohort 3**: 3 x 10^{13} vg/kg (9-12) 8 subjects treated

Primary Endpoint

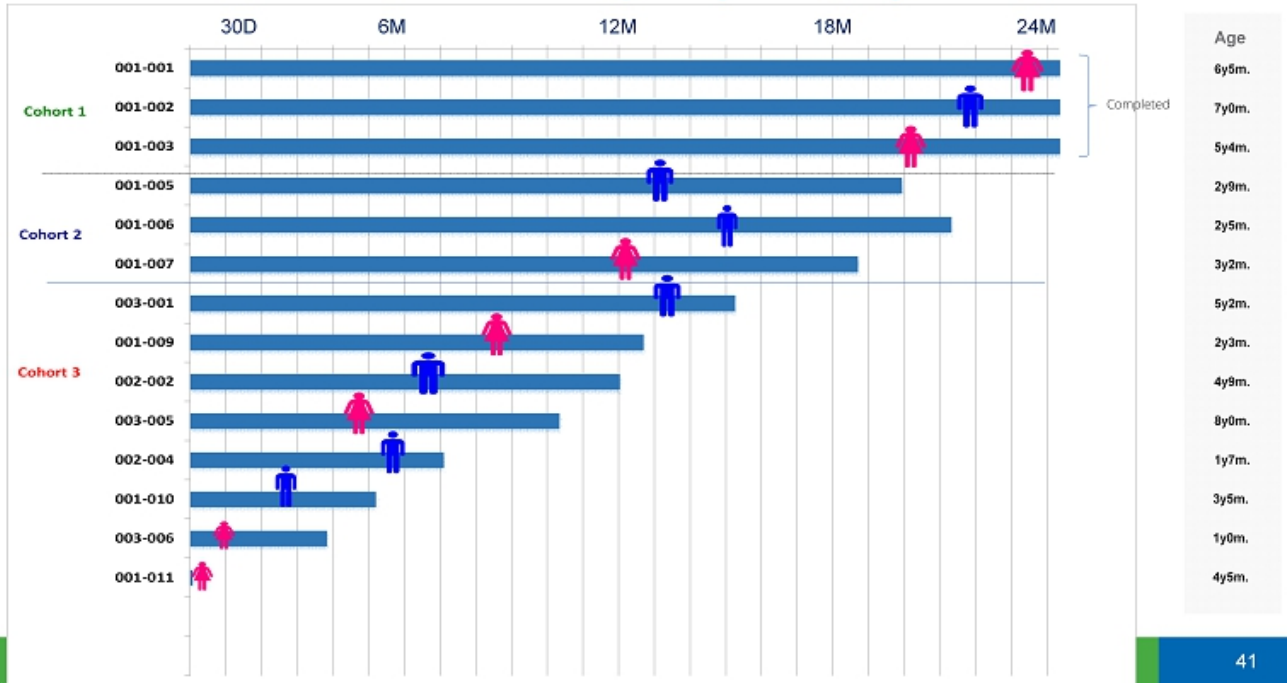
- Safety

Secondary Endpoints

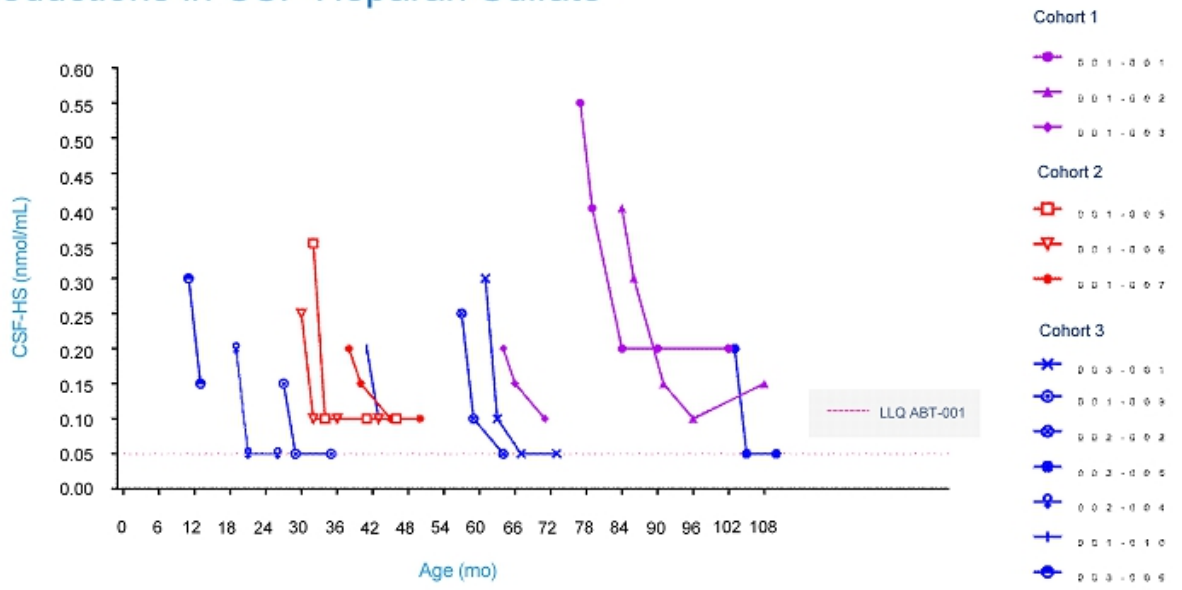
- Cerebrospinal Fluid (CSF) and/or urinary HS and/or GAGs
- CSF and serum SGSH enzyme activity
- Liver, spleen and brain volume by MRI
- Neurocognitive function as measured by Leiter International Performance Scale and the Mullen Scales of Early Learning
- Adaptive functioning, by Vineland Adaptive Behavior Scale (caregiver report)

ClinicalTrials.gov: NCT02716246; Study Sponsor Abeona Therapeutics Inc

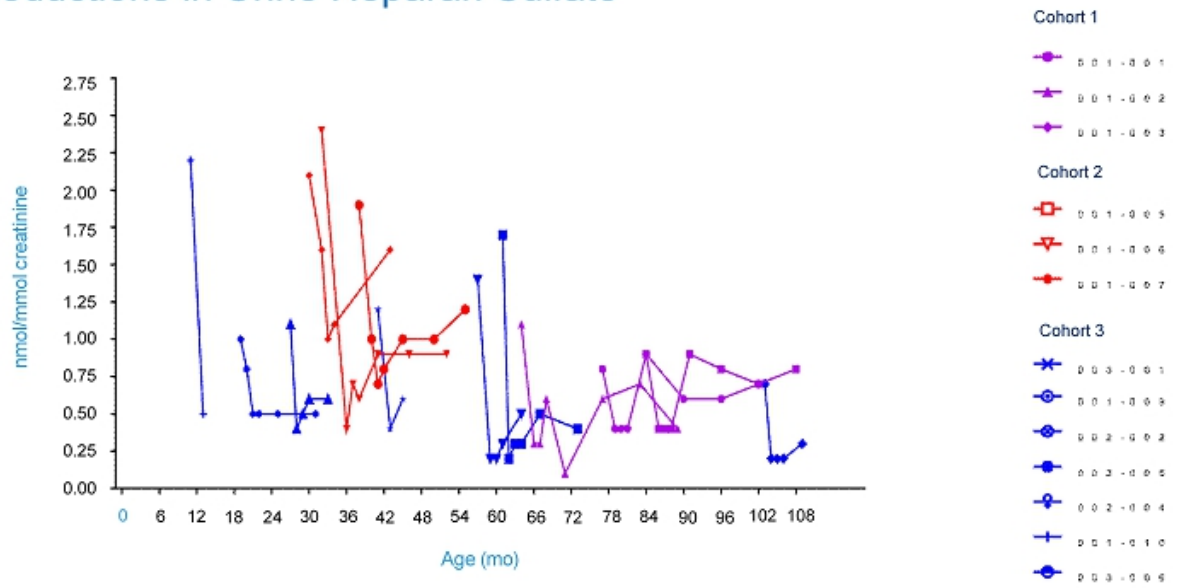
Current Enrollment and Follow up: Days Post-Injection



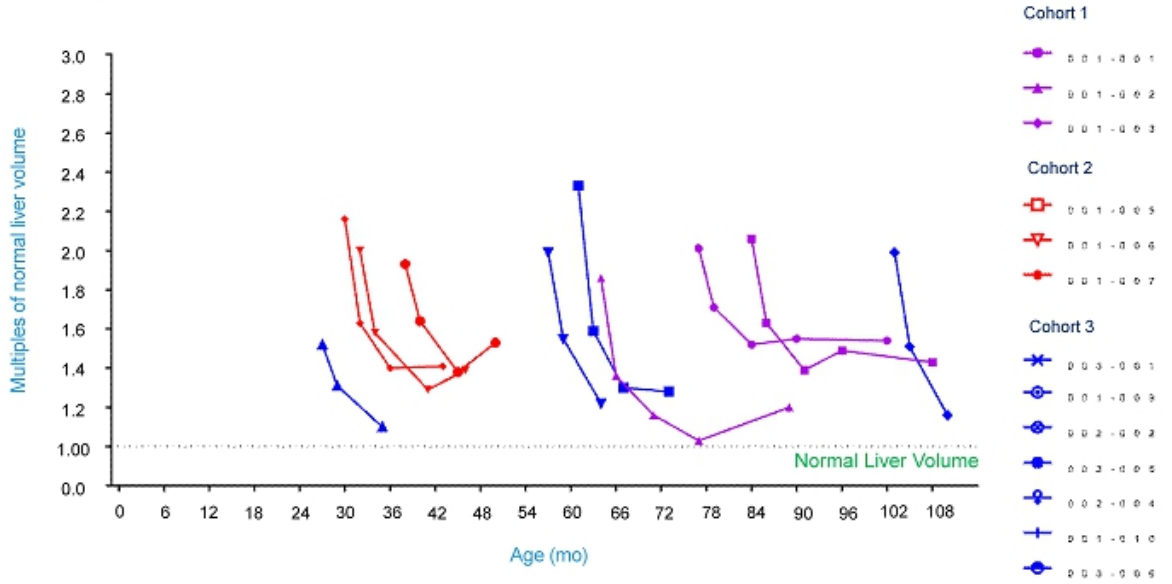
Reductions in CSF Heparan Sulfate



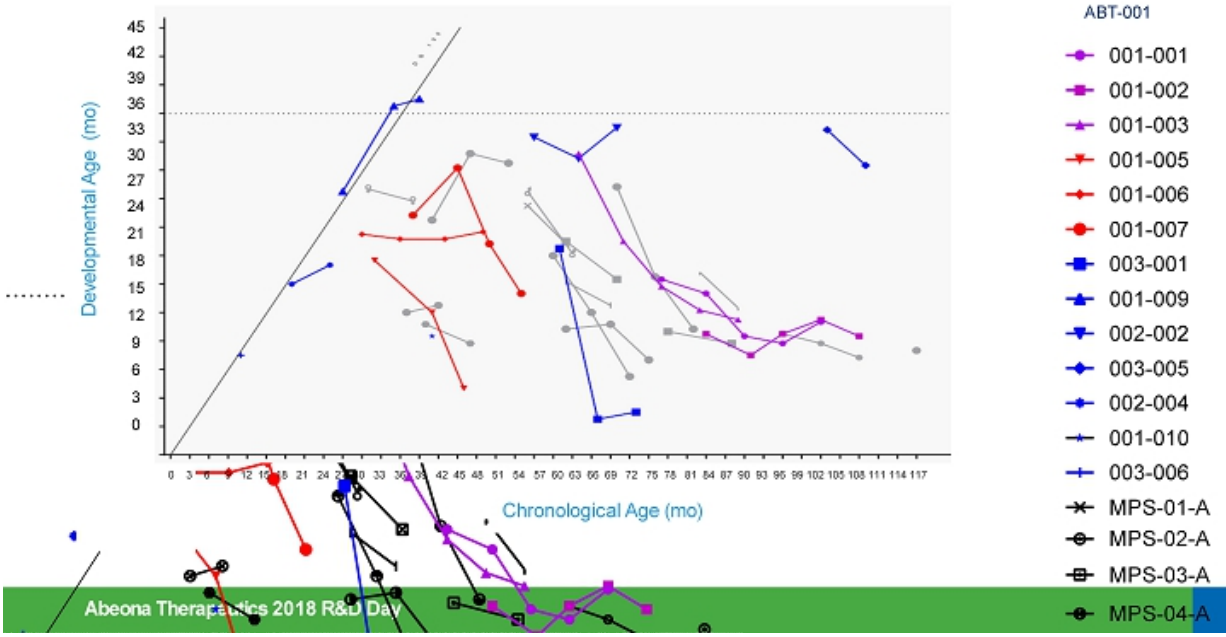
Reductions in Urine Heparan Sulfate



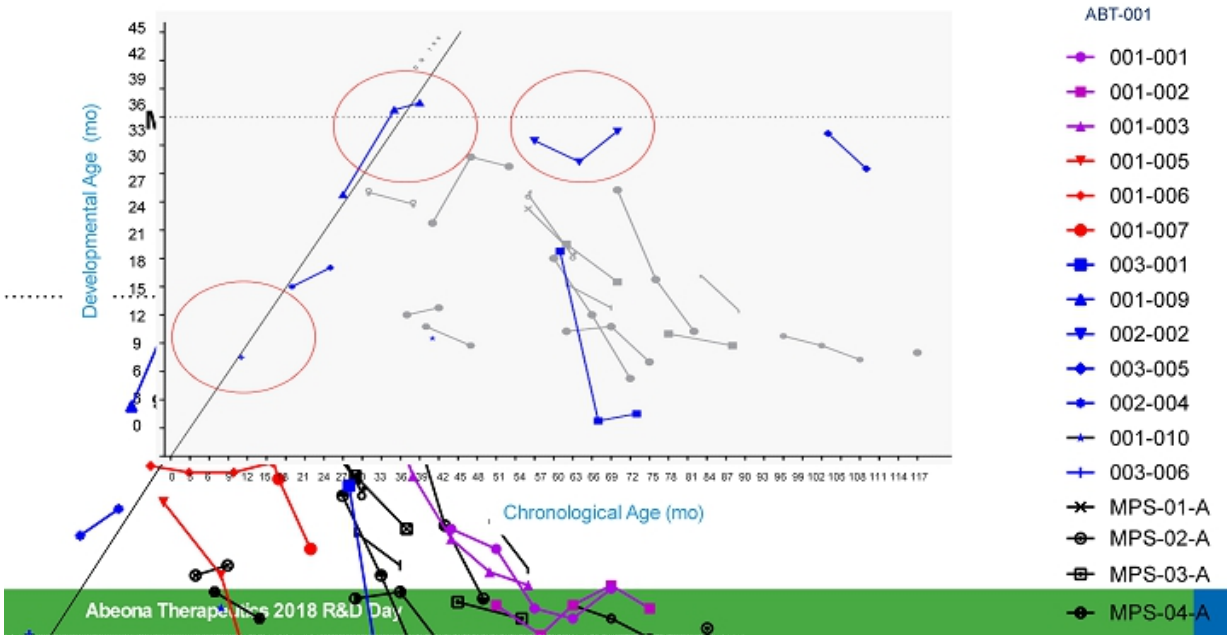
Reductions in Liver Volumes



Mullen Developmental Age: ABT-001 vs. NHS



Mullen Developmental Age: ABT-001 vs. NHS



Global Regulatory Guidance Supportive of Development Program

Clinical

- Acknowledged substantial reduction in disease biomarkers
- Evidence of neurological improvements continued to be demonstrated
- Natural History Comparator Control may be acceptable, which we may expand to increase power comparisons
- Plan to include a few subjects with ABO-102 manufactured using Baculovirus system

CMC/Quality/Nonclinical

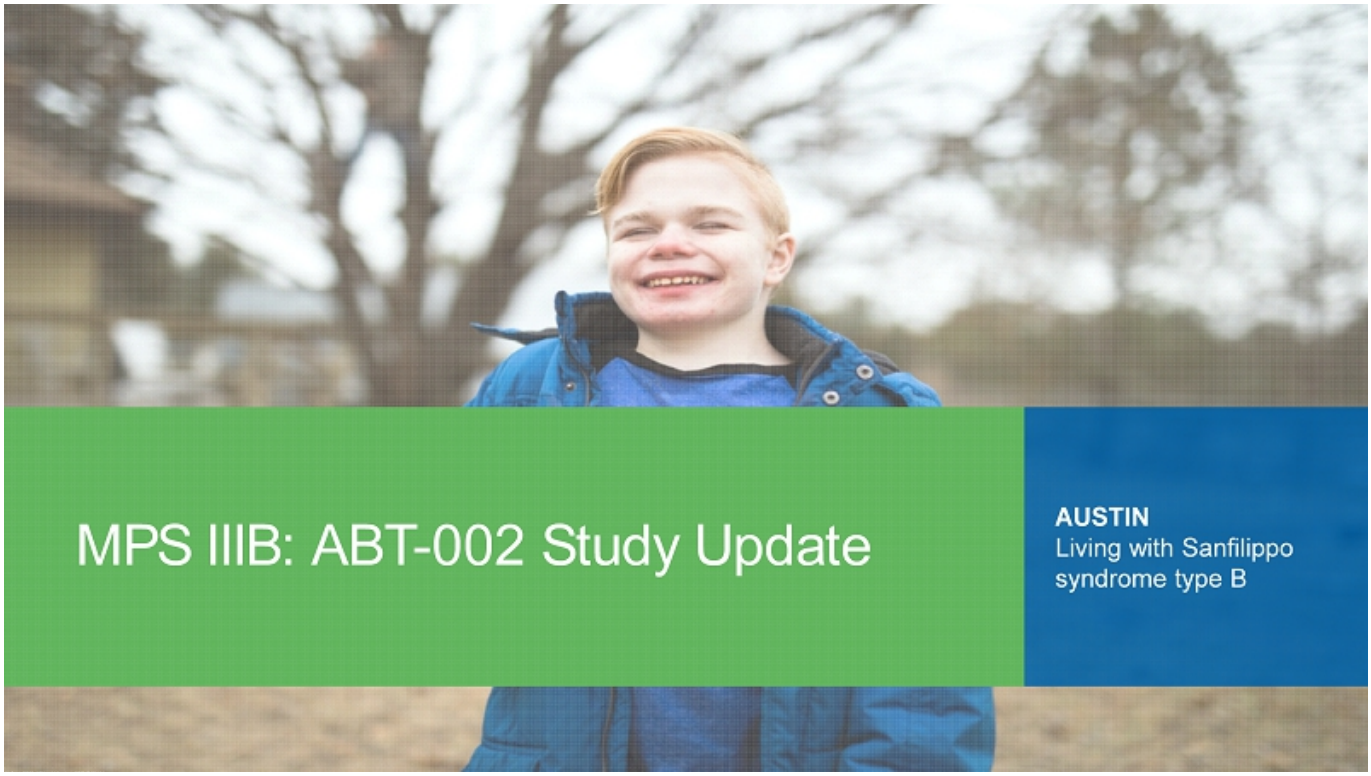
- Transition to Baculovirus-insect cell system expression system is acceptable
  - Demonstrate comparability to the current clinical trial material
- Plan for establishing an in vitro potency assay are acceptable

Summary of MPS IIIA ABO-102 Phase 1/2 Study Data

- N=14 as of November 2018
  - Length of follow up in cohort 3: 6 months (N=6 of 8 total), 12 months (N=3 of 8 total)
- Clear dose-response, and sustained improvement in biomarkers (HS CSF, Liver Volume)
- Encouraging neurocognitive signals seen in younger, higher functioning patients in cohort 3
  - Caregiver observations include:
    - Reports of improved attention, interaction with siblings/schoolmates/environment
    - Improved sleep
    - Improved speech in young patients
      - Enrolled at age 2.3 years: "putting adjectives and adverbs into complete sentences"
- Safety: ABO-102 has been well tolerated to date
  - No serious drug related adverse events (n=14 subjects)
  - SGSH ELISpot negative
  - Length of Follow up as of November 2018:
    - Cohort 1: 27-30 months; Cohort 2: 19-21 months; Cohort 3: 1-16 months

Next Steps

- Amend protocol criteria in ABT-001 Phase 1/2 study to exclusively enroll patients with greater neurological reserve (younger, higher functioning)
- Continued enrollment in the MPS IIIA program
- Older/lower functioning eligible patients may participate in a separate study
- Confer with FDA through RMAT designation as more data become available


MPS IIIB: ABT-002 Study Update
AUSTIN
Living with Sanfilippo syndrome type B

A Phase 1/2 Clinical Trial (AAV-NAGLU) for MPS IIIB

2-year, Open-label, dose-escalation global clinical trial

Intravenous Dosing

- **Cohort 1**: 2 x 10^{13} vg/kg (n=3 subjects) 1 patient treated (4.2 y/o) and 1 patient enrolled
- **Cohort 2**: 5 x 10^{13} vg/kg (n=3-6 subjects planned)

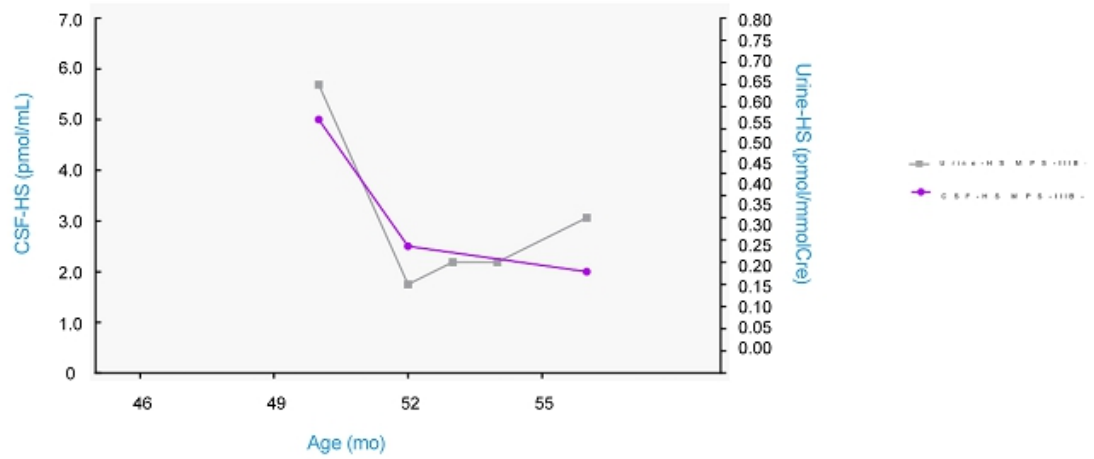
Primary Endpoint

- Safety

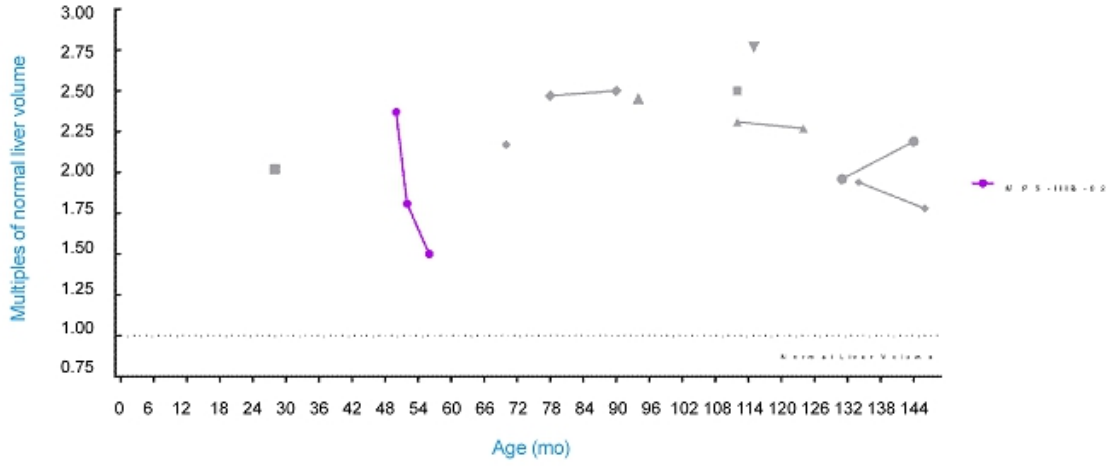
Secondary Endpoints

- CSF and/or urinary HS and/or GAG
- CSF and serum NAGLU enzyme activity levels
- Liver, spleen and brain volume (MRI)
- Neurocognitive function as measured by Leiter International Performance Scale and the Mullen Scales of Early Learning
- Adaptive functioning, by Vineland Adaptive Behavior Scale (caregiver report)

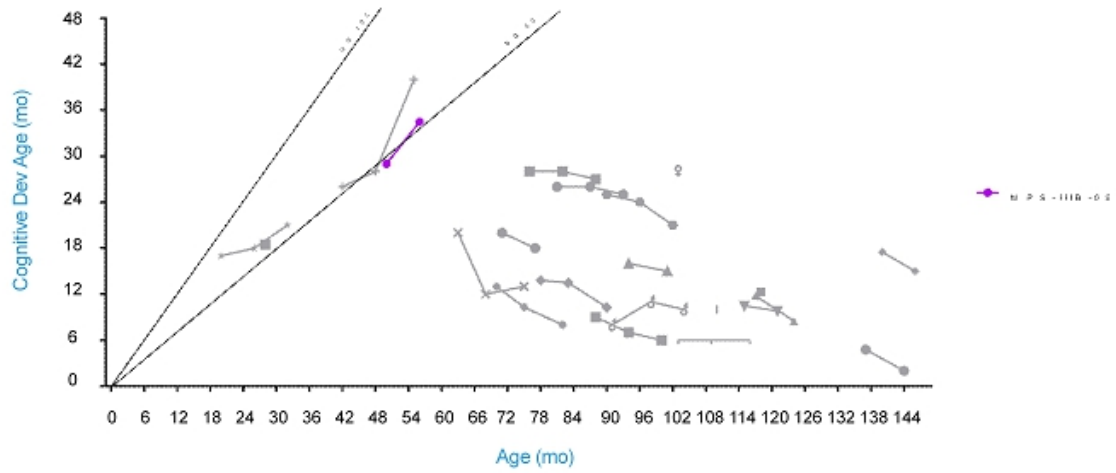
Reductions in CSF and Urine Heparan Sulfate



Reduction in Liver Volume ABT-002 vs. Natural History



Developmental Age vs. Natural History



Truxal et al. 2016; Whitley et al. 2018

MPS IIIB Next Steps

Additional sites are being activated to increase the rate of enrollment

- US, Germany, France, and the U.K.

Pre-screening Protocol

- Will help provide more access to screening for pre-existing antibodies against AAV9


AAV GMP MANUFACTURING

Elisa Linton Center: GMP Manufacturing of AAV Products

- FDA Division of Manufacturing and Product Quality (DMPQ) supports clinical or single use commercial facility
- AAV Processing Facility:
  - Separate Upstream and Downstream Suites
  - Capable of Clinical and Commercial Production
  - Initial Production to support Clinical Needs

Abeona GMP AAV Manufacturing Facility Supportive of Clinical Translation

CellSTACKS
6,320 cm^2

Hyperstacks
18,000 cm^2

- ✓ 46 Staff Members
- ✓ Seasoned Process Experts
- ✓ Strong Management Team
- ✓ Prior Manufacturing Experience
- ✓ Effective Quality System
- ✓ Instrumentation / Equipment
- ✓ FDA Inspection Experience

Eppendorf Bioblu
Scalable to 40L

iCellis Nano
40,000 cm^2


ABO-202
Inspiring Hope for Patients with
Infantile Batten Disease (CLN1)
Leighton
Living with CLN1

ABO-202: scAAV9 for Treatment of Infantile Batten Disease – CLN1

- Infantile Neuronal Ceroid Lipofuscinosis (INCL)
- Exclusive, worldwide license secured for AAV9 for treatment of CLN1
- Severe neurodegenerative lysosomal storage disease, currently with no approved treatment
- Caused by mutations in the CLN1 gene, encoding the soluble lysosomal enzyme palmitoyl-protein thioesterase-1 (PPT1)
- Onset of symptoms between 6 to 24 months of age
  - Progressive visual failure, cognitive decline and loss of fine and gross motor skills develop by 2-3 years of age
  - By 5 years of age there is loss of light perception, complete loss of motor skills and social interaction; myoclonus and seizures can appear eventually
  - Death usually occurs by 7 years of age
- Therapeutic approach is a scAAV9 vector with codon-optimized CLN1 transgene

UT Southwestern Medical Center

THE UNIVERSITY of NORTH CAROLINA at CHAPEL HILL

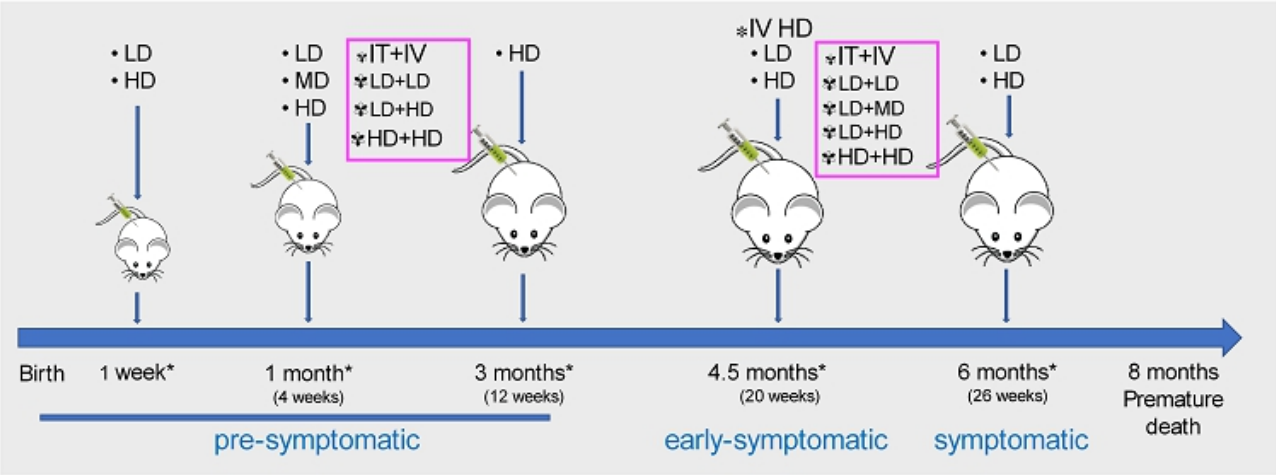
Therapeutic Approach: Combination intravenous & intrathecal dosing

- Demonstrated efficacy in CLN1 mice support rationale for clinical dosing by both intrathecal (IT) and intravenous (IV) simultaneously
  - IT: Intrathecal (lumbar puncture)
  - IV: Intravenous (tail vein)

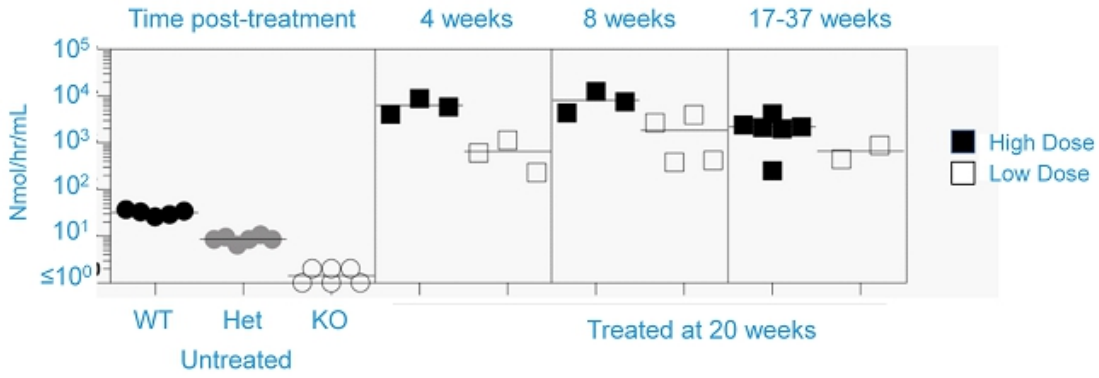
Steven Gray, Ph.D. - Batten Researcher

- Potential Benefits of IT/IV dosing include:
  - Combination dosing overcomes IT dose limitations due to volume constraints
  - "Double exposure" to the CNS for broad and distributed coverage
  - Treatment with higher overall dose may provide greater therapeutic efficacy, especially in older patients
  - Combination dosing with both a high intrathecal and high intravenous dose shows significant efficacy in CLN1 mice

ABO-202: IND-Enabling Efficacy Study Designs

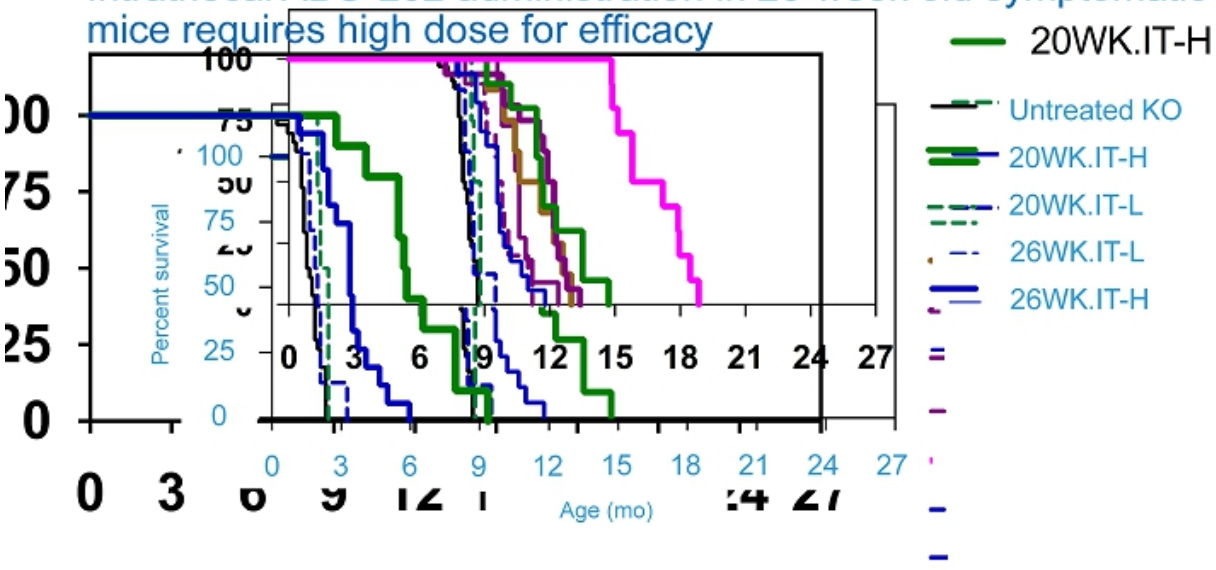


ABO-202 intrathecal delivery results in supraphysiological PPT1 activity levels in serum CLN1 mice

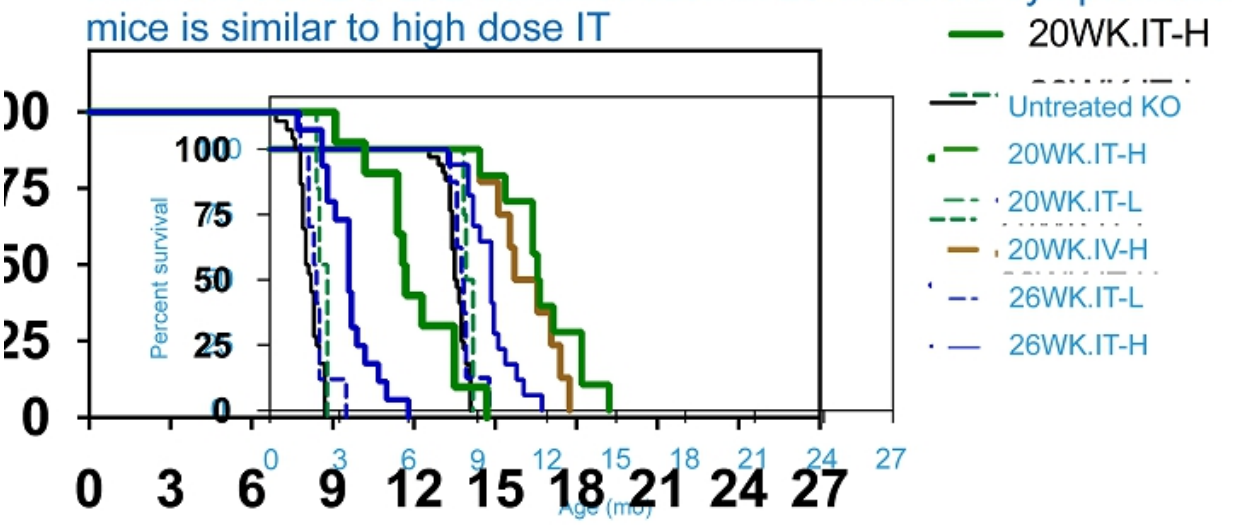


Sustained supraphysiological levels of active PPT1 in CLN1 treated at 20 weeks of age compared to wild type (wt) or CLN1 heterozygote (Het) and knockout (KO) mice

Intrathecal ABO-202 administration in 20-week old symptomatic mice requires high dose for efficacy

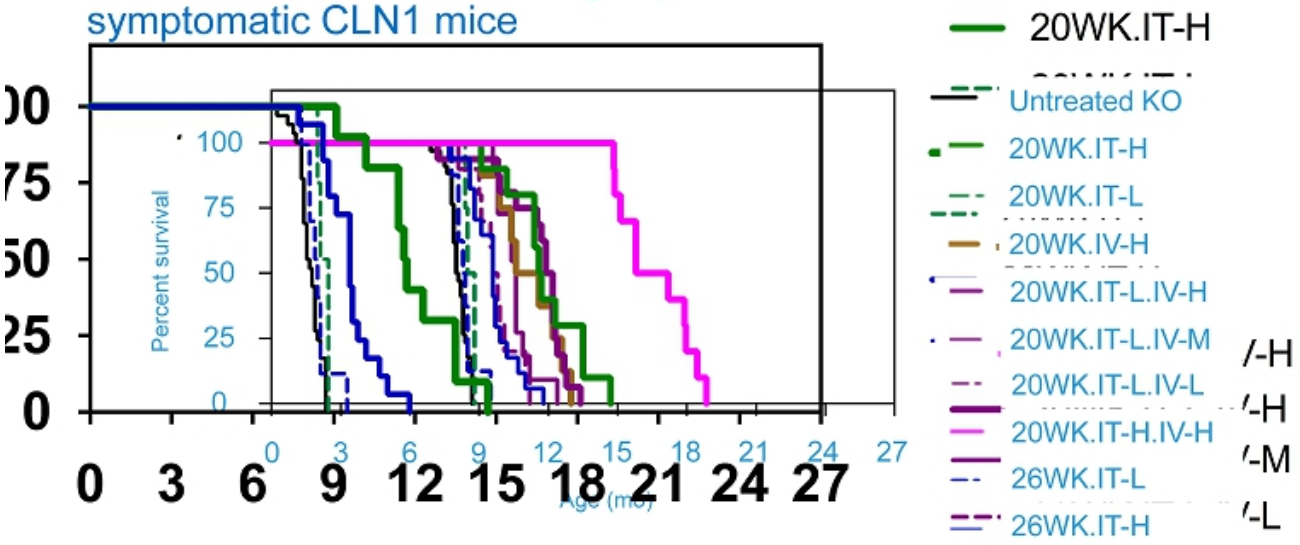


Intravenous ABO-202 administration in 20-week old symptomatic mice is similar to high dose IT

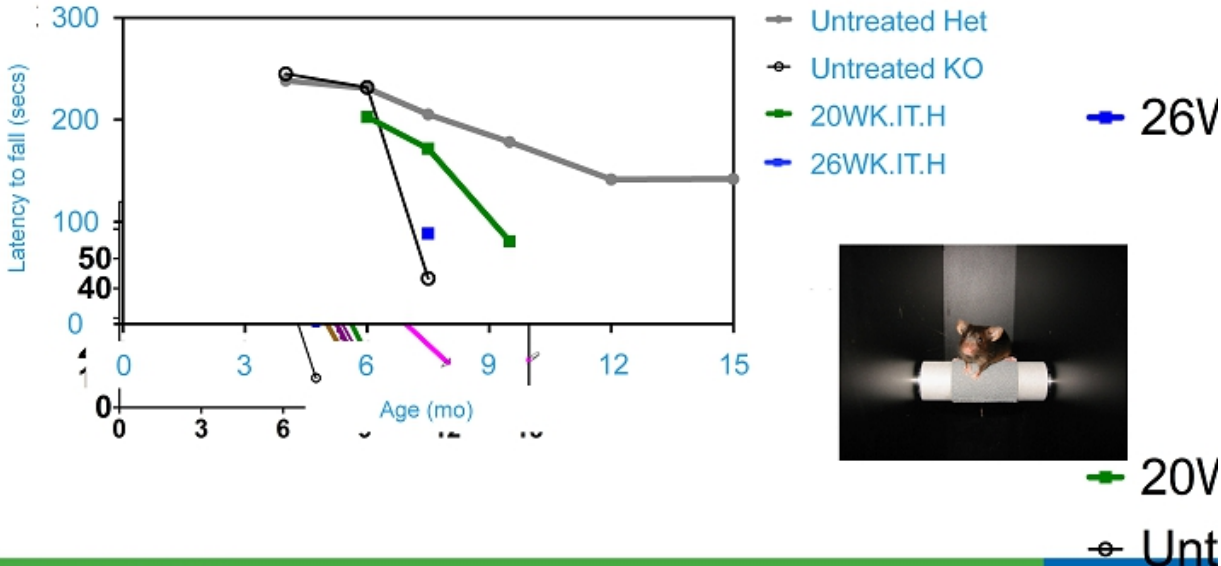


IV and IT results in older animals demonstrate similar survival efficacy

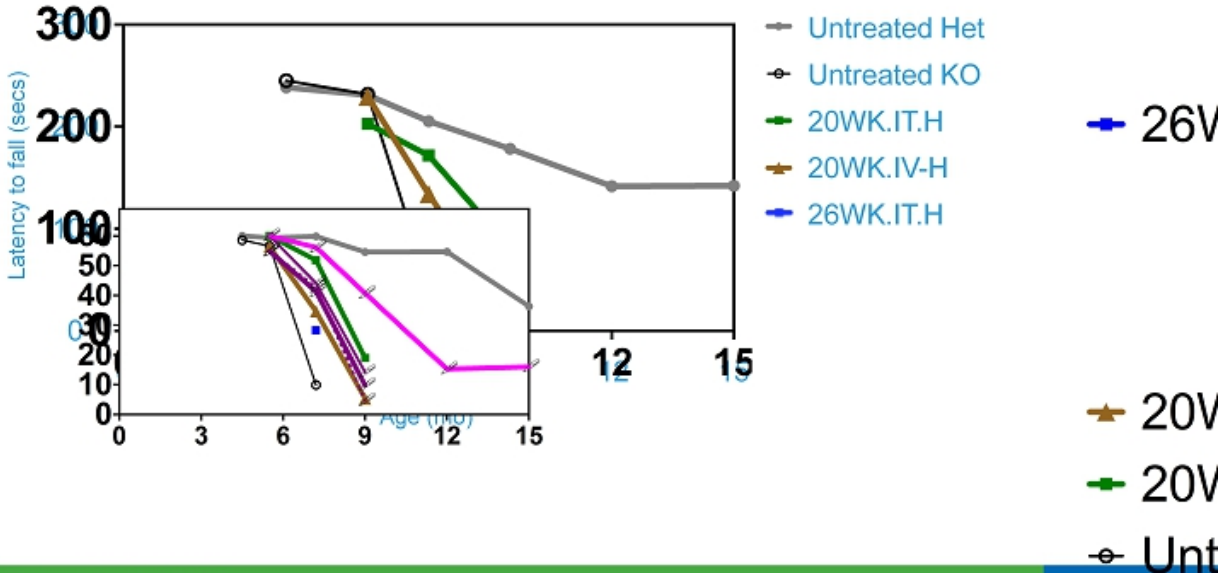
ABO-202 combination dosing improves survival in older symptomatic CLN1 mice



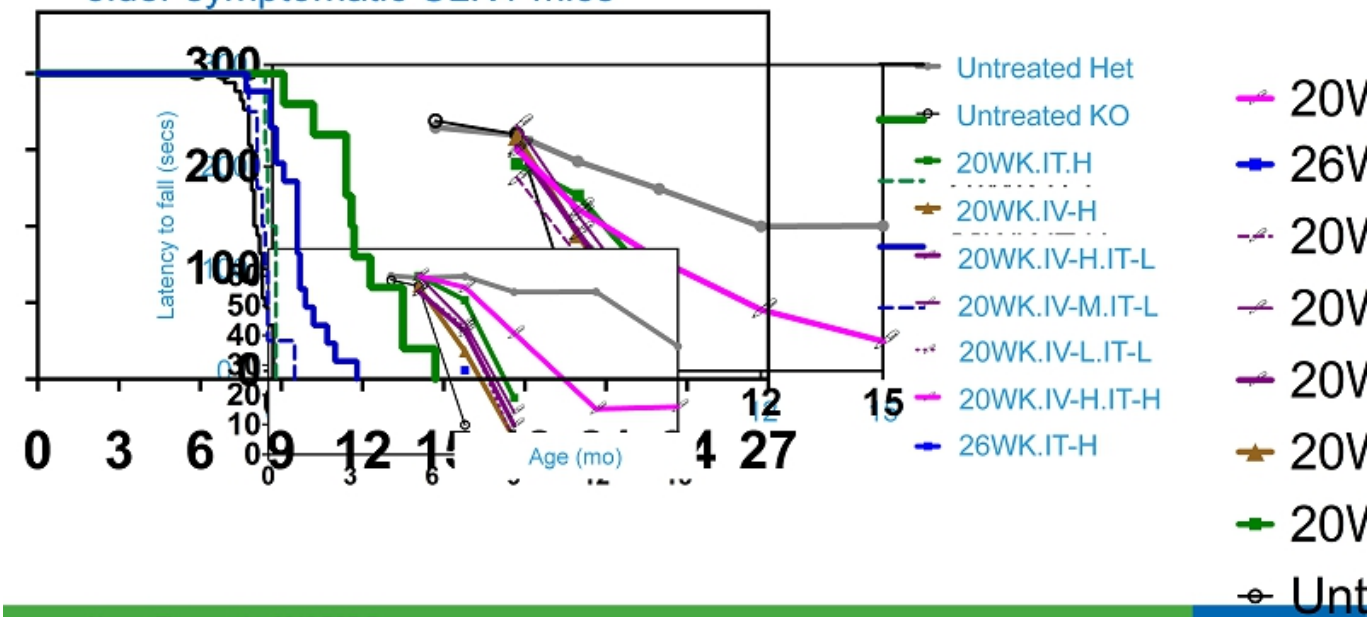
ABO-202 intrathecal administration improves motor function (rotarod)



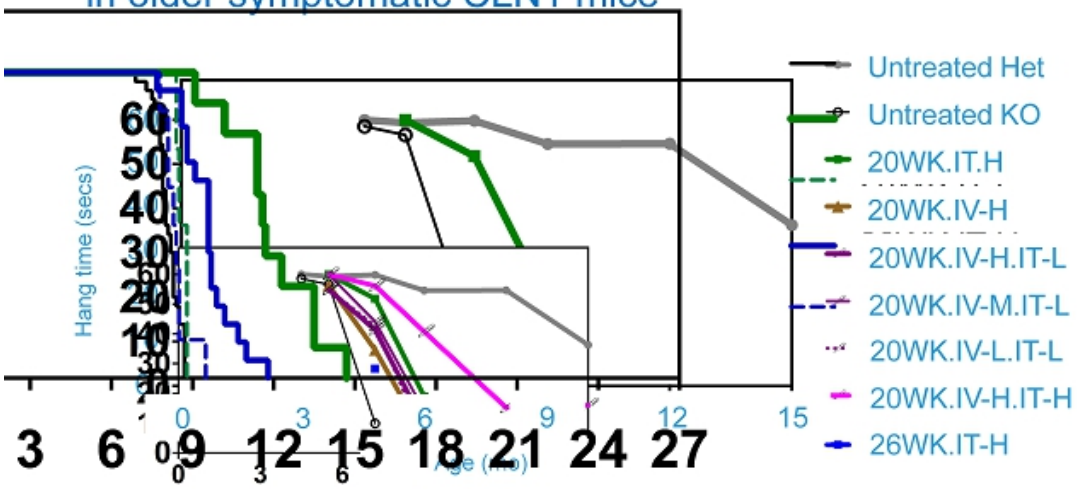
ABO-202 intravenous administration improves motor function (rotarod) similar to intrathecal administration



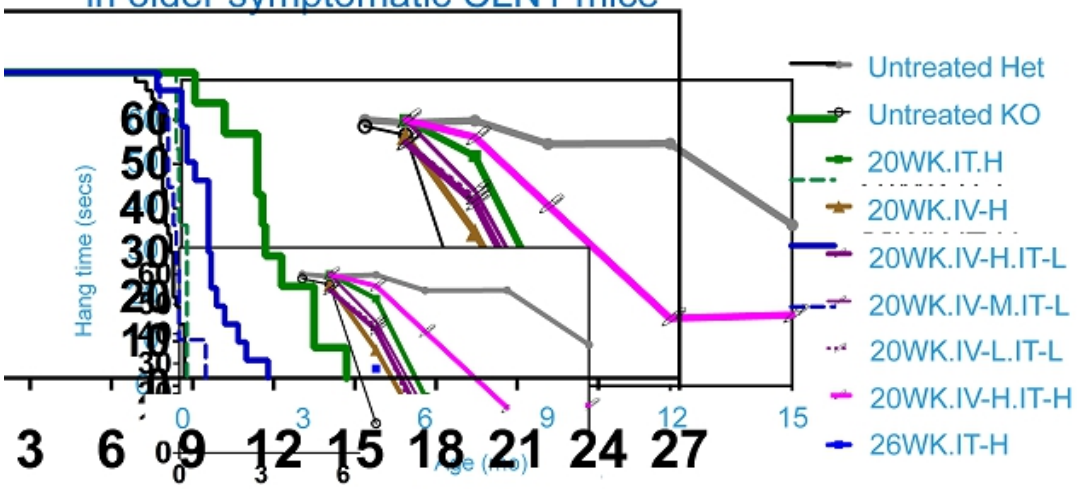
ABO-202 combination dosing improves motor function (rotarod) in older symptomatic CLN1 mice



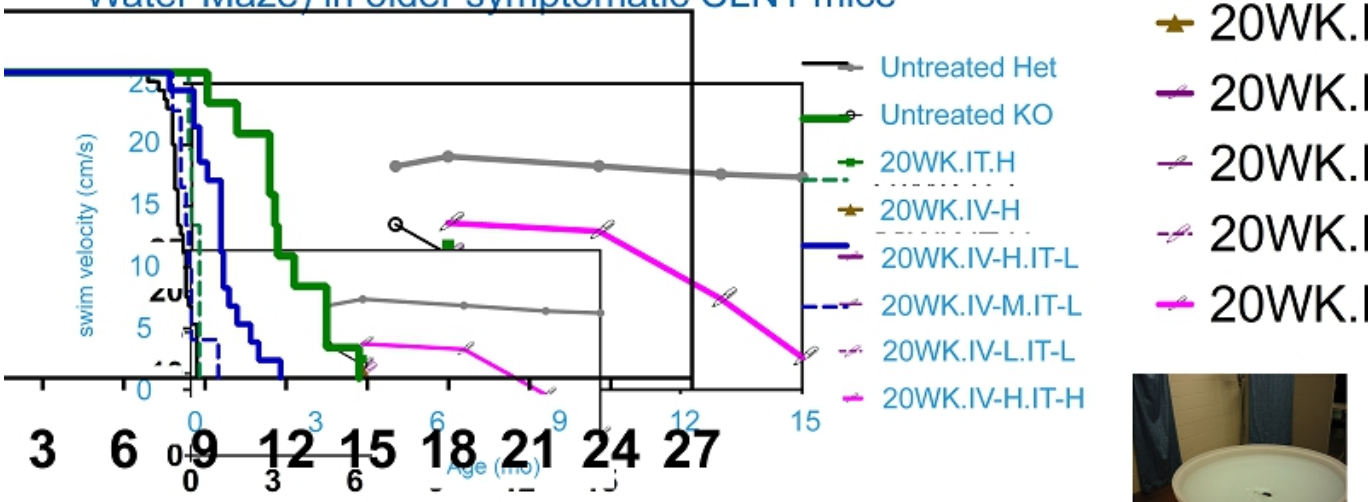
ABO-202 combination dosing improves motor function (wire hang) in older symptomatic CLN1 mice



ABO-202 combination dosing improves motor function (wire hang) in older symptomatic CLN1 mice



ABO-202 combination dosing improves cognition & learning (Morris Water Maze) in older symptomatic CLN1 mice



ABO-202: Phase 1/2 Clinical Trial

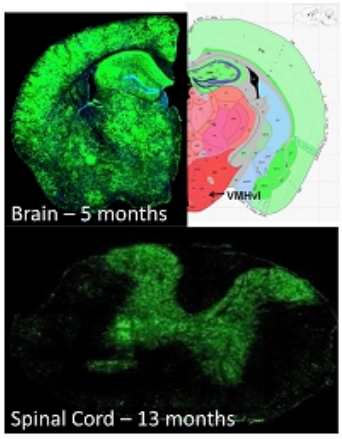
- **IND-enabling toxicology studies completed**
  - Strong safety, with no significant toxicology findings in the combination dose escalation study
- **IND-enabling efficacy studies demonstrate normalized survival, muscle function and cognition**
  - Combination Dosing of intravenous and intrathecal administrations enhance therapeutic opportunity
- **IND submission anticipated in Q1 2019**
- **Phase 1/2 GMP ABO-202 manufacturing completed**
- Trial outcomes supported by large, multi-year Natural History Study
- World-leading investigators and clinical sites for CLN1:
  - University of Rochester (Jonathon Mink & Erika Augustine)
  - University of Hamburg (Angela Schultz)

Building New Futures for Patients with Juvenile Batten Disease (CLN3)

Theo
Living with CLN3

scAAV9 for the treatment of Juvenile Batten Disease (CLN3)

- Juvenile Neuronal Ceroid Lipofuscinosis (JNCL)
- Exclusive, Worldwide License secured for AAV9 for treatment of CLN3
- Caused by Autosomal recessive (inherited) mutation in the CLN3 gene
  - Initially presents as blindness, progressing to behavioral issues, sleep disturbances, seizures, cognitive loss, motor abnormalities, premature death (late teens-early 20s)
  - Neurodegeneration occurs in thalamus, cortex, and hippocampus, with inclusions observed throughout the CNS
  - Estimated incidence of 1:100,000 births
  - Therapeutic Approach is utilizes a scAAV9 vector



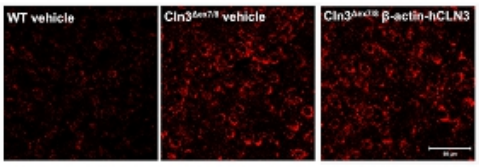
- scAAV9 vector show broad CNS distribution (green color) in CLN3 mouse brain and spine cells
- 5-13 months after intravenous injection in CLN3 Batten mice

Bosch et al 2016, J. Neuroscience, 36

scAAV9 for the treatment of Juvenile Batten Disease (CLN3)

Preclinical summary:

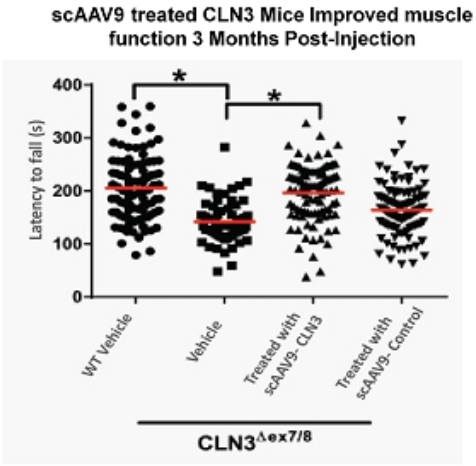
- Multi-year POC studies from Univ. Nebraska Medical Center
- Intravenous delivery of scAAV9 in CLN3$^{\Delta ex7/8}$ mice
  - Normalization of cognitive & muscle function
  - No Safety issues
  - Key finding: AAV9-CBA promoter not effective treatment



- 1 year IND enabling study in CLN3$^{\Delta ex7/8}$ mice – complete
  - No toxicities observed
- Acute non-human primate study – confirms CLN3 expression in target tissue

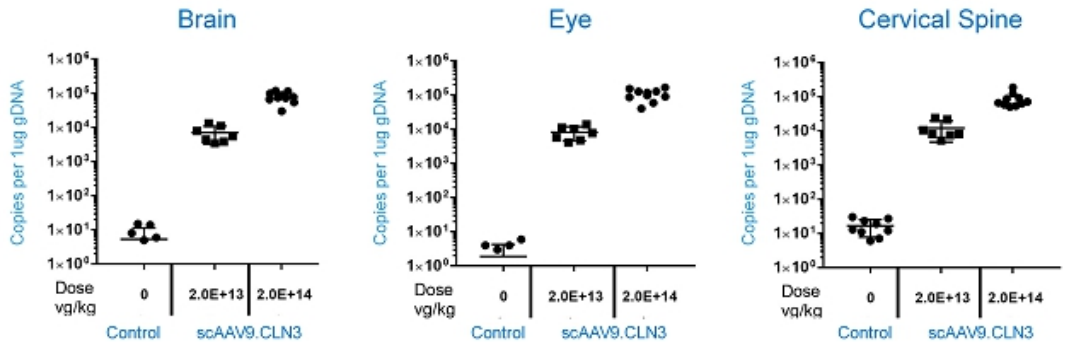
Bosch et al 2016, J. Neuroscience, 36

Proof of concept studies:



Intravenous delivery of scAAV9.CLN3 results in significant, dose-dependent biodistribution into the CNS and peripheral organs in ADULT CLN3 mice

Adult mice assessed 1 month post ABO-202 administration

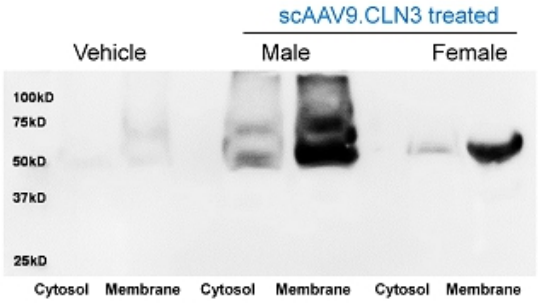


Same trend for: Lumbar Spine, Heart, Lung, Spleen, Kidney

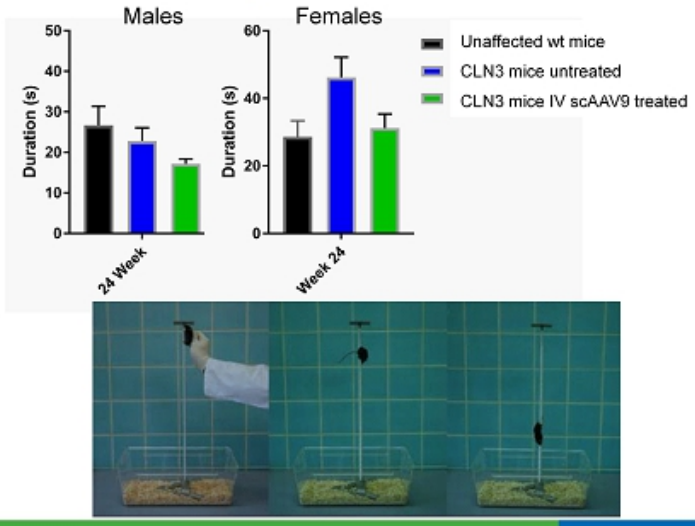
Abeona Unpublished data

Intravenous delivery of scAAV9.CLN3 – biopotency and efficacy

Restored expression in peripheral tissues one month post-injection in CLN3 mice



Improved motor function (pole descent) 6 months post-injection in CLN3 mice



Abeona Unpublished data

2019 Batten Disease Clinical Trials: World Leading NCL Centers and Natural History Experts

Jonathon Mink, MD, Ph.D.
University of Rochester

Erika Augustine, MD
University of Rochester

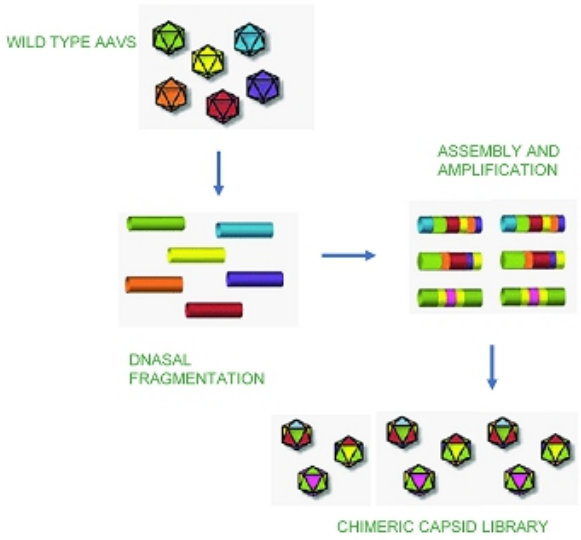
Heather Adams, Ph.D.
University of Rochester

Angela Schultz, MD
University of Hamburg

- Combined >15 years of Batten Disease Natural History Study Principal Investigators
- Subject matter experts on Batten disease manifestations, neurological assessments and clinical trials
- Clinical trial investigators for multiple neurological and lysosomal storage diseases


Next Generation AIM™ AAV
Platform

AIM™: Next Generation AAV Vector Platform



AIM™ Vector Platform

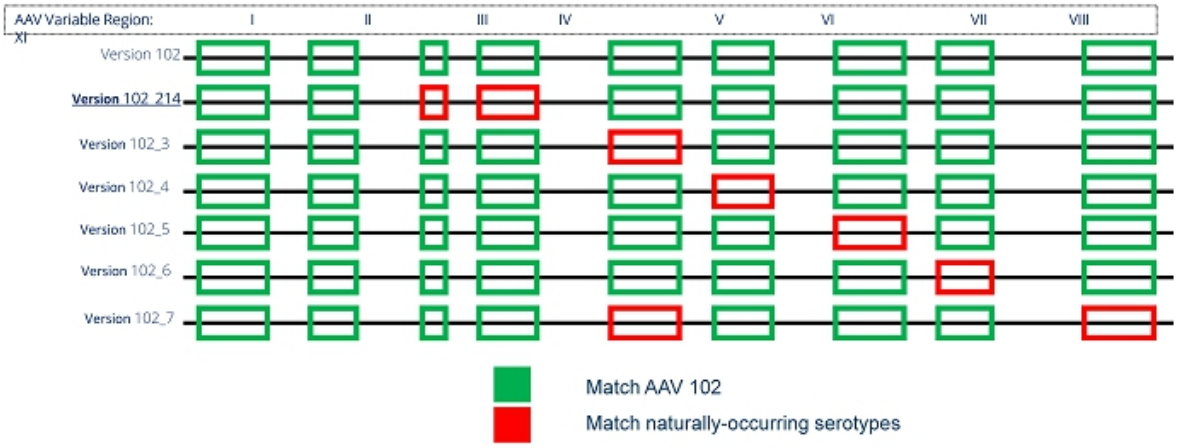
- AAV viral vector platform selected to target CNS, lung, skin, muscle, liver and other tissues
- Initial library of 64 first generation novel AAV Capsids

Key Advantages of AIM™

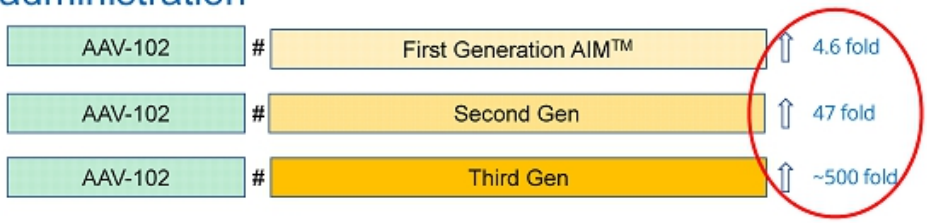
- First generation demonstrated increased gene delivery efficiency to specific tissues
- Second and third generations have increased tissue tropisms
  - Over 100 capsids under evaluation
- Potential for redosing previously treated AAV subjects

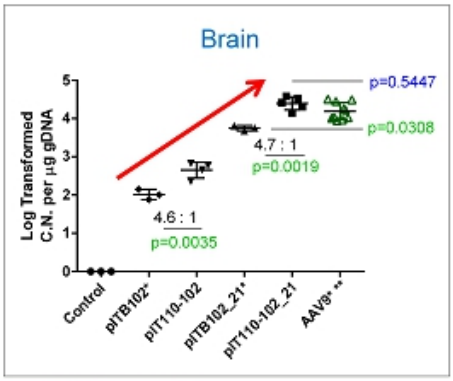
Improving Second and Third Generation of AIM™ AAV Capsids

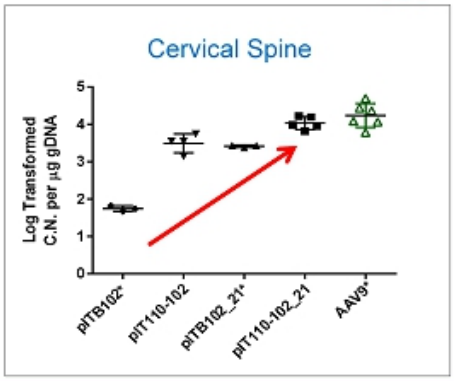
Capsid 102 Rational Design Evaluation



Case Study: AIM™ Capsid enhanced tissue tropism for AAV after intravenous administration

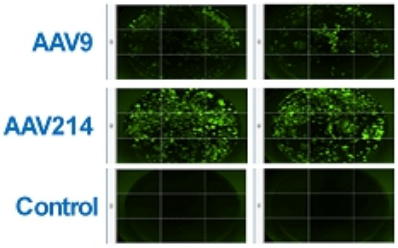




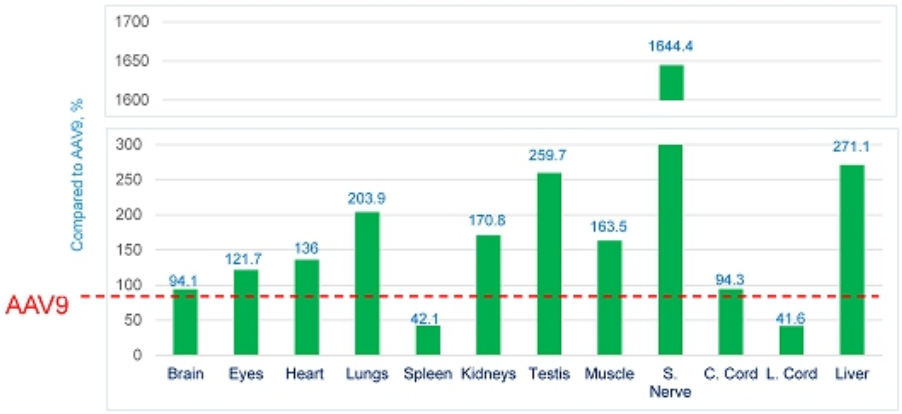


Case Study: Third generation intravenous delivery of AAV214 crosses blood-brain-barrier in mice

Tools for comparing tropisms

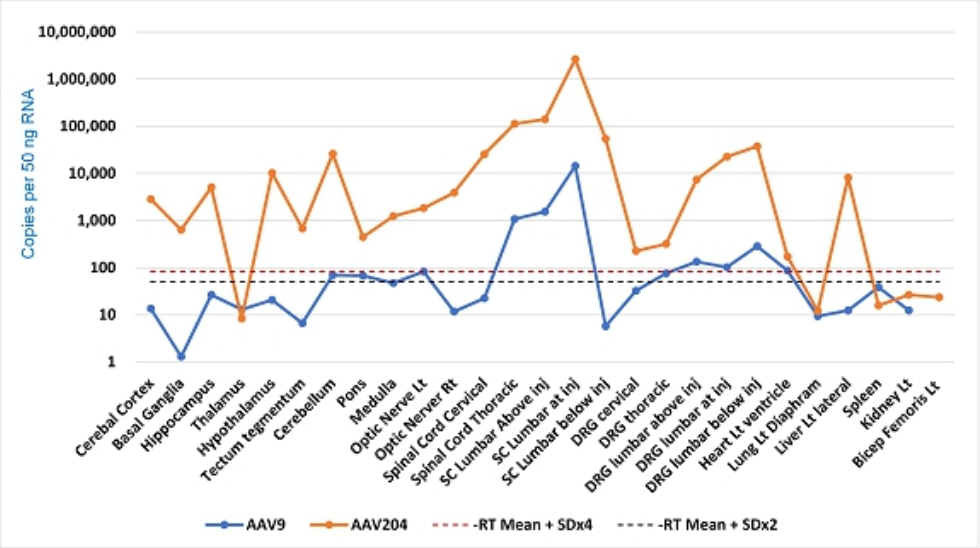


IN VITRO POTENCY ASSAY



IN *VIVO* IV DELIVERY IN MICE

Intrathecal delivery of AAV204 into Non-Human Primates: Penetrating the CNS

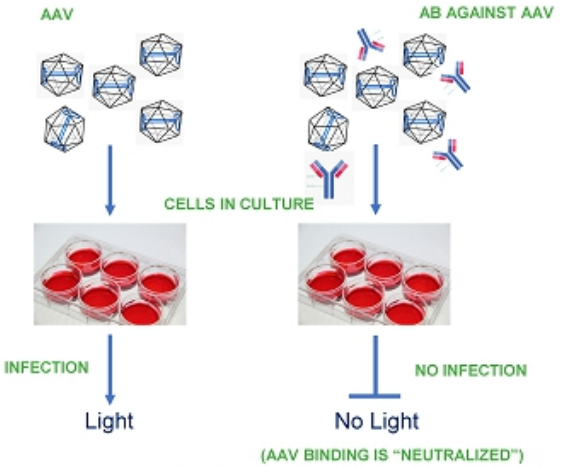


- Intrathecal delivery
- 30 Days post dosing
- Same promoter
- 10-100 fold increased delivery & expression over AAV9

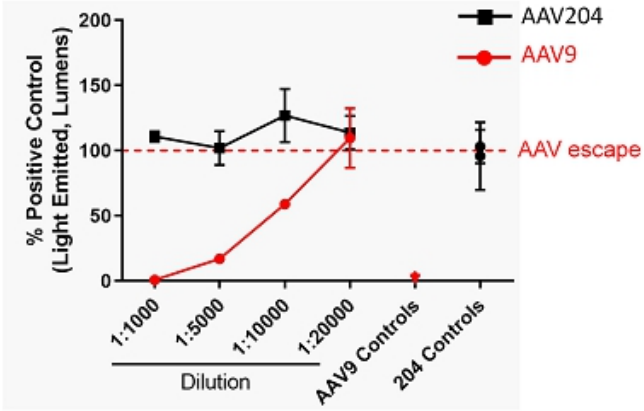
AAV204 Escapes AAV9 Neutralizing Antibodies

Neutralizing Antibody Assay:

- AAV9 or AAV204 mixed with serum from AAV9 treated subject
- Added onto cells in culture (MOI 2.5E5)
- Readout of light emitted from the cells



- Serum from AAV9 treated subject 60 Days Post-Treatment
- Total antibodies against AAV9 tittered @ > 1:1E6 by ELISA



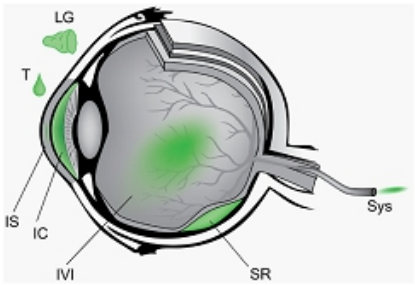
Controls = anti-AAV9 antibodies from commercial source added to virus preps at 5 or 0.5 ug (expected result for AAV9 is no signal)

AIM™ AAV-204: A Tool for Delivering Genes that Target Eye Disorders

- Multiple Routes of Administration
- ABO-50X Product Pipeline: Studies in Non-Human Primates

AIM™: Expanding the Gene Therapy Toolbox for Eye Disorders

- **The Eye is immune privileged tissue**
- **Very small amounts of virus necessary for therapeutic benefit**
  - 100-200 uL @ 1E+12 vg/mL per eye (total dose of 1E+10 vg/eye) in humans
  - 1 uL @ 5E+12 vg/mL per eye (total dose of 5E+9 vg/eye) in mice
- **Non-invasive *in vivo* imaging to monitor efficacy/safety in animals**
  - Scanning laser ophthalmoscopy (SLO)
  - Optical coherence tomography (OCT)
  - Multi-photon microscopy
  - Fluorescein angiography
- **Well characterized cell-specific promoters**
- **Multiple unmet clinical need of inherited retinal dystrophies**
  - Primarily with recessive inheritance pattern
- **Current method of administration (sub-retinal) is surgery intensive**
- **Preferred route of administration = intravitreal**



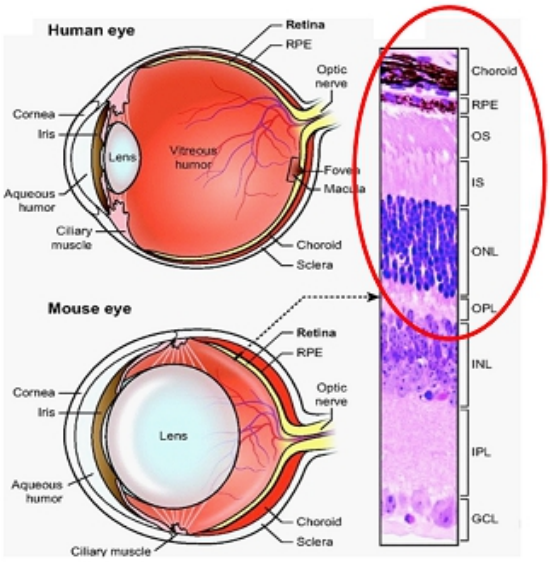
Approaches for eye treatment:
LG, lacrimal gland; T, topical eye drop; IS, intra stromal of cornea; IC, intra-cameral, i.e., anterior chamber; IVI, intravitreal; SR, sub-retinal; Sys, systemic.

Ref: Khabou et al.2016 : Mechanisms of Enhanced Transduction by AAV2-7m8, Willett, Front. Immunol.,2013

80% of Inherited Retinal Dystrophies Affect Photoreceptors & Retinal Pigmented Epithelial Layers

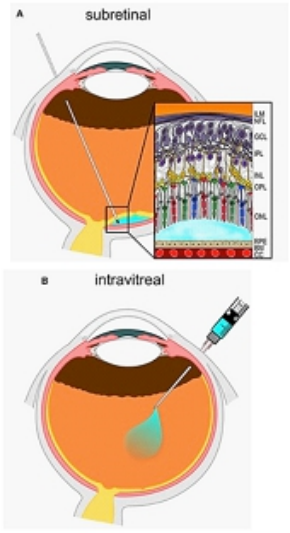
Diseases affecting photoreceptor and RPE cells

- RP (rec., dom. and X-linked)
- BEST
- LCA
- CRD
- Stargardt's
- Choroideremia
- Usher Syndrome
- Achromatopsia

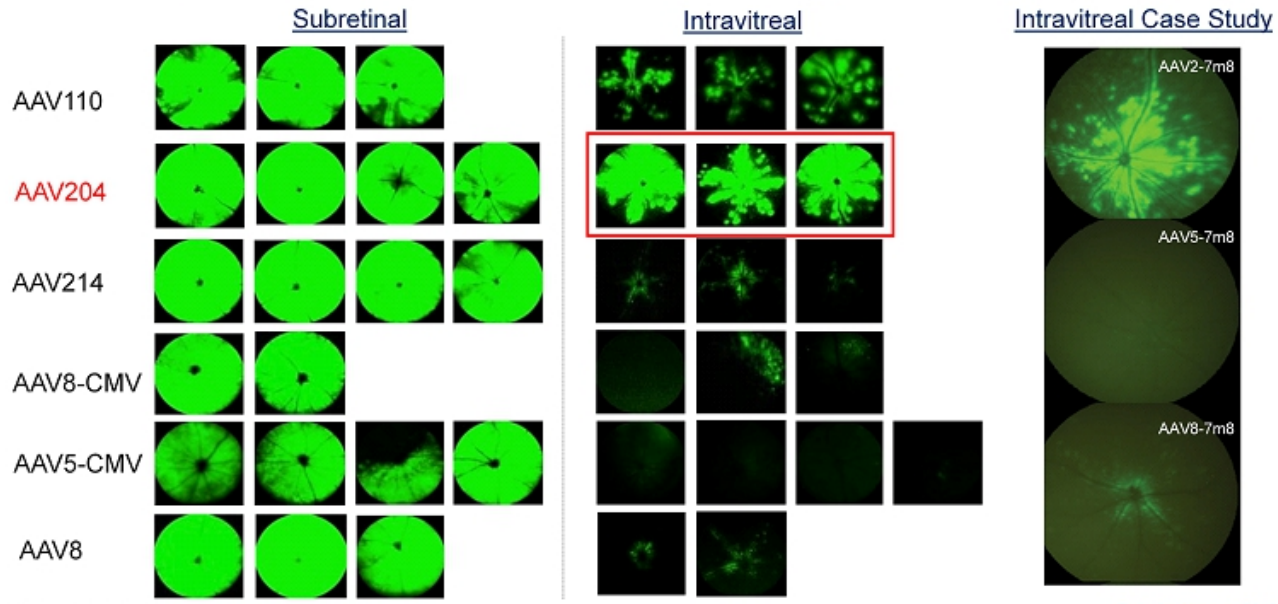


AIM™ for the Eye:
Enabling Gene Therapy Delivery for Ophthalmology Diseases

	Subretinal	Intravitreal
Pros	Direct contact between vector and target cells	Injection can be performed at any out-patient eye clinic
	FDA-approved route of AAV administration (Luxturna)	Administration does not cause retinal detachment
Cons	Injection causes detachment of retina with underlying RPE	Vector must traverse vitreous humor and inner retina to reach target cells
	Detachment of macula may cause permanent damage	Few vectors have been identified which can reach the photoreceptors and RPE
	Requires OR and trained retina surgeon for administration	



AIM™ Capsids Demonstrate Enhanced Delivery to the Eye

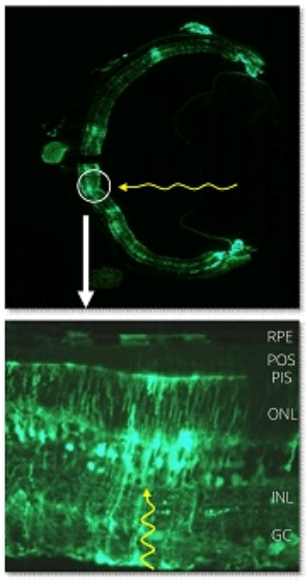


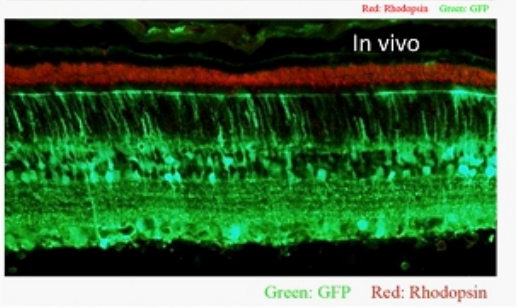
10 Days post-injection in mice

Ref: Khabou et al.2016

Intravitreal Administration of AAV204 to Non-Human Primate (NHP) Eye

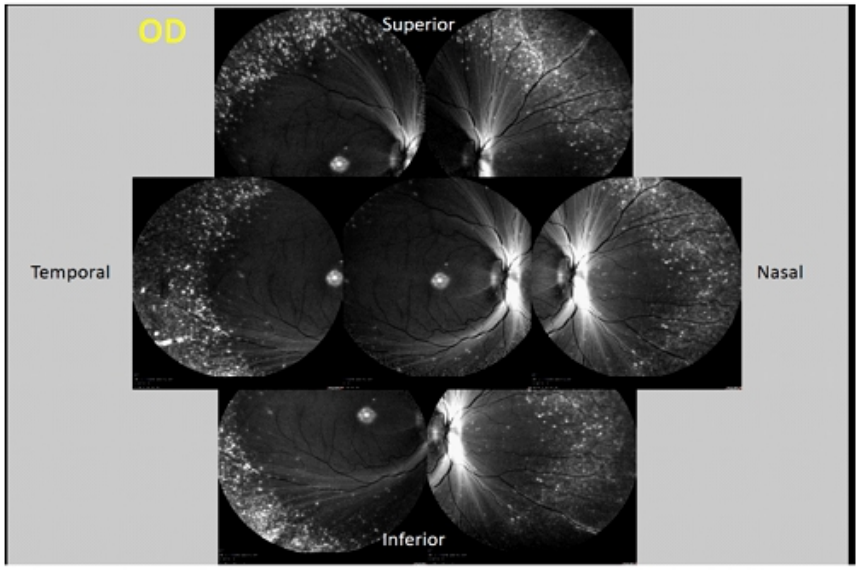
Widespread Retinal Transduction





- TIME: 6 WEEKS POST INJECTION

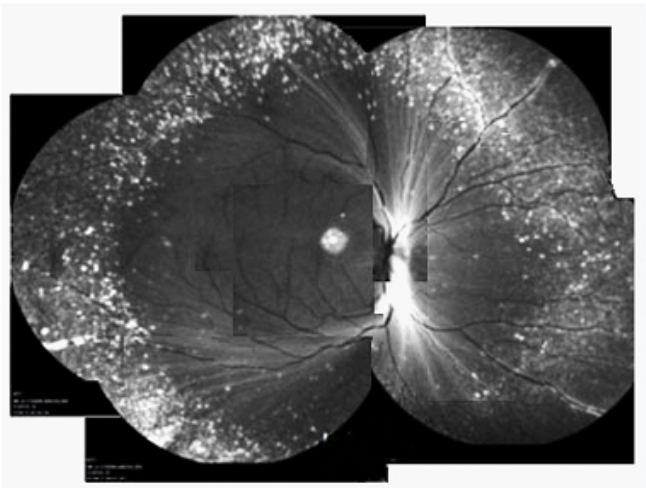
AAV204-GFP NHP Intravitreal Administration Demonstrates Significant Distribution and Expression – Live Eye Imaging

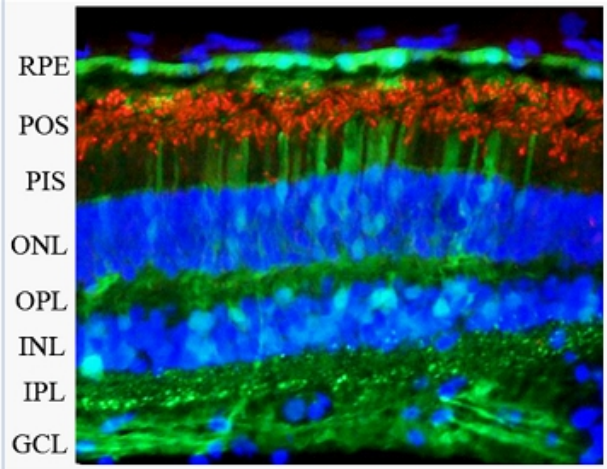


Abeona NHP study comparisons:

- Administered 1.5E12 vg
- Assessed at 6 weeks
- Assessed 50% earlier and at 30% of dose compared to others

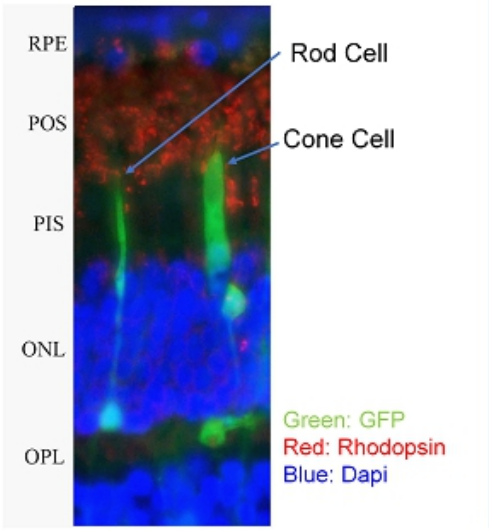
Intravitreal Administration of AAV204 Results in Significant Transduction in the Peripheral Retina and Foveal Region





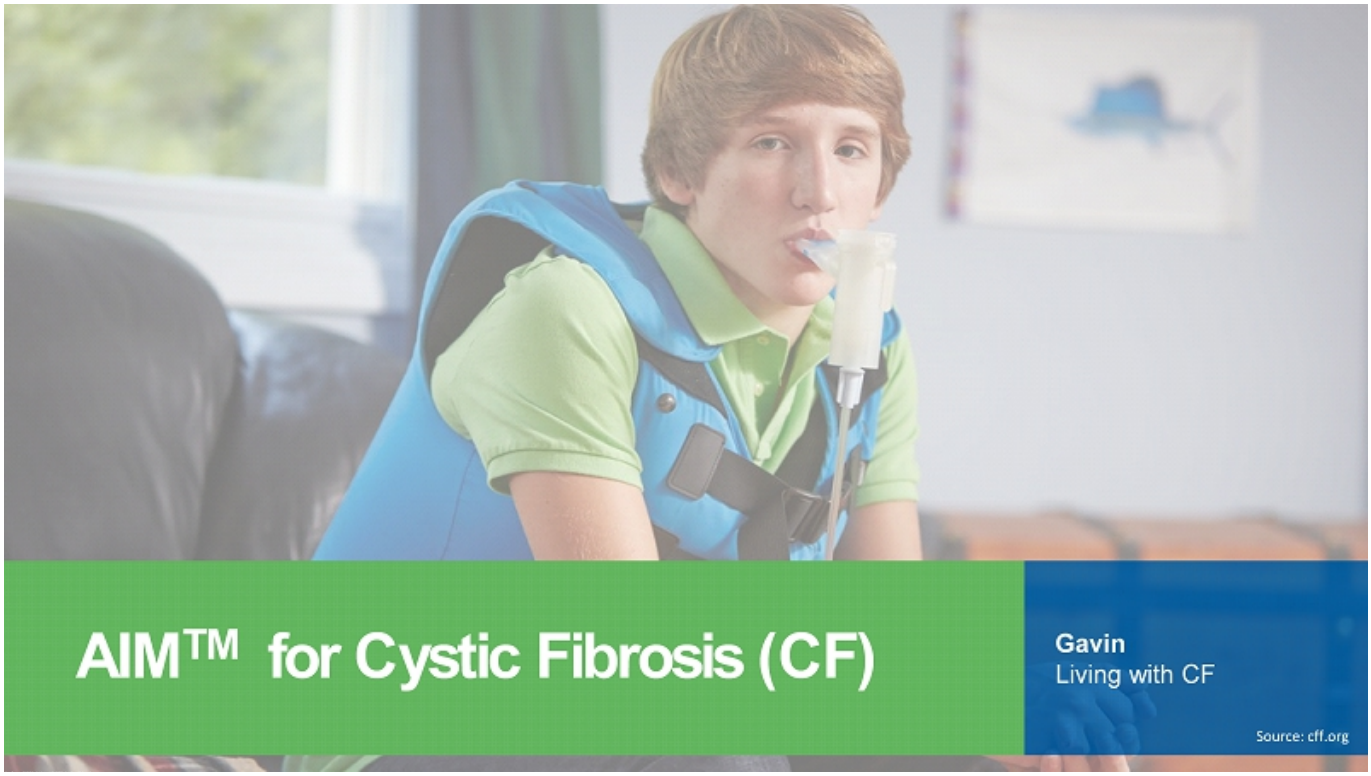
Green: GFP Red: Rhodopsin Blue: Dapi

AAV204 Efficiently Transduces Rods & Cones in Macula in Non-human primate Retina After Intravitreal Injection



Summary: AIM™ vectors enable clinical translation for eye diseases

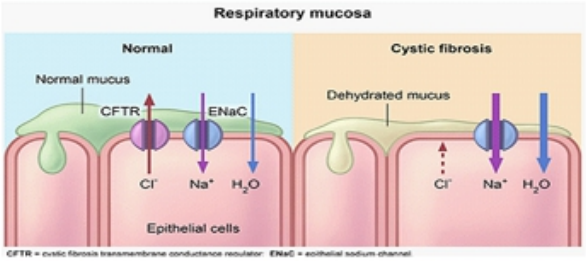
- 80% of genetic eye disorders occur in the photoreceptors
- Intravitreal delivery of small volume gene therapies can occur in out-patient clinics
- Mouse studies identified multiple AIM™ vectors with high retinal tropism by subretinal and intravitreal injections
- Vector tropism translated from mice to non-human primates
- Cones, rods, RPE – multiple cell types able to be targeted by AIM™ vector
- Data suggest AIM™ capsid may escape neutralizing antibodies against natural serotypes - Enables potential redosing


AIM™ for Cystic Fibrosis (CF)
Gavin
Living with CF
Source: cff.org

ABO-401 (AIM™ AAV204.CFTR) for CF

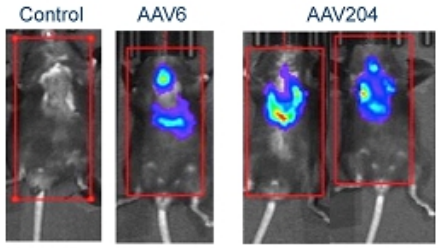
- CF is a progressive, genetic disease that causes persistent lung infections and limits the ability to breathe over time
- CF is caused by a mutation in the gene cystic fibrosis transmembrane conductance regulator (CFTR)
- CF affects at least 30,000 people in the United States; between 900 and 1,000 new cases are diagnosed every year
- ABO-401: First AIM™ candidate using novel AAV capsid
- Potential follow-on products for lung disorders

AAV204 demonstrates enhanced lung tropism

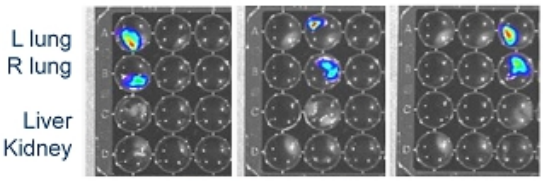


Historical Challenges to CF gene therapy:
1. Packaging *CFTR* in an AAV is size restricted
2. Delivery of AAV to lung cells

Opportunity: new AIM vector with lung tropism - novel transgene + AIM capsid



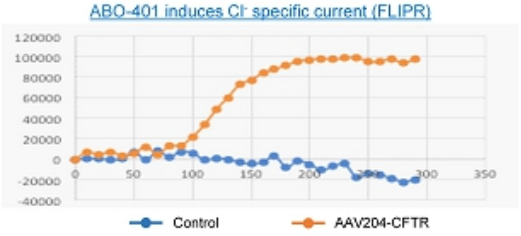
AAV204 demonstrates ~3-5x higher expression in lung after delivery compared to AAV6



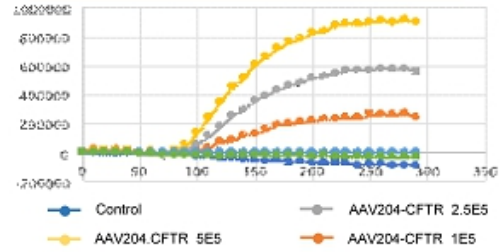
Ex vivo organ imaging

ABO-401 is a membrane-localized CFTR transgene that Corrects CFTR chloride current in CF mice

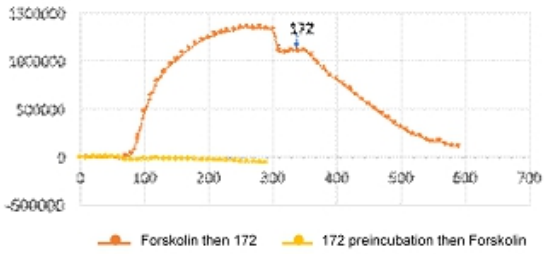
ABO-401 induces Cl- specific current (FLIPR)



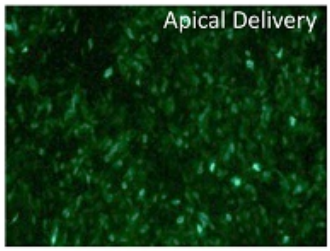
ABO-401 dose dependent induction of Cl- specific current



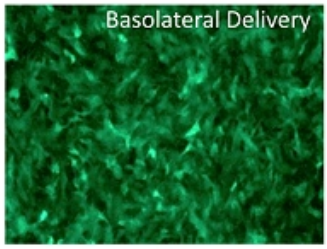
ABO-401 efficacy can be inhibited by a CFTR specific small molecule inhibitor 172



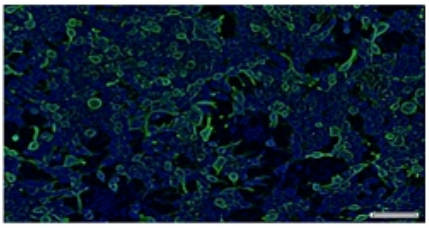
ABO-401 Can Address All CFTR Mutations



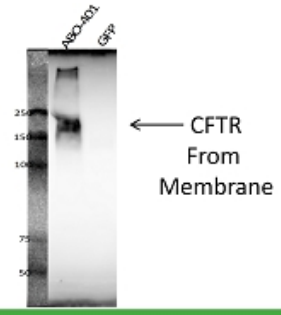
Human delta508 CF bronchial cells polarized airway cultures with AAV204-GFP



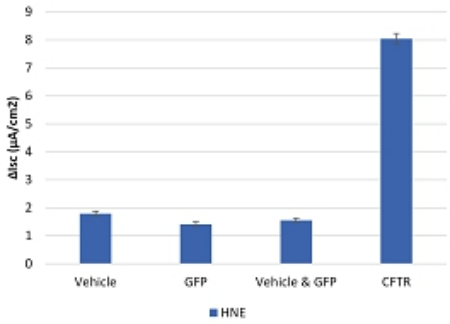
Human delta508 CF nasal epithelial cells polarized airway cultures with AAV204-GFP



ABO-401 produces CFTR that is membrane localized in human CF cells
AAV 293

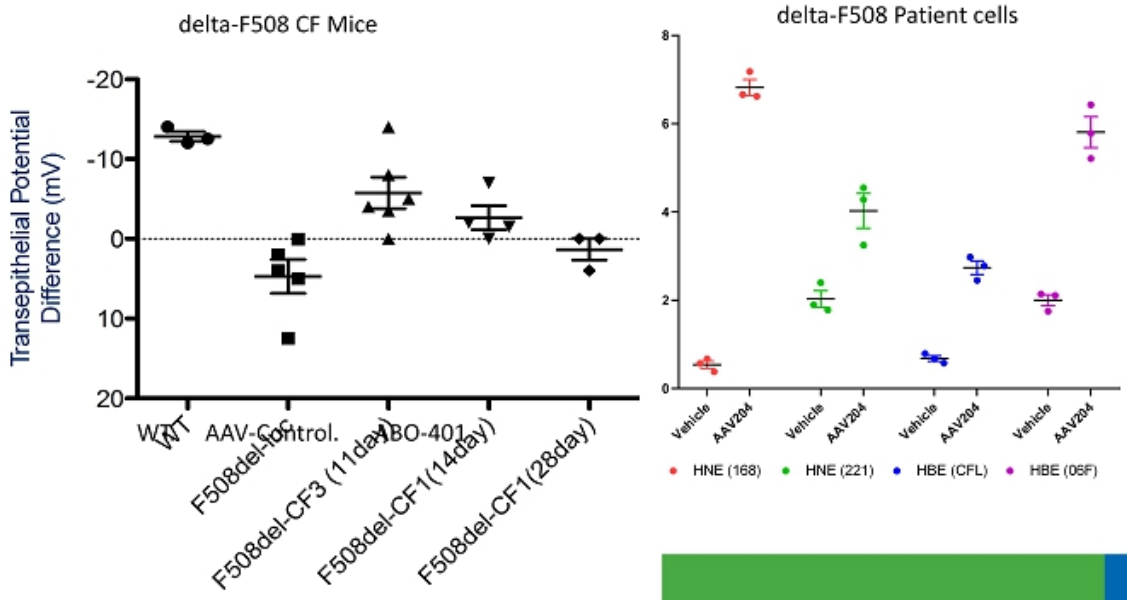


ABO-401 Restores CFTR current in human CF Patient cells



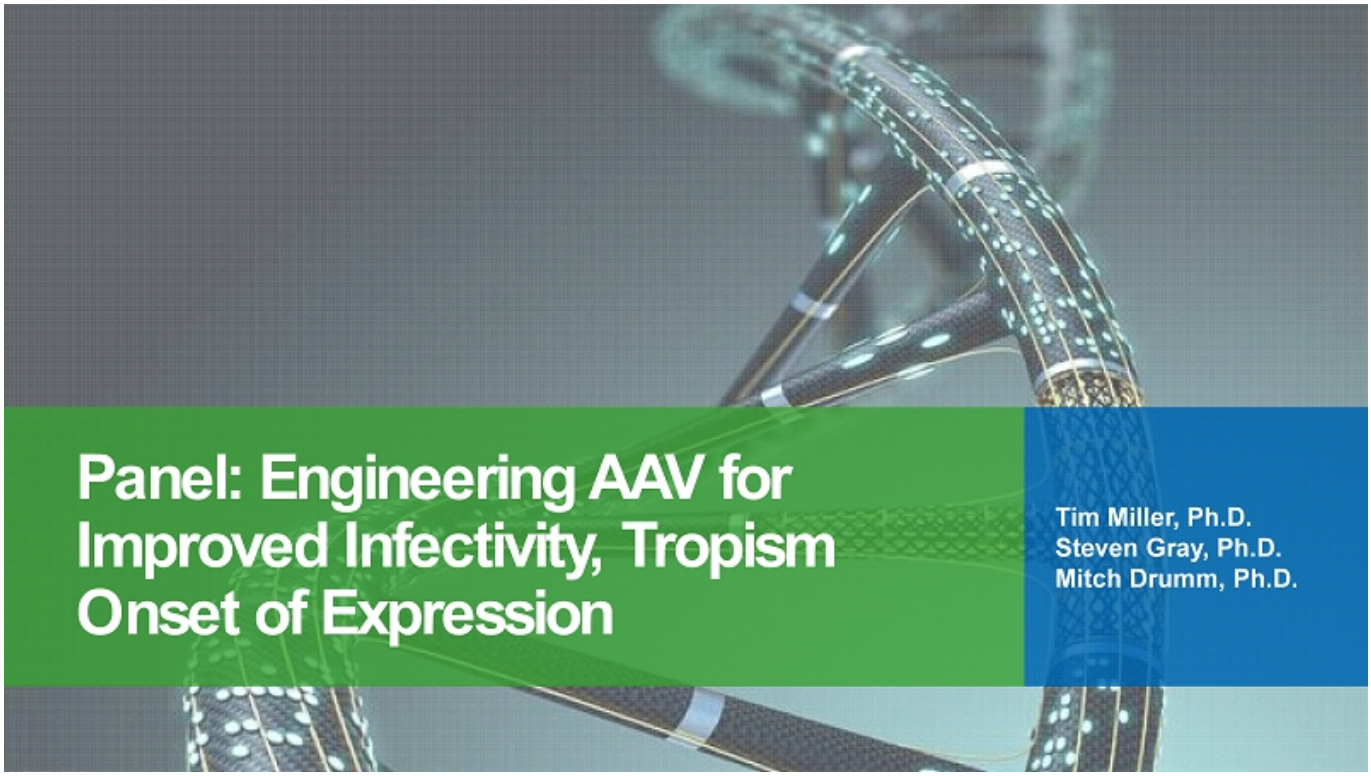
CF Patient Explant Cultures

ABO-401 Correct delta-F508 mutation in CF Mice and Human CF Patients



ABO-401 – Advancing Gene Therapy for the Treatment of All human CF Mutations

- AAV-204 efficiently targets lung cells
- ABO-401 corrects the underlying CF chloride channel deficit
- ABO-401 can address all CFTR mutations, including delta-F508 (50-60% of human population)
- Transduces ***human*** CF patient bronchial and nasal epithelial cells
- Corrects chloride current in dF508 CF cells


Panel: Engineering AAV for Improved Infectivity, Tropism Onset of Expression
Tim Miller, Ph.D.
Steven Gray, Ph.D.
Mitch Drumm, Ph.D.


Abeona
THERAPEUTICS

Exhibit 99.2

Abeona Therapeutics Details Pathway for Advancing Lead Clinical Programs and Unveils New Cystic Fibrosis Program Born from Next Generation AIM™ Vector Platform at 2018 R&D Day

EB-101 pivotal trial for Recessive Dystrophic Epidermolysis Bullosa planned for mid-2019 enrollment

Expanding Phase I/II study of ABO-102 for Sanfilippo syndrome type A (MPS IIIA)

Novel AIMTM AAV vector with CFTR minigene addresses all mutations of Cystic Fibrosis

NEW YORK and CLEVELAND, Dec. 06, 2018 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel cell and gene therapies for life-threatening rare genetic diseases, today announced key pipeline updates during the Company's 2018 R&D Day.

"The important clinical and preclinical updates we shared today further establish Abeona's pathway to bring long-term value to our shareholders and hope to our patients," said João Siffert, M.D., interim Chief Executive Officer, Chief Medical Officer and Head of R&D. "We are very pleased to share next steps for our lead programs and to unveil the potential of the novel AIMTM AAV vector platform that could be a catalyst for the next generation of gene therapy."

EB-101 for Recessive Dystrophic Epidermolysis Bullosa (RDEB)
Abeona is developing gene-corrected cell therapy EB-101 for the treatment of RDEB, a skin disease characterized by chronic epidermal wounds in which patients suffer from pain, itching, and widespread complications impacting quality-of-life and life expectancy.

The Company expects to initiate a pivotal clinical trial evaluating the potential of EB-101 for the treatment of RDEB in the middle of 2019. The VITAL Study will be a multicenter, randomized, Phase III clinical trial assessing 10-15 patients treated with EB-101 compared to intra- patient untreated wounds. The primary outcome measure of the study will be the proportion of treated wounds with >50% healing at three months, with secondary endpoints of investigator global assessment of wounds and changes in pain and itch from baseline.

The Company also reported that it has established GMP manufacturing capability for EB-101 at its gene therapy manufacturing facility in Cleveland. The facility, known as the Elisa Linton Center for Rare Disease Therapies, can produce clinical product, and has scalable capacity to support the potential commercial launch of EB-101.

"We believe that we are strongly positioned to initiate a pivotal trial evaluating EB-101 by mid-2019 thanks to the important CMC work undertaken by colleagues at our gene therapy manufacturing facility in Cleveland, which also addressed guidance received through frequent regulatory interactions afforded by the Regenerative Medicine Advanced Therapy and other designations we hold. We believe this work is critical for our path towards BLA filing," added Dr. Siffert.

ABO-102 for Sanfilippo Syndrome Type A (MPS IIIA)
Abeona is developing novel gene therapy ABO-102 for the treatment of MPS IIIA, a lysosomal storage disease with no approved treatment that is characterized by neurodevelopmental decline, behavior abnormalities, seizures, loss of speech or vision, an inability to sleep, and premature death.

The Company plans to amend its ongoing Phase I/II trial evaluating ABO-102 for MPS IIIA to enroll patients at earlier stages of disease. ABO-102 has been well tolerated to date with no serious drug-related adverse events. The study has also demonstrated a substantial, dose-related improvement in biomarkers, including reductions in cerebrospinal fluid heparan sulfate levels and liver volume in patients treated with ABO-102. Investigators also observed encouraging neurocognitive signals in younger, higher functioning patients enrolled in the higher dose of Cohort 3. Patients unable to participate in the modified Phase I/II study may be eligible to enroll in other studies within our MPS IIIA program.

"The encouraging data generated to date and our interactions with the FDA and EMA have informed the advancement of our Phase I/II trial, which will seek to enroll patients likely to receive the most benefit from treatment," added Dr. Siffert.

ABO-401 for Cystic Fibrosis
Abeona presented new pre-clinical data today from the Company's first program produced by the novel AIMTM vector platform for gene therapy delivery. The data suggest that ABO-401, based on the vector AAV204, efficiently targets lung cells and that ABO-401 corrects the underlying cystic fibrosis (CF) chloride channel deficit, regardless of underlying mutations of the CF transmembrane conductance regulators, including the most common CF mutation, delta-F508.

AIMTM Vector Platform Targeting the Eye
The Company presented non-human primate data suggesting that next-generation AIM TM AAV vectors can efficiently target the retinal epithelium after intravitreal injection, creating the potential for new pipeline candidates that can address multiple eye disorders. Also presented were data showing that certain AIMTM capsids with high tropisms for central nervous system tissue can evade neutralizing antibodies against naturally occurring AAV serotype, and potentially enable redosing in patients that have previously received an AAV injection.

"Our AIMTM vector platform enables the potential for gene therapy for patients living with cystic fibrosis, regardless of mutation, which could change the landscape of treatment and alter the course of this progressive, genetic disease," said Timothy J. Miller, Ph.D., co-Founder, President, and Chief Scientific Officer. "We are very encouraged by the preclinical data presented today demonstrating delivery and correction of the underlying genetic deficit in CF patient cells. Furthermore, we are very excited to show the capability of the AIMTM vectors for delivering genes to the eye and are excited about their potential as the next generation of gene therapy across tissue types."

ABO-202 for Infantile Batten Disease (CLN1)
Abeona is developing ABO-202 for the treatment of CLN1, a rare and fatal autosomal recessive genetic disorder with no approved treatment, which is characterized by vision impairment and rapid neurological regression.

The Company presented new preclinical data today that will inform the submission of an investigational new drug application (IND) for ABO- 202 in the first quarter of 2019. Findings from a combination pre-clinical, dose-escalation study suggest that ABO-202 may have a favorable safety profile, with no significant toxicology findings. Other IND-enabling studies also demonstrated normalized survival, improvement of motor function and cognition in affected mice treated with ABO-202, and that combination dosing of intravenous and intrathecal administrations may enhance the therapeutic potential of ABO-202.

Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates and is the only company with RMAT designation for two investigational therapies (EB-101 and ABO-102).

About Abeona Therapeutics
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include EB-101 (gene-corrected cell therapy) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and ABO-101 (AAV- NAGLU), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type B (MPS IIIB). Abeona is also developing ABO- 201 (AAV-CLN3) gene therapy for CLN3 disease, ABO-202 (AAV-CLN1) for treatment of CLN1 disease, EB-201 for epidermolysis bullosa (EB), ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder ABO-302 using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases, and ABO-401 using a novel AAV vector platform, AIM™ with CFTR minigene addresses all mutations of cystic fibrosis. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates and is the only company with RMAT designation for two investigational therapies (EB-101 and ABO-102).
For more information, visit www.abeonatherapeutics.com.

Forward Looking Statements

This press release contains certain statements that are forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties. These statements include statements regarding our pipeline and product portfolio, approval by regulatory agencies relative to amendments to INDs and to protocol amendments including related to ABO-102, timelines for initiation of further clinical studies including the pivotal study for EB-101, the establishment and scalability of manufacturing capabilities, the capabilities of the novel AIM™ vector platform in development; the market opportunities for the Company's products and product candidates, the ability to generate long term shareholder value, meet patient expectations, and achieve the company's goals and objectives. We have attempted to identify forward looking statements by such terminology as "may," will," "anticipate," "believe," "estimate," "expect," "intend," and similar expressions.

Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, numerous risks and uncertainties, including but not limited to: continued interest in our rare disease portfolio, our ability to submit protocols and protocol amendments to regulatory agencies, our ability to initiate and enroll patients in clinical trials, the adequacy of manufacturing capabilities, the impact of competition, the ability to secure licenses or establish intellectual property rights for any technology that may be necessary to continue to develop and commercialize our products, the ability to achieve or obtain necessary regulatory approvals, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, and other risks as may be detailed from time to time in the Company's annual reports on Form 10-K and quarterly reports on Form 10-Q and other reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward- looking statements or update them to reflect events or circumstances occurring after the date of this presentation, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

Investor Contact:
Christine Silverstein
SVP, Finance & Investor Relations
Abeona Therapeutics Inc.
+1 (646) 813-4707
csilverstein@abeonatherapeutics.com

Media Contact:
Scott Santiamo
Director, Corporate Communications

Abeona Therapeutics Inc.
+1 (718) 344-5843
ssantiamo@abeonatherapeutics.com