Corporate Presentation

December 2020

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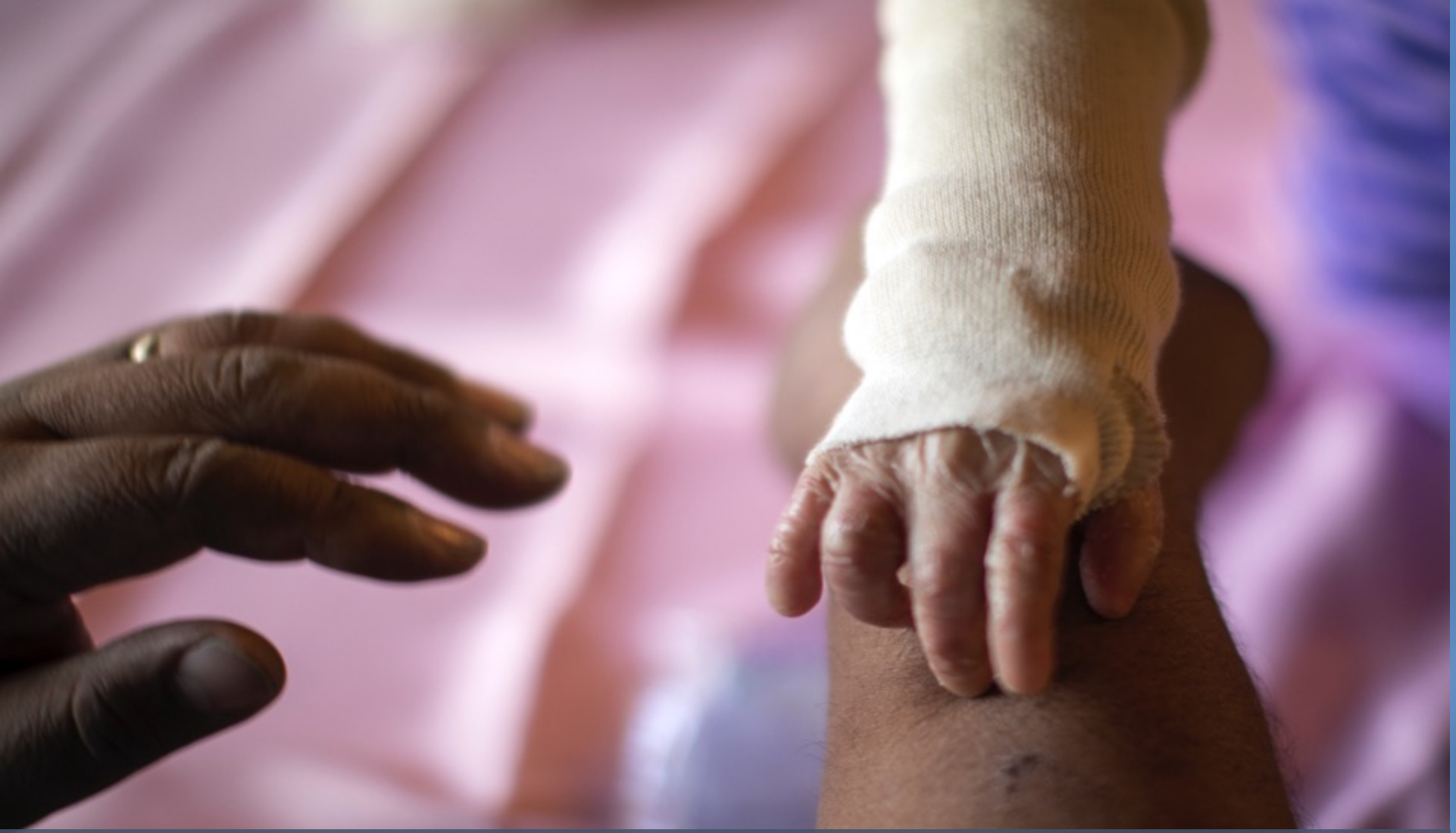
A Fully-Integrated Gene & Cell Therapy Company Focused on Rare Diseases With No Approved Treatments

Comprehensive Gene & Cell Therapy Capabilities	Late-Stage First-to-Market Opportunities	Breakthrough Therapy with Gene Corrected Cell Therapy	AAV9 and Proprietary AAV (AIM™) Programs	Gene & Cell Therapy Expertise and Manufacturing Capabilities	Robust Pipeline of Clinical Stage and Preclinical Programs

- EB-101 breakthrough therapy in pivotal Phase 3 study for RDEB, supported by evidence of multi-year wound healing data
- AAV gene therapies with early signs of clinical benefit in MPS IIIA and clear biologic effect in MPS IIIB in Phase 1/2 trials
- Novel AIM™ AAV capsid platform: in vivo proof of concept data for efficient intravitreous and subretinal delivery in NHP as well as tropism for other organs, including CNS
- State-of-the-art cGMP manufacturing for clinical and commercial grade gene and cell therapy products
- $104M in cash, cash equivalents, receivables and marketable securities (Sept 30, 2020)

Robust Pipeline



EB-101: Gene-Corrected Cell Therapy for RDEB

- ✓ Orphan Drug Designation (FDA)
- ✓ Orphan Drug Designation (EU)
- ✓ Rare Pediatric Disease Designation (FDA)
- ✓ Breakthrough Therapy Designation (FDA)
- ✓ Regenerative Medicine Advanced Therapy Designation (FDA)

Epidermolysis Bullosa (EB): Devastating Inherited Connective Tissue Disorder

Recessive Dystrophic Epidermolysis Bullosa (RDEB):

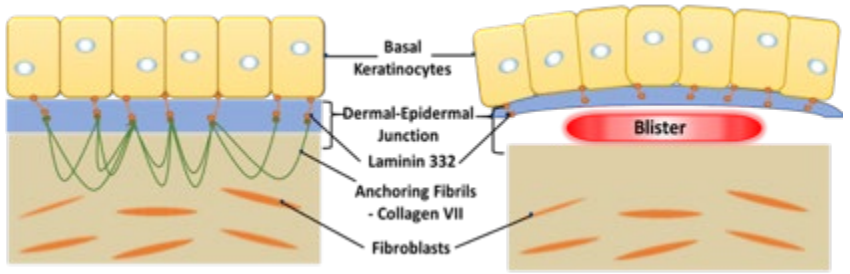
- Most severe form of EB
- Primarily characterized by skin blisters and erosions
- Caused by mutations in COL7A1 gene, which encodes type VII collagen
- Est. 2,500 U.S. patients

Up to 80% of patient's body covered in wounds, leading to:

- Severe pain and widespread scarring
- Debilitating and life-threatening systemic complications
- Up to 90% of RDEB patients are at risk for squamous cell carcinoma (SCC)

50% of generalized severe patients die before 35

75% die before 40

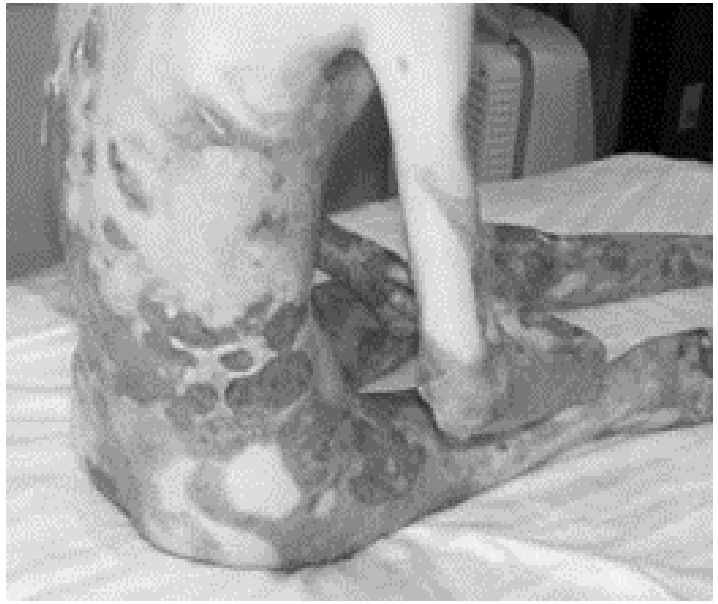


normal skin affected skin

The lack of functioning anchoring fibrils in RDEB patients leads to skin blistering and tears with minor trauma

Recognizing the Full Burden of RDEB

Clinical



Large, chronic wounds comprise the main clinical burden in RDEB and are correlated with pain.

Up to 90% of RDEB patients are at risk of developing SCC.

Economic

Annual wound dressing cost per patient is up to $245,000.

U.S. families characterized the economic impact of managing RDEB as “high” or “severe.”

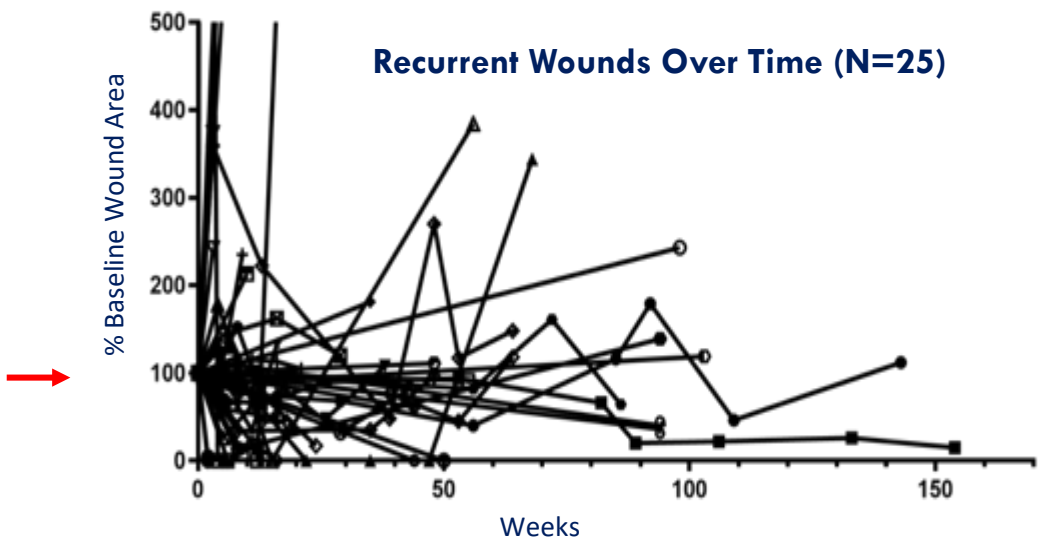
Humanistic

Many patients have anxiety and depression.

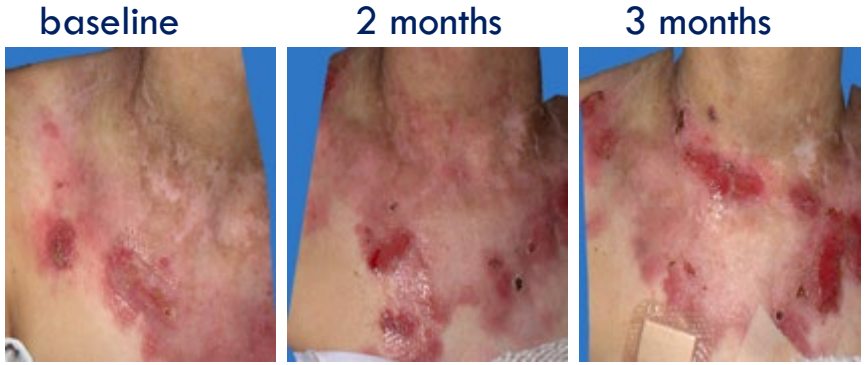
67% of divorced parents reported RDEB as a major/primary factor.

Tang et al. Society for Pediatric Dermnatology 2020 Poster Presentation

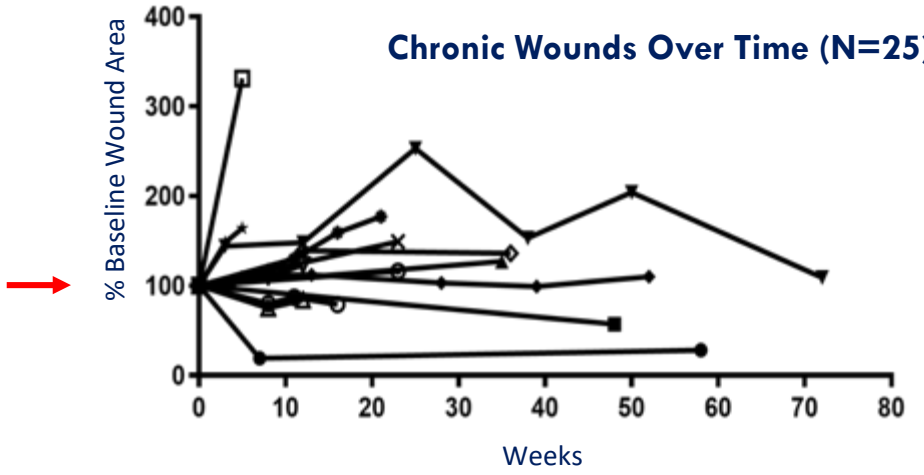
Recurrent and Chronic Wounds Have Distinct Time Courses



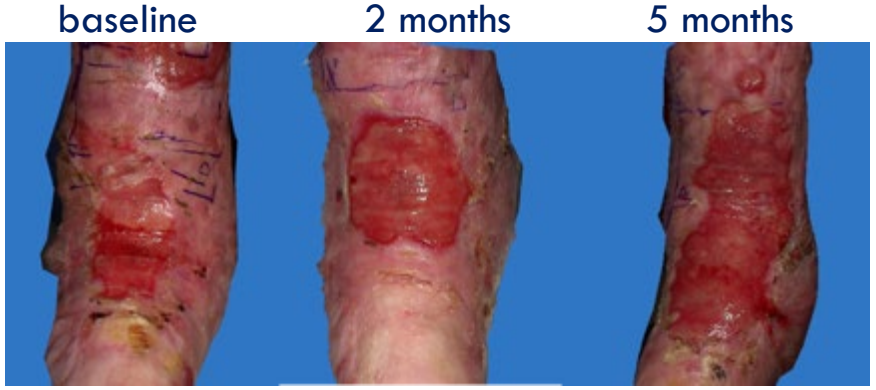
Recurrent



Time to heal (6 weeks)
Time to re-blister (3 weeks)



Chronic

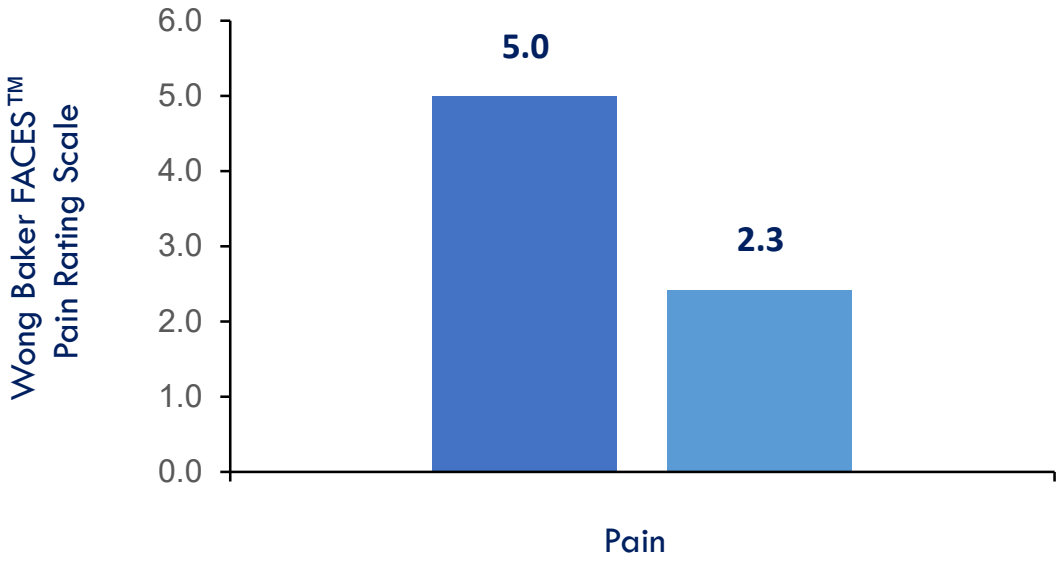


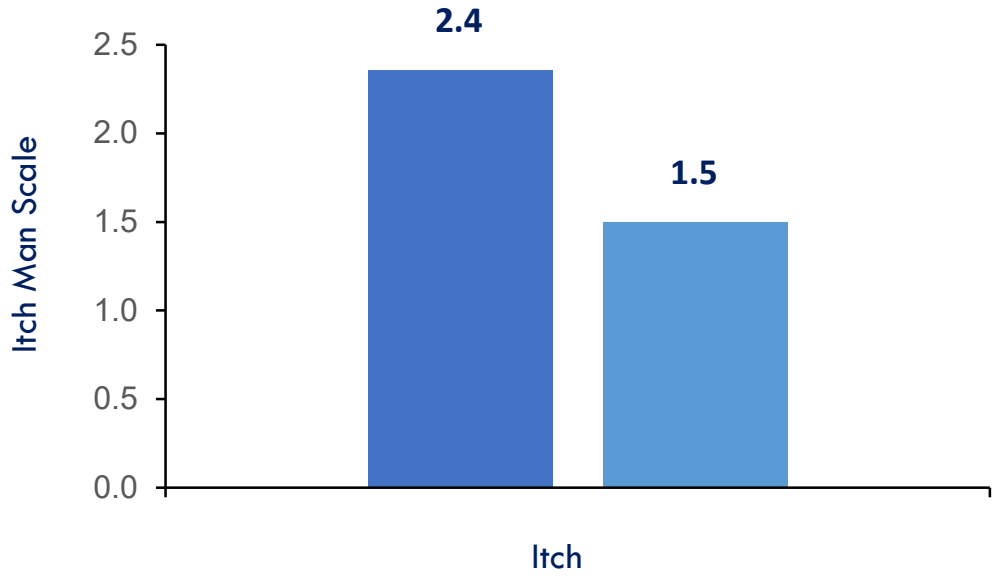
Natural history of chronic wounds in patients with recessive dystrophic epidermolysis bullosa; Solis, D. et al.; Journal of Investigative Dermatology, Volume 137, Issue 5, S37

Recurrent Wounds Over Time (N=25)

Large, Chronic Open Wounds Cause Greatest Pain and Itch

Sequentially Photographed Wounds: N=25 patients, 62 wounds





	≤19 cm^2	20-39 cm^2	≥40 cm^2	Mean Size	Mean Duration
Recurrent	64%	21%	15%	26 cm^2	5 years
Chronic Open	27%	20%	53%	118 cm^2	7 years

Natural history of wounds in patients with recessive dystrophic epidermolysis bullosa; Teng et al., Abstract #251; Society of Investigational Dermatology Annual Meeting, 2019

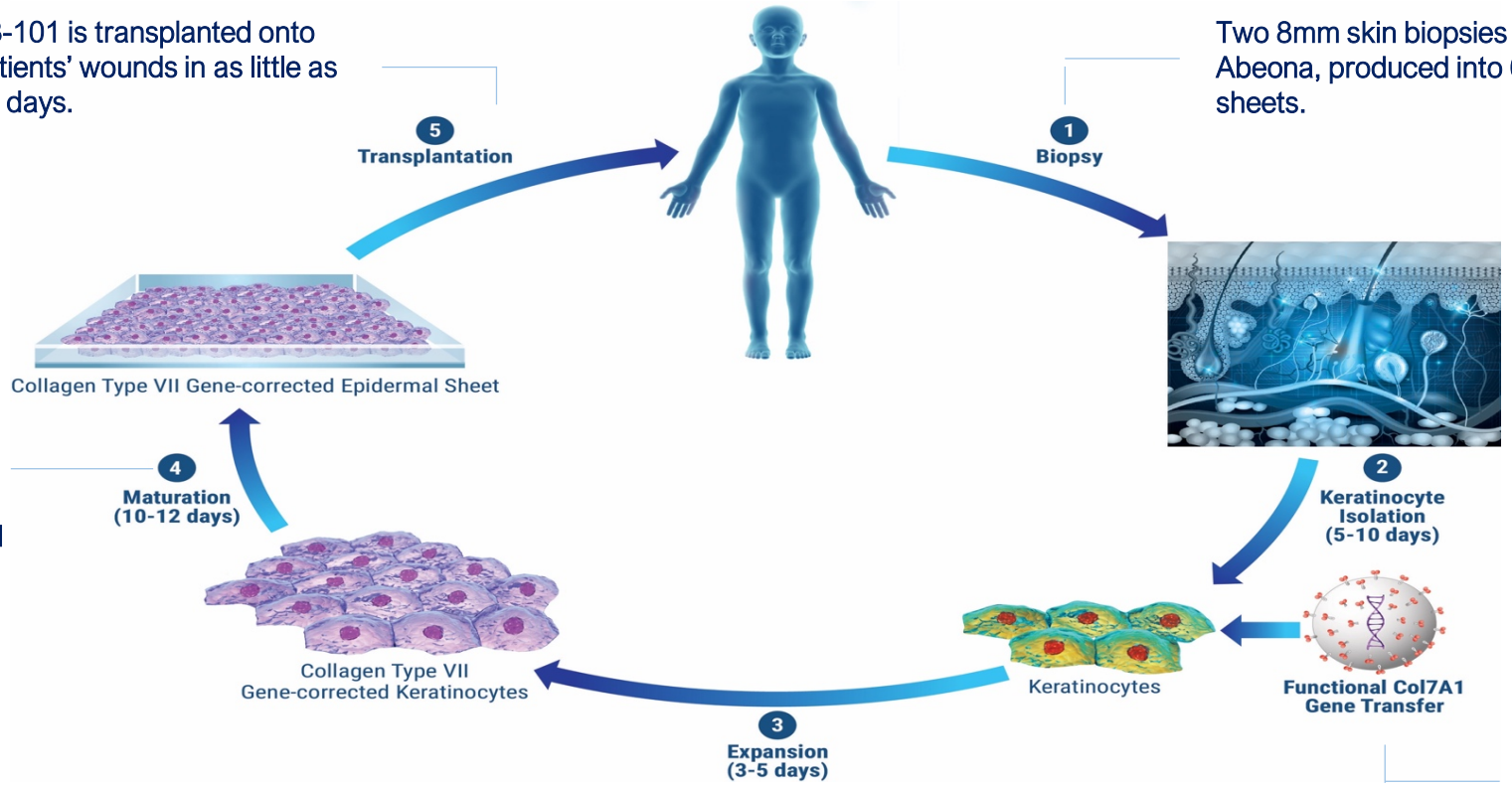
EB-101: Ex-Vivo Autologous Gene Corrected Cell Therapy

EB-101 RESTORES FUNCTIONAL COLLAGEN VII TO PATIENT'S OWN KERATINOCYTES AND THEIR PROGENITORS

- PERSONALIZED TREATMENT
- BIOPSY TO PATIENT-READY IN ~4 WEEKS
- 2 SKIN BIOPSIES = 6 SHEETS = 240 CM^2

EB-101 is transplanted onto patients' wounds in as little as 26 days.

Two 8mm skin biopsies shipped to Abeona, produced into 6-8 ~40 cm^2 sheets.



Keratinocyte cells expanded and prepared the for next step.

10-12 days of continued cell maturation and growth.

Keratinocyte cells expanded and harvested as 5.5x7.5cm sheets.

Functional Type VII collagen transduced into keratinocyte cells; cell maturation leads to gene-corrected type VII collagen expression.

EB-101 Pivotal Phase 3 VIITAL™ Study

VIITAL
PHASE 3 STUDY

Study Design	• Multi-center, randomized trial led by Stanford University • 10-15 RDEB patients, with approx. 30 chronic wound sites treated in total • Follow-up visits 1-6 months, then in a long-term follow-up protocol until year 5

Study Endpoints	• Proportion of wounds with >50% healing, comparing treated with untreated wound sites on the same patient
	• Patient's global impression of change in pain from baseline
	• Patient-reported outcomes assessing pain during:
	  – Dressing changes
	  – Pain impact
	  – Physical function

Enrollment completion expected in first half of 2021

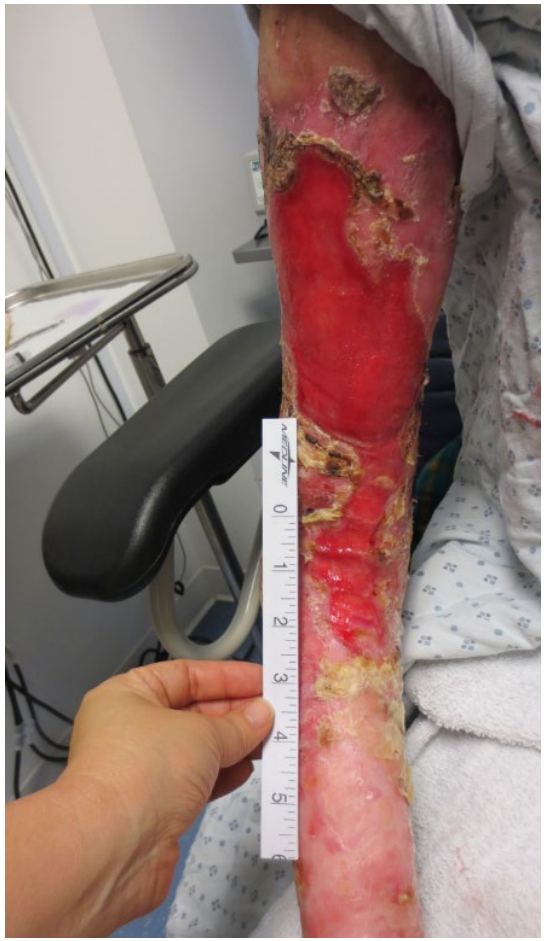
VIITAL™ Study Supported by EB-101 Phase 1/2a Study for RDEB

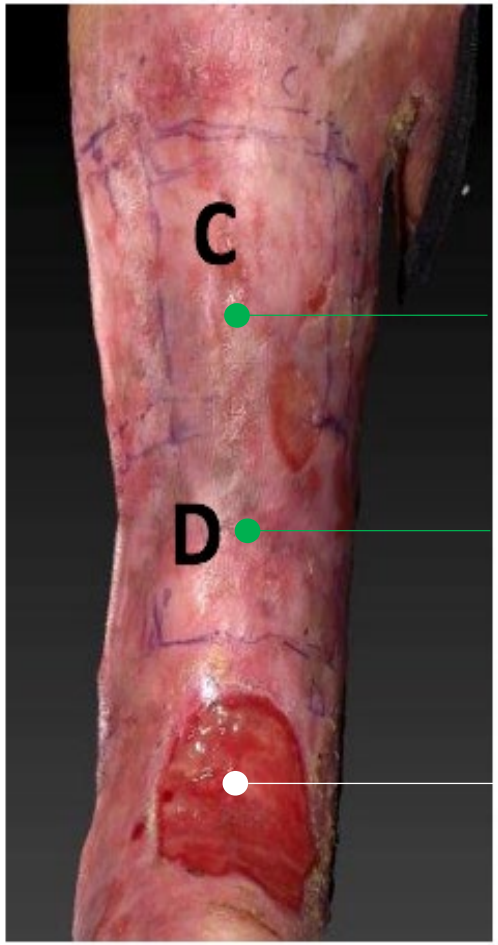
Study Description	• A Phase 1/2a Single Center Trial of Gene Transfer for Recessive Dystrophic Epidermolysis Bullosa (RDEB) using EB-101 for autologous tissue transplantation
Study Design	• Open-label, interventional study • Seven patients with RDEB (ages 18 to 45 years) • Follow-up visits at 1-12 months post treatment; yearly thereafter until year 5

- Phase 1/2a study addressed wounds of increasing severity and complexity
- Study participants had challenging wounds representative of those most troublesome for the RDEB population
- Learnings from program provided essential guidance for future wound treatment, de-risked Phase 3 study
- Potential to address most wounds, regardless of size or duration

EB-101 Treated Large, Chronic Wounds in a Phase 1/2 Study

Baseline 9 months





Durable wound healing

- Healing lasted for 3+ to 5+ years after treatment
- Wound healing of large wounds was associated with no pain

Favorable safety profile 5 years after treatment

- Longest safety follow up of any gene therapy in development for RDEB

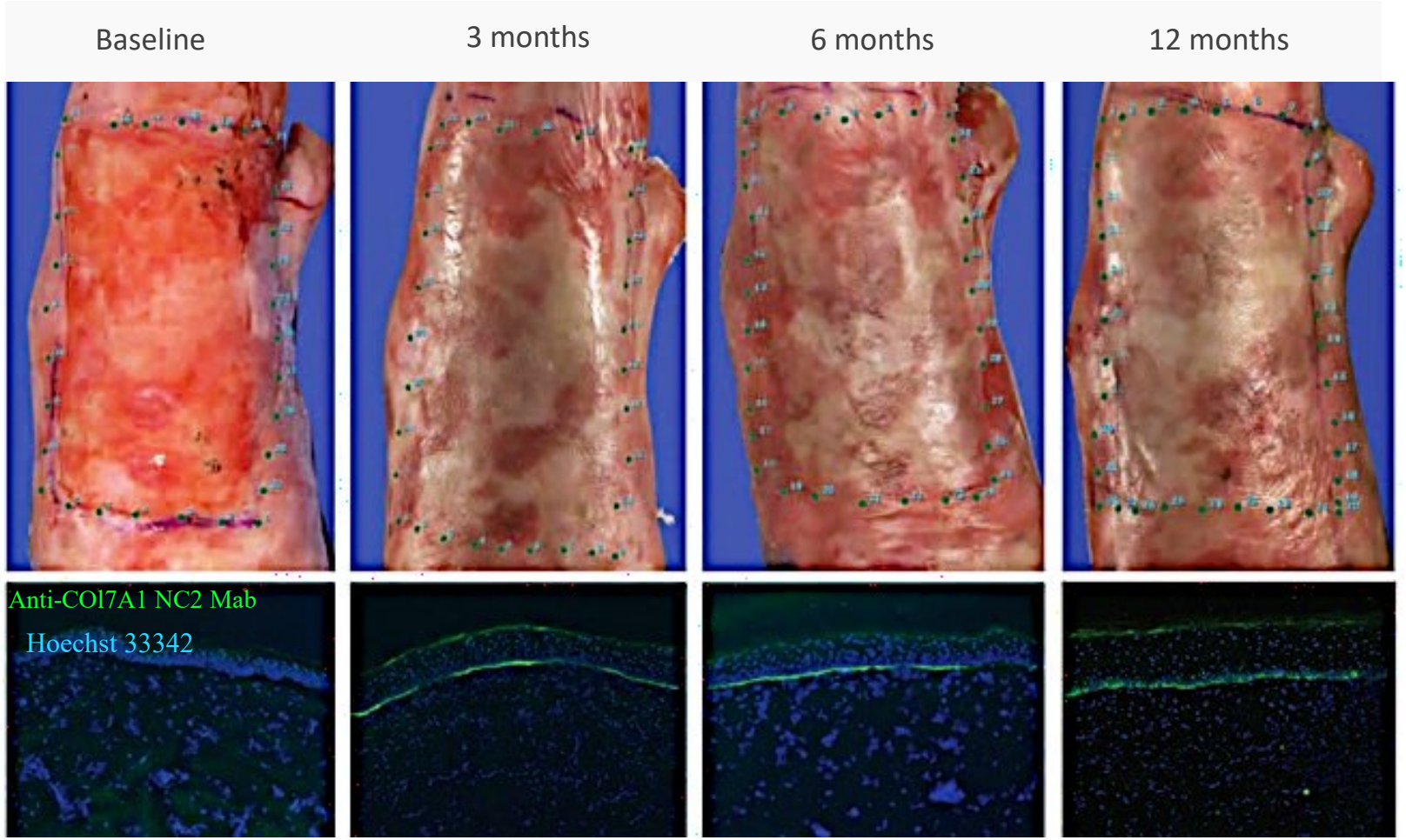
Evidence of treating the underlying cause

- Continuous Type VII collagen expression seen 2+ years after treatment

Eichstadt et al. JCI Insight 2019

EB-101 Restored Collagen VII that Forms Functional Anchoring Fibrils

Phase 1/2 Study Results



Green line shows collagen expression post-treatment

EB-101 Demonstrated Durable Efficacy

Phase 1/2 Study Results

Participant	Site	Location	Wound Age (years)	3 months	6 months	12 months	2 years	3 years	4 years	5 years
1	A	R distal forearm	>5							
	B	L forearm	>5							
	C	R proximal forearm	>5							
	D	R shoulder	>5							
	E	L arm	<1							
	Z	R arm	Induced							
2	A	Central chest	>5							
	B	L shoulder	>5							
	C	R forearm	3-5							
	D	R posterior shoulder	>5							
	E	Lower back	>5							
	Z	R upper chest	Induced							
3	A	R lateral hand	3-5							
	B	R medial hand	3-5							
	C	L ventral foot	3-5							
	D	L hand	3-5							
	E	R foot	3-5							
	Z	L ventral foot	Induced							
4	A	L distal forearm	>5							
	B	L medial forearm	>5							
	C	L proximal forearm	>5							
	D	R lateral forearm	>5							
	E	R distal forearm	>5							
	Z	R medial forearm	Induced							
5	A	L upper arm	16							
	B	L upper arm	16							
	C	R upper arm	16							
	D	R upper arm	16							
	E	R upper arm	16							
	F	R upper arm	16							
6	A	R lateral back upper	20							
	B	R middle back upper	20							
	C	R medial back upper	20							
	D	R lateral. back lower	20							
	E	R middle back lower	20							
	F	R medial back lower	20							
7	A	R back corner	20							
	B	R lateral outer leg	20							
	C	R Back central	20							
	D	R Back medial	20							
	E	R foot front anterior	20							
	F	Back upper corner	20							

- Enrolled patients with large wounds not eligible for clinical trials with other gene therapies in development
- Wounds up to **400 cm^2** and open **3-20 years**

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

Proportion of Wounds with ≥ 50% and ≥75% Healing

Phase 1/2 Study Results

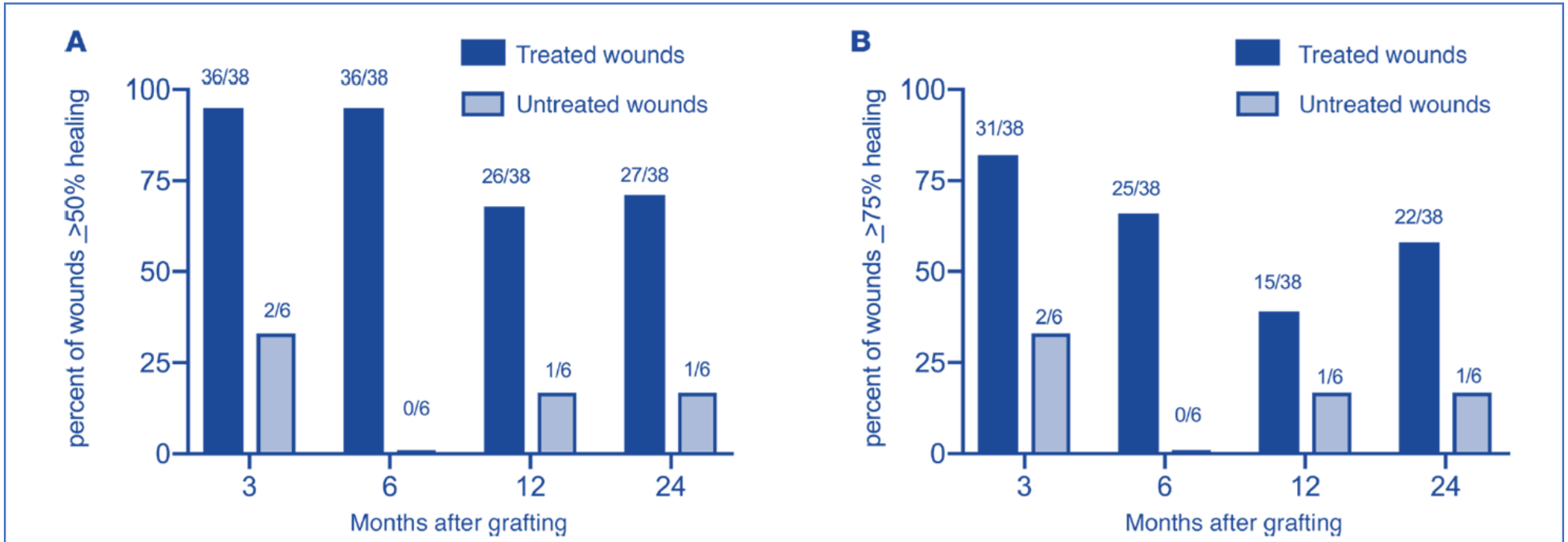


Figure 3. Wound healing in treated and untreated wounds. Summary of treated wounds and untreated control wounds that achieved ≥50% wound healing (**A**) and wounds that achieved ≥75% wound healing (**B**) at designated time points after treatment, as assessed by Investigator Global Assessment. Black bars represent treated sites; light gray bars represent untreated control wounds.

Average wound area healed per patient was 130 cm^2 and 120 cm^2 (up to 157 cm^2) at 3- and 6-months, respectively

(Note: healed area was calculated based on minimum % healing per wound site, e.g. 50% used for wound sites that healed ≥ 50%)

Eichstadt et al. JCI Insight 2019

ABO-102* and ABO-101 Clinical Programs for MPS III

- ✓ Orphan Drug Designation (FDA)
- ✓ Orphan Drug Designation (EU)
- ✓ Rare Pediatric Disease Designation (FDA)
- ✓ Fast Track Designation (FDA)
- ✓ Regenerative Medicine Advanced Therapy Designation* (FDA)
- ✓ Priority Medicines Designation* (EMA)

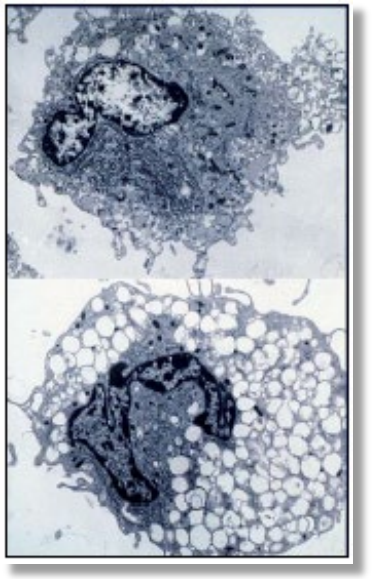
Sanfilippo Syndrome (MPS III)

Inherited monogenic disorders causing lysosomal enzyme deficiency

- Global incidence varies by regions and it is estimated 0.17-2.35 per 100,000 births*
- Two most common forms categorized by deficient enzymes:
  - MPS IIIA (SGSH), MPS IIIB (NAGLU)
- Abnormal accumulation of glycosaminoglycans (GAGs; heparan sulfate (HS))
- Language and cognitive decline, behavioral abnormalities, seizures, sleep disturbances
- Most children with MPS III have only ~60% of typical cognitive capacity by age 3 years
- 70% of children with MPS III do not survive to age 18 years

No approved treatments available

Normal cell

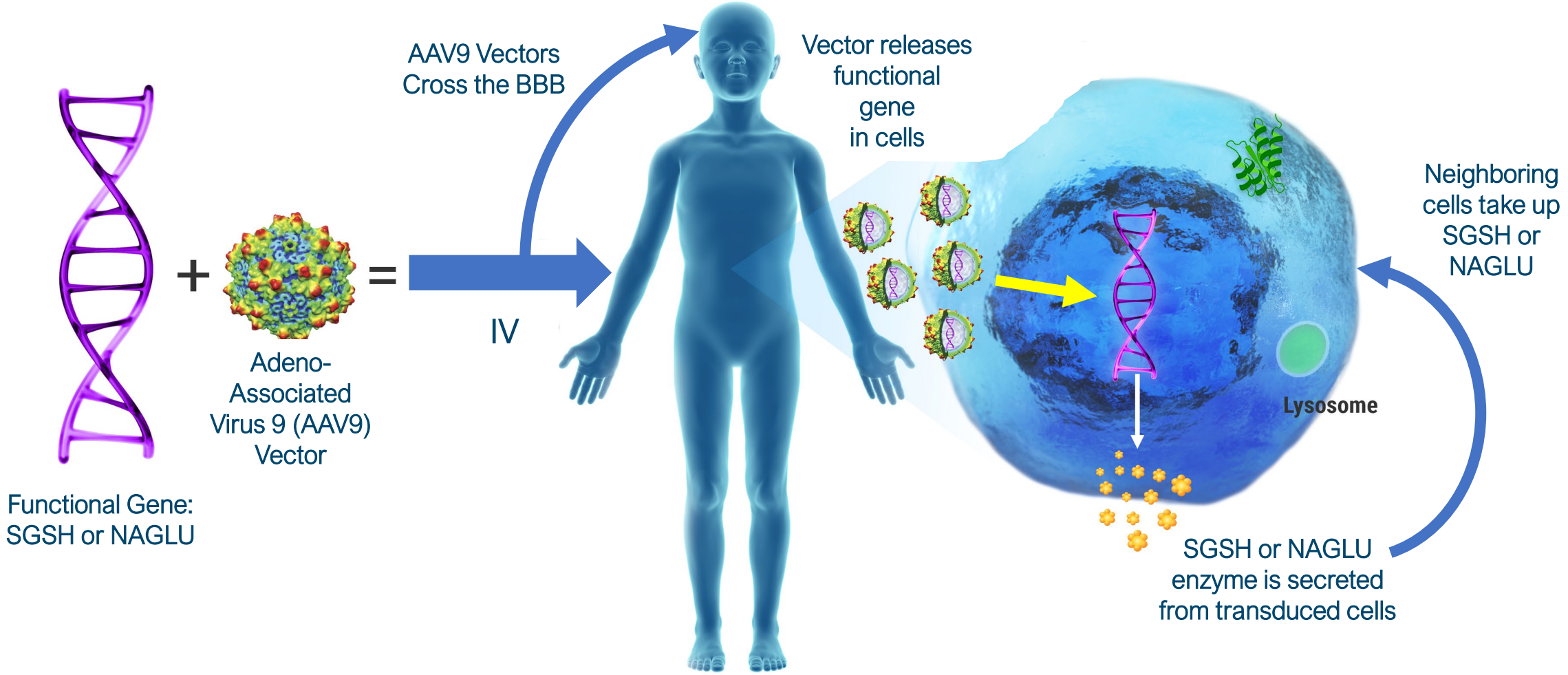


Cell with lysosome deficiency

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

ABO-102 and ABO-101: AAV Gene Therapies for MPS IIIA and MPS IIIB

Mechanism of Action



Phase 1/2 Open-label, Dose-escalation Clinical Trials in MPS IIIA and IIIB

Study Design

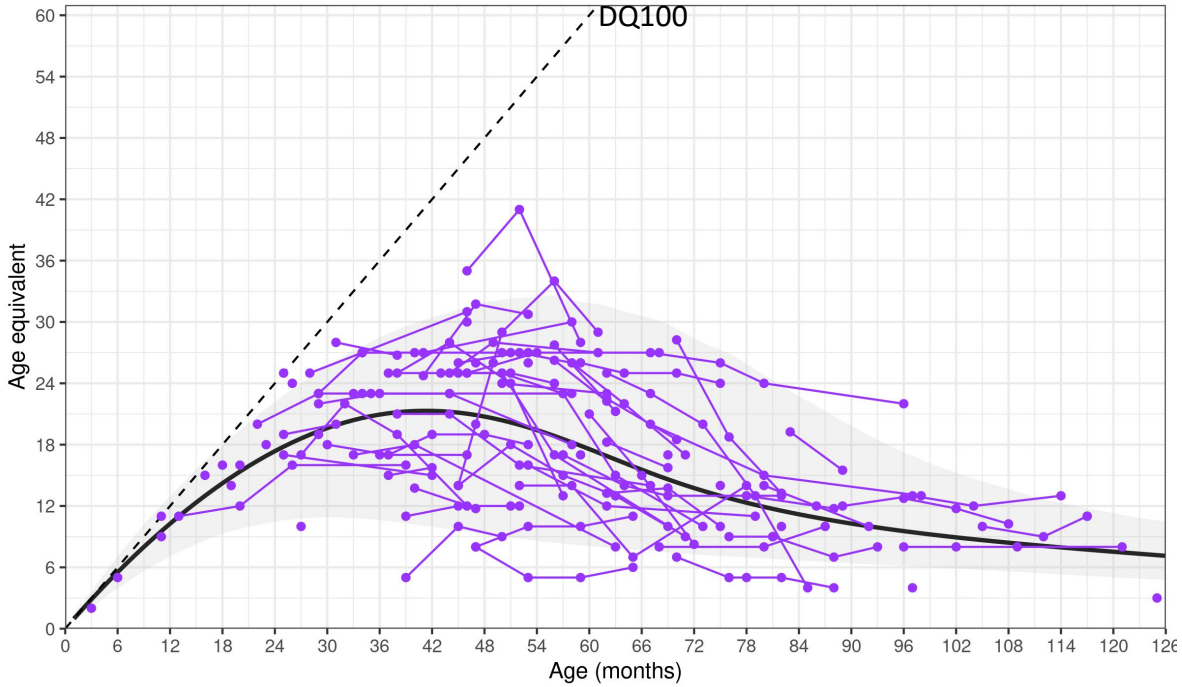
	transpher A study (ABT-001)	transpher B study (ABT-002)
Study Description	• Single IV dose of ABO-102 (scAAV9.U1.hSGSH) for MPS IIIA	• Single IV dose of ABO-101 (rAAV9.CMV.hNAGLU) for MPS IIIB
Enrollment Status	✓ Cohort 1: 5 x 10^{12} vg/kg (n=3) ✓ Cohort 2: 1 x 10^{13} vg/kg (n=3) • Cohort 3: 3 x 10^{13} vg/kg (n= 11, up to 16)	✓ 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=2, up to 8)
Primary Endpoints	• Neurodevelopmental scores post treatment vs. untreated patients enrolled in natural history studies based on Mullen Scales of Early Learning (MSEL) • Product safety	
Secondary Endpoints	• Behavior evaluations, quality of life, enzyme activity, heparan sulfate levels, and brain and liver volume	

Enrollment completion expected in the first quarter of 2021

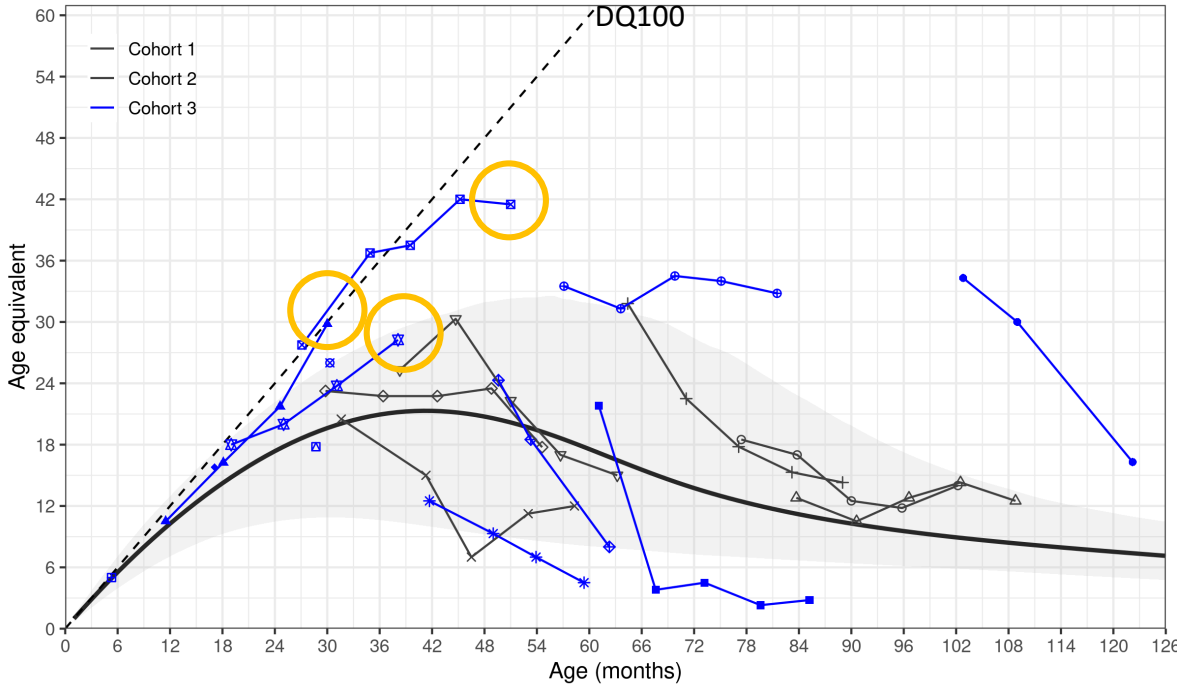
*Clinical study protocol states 3 subjects in Cohort 1; however, due to exceptional circumstances and following robust safety profile and positive review from DSMB, trial was cleared in Europe to advance to Cohort 2 dose

Neurocognitive Development of Youngest Patients Preserved 18-24 months Post Treatment compared with Natural History

Cognitive Natural History Data (92 children)



transpherA study

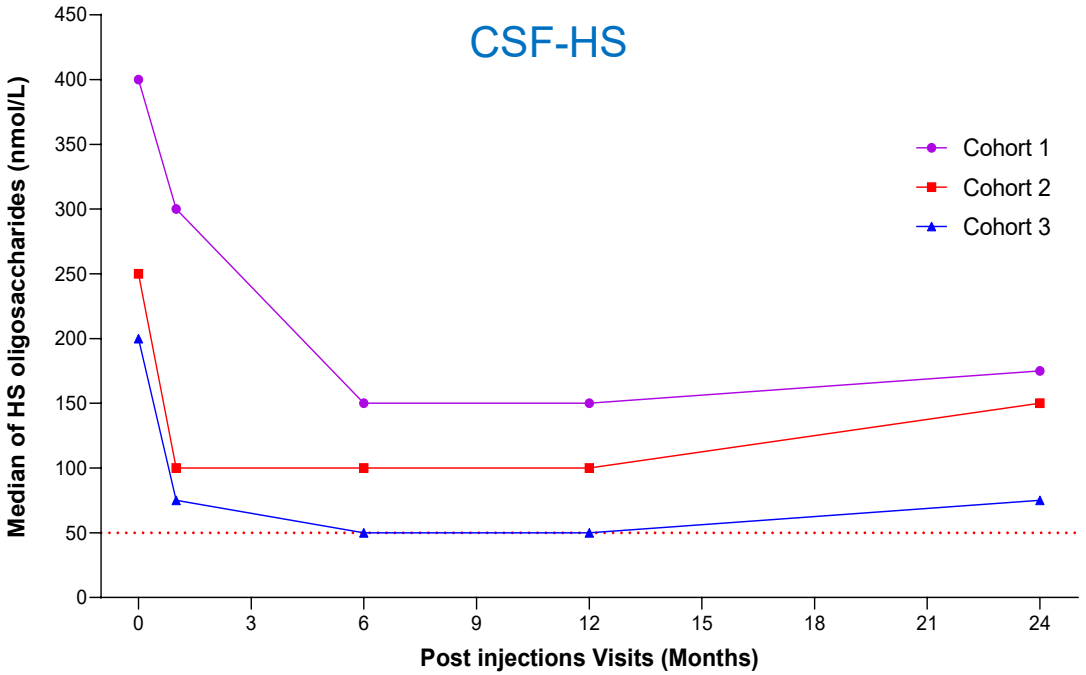


- Black Solid Line: Typical developmental pattern for children with MPS IIIA (natural history)
- Gray Shaded Area: Variability from patient-to-patient differences and measurement error
- Black Dashed Line: Expected development for children without disease

Truxal *et al, Mol Genet Metab,* 2016
Shapiro *et al, J Pediatrics*, 2016
Burhman *et al, J Inherit Metab Dis* 2014
Wijburg *et al,* WORLD Symposium, 2018

Post-treatment Improvement in Disease-Specific CNS Biomarkers in MPS IIIA and MPS IIIB

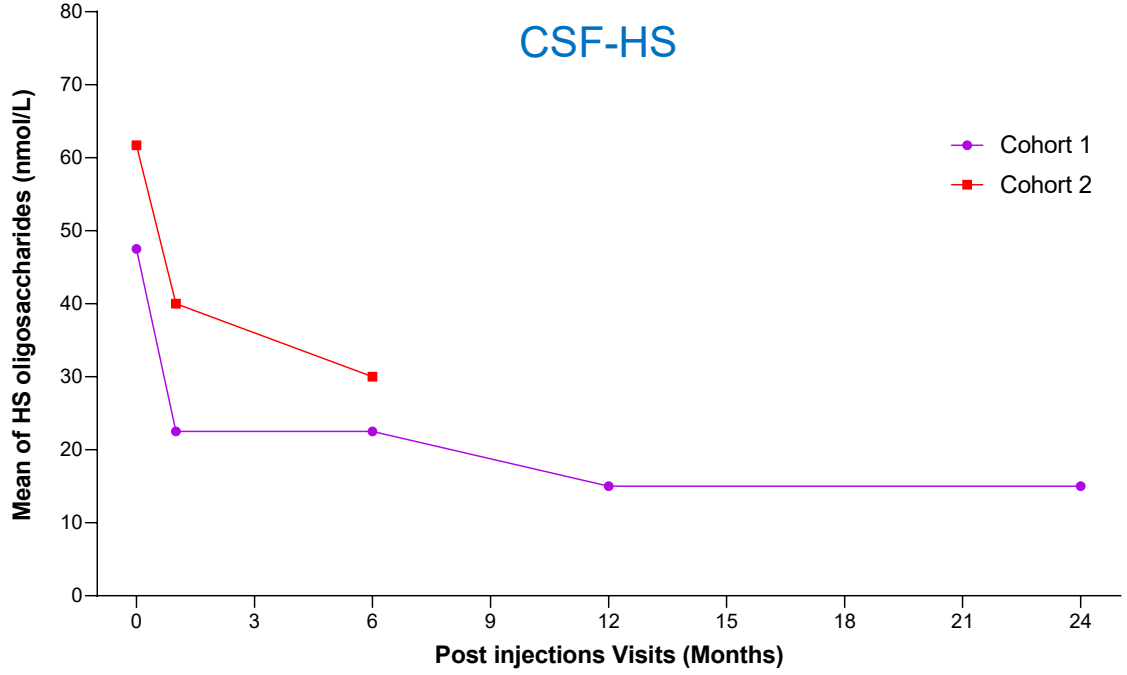
transpher A study



No Patients	Screening	Month 1	Month 6	Month 12	Month 24
Cohort 1	3	3	3	2	2
Cohort 2	3	3	3	3	3
Cohort 3	8	8	8	8	4

ABO-102 showed rapid, dose-dependent, and sustained reduction in CSF heparan sulfate

transpher B study

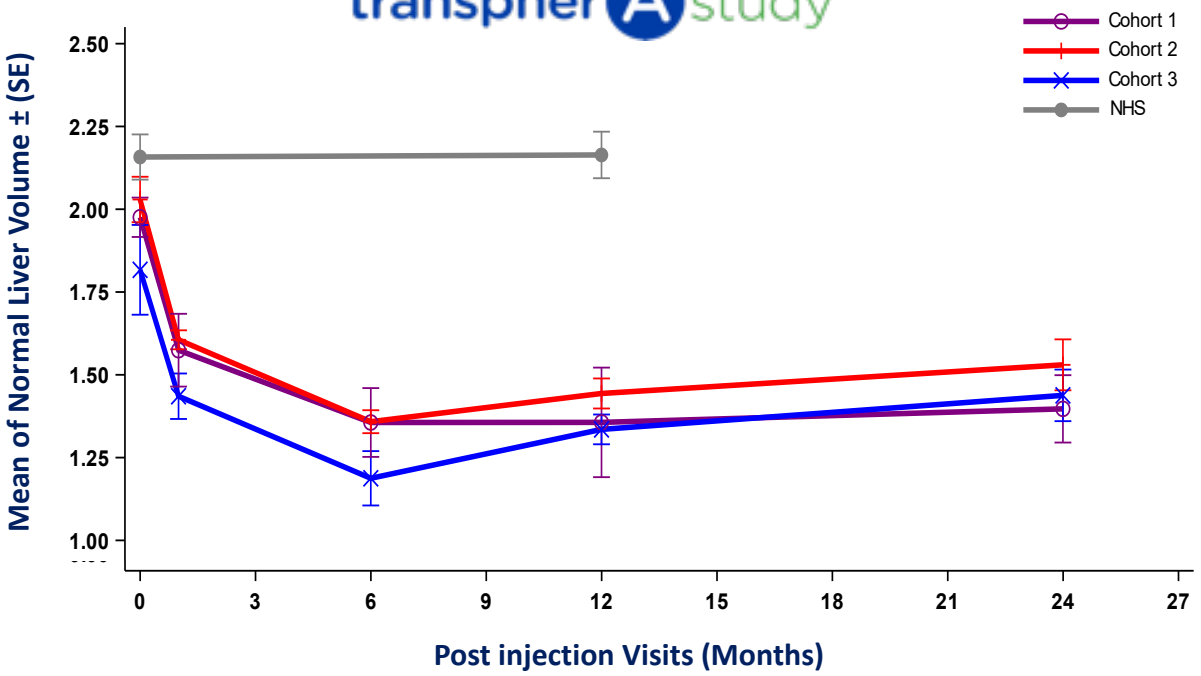


No Patients	Screening	Month 1	Month 6	Month 12	Month 24
Cohort 1	2	2	2	2	1
Cohort 2	3	4	1		

ABO-101 showed improvement in CSF heparan sulfate

Post-treatment Reduction in Liver Volume in MPS IIIA and IIIB

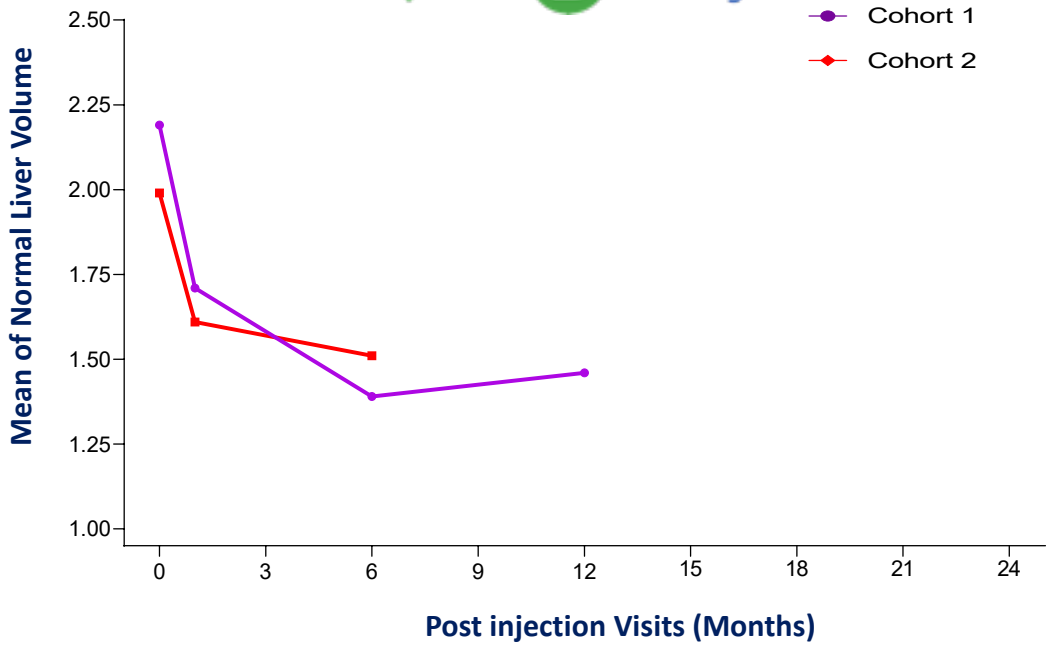
transpher A study



No Patients	Screening	Month 1	Month 6	Month 12	Month 24
Cohort 1	3	3	3	3	3
Cohort 2	3	3	3	3	3
Cohort 3	7	8	8	8	2

ABO-102 showed durable, dose-dependent reduction in liver volume post treatment

transpher B study



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

ABO-101 showed early signs of reduction in liver volume post treatment

NHS data: Truxal et al, 2016, Mol Genet Metab

Consistent Safety and Clinical Benefit in Phase 1/2 Studies with ABO-102 and ABO-101

transpher A study

ABO-102 was well-tolerated

- No infusion reactions
- No treatment-related SAEs
- No clinically significant AEs 0.5-50 months (n=16)

Preliminary evidence of clinical benefit

- Preservation of neurocognitive development in the 3 young patients treated <30 months of age in Cohort 3 (18-24 mos. of follow-up)
- Rapid and sustained, dose-related reduction in disease-specific biomarkers (e.g. heparan sulfate in cerebrospinal fluid and liver volume)

transpher B study

ABO-101 was well-tolerated

- No infusion reactions
- No treatment-related SAEs
- No clinically significant AEs 3-31 months (n=9)

Clear biologic effect

- Decreased CSF HS levels (up to 12 mos.)
- Reduction in plasma and urine HS and GAGs
- Reduction in liver volume
- Neurological assessments pending resumption of visits post-COVID

As of July 2020

In-House GMP Manufacturing

Fully-Integrated, Independent, and Scalable cGMP Manufacturing

Control of supply chain, including timelines and cost

- 26,000 sq. ft multi-purpose facility in Cleveland
- Scalable cGMP capacity
- State-of-the-art laboratories to support CMC development for process and analytics
- Experienced and trained staff in Quality, Validation, Process Development, and Assay Development

Clinical and commercial grade manufacturing capability

- EB-101 Phase 3 manufacture ongoing; retrovirus manufacturing in late stage development
- Scalable capacity to support EB-101 commercial launch
- 200L AAV manufacturing GMP upstream capacity; process development for 500L underway
- Supportive of development programs, capable of clinical and commercial AAV production

Anticipated Milestones

EB-101

- Complete enrollment in pivotal Phase 3 VIITAL™ study in H1 2021, depending upon impact from COVID-19 pandemic
- Topline results from VIITAL™ study in late-2021

ABO-102 and ABO-101 (AAV-based Gene Therapies)

- Complete enrollment in ABO-102 MPS IIIA and ABO-101 MPS IIIB studies in Q1 2021
- Updated neurocognitive data from MPS IIIA and clinical data from MPS IIIB studies in Q1 2021
- Update on U.S. regulatory pathway for ABO-102 in MPS IIIA

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