Passion, Perspective, and Progress
One Community’s Ongoing Drive to Develop a Potential Gene Therapy for a Rare Pediatric Disease

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Sanfilippo Syndrome or Mucopolysaccharidosis Type III (MPS III)

- Rare disease impacting an estimated 1 in 70,000
- Genetic disease caused by a missing enzyme that breaks down sugar molecules in cells
- Four types, designated as A, B, C, and D
- No visible symptoms at birth, kids achieve typical milestones until about age 2
- Significantly challenging to diagnose
- Progressive physical and mental loss – and, ultimately fatal
- 70% of children with this don’t see their 18th birthday
- No available treatment or therapy on the market
A Part of University of Minnesota History: Dr. Sylvester Sanfilippo

“His name is associated with a rare and fatal childhood disease, which he helped identify 50 years ago. His satisfaction was that he lived long enough to know that gene therapy breakthroughs could soon produce a cure.”

McEnroe, P. (2013, May 10) Obituary: Dr. Sylvester Sanfilippo, pediatrician and researcher, Star Tribune
Investigational Gene Therapies for MPS IIIA and MPS IIIB

- Provide correct form of SGSH or NAGLU gene
- Deliver via Adeno-Associated Virus (AAV)
- Administer single intravenous infusion
- Seek long-term expression
The Slow Burn and Build

1995
Dr. Neufeld

1995
Kirby Wilson

1996
Dr. Hong Zhao

1997
Dr. Muenzer

2003
Drs. Fu and McCarty

The Wilson Family

1997
Muenzer
Abeona Therapeutics: The Origin Story
From Private to Public: Celebrating 5 Years!

- Founded in March 2013 around technology for MPS IIIA & IIIB
- Completed merger in May 2015 with public company
- Re-emerged as Abeona Therapeutics
Collaborating on Clinical Trial Readiness

- Prospective Natural History Study for MPS IIIA and MPS IIIB

- 25 Participants in Total: 15 with MPS IIIA and 10 with MPS IIIB

- Assessments completed at 0, 6, and 12 months

- Established standards and outcome measures for future MPS III clinical trials
May 2016: Launch of Phase 1/2 Global Clinical Trial ABO-102 for MPS IIIA

Columbus, OH, USA

Santiago de Compostela, Spain

Adelaide, Australia
Dec 2017: Launch of Phase 1/2 Clinical Trial ABO-101 for MPS IIIB

Columbus, OH, USA

Santiago de Compostela, Spain

Adelaide, Australia
Collaborating on Future Clinical Studies
ABO-102 for MPS IIIA and ABO-101 for MPS IIIB

- Clinical investigation extending beyond the original Phase 1/2 trials
- Announcement on October 16, 2017
- 9 of 12 Founding Foundations
- $13.85 M Grant funded
- Abeona matched funding and Resources
- Extended studies planned for initiation yet this year
The Perspective I Have Gained

- When there is no light at the end of the tunnel... CLIMB HIGHER.

- The power of the parent [patient] is not to be underestimated.

- My threshold for misery and challenge has been significantly elevated.

- We are all connected.

- Tell your story.

- Be kind.
The Weight of a Mountain
“Alone we can do so little; together we can do so much.”
– Helen Keller

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