Hello to the International Leadership in the MPS III Community,

I write to you on this special day that has been designated for the global recognition and deserved focus on those living with or affected by rare disease. This is a wonderful opportunity to bring more light to the everyday impact that navigating the challenges presented by rare disease has, beyond the responsibilities of daily life. Given the focus of our research efforts, rare disease is quite constantly on our minds but providing this additional insight to our team has brought further meaning. Thank you to those who have shared your perspectives with us. The following is an update that comes after continued work with the 9 collaborating foundations for additional studies investigating extended populations of children affected by MPS IIIA and MPS IIIB.

This is a long message with the purpose of providing greater insight into the different MPS III ongoing and planned studies and other updates from Abeona. First, I'll start with the news that João Siffert, M.D. has been appointed as CEO after serving as the interim-CEO for several months. For the time, he will continue to serve also as head of Research and Development and Chief Medical Officer until those roles can be filled.

Next, I'll elaborate on items we have shared previously and summarize notable updates for the ongoing and planned studies for the investigation first of MPS IIIA programs and then MPS IIIB.

**ABT-001: Ongoing Phase 1/2 Clinical Trial for ABT-102 gene therapy for individuals diagnosed with MPS IIIA**

Our team met with both FDA and EMA regarding the progress to date on ABT-001, and it was decided that the eligibility criteria in ABT-001 Phase 1/2 study will be modified to enroll additional patients with greater function or who have experienced less neurologic decline. The listing in clinicaltrials.gov for this study is now revised with the following modifications. Those interested or with questions should contact their child's physician to determine eligibility.

- Inclusion criteria has been adjusted to include Age 6 months to 2 years or children older than 2 years with a minimum cognitive DQ of 60 or above calculated by Bayley Scales of Infant and Toddler Development
- Total number of anticipated participants adjusted from 16 to 22. This means we are continuing enrollment across sites for a total of up to 8 more participants in Cohort 3.
- Study completion date changed to Dec 2021 (from Dec 2020) to account for additional participants
- Exclusion criteria has been modified to account for:
    - Previous treatment by HSCT or participation in gene/cell therapy or ERT clinical trial
    - Any vaccination with viral attenuated vaccines less than 30 days prior to scheduled date of treatment
    - Subjects with positive response for the ELISPOT for T-cell responses to AAV9
- In addition to a primary outcome of safety, the change from baseline in the Age Equivalent Developmental Score (MSEL or Kaufman) compared with natural history study data will be measured
- Additional secondary outcomes have been incorporated which include:
    - Change from baseline in the Cognitive Age Equivalent compared to natural history study, calculated using Bayley Scales of Infant and Toddler Development
    - PedsQL total score
    - Parent quality of life, using a tool called the Parenting Stress Index
    - Analysis in plasma, saliva, urine, feces of vector shedding

- For outcomes already listed on ct.gov – change to 24 month timeframe (currently lists 12 months)

**ABT-003: Additional Clinical Trial for ABT-102 gene therapy for individuals diagnosed with MPS IIIA and have further disease progression**
As a result of the changes outlined above, we have adjusted the additional planned trial, ABT-003, to complement ABT-001 and to investigate the effects of ABO-102 in eligible patients with more progressed or increased neurological impact of the disease.

- Enrollment criteria to complement upper limits for ABT-001 for no eligibility gap between these two studies
- Inclusion/exclusion criteria and number of participants are not yet available for release
- Protocol submission to regulatory agencies is imminent for Spain, Australia, and the United States
- In parallel, preparations with sites in Spain, Australia, and the United States are well underway and will be limited to these countries
- Currently, it's not certain which country will be ready first but we will not wait for all three to initiate enrollment
- More information will be provided as available

**ABT-002: Ongoing Phase 1/2 Clinical Trial for ABT-102 gene therapy for individuals diagnosed with MPS IIIB**
This study is still early with participants with safety as the highest priority followed by getting data to inform on dose. There have been recent updates to the clinicaltrials.gov listing for this study, summarized below:

- Updated Responsible Party and main contact as Abeona
- Added exclusion criterion of treatment with prior ERT
- Modified age criteria for enrollment to 6 months and above
- Removed secondary endpoint of change in brain volume by MRI
- Added Spain as an active clinical site
- Efforts continue to initiate sites in UK, Germany, and France

Thank you again, as I realize this is a large amount of information. Lastly, I'll be using this format for sending group emails in order to provide people with the option to unsubscribe should they choose to no longer receive updates from Abeona.

Warmest regards,

Michelle

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