PEPGEN ADVOCACY- 3RD QUARTER NEWSLETTER

a quarterly newsletter for the patient advocacy organizations and communities engaged with PepGen

MYOTONIC DYSTROPHY TYPE 1

- *The FDA has Lifted the Clinical Hold on its Investigational New Drug Application for FREEDOM-DM1 Phase 1 Study of PGN-EDODM1 for Myotonic Dystrophy Type 1 (DM1). Click here for the official press release.*
- *The United States Food and Drug Administration (FDA) granted Orphan Drug Designation to PGN-EDODM1 for the treatment of DM1.*
- *PepGen received a No Objection Letter (NOL) from Health Canada for its Clinical Trial Application (CTA) to initiate the FREEDOM-DM1 Phase 1 study in people living with DM1*
  - *oFREEDOM-DM1 is a randomized, placebo- controlled, single ascending dose (SAD) study, intended to enroll approximately 24 adults living with DM1 to evaluate the safety and tolerability of PGN-EDODM1.*
- *PepGen presented at the annual Myotonic Dystrophy Foundation conference in Washington D.C. in September. It was wonderful to see new and familiar community members, and share pre-clinical and clinical updates for DM1. Details can be found here.*

"We have worked closely with the FDA to resolve their questions expeditiously and are pleased that the clinical hold on our DM1 program in the United States has been lifted. Our novel PGN-EDODM1 approach targets the toxic RNA species responsible for the disease, and the strength of our Enhanced Delivery Oligonucleotide (EDO) safety preclinical package has enabled us to launch this study in both the U.S. and internationally at doses that we believe could provide a clinically meaningful benefit to patients. We are very pleased after review of our existing safety data that the FDA agreed with our proposed starting dose of 5 mg/kg, moving up to 10 mg/kg and 20 mg/kg,"
James McArthur, Ph.D., President and CEO of PepGen.

DUCHENNE MUSCULAR DYSTROPHY

The CONNECT 1 and CONNECT 2 clinical studies are designed to evaluate the potential safety and efficacy of PGN-EDO51 in a broad population of boys and young men with DMD amenable to exon 51 skipping.

- *CONNECT1, our Canadian study will be open for recruitment soon, and details of the study will be shared with the community.*
- *Plans for CONNECT2, our global study are ongoing.*

Q3 PATIENT ADVOCACY HIGHLIGHTS

Celebrated World Duchenne Awareness Day by hosting a Jett Foundation webinar watch party with all staff

Hosted a DMD and DM1 Trivia Luncheon internally to spread awareness and educate staff

Hosted a “Duchenne Summer Movie Night” in our office cafeteria for all staff

Attended and raised over $1000 USD through internal activities for the MDA Walk, held at Castle Island in Boston MA

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