

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

Duchenne Muscular Dystrophy (DMD)

In May, PepGen received clearance by Health Canada to begin a Phase 2 clinical trial, CONNECT1-EDO51.

- CONNECT1-EDO51 is an open label, multiple ascending dose (MAD) clinical trial of PGN-EDO51 in people with DMD amenable to an exon 51 skipping approach.
- CONNECT1-EDO51 will assess dystrophin levels, exon skipping data and safety data following multiple doses of PGN-EDO51.
- PepGen expects to begin dosing patients with PGN-EDO51 in the second half of 2023.

CONNECT2-EDO51, a multinational study of PGN-EDO51, is planned.

Preclinical data for PGN-EDO51 and information about PepGen's clinical program were presented at the American Academy of Neurology meeting.

Myotonic Dystrophy Type 1 (DM1)

In May, PepGen filed an investigational new drug application for PGN-EDODM1 to initiate a clinical trial in people with DM1.

- PepGen received a clinical hold notice from the U.S. Food and Drug Administration (FDA).
- While disappointed, we will work closely with the FDA to lift the hold as quickly as possible.
- In parallel, we continue to pursue the advancement of PGN-EDODM1 into the clinic outside the U.S.

Preclinical data for PGN-EDODM1 and information about PepGen's clinical program were presented at the American Academy of Neurology meeting.



ADVOCACY HIGHLIGHTS

- PepGen talked with the myotonic dystrophy community on a “Meet the Drug Developers” [webinar with the Myotonic Dystrophy Foundation](#).
- [PepGen talked to the Canadian DMD community](#) about PGN-EDO51 and the start of the CONNECT1-EDO51 trial with Defeat Duchenne Canada.
- PepGen presented a summary of its DMD data and discussed our programs and the needs of the DMD community with families at our booth at the CureDuchenne meeting in San Diego.
- David Shortlidge, his family and his service dog, Bandit, visited PepGen’s new offices and labs and spoke to the PepGen Pioneers.



David, who lives with Duchenne muscular dystrophy, speaks with James McArthur, PhD, President and Chief Executive Officer, and Michelle Mellion, MD, Senior Vice President, Head of Clinical Development at the PepGen office.

Recent Corporate Highlights

In April 2023, PepGen gave an oral and poster presentation on the preclinical data supporting the development and advancement of PGN-EDODM1 into clinical studies, the design of the proposed Phase 1 clinical trial, FREEDOM-DM1, and preclinical and Phase 1 PGN-EDO51 data at the American Academy of Neurology 2023 Annual Meeting.

