



PEPGEN ADVOCACY

A QUARTERLY NEWSLETTER FOR THE PATIENT ADVOCACY ORGANIZATIONS AND COMMUNITIES ENGAGED WITH PEPGEN

DUCHENNE MUSCULAR DYSTROPHY

- January 8th: PepGen announced that we successfully dosed the first person in the CONNECT1-EDO51 study. Read more [here](#).
 - Preliminary data for the safety, exon 51 skipping and dystrophin production is expected in mid-2024 for the 5 mg/kg cohort.
- March 4th: PepGen announced clearance of our Clinical Trial Application (CTA) by the United Kingdom Medicines & Healthcare Products Regulatory Agency to begin CONNECT2-EDO51, a Phase 2 Clinical Trial designed to support potential accelerated approval of PGN-EDO51 in the USA for the Treatment of Duchenne Muscular Dystrophy. Read more [here](#).
- March 13th: PepGen received United States FDA Orphan Drug and Rare Pediatric Disease Designations for PGN-EDO51 for the Treatment of Duchenne Muscular Dystrophy. Read more [here](#).

MYOTONIC DYSTROPHY TYPE 1

- February 20th: PepGen received U.S. FDA Fast Track Designation for PGN-EDODM1 for the Treatment of Myotonic Dystrophy Type 1. Read more [here](#).

CONNECT1-EDO51

The CONNECT1-EDO51 clinical trial is a Phase 2 research study for people who are living with DMD. The study will test whether an investigational drug, called PGN-EDO51, is safe and tolerable for boys and men with Duchenne, amenable to exon 51 skipping. CONNECT1-EDO51 is actively enrolling in Canada. More information can be found by visiting the websites below:

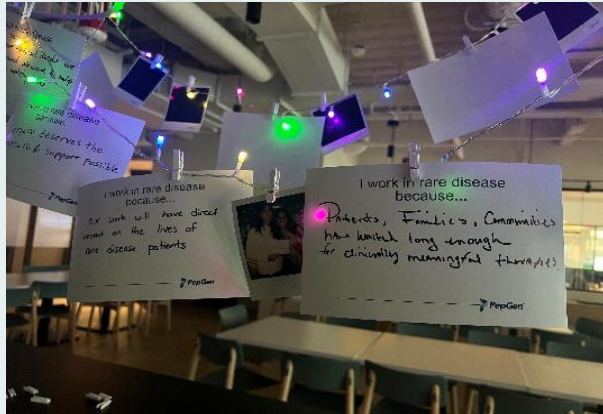
- [Connect1 Study Website](#)
- [Clinicaltrials.gov Listing](#)

FREEDOM-DM1

The FREEDOM-DM1 study is a Phase 1 study that is exploring whether a single dose of the investigational drug, called PGN-EDODM1, is safe and tolerable for people with DM1 compared to placebo. FREEDOM-DM1 is actively enrolling in the US and Canada. More information can be found by visiting the websites below:

- [FREEDOM-DM1 Website](#)
- [Clinicaltrials.gov Listing](#)

Q1 COMMUNITY HIGHLIGHTS



RARE DISEASE DAY

Staff gathered on Rare Disease Day to celebrate and honor those who are impacted by a rare disease by hosting a watch party of Jett Foundation's Thriving With Duchenne Rare Disease Day [webinar](#). We also filled out and shared cards describing why we work in rare disease and what we have learned from the communities that we serve. Check out PepGen lighting up for Rare [here!](#)



2024 CONFERENCE SEASON

PepGen recently attended the 2024 Muscular Dystrophy Association conference where we had a booth & presented posters on PGN-EDODM1 and PGN-EDO51. PepGen also attended the first of a six-part regional Myotonic Dystrophy Foundation conference series where we had a table & presented on PGN-EDODM1. Our team enjoyed connecting with the neuromuscular disorder community at both meetings.



DMD COMMUNITY SCIENCE DAYS

The Patient Advocacy team hosted our first annual Community Science Day for families living with DMD in January. Science Camp activities included a lab tour and STEM activities with our fellow PepGen staff. We look forward to hosting more sessions in the future.

Previous versions of this newsletter and their translations are available at pepgen.com/community

