



PEPGEN ADVOCACY

PepGen is greatly appreciative of the Duchenne Muscular Dystrophy (DMD) and Myotonic Dystrophy Type 1 (DM1) communities for an incredible year of engagement, learning, and progress. 2024 was a year of great achievement at PepGen. This year, we accomplished the following:

- Released the first data from CONNECT1-EDO51, a Phase 2 trial for DMD
- Received FDA orphan drug and rare pediatric disease designations for PGN-EDO51
- Received FDA fast track designation for PGN-EDODM1
- Learned from the community at both conferences and internal events

MYOTONIC DYSTROPHY TYPE 1

FREEDOM-DM1

The FREEDOM-DM1 study is a Phase 1 placebo-controlled study that is exploring whether a single dose of the investigational candidate, PGN-EDODM1, is safe and tolerable for people with DM1.

In November 2024, PepGen announced that the 10 mg/kg cohort of the FREEDOM-DM1 study is fully enrolled. We expect to report safety, splicing correction and functional outcome measures from both the 5 mg/kg and 10 mg/kg dose cohorts by the end of the first quarter of 2025 and expect to report results from the 15 mg/kg cohort in the second half of 2025.

FREEDOM-DM1 is actively enrolling 24 adults living with DM1 in the United States, Canada, and the United Kingdom. Visit the [FREEDOM-DM1 study website](#) and [Clinicaltrials.gov](#)



FREEDOM2-DM1

FREEDOM2-DM1 is a Phase 2 randomized, double-blind, placebo-controlled, multiple ascending dose clinical study evaluating the investigational candidate, PGN-EDODM1 in approximately 24 adults living with DM1 in Canada, the United Kingdom, and, subject to regulatory clearance, the United States.

PepGen dosed the first person in FREEDOM2-DM1 the fourth quarter of 2024.

Visit the [FREEDOM2-DM1 page on Clinicaltrials.gov](#)





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DUCHENNE MUSCULAR DYSTROPHY

CONNECT1-EDO51

CONNECT1-EDO51 is a Phase 2, open-label, multiple ascending dose (MAD) clinical trial, being conducted in Canada, evaluating the safety and tolerability of the investigational candidate, PGN-EDO51 in approximately 10 people at least 6-16 years old, with DMD amenable to exon 51 skipping.

In November 2024, PepGen announced the following protocol changes to the CONNECT1-EDO51 study based on learnings from the first cohort:

- Adjusting the eligible age group from 8 years of age and older to 6-16 years of age
- Adjusting the Performance of Upper Limb (PUL) test entry score from 3 to 4 for inclusion

In December 2024, PepGen announced that the 10 mg/kg cohort was fully enrolled. CONNECT1-EDO51 is actively enrolling in Canada. Visit the CONNECT1-EDO51 page on [Clinicaltrials.gov](https://clinicaltrials.gov)



CONNECT2-EDO51

CONNECT2-EDO51 is a multinational, placebo-controlled Phase 2 clinical trial for people who are living with DMD amenable to exon 51 skipping. The trial will study safety and tolerability, as well as levels of dystrophin in skeletal muscle, following monthly intravenous doses of the investigational candidate, PGN-EDO51.

CONNECT2-EDO51 is open in the United Kingdom. PepGen continues to engage with regulators in the European Union.

In December 2024, PepGen received a clinical hold notice from the U.S. Food and Drug Administration (FDA) regarding our Investigational New Drug (IND) application to initiate the CONNECT2-EDO51 clinical trial in the U.S. PepGen intends to work closely with the FDA to resolve the hold as quickly as possible.

Our open-label CONNECT1-EDO51 multiple ascending dose study of PGN-EDO51 in boys and young men living with DMD continues as planned in Canada.



PATIENT ADVOCACY HIGHLIGHTS



DM1 Lunch and Learn

In November, we hosted Dr. Thurman Wheeler, Ashley Segovia, Jesse Segovia, and Jeannine DeSoi for a DM1 lunch and learn. Our PepGen pioneers learned about DM1 from the unique perspectives of a physician, community member, and caregiver. We are always delighted to host community members at our office, and have our pioneers learn directly from them.

'Brothers' Movie Screening

In honor of Mental Health awareness month in October, we hosted a screening of the movie 'Brothers.' Created by Michael Norton, a DMD community member, 'Brothers' tells the story of his family's mental health challenges and DMD experiences. Learn more about Michael and 'Brothers' [here](#).

Action Duchenne Annual International Conference

November 8-9, Bassem Morcos, Medical Director, attended Action Duchenne's Annual International Conference in Hinckley, United Kingdom. Bassem talked to the community about how we use community insights in our drug development programs.

PPMD Drug Development Round Table

On November 15, Alayna Tress, Director of Patient Advocacy, attended Parent Project Muscular Dystrophy's Drug Development Round Table in New York City. Alayna had the great opportunity to speak about our clinical trial Diversity, Equity, and Inclusion (DEI) efforts, and learn from other roundtable participants.

DM1 Japan Community Presentation

On December 7, Jane Larkindale, VP of Clinical Science, presented to the advocacy group DM1 Japan. We were grateful to have the opportunity to speak about PepGen's technology, DM1 program, and FREEDOM trials. .

Social Media Spotlight

DMD Panel

In September, we celebrated World Duchenne Awareness Day by hosting an educational DMD panel. It was a truly inspiring event, and we are so grateful to the amazing panelists. Check out some of the highlights in [this video](#).

Professor PepGen

Earlier this year, we kicked off a new educational social media series called Professor PepGen! The series educates the communities we serve on the basics of clinical trials, in an effort to help them navigate the sometimes-complicated clinical trial landscape. [Check out Lesson 3 here](#).

