



PEPGEN'S 2024 MID-YEAR ADVOCACY UPDATE

DUCHENNE MUSCULAR DYSTROPHY

CONNECT1-EDO51



In July 2024, [PepGen reported clinical data](#) from the 5 mg/kg PGN-EDO51 dose cohort of CONNECT1-EDO51, including safety, exon 51 skipping, and dystrophin production data. CONNECT1-EDO51 is a Phase 2, open-label, multiple ascending dose (MAD) clinical trial, being conducted in Canada, evaluating safety and tolerability of PGN-EDO51 in approximately 10 people at least 8 years old, with DMD amenable to exon 51 skipping. **At the 5 mg/kg dose:**

- PGN-EDO51 was well tolerated by all study cohort participants through week 13
- Mean exon skipping in biceps was 2.15% greater at week 13 compared to baseline
- Mean muscle-adjusted dystrophin level was 1.49% of that of unaffected individuals, 0.70% increase after 4 doses, measured at week 13
- Mean absolute dystrophin level was 0.61% of that of unaffected individuals, 0.26% increase, after 4 doses, measured at week 13

CONNECT1-EDO51 is actively enrolling in Canada. Visit the [CONNECT1 study website](#) and [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 placebo-controlled study will study safety and tolerability, as well as levels of dystrophin in skeletal muscle, following monthly intravenous doses of PGN-EDO51 administered to participants with Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping.

CONNECT2-EDO51 is open in the [United Kingdom](#). The Company continues to engage with regulators in the European Union and expects to open the clinical trial in the United States by year-end, subject to regulatory clearance.

MYOTONIC DYSTROPHY TYPE 1

FREEDOM-DM1



The FREEDOM-DM1 study is a Phase 1 study that is exploring whether a single dose of the investigational drug, PGN-EDODM1, is safe and tolerable for people with DM1 compared to placebo. PepGen anticipates reporting clinical results from the at least the first cohort of the FREEDOM clinical trial, including safety, splicing correction, and functional outcome measures in Q4 of 2024.

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



On August 8, [PepGen announced](#) that both Health Canada and the United Kingdom Medicines and Healthcare products Regulatory Agency have cleared the Company's clinical trial application submissions for the FREEDOM2 trial, and PepGen expects to initiate participant dosing in the second half of 2024.

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



PATIENT ADVOCACY HIGHLIGHTS



WALK TO DEFEAT DUCHENNE

On May 22nd, PepGen Pioneers participated in Defeat Duchenne Canada's Annual Walk to Defeat Duchenne. Our staff met to honor the DMD community and took a lap around our office neighborhood in support of the efforts that Defeat Duchenne Canada make every day for those living with DMD.



Cure Duchenne FUTURES Annual Meeting

Our Director of Patient Advocacy, Alayna Tress, along with our Medical Director, Bassem Morcos recently attended the 2024 Cure Duchenne FUTURES Conference. Our team made connections both at our booth and during informative sessions throughout the conference discussing hot topics such as access, treatment options, and ways to incorporate more diversity within a busy clinical trial landscape. PepGen also participated in an industry [panel on exon skipping](#).



MDF Regional Conference Series

This past spring, Alayna Tress, Director of Patient Advocacy, Jenny Shoskes, Associate Director of Clinical Development, and Jane Larkindale, VP of Clinical Science traveled nationwide to the Myotonic Dystrophy Foundation's first ever Regional Conference Series. Our team members enjoyed interacting with DM1 community members all over the country and attending informative sessions as well as hosting our booth and presenting information on PGN-EDODM1 to the community.

PPMD's 30th Annual Meeting

Our Director of Patient Advocacy, Alayna Tress, and our Medical Director, Bassem Morcos, also attended PPMD's 30th Annual Meeting at the end of June. Our team had insightful conversations with DMD community members both at our booth and during a panel with other industry partners. In these conversations, PepGen discussed crucial topics such as Diversity & Inclusion, and how we can represent the DMD community members more broadly as we continue to enroll our clinical trials. Community members also provided valuable input on what is most important to them as they navigate their journeys with DMD with their families and support teams. PepGen was appreciative for the opportunity to participate in the ["Research Row" panel](#).