



PEPGEN ADVOCACY

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

Myotonic Dystrophy Type 1 (DM1)



In February 2025, PepGen reported initial clinical data from the 5 and 10 mg/kg dose cohorts of the FREEDOM-DM1 study. These included safety, tolerability and splicing (a measure of whether the drug is doing what it is intended to do) data as well as measurements of how much drug is getting into muscle. The results were as follows:

- Mean splicing correction was 12.3% at 5 mg/kg (n=6) and 29.1% at 10 mg/kg (n=4) 28 days post-dosing
- As dose increased from 5 mg/kg to 10 mg/kg, more drug was observed in muscle tissue. This increase was approximately consistent with the increase in splicing correction.
- No significant improvements were seen in measurements of function after a single dose, but these changes are anticipated to take longer than corrections in splicing
- PGN-EDODM1 was observed to have a favorable emerging safety profile in the 5 and 10 mg/kg cohorts
- One person had a treatment-related serious adverse event of abdominal pain in the 10 mg/kg cohort that was potentially confounded by use of another drug taken on the morning of PGN-EDODM1 dosing

FREEDOM is a Phase 1 placebo-controlled clinical study exploring whether a single dose of the investigational candidate, PGN-EDODM1, is safe and tolerable for people with DM1, aged 18-50.

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



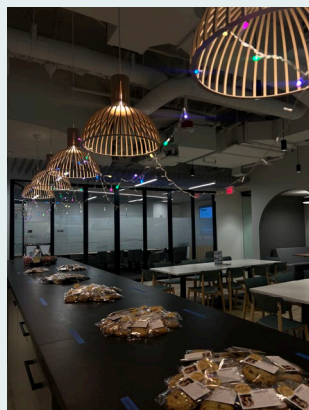
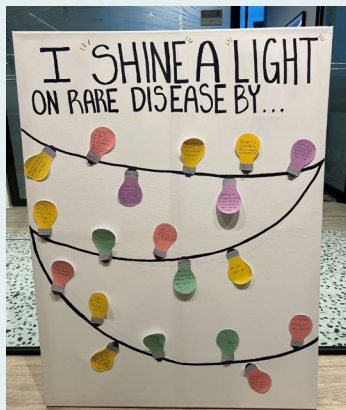
FREEDOM2-DM1 is a Phase 2 randomized, double-blind, placebo-controlled, multiple ascending dose clinical study evaluating increasing doses of the investigational candidate, PGN-EDODM1 in approximately 24 adults living with DM1 and is currently open in Canada and the United Kingdom.

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

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



PATIENT ADVOCACY HIGHLIGHTS

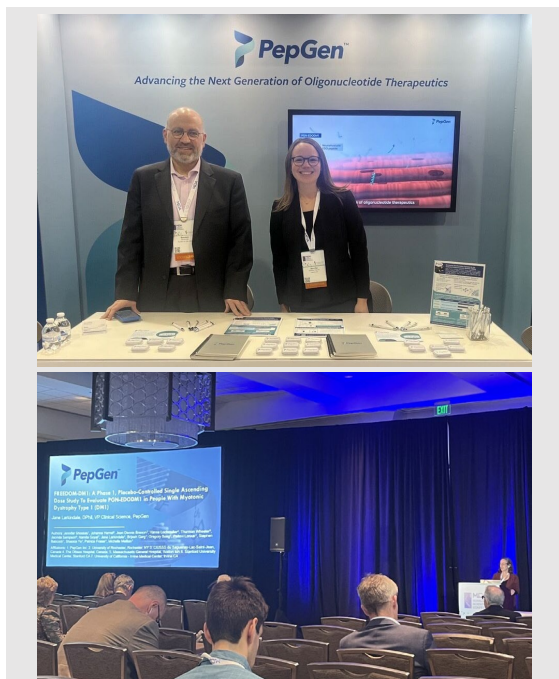


Rare Disease Day

On February 27, we celebrated Rare Disease Day in our office. During this event, we reflected on how we shine a light on rare diseases in a #LightUpForRare activity. The following day, our Pioneers tuned in to Jett Foundation's "Thriving with Duchenne" webinar. It was a great opportunity to hear from some of the incredible and inspirational members of the DMD community.

MDA Conference

March 16-19, we attended the Muscular Dystrophy Association's annual clinical and scientific conference in Dallas, TX. We discussed initial data from the ongoing CONNECT1-EDO51 clinical trial and the recently announced initial results from the FREEDOM-EDODM1 clinical trial. See our publications from the conference [here](#).



Social Media Spotlight

Our social media series, Professor PepGen, 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 6: What groups are involved in running a clinical trial?](#)



For US audiences only

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