



PEPGEN ADVOCACY

Myotonic Dystrophy Type 1 (DM1) *DM1 Program Updates*



PepGen expects to report data from the 15 mg/kg cohort of its FREEDOM-DM1 study in people with DM1 during the second half of 2025. FREEDOM is a Phase 1 placebo-controlled clinical study in approximately 24 adults living with DM1 exploring whether a single dose of the investigational candidate, PGN-EDODM1, is safe and tolerable for people with DM1, aged 18-50.

Earlier this year, PepGen reported initial clinical data from the 5 mg/kg and 10 mg/kg dose cohorts of the FREEDOM-DM1 study. A single 10 mg/kg dose resulted in mean mis-splicing correction of 29%, with a favorable emerging safety profile (data as reported from February 24, 2025).

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



PepGen expects to report data from the 5 mg/kg cohort of the multiple-dose study FREEDOM2-DM1 study in the first quarter of 2026. 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.

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

To learn more about PepGen and our development pipeline, visit www.pepgen.com.





PATIENT ENGAGEMENT HIGHLIGHTS

MDF Annual Conference

May 2nd-4th, we had the wonderful opportunity to attend the Myotonic Dystrophy Foundation's Annual Conference in Indianapolis, Indiana. Our Vice President of Clinical Science, Jane Larkindale D.Phil, presented an update on our DM1 program. It was an incredible weekend of connection with DM1 community members, scientists, and doctors.

Conference Recap with PepGen's VP of Clinical Science Jane Larkindale D. Phil

What was one new thing you learned at the MDF conference?

"That's difficult – scientifically, I learned about how the repeat expansion that causes DM1 may change in length in different parts of the body and over time. I also talked to a lot of people about childhood DM1, as that has not been studied as much as DM1 in adults, and people are only now considering how a new drug could be developed to treat children who live with DM1."

What did you share with the community at the MDF conference?

"We shared the data from the first two dose cohorts of FREEDOM-DM1, and our excitement about the mean mis-splicing correction of 29% after a single dose of PGN-EDODM1. Mis-splicing is the primary driver of DM1 symptoms, so we were very happy to see this result in conjunction with a favorable emerging safety profile."

Community Advisory Board

After the MDF conference, we participated in a community advisory board run by the European Patients Academy on Therapeutic Innovation (EUPATI). An insightful group of community advisors from all around the world shared invaluable feedback on our DM1 program. We discussed the advisors' experiences with DM1, how to effectively present data to the community, how to best support clinical trial participants, and more. Community advisory boards are a critical part of our clinical trial design process, and we are deeply grateful to all advisors who participated.

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. In April, Professor PepGen taught Lesson 6: What groups are involved in running a clinical trial? Check it out [here](#)!

Curious how our amazing PepGen Pioneers connect and relax? Well, in June, we kicked off summer on a Boston Duck Boat Tour. Check out some pictures from our day out [here](#)!

For US Audiences Only

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