A quarterly newsletter for the patient advocacy organizations and communities engaged with PepGen

DUCHENNE MUSCULAR DYSTROPHY- CONNECT1-EDO51 and CONNECT2-EDO51 clinical studies are planned for 2023

March 2023: PepGen announced new nonclinical data at the MDA conference in Dallas that highlights the potential for PGN-EDO51 to produce meaningful amounts of dystrophin:

- *mdx* mouse: A single, 30 mg/kg dose of PGN-EDO23 (mouse equivalent of PGN-EDO51) resulted in 52.5% exon 23 skipping and **22.5% unaffected dystrophin levels in the biceps** that was sustained for up to four weeks.
- *mdx* mouse: Repeat dosing of 30 mg/kg PGN-EDO23 once every 4 weeks for 4 doses resulted in 91.5% exon 23 skipping and 82.3% unaffected dystrophin levels demonstrating accumulation of dystrophin compared to single doses (**3.7-fold increase in dystrophin after 4 doses compared to 1**).
- Non-human primates (NHPs): A single-dose of PGN-EDO51, our clinical candidate, showed levels of exon skipping similar to that measured in our single dose healthy volunteer study. **Repeat dosing of PGN-EDO51 in NHPs resulted in 14-fold higher levels of exon skipping after 4 doses compared to 1** at 20 mg/kg.

PepGen is planning to initiate two clinical trials to assess the safety and efficacy of repeat-doses of PGN-EDO51 in boys and young men living with DMD.

- CONNECT1-EDO51 is a Phase 2 open-label MAD clinical trial to be initiated in Canada in 2023 that is expected to report dystrophin, exon skipping and safety data in 2024.
- CONNECT2-EDO51, a Phase 2, multinational randomized placebo-controlled clinical trial designed to potentially support a future accelerated approval pathway expected to be initiated in the second half of 2023.

MYOTONIC DYSTROPHY TYPE 1- FREEDOM-DM1 study of PGN-EDODM1 planned for 2023

March 2023: PepGen announced nonclinical data at the MDA conference in Dallas that shows that PGN-EDODM1 addresses the fundamental cause of DM1, **liberating MBNL1 protein from the repeat sequence and correcting splicing in cells** from a donor with DM1.

PepGen plans to initiate the FREEDOM-DM1 clinical trial in 2023, a Phase 1 multinational, placebo-controlled randomized single-ascending dose study to assess safety and explore markers of efficacy in people with DM1.

COMMUNITY ENGAGEMENT

PepGen strives to work with the DM1 and DMD communities to ensure that our programs meet the needs of those we hope to help. In the first quarter of 2023 we have:

- Held formal advisory committee meetings with representatives of the DM1 and DMD communities to learn more about how to best design our trials.
- Developed new materials for explaining our programs to community members.
- Completed our photojournalism project and displayed portraits of community members throughout our new office and lab space in Boston.