



PepGen to Present Late-Breaking Data from Novel Enhanced Delivery Oligonucleotide (EDO) Pipeline at 2021 Muscular Dystrophy Association Virtual Clinical and Scientific Conference

March 15, 2021

EDO platform demonstrates potentially best-in-class safety and efficacy profile in addressing DMD and DM1 disease phenotypes; advancing rapidly to the clinic with first-in-human studies expected to initiate in early 2022

Boston, Mass. and Oxford, UK (March 15, 2021) – PepGen, an emerging biotechnology company focused on developing transformative therapies for individuals living with neuromuscular and neurologic diseases, will present data for its novel enhanced delivery oligonucleotide (EDO) platform, and therapeutic programs for myotonic dystrophy type 1 (DM1) and Duchenne muscular dystrophy (DMD) at the 2021 Muscular Dystrophy Association (MDA) Virtual Clinical and Scientific Conference. Data from PepGen's DM1 program will be presented at a late-breaking session on Thursday, March 18, 2021 at 6 p.m. ET, and data from the company's DMD program will be available as a poster presentation throughout the conference from March 15 – 18, 2021.

"We are excited to share publicly, and for the first time, data from PepGen's transformational EDO platform addressing DM1 and DMD with the MDA community," said James McArthur, PhD, President and CEO of PepGen. "We are encouraged by strong preclinical results demonstrating well-tolerated *in vivo* efficacy that surpasses levels of exon skipping seen with other approaches to DMD, and the achievement of long-term correction of splicing defects in DM1 with a single treatment. Our team at PepGen is well-positioned to advance our lead candidate for DMD, PGN-EDO51, into the clinic in early 2022 and to accelerate the development of our DM1 program in late 2022."

In addition to the current DM1 and DMD pipeline, PepGen continues to advance additional disease-modifying oligonucleotide therapeutics for other DMD patient populations, and other severe neuromuscular and neurological diseases towards clinical development.

Title: *A novel enhanced delivery oligonucleotide (EDO) therapeutic demonstrates considerable potential in treating myotonic dystrophy type 1 (DM1)*

Date & Time: *Late-Breaking Presentation – Thursday, March 18, 2021 at 6 p.m. ET*

PepGen's DM1 candidate PGN-EDODM1 is a peptide conjugated disease-modifying oligonucleotide using EDO technology to efficiently deliver the therapeutic PMO to affected tissues, including cardiac and skeletal muscle cells and the central nervous system (CNS), by blocking MBLN1 binding to the toxic CUG hairpin loop. Data to be presented at MDA 2021 will show that a single dose of PGN-EDODM1 in mice was well-tolerated and demonstrated sustained correction of mRNA mis-splicing and complete amelioration of the DM1 myotonia phenotype for up to three months. The data will be presented by Ashling Holland, PhD, a Principal Scientist at PepGen.

[PepGen – MDA conference DM1 presentation](#)

Title: *A novel enhanced delivery oligonucleotide (EDO) therapeutic demonstrates considerable potential in treating Duchenne muscular dystrophy*

Date & Time: *Poster presentation available throughout the conference from March 15-18, 2021*

In spite of an existing approved drug for DMD exon 51 skipping, responsive patients still live with a tremendous unmet medical need due to inadequate therapeutic oligonucleotide delivery to affected tissues. PepGen's EDO technology has demonstrated exceptional dystrophin restoration in *mdx* disease model mice of up to 54% of wild-type levels in the tibialis anterior, and up to 12% in the heart following a single low intravenous dose of 10 mg/kg. Furthermore, non-human primate (NHP) studies have shown that PepGen's lead candidate, PGN-EDO51, achieves exon 51 skipping levels of 58% in the tibialis anterior and 19% in the heart with a single intravenous dose of 20 mg/kg. An extensive review of PGN-EDO51 biodistribution in NHP illustrated that high concentrations of therapeutic were achieved in critical tissues, including skeletal, cardiac and smooth muscle. PGN-EDO51 has also shown delivery to the CNS across the blood-brain barrier. The safety and efficacy data presented in DMD provide strong support for the advancement of PGN-EDO51 into the clinic in early 2022.

[PepGen – MDA conference DMD poster](#)

About PepGen

PepGen, Inc. is a biotechnology company focused on transforming the care of patients with neuromuscular diseases through the peptide-mediated delivery of nucleic acid therapeutics. Recognizing a deep need for a paradigm shift in genomic medicine, PepGen's proprietary enhanced delivery oligonucleotides (EDOs) strive to achieve clinical potential of these therapeutics by providing reliable, safe and efficacious delivery to critical disease targets. PepGen raised a \$45 million Series A in December 2020 with RA Capital Management as the lead investor; Oxford Sciences Innovation (OSI), CureDuchenne Ventures and the University of Oxford also participated in the round. The company was founded in 2018 with an initial seed investment from OSI. For more information, visit <https://pepgen.com/>.

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