PepGen Announces First Participant Dosed in a Phase 1 Clinical Trial of PGN-EDO51 for the Treatment of Duchenne Muscular Dystrophy

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Initiation of Phase 1 Study Marks PepGen's Transition to a Clinical Stage Company

This Phase 1 Healthy Normal Volunteer (HNV) Study will Evaluate Safety and Tolerability as the Primary Objective, and will Begin to Evaluate the Delivery and Target Engagement of PGN-EDO51 to Enable Subsequent Patient Trials

BOSTON, April 6, 2022 – PepGen, Inc., advancing the next generation of oligonucleotide therapies with the goal of transforming the treatment of severe neuromuscular and neurological diseases, today announced a critical clinical milestone for PGN-EDO51, the company's lead product candidate for the treatment of Duchenne muscular dystrophy (DMD) patients whose mutations are amenable to an exon 51 skipping approach. Following the acceptance of PepGen's Clinical Trial Application (CTA) by Health Canada last month, the company has now dosed the first healthy volunteer participant in a Phase 1 single ascending dose (SAD) clinical trial of PGN-EDO51.

PGN-EDO51 utilizes PepGen's proprietary Enhanced Delivery Oligonucleotide (EDO) platform technology, which leverages a novel cell-penetrating peptide designed to improve the delivery, uptake and activity of therapeutic oligonucleotides. PGN-EDO51 utilizes the EDO technology to deliver an exon 51 skipping oligonucleotide, which allows mutations in the gene to be bypassed producing a slightly smaller, yet functional version of the dystrophin protein. As individuals with DMD lack this critical protein, such an approach has the potential to be transformative for the 13% of patients who are amenable to treatment with an exon 51-skipping oligonucleotide.

"DMD is a devastating rare disease that places a significant burden on the lives of patients and their families. There are limited therapeutic options available, and our mission is to deliver a life-changing therapeutic to this community in need," stated James McArthur, Ph.D., President and CEO of PepGen. "The initiation of our Phase 1 HNV study is a significant milestone for PepGen and marks our transition to a clinical stage company. We look forward to reporting topline data from this trial by the end of 2022 and expect that this data will enable our future studies in DMD patients to initiate in early 2023."

PepGen's Phase 1 HNV study for PGN-EDO51 is an SAD trial evaluating the safety and tolerability of this product candidate in approximately 40 healthy adult males. Following IV administration of PGN-EDO51, trial participants will be evaluated by a safety review committee prior to progression to the next dose level. The primary objective for this study will be to assess the safety and tolerability of PGN-EDO51. The pharmacokinetics of PGN-EDO51 and exon 51 skipping in bicep biopsies from healthy volunteers will also be evaluated as secondary exploratory endpoints to provide a baseline for this therapeutic in subsequent clinical trials in DMD patients. The trial will be conducted in Canada.

About PGN-EDO51

PGN-EDO51, PepGen's lead clinical candidate for the treatment of Duchenne muscular dystrophy (DMD), utilizes the company's proprietary Enhanced Delivery Oligonucleotide (EDO) technology to deliver a therapeutic oligonucleotide that targets the root cause of this devastating disease. PGN-EDO51 is designed to skip exon 51 of the dystrophin transcript, an established therapeutic target for approximately 13% of DMD patients, thereby restoring the open reading frame and enabling the production of a truncated, yet functional dystrophin protein. In preclinical studies PepGen observed that treatment of non-human primates with PGN-EDO51 resulted in greater levels of exon-skipping when compared to the most clinically-advanced peptide-conjugated oligonucleotide therapeutic in head-to-head studies, which could translate to higher levels of dystrophin production in patients. Cross-trial comparisons with publicly-available data also suggested that PGN-EDO51 treatment yields the highest rate of exon 51 skipping reported by any approved therapeutic or known development candidate.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a recessive, X-linked muscle-wasting disease that predominantly affects males. This debilitating disease arises due to genetic mutations in the gene encoding dystrophin, a protein critical for healthy muscle function, and is one of the most prevalent rare genetic diseases, with an incidence rate of approximately one in every 3,500 to 5,000 male births. DMD is characterized by progressive muscle weakness, which leads to patients losing the ability to walk, a loss of upper body function, cardiac issues and difficulties breathing. DMD is invariably fatal by young adulthood. Despite significant advances in treatments for this devastating disease, current therapies are limited by poor delivery to muscle tissue and have yet to establish meaningful clinical benefit for DMD patients.

About PepGen

PepGen, Inc. is a biotechnology company advancing next-generation oligonucleotide therapies with the goal of transforming the treatment of severe neuromuscular and neurological diseases. PepGen's proprietary Enhanced Delivery Oligonucleotides (EDOs) are designed to target the underlying causes of rare genetic diseases, such as Duchenne muscular dystrophy (DMD) and myotonic dystrophy type 1 (DM1), in a safe and effective manner. In preclinical studies, PepGen's enhanced delivery peptides demonstrated highly effective cell penetration and delivery of therapeutic candidates to multiple tissue types, including cardiac tissue. PepGen is backed by a strong syndicate of investors including RA Capital Management, Oxford Sciences Enterprises, and others. The company is headquartered in Boston, Mass. For more information, visit www.pepgen.com or follow PepGen on Twitter and LinkedIn.

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