PepGen Inc. Announces Clearance of CTA by Health Canada to Begin the FREEDOM-DM1 Phase 1 Study of PGN-EDODM1 in Patients with Myotonic Dystrophy Type 1

September 6, 2023

The U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation to PGN-EDODM1 for the treatment of Myotonic Dystrophy Type 1 (DM1)

BOSTON, Sept. 06, 2023 (GLOBE NEWSWIRE) -- PepGen Inc. (Nasdaq: PEPG), a clinical-stage biotechnology company advancing the next generation of oligonucleotide therapies with the goal of transforming the treatment of severe neuromuscular and neurological diseases, today announced it has received a No Objection Letter (NOL) from Health Canada for its Clinical Trial Application (CTA) to initiate the FREEDOM-DM1 Phase 1 study of PGN-EDODM1 in patients with myotonic dystrophy type 1 (DM1). In addition, the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to PGN-EDODM1 for the treatment of myotonic dystrophy type 1.

The FREEDOM-DM1 clinical trial is a randomized, placebo-controlled, single ascending dose (SAD) study, intended to enroll approximately 24 adult patients with DM1 to evaluate the safety and tolerability of PGN-EDODM1. In addition to safety, oligonucleotide muscle concentrations and PGN-EDODM1 correction of mis-splicing of transcripts, clinical functional outcomes impacted in DM1 will also be assessed 28 days and at later time points following a single dose of PGN-EDODM1. The approved dose escalation from the initial 5 mg/kg dose cohort will be to 10 mg/kg and then to 20 mg/kg. The decision to escalate to the next dose will be determined based on clinical data from the prior dose cohorts

“We are very pleased to have received Health Canada's clearance of PepGen’s latest CTA for PGN-EDODM1, which allows PepGen to continue striving toward developing truly transformative medicines that have the potential to bring clinically meaningful outcomes to individuals with neuromuscular and neurological diseases. We expect to report initial results from this study in 2024,” said James McArthur, Ph.D., President and CEO of PepGen. “People living with DM1 have a significant unmet medical need with currently no approved disease-modifying therapies, and the initiation of this trial plus the FDA’s granting of orphan drug designation for PGN-EDODM1 signifies another important step toward addressing that need.”

Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug product intended to treat a rare disease or condition, which is generally a disease or condition that affects either (i) fewer than 200,000 individuals in the United States, or (ii) more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making the product available in the United States for this type of disease or condition will be recovered from sales of the product.

As reported earlier today, PepGen will present new preclinical data on PGN-EDODM1 at the 6th Ottawa International Conference on Neuromuscular Disease and Biology (NMD) being held on September 7-9, 2023 in Ottawa, Canada and at the 2023 Myotonic Dystrophy Foundation (MDF) Annual Conference being held on September 7-9, 2023 in Washington, D.C.

As previously communicated, PepGen received a clinical hold notice from the FDA regarding its Investigational New Drug (IND) application to initiate a Phase 1 study of PGN-EDODM1 in patients with DM1. PepGen continues to work closely with the FDA to resolve the clinical hold in the United States as quickly as feasible.

About PGN-EDODM1

Our most advanced product candidate in the DM1 program, PGN-EDODM1, is designed to deliver a peptide conjugated antisense oligonucleotide (ASO) to restore cellular function. DM1 is caused by CUG repeats that form hairpin loops in the DMPK RNA that cause sequestering of the MBNL1 protein, a key RNA processing factor protein. This results in downstream mis-splicing events and aberrant expression of many proteins that play a critical role in muscle contraction and relaxation. By blocking the toxic CUG repeats, the goal of PGN-EDODM1 is to restore functional downstream splicing and muscle function.

About myotonic dystrophy type 1 (DM1)

Myotonic dystrophy type 1, or DM1, is a genetic disorder that affects many parts of the body. DM1 (also known as Steinert’s disease) is the most prevalent form of the condition and generally the most severe. DM1 affects an estimated 40,000 people in the U.S. and 70,000 in the EU. With an average life expectancy of 45-60 years, patients typically present with myotonia (stiff or contracted muscles), muscle weakness, and cardiac and respiratory abnormalities. Many patients also experience excessive daytime sleepiness, fatigue, and issues with gastrointestinal or cognitive dysfunction that greatly affect their quality of life. The congenital form of DM1 is the most severe version and can be life-threatening.

About PepGen

PepGen Inc. is a clinical-stage biotechnology company advancing the next-generation of oligonucleotide therapies with the goal of transforming the treatment of severe neuromuscular and neurological diseases. PepGen’s Enhanced Delivery Oligonucleotide, or EDO, platform is founded on over a decade of research and development and leverages cell-penetrating peptides to improve the uptake and activity of conjugated oligonucleotide therapeutics. Using these EDO peptides, we are generating a pipeline of oligonucleotide therapeutic candidates that are designed to target the root cause of serious diseases.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. These statements may be identified by words such as “aims,” “anticipates,” “believes,” “could,” “estimates,” “expects,” “forecasts,” “goal,” “intends,” “may,” “plans,” “possible,” “potential,” “seeks,” “will,” and variations of these words or similar expressions that are intended to identify forward-looking statements. Any such statements in this press release that are not statements of historical fact may be deemed to be forward-looking statements. These forward-looking statements include, without limitation, statements regarding the potential therapeutic benefits and safety profile of our product candidates, initiation and timeline of the Phase 1 study of PGN-EDODM1, the possible benefits conferred by orphan drug designation, and planned regulatory interactions in the U.S. and elsewhere.

Any forward-looking statements in this press release are based on current expectations, estimates and projections only as of the date of this release and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to that we may experience delays or fail to successfully initiate or complete our planned clinical trials for P PGN-EDODM1; our interpretation of clinical and preclinical study results may be incorrect; our product candidates may not be safe and effective; there may be delays in regulatory review, clearance to proceed or approval by regulatory authorities with respect to our programs, including clearance to commence planned clinical studies of our product candidates, including PGN-EDODM1, and to resolve the FDA clinical hold for the proposed Phase 1 clinical trial of PGN-EDODM1; changes in regulatory framework that are out of our control; and we are dependent on third parties for some or all aspects of our product manufacturing, research and preclinical and clinical testing. Additional risks concerning PepGen's programs and operations are described in our most recent annual report on Form 10-K and quarterly report on Form 10-Q that are filed with the SEC. PepGen explicitly disclaims any obligation to update any forward-looking statements except to the extent required by law.

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