PepGen Receives U.S. FDA Orphan Drug and Rare Pediatric Disease Designations for PGN-EDO51 for the Treatment of Duchenne Muscular Dystrophy

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BOSTON, March 13, 2024 (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 that the U.S. Food and Drug Administration (FDA) granted both orphan drug and rare pediatric disease designations for PGN-EDO51, an investigational therapeutic for Duchenne muscular dystrophy (DMD) patients whose mutations are amenable to an exon 51 skipping approach. PepGen is evaluating PGN-EDO51 for the treatment of DMD in the ongoing CONNECT1 Phase 2 trial, and expects to begin enrolling patients in the CONNECT2 Phase 2 trial later this year.

“Receiving these FDA designations for PGN-EDO51 emphasizes the critical demand for novel and enhanced therapeutic options for people living with DMD,” said James McArthur, Ph.D., President and CEO of PepGen. “With our CONNECT1 Phase 2 trial underway, we look forward to reporting preliminary data this year.”

Orphan designation is granted by the FDA to advance the evaluation and development of new treatments 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 on the basis of certain facts and circumstances. Under the Orphan Drug Act, the FDA may provide sponsors incentives including tax credits for qualified clinical trials, FDA user-fee benefits, and seven years of market exclusivity in the United States after approval.

The FDA defines a "rare pediatric disease" as a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years. Under the Rare Pediatric Disease program, the FDA may award priority review vouchers to sponsors who have been granted rare pediatic disease designation for its drug. A sponsor who has been granted the designation and receives an approval for a drug for a rare pediatric disease may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product.

For more information on these designations, please visit the FDA website’s Orphan Drug and Rare Pediatric Disease webpages.

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 is designed to target 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 aiming to restore the open reading frame and enabling the production of a truncated, yet functional dystrophin protein.

In preclinical studies, PepGen observed that administration of PGN-EDO51 to NHPs resulted in greater levels of exon-skipping when compared in head-to-head studies against a molecule that we believe is structurally equivalent to the most clinically-advanced peptide-conjugated oligonucleotide therapeutic candidate. Higher levels of exon skipping may translate to higher levels of dystrophin production in patients. PGN-EDO51 also exhibited the highest level of exon 51 skipping in nonhuman primate skeletal muscles, including the diaphragm, reported for any approved therapeutic or known development candidate at tolerable target dose levels, based on cross-trial comparisons of publicly available data.

In a Phase 1 Healthy Volunteer single ascending dose study, PGN-EDO51 exhibited six-times higher mean exon 51 skipping than the naked oligonucleotide based on cross-trial comparisons of publicly available data.

About Duchenne Muscular Dystrophy (DMD)

Duchenne muscular dystrophy (DMD) is an X-linked recessive, progressive, muscle-wasting disease that predominantly affects males. This debilitating disease is caused by genetic mutations in the gene encoding dystrophin, a protein necsessary for normal 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 exon skipping therapies are thought to have limited impact on disease progression due to low levels (<5%) of dystrophin production.

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 therapeutic potential and safety profile of our product candidates, including PGN-EDO51, our technology, including our EDO platform, the design, initiation and conduct of clinical trials, including the CONNECT1-EDO51 and CONNECT2-EDO51 trials, expected timelines and preliminary data reports from our clinical trials, including the CONNECT1-EDO51 and CONNECT2-EDO51 trials, regulatory interactions, and our financial resources and cash runway.

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 risks related to: delays or failure to successfully initiate or complete our ongoing and planned development activities for our product candidates, including PGN-EDO51; our ability to enroll patients in our clinical trials, including CONNECT1-EDO51 and CONNECT2-EDO51; our interpretation of clinical and preclinical study results may be incorrect, or that we may not observe the levels of therapeutic activity in clinical testing that we anticipate based on prior clinical or preclinical results, including for PGN-EDO51; our product candidates, including PGN-EDO51, may not be safe and effective or otherwise demonstrate safety and efficacy in our clinical trials; adverse outcomes from our regulatory interactions, including 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, such as PGN-EDO51, or other regulatory feedback requiring modifications to our development programs, including in each case with respect to CONNECT1-EDO51 and CONNECT2-EDO51; changes in regulatory framework that are out of our control; unexpected increases in the expenses associated with our development activities or other events that adversely impact our financial resources and cash runway; and our dependence 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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