ReveraGen

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ReveraGen BioPharma Selected as Awardee for Inaugural National Institutes of Health's Therapeutics for Rare and Neglected Diseases Program for its Novel Dissociative Glucocorticoid Analogue

ReveraGen's VBP15 to be developed in collaboration with NIH for treatment of Duchenne Muscular Dystrophy

Rockville, MD – ReveraGen BioPharma, Inc., a biopharmaceutical company developing alternatives to traditional steroid compounds, including dissociative glucocorticoid analogues for use in muscular dystrophy, has been selected by the National Institutes of Health's (NIH) Therapeutics for Rare and Neglected Diseases (TRND) program, to partner on the development of a new treatment of Duchenne muscular dystrophy (DMD).

Chronic treatment with traditional glucocorticoids, such as prednisone, is the prevailing standard of care in DMD, but this treatment is limited due to side effects. ReveraGen's VBP15, a novel dissociative glucocorticoid, is designed to maintain the efficacy of traditional steroids, yet elicit fewer side effects. Preliminary pre-clinical research with VBP15 suggests that it could have potent NFkB inhibition, anti-inflammatory activity, and improvement in strength in pre-clinical models of Duchenne muscular dystrophy.

"This partnership with NIH/TRND provides ReveraGen with the opportunity to facilitate the next steps in the research and development of VBP15," said Edward Connor, MD, a member of the Board of Directors and interim CEO of ReveraGen BioPharma. "Public-private partnerships are an important way of advancing therapeutics in orphan diseases and the team at TRND provides significant experience in orphan drug development."

The TRND program is a new initiative for NIH to assist in the development of new drugs for rare and neglected diseases. To develop new medicines, TRND establishes partnerships with academic, government, biopharmaceutical, and patient advocacy groups to focus on the discovery, optimization, and pre-clinical testing of new drugs. VBP15 was selected through a national competitive process as one of a select few inaugural compounds in the TRND program.

"TRND selected ReveraGen's work on VPB15 because of its potential to move forward into later stage development in diseases for which there is little or no therapy," said John McKew, PhD, chief of TRND's Therapeutic Development Branch and director of TRND. "Our staff is equipped with the drug development expertise and the laboratory resources to assist its partners in advancing promising candidates for rare disorders through the drug development process."

Led by ReveraGen's principal investigator Erica Reeves, PhD, along with company founders Eric Hoffman, PhD, John McCall, PhD, and Kanneboyina Nagaraju, DVM, PhD, the TRND partnership will move to finalize VPB15 process development and conduct pre-clinical evaluation, including investigational new drug-enabling studies. Beth Burnside, PhD, who recently joined ReveraGen's leadership team as senior vice president of Development and Operations, brings a broad background in drug development to the group and will also be a scientific leader in the collaboration.

Strongly echoing Connor's sentiments regarding the importance of partnerships in drug development, Muscular Dystrophy Association Chairman of the Board R. Rodney Howell, MD said, "MDA is delighted to see that its initial investment in the early stages of development of VBP15 has resulted in positive results such that the NIH/TRND program has decided to invest resources into further development of this as a therapy for individuals with Duchenne muscular dystrophy."

"Glucocorticoids are important in the treatment of many diseases, yet their broad mechanism of action has been challenging to refine to more targeted drugs," offered Dr. Peter Kim, director of the Sheikh Zayed Institute for Pediatric Surgical Innovation at Children's National Medical Center in Washington, DC, where much of the VBP15 research is currently conducted. "The pioneering work that the ReveraGen staff has been doing on these dissociative glucocorticoids is the type of innovative research we foster at the Sheikh Zayed Institute. We look forward to seeing the work that comes from ReveraGen's partnership with TRND."

ReveraGen is also supported by investments from the Muscular Dystrophy Association's Venture Philanthropy and the Foundation to Eradicate Duchenne. Joel Wood, a parent of a child with Duchenne, and Director of Foundation to Eradicate Duchenne, commented, "I have seen first-hand the devastating side effects from long-term glucocorticoid use – my son's bones became so brittle that fractures put him in a wheelchair. I am happy to see the progress in ReveraGen's work and the collaboration with TRND in assessing these investigational drugs."

ReveraGen and TRND personnel are now working together as a joint project team on several aspects of product development for VBP15, with the goal of conducting the necessary studies to enable an IND Application.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is an inherited, rapidly progressive form of muscular deterioration affecting approximately 1 in 3,500 male births worldwide; there is no known cure. Boys with DMD lose muscle strength and function during the first decade of life, typically becoming non-ambulatory by their second decade. Death among DMD patients is common as early as age 25, typically from lung muscle dysfunction. Daily prednisone (a glucocorticoid) is considered a standard of care in patients with DMD. Studies have shown that prednisone treatment is associated with improved muscle function and delay in progression of the disease. Long-term glucocorticoid use, however, may be problematic due to significant systemic side effects that may cause the therapy to be discontinued.

About ReveraGen BioPharma, Inc.

ReveraGen BioPharma, Inc., is an early-stage biopharmaceutical company focused on the development of novel dissociative glucocorticoid compounds for the treatment of Duchenne muscular dystrophy and other inflammatory diseases that fulfill substantial medical needs for improvement in glucocorticoid therapy. ReveraGen was founded in 2008 through a technology transfer from Children's National Medical Center, as part of a commitment to translational research into new and innovative medication to address unmet medical needs in rare and orphan diseases in children. For more information on ReveraGen, please visit www.reveragen.com.