VISION-DMD

Vamorolone (VBP15) Top Line Data Shows Improvements in Strength and Endurance in Boys with Duchenne Muscular Dystrophy

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ReveraGen BioPharma, with the Cooperative International Neuromuscular Research Group (CINRG), conducted an international clinical trial of vamorolone at 11 CINRG sites in 6 countries (US, Canada, Australia, Israel, UK, and Sweden).

This was an open-label, sequential dose-ranging study evaluating 4 dose levels (0.25, 0.75, 2.0, and 6.0 mg/kg/day) of vamorolone in 48 boys with DMD, 4-7 years old. The initial two-week treatment period was followed by a 6-month extension at the same dose levels (clinicaltrials.gov; NCT02760264, NCT02760277).

The study showed dose-related improvements of multiple function tests of strength and endurance. Clinical efficacy was demonstrated at the 2 mg/kg/day and the 6 mg/kg/day vamorolone doses compared to data from untreated patients in the CINRG Duchenne Natural History Study and was of similar magnitude to that seen in prednisone-treated patients in the historical CINRG prednisone trial.

Vamorolone exhibited limited metabolic disturbance and bone turnover change at doses of 2.0 or 6.0 mg/kg/day.

In animal models of DMD, vamorolone was shown to be a dissociative steroid. It displayed less side effects than traditional steroids and was superior for muscle strength and membrane stabilization, two key efficacy aspects specific for DMD therapy.

“These early clinical data are encouraging,” said Paula Clemens, MD, Professor of Neurology at the University of Pittsburgh School of Medicine, Study Chair, and Medical Director of the CINRG group. “We are grateful for the participation of patients and their families, and we owe a great deal of the success of the program to their efforts,” she continued.

“The side effects of anti-inflammatory steroids often detract from patient quality of life, particularly in children,” noted Eric Hoffman, PhD, Professor of Pharmaceutical Sciences at Binghamton University – SUNY and CEO of ReveraGen BioPharma, the clinical trial Sponsor. “The potential of vamorolone to reduce this burden may improve the quality of life of children with DMD and their families,” he said. Adverse effects were measured in the trial.

Vamorolone has been developed under a venture philanthropy model, with funding from a dozen international non-profit foundations, as well as US and EU governments. “The National Center for Advancing Translational Sciences — NCATS — at the National Institutes of Health partnered with ReveraGen to aid in vamorolone’s pre-clinical development,” Hoffman said. “Clinical trial support from National Institute of Neurological Disorders and Stroke – NINDS – made the DMD trials possible.” Joel Wood, President, Foundation to Eradicate Duchenne, noted, “I have personally seen both the good and the ugly of steroids; the potential for having a safer alternative is a major step forward for our families.”

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About Duchenne muscular dystrophy

Duchenne muscular dystrophy (DMD) is a rare genetic disease that predominantly affects young boys. Loss of the large dystrophin protein in muscle leads to persistent damage to myofibers. DMD is a progressive disease, with gradual deterioration of muscle and ensuing weakness over 20 years, leading to loss of walking abilities, and shortened lifespan.

About ReveraGen BioPharma

ReveraGen was founded in 2008 to develop first-in-class dissociative steroidal drugs for Duchenne muscular dystrophy and other chronic inflammatory disorders. The development of ReveraGen's lead compound, vamorolone, has been supported through partnerships with foundations worldwide, including Muscular Dystrophy Association USA, Parent Project Muscular Dystrophy, Foundation to Eradicate Duchenne, Save Our Sons, JoiningJack, Action Duchenne, CureDuchenne, Ryan's Quest, Alex's Wish, DuchenneUK, Pietro's Fight, Michael's Cause, and Duchenne Research Fund. ReveraGen has also received generous support from the US Department of Defense CDMRP, National Institutes of Health (NCATS, NINDS, NIAMS), and European Commission (Horizons 2020). www.reveragen.com

About vamorolone

Vamorolone (previously VBP15) binds to the same cellular receptors as traditional glucocorticoid drugs, but unlike these, does not enable dimerization of the drug/receptor complexes. This leads to a separation (dissociation) of anti-inflammatory benefit from safety concerns. In published Phase I studies in healthy adult volunteers, vamorolone showed reduction or loss of most side effects of glucocorticoids, as measured by blood biomarkers over a 2-week treatment period. Vamorolone has been granted Orphan Drug status by both FDA and EMA, and received Fast Track designation by the FDA.

About the Cooperative International Neuromuscular Research Group (CINRG)

CINRG was founded in 2000 as an international academic clinical trial network, with a focus on pediatric neuromuscular disease. CINRG has enrolled over 1,500 patients into clinical research studies. Recent studies include the CINRG Duchenne Natural History Study (DNHS) with 440 DMD patients and over 100 healthy peers followed by expert neuromuscular physicians in 20 sites in 10 countries. See www.cinrgresearch.org

VISION-DMD Programme: The on-going clinical development of Vamorolone is undertaken within the VISION-DMD programme. This is a US-European collaboration coordinated the University of Newcastle and led by the Sponsor ReveraGen Biopharma. More details@ http://vision-dmd.info.

Vision-DMD: This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 667078 and the US National Institutes of Health under grant agreement NINDS R44 NS095423.