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Vamorolone treatment of Duchenne muscular dystrophy patients leads to improvements in motor function

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A publication in the journal ***Neurology*** was released today describing efficacy studies of vamorolone, a treatment option being evaluated as a potentially safer alternative to corticosteroids, in boys with Duchenne muscular dystrophy.

"Vamorolone showed marked improvement of multiple tests of strength and endurance in boys treated with either 2 or 6 mg/kg/day" said Dr. Paula Clemens, Study Chair, Professor of Neurology at the University of Pittsburgh School of Medicine.

The ***Neurology*** publication reports an open-label, multiple-ascending dose study of vamorolone in 48 boys with DMD (4 to <7 years, steroid-naïve) (clinicaltrials.gov; NCT02760264, NCT02760277). Doses of vamorolone were 0.25, 0.75, 2.0 and 6.0 mg/kg/day. The 2.0 mg/kg/day dose group met the primary efficacy outcome of improved muscle function (time to stand; 24 weeks vamorolone treatment versus both natural history controls [p=0.04], and the 0.25 mg/kg/day lowest vamorolone dose group [p=0.02]), without evidence of most adverse effects of glucocorticoids. The 6.0 mg/kg/day dose also showed significant improvements in time to run 10 meters and 6-minute walk test versus the 0.25 mg/kg/day dose group (p=0.006; p=0.002 respectively). A biomarker of bone formation, osteocalcin, increased in vamorolone treated boys, suggesting possible loss of bone morbidities seen with corticosteroids. Biomarker outcomes for adrenal suppression and insulin resistance were also lower in vamorolone-treated DMD patients, relative to published studies of corticosteroid therapy.

"The leverage of the Cooperative International Neuromuscular Research Group [CINRG] enabled the rapid recruitment, and the robust measurements of outcomes in the 48 boys enrolled, and the comparisons to previous CINRG studies", said Lauren Morgenroth, Chief Operating Officer of TRiNDS, the coordinating center for the trial. Patients that completed the clinical trial reported in the ***Neurology*** publication had the option of continuing on vamorolone though a 2-year long-term extension study (VBP15-LTE; NCT03038399), and 46 of 48 patients have enrolled in the extension study to continue vamorolone treatment. Patients completing the 2-year long-term extension study are given the option for further long-term treatment with vamorolone via an Expanded Access Protocol (EAP; NCT03863119). The majority of patients, families, and physicians have opted for continued vamorolone treatment, rather than transitioning to corticosteroids (standard of care).

"Vamorolone appears to be the first dissociative steroidal anti-inflammatory drug that is able to retain efficacy of corticosteroids while reducing side effect profiles," said Dr. Eric Hoffman, CEO and co-founder of ReveraGen. "Vamorolone thus holds potential for the many chronic inflammatory diseases where corticosteroid treatment is standard of care, yet side effects detract from patient quality of life," he continued.

A pivotal trial of vamorolone is currently enrolling participants (4 to <7 years boys with DMD that have not previously been treated with corticosteroids; NCT03439670). "We are currently 33% enrolled into the new pivotal study that we hope will lead to authorization of vamorolone," said Dr. Michela Guglieri, Senior Lecturer and Honorary Consultant, Institute for Genetic Medicine, Newcastle University. "We

hope to be fully enrolled by the end of 2019, and are working to provide long-term access to vamorolone for all patients finishing the 12-month trial," she continued.

The clinical trial reported in ***Neurology*** received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement number 667078, and the US National Institutes of Health, National Institute of Neurological Disorders and Stroke (R44NS095423).

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About Duchenne muscular dystrophy

Duchenne muscular dystrophy is a rare genetic disease that predominantly affects young boys. Loss of the large protein, dystrophin, in muscle leads to persistent damage to muscle. DMD is a progressive disease, with gradual loss of muscle and 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 www.trinds.com