VISION-DMD

Top-line data of 18-month vamorolone treatment of Duchenne muscular dystrophy patients shows continued improvement of symptoms with reduction in corticosteroid safety concerns
Rockville MD – 5 October 2019

A late-breaking presentation at the 24th International Annual Congress of the World Muscle Society (WMS) in Copenhagen, Denmark reported top-line data from 18-month treatment of Duchenne muscular dystrophy (DMD) patients (VBP15-LTE trial). The data was presented by Dr. Eric Hoffman, CEO of ReveraGen.

Vamorolone is a first-in-class steroidal anti-inflammatory in development as treatment for DMD to substitute for corticosteroids (prednisone, deflazacort) that are currently recommended. 48 DMD patients treated for 6 months over a broad dose range (0.25 to 6.0 mg/kg/day) showed dose-related improvements in multiple gross motor outcomes (Hoffman et al. 2019). Upon exiting the 6-month trial, nearly all patients and their physicians chose to continue vamorolone treatment. 45 boys enrolled in a 2-year long-term extension study (VBP15-LTE; NCT03038399), and all dose escalated to 2.0 or 6.0 mg/kg/day of vamorolone.

At the WMS presentation, Dr. Hoffman reported motor function data from 23 patients treated with 2.0 or 6.0 mg/kg/day vamorolone for at least 18 months compared to matching steroid-naïve patients from the Duchenne Natural History Study (DNHS) conducted by the Cooperative International Neuromuscular Research Group (CINRG) (McDonald et al. 2018). Vamorolone treatment significantly improved the velocity of 10-meter run/walk (p=0.005), and 4-stair climb (p=0.036) relative to historical controls. Time to stand from supine showed significant improvements within the vamorolone-treated patients from baseline to 18-months (log seconds p=0.007; velocity p=0.017), but comparison to CINRG DNHS steroid-naïve patients did not reach significance (p=0.08). In addition, motor function outcomes of vamorolone-treated DMD boys were compared to age-matched prednisone-treated CINRG clinical trial patients (Escolar et al. 2011). Both groups (vamorolone and prednisone) showed similar improvements in these gross motor outcomes.

Vamorolone-treated DMD boys showed normal growth rates, and less physician-reported weight gain and Cushingoid features compared to published studies of prednisone and deflazacort (Griggs et al. 2016). The molecular and clinical data suggest that vamorolone is a dissociative steroidal drug that maintains efficacy and has a lower level of the adverse effects that are seen with the currently recommended corticosteroids for the treatment of DMD.

“Our findings suggest that vamorolone results in improvement in DMD patient function, similar to that of corticosteroids, but with less side effects, including no stunting of the growth of DMD children”, said Dr. Hoffman. DMD patients show poor compliance with corticosteroid treatment, even though it is standard of care, and this is primarily due to intolerable side effects (Cowen et al. 2019). “The poor growth of DMD boys after taking corticosteroids is of concern to patients and families, and the lack of this growth failure with vamorolone treatment is an objective outcome of improved safety”, said Dr. Paula Clemens, Study Chair, Professor of Neurology at the University of Pittsburgh.

The vamorolone studies were carried out by the Cooperative International Neuromuscular Research Group (CINRG), an international collaborative of expert pediatric neuromuscular research centers, with studies coordinated by TRiNDS LLC and ReveraGen BioPharma.

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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 (Horizon 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

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.