Immediate release

Vamorolone (VBP15) First-in-patient Studies in Duchenne Muscular Dystrophy Published
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A clinical trial of vamorolone in 48 boys with Duchenne muscular dystrophy entitled "*Phase IIa trial in Duchenne muscular dystrophy shows vamorolone is a first-in-class dissociative steroidal anti-inflammatory drug*" has been published in *Pharmacological Research*. The publication describes a multiple ascending dose clinical trial covering a broad range of doses of vamorolone (0.25, 0.75, 2.0 and 6.0 mg/kg/day), with 2 weeks of vamorolone treatment, followed by a 2-week washout period. The trial was carried out by ReveraGen BioPharma, with the Cooperative International Neuromuscular Research Group (CINRG), at 11 CINRG sites in 6 countries (US, Canada, Australia, Israel, UK, and Sweden) (clinicaltrials.gov; NCT02760264). The published paper presents results of clinical safety, pharmacokinetics and pharmacodynamic biomarkers.

The authors report that vamorolone was safe and well-tolerated through the highest dose tested (6.0 mg/kg/day, or about 9-times typical prednisone dose in DMD) and showed pharmacokinetics similar to prednisone. "Our use of blood biomarkers in this study was innovative," noted Laurie Conklin MD, lead author on the study. "Based on blood testing, we were able to show that vamorolone has less potential for side effects than deflazacort and prednisone, but still shows strong anti-inflammatory activity."

The trial was fully recruited in 13 months (first subject enrolled to last subject enrolled). "Given the narrow age rangeof 4 to <7 years, the rarity of DMD, and the requirement that subjects be steroid-naive, the rapid recruitment by the CINRG group was quite remarkable," noted Jesse Damsker, PhD, Vice President of Operations at ReveraGen. All 48 patients continued into the 24-week extension study (VBP15-003), and 46 of 48 completed the extension and remain enrolled in the 2-year long-term extension (VBP15-LTE).

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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 DMD 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 in 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