ReveraGen BioPharma Inc initiates VISION-DMD Phase 2a Study for Treatment of Duchenne muscular dystrophy

Enrollment has begun in the Phase 2a clinical trial of vamorolone (VBP15) in the treatment of boys with Duchenne muscular dystrophy (DMD). The Phase 2a trial sponsored by ReveraGen BioPharma, is an open-label, multiple ascending dose study that will be followed by a 2a extension trial. The Phase 2a trials are part of the VISION-DMD project: An Innovative Steroid-like Intervention on DMD.

Vamorolone is a dissociative steroidal agent that aims to separate safety concerns of traditional glucocorticoid drugs, such as prednisone and deflazacort, from key aspects of efficacy. During the Phase 2a trial, 48 steroid-naïve boys aged 4-7 will be enrolled into four dose cohorts sequentially (0.25 mg/kg, 0.75 mg/kg, 2.0 mg/kg, and 6.0 mg/kg) and receive vamorolone once daily for 2 weeks followed by 2 weeks of follow-up off treatment. Participants will be rolled over into the Phase 2a extension at the same dose level and dosing will continue for 24 weeks. The primary outcome of the 2a trial is acute safety and tolerability and pharmacokinetics of vamorolone administration. The primary outcome of the Phase 2a extension trial is long-term safety and tolerability, efficacy as measured by the Time to Stand Test, and safety as measured by body mass index. Both studies include innovative study design aspects including collection of pharmacodynamic safety and efficacy biomarkers. Enrollment will take place at sites of the Cooperative International Neuromuscular Research Group (CINRG) network within the United States, Canada, Israel, United Kingdom, Sweden and Australia. For more information on the trial visit: http://vision-dmd.info/2a-trial-information/

The development of vamorolone as an alternative to traditional glucocorticoids for DMD is currently funded through a Venture Philanthropy model including an international community of patient groups and US and European public grants. The VISION-DMD clinical program includes the Phase 2a and Phase 2a extension studies and the planned Phase 2b and Phase 2b extension studies. The Phase 2a trials are funded by a grant from the National Institute of Neurological Disorders and Stroke (NINDS) of the National Institutes of Health. Planning for the Phase 2a trials was funded by the National Institute of Arthritis, Musculoskeletal, and Skin Diseases (NIAMS) of the National Institutes of Health. The Phase 2b trials are expected to begin the second half of 2017 and will be part funded by a grant from the European Commission Horizon 2020 programme. Phase I clinical trials in healthy adults were supported by the Muscular Dystrophy Association USA and three United Kingdom foundations (Joining Jack, Duchenne Children’s Trust, and Duchenne Research Foundation). Preclinical funding was provided by DMD foundations and US government: Foundation to Eradicate Duchenne (USA); Parent Project Muscular Dystrophy (USA); Muscular Dystrophy Association (USA); Action Duchenne (UK), Save Our Sons (Aus); Michael’s Cause (USA), Pietro’s Fight (USA), Alex’s Wish (UK), Ryan’s Quest (USA); and US public grants from National Institutes of Health; and Department of Defense CDMRP.

About

ReveraGen BioPharma (Sponsor): ReveraGen is a privately held clinical-stage drug development company focused on neuromuscular disease. ReveraGen’s lead compound, vamorolone, is a novel anti-inflammatory drug in development for DMD.
**VISION-DMD Consortium:**

A US-European consortium led by ReveraGen Biopharma and Coordinated by the University of Newcastle (Prof Kate Bushby’s Group) will deliver the VISION-DMD programme. Partners include the United Parent Projects Muscular Dystrophy (UPPMD) global patient advocate group; the Cooperative International Neuromuscular Research Group (CINRG), led by Paula Clemens - University of Pittsburgh and supported by the Therapeutic Research in Neuromuscular Disorders Solutions (TRINDS) management team; the European Clinical Research Infrastructure Network (ECRIN); the University Hospital Motol and Ceratium Limited. More details @ [http://vision-dmd.info/consortium/](http://vision-dmd.info/consortium/).

**Further information**

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See also [http://vision-dmd.info/](http://vision-dmd.info/)

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