VISION-DMD

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

A first-in-patient Phase 2a study of vamorolone, a first-in-class dissociative steroid drug, in 48 DMD boys (4 to <7 years; VBP15-002) successfully completed in November 2017 with dose escalations to 10-times typical prednisone/deflazacort dose in DMD. Participants are continuing in a six month extension (VBP15-003) and two year long-term extension (VBP15-LTE).

Data from the Phase 2a study was supportive of initiation of a Phase 2b clinical trial of vamorolone (VBP15) in 120 boys with DMD (4 to <7 years). The Phase 2b trial is a blinded, placebo- and prednisone-controlled trial. For the initial 24 weeks, patients will be randomized to vamorolone (50%), prednisone (25%) and placebo (25%). For the 2nd 24 week period, all patients will receive vamorolone. The Phase 2b trial is expected to begin enrollment in March 2018.

The Phase 2 program is sponsored by ReveraGen BioPharma, with support from NIH NINDS, European Commission, and non-profit foundations. The Phase 2 trials of vamorolone are part of the VISION-DMD project, with Study Chairs Dr. Paula Clemens (University of Pittsburgh), and Dr. Michela Guglieri (Newcastle University).

Vamorolone is a dissociative steroidal agent that is designed to retain and increase the efficacy of corticosteroids (prednisone and deflazacort), while reducing the side effects (safety concerns).

The primary outcomes of the Phase 2b trial are a comparison of efficacy of two doses of vamorolone against placebo, and comparison of safety against prednisone (weight gain, bone symptoms, metabolic disturbance, and others). The trials were designed with the involvement of parents of DMD children, with an effort to minimize burden on the families. For more information on the trial contact Becky Davis becky.davis@newcastle.ac.uk (Europe) or Andrea Smith asmith@trinds.com (North America).

The development of vamorolone has been carried out using 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 Phase 2b and 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 (NIH). Planning for the Phase 2a trials was funded by the National Institute of Arthritis, Musculoskeletal, and Skin Diseases (NIAMS) of the NIH. The Phase 2b trials are funded in part 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 NIH; 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 and other chronic inflammatory states.

VISION-DMD Consortium:

A US-European consortium led by ReveraGen Biopharma, with the University of Newcastle, University of Pittsburgh, and TRiNDS LLC. Partners include the United Parent Projects Muscular Dystrophy (UPPMD) global patient advocate group; the Cooperative International Neuromuscular Research Group (CINRG); the European Clinical Research Infrastructure Network (ECRIN); the University Hospital Motol and Ceratium Limited. More details@ http://vision-dmd.info/consortium/.

Further information

Contact: Eric P. Hoffman, PhD, CEO, ReveraGen Biopharma, Inc. ericphoffman@gmail.com

See also 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.

ReveraGen BioPharma www.reveragen.com

CERATIUM helping research happen www.ceratium.eu

Newcastle University www.newcastle-muscle.org

ECRIN EUROPEAN CLINICAL RESEARCH INFRASTRUCTURE NETWORK www.ecrin.org

United Parent Projects Muscular Dystrophy www.uppmd.org

FN MOTOL www.fnmotol.cz

CINRG www.cinrgresearch.org

TRiNDS www.trinds.com