ReveraGen BioPharma receives National Institutes of Health ethics grant to return data to participants in vamorolone clinical trials

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The National Institute of Neurological Disorders and Stroke (NINDS) of the National Institutes of Health (USA) has awarded an ethics research grant to ReveraGen BioPharma to return patient-level and aggregate data to Duchenne muscular dystrophy (DMD) patients and their families participating in clinical trials of vamorolone.

Vamorolone is a first-in-class small molecule drug that has been shown to significantly improve patient muscle function in published open label trials. "We recognize and greatly appreciate the considerable time and effort required of families participating in clinical trials, and we strive to return information on the results of the trial to patient families and quickly as possible," said Dr. Laurie Conklin, Director of Regulatory Affairs at ReveraGen and project lead.

Clinical trials often are 'blinded' to preserve the integrity and validity of clinical trials. However, such blinding typically leads to patients not knowing whether they received drug or placebo, or how their response to the experimental drug compared to others in the trial. Returning results to patients and families requires identifying specific patients within a clinical trial, at the family's request.

"I have worked with many of the families within the vamorolone trials as a patient advocate within ReveraGen", said Suzanne Gaglianone, patient liaison at ReveraGen. "My son and I have participated in multiple clinical trials, and I fully recognize the desire of families to find out details on how their son did while taking part in the trial", she continued. Ms. Gaglianone will manage patient contacts, helping them identify their son in databases, and coordinating the return of patient-level and aggregate data to them.

"ReveraGen's approach is highly innovative, as well as needed", said Abby Bronson, Senior Vice President Research Strategy, Parent Project Muscular Dystrophy. "We are delighted to collaborate with ReveraGen in facilitating return of clinical trial data to our patients and families, and to learn from this novel research grant", she continued.

"The Muscular Dystrophy Association is delighted to see ReveraGen share back with patient's their own clinical trial data", noted Kristin Stephenson, Executive Vice President, Chief Advocacy and Care Services Officer of the Muscular Dystrophy Association, "this is an exciting opportunity for clinical trial participants."

"The Foundation to Eradicate Duchenne has been a long-term collaborator on the vamorolone program in DMD, and this pioneering effort to give results back to the patients in the trials is warmly welcomed," said Joel Wood, CEO of FED.

Dimitrios Athanassiou, Board member of World Duchenne noted, "Returning data to the trial participants is a first in the DMD community, and we hope that other trial sponsors adopt this model."

About *Vamorolone* – first-in-class dissociative steroid

Vamorolone is a first-in-class drug candidate that binds to the same receptors as corticosteroids but modifies the downstream activity of the receptors. This has the potential to 'dissociate' efficacy from typical steroid safety concerns and therefore could replace existing corticosteroids, the current standard of care in children and adolescent patients with DMD. In published open label studies, vamorolone showed improvements in motor skills in DMD boys. There is significant unmet medical need in this patient group as high dose corticosteroids have severe systemic side effects that detract from treatment compliance and patient quality of life. The United Kingdom MHRC has awarded vamorolone Priority Innovative Medicines designation.

The currently ongoing 48-week Phase IIb VISION-DMD study (VBP15-004; NCT03439670) is designed as a pivotal trial to demonstrate efficacy and safety of vamorolone compared with prednisone and placebo in 120 boys aged 4 to <7 with DMD that have not yet been treated with corticosteroids. For more information: https://vision-dmd.info/2b-trial-information.

Vamorolone is being developed by US-based ReveraGen BioPharma Inc. with participation in funding and design of studies by several international non-profit foundations, the US National Institutes of Health, the US Department of Defense and the European Commission's Horizon 2020 program. In November 2018, Santhera acquired from Idorsia the option to an exclusive sub-license to vamorolone in all indications and all countries worldwide (except Japan and South Korea).

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