VISION-DMD RELEASES WHITE PAPER ON RETURNING INDIVIDUAL CLINICAL TRIAL DATA TO PARTICIPANTS

Newcastle, United Kingdom, March 10, 2021 – VISION-DMD today released a white paper on returning meaningful individual clinical trial results. The focus of the white paper 'Returning Individual Clinical Trial Data Back To Participants' is to address the ethical and technical challenges, using the vamorolone clinical trial experience.

The white paper highlights the need for clinical trials to consider the return of patient study data directly to the families participating, allowing access to their clinical trial data without compromising the study, to provide an opportunity for participants to connect their clinical trial data with their clinical care information. This white paper concludes that returning data promotes autonomy in patients and encourages future research participation with both clinical and individual relevance.

"Patients participate in a clinical trial to generate new knowledge to advance drug and technology development. During a clinical trial, data on individuals is collected to generate aggregated information to support the study objectives." Says Dr. Conklin, Medical and Regulatory Director, ReveraGen BioPharma. "Often, this individual patient data will not be returned to the participants, even when this is requested."

In the white paper, Dr. Conklin and Dr. Peay address the ethical and technical challenges of returning meaningful clinical trial results. They propose a framework that ensures independent data return without compromising patient anonymity and study integrity.

The challenges highlighted in this white paper are:
1. Ethical implications in returning clinical data
2. Assessment of meaningful research results
3. Understanding research results
4. Barriers in data integrity and sharing

These challenges were investigated by ReveraGen Biopharma, the drug sponsor/developer in the VISION-DMD clinical trial programme for the innovative new steroid-like drug vamorolone in boys with Duchenne Muscular Dystrophy. Following conversations with patient families during the vamorolone studies, the ReveraGen team developed an approach to provide patient study data directly to participant families, allowing them to share and connect their clinical trial data with their clinical care information. This approach received funding through a grant from the U.S. National Institutes of Health Bioethics, led by Dr. Laurie Conklin and Dr. Eric Hoffman.

Click here to read the White Paper 'Returning Individual Clinical Trial Data Back To Participants'

For more information about the study: www.vision-dmd.info
To request the return of data please contact: suzanne.gaglianone@reveragen.com
For project enquiries contact: vision-dmd_team@ceratium.eu

This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 667078

About DMD

Duchenne Muscular Dystrophy (DMD) is a rare, muscle wasting disease. Boys progressively weaken and lose the ability to walk. The progressive muscle weakness leads to serious medical problems, particularly issues relating to the heart and lungs. Young men with DMD typically live into their late twenties. Corticosteroids are widely recognised to increase muscle strength and delay disease progression but global acceptance as the standard of care is variable due to severe side effects.

VISION-DMD and EU funding for rare diseases

VISION-DMD is an international project with partners from the US, UK, Czech Republic, and the Netherlands bringing together SME's, universities, patient organisations and leading clinicians in this exciting and innovative project. Rare diseases including DMD are a priority area of EU research funding. More than €1.4 billion has been spent on over 200 research and innovation FP7 and H2020 projects in the area of rare diseases facilitating the formation of multidisciplinary teams from universities, research organisations, healthcare providers, SMEs, industry and patient organisations from across Europe and beyond.

Patient group funding has been critical to the clinical development of vamorolone through a venture philanthropy model. For more information see: https://vision-dmd.info/patient-group-funders/

About vamorolone

Vamorolone is an innovative steroid-like drug designed to retain or improve corticosteroid efficacy and increase membrane stabilisation with reduced side effects. Vamorolone aims to increase the therapeutic window, slow disease progression, and improve quality of life and lifespan for all DMD patients. Results of Phase 2a studies have found vamorolone has an acceptable safety and tolerability profile with no clinically significant safety concerns. 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.

Phase 2b clinical trials

The Phase 2b study is a 48-week study (placebo arm for the first 24 weeks) testing vamorolone and the comparator, Prednisone, in 121 steroid naive DMD boys aged from 4 to 7 years old. The trial is fully recruited and is taking place at study sites in eleven countries across Europe, North America and Australia.

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