VISION-DMD

ReveraGen Announces First Patient Enrollment in International Pivotal Trial of Vamorolone in Duchenne Muscular Dystrophy

VISION-DMD Study supported by National Institutes of Health and European Commission's Horizon 2020 programme, Examines Efficacy and Safety of Vamorolone Compared to Placebo and Prednisone

ROCKVILLE, Maryland, August 22, 2018

ReveraGen BioPharma, Inc. today announced the initiation and first patient enrolment in a double-blind, placebo- and prednisone-controlled clinical trial (VBP15-004) of vamorolone, a first-in-class dissociative steroidal anti-inflammatory, in Duchenne muscular dystrophy. This VISION-DMD study (clinicaltrials.gov NCT03439670) will enrol approximately 120 patients at ~30 sites in US, Canada, Israel, United Kingdom, Sweden, Australia, Netherlands, Belgium, and Czech Republic.

"This trial builds on the promising preliminary safety and efficacy data in our completed Phase 2a trial of 48 DMD boys", said Eric Hoffman, Ph.D., CEO of ReveraGen, and study Sponsor. In publicly released data, six months treatment with vamorolone showed dose-responsive improvements in timed function tests, and improved safety relative to historical comparators treated with glucocorticoids.

Glucocorticoids, such as prednisone and deflazacort (Emflaza) are considered standard of care, yet the many side effects of these drugs lead to decreased quality of life of patients and their families. "DMD patients need an alternative to glucocorticoid standard of care, and it is our hope that this new VISION-DMD trial will provide data towards this end," said Michela Guglieri, M.D., Study Co-Chair and Hon. Consultant in Human Genetics – Neurologist at The Newcastle upon Tyne Hospitals and NHS Foundation Trust in the United Kingdom.

Vamorolone is a first-in-class drug with a novel chemistry that binds to the same receptors as glucocorticoids but changes the downstream activity of the receptors. This has the potential to 'dissociate' the efficacy from safety concerns, as suggested in multiple animal models of chronic inflammatory states, as well as Phase 1 clinical trials in adult volunteers, and the aforementioned Phase 2a study in 48 DMD boys.

Vamorolone has been developed under a venture philanthropy model, with participation in funding and design of studies by 12 international non-profit foundations, US National Institutes of Health, US Department of Defense, and European Commission Horizon 2020 program.

About the VISION-DMD VBP15-004 study.

Boys with DMD that have not yet been treated with glucocorticoids, and who have had their 4th birthday but not yet had their 7thbirthday will be randomized to one of four groups: low dose vamorolone (2 mg/kg/day), high dose vamorolone (6 mg/kg/day), prednisone (0.75 mg/kg/day), or placebo. After the initial 24-week treatment period, the prednisone and placebo groups will cross-over to low dose or high dose vamorolone. The second treatment period then has all patients treated for an additional 24 weeks with vamorolone. Clinical outcomes for efficacy include timed function tests and measures of muscle strength and endurance. Clinical outcomes for safety include monitoring of bone changes, weight

changes, cataracts, and biomarkers of metabolic disturbances. Enrolment is expected to take about 12 months, with total study duration of about 24 months.

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 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

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