ReveraGen Receives $3.3 Million NIH Commercialization Readiness Pilot Grant for NDA Preparations for Vamorolone in Duchenne Muscular Dystrophy

-- Funds will support NDA preparatory activities for anticipated filing in 2021 --

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Rockville, Maryland—ReveraGen, Inc., a privately held company developing vamorolone (VBP15) as a potential safer alternative for corticosteroid treatment in Duchenne muscular dystrophy and other disorders, today announced an award of a $3.3 million grant from the National Institute of Neurological Disorders and Stroke (NINDS) at National Institutes of Health (NIH). The two-year SBIR/STTR Commercialization Readiness Pilot (CRP) Program award will provide additional funding towards the new drug application (NDA) preparation, enabling timely filing after the read-out of the fully-enrolled registration study (VBP15-004; NCT03439670) anticipated in Q2 2021.

The Commercialization Readiness Pilot (CRP) program is designed to aid companies with previously funded SBIR/STTR Phase II/IIB projects as they advance to commercialization. ReveraGen has held a NINDS SBIR Phase II grant that enabled completion of Phase 2a clinical trials that showed dose-responsive improvement of vamorolone-treated DMD participants at both 24-weeks and 18-months treatment. A subsequent NIH SBIR Phase IIB award has supported the ongoing pivotal trial in 121 DMD boys recruited at 30 sites in 11 countries.

“This generous support from NIH is enabling the execution of the global regulatory and global intellectual property strategy of vamorolone, which we are developing jointly with Santhera,” said **Dr. Eric Hoffman, VP Research and principal investigator at ReveraGen** on the award.

“Upfront of the CRP application, ReveraGen had carried out a formal gap analysis to identify pending items required for the upcoming NDA submission, and the CRP award will be beneficial in addressing these,” noted **Dr. Jesse Damsker, VP Operations at ReveraGen**.

“We congratulate our partner ReveraGen to this grant which not only provides support in advancing vamorolone towards FDA submission and commercialization but also stands for recognition of the drug’s promising potential,” added **Dario Eklund, Chief Executive Officer of Santhera**.

ReveraGen has open INDs for development of vamorolone in both Duchenne muscular dystrophy and Becker muscular dystrophy. Studies of vamorolone in animal models of multiple chronic inflammatory diseases (multiple sclerosis, asthma, inflammatory bowel disease, arthritis and others) have shown efficacy similar to corticosteroids, and an improved safety profile. Studies of vamorolone

in DMD boys have shown loss of the stunting of growth seen with treatment with corticosteroid standard of care (deflazacort, prednisone), with less physician-reported safety concerns.

Structure/activity studies comparing the active metabolites of glucocorticoid receptor ligands (vamorolone, deflazacort, prednisone) have shown differential activity in binding co-activators and co-repressors, leading to vamorolone showing unique mechanisms of action. Specifically, vamorolone binding leads to less positive gene transcription activity associated with safety concerns, while retaining potent anti-inflammatory activity.

Recently, Santhera Pharmaceuticals has acquired a world-wide license to vamorolone in all indications. ReveraGen continues to manage the clinical programs and carry out NDA preparations.

About Vamorolone
Vamorolone is a first-in-class drug candidate that binds to the same receptor as corticosteroids but modifies its downstream activity and as such is a dissociative partial agonist. This mechanism has the potential to 'dissociate' efficacy from typical steroid safety concerns and therefore vamorolone could emerge as a promising alternative to existing corticosteroids, the current standard of care in children and adolescent patients with DMD. There is substantial unmet medical need in this patient group as high-dose corticosteroids have significant systemic side effects that diminish patient quality of life. The fully enrolled, pivotal Phase 2b VISION-DMD trial (VBP15-004, https://vision-dmd.info/2b-trialinformation) is currently being conducted at study sites across North America, Europe, Israel and Australia and topline 6-month data are expected in Q2-2021, paving the way for a US NDA submission in Q4-2021. Vamorolone has been granted Orphan Drug status in the US and in Europe, and has received Fast Track and Rare Pediatric Disease designations by the US FDA and Promising Innovative Medicine (PIM) status from the UK MHRA. Vamorolone was discovered by US-based ReveraGen BioPharma, Inc. and is being developed in collaboration with Santhera, which owns worldwide rights to the drug candidate in all indications. The vamorolone development program has received funding from several international non-profit foundations and patient organizations, the US National Institutes of Health, the US Department of Defense and the European Commission's Horizon 2020 program. See www.reveragen.com; https://vision-dmd.info.

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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