ReveraGen and Santhera Announce FDA Orphan Grant Funding for Clinical Trial with Vamorolone in Becker Muscular Dystrophy

Rockville, MD, USA, and Pratteln, Switzerland, September 27, 2021 – ReveraGen Biopharma and Santhera Pharmaceuticals (SIX: SANN) announce that ReveraGen has received a USD 1.2 million grant from the FDA under their "Clinical Studies of Orphan Products Addressing Unmet Needs of Rare Diseases (R01)" grants program. The grant adds to existing grants from the National Institutes of Health, NIAMS, and the Foundation to Eradicate Duchenne to initiate a clinical trial of vamorolone in adults and children with Becker muscular dystrophy, a progressive muscle wasting disease similar to Duchenne muscular dystrophy, but usually milder.

Vamorolone, a dissociative steroid drug, that has shown retention of efficacy and reduction of safety concerns typically associated with corticosteroids in Duchenne muscular dystrophy (DMD) will now be tested in a 24-week clinical exploratory trial in Becker muscular dystrophy (BMD). BMD is caused by mutations of the DMD gene but shows residual dystrophin protein in muscle, and variable onset and progression of muscle weakness. The double-blind trial will test efficacy and safety of daily vamorolone on motor outcomes and established biomarker outcomes, with participants randomized 2:1 vamorolone or placebo. The clinical trial plans to enroll at sites in Padova (Italy) and Pittsburgh (USA).

"There are currently no approved drugs for BMD in any country, and there is a high unmet need," said **Paula Clemens, MD, Professor of Neurology at the University of Pittsburgh School of Medicine**, and co-principal investigator on the FDA, NIH and Foundation awards.

The mechanisms of actions, providing basis for vamorolone efficacy as demonstrated in the pivotal VISION-DMD study in the more severe DMD, are felt to be highly relevant to BMD too. In addition, vamorolone is hypothesized to increase dystrophin protein levels in BMD via inhibition of microRNAs that deleteriously target dystrophin, and this may further complement the mechanism of action specifically in BMD [1-3].

"While the drug development pipeline has greatly expanded for DMD in recent years, there are very few clinical investigational efforts underway for BMD," said **Elena Pegoraro, MD, PhD, Professor of Neurology at the University of Padova in Italy**. "Corticosteroids are often not tolerated by patients with BMD due to their side effects. Therefore, the lessened side effect burden of vamorolone seen in DMD trials may prove important to the underserved BMD patient community," she continued.

"The Foundation to Eradicate Duchenne is pleased to provide support of the vamorolone program to include BMD patients," said **Joel Wood, President of Foundation to Eradicate Duchenne**.

Vamorolone was discovered by US-based ReveraGen BioPharma, Inc. and is being developed in collaboration with Santhera who owns worldwide rights to the drug candidate for all indications.

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 [4-6]. 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 many patient groups where corticosteroids are standard of care or hold therapeutic promise, but side effects limit prescription and patient adherence. US NDA submission for DMD is anticipated in Q1-2022. Vamorolone has been granted Orphan Drug status in the US and in Europe for DMD, and has received Fast Track and Rare Pediatric Disease designations by the US FDA and Promising Innovative Medicine (PIM) status from the UK MHRA for DMD.

References:

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About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for rare neuromuscular and pulmonary diseases with high unmet medical need. Santhera has an exclusive license for all indications worldwide to vamorolone, a first-in-class dissociative steroid with novel mode of action, which was investigated in a pivotal study in patients with DMD as an alternative to standard corticosteroids. The clinical stage pipeline also includes lonodelestat (POL6014) to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases as well as an exploratory gene therapy approach targeting congenital muscular dystrophies. Santhera out-licensed rights to its first approved product, Raxone® (idebenone), outside North America and France for the treatment of Leber's hereditary optic neuropathy (LHON) to Chiesi Group. For further information, please visit www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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, Duchenne Research Fund, and Jesse's Journey. 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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