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DRAFT V2

Vamorolone Designated Promising Innovative Medicine (PIM) for Treatment of Duchenne Muscular Dystrophy by the UK MHRA

Pratteln, Switzerland, October 21, 2019 – Santhera Pharmaceuticals (SIX: SANN) announces that the UK's Medicines and Healthcare Products Regulatory Agency (MHRA) has informed ReveraGen BioPharma about having designated vamorolone a Promising Innovative Medicine (PIM) in the treatment of Duchenne muscular dystrophy (DMD).

"We congratulate ReveraGen to this success and are excited about the PIM designation as it further validates the potential of vamorolone as an innovative treatment approach addressing the high unmet medical need in young patients with DMD," said **Thomas Meier, PhD, CEO of Santhera**.

The PIM designation indicates that the UK MHRA considers vamorolone a promising candidate for the Early Access to Medicines Scheme (EAMS). In the UK, the EAMS, of which PIM is the first step, aims to give patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorization when there is a clear unmet medical need.

Vamorolone is a first-in-class steroidal anti-inflammatory investigational drug in development as treatment for DMD. Data from non-clinical and clinical studies indicated that vamorolone treatment results in a persistent improvement of muscle function in DMD patients with less adverse effects typically reported for traditional corticosteroids [1-6].

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.

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. 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 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 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 is building a Duchenne muscular dystrophy (DMD) product portfolio to treat patients irrespective of causative mutations, disease stage or age. A marketing authorization application for Puldysa® (idebenone) is currently under review by the European Medicines Agency. Santhera has an option to license vamorolone, a first-in-class dissociative steroid currently investigated in a pivotal study in patients with DMD to replace standard corticosteroids. The clinical stage pipeline also includes POL6014 to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases, as well as omigapil and an exploratory gene therapy approach targeting congenital muscular dystrophies. Santhera out-licensed ex-North American rights to its first approved product, Raxone® (idebenone), for the treatment of Leber's hereditary optic neuropathy (LHON) to Chiesi Group. For further information, please visit www.santhera.com.

Raxone® and Puldysa® are trademarks of Santhera Pharmaceuticals.

Author

Comment [1]: For consideration: this sentence is quite redundant to the last sentence of the section above about vamorolone. I would thus remove it here.

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

References:

[1] Hoffman EP et al. (2019). Vamorolone trial in Duchenne muscular dystrophy shows dose-related improvement of muscle function. Neurology 93(13):e1312-e1323. doi:10.1212/WNL.0000000000008168

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[3] Conklin LS et al. (2018). Phase IIa trial in Duchenne muscular dystrophy shows vamorolone is a first-in-class dissociative steroidal anti-inflammatory drug. Pharmacol Res. 136:140-150. doi: 10.1016/j.phrs.2018.09.007.

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[5] Heier CR at al. (2013). VBP15, a novel anti-inflammatory and membrane-stabilizer, improves muscular dystrophy without side effects. EMBO Mol Med 5: 1569–1585

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REVIEW	DEADLINE	APPROVAL
V2	Oct 18	Thomas Meier (share with EH?)
V3		Anne Atkins Oliver Strub
V4		Thomas Meier Distribution to Management Team Distribution to Board
Final		Thomas Meier (clearance for publication)