Santhera Pharmaceuticals (Switzerland) Ltd
Hohenrainstrasse 24, 4133 Pratteln
Switzerland
Phone: +41 61 906 89 50 | Fax: +41 61 906 89 51

Thomas Meier, PhD

CEO

+41 61 906 89 64

thomas.meier@santhera.com

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Dear representative of the DMD Patient Community,

Just a few days ago, we have announced that **Santhera Pharmaceuticals** entered into an agreement with **Idorsia Pharmaceuticals** for the exclusive sub-license to *vamorolone*, which is in clinical development as potential treatment for Duchenne muscular dystrophy (please refer to the news releases dated 20 November 2018 available on our website).

As stated in the joint news release, this agreement grants Santhera the exclusive option to a sub-license from Idorsia to *vamorolone* worldwide, except for Japan and South Korea. Santhera can exercise this option at the time when data from the currently ongoing Phase IIb VISION-DMD trial becomes available, which is currently expected for 2020.

Vamorolone was discovered by US-based **ReveraGen BioPharma** and has been developed with participation in funding and design of studies by 12 international non-profit foundations, the US National Institutes of Health, the US Department of Defense and the European Commission's Horizon 2020 program.

ReveraGen supported the agreement between Idorsia and Santhera and **Eric Hoffman, PhD**, Chief Executive Officer of ReveraGen, stated in our news release: "Our hope for *vamorolone* is that it can replace existing glucocorticoids in DMD therapy. Early clinical development of *vamorolone* in patients with DMD, using an innovative approach with an array of pre-selected biomarkers in multiple contexts of use, suggests that *vamorolone* preserves anti-inflammatory efficacy while decreasing steroid-associated safety concerns. I am delighted to work with Santhera to advance this exciting therapeutic candidate for patients with DMD."

Santhera's role in the development of vamorolone for the treatment of young patients with DMD

The venture philanthropy model with the widespread and generous support of the Duchenne community and family foundations funded the preclinical and Phase I work and was instrumental in advancing *vamorolone* through the early development process.

Currently, ReveraGen is conducting the VISION-DMD Phase IIb trial in boys ages 4-<7 years old. We are enthusiastic about this trial as it will provide the data needed by regulators to evaluate *vamorolone* as a potential treatment for patients with DMD. The trial is fully funded with support of a USD 15 million payment from Idorsia to ReveraGen – and indirectly by us, since our upfront payment of USD 20 million to Idorsia includes USD 15 million for the trial. ReveraGen remains fully responsible for conducting and completing the trial as planned.

Santhera's commitment towards registration of idebenone for patients with later stage DMD

In parallel, we further advanced *idebenone* towards regulatory approval in Europe and the US. In the past months, we have collected additional data, from natural history studies and patients treated long-term with *idebenone*, which support the data from our development program, most notably from the positive Phase III DELOS trial. A comparative analysis of the Phase III DELOS trial outcome with new data from natural history studies showed that the treatment effect with *idebenone* observed in the DELOS trial can be linked to a delay in the initiation of assisted ventilation by three years, which is of high clinical relevance. Data from patients treated up to four years suggest that the therapeutic effect of *idebenone* is maintained longer term. These findings will be included in the regulatory dossier in preparation of marketing authorization applications for *idebenone* in DMD in Europe and the U.S. in 2019.

Strategic fit and Santhera's long-term commitment to support the DMD Community

We are excited to be working to bring *vamorolone* to patients in parallel with our development of *idebenone* addressing respiratory function decline in DMD. We see *vamorolone*, which is currently investigated in young patients with DMD, fully synergistic to *idebenone*, a potential treatment of respiratory disease aspects in older patients with DMD. These two drugs have the opportunity to address the entirety of Duchenne community, regardless of age or mutation. *Vamorolone* has the potential to become a new standard of care in DMD and provide patients with the benefits of steroids but potentially with a reduced risk of side effects typically seen in glucocorticoids.

Providing treatments for DMD where a high medical need exists is a priority for Santhera and we have been working as a trusted member of the Duchenne community for over 10 years developing *idebenone* for respiratory decline in DMD. Now, we look forward to partnering with ReveraGen, as both of our companies have a deep commitment to the DMD community and Santhera has the regulatory and commercial infrastructure to make *vamorolone* available to patients as soon as possible.

Santhera looks forward to our continued work together and thanks you for the support of our efforts to bring treatments to all boys with Duchenne. Should you have questions, I kindly ask you to contact your Santhera representative of our Patient Advocacy or Medical Affairs teams.

We are looking forward to meeting you at one of the upcoming DMD Community Conferences.

Sincerely yours,

Thomas Meier, PhD, CEO