

*Santhera will hold a conference call today, May 23, 2019, at 13:00 CET, 12:00 GMT, 07:00 EST. Details at the end of statement.*

## **Santhera Enters into License Agreement with Chiesi Group for Raxone® in LHON Valued at up to CHF 105 Million**

- *Transaction allows Santhera to advance its long-term growth strategy by focusing on the development of its clinical-stage neuromuscular and pulmonary programs*
- *Deal includes upfront cash payment of CHF 50 million (EUR 44 million) which is due after closing of the transaction*

**Pratteln, Switzerland, May 23, 2019 – Santhera Pharmaceuticals (SIX: SANN) announces that it has entered into an exclusive license agreement with Chiesi Farmaceutici, an international research-focused healthcare group (Chiesi Group), under which Chiesi Group will in-license Raxone® for the treatment of LHON for a total consideration of up to CHF 105 million (EUR 93 million), comprising an upfront cash payment of CHF 50 million (EUR 44 million) and near- to mid-term sales milestone payments of up to CHF 55 million (EUR 49 million).**

Chiesi Group is in-licensing the rights to Raxone in LHON and all other ophthalmological indications worldwide except the US and Canada, where Santhera retains rights. In a second step, following the completion of certain reimbursement and post-regulatory commitments on the part of Santhera, Chiesi Group has the option to fully acquire Santhera's Raxone business. The transaction enables Santhera to focus on the business areas core to its long-term growth strategy by advancing its clinical-stage neuromuscular and pulmonary programs.

"Since the approval in 2015 we have successfully launched Raxone in Europe and reported strong year-on-year sales increases. In the past eighteen months we also strategically diversified our pipeline. This license agreement for Raxone in LHON, our sole neuro-ophthalmology asset, will allow us to create significant value for our shareholders as it provides financial resources enabling us to focus on delivering innovation to patients with neuromuscular and pulmonary diseases of great unmet medical need," said **Thomas Meier, PhD, Chief Executive Officer of Santhera.**

"We have built a strong portfolio in neuromuscular and pulmonary rare disease areas with last year's acquisition of option-rights to *vamorolone* for Duchenne muscular dystrophy (DMD) and other inflammatory diseases, and the acquisition of POL6014 for cystic fibrosis and other pulmonary disorders. Major upcoming inflection points will be the decision on our marketing authorization application for Puldysa® in DMD in Europe, which we plan to submit shortly. Another milestone expected for next year are results of the pivotal trial with *vamorolone* in DMD. With positive results and upon exercising the option to acquire this asset, we could seek regulatory approvals in the US and Europe. We are delighted to be working with Chiesi Group, a global company with an established commercial infrastructure and a strong reputation for delivering innovation in rare diseases and areas of special care, including ophthalmology. We will work closely with Chiesi Group to ensure a smooth handover of the Raxone business and a seamless supply of this only approved medication for the treatment of this devastating form of vision loss to patients in need."

“Chiesi Group has a strong commitment in the orphan diseases area, bringing innovative treatments forward to patients affected by rare or ultra-rare disorders and improving the quality of life of people with rare diseases is one of the most important goals. This strategic partnership reinforces this commitment with a therapeutic offer for patients affected by LHON, a neurodegenerative disease targeting the optic nerve and characterized by sudden vision loss”, commented **Ugo Di Francesco, Chief Executive Officer, Chiesi Group**.

Santhera will license and transfer to Chiesi Group all of its rights for the development, commercialization and distribution of Raxone for the treatment of LHON and any other potential ophthalmological indications for all territories worldwide except the US and Canada.

The closing of the transaction is subject to customary approvals and is currently expected to occur in the third quarter 2019. As of that date, the advance payment of CHF 50 million (EUR 44 million) will also become due.

In an interim phase Santhera will continue to provide medical, technical, logistical and scientific support with regard to ongoing market authorization activities and/or market access undertakings for several months. In this context, the parties agreed that Santhera will continue to commercialize Raxone for LHON in France until ongoing pricing and reimbursement negotiations have been finalized. Santhera will also be responsible to complete the ongoing Post Authorization Measures, expected by 2021, in conjunction with the centralized European Marketing Authorization granted in 2015.

Following the transaction, Santhera will focus its resources on advancing its late-stage clinical pipeline of products for the treatment of neuromuscular and pulmonary diseases. In particular, Santhera intends to fully exploit its strategic position in DMD therapy development, with ongoing programs to help all DMD patients irrespective of causative mutations, disease stage or age. Addressing the needs of advanced DMD patients experiencing respiratory dysfunction, Santhera plans to submit a conditional marketing authorization application (MAA) for Puldysa® (idebenone) to the EMA. Pivotal study data for *vamorolone* as treatment of younger, still ambulatory DMD patients is expected in 2020. In addition, Santhera's expertise in the respiratory area through its activities in the DMD space led to the acquisition of the promising clinical-stage asset POL6014, which has the potential to treat cystic fibrosis (CF) and other pulmonary diseases. Other pipeline elements include strategies to treat congenital muscular dystrophies.

Centerview Partners acted as exclusive financial advisor to Santhera for this license agreement.

#### **Santhera Conference Call**

Santhera will hold a conference call today, May 23, 2019 at 13:00 CET, 12:00 GMT, 07:00 EST to discuss the license agreement with Chiesi Group for Raxone. Participants are invited to dial one of the following numbers about 10 minutes before the call is due to start (no code required):

Europe: +41 58 310 50 00

UK: +44 207 107 0613

USA: +1 631 570 5613

### **About Leber's Hereditary Optic Neuropathy and the Therapeutic Use of Raxone**

Leber's hereditary optic neuropathy (LHON) is a heritable genetic disease causing profound vision loss and blindness. The disease presents predominantly in young, otherwise healthy adult males as rapid, painless loss of central vision, usually leading to permanent bilateral blindness within a few months of the onset of symptoms. About 95% of patients harbor one of three pathogenic mutations of the mitochondrial DNA, which cause a defect in the complex I subunit of the mitochondrial respiratory chain. This defect leads to decreased cellular energy (ATP) production, increased reactive oxygen species (ROS) production and retinal ganglion cell dysfunction, which cause progressive loss of visual acuity and blindness.

Raxone (idebenone), a synthetic short-chain benzoquinone and a cofactor for the enzyme NAD(P)H:quinone oxidoreductase (NQO1), circumvents the complex I defect, reduces and scavenges ROS, restores cellular energy levels in retinal ganglion cells and promotes recovery of visual acuity. Current data demonstrate that up to 50% of patients benefit from treatment and are protected from progression of visual acuity loss or experience a clinically relevant recovery of visual acuity.

Raxone for the treatment of LHON was granted orphan drug status in the EU, US, Switzerland and South Korea.

### **About Chiesi Group**

Based in Parma, Italy, Chiesi Farmaceutici is an international research-oriented group with over 80 years' experience in the pharmaceutical sector, and is present in 27 countries. The group researches, develops and commercialises innovative medicines in the respiratory disease, special care and rare disease therapeutic areas. The Group's Research & Development centre is based in Parma (Italy) and integrated with 6 other important research and development groups in France, the USA, the UK and Sweden, to promote its pre-clinical, clinical and registration programmes. The Group employs around 5,700 people. [www.chiesi.com](http://www.chiesi.com).

### **About Santhera**

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for rare and other diseases with high unmet medical needs. The portfolio comprises clinical stage and marketed treatments for neuro-ophthalmologic, neuromuscular and pulmonary diseases. Santhera's Raxone® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein, Israel and Serbia for the treatment of Leber's hereditary optic neuropathy (LHON) and is currently commercialized in more than 20 countries. For further information, please visit [www.santhera.com](http://www.santhera.com).

*Raxone® and Puldysa® are trademarks of Santhera Pharmaceuticals.*

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