

## **Santhera Receives Positive Opinion for Orphan Drug Designation in the EU for POL6014 in Cystic Fibrosis**

**Pratteln, Switzerland, October 15, 2018 – Santhera Pharmaceuticals (SIX: SANN) announces that the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA) has issued a positive opinion on orphan drug designation for POL6014 in the treatment of cystic fibrosis (CF), a rare pulmonary disease affecting around 35,000 people in the European Union.**

“Obtaining orphan drug designation for POL6014 in the European Union is an important regulatory milestone for our development program with POL6014,” stated **Kristina Sjöblom Nygren, MD**, Chief Medical Officer and Head of Development of Santhera. “With this positive opinion the COMP acknowledges the needs of patients with cystic fibrosis, and for a novel treatment approach with POL6014 to support these patients.”

POL6014, an innovative, potent and selective inhibitor of human neutrophil elastase (hNE) licensed from Polyphor, is entering Phase I/II clinical development for the treatment of CF.

The positive opinion from the EMA’s COMP will be sent to the European Commission (EC), which is expected to grant the orphan drug designation within 30 days. The orphan drug designation will provide Santhera with regulatory and financial incentives to develop POL6014 in the treatment of CF.

### **About POL6014**

POL6014 is a highly potent and selective inhibitor of human neutrophil elastase (hNE) and was shown to reach high concentrations in the lung when administered by inhalation via an optimized eFlow® nebulizer (PARI Pharma GmbH). A first-in-man Phase I study in healthy volunteers and a Single Ascending Dose (SAD) safety and tolerability Phase I study in CF patients have successfully been completed. The drug candidate was well tolerated and showed evidence of strong elastase inhibition as previously demonstrated in animal models. In addition, POL6014 may show therapeutic benefit for a range of neutrophilic pulmonary diseases with high medical need such as non-cystic fibrosis bronchiectasis (NCFB), alpha-1 antitrypsin deficiency (AATD) or primary ciliary dyskinesia (PCD). POL6014 has EU orphan drug designation for the treatment of AATD and PCD.

### **About Cystic Fibrosis**

Cystic fibrosis (CF) is a rare, hereditary, life-threatening, progressive disease affecting approximately 70,000 patients in the U.S. and Europe and is characterized by persistent lung infection and chronic inflammation thereby limiting the ability to breathe over time. Activated or necrotic neutrophils liberate human neutrophil elastase (hNE) in the lung that causes damage to structural, cellular and soluble components of the pulmonary microenvironment. High levels of hNE play a central role in the pathophysiology of CF and correlate with disease severity as measured by functional lung parameters. Inhibition of hNE is expected to stop or slow the damage to lung tissue and may help to improve the overall quality of life for individuals with CF.

**About Santhera**

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for orphan 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 and Israel for the treatment of Leber's hereditary optic neuropathy (LHON) and currently commercialized in more than 20 countries. For further information, please visit [www.santhera.com](http://www.santhera.com).

*Raxone® is a trademark of Santhera Pharmaceuticals.*

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