Santhera Drug Wins European Approval for Rare Blindness Disease

By Adam Feuerstein Follow | 09/09/15 - 01:01 AM EDT

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Liestal, Switzerland (TheStreet) -- European regulators approved Wednesday a new drug from **Santhera Pharmaceuticals** (SANN.SW) to treat a rare, inherited disease which causes progressive vision loss and blindness.

The Santhera drug, Raxone, is the first approved treatment for Leber hereditary optic neuropathy (LHON), which affects approximately 10,000 patients (mostly males) in Europe.

The Raxone commercial launch will commence in the coming weeks, said Santhera CEO Thomas Meier. He declined to discuss how much the company expects to charge for Raxone except to say that it will be "priced as a typical orphan disease drug."

Raxone is a pill designed to stimulate mitochondria, the energy-producing organelle found inside cells. In LHON, a defect in the mitochondrial DNA causes retinal ganglion cells -- a type of nerve cell connecting the eye to the brain -- to lose energy and stop functioning. When retinal ganglion cells stop working, patients experience progressive vision loss and eventual blindness.

Raxone doesn't correct the defective mitochondrial DNA underlying LHON. Instead, the drug circumvents the genetic defect and restores energy levels to the retinal ganglion cells. European regulators approved Raxone based on data from a clinical trial and an expanded access program showing the drug mitigates and reverses vision loss in LHON patients.

RBC Capital analyst Simos Simeonidis forecasts $29 million in peak Raxone LHON sales based on annual pricing of $43,000.

The LHON indication makes up just a fraction of RBC's Santhera valuation because the company is also developing Raxone to improve the lung function of Duchenne muscular dystrophy (DMD) patients. The progressive weakening of muscles in the chest of DMD patients leads to respiratory disease and breathing problems. Santhera hopes to submit Raxone approval filings for DMD in the U.S. and Europe by the end of the year, said Meier.