Sight for sore eyes?

An experimental eye solution may recover eyesight for patients with neurotrophic keratitis

By Elena Berton

An experimental ophthalmology drug has shown promise as a treatment for rare degenerative eye disease neurotrophic keratitis and could potentially reach the market as early as 2016 if the results of an ongoing mid-stage study confirm these early findings.

The therapy also holds the potential to treat other serious eye conditions such as dry eye syndrome, glaucoma and retinitis pigmentosa, according to its developer, Italian biopharmaceutical company Dompé. If successful, the treatment would pave the way for Dompé to enter the $19 billion ophthalmology market currently dominated by large drug makers.

"Our ambition is to bring to the international market a number of treatments that will address unsolved problems thanks to our own R&D," said Dompé Chief Executive Eugenio Aringhieri. "We believe that in the next 5 years we can transform the value of the company."

Neurotrophic keratitis is a rare degenerative eye disease affecting one in 5000 people worldwide and has no current targeted treatment. It is characterized by progressive damage of the cornea caused by eye surgeries, existing conditions such as herpes infections and diabetes, as well as misuse of contact lenses and certain eye medications.

The arrival of blockbusters such as Lucentis (Novartis, Basel, Switzerland) and Eylea (Bayer HealthCare, Leverkusen, Germany) has transformed this historically slow-growth, generic-focused market into a high-growth sector with annual growth rates twice that of the overall pharmaceutical industry. The global ophthalmology market is expected to grow at 5.2% annually to reach an estimated value of $21.6 billion in 2018, according to Transparency Market Research.[1]

Dompé's experimental eye solution, which is based on a proprietary recombinant human nerve growth factor (a protein that stimulates the growth and survival of neurons), aims to recover eyesight by restoring the nerve fibres that maintain the functions of the cornea — a completely transparent membrane, devoid of blood vessels — and heal ulcers on its surface. The preliminary findings from an early and mid-stage trial were presented at a meeting of the European Society of Cornea and Ocular Surface Disease Specialists in London on 13 September 2014.

Phase II trial underway

A Phase II double-blind trial is underway in 39 centres in nine European countries and is currently testing 90 patients with mid- and advanced-stage neurotrophic keratitis. The goal of the study, which recruited a total of 141 patients by December 2014, according to Dompé, is to assess the efficacy of two concentrations of the drug compared with placebo on complete healing of corneal lesions in addition to eyesight improvement.

Although it's not yet possible to know which patients received the active drug and which ones were treated with placebo, researchers have confirmed that five patients who had to be unmasked because their condition worsened while taking part in the study had in fact been treated with placebo.

These early findings follow encouraging data from the Phase I segment of the study, when 18 patients reported a considerable reduction of the symptoms linked to neurotrophic keratitis without any significant side effects. Scientists

IN SHORT

An experimental ophthalmology drug has shown promise as a treatment for neurotrophic keratitis, a rare degenerative disease. In this article, further details of the therapy and current trial information are discussed.

found that 73% of the patients reported the complete closure of the corneal wound after 8 weeks of treatment, while for those taking the highest concentration of the drug the lesions on the cornea took less time to heal.

Orphan drug

FDA and the European Medicines Agency have designated Dompé's treatment as an orphan drug. Although orphan drugs generally follow the same regulatory development path as any other pharmaceutical product, their developers face lessened clinical trial burdens because it may not be possible to test more than a thousand patients in a late-stage trial since far fewer people may be affected by the disease in question. Dompé aims to start discussions with regulators to try to obtain an accelerated approval and market the treatment as a therapy for neurotrophic keratitis without the need of further clinical studies, which could pave the way for a launch in the fourth quarter 2016 if the drug meets all its goals in the current trial.

Further mid-stage trials are underway to test the drug's potential as a therapy to treat dry eye syndrome and other conditions affecting the back of the eye, such as glaucoma and retinitis pigmentosa — a rare inherited degenerative disease that can lead to blindness for which there is no current treatment. Results for the retinitis pigmentosa study are expected to be available later this year.

REFERENCE

1. http://www.transparencymarketresearch.com/ophthalmic-drugs-market.html

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