News Release

Vekacia® (Ciclosporin 1mg/mL Eye Drops) Pivotal Trial Meets Primary and Key Secondary Endpoints

May 11, 2016 – Santen Europe (Geneva, Switzerland) and Santen Pharmaceutical Co., Ltd. (Osaka, Japan) have today announced that the Phase III clinical trial of its investigational drug, ciclosporin 1mg/mL eye drops, (proposed trade name Vekacia®), has met its primary and key secondary endpoints, demonstrating the investigational drug's efficacy and tolerability, versus placebo[1], in the treatment of active, severe vernal keratoconjunctivitis (VKC) in paediatric patients.[i] The trial, Vektis, was a multicentre, randomised, double blind, three parallel-arm, placebo-controlled study in patients aged 4–18 years.

VKC is a severe and recurrent allergic eye condition that mainly affects children (predominantly boys) and young adults, and which results in intense itching photophobia, painful eyes, and potentially even permanent loss of vision.[ii] The condition is characterised by severe inflammation of the ocular surface, including the conjunctivae and cornea (keratitis). Currently available anti-allergy pharmacological therapies for VKC have shown to be effective in mild-to-moderate forms of the disease, but they do not target the underlying immune process that initiates and perpetuates the allergic ocular surface inflammation – that requires a more potent agent.

Vekacia® contains ciclosporin, an immunomodulator that moderates the allergic response and inflammation associated with VKC.[ii] Vekacia® has been granted orphan drug status by the European Commission (EC) for the treatment of VKC, and if approved by the European Medicines Agency (EMA) will be the first drug that targets the underlying inflammation that causes VKC signs and symptoms.

"With these very encouraging results from Vektis, Vekacia® is delivering on its promise to provide an innovative and effective treatment that significantly improves outcomes for children with severe VKC," said Shigeo Taniuchi, Head of Santen Europe. "Our investment in VKC reflects our recognised commitment to eye care and to developing paediatric medicines that protect children's vision and restore quality of life."

Detailed results will be presented in due course at relevant scientific meetings.

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About VKC

VKC is a severe, recurrent, chronic allergic eye disease that causes inflammation of the conjunctiva (the membrane that lines the eyelid) and the cornea (the transparent layer in front of the pupil). It mainly affects children (predominantly boys) living in warm, dry climates and tends to resolve at puberty. The disease is characterised by seasonal or perennial symptoms, including itching, photophobia, tearing and burning, that worsen in the spring or early autumn.

VKC is a rare disease with a prevalence of 3.2 in 10,000 people in Europe.[iii]

[1] In this setting the placebo was a 'vehicle' with the equivalent delivery system as Vekacia®, minus the active pharmaceutical component.

About ciclosporin 1mg/mL eye drops (proposed trade name Vekacia®)

Vekacia® is a medicine that contains ciclosporin A (CsA) (1 mg/mL). It is administered as eye drops and has been developed as a sterile, positively charged (i.e. cationic), oil-in-water emulsion. This cationic emulsion – developed using Santen's proprietary Novasorb® technology – optimises the delivery of ciclosporin into the eye[iv] by binding with the overall negative charge of the ocular surface. Novasorb® increases the length of time that the ciclosporin remains on the ocular surface (residence time), thereby optimising its therapeutic effects.

About Vektis

The Phase III Vektis study was an international, multicentre, randomised, double-blind, three parallel-arm, vehicle (placebo)-controlled trial designed to assess the efficacy and tolerability of Vekacia® (ciclosporin) 1mg/mL eye drop emulsion in the treatment of severe VKC in paediatric patients. The study included 169 children and adolescents between the ages of 4–18 years who had severe VKC with severe keratitis. Participants were treated for four months, either with Vekacia® 1 mg/mL four times a day; or with Vekacia® 1 mg/mL twice a day and placebo twice a day; or with placebo four times a day. This was followed by an 8-month safety follow up period. Vektis met its primary and key secondary endpoints, with significantly greater improvement across efficacy measures, versus placebo. Vekacia® was also shown to be well tolerated.[i] Safety results are in accordance with other topical ciclosporin preparations.

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About Santen

As a specialty company dedicated to the ophthalmic field, Santen carries out research, development, sales, and marketing of pharmaceuticals. Santen is the market leader in Japan for prescription ophthalmic pharmaceuticals and sells products in over 50 countries. As a leading company in the field of ophthalmology, Santen aims to contribute to society by supplying valuable products and services to satisfy unmet medical needs. For more details, please see Santen's website (www.santen.com).

Santen Forward-looking Statements

Information provided in this press release contains forward-looking statements. The achievement of these forecasts is subject to risk and uncertainty from various sources. Therefore, please note that the actual results may differ significantly from the forecasts. Business performance and financial conditions are subject to the effects of changes in regulations made by the governments of Japan and other nations concerning medical insurance, drug pricing and other systems, and to fluctuations in market variables such as interest rates and foreign exchange rates.

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[i] Santen data on file.
[ii] Leonardi A. Management of vernal keratoconjunctivitis. *Ophthalmol Ther* 2013;2:73–88.
[iii] Bremond-Gignac D, et al. Prevalence of vernal keratoconjunctivitis: a rare disease? *Br J Ophthalmol* 2008;92:1097-1102.
[iv] Lallemand F, et al. Successfully improving ocular drug delivery using the cationic nanoemulsion, Novasorb. *J Drug Deliv* 2012;2012:604204.