

## **Santen Announces Phase III SAKURA Program Topline Results in Patients with Non-Infectious Uveitis of the Posterior Segment**

*– Topline Data from Largest Global Clinical Development Program in Non-Infectious Uveitis of the Posterior Segment Support Efficacy and Safety of OPSIRIA® –  
– Santen to Prepare for Submission to Regulatory Authorities –*

November 28, 2016, Osaka, Japan – Santen Pharmaceutical Co., Ltd. (hereinafter, “Santen”), a specialty ophthalmology company headquartered in Osaka, Japan, today announced the topline results of its SAKURA (Sirolimus study Assessing double-masked Uveitis tReAtment) global clinical development program designed to confirm the efficacy, safety, and optimal dose between three active doses of sirolimus intravitreal injection as monotherapy for patients with non-infectious uveitis of the posterior segment. The SAKURA Program met its objective, demonstrating that OPSIRIA (440 µg sirolimus injection, development code: DE-109) can effectively and safely reduce intraocular inflammation (as measured by vitreous haze).

Findings from SAKURA Study 1, the first Phase III trial, established the efficacy and safety of OPSIRIA as a potential treatment for non-infectious uveitis of the posterior segment. In SAKURA Study 2, the second Phase III trial, the difference in the effect (vitreous haze) between the low dose of sirolimus injection (44 µg) and OPSIRIA was not statistically significant, though clinical findings provide supportive evidence confirming the efficacy of the product. Based on the totality of the data from the SAKURA Program, Santen plans to file a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA).

“OPSIRIA will address a significant need in the management of non-infectious uveitis of the posterior segment as a locally-delivered option for this orphan disease. Santen is looking forward to filing an NDA in the U.S. in early 2017,” said Naveed Shams, MD, PhD, Chief Scientific Officer and Head of Global Research and Development at Santen.

### **About SAKURA**

The SAKURA Program is the largest Phase III global clinical program to date evaluating patients with non-infectious uveitis of the posterior segment. Both SAKURA Study 1 and Study 2 were multinational, randomized and double-masked, assessing the efficacy and safety of OPSIRIA as monotherapy in patients with non-infectious uveitis of the posterior segment. The primary endpoint of the studies was to achieve a vitreous haze score<sup>1</sup> of zero at month five. SAKURA Studies 1 and 2 enrolled patients under the same protocol through six months of treatment. Eligible patients were randomized into one of three active treatment arms (44 µg, 440 µg, 880 µg).

### **About OPSIRIA**

OPSIRIA (440 µg sirolimus injection) is a first-in-class intravitreal, locally-delivered,

targeted, immunoregulator being investigated for the treatment of non-infectious uveitis of the posterior segment – a progressive and chronic inflammatory disease of the eye. OPSIRIA regulates the immune system through the inhibition of mTOR which acts by interrupting the inflammatory cascade that leads to T-cell activation, differentiation and proliferation, and production of interleukin-2 (IL-2), as well as other pro-inflammatory cytokines and, also, promoting immune tolerance by inducing T regulatory cells (Tregs).

OPSIRIA was granted orphan drug designation by the U.S. Food and Drug Administration (FDA) and the European Commission (EC) in 2011.

### **About Uveitis**

Uveitis is a leading cause of preventable blindness in working-age adults and is estimated to account for 10 to 15 percent of cases of total blindness in the developed world.<sup>2</sup> It is characterized by intraocular inflammation that is often chronic, can flare up at any time and can lead to visual impairment and vision loss. Non-infectious uveitis of the posterior segment includes intermediate, posterior and panuveitis.

### **About Santen**

As a specialty company dedicated to the ophthalmic field, Santen carries out research, development, marketing, and sales 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](http://www.santen.com)).

### **Santen Forward-looking Statements**

Information provided in this press release contains so-called "Forward-looking Statements." The realizations of these forecasts are 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 condition are subject to the effects of change 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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<sup>1</sup> Standardized Uveitis Nomenclature [SUN] Photographic scale.

<sup>2</sup> Durrani OM, Tehrani NN, Marr JE, Moradi P, Stavrou P, Murray PI. Degree, duration, and causes of visual loss in uveitis. *Br J of Ophthalmol*. 2004;88(9):1159-1162.

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