

# **News Release**

# ASTELLAS ANNOUNCES RESEARCH COLLABORATION WITH HARVARD MEDICAL SCHOOL INVESTIGATOR FOR TREATMENT OF RETINAL DEGENERATION

- AIMing to establish a new treatment for ophthalmologic diseases –

*Tokyo, October 14th, 2014 ---* Astellas Pharma Inc. (Tokyo: 4503, President & CEO: Yoshihiko Hatanaka, "Astellas") announced today a research collaboration with an investigator at Harvard Medical School ("Harvard"), located in Boston, Massachusetts, USA, focused on discovering the pathologic mechanism for retinitis pigmentosa and identification of new therapeutic targets. Astellas and Harvard will work with the aim to provide a new treatment option with a view of a gene therapy, to retinitis pigmentosa patients who have difficulty maintaining good vision.

Retinitis pigmentosa is a retinal degenerative disease caused by genetic mutations and characterized by loss of peripheral and night vision, followed by loss of color vision and complete blindness. The pathogenesis of the retinal degeneration is still not fully understood and an effective treatment has yet to be established.

This collaborative research will be led by Constance L. Cepko, Ph. D., Professor of Genetics and of Ophthalmology at Harvard, who is also an investigator of the Howard Hughes Medical Institute, and is a worldrenowned authority on ophthalmology and gene therapy. Using adeno-associated virus vectors, genes that prolong vision in those who inherit retinitis pigmentosa will be identified and verified. Astellas will support the collaboration for up to three years. If the collaboration is successful in identifying genes that lead to promising treatment options for patients, Astellas will conduct further drug discovery on such promising candidates, and subsequent development and commercialization under an exclusive license from Harvard.

"For the purpose of enhancing the ability to generate new drugs, Astellas is looking for challenges in new therapeutic areas and novel technology platforms as well as new drug discovery opportunities that satisfy unmet medical needs through collaboration with external partners. This collaboration with Professor Cepko at Harvard is a part of this strategy. It is our hope that this collaboration to advance drug discovery will bring light to patients

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who struggle with retinitis pigmentosa," said Kenji Yasukawa, Ph. D., Senior Vice President and Chief Strategy Officer, Astellas.

Dr. Cepko had the following to say: "We are hopeful that some of our candidates will preserve the color and daylight vision of RP patients, regardless of the disease gene in their family. These treatments may also be effective in people that have other types of ocular disease, such as age-related macular degeneration, or glaucoma."

This partnership has been led by Innovation Management ("AIM"), Astellas.

#### About AIM

Innovation Management ("AIM") was a new division established in October 2013 in order to enhance and accelerate the process of screening and acquiring external opportunities to strengthen innovation at the preclinical development stage. AIM oversees strategic alliance activities with external partners and is responsible for a series of activities at acquiring external innovation opportunities in the preclinical development stage, such as strategy planning, screening, scientific assessment and alliance negotiations, so that strategic external business alliances can be strategically and systematically performed.

### **About Astellas**

Astellas Pharma Inc., located in Tokyo, Japan, is a pharmaceutical company dedicated to improving the health of people around the world through the provision of innovative and reliable pharmaceuticals. Astellas has approximately 18,000 employees worldwide. The organization is committed to becoming a global category leader in Urology, Immunology (including Transplantation) and Infectious diseases, Oncology, Neuroscience and Diabetes Mellitus (DM) Complications and Kidney diseases. For more information on Astellas Pharma Inc., please visit the company website at www.astellas.com/en.

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