PRESS RELEASE

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ALK-001 Receives FDA Orphan Drug Designation for the Treatment of Stargardt's Disease

Alkeus Pharmaceuticals Licenses Novel Compounds from Columbia University for Potential Treatments of Ophthalmologic Conditions such as Dry-AMD and Stargardt Disease

New York, NY and **Cambridge, Mass.** - Alkeus Pharmaceuticals and Columbia University announced today that they have entered into a license agreement for a set of potential therapies for the treatment of dry age-related macular degeneration (dry-AMD), Stargardt disease, and other degenerative diseases of the eye. Left untreated, these conditions often lead to impaired vision and even blindness.

Dr. Ilyas Washington, inventor of the technology and the Michael Jaharis Assistant Professor in the Department of Ophthalmology at Columbia University Medical Center, has found in preclinical testing that the compounds can reduce accumulation of certain toxic pigments in the eye. These pigments, which accumulate with age, are thought to be partially responsible for the vision loss associated with dry-AMD and Stargardt disease. Other conditions that can lead to impaired vision and are also associated with the accumulation of vitamin A aggregates may also be addressed, including Best's disease and certain forms of retinitis pigmentosa and of cone rod dystrophy.

"We have developed a modified vitamin A with the hope of halting vision loss or even restoring ocular function," Dr. Washington says. "Humans have evolved to make use of natural micronutrients or vitamins that regulate a wide variety of physiological functions.

By altering the chemical structure of these vitamins, we can potentially regulate or enhance the biological processes that they control."

Dr. Leonide Saad, Chief Executive Officer of Alkeus, explained, "These vitamin A aggregates accumulate in dry-AMD and, to an even faster extent, in Stargardt disease due to a genetic defect that exacerbates this process. Ilyas has elegantly shown, in a mouse model of Stargardt, that slightly changing vitamin A significantly reduces the rate of formation of these aggregates, eventually preserving visual function.

"While these results are at the preclinical stage, the way vitamin A interacts with the human body is very well understood and gives us confidence of the low risk and the high potential to tackle these serious ocular diseases," said Dr. Saad.

Other therapeutic compositions have been evaluated to reduce the accumulation of these vitamin A aggregates employing more invasive techniques, but the compounds developed by Dr. Washington rely on a novel and innocuous mechanism of action that strengthens the specific chemical bonds that have to be broken in order to form these aggregates.

"The problem with other approaches is that they affect the way vitamin A is processed in the eye, which often results in visual side effects such as slowed dark adaptation, hallucinations and night blindness," commented Dr. Saad.

"We are very pleased to be working with Alkeus for the continued development of these therapies. In Alkeus, we believe we have found the best partner to efficiently and effectively drive commercialization of these therapies," said Donna See, who oversees portfolio strategy and marketing for Columbia Technology Ventures, the technology transfer office of Columbia University. "As yet, there is no therapy to address impaired vision resulting from dry-AMD, a condition which affects millions of people around the world. Perhaps even more urgent is a therapy for the children who are affected by Stargardt. We look forward to hopefully one day being able to offer a way to help these patients and their families."

About dry age-related macular degeneration: Dry-AMD is the number one cause of blindness in the western world with several million Americans living with poor vision and at a high risk of turning blind. With the aging population, the number of patients with AMD is expected to double by 2020. Although dry-AMD symptoms progress more slowly than the wet form of the disease, dry-AMD represents nearly 90% of all the AMD cases in the United States, and remains without any treatment, contrary to wet-AMD which can be treated with periodic ocular injections.

About Stargardt disease: Stargardt disease is the most common form of inherited juvenile macular degeneration. It is a rare disease with a prevalence of one in 10,000 persons, or an estimated 30,000 people in the United States. Patients with Stargardt often start losing vision during their teenage years and will in most cases turn blind before becoming adults. As for dry-AMD, there is no treatment or cure.

About Columbia Technology Ventures

A leading academic and research university, Columbia University continually seeks to advance the frontiers of knowledge and to foster a campus community deeply engaged in understanding and addressing the complex global issues of our time. Columbia University's technology transfer office, Columbia Technology Ventures, manages Columbia's intellectual property portfolio and serves as the university's gateway for companies and entrepreneurs seeking novel technology solutions. Our core mission is to facilitate the transfer of inventions from academic research to outside organizations for the benefit of society on a local, national and global basis. For more information on Columbia Technology Ventures, please visit www.techventures.columbia.edu.

About Alkeus Pharmaceuticals

Alkeus is a clinical development biopharmaceutical company focused on developing new treatments for blindness and other serious ophthalmic conditions. Its lead compound, ALK-001, specifically addresses the excessive accumulation of toxic debris in the eyes of

patients with dry age-related macular degeneration and Stargardt's disease. For more information, please visit www.alkeus.com or email us at info@alkeus.com

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