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Alectos Therapeutics Announces FDA Orphan Drug Designation for MK-8719: An Investigational Small-molecule OGA Inhibitor for Treatment of Progressive Supranuclear Palsy

- **Advances Novel Tau-directed Strategy for Treating Neurodegenerative Disease**
- **Phase 1 Clinical Trial Completed**

Vancouver, British Columbia (April 20, 2016) – Alectos Therapeutics Inc. today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to OGA inhibitor MK-8719 for the treatment of progressive supranuclear palsy (PSP). Alectos also announced completion of a Phase 1 clinical trial in its ongoing collaboration with Merck, known as MSD outside the U.S. and Canada. This collaboration aims to develop agents that modulate the O-GlcNAcase (OGA) enzyme as a disease-modifying therapy for treatment of various neurodegenerative diseases. This follows the achievement of a previously undisclosed preclinical research milestone in 2012 under the same agreement.

“We are gratified that our collaboration with the exceptional team at Merck has progressed to clinical testing of this novel disease-modifying therapeutic approach for patients affected by PSP and other tauopathies” Ernest McEachern, Ph.D., CEO of Alectos, said. “This is an important validation of our scientific leadership in the area of OGA modulators, and we look forward to working with Merck to realize the full value of this novel mechanism for a range of neuroscience disorders”.

MK-8719 is an orally available potent and selective small-molecule OGA inhibitor planned for evaluation for the treatment of PSP. Like Alzheimer’s disease, PSP belongs to the family of neurodegenerative disorders known as tauopathies; these diseases are associated with the formation and progressive spread of toxic oligomers of tau protein in the brain. Preclinical data that has been independently confirmed by several groups provides evidence that OGA inhibition reduces tau pathology and associated neurodegeneration. Extensive preclinical validation, the oral bioavailability of MK-8719, and the use of biomarkers including a novel OGA PET ligand to establish target engagement in the brain set MK-8719 apart from previous efforts to treat tau related disease.

“Merck has a strong commitment to neuroscience.” said Darryle D. Schoepp, Ph.D., Vice President, Neuroscience Discovery, Merck Research Laboratories. “We are pleased to have advanced this first in class molecule in collaboration with Alectos and look forward to testing this novel mechanism in patients”.

Clinical development of MK-8719 was initiated with a single ascending dose Phase 1 study in healthy adult subjects to evaluate the safety, tolerability, and plasma pharmacokinetics of MK-8719. Single doses of MK-8719 up to 1200 mg were administered revealing favorable human pharmacokinetics and pharmacodynamic effects of OGA inhibition in blood. Brain OGA target engagement has also been assessed with a directed PET ligand in additional Phase 1 work that will support dose selection for efficacy studies. These data support further clinical development of MK-8719 and investigation of safety/tolerability and efficacy in PSP patients.

About Orphan Drug Designation

The FDA Orphan Drug Designation program provides a special status to drugs and biologics intended to treat, diagnose or prevent so-called orphan diseases and disorders that affect fewer than 200,000 people in the U.S. This designation provides for a seven-year marketing exclusivity period against competition, as well as certain incentives, including federal grants, tax credits and a waiver of PDUFA filing fees. The approval of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval.

About Progressive Supranuclear Palsy

PSP is a progressive neurodegenerative disorder, with an estimated worldwide incidence of six per 100,000 people. The disease affects approximately 20,000 individuals in the U.S. and 35,000 individuals in Europe. The most common features of PSP are loss of balance, blurred vision, and problems controlling eye movement. Other PSP symptoms, such as slowed movement and behavioral or cognitive changes, are similar to other brain disorders, particularly Parkinson's disease. The course of PSP is progressive and predisposes individuals to serious complications, such as choking, pneumonia, head injury and fractures caused by falls. Currently, there are no approved treatments for PSP. It is one of more than 20 different neurodegenerative disorders, including Alzheimer's disease, that are characterized by abnormalities in the brain protein tau. For more information on PSP, please visit www.psp.org.

About Alectos Therapeutics Inc.

Alectos Therapeutics is a private pharmaceutical company dedicated to the discovery and development of novel small-molecule drugs for human disease. Alectos was founded in 2007 as a spin-off company based on technologies initially discovered in the laboratory of Professor David Vocadlo at Simon Fraser University, who also serves as Alectos CSO. For more information, please visit our website at www.alectos.com. Digital images are available by request.

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