



Inflammasome Therapeutics Completes Enrollment in Study for First Oral Dual Inflammasome Inhibitor for Ophthalmic and Neuroinflammatory Diseases

- **Drug halts activation of two key inflammasomes believed to be key in progression of diseases with large unmet needs**
- **Potential treatment for wide range of diseases including ALS, Alzheimer’s, Parkinson’s, Multiple Sclerosis, Lupus, Macular Degeneration and Thyroid Eye Disease – all believed to progress due to inflammatory activity caused by inflammasome activation**

Newton, MA (September 10, 2024) – Inflammasome Therapeutics, a private company, has completed enrollment of a Phase I PK/Safety study of its new brain- and retina-penetrant drug K9, which has application in the treatment of multiple neuroinflammatory, degenerative diseases (such as Alzheimer’s, MS, ALS, Parkinson’s, geographic atrophy) and autoimmune diseases such as lupus and thyroid eye disease.

“These diseases all involve a type of inflammation called inflammasome activation, and the activation of two specific inflammasomes catalyzes the progression of these diseases. K9, taken as a tablet, penetrates the brain and retina and blocks both inflammasomes”, said Dr. Paul Ashton, CEO and cofounder of Inflammasome Therapeutics. “Drugs that block a single inflammasome have been found to have only moderate or no efficacy in models of MS and macular degeneration; we have seen dramatically superior results with our Kamuvudine molecules that block multiple inflammasomes.”

Delivering therapies across the tightly controlled blood-brain and blood-retina barriers to reach disease targets that are responsible for driving smoldering neuroinflammation within the central nervous system has been a formidable challenge in advancing innovation in neurological and retinal diseases. “Our data reveal that Kamuvudines have disease-modifying potential by targeting inflammation directly in the central nervous system as well as in the periphery,” said Dr. Jayakrishna Ambati, Inflammasome Therapeutics’ co-founder.

The successful completion of this study paves the way for further clinical trials of the drug, which Inflammasome Therapeutics expects to commence later this year.

The company’s other dual inflammasome inhibitor entered clinical trials earlier this year for geographic atrophy, a late stage of macular degeneration that is one of the most common causes of blindness in the elderly without a meaningfully effective treatment. That drug, K8, is delivered via a sustained delivery implant that is injected directly into the eye and releases minute but effective amounts of drug for three months.

Dr. Ambati has spent more than a decade leading research into the role of inflammasomes in triggering the inflammation that is believed to be the underlying cause of several neurodegenerative, autoimmune, and ophthalmic conditions for which there are no cures. Earlier, his research found that people taking a type of anti-HIV drug (called NRTIs) have a far lower risk of developing Alzheimer's disease and geographic atrophy. Unfortunately, NRTIs also have substantial toxicity. This led Dr. Ambati and his team to create new molecules formed by altering the structure of NRTIs to remove their toxicity creating a new class of drugs, Kamuvudines. Kamuvudines, have the same inflammasome-inhibiting action as their predecessors, but none of the toxicity.

Inflammasome Therapeutics (<https://www.inflam.com>) was founded by Jayakrishna Ambati, M.D. and Paul Ashton, Ph.D. to advance therapies for prevalent, degenerative diseases and to advance novel delivery technologies for sustained release of therapeutic agents and compounds. The company combines scientific excellence with proven development expertise and works to create breakthrough products via a mixture of licensing agreements and internal development.

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