Complement Therapeutics Receives FDA IND Clearance to Advance CTx001 into Opti-GAIN, a Phase I/II Clinical Trial in Geographic Atrophy secondary to AMD

- CTx001 is an investigational AAV-based gene therapy designed to modulate multiple pathways of the complement system
- Opti-GAIN is a first-in-human trial to evaluate the safety, tolerability, and preliminary efficacy of CTx001 as a potential one-time treatment for GA
- Opti-GAIN builds on insights from i-GAIN, a natural history study that has enrolled over 230 participants in the UK and US

Munich, Germany – 8th Oct 2025 - Complement Therapeutics GmbH (CTx), a biotechnology company developing next-generation therapeutics for complement-mediated diseases, today announced that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for CTx001, the company's lead gene therapy candidate. The IND clearance enables initiation of the Opti-GAIN Phase I/II clinical trial in patients with Geographic Atrophy (GA) secondary to Age-related Macular Degeneration (AMD). A condition that has a global prevalence of 5 million, affecting 1.5 million people in the US alone.

CTx001 is an adeno-associated virus (AAV)-based gene therapy designed to deliver a truncated version of Complement Receptor 1 (mini-CR1), enabling long-term modulation of the classical and alternative pathways of the complement cascade. GA is an advanced form of dry AMD that leads to irreversible vision loss, with limited therapeutic options currently available.

"FDA clearance of the IND for CTx001 is a major milestone for Complement Therapeutics and our mission to transform the treatment landscape for GA," said Dr. Rafiq Hasan, Chief Executive Officer of Complement Therapeutics. "It is a testament to the dedication, talent, and vision of our team that we've progressed from a university spinout to a clinical-stage company in just four years. With Opti-GAIN, we are entering the clinic with a highly innovative gene therapy candidate that has the potential to deliver durable, one-time treatment benefits for patients affected by this devastating disease."

The Opti-GAIN (Optimised Geographic Atrophy INterventional) trial is an international first-in-human, open-label, Phase I/II study designed to evaluate the safety, tolerability, and preliminary efficacy of CTx001. The trial will enrol patients across leading retinal centres with first patient dosing expected in the US in Q1 2026.

The trial design is informed by data from i-GAIN, a natural history study involving over 230 participants, which has provided valuable insights into disease progression, imaging biomarkers, and patient stratification in GA.

About Complement Therapeutics GmbH:

Complement Therapeutics GmbH (CTx) is a German headquartered early-stage biotechnology company focused on the research and development of novel therapeutics for complement-mediated diseases. The Company is a spinout from the University of Manchester and is based on the pioneering research of its founders into novel targets within the complement cascade.

Our lead investigational product (CTx001) is being evaluated as a potential gene therapy for GA, a leading cause of blindness. Additional programs will evaluate potential therapeutic opportunities in other complement-mediated conditions.

The Company has subsidiaries in the UK (Complement Therapeutics Ltd) and in the USA (Complement Therapeutics Inc) as well as research laboratories in Stevenage, UK.

About Geographic Atrophy

Geographic Atrophy (GA) is a leading cause of blindness in the elderly and represents the advanced stage of dry age-related macular degeneration. It is characterised by the progressive degeneration of photoreceptors, retinal pigment epithelium, and choriocapillaris, resulting in irreversible vision loss. GA affects over 5 million people globally and remains a significant unmet clinical need.

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For more information please visit: https://complementtx.com/

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