Complement Therapeutics Announces First Patient Dosed with CTx001 in the Phase I/II Opti-GAIN Study for Geographic Atrophy Secondary to AMD

- First patient dosed in Opti-GAIN, a first-in-human Phase I/II clinical trial of CTx001 in Geographic Atrophy secondary to AMD
- CTx001 is an investigational AAV-based gene therapy designed to deliver mini-CR1, a potent modulator of multiple pathways of the complement system
- Opti-GAIN is supported by Pre-GAIN, an ongoing natural history study designed to provide short-term GA progression insights and support the development of novel endpoints

Munich, Germany – 25 Mar 2026 - Complement Therapeutics GmbH (CTx), a clinical-stage biotechnology company developing next-generation therapeutics for complement-mediated diseases, today announced that the first patient has been dosed in Opti-GAIN, the Company's first-in-human Phase I/II clinical trial of CTx001 in patients with Geographic Atrophy (GA) secondary to Age-related Macular Degeneration (AMD).

Opti-GAIN is a multi-centre Phase I/II study evaluating the safety, tolerability and preliminary efficacy of CTx001 administered via a single subretinal injection. Part 1 is an open-label, dose-escalation study across three dose cohorts, followed by a dose-expansion phase in Part 2. The Opti-GAIN study is being conducted by the UK subsidiary of the company.

"Geographic Atrophy remains an area of significant unmet need, and there is a clear need to evaluate novel approaches for patients facing progressive vision loss," said Dr Arshad M. Khanani, the trial's Chief Investigator and Director of Clinical Research at Sierra Eye Associates, Reno, Nevada, USA. "I am pleased to be involved in this first-in-human study and to have administered CTx001 to the first patient in Opti-GAIN. I look forward to working with Complement Therapeutics to deepen our understanding of this promising one-time investigational gene therapy approach in patients with GA secondary to AMD."

CTx001 is an investigational AAV2-based gene therapy designed to transduce retinal cells with a construct encoding mini-CR1, a truncated and secreted form of Complement Receptor 1 that can modulate both the alternative and classical complement pathways. Subretinal delivery of CTx001 enables local retinal production of mini-CR1, whilst the small size of mini-CR1 may support penetration across Bruch's membrane resulting in broad ocular biodistribution, including the choriocapillaris.

Consequently, CTx001 builds on clinically validated complement biology in GA whilst aiming for a potentially best in class profile due to broad ocular biodistribution, strong potency and modulation of multiple complement pathways combined with extended durability.

The Opti-GAIN study is being advanced alongside Pre-GAIN, the Company's ongoing natural history study in GA currently enrolling in both the United States and the United Kingdom.

Together, these studies are intended to support patient selection and the evaluation of novel structural and functional endpoints, including ellipsoid zone (EZ) and focal Optical Coherence Tomography (OCT)-based microperimetry. Opti-GAIN is among the first studies to prospectively evaluate focal OCT-based microperimetry, an approach that has the potential to advance how the treatment effect is assessed in Geographic Atrophy.

"Dosing the first patient in Opti-GAIN is an important milestone for Complement Therapeutics and for the advancement of CTx001 in Geographic Atrophy," said Dr Rafiq Hasan, Chief Executive Officer of Complement Therapeutics. "We believe the combination of a differentiated asset and an integrated clinical development strategy sets CTx001 apart. By advancing Opti-GAIN alongside Pre-GAIN, we are generating natural history and interventional data to better inform patient selection, endpoint strategy and future clinical development."

Dr Muhammad Ali Memon, Chief Medical Officer of Complement Therapeutics, added: "The precise surgical dosing strategy, adaptive immunomodulatory regimen and well-characterised patient population, from Complement's non-interventional i-GAIN and Pre-GAIN studies, provides a strong platform for CTx001 to demonstrate a therapeutic effect within Opti-GAIN".

More information about the Company's clinical studies is available at ClinicalTrials.gov, including Opti-GAIN (NCT07392255) and Pre-GAIN (NCT07144137).

About the CTx001 program

CTx001 is an investigational AAV2-based gene therapy in development for Geographic Atrophy secondary to Age-related Macular Degeneration. The therapy is designed to deliver mini-CR1, a truncated and secreted form of Complement Receptor 1, to enable sustained local modulation of multiple complement pathways following a single subretinal injection.

About the Opti-GAIN Trial

Opti-GAIN is a Phase I/II multi-centre clinical study evaluating the safety, tolerability and efficacy of CTx001 administered via a single subretinal injection in 75 participants with Geographic Atrophy secondary to Age-related Macular Degeneration. Safety and efficacy will be assessed regularly over 2 years, followed by annual long-term safety follow-up for up to 5 years.

About the Pre-GAIN Trial

Pre-GAIN is a multi-centre, non-interventional, natural history study designed to provide insights into the short-term progression of Geographic Atrophy secondary to Age-related Macular Degeneration. The study is intended to characterise structural and functional measures of GA progression and may help identify participants for Opti-GAIN.

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.

About Complement Therapeutics GmbH:

Complement Therapeutics GmbH (CTx) is a German-headquartered clinical-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.

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

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