ONL Therapeutics Announces Randomization of First Patient in Global Phase 2 GALAXY Trial of Xelafaslatide (ONL1204) in Patients with Geographic Atrophy (GA) Associated with Dry AMD

Approvals received from World Health Organization and United States Adopted Names Council establishing "xelafaslatide" as nonproprietary name for ONL1204 Ophthalmic Solution

ANN ARBOR, Mich., **Oct. 28, 2025** – ONL Therapeutics, Inc., a clinical-stage biopharmaceutical company developing novel therapies for protecting the vision of patients with retinal disease, today announced that the first participant has been randomized in the company's Phase 2 GALAXY trial. GALAXY is a global Phase 2 clinical trial designed to evaluate the efficacy and safety of xelafaslatide (formerly ONL1204) in patients with geographic atrophy (GA) associated with dry age-related macular degeneration (AMD).

Xelafaslatide is an investigational first-in-class small molecule Fas inhibitor designed to protect key retinal cells, including photoreceptors, from cell death that occurs across a range of retinal diseases and conditions, including GA associated with dry AMD. Xelafaslatide is the newly established nonproprietary name for ONL1204 Ophthalmic Solution following approval from the World Health Organization and the United States Adopted Names Council.

"GA remains a devastating condition with a high unmet need. We are excited to be a part of the GALAXY trial as it provides an excellent opportunity to further understand the therapeutic potential of xelafaslatide," said Dr. David R.P. Almeida, M.D., M.B.A., Ph.D., FRCSC, executive chairman of Erie Retina Research in Erie, Pennsylvania. "With its novel therapeutic pathway targeting Fas, and dosing every three to six months, xelafaslatide has the potential to make a significant positive impact for patients while also lowering the treatment burden associated with currently approved GA therapies."

"The continued support of the retina specialist community for the GALAXY trial underscores the strong interest in xelafaslatide and its unique and differentiated mechanism of action targeting Fas," said David N. Zacks, M.D., Ph.D., chief scientific officer of ONL Therapeutics. "We are committed to advancing xelafaslatide as a potential breakthrough neuroprotection therapy for GA to help clinicians address the needs of

patients facing this progressive, vision-threatening disease. Enrolling the first patient is a critical step in our search for more effective and durable GA treatments."

GALAXY (NCT06659445) will enroll approximately 324 patients across sites in the US, Canada and the EU. The trial will build on data from a Phase 1b study that demonstrated xelafaslatide to be generally safe and well tolerated with encouraging efficacy signals observed after six months. Xelafaslatide, which is delivered via intravitreal injection, will be studied across three experimental arms, including two dose levels and two treatment frequencies (every 12 weeks or every 24 weeks). The primary endpoint is the rate of growth of the GA lesion area in patients treated with xelafaslatide versus sham as assessed by fundus autofluorescence (FAF) measured at 48 weeks. Additional timepoints will be measured out to 72 weeks, and an active reference arm will be applicable to patients at US sites only.

About Xelafaslatide (ONL1204 Ophthalmic Solution)

Xelafaslatide (ONL1204) is an investigational first-in-class small molecule Fas inhibitor designed to protect key retinal cells, including photoreceptors, from cell death that occurs across a range of retinal diseases and conditions. Death of these retinal cells, through both direct and inflammatory signaling pathways, is the root cause of vision loss and the leading cause of blindness. The company's later stage clinical development program for xelafaslatide includes a Phase 2 study for the treatment of GA associated with AMD (NCT06659445) and a completed Phase 2 study in the U.S. for the treatment of macula-off retinal detachment (RD) (NCT05730218), a condition for which the compound has been granted orphan drug designation by the United States Food and Drug Administration (FDA). The company has also completed a Phase 1b clinical trial in patients with GA associated with AMD (NCT04744662), a Phase 1b clinical trial in patients with progressing open-angle glaucoma (NCT05160805) and a Phase1 clinical trial in macula-off RD patients at sites in Australia and New Zealand (NCT03780972).

About Geographic Atrophy (GA) Associated with Dry Age-related Macular Degeneration (AMD)

AMD has become a major cause of visual disability and legal blindness globally. Although generally affecting only the central retina (macula), this region of photoreceptors provides the visual acuity necessary for reading, driving, and the performance of fine vision-related tasks. Associated with aging, cigarette smoking, obesity, diets low in certain nutrients, a lifestyle related to cardiac risk, and a growing list of genetic factors, AMD is becoming an increasingly prevalent public health concern, especially as the global population ages. GA, also called atrophic AMD, is an advanced form of AMD.

About ONL Therapeutics
ONL Therapeutics (ONL) is a clinical-stage biopharmaceutical company committed to developing first-in-class therapeutics to protect and improve the vision of patients with retinal disease. By advancing a breakthrough technology designed to protect key retinal cells from Fas-mediated cell death, ONL is pioneering a new approach to preserving vision.

For more information about ONL Therapeutics, please visit www.onltherapeutics.com.

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