Oculis Announces Agreement with FDA on a Special Protocol Assessment (SPA) for Optic Neuritis Registrational Trial

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- *Special Protocol Assessment (SPA) agreement with the U.S. FDA provides regulatory alignment on the registrational path for Privosegtor in optic neuritis (ON)*
- *FDA Breakthrough Therapy and EMA PRIME designations in ON underscore the significant unmet need and therapeutic potential of Privosegtor as a neuroprotective candidate*

ZUG, Switzerland, May 7 2026 -- Oculis Holding AG (Nasdaq: OCS / XICE: OCS) ("Oculis"), a global biopharmaceutical company focused on breakthrough innovations to address significant unmet medical needs in ophthalmology and neuro-ophthalmology, today announced that it has received written agreement from the U.S. Food and Drug Administration (FDA) under a Special Protocol Assessment (SPA) regarding PIONEER-1, the first registrational trial within the PIONEER Program evaluating Privosegtor for the treatment of optic neuritis (ON). This formal FDA agreement confirms the design and planned analysis of the PIONEER-1 study are adequate to address the objectives necessary to support a future NDA submission, subject to a successful trial outcome and FDA review of the complete submission.

Privosegtor is a novel peptoid small molecule that crosses the blood-brain and retinal barriers; it has the potential to become the first neuroprotective therapy for ON, with broad applicability in other neuro-ophthalmic and neurological diseases. Following the successful Phase 2 ACUITY trial, Oculis launched the PIONEER program which includes two pivotal trials to support registrational plans for Privosegtor in ON.

The PIONEER-1 Phase 3 study will evaluate Privosegtor in patients with ON across a broad population, including those with and without multiple sclerosis (MS). The primary endpoint is defined as the proportion of patients achieving at least a 15-letter gain from baseline in low-contrast visual acuity (LCVA) at Month 3, a well-established endpoint for clinically meaningful visual function in ophthalmology trials. Patients will be followed for 12 months to assess Privosegtor's long-term safety and tolerability. Dosing and patient enrollment criteria will closely mirror those of the Phase 2 ACUITY trial, in which Privosegtor + steroid showed substantial improvements in vision at Month 3, which persisted through Month 6, as measured by LCVA, along with consistent anatomical and biological neuroprotective benefits compared with placebo + steroid. The most common drug-related adverse events (AEs) were headache and acne (each in two participants; 10.5%). No drug-related serious AEs or AEs leading to treatment or study discontinuations occurred. These positive findings supported the granting of **Breakthrough Therapy designation** by the U.S. FDA and **Pri**ority **Me**dicines (**PRIME**) designation by the European Medicines Agency (EMA) for the treatment of ON.

Riad Sherif, M.D., Chief Executive Officer of Oculis, remarked, "The FDA SPA agreement for the PIONEER-1 trial, following Breakthrough Therapy and PRIME designations from the FDA and EMA, clarifies our path to NDA and validates our scientific approach. With a potential $7 Billion U.S. market in acute optic neuropathies, Privosegtor aims to address a critical gap in neuroprotection in neuro-ophthalmology and beyond."

Mark Kupersmith, M.D., Chief Medical Advisor, Neuro-ophthalmology and Professor, Vice chair translational research, Chair NORDIC at Icahn School of Medicine at Mount Sinai Hospital, New York, added: "Privosegtor has demonstrated compelling results in the treatment of optic neuritis with improvement of visual function combined with positive anatomical and biological measures of nerve cell preservation. The consistency of the results observed for all three determinations provides hope for patients suffering from optic neuropathies, many of whom have permanent visual deficits. I think this new therapy has the potential to bridge the gap from the lab to patients for neuroprotection in ophthalmology and neurology. I look forward to continuing our collaborative work with Oculis to further advance this promising candidate through late-stage clinical development."

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About Privosegtor
Privosegtor, a novel peptoid small-molecule candidate that crosses the blood-brain and retinal barriers, has the potential to become the first neuroprotective therapy for optic neuritis (ON) and other neuro-ophthalmic diseases. Positive results from the ACUITY Phase 2 trial demonstrated Privosegtor's neuroprotective potential through anatomical preservation of the retina and improvements in visual function after an acute episode of optic neuritis. Consistent results were observed in animal models of neuroinflammation and neurodegeneration, where Privosegtor preserved retinal ganglion cell damage and was associated with improvements in mobility (clinical function disability). Privosegtor has received Breakthrough Therapy designation from the FDA and Priority Medicines (PRIME) designation by the European Medicines Agency (EMA) as well as Orphan Drug from both the FDA and the EMA for ON. Privosegtor is currently being evaluated in Oculis' PIONEER (Privosegtor Investigation in Optic Neuropathies Efficacy Evaluation Research) program which includes two registrational trials in ON and one registrational trial in non-arteritic anterior ischemic optic neuropathy (NAION). In addition to its potential neuroprotective effect on the optic nerve, Privosegtor could also have wide applicability in treating other neuro-ophthalmic and neurological indications.

Privosegtor is an investigational drug and has not received regulatory approval for commercial use in any country.

About Optic Neuritis

Optic Neuritis (ON) is a rare condition characterized by an acute inflammation of the optic nerve that can lead to permanent visual impairment. It affects up to 8 in 100,000 people worldwide with a U.S. annual incidence estimated to be >30,000 and often represents the first sign of multiple sclerosis[1,2]. It mainly occurs in adults between the age of 20 and 40 years and is more frequent in women (2:1)[3]. ON is a type of neuropathy (nerve disease) that happens when acute inflammation of the optic nerve affects the signals traveling from the eyes through the brain, causing pain, vision loss and other symptoms. The cells that make up the optic nerve have a lipid protective coating called a myelin sheath, which is preferentially damaged in ON. Without myelin, the optic nerve cells can't send signals properly and axons can be irreversibly lost. To date there is no specific therapy approved for acute optic neuritis and the unmet needs remain for therapies that can prevent vision loss after an acute episode by reducing nerve cell permanent damage or death.

About Non-arteritic Anterior Ischemic Optic Neuropathy
Non-arteritic anterior ischemic optic neuropathy (NAION) is an acute optic nerve disorder that causes permanent visual impairment in >60% of affected patients[3]. It is the most common cause of acute optic nerve injury in individuals over 50 years old[4] and affects up to 10.2 per 100,000 people worldwide[5] with a U.S. annual incidence estimated to be >30,000[4,6,7]. In NAION, the optic nerve head region swells and there is painless sudden vision loss. The swelling eventually resolves, but the optic nerve axons and neuronal cell bodies (in the retina) are permanently lost, leading to

significant irreversible visual impairment or even blindness[8]. There are no approved therapies for NAION and the unmet medical need is for therapies that preserve vision and provide neuroprotection for patients suffering from NAION.

About Special Protocol Assessment (SPA)
A Special Protocol Assessment (SPA) is a written agreement between a sponsor and the U.S. Food and Drug Administration (FDA) regarding the design, endpoints, and planned statistical analyses of a clinical trial intended to support a marketing application. SPAs are intended to document FDA's agreement that the proposed trial design is adequate to support regulatory approval, provided the study is conducted as agreed and achieves its prespecified objectives. SPA agreement is generally sought for pivotal Phase 3 registrational trials intended to form a primary basis of evidence of effectiveness. SPA agreement provides greater regulatory clarity and predictability for drug development programs, but does not guarantee approval of a marketing application, which remains subject to the successful completion of the trial, submission of all required data, and FDA review.

About Oculis

Oculis is a global biopharmaceutical company (Nasdaq: OCS; XICE: OCS) focused on breakthrough innovations to address significant unmet medical needs in neuro-ophthalmology and ophthalmology. Oculis' highly differentiated late-stage clinical pipeline includes three core product candidates: OCS-01, an eye drop in pivotal registration studies, aiming to become the first non-invasive topical treatment for diabetic macular edema (DME); Licaminlimab, a novel, topical anti-TNFα in registrational trial, which is being developed with a genotype-based approach to drive precision medicine in dry eye disease (DED), and Privosegtor, a breakthrough neuroprotective candidate in the PIONEER program which consists of studies intended to support registration plans for treatment in optic neuropathies like optic neuritis (ON) and non-arteritic anterior ischemic optic neuropathy (NAION), with potentially broad clinical applications in various other neuro-ophthalmic and neurological diseases. Headquartered in Switzerland with operations in the U.S., Iceland and Switzerland, Oculis is led by an experienced management team with a successful track record and supported by leading international healthcare investors.

For more information, please visit: www.oculis.com

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Cautionary Statement Regarding Forward Looking Statements

This press release contains forward-looking statements and information. For example, statements regarding the potential benefits of the Company's product candidates, the initiation, timing, progress and results of current and future clinical trials, Oculis' research and development programs, regulatory and business strategy; Oculis' future development plans including the potential broad applicability of the Company's product candidates into additional indications; the timing or likelihood of regulatory filings and approvals; and statements about market opportunity, are forward-looking. All forward-looking statements are based on estimates and assumptions that, while considered reasonable by Oculis and its management, are inherently uncertain and are inherently subject to risks, variability, and contingencies, many of which are beyond Oculis' control. These forward-looking statements are provided for illustrative purposes only and are not intended to serve as, and must not be relied on by an investor as, a guarantee, assurance, prediction or definitive statement of a fact or probability. Actual events and circumstances are difficult or impossible to predict and will differ from assumptions. All forward-looking statements are subject to risks, uncertainties and other factors that may cause actual results to differ materially from those that we expected and/or those expressed or implied by such forward-looking statements. Forward-looking statements are subject to numerous conditions, many of which are beyond the control of Oculis, including those set forth in the Risk Factors section of Oculis' annual report on Form 20-F and any other documents filed with the U.S. Securities and Exchange Commission (SEC). Copies of these documents are available on the SEC's website, www.sec.gov. Oculis undertakes no obligation to update these statements for revisions or changes after the date of this release, except as required by law.

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