Oculis Announces U.S. FDA Breakthrough Therapy Designation Granted to Privosegtor for Treatment of Optic Neuritis

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ZUG, Switzerland, Jan. 06, 2026 (GLOBE NEWSWIRE) --

- *Breakthrough Therapy Designation granted to Privosegtor, a neuroprotective candidate, for the treatment of optic neuritis*
- *Privosegtor is advancing in the registrational PIONEER program across 2 key optic neuropathies, representing an unaddressed potential market of $7 billion in the U.S.*
- *Privosegtor achieved an average gain in Low Contrast Visual Acuity (LCVA) of 18 letters compared to IV steroid alone at month 3 in the ACUITY trial*

ZUG, Switzerland, January 6, 2026 -- Oculis Holding AG (Nasdaq: OCS / XICE: OCS) ("Oculis"), a global biopharmaceutical company focused on breakthrough innovations to address significant unmet medical needs in neuro-ophthalmology and ophthalmology, today announced that its neuroprotective candidate Privosegtor was granted breakthrough therapy designation by the U.S. Food and Drug Administration (FDA) for treatment of optic neuritis (ON).

Privosegtor, a novel peptoid small molecule designed to cross both the blood–brain and retinal barriers, has the potential to become the first neuroprotective therapy for optic neuropathies. These serious conditions carry a significant unmet need, because they can lead to permanent vision loss from nerve cell damage or death. There are no neuroprotective treatments currently available and together, they represent a potential market of $7 billion in the U.S. alone.

The FDA's Breakthrough Therapy Designation for Privosegtor is supported by visual -function results from the Phase 2 ACUITY trial in optic neuritis (ON), a rare, sight-threatening neuro-ophthalmic condition that is often the first clinical manifestation of multiple sclerosis. In the trial, Privosegtor delivered substantial improvement in LCVA along with consistent anatomical and biological benefits compared with placebo, reinforcing its potential as a neuroprotective treatment across both neuro-ophthalmic and neurological diseases.

In the ACUITY trial, Privosegtor produced substantial vision improvements on the 2.5% ETDRS Low Contrast Letter Acuity chart. Patients receiving Privosegtor 3 mg/kg/day plus IV methylprednisolone gained an average of 18 letters at three months compared with placebo plus IV methylprednisolone. For context, a 15-letter (three-line) gain represents roughly a two-fold improvement in visual resolution and is considered clinically meaningful for daily visual functioning. Privosegtor also showed anatomical preservation of retinal and optic nerve structure, which are typically damaged during acute optic neuritis. Additional analyses showed reduced neurofilament release, a biomarker of decreased neuroaxonal injury seen in conditions such as multiple sclerosis. 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.

Following a successful meeting with the FDA in 2025, Oculis launched the PIONEER program, which includes three pivotal trials to support registration plans for Privosegtor in ON and a second rare neuro-ophthalmic disease, NAION. These two optic neuropathies represent a potential market opportunity of potentially exceeding $7 billion in the U.S. alone, given the significant unmet medical need. The first trial in the program, PIONEER-1 in ON, was initiated in Q4 last year. This global study spans three continents. Sites activation is underway, and enrollment is expected to begin shortly.

Riad Sherif, M.D., Chief Executive Officer of Oculis, stated, "Today's Breakthrough Therapy Designation underscores Privosegtor's significant potential as a first-of-its-kind neuroprotective therapy for people living with optic neuritis, and highlights our commitment to redefining what's possible for patients suffering from neuroaxonal loss. With the ACUITY results and Privosegtor now progressing as a neuroprotective platform across key neuro-ophthalmic diseases, Oculis is uniquely positioned to reshape the treatment landscape in areas with substantial unmet needs, and 2026 is shaping up to be a milestone-rich year across our late-stage portfolio."

Mark Kupersmith, M.D., Chief Medical Advisor, Neuro-Ophthalmology, added: "The ACUITY trial delivered truly groundbreaking results, demonstrating for the first time in a single study that a drug candidate consistently improves visual function alongside anatomical and biological evidence of neuroprotective benefit. Significant unmet medical needs remain, as patients with optic neuritis—more often young women and frequently experiencing the first sign of multiple sclerosis—are still at high risk of permanent visual loss."

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About Privosegtor
Privosegtor, a novel peptoid small-molecule candidate that penetrates 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 Orphan Drug designation from both the FDA and the EMA for ON and is now entering registrational trials for this indication, as well as a registrational trial in non-arteritic anterior ischemic optic neuropathy (NAION), as part of Oculis' PIONEER (Privosegtor Investigation in Optic Neuropathies Efficacy Evaluation Research) program. 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. incidence estimated to be >30,000 and often represents the first sign of multiple sclerosis[1]. It mainly occurs in adults between the age of 20 and 40 years and is more frequent in women (2:1)[2]. 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. 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 the ACUITY Trial Supporting Breakthrough Therapy Designation
The Phase 2 ACUITY (Acute OptiC NeUrITis of DemYelinating Origin) trial was a randomized, double-blind, placebo-controlled, multi-center trial, designed to evaluate a once-daily intravenous infusion of Privosegtor over five days compared with placebo, in patients with acute optic neuritis receiving steroids. In addition to safety, other secondary efficacy endpoints were measured to evaluate the potential of Privosegtor on neuroprotection and visual function improvement in acute optic neuritis patients. The study randomized 36 eligible patients aged between 18 to 60, with recent onset (visual loss symptoms) of unilateral acute optic neuritis with a demyelinating origin, of which 33 patients received Privosegtor 2mg or 3 mg/kg/day plus IV methylprednisolone, or placebo plus IV methylprednisolone for five days.

About Breakthrough Therapy Designation[9]
Breakthrough therapy designation is intended to expedite the review of drugs for serious or life-threatening conditions. The criteria for breakthrough therapy designation require preliminary clinical evidence that demonstrates the drug may have substantial improvement on at least one clinically significant endpoint over available therapy. Approaches to demonstrating substantial improvement include the following:

- Direct comparison of the new drug to available therapy shows a much greater or more important response
- If there is no available therapy, the new drug shows a substantial and clinically meaningful effect on an important outcome when compared with a placebo or a well-documented historical control.
- The new drug added to available therapy results in a much greater or more important response compared to available therapy in a controlled study or to a well-documented historical control.
- The new drug has a substantial and clinically meaningful effect on the underlying cause of the disease, in contrast to available therapies that treat only symptoms of the disease, and preliminary clinical evidence indicates that the drug is likely to have a disease modifying effect in the long term (e.g., a sustained clinical benefit compared with a temporary clinical benefit provided by available therapies).
- The new drug reverses or inhibits disease progression, in contrast to available therapies that only provide symptomatic improvement.
- The new drug has an important safety advantage that relates to serious adverse reactions (e.g., those that may result in treatment interruption) compared with available therapies and has similar efficacy.

A breakthrough therapy designation conveys more intensive FDA guidance on an efficient drug development program, an organizational commitment involving senior managers, and eligibility for rolling review and priority review. FDA will review the full data submitted to support approval of drugs designated as breakthrough therapies to determine whether the drugs are safe and effective for their intended use before they are approved for marketing.

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: 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; OCS-01, an eye drop in pivotal registration studies, aiming to become the first non-invasive topical treatment for diabetic macular edema (DME); and Licaminlimab, a novel, topical anti-TNFα in Phase 2, which is being developed with a genotype-based approach to drive precision medicine in dry eye disease (DED). Headquartered in Switzerland with operations in the U.S. and Iceland, 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, including planned interactions with the FDA and potential benefits of breakthrough therapy designation; Oculis' future development plans; the timing or likelihood of regulatory filings and approvals; statements about market opportunity, and the Company's expected financial position and cash runway, 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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