PanOptica Reports Positive Results from Phase 1/2 Clinical Trial of PAN-90806, a Novel Topical Anti-VEGF Eye Drop

Late-Breaking Data Presented During Retina Subspecialty Day at AAO Annual Meeting Show Positive Biological Activity in Patients with Wet AMD

Next-Generation Formulation Scheduled to Enter New Phase 1/2 Clinical Trial in 2017

BERNARDSVILLE, N.J—October 14, 2016—PanOptica, Inc., a private biopharmaceutical company focused on licensing and developing innovative ophthalmology therapies, today reported positive data from a Phase 1/2 study of PAN-90806, a topical anti-vascular endothelial growth factor (anti-VEGF) eye drop in development for the treatment of neovascular eye diseases. Based on these results, the company plans to initiate a Phase 1/2 clinical trial of a next-generation formulation of topical PAN-90806 in patients with neovascular age-related macular degeneration (wet AMD) in 2017.

Scott W. Cousins, M.D., Robert Machemer Professor of Ophthalmology and Immunology, Vice Chair for Research, and Director of the Duke Center for Macular Diseases at Duke Eye Center, presented late-breaking data on behalf of investigators from the Phase 1/2 study in 50 treatment-naïve patients with wet AMD that received topical PAN-90806 during the 2016 Retina Subspecialty Day at the American Academy of Ophthalmology (AAO) Annual Meeting in Chicago. An independent panel of retina experts that included Dr. Cousins confirmed positive biological response to topical PAN-90806 in approximately 45-50% of treated patients, including outcomes such as vascular leakage, lesion morphology and vision. The reviewers also observed signals of anti-VEGF biological activity across all PAN-90806 monotherapy dose arms (n=40), including at the lowest doses.

“We are encouraged by our early clinical experience with PAN-90806 and its potential to empower patients to take a more active role in managing chronic treatment of their AMD,” said Dr. Cousins “If proven safe and effective through additional clinical trials, PAN-90806 topical anti-VEGF eye drop would reduce or eliminate injection-related risks and would be compatible with any combination therapy administered in any way.”

No treatment-related systemic adverse events (AEs) were reported. Local AEs were limited to ocular surface findings at the higher doses (most commonly punctate keratopathy) that were reversible upon discontinuation of treatment.

Dr. Cousins also reported on recent progress with the development of a new suspension formulation of PAN-90806, which demonstrates reduced corneal concentrations and a reduced risk of adverse corneal findings in exploratory non-clinical pharmacokinetic and toxicology studies, while maintaining excellent dose-dependent target tissue distribution to the central choroid and central retina.

“Based on the clinical observations of safety and tolerability exhibited in the first-in-man PAN-90806 Phase 1/2 study, along with the significant progress toward a better-tolerated next

generation formulation, we plan to pursue a higher dose range in our new Phase 1/2 study for topical PAN-90806 as monotherapy next year," stated Paul G. Chaney, President and Chief Executive Officer, PanOptica. "PAN-90806 is a potent, selective, small-molecule VEGF receptor blocker with unusually favorable characteristics for topical delivery to the back-of-the-eye. The next trial has the potential to define the optimal dose, regimen and path toward registration of the first effective topical treatment for wet AMD."

PAN-90806 also is being studied in a Phase 1 trial of patients with Proliferative Diabetic Retinopathy, and may have utility in other chronic neovascular eye diseases, including retinal vein occlusion, and other VEGF-dependent neovascular eye diseases. Retina specialists also have expressed interest in studying PAN-90806 as a potential strategy for preventing the progression to wet AMD in patients with high-risk dry AMD.

About PAN-90806
PAN-90806 is a potent and selective inhibitor of VEGF receptor signaling. VEGF is a protein that plays a critical role in angiogenesis (the formation of new blood vessels) and increased permeability (leakage from blood vessels), two pathological processes that contribute to the vision loss associated with wet AMD. In pre-clinical research using validated ocular angiogenesis models, topically administered PAN-90806 suppressed the formation of new blood vessels. In non-clinical pharmacokinetic studies, topical administration of PAN-90806 achieved significant and sustained levels in the retina and choroid of multiple species, supporting further investigation of PAN-90806.

About PanOptica
PanOptica, Inc., is a private, venture-backed biopharmaceutical company focused on licensing and developing a portfolio of exciting and innovative therapeutics for major ophthalmic diseases. In April 2014, the company closed a $45 million Series B financing backed by Third Rock Ventures, Novo Ventures, and founding investor SV Life Sciences. The company was co-founded by Paul Chaney, past president of OSI-Eyetech and Martin Wax, MD, a noted academic clinician scientist working in glaucoma, and most recently VP of research and development and head of ophthalmology discovery and pre-clinical development at Alcon Labs, Inc. The company seeks early-stage assets translated from other diseases and develops select candidates through human clinical proof of concept. For more information, please visit www.panopticapharma.com.

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