PanOptica Anti-VEGF Eye Drop Shows Promise in Treatment of Neovascular (Wet) AMD

- First Topical Anti-VEGF Eye Drop to Demonstrate both Safety and Biological Response as Monotherapy in Treatment-naïve Patients with Wet AMD -

- Phase 1/2 Data Presented at OIS@AAO Support Continued Clinical Development of PAN-90806 -

MOUNT ARLINGTON, N.J., Oct. 10, 2019 — PanOptica, Inc., a private biopharmaceutical company focused on developing innovative ophthalmology therapies, today reported positive and unprecedented clinical data for PAN-90806, a once-daily topical formulation of a small-molecule anti-vascular endothelial growth factor (anti-VEGF) eye drop for the treatment of neovascular eye diseases. At the Ophthalmology Innovation Summit at the American Academy of Ophthalmology annual meeting (OIS@AAO) in San Francisco, the company presented topline results from a Phase 1/2 dose-ranging clinical trial in which PAN-90806 was the first topical anti-VEGF eye drop to demonstrate both safety and biological response as monotherapy in treatment-naïve patients with neovascular age-related macular degeneration (wet AMD).

More than half of participants receiving once-daily topical PAN-90806 ophthalmic suspension for 12 weeks completed the study without needing rescue with anti-VEGF intraocular injection medication. Of those patients, 88% experienced either clinical improvement or stability of their disease, as confirmed by a panel of independent retina experts, with no serious or severe adverse effects related to PAN-90806.

“The Phase 1/2 trial results constitute the most robust set of data exploring a topical anti-VEGF eye drop as monotherapy, and the favorable safety profile and biological response to PAN-90806 support its continued clinical development in wet AMD and other neovascular eye diseases,” said Paul G. Chaney, president and chief executive officer of PanOptica. “The results suggest that topical ocular PAN-90806 may provide clinical benefit and substantially reduce the injection burden for patients with wet AMD, while increasing their chances for improved vision outcomes though maintenance of anti-VEGF therapy during the chronic management of their disease. We are now engaging with potential strategic partners in an effort to support the rapid development, registration, and commercialization of PAN-90806.”

Based on the strength of these data, PanOptica is eager to begin pivotal trials and has retained JMP Securities to represent the company in merger and acquisition discussions and to manage ongoing and future dialogue with potential strategic partners.

Phase 1/2 Neovascular AMD Trial Data

The double-masked, dose-ranging Phase 1/2 trial randomized 51 treatment-naïve patients to one of three once-daily doses of PAN-90806 monotherapy: 2 mg/mL (n = 16), 6 mg/mL (n = 18), or 10 mg/mL (n = 17). The primary endpoint was safety/tolerability; the secondary endpoint was anti-VEGF biological response. The study protocol allowed for rescue medication injections with ranibizumab beginning Week 2 of the 12-week study; investigators followed patients through one month (Week 16) following PAN-90806 discontinuation.
The independent Safety Monitoring Committee characterized PAN-90806 as reasonably safe and well-tolerated, with no major or serious drug-related safety concerns or trends. PAN-90806 ophthalmic suspension also exhibited improved tolerability and significantly improved safety over the previous, abandoned clinical solution. Key safety results are summarized below:

- Nine patients (17.6%) reported at least one PAN-90806-related adverse event (AE); none were serious. Five patients (9.8%) reported six PAN-90806-related corneal AEs.
- Three patients discontinued eye drop therapy prior to their Week 12 visit; two of these discontinuations were considered related to PAN-90806.
- Reported ocular adverse events were consistent with those observed in an AMD population and following treatment with topical ocular medications in general.
- Reported non-ocular events were consistent with those observed in elderly populations.

“The Phase 1/2 safety results and findings associated with anti-VEGF biological activity suggest that a topical eye drop may make anti-VEGF therapy safer, less burdensome, and more sustainable by helping patients avoid the frequent intravitreal injections that current regimens require,” commented Allen C. Ho, MD, FACS, Wills Eye Hospital Attending Surgeon and Director of Retina Research, Professor of Ophthalmology, Thomas Jefferson University, Mid Atlantic Retina, President, Retina Society. “Once-daily topical ocular PAN-90806 creates the opportunity to change the wet AMD treatment paradigm by potentially improving patient adherence, reducing losses to follow-up, and maintaining early patient benefits obtained with initial injection treatment throughout what may often be chronic, lifelong, anti-VEGF therapy.”

**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 in the form of an eye drop 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, further supporting the clinical investigation of PAN-90806.

**About PanOptica**

PanOptica, Inc., is a private biopharmaceutical company focused on developing innovative ophthalmology therapies. Investors include Third Rock Ventures and SV Health Investors. 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 vice president 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](http://www.panopticapharma.com).

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