November 7, 2019

Ocuphire Pharma Announces Results from Two Phase 2 Clinical Studies of Nyxol® Eye Drops

Results of MIRA-1 and ORION-1 Phase 2b Studies Confirm Drug Product Profile and Provide Basis for Additional Patent Claims

Nyxol Development to be Advanced in Night Vision Disturbances and Reversal of Mydriasis into Phase 3, and Presbyopia into Phase 2

FARMINGTON HILLS, Mich.--(BUSINESS WIRE)-- Ocuphire Pharma, Inc., a clinical-stage pharmaceutical company focused on the development and commercialization of therapies to treat patients with a variety of ophthalmic disorders, announced statistically significant and clinically meaningful results from its recently completed two Phase 2b studies of its lead drug candidate, Nyxol Eye Drops.

Top-line results of the primary and secondary endpoints of the MIRA-1 and ORION-1 studies provided confirmatory and novel data on a single dose and a once-daily, evening dosing regimen for Nyxol's pupil modulation effects. Results included a clear improvement in visual acuity, and Nyxol was affirmed to have an attractive safety and tolerability profile in both acute and chronic settings. Based on these data, combined with data from five prior trials, Ocuphire is prioritizing its clinical program to develop Nyxol in three important indications with currently unmet or poorly addressed needs: moderate-to-severe night vision disturbances (NVD), reversal of mydriasis (RM) and presbyopia. During the first half of 2020, Ocuphire plans to initiate two Phase 3 registration studies with Nyxol in NVD and RM, and a Phase 2 study to assess the combination of Nyxol plus a miotic agent in presbyopia.

MIRA-1 (NCT04024891) was designed as a multi-center, randomized, double-masked, placebo-controlled, crossover, single-dose Phase 2b trial with 32 healthy subjects (average age 28) to study 1% Nyxol's safety and efficacy in reducing pharmacologically induced mydriasis (dilation of pupil for eye exams). The primary endpoint was successfully met for the change from maximum pupil diameter (PD) at 2 hours post-treatment with one drop of 1% Nyxol compared to placebo ($p<0.0001$; ranked ANCOVA). Key secondary endpoints met statistical and clinical significance including Nyxol reducing the pupil diameter sooner than placebo at additional timepoints of 1 hour ($p<0.0001$) and 4 hours ($p<0.0001$) with two widely used mydriatic agents (i.e. parasympatholytic (tropicamide) and adrenergic (phenylephrine) eye drops). Also, Nyxol returned more subjects to their baseline vision (accommodation) at 2 hours as compared to placebo ($p=0.008$) after dilation with tropicamide. Overall, Nyxol was well-tolerated with no burning, no ptosis, no adverse events (AEs) that were probably related other than transient mild-to-moderate conjunctival redness (on-target effect, with no request for redness relievers such as Lumify®), and no severe adverse events (SAEs).

ORION-1 (NCT03960866) was designed as a multi-center, randomized, double-masked, placebo-controlled, multiple-dose Phase 2b study in 39 subjects (average age 60) with elevated intraocular pressure (IOP) ≥ 22 and ≤30 mmHg, evaluating safety, dosing schedule, pupil modulation, vision performance and IOP efficacy following once daily evening 1% Nyxol drop dosing for 14 days. The primary endpoint of change from baseline in mean diurnal IOP at Day 15 was not statistically significant, but there was a trend toward greater IOP lowering in subjects with lower pressure baselines with Nyxol as compared to placebo. Important to the NVD and presbyopia indications, the planned secondary endpoints with evening daily dosing of Nyxol were successfully met including a statistically significant and clinically relevant mean reduction in PD of 20% (1mm smaller pupil size) at both Day 8 and Day 15, that was sustained through 36 hours post-dose at Day 16. Further, Nyxol as a single agent showed statistical significance 1-line or greater of improvement from baseline in near visual acuity, with a trend 2-lines or greater at Day 8, Day 15, and Day 16. Nyxol was well-tolerated with no burning, no ptosis, no tachyphylaxis, no rebound, and no other AEs or SAEs. Unlike other topical drugs, there was no statistically significant difference in redness (conjunctival hyperemia) in the morning between Nyxol and placebo with evening dosing.

“Ocuphire is emerging as a new and relevant company in the ophthalmology and optometric arena,” stated Dr. Eliot Lazar, Clinical Ophthalmologist and President at elCON Enterprises, Inc. “Their newest data set demonstrates a clear signal that met all relevant pupil modulation, vision performance, and safety endpoints. This will hopefully translate to a novel therapeutic agent (Nyxol) that will aid in remediating night vision disturbances, a true unmet need. Similarly, Nyxol is expected to be a valuable tool in the reversal of mydriasis which will assist clinicians and patients alike. This data establishes the foundation to move forward and advance their technology to the next stages of development.”

"The MIRA-1 and ORION-1 Phase 2b studies clearly validate Nyxol’s overall product profile from prior studies,” stated Dr. Paul Karpecki, Clinical Director of Cornea at Kentucky Eye Institute. “Importantly, the consistent and clinically relevant reduction in pupil diameter, improvement in near visual acuity and accommodation, and overall safety profile with only transient mild redness support the adoption of Nyxol in physician practices.”

The Ocuphire team, including its key clinical advisors, will continue to evaluate the complete datasets from MIRA-1 and ORION-1 as it prepares for the End of Phase 2 meeting with the FDA for the overall Nyxol program. Full results from these studies will be submitted for presentation at upcoming medical meetings.

“We are excited to see Nyxol advance to Phase 3, particularly in NVD, which was the original inspiration for the drug and includes millions of individuals with no treatment options today,” said Mina Sooch, Chair, President, and Chief Executive Officer of Ocuphire. “Additionally, reversing pupil dilation following the tens of millions of eye exams in doctor’s offices represents a unique single-dose treatment opportunity with a simpler and faster regulatory path to approval given its an acute versus chronic indication.”

Based on findings from the two studies, Ocuphire has bolstered its intellectual property portfolio with the filing of two more patent applications relating to Nyxol as a single agent or in combination with other approved ophthalmic agents in the treatment of presbyopia, reversal of mydriasis and glaucoma. All of the global intellectual property covering Nyxol is wholly owned by Ocuphire.

About Nyxol

Nyxol is a novel eye drop drug candidate for multiple front-of-the-eye disorders, including night vision disturbances (NVD), reversal of mydriasis (RM), presbyopia, and normal-tension glaucoma. Nyxol is a proprietary ophthalmic formulation of phentolamine mesylate, an approved alpha-1 and alpha-2 inhibitor, allowing a more efficient 505(b)(2) development pathway for approval. With safety and efficacy data from seven Phase 1 and Phase 2 trials, Nyxol has demonstrated a differentiated target product profile that includes moderately reducing pupil size (which leads to improved vision night and day at both far and near distances), lowering intraocular pressure in normal range, and convenient once-daily evening dosing with durable effects. Nyxol was originally invented by Dr. Gerald Horn, an ophthalmologist and laser vision specialist in Chicago, who also invented the recently-approved redness reliever eye drop Lumify®.

About Night Vision Disturbances

NVD causes people to experience glare, halos, starbursts, and poor vision in dim light. NVD is a significant unmet need, as it affects more than an estimated 4 million people in the U.S., and there is no approved drug therapy.

About Medically-Induced Mydriasis

Medically-induced mydriasis, or dilation, occurs as part of more than 80 million eye exams conducted in the U.S. every year, as well as millions of specialty exams. Mydriasis increases light sensitivity and impairs vision for hours, and there is currently no treatment available on the market.

About Presbyopia

Presbyopia is the progressive loss of the eye's ability to focus on near objects, and it affects everyone to some degree with aging (over 100 million people in the U.S.), generally beginning at around 40 years. There is no cure for the condition, which is generally treated with corrective lenses, however the approach of using pupil modulation is being investigated in clinical trials.

About Ocuphire

Ocuphire is a clinical-stage biopharmaceutical company engaged in the development and commercialization of drugs to treat important ophthalmic disorders. The company's lead drug candidate, preservative-free Nyxol Eye Drops, is being developed for multiple front-of-the-eye indications. Please visit www.clinicaltrials.gov to learn more about our past clinical trials. For more information, please visit www.ocuphire.com.

Forward-looking statements are made in this press release and you are cautioned not to put any undue reliance on such statements. There are a number of risks and uncertainties that could cause actual events to differ from the expectations indicated in these forward-looking statements. This press release shall not constitute an offer to sell or the solicitation of an offer to buy, nor shall there be any sale of these securities in any state or jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such state or jurisdiction.

View source version on businesswire.com:
https://www.businesswire.com/news/home/20191107005535/en/

Investor Contact:
Mina Sooch
President and CEO
msooch@ocuphire.com
248-681-9815

Source: Ocuphire Pharma, Inc.