Oyster Point Pharma Announces Positive Results in ONSET-2 Phase 3 Trial of OC-01 Nasal Spray for the Treatment of the Signs and Symptoms of Dry Eye Disease

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ONSET-2 met the prespecified primary endpoint in both doses tested, demonstrating statistically significant improvement in Schirmer's score from baseline to Week 4 in subjects receiving OC-01 nasal spray versus control (p<0.0001)

Key secondary symptom endpoints also met in the 1.2 mg/ml dose group, showing symptom improvement at Week 4 (p=0.002) and as early as Week 2 (p=0.009) compared to control

NDA submission planned for 2H 2020

Conference call and live webcast scheduled for 8:00 a.m. ET to review ONSET-2 topline data

PRINCETON, N.J., May 11, 2020 (GLOBE NEWSWIRE) -- Oyster Point Pharma, Inc. (Nasdaq: OYST), a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of first-in-class pharmaceutical therapies to treat ocular surface diseases, announced positive top-line results from the Phase 3 ONSET-2 study in dry eye disease. The ONSET-2 trial met the primary endpoint, where a greater percentage of subjects treated with 0.6 mg/ml and 1.2 mg/ml of OC-01 gained ≥10 mm on Schirmer's score, a measure of tear film production, compared to control. Key secondary endpoints were met, including an improvement in eye dryness score (EDS) in the normal clinic environment as well as mean change in Schirmer's score in the 1.2 mg/ml dose group. The overall safety profile of OC-01 nasal spray was found to be consistent with prior data without any new safety signals.

"The results announced today demonstrate impressive improvements in signs and symptoms of dry eye disease," said Dr. Preeya Gupta, Associate Professor of Ophthalmology at Duke University School of Medicine and member of Oyster Point Pharma's medical advisory board. "This novel treatment approach for dry eye disease is a much needed addition to the dry eye treatment armamentarium. The ONSET-2 study results represent outcomes from a broad population of dry eye patients and we believe they should translate to treating patients in the clinic."

Jeffrey Nau, PhD, MMS CEO, Oyster Point Pharma, said, "The ability to show statistically significant sign and symptom endpoints within the same clinical trial has been elusive in dry eye disease. ONSET-1 and ONSET-2 have independently met endpoints of both signs and symptoms in their respective trial populations. The ability to meet this high bar in the ONSET-2 population consisting of mild, moderate, and severe subjects is even more notable and speaks to the broad applicability of OC-01 to treat dry eye patients. We look forward to submitting the New Drug Application to FDA for OC-01 nasal spray to treat signs and symptoms of dry eye disease in the second half of 2020. If approved by the FDA, we remain on track for a planned U.S. launch in the fourth quarter of 2021."

ONSET-2 Topline Results

The ONSET-2 Phase 3 trial was a multicenter, randomized, double-masked, vehicle-controlled clinical trial designed to evaluate the safety and efficacy of OC-01 (varenicline) nasal spray for the signs and symptoms of dry eye disease. The study enrolled 758 subjects at 22 centers in the United States and investigated two doses of OC-01 nasal spray, 0.6 mg/ml and 1.2 mg/ml, as compared to control (vehicle) nasal spray. Subjects were administered OC-01 nasal spray twice daily for 4 weeks.

For the primary endpoint, both tested doses of OC-01 showed a statistically significant improvement in subjects gaining ≥ 10 mm in Schirmer's score at Week 4 as compared to control. In the 0.6 mg/ml subject dose group, the percentage of subjects gaining ≥10mm on Schirmer's score was 44% (p<0.0001 vs. control). In the 1.2 mg/ml subject dose group, the percentage of subjects gaining ≥10mm on Schirmer's score was 47% (p<0.0001 vs. control). In the control group, the percentage of subjects gaining ≥10mm on Schirmer's score was 26%.

Additionally, consistent with the ONSET-1 clinical trial, there was a statistically significant improvement in mean change in Schirmer's score at Week 4 in both doses tested as compared to control. In the group of subjects treated with the 0.6 mg/ml dose at Week 4, the mean change from baseline in Schirmer's score was 11.0 mm (p<0.0001 vs. control). In the 1.2 mg/ml dose group at Week 4, the mean change from baseline on Schirmer's score was 11.2 mm (p<0.0001 vs. control). Mean change from baseline on Schirmer's score in the control group was 5.9 mm.

Multiple secondary endpoints were assessed in ONSET-2 including measurement of symptoms in the normal clinic environment as well as in a controlled adverse environment chamber. Eye Dryness Score measured in the normal clinic environment demonstrated statistically significant results in the 1.2 mg/ml dose group at Week 4 (p=0.002 vs. control), although the 0.6 mg/ml dose was not statistically significant (p=0.07). The 0.6 mg/ml and 1.2 mg/ml doses of OC-01 nasal spray did not meet the secondary endpoint for patient-reported symptoms of eye dryness in the Controlled Adverse Environment® (AE) (Ora®, Andover, MA) at Week 4. However, both OC-01 doses exhibited a directional benefit as compared to control. The statistical power for assessing this endpoint was negatively impacted by a decrease in the sample size due to the subjects being unable to be assessed as a result of the coronavirus pandemic. In addition, there were a number of subjects who did not meet criteria for treatment in the chamber, which further reduced statistical power.

Additionally, the 0.6 mg/ml (nominal p=0.049 vs. control) and 1.2 mg/ml (nominal p=0.009 vs. control) dose of OC-01 nasal spray showed statistical significance in Eye Dryness Score measured at Week 2.

OC-01 was well-tolerated in the ONSET-2 clinical trial, and the adverse event profile was consistent with the ONSET-1 clinical trial. The most common

adverse event experienced in the treatment groups was sneeze, which occurred with 50% of nasal spray administrations, was transient (majority of sneezes occurred within the first minute following administration), and mild in severity. There were no reports of serious adverse events related to nasal administration. The number of subjects with treatment emergent adverse events related to study drug leading to discontinuation was 2% or less in either treatment group.

Conference Call Details and Webcast

Oyster Point Pharma will host a live conference call and webcast today at 8:00 am Eastern Time to discuss the ONSET-2 topline data, the first quarter 2020 financial results and provide a business update. To access the live call by phone, please dial (855) 548-1220 (US/Canada) or (602) 563-8619 (International). The conference ID number is 6356809. The webcast will be made available on the company's website at www.oysterpointrx.com under the "Events & Presentations" section of the company's website at https://edge.media-server.com/mmc/p/dngsj6eh.

A telephone replay will be available for approximately 7 days following the live conference call. To access the telephone replay, please dial (855) 859-2056 (US/Canada) or (404) 537-3406 (International). The conference ID number is 6356809**.** A replay of the webcast will be available for approximately 30 days following the live audio webcast.

About OC-01 Nasal Spray

OC-01 is a highly selective nicotinic acetylcholine receptor (nAChR) agonist, being developed as a preservative free nasal spray to treat the signs and symptoms of dry eye disease (DED). The parasympathetic nervous system, the "rest and digest" system of the body, controls tear film homeostasis partially via the trigeminal nerve which is accessible within the nose. Administered as a preservative-free, aqueous nasal spray, OC-01's novel mechanism of action activates the trigeminal parasympathetic pathway in the nasal cavity to stimulate natural tear film production. Human tear film is a complex mixture of more than 1,500 different proteins, including growth factors and antibodies, as well as numerous classes of lipids and mucins. This complex tear film coating is responsible for forming the primary refracting surface of the cornea, as well as protecting and moisturizing the cornea.

About Oyster Point Pharma

Oyster Point Pharma is a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of first-in-class pharmaceutical therapies to treat ocular surface diseases. Oyster Point Pharma's lead product candidate, OC-01 nasal spray, a highly selective nicotinic acetylcholine receptor (nAChR) agonist, is being developed to treat the signs and symptoms of dry eye disease. OC-01 nasal spray's novel mechanism of action is designed to re-establish tear film homeostasis by activating the trigeminal parasympathetic pathway to stimulate the glands and cells responsible for natural tear film production, known as the lacrimal functional unit (LFU).

About Dry Eye Disease

Dry eye disease is a chronic, progressive condition that impacts more than 30 million Americans and is growing in prevalence. An estimated 16 million U.S. adults have been diagnosed with dry eye disease, a multifactorial condition of the ocular surface characterized by disruption of the tear film. A healthy tear film protects and lubricates the eyes, washes away foreign particles, contains growth factors and antimicrobial components to reduce the risk of infection, and creates a smooth surface that contributes refractive power for clear vision. Dry eye disease can have a significant impact on a person's day-to-day quality of life, as it can cause persistent stinging, scratching, burning sensations, sensitivity to light, blurred vision, and eye fatigue. Despite the large prevalence of dry eye and the burden of the disease, there remains a significant unmet need for effective therapies.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on our current beliefs, expectations and assumptions and on information currently available to us. The forward-looking statements in this press release represent our views as of the date of this press release. These statements may include but are not limited to statements regarding our plans for and the anticipated benefits of and safety of our product candidates, the timing, objectives and results of the clinical studies and anticipated regulatory and development milestones, including potential timing of NDA submission and potential commercialization. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. Forward-looking statements may involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from those expressed or implied by the forward-looking statements. Accordingly, readers are cautioned not to place undue reliance on these forward-looking statements. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements. Important factors that could cause our actual results to differ materially are detailed from time to time in the reports we file with the Securities and Exchange Commission, copies of which are posted on our website and are available from us without charge. However, new risk factors and uncertainties may emerge from time to time, and it is not possible to predict all risk factors and uncertainties.

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