PRESS RELEASE

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Ascelia Pharma Successfully Meets Primary Endpoint with Strong Headline Results in Orviglance Phase 3 Study

Ascelia Pharma AB (publ) (ticker: ACE), a biotech focused on improving the life of people living with rare cancer conditions, today announced that liver imaging drug candidate, Orviglance®, significantly improved visualization of focal liver lesions, successfully meeting the primary endpoint in the pivotal Phase 3 study SPARKLE. Investors and analysts are invited to the virtual Investor Update: "Bringing Orviglance to Patients", on Tuesday, 7 May at 14:00 CEST

- The Phase 3 study demonstrated strong superiority in visualization of focal liver lesions with Orviglance (CMRI) vs. unenhanced MRI with statistical significance for all three readers (<0.001)
- Orviglance is in development as a first-in-class contrast agent for use in liver MRI in patients with severely impaired kidney function and has been granted FDA Orphan Drug Designation
- Orviglance addresses a global USD 800 million market annually
- The Phase 3 data marks the completion of Orviglance clinical development with nine studies in 286 patients and healthy volunteers
- Submission of the New Drug Application (NDA) file to the US Food and Drug Administration (FDA) is expected by mid-2025
- Investors, analysts and media are invited to the virtual Investor Update and live Q&A on next steps: "Bringing Orviglance to Patients", on Tuesday, 7 May 2024 at 14:00 CEST

The pivotal Phase 3 study, SPARKLE, successfully met the primary endpoint and demonstrated that the company's magnetic resonance imaging (MRI) contrast agent, Orviglance significantly improved the visualization of focal liver lesions compared to unenhanced MRI. The results for all three readers were highly statistically significant (P values <0.001).

The reliability of the data is strong and conclusive for all readers – this includes an acceptable level of variability.

Common adverse events in this vulnerable patient population were in line with previous studies with Orviglance, such as mild- to moderate nausea. No serious adverse drug reactions were observed.

"We are thrilled to announce these strong and convincing Phase 3 results for Orviglance. This is the most significant milestone achievement for Ascelia Pharma so far", said Magnus Corfitzen, CEO of Ascelia Pharma. "Following considerable challenges during the SPARKLE study, we are very excited to complete the Orviglance clinical development with these strong results in Phase 3. We now look forward to advancing Orviglance through the registration process and make it available for patients."

The Company will now focus on bringing Orviglance through the regulatory submission and approval process. In parallel, we will continue to advance launch readiness and dialogue with potential commercialization partners to make Orviglance available to patients who need high-quality liver imaging without gadolinium-related safety risk.

“On behalf of the entire Ascelia Pharma team, I would like to thank patients and investigators involved in the SPARKLE study as well as other partners instrumental in completing the successful data read-out. We look forward to discussing the results with the medical community and regulatory authorities”, said Magnus Corfitzen.

The Company expects to submit the New Drug Application (NDA) file to the US Food and Drug Administration (FDA) by mid-2025 to obtain regulatory approval.

Investors, analysts, and media are invited to the virtual Investor Update: “Bringing Orviglance to Patients”, on Tuesday, 7 May 2024 at 14:00 CEST. In this update and live Q&A, executive management will further explain the strong headline results and share plans for the upcoming regulatory and commercialization activities. A link to the event will be available on the company’s website (www.ascelia.com).

Clinical development of Orviglance has now been completed with consistent positive efficacy and safety data from 286 patients and healthy volunteers in nine studies, of which the SPARKLE is the last and pivotal study. Patient enrollment in the global multi-center SPARKLE study was completed early 2023, with MRI data from 85 patients with known or suspected focal liver lesions and severely impaired kidney function. In accordance with regulatory requirements, the improvement of visualization of lesions was evaluated by three independent radiologist readers. In mid-2023, the unexpected discovery of high intra-reader variability in the study image scoring by the readers prevented the Company from evaluating the efficacy data from SPARKLE. Therefore, a new evaluation of the images with new readers was required. The company successfully completed the re-evaluation according to the planned timeline with the announcement today.

Orviglance has been granted an Orphan Drug Designation by the US Food and Drug Administration (FDA) for liver MRI in patients with severely impaired kidney function. These patients have the highest risk of developing the serious and potentially fatal condition Nephrogenic Systemic Fibrosis (NSF) after exposure to the currently available gadolinium-based contrast agents. Regulatory bodies have issued warnings for the use of these agents in this vulnerable patient population. Therefore, there is a need for an alternative solution to detect and visualize focal liver lesions in patients with impaired kidney function, who today typically receive inferior unenhanced MRI. Liver MRI is a cornerstone in cancer care, as liver metastases are common in many cancer types and often the cause of mortality. Orviglance aims to give patients with impaired kidney function access to safe and effective liver imaging to live longer and healthier lives. The unmet need for these patients represents an addressable market potential of USD 800 million globally.

*The Company considers mid-2025 to be the period from the second half of Q2 to the first half Q3.

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About Us

Ascelia Pharma is a biotech company focused on orphan oncology treatments. We develop and commercialize novel drugs that address unmet medical needs and have a clear development and market pathway. The company has two drug candidates – Orviglance and Oncoral – in clinical development. Ascelia Pharma has global headquarters in Malmö, Sweden, and is listed on Nasdaq Stockholm (ticker: ACE). For more information, please visit http://www.ascelia.com.

About Orviglance

Orviglance (manganese chloride tetrahydrate) is a novel oral contrast agent for MR-imaging developed to improve the detection and visualization of focal liver lesions (including liver metastases and primary tumors) in patients with reduced kidney function. These patients are at risk of serious side effects from the currently available class of gadolinium-based contrast agents. Orviglance, has been granted an Orphan Drug Designation by the US Food and Drug Administration (FDA). A clinical program of nine studies, including the pivotal global Phase 3 study SPARKLE, has successfully been completed with strong and consistent efficacy and safety results.

About Oncoral

Oncoral is a novel irinotecan chemotherapy tablet developed initially for the treatment of gastric cancer. Irinotecan chemotherapy has an established potent anti-tumor effect. Oncoral is a daily tablet with the potential to offer better patient outcomes with improved safety following the daily dosing at home compared to intravenous high-dose infusions at the hospital. Following successful Phase 1 results, Oncoral is now prepared for Phase 2 clinical development.

This information is information that Ascelia Pharma is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact persons set out above, at 2024-05-02 11:12 CEST.

Attachments

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