PRESS RELEASE

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Ascelia Pharma Announces Completion of Full Study Report Reinforcing the Successful Outcomes of Orviglance Phase 3 Study SPARKLE

Ascelia Pharma AB (publ) (ticker: ACE), a biotech focused on improving the life of people living with rare cancer conditions, today announced the completion of the Full Study Report for the pivotal Phase 3 Study for Orviglance® liver imaging drug candidate, which includes the previously announced strong results of primary endpoints. In addition, the results of secondary endpoints further reinforce the successful study outcomes and support the New Drug Application (NDA) process.

Ascelia Pharma announced on 2 May 2024, that 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 in patients with severely impaired kidney function.

The Full Study Report has now been completed and includes the previously announced strong results of primary endpoints and safety. The report also includes the results of secondary endpoints, which further reinforce the successful study outcomes and support the NDA process and potential clinical value of Orviglance.

"With the Full Study Report, we are pleased to pass yet another milestone on our path to obtaining regulatory approval and entering into commercialization partnerships for Orviglance", says Magnus Corfitzen, CEO of Ascelia Pharma.

Primary endpoint concludes improved visualization with Orviglance
As previously announced, the primary efficacy of Orviglance in liver MRI was demonstrated by improved visualization of focal liver lesions in Orviglance-enhanced* images compared to unenhanced images. For this endpoint, three independent, blinded readers assessed border delineation (BD) and lesion contrast (LC) on scales from 1 ('poor') to 4 ('excellent') of all lesions for each patient**. Overall, Orviglance enhanced visualization from 'moderate' or 'good' to 'good' or 'excellent'.

- For unenhanced images, the median BD and LC scores ranged from 2.1 to 3.0 across readers
- For Orviglance-enhanced images, the median BD and LC scores increased to 3.0 and 4.0 across readers
- These increases were statistically significant ($p<0.001$) for all three readers
- Orviglance also provided superior visualization compared to unenhanced images across pre-defined sub-groups for all three readers. These sub-groups include lesion type, age, sex, degree of renal impairment, and magnetic field strength

Secondary endpoints reinforce successful SPARKLE outcomes
The results of the secondary efficacy endpoints support the positive primary analysis and confirm the robustness of the positive results.

Key secondary efficacy analyses include e.g.,

- Detection of lesions: across all readers at least one additional lesion was detected in 40-52% of patients with Orviglance compared to unenhanced MRI across readers
- Detection of small lesions: The mean size of the smallest lesions was 2 mm smaller with Orviglance than with unenhanced MRI

The results for other secondary endpoints generally support the superiority of Orviglance to unenhanced MRI and no analysis favours unenhanced MRI. These endpoints include quantitative assessment of signal intensity in the images, recommended next step in treatment and reader confidence in detection and localization of lesions. Results for Orviglance were comparable regardless of whether unenhanced images were included in the evaluation.

No safety concerns with Orviglance
The primary safety analysis concluded that no serious adverse drug reactions were observed. Adverse drug reactions were classified as mild (86%) or moderate (14%) and of short duration. They were most commonly gastrointestinal related, such as nausea (15% of all patients), diarrhea (10% of all patients) and vomiting (5% of all patients). The safety results in this vulnerable patient population were consistent with previous studies.

The positive results of the primary and secondary endpoints for SPARKLE successfully conclude the clinical development for Orviglance.

“The positive and robust results for SPARKLE confirm our confidence in Orviglance and will be valuable in our discussions with regulators to advance Orviglance to approval”, says Andreas Norlin, CSO of Ascelia Pharma.

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

As per industry practice and FDA guidance:
* Orviglance-enhanced images consist of combined Orviglance-enhanced plus unenhanced images
** Only patients with detection of the same lesions in both unenhanced and Orviglance-enhanced images are included in the primary analysis

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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.

Attachments

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