PRESS RELEASE

Malmö, 19 November 2018

Ascelia Pharma receives positive feedback from FDA on design of the pivotal Phase 3 study with Mangoral®

Ascelia Pharma AB today announced that the company has successfully concluded discussions with the US Food and Drug Administration (FDA) regarding the study design for the single pivotal Phase 3 trial for Mangoral® as a contrast agent in patients with focal liver lesions and severe renal impairment. Ascelia Pharma will now complete the preparatory work to start the trial and expects to enroll the first patient during the second half of 2019.

Mangoral® is an oral contrast agent for liver MRI developed to facilitate the visualization of focal liver lesions, including liver metastases, using Magnetic Resonance Imaging (MRI) in adult patients where use of the current gold standard - gadolinium-based contrast agents - may be medically inadvisable or cannot be administered.

The Phase 3 trial will be a multicenter, open-label study with the overall objective to evaluate the safety and diagnostic efficacy of Mangoral® in patients with known or suspected focal liver lesions and severe renal impairment. The study will be conducted at premier hospitals globally.

The primary efficacy variable will be based on number of lesions visualized and semi-quantitative and semi-qualitative parameters. Comparison will be between combined MRI (Mangoral®-enhanced MRI plus unenhanced MRI) as compared to unenhanced MRI.

"We are very excited to move Mangoral® into the pivotal phase of development and initiate this global multi-center study," said Magnus Corfitzen, CEO of Ascelia Pharma.

"If the study is successful, it could enable approval of the only non-gadolinium liver MRI drug for patients who are at risk of serious side effects from the gadolinium-based contrast media available on the market today. The FDA has been very supportive and collaborative and has provided valuable feedback on the design of the Phase 3 study. We are confident that we have a robust trial design with the potential to repeat and confirm the excellent data observed in our Phase 2 studies, and support approval."

For further information, please contact:
Magnus Corfitzen, CEO, +46 735 179 110

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About Mangoral®

Mangoral® is an investigational new drug for diagnostic use as an MRI contrast agent for enhancement of the liver tissue. The drug candidate is being developed for facilitating the visualization of focal liver lesions, including liver metastases, in patients where use of gadolinium-based contrast agents may be medically inadvisable or cannot be administered.

Liver metastases are the most frequent type of malignant focal liver lesion. Metastasis to the liver often occurs in progressive cancer disease and is associated with substantially reduced survival. In fact, the liver is one of the most frequent – and often the first – site of metastasis. About 70% of all patients with colorectal cancer will develop liver metastases at some point in their lifetime, and one-third of these will have metastases confined only to the liver. Early detection and localization of liver metastases is critical for optimal patient management.

Mangoral® is the first contrast agent in the world to obtain Orphan Drug Designation by the FDA for use in liver MRI in patients where use of gadolinium-based contrast agents may be medically inadvisable, or where gadolinium-based contrast agents cannot be administered.

Mangoral® is orally administered and consists of manganese combined with absorption promoters to increase manganese absorption in the small intestine, a prerequisite for a high uptake of manganese into the liver tissue, which is the optimal condition for obtaining high imaging quality. Manganese is a natural trace element and after absorption from the gastrointestinal tract it is efficiently taken up by normal liver cells, known as hepatocytes. Due to the retention of manganese in the hepatocytes and its paramagnetic properties, the contrast agent clearly enhances the liver tissue in MR imaging whilst the liver metastases do not accumulate manganese. Therefore, the liver metastases will become clearly detectable against the enhanced liver tissue on the MR image. From the liver the manganese is excreted via the bile. The route of administration, uptake and excretion of Mangoral® means that only very small amounts of manganese reach the systemic blood circulation.

About Ascelia Pharma

Ascelia Pharma is an orphan oncology drug development company focused on novel drugs with an established mode of action to improve the life expectancy and quality of life for people living with cancer. The company is located at Medeon Science Park in Malmö, Sweden, in the middle of Medicon Valley, a leading European life science cluster. For more information, please visit www.ascelia.com