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Ascelia Pharma presents clinical development plan for Oncoral as a novel chemotherapy

Ascelia Pharma AB (publ) (ticker: ACE) today announced the clinical development plan for Oncoral. With Oncoral, Ascelia Pharma has the opportunity to develop a novel oral chemotherapy with the potential to offer both efficacy and safety benefits to cancer patients. The planned Phase 2 study, for the treatment of gastric cancer, is expected to start in H2 2021. Further details of the Oncoral development plan can be found on its dedicated website.

Development plan – Phase 2 in gastric cancer

With encouraging Phase 1 data, Ascelia Pharma has prepared the next steps of clinical development. The planned Phase 2 study, which is expected to commence in H2 2021, will address metastatic gastric cancer, which is a serious disease with large unmet medical need for novel safe and effective therapies. The clinical guidelines and clinical data support efficacy of the active substance in Oncoral – irinotecan – in patients with gastric cancer. In gastric cancer, there is also an opportunity to obtain Orphan Drug Designation in the US and the EU.

The Phase 2 study will be a randomized controlled multicenter study of Oncoral added to Standard of Care, compared to Standard of Care alone. The primary endpoint will be progression-free survival, which is standard for an oncology Phase 2 study. Secondary endpoints will include response rate, overall survival, pharmacokinetics, safety, and tolerability.

For subsequent development, there is potential for label expansion into other solid tumor indications where irinotecan has also proved efficacious.

The development of Oncoral is supported by a Scientific Advisory Board of leading oncologists, who all share the company's view that Oncoral, a daily tablet formulation of irinotecan, would be a valuable additional treatment option for cancer patients, especially in later disease stages.

Oncoral – based on the well-established chemotherapy irinotecan

The active substance in Oncoral is irinotecan. Irinotecan is a well-established chemotherapy with documented anti-cancer effects. It is approved for treating colorectal and pancreatic cancer as well as for gastric cancer in Japan.

Today, the administration of irinotecan is intravenous (IV) bolus infusions, typically as a high dose every third week. Oncoral is a novel patented tablet formulation of irinotecan for daily dosing at home with potential to offer better patient outcomes.

Oncoral – potential to improve both efficacy and safety

Intravenous chemotherapy is often a trade-off between desired treatment effect and tolerability for the patient. With Oncoral as a daily irinotecan tablet there is a potential to improve both efficacy and

tolerability compared to IV administration. In addition, it may offer convenience for the patient and at the same time reduce hospital costs with home administration.

Efficacy

The potential to improve efficacy is based on a fivefold higher conversion rate of irinotecan to the cytotoxic active metabolite SN-38 when dosed orally compared to an IV infusion. In addition, the principle of frequent, low daily dosing, also called metronomic dosing, may optimize the exposure of SN-38 and thus maximize the anti-tumor effect. Several non-clinical and clinical studies provide proof of concept for metronomic dosing, including improved patient outcomes. In the Phase 1 study with Oncoral, clinical benefits with stable disease were observed even in patients that previously received IV irinotecan.

Safety

Conventional IV bolus administration of irinotecan is associated with toxicity. Most patients experience gastrointestinal and hematological side effects, of which approximately 30% are severe or life-threatening (grade 3 or 4, ref: Camptosar® prescribing information).

Frequent low dosing, avoiding high peak plasma levels, may reduce toxicity and complications compared to high-dose IV infusions. Oral daily administration also brings the opportunity to adjust dosing quickly in case of acute toxicity. In the Phase 1 study, Oncoral was well tolerated and the hematological toxicities were only mild to moderate, grade 1 or grade 2.

Dedicated website for Oncoral

Additional information about Oncoral, including filmed presentations from Ascelia Pharma's CEO and Chief Medical Officer and a member in the Scientific Advisory Board, is found on the dedicated website for Oncoral: [Oncoral development: Phase 2 and onwards – ASCELIA](#)

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About Ascelia Pharma

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 – Mangoral 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 www.ascelia.com.

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.

About gastric cancer

Gastric cancer is a global public health problem. It is the third most common cause of cancer death with one million new cases every year. Most patients, particularly in Western countries, are diagnosed with the disease too advanced for curative surgery. For these patients, chemotherapy is the standard treatment, although low tolerability impacts clinical outcomes, and the median survival of patients is less than one year. Consequently, there is a great unmet medical need for novel safe and effective therapies.