Press Release
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MEDIVIR

Medivir obtains IND approval for fostrox - the first oral, liver-targeted treatment for advanced liver cancer

- *FDA clearance of Investigational New Drug (IND) application to evaluate fostrox (fostroxacitabine bralpamide) in combination with Lenvima® vs Lenvima alone in a randomized phase 2b study in second-line advanced liver cancer (hepatocellular carcinoma, HCC).*
- *Phase 1b/2a data has demonstrated that the combination of fostrox + Lenvima has shown a manageable safety profile and encouraging anti-tumor activity in second-line population, including a median time to progression (TTP) of 10.9 months* [1].
- *Medivir plans to recruit patients in at least 8 countries across USA, Europe and Asia, aiming for study read-out in 2027.*

Stockholm, Sweden — Medivir AB (Nasdaq Stockholm: MVIR), a pharmaceutical company focused on developing innovative treatments for cancer in areas of high unmet medical need, today announced the approval of the US Investigational New Drug application (IND) for evaluating fostrox + Lenvima vs Lenvima alone in a phase 2b study in 2nd line advanced HCC.

"Opening of the IND for fostrox in combination with Lenvima in a randomized phase 2 study, is a significant milestone in Medivir's mission to improve life for patients with advanced liver cancer. Our recently closed phase 1b/2a study has shown a manageable safety profile and promise of anti-tumor activity beyond current second-line alternatives in advanced HCC. Through this phase 2b study, we aim to make the fostrox + Lenvima combination the first, approved second line treatment option after immunotherapy." says Dr. Pia Baumann, CMO at Medivir.

The randomized phase 2b study will evaluate the combination of fostrox + Lenvima vs Lenvima alone in second-line advanced HCC patients who have previously been treated with an immunotherapy combination. The primary endpoint is Objective Response Rate with secondary endpoints including duration of response (DoR), progression-free survival (PFS), overall survival (OS), safety and Quality of Life. Principal Investigator of the study is Prof. Maria Reig, Director of the Barcelona Clinic Liver Cancer (BCLC) and the Liver Oncology Unit at the Hospital Clinic of Barcelona in Spain, and one of the most renowned global experts in the field of liver cancer.

"*There is a significant unmet medical need for patients suffering from liver cancer, especially in second-line advanced liver cancer, where there are no approved treatment options available after an immunotherapy combination*," said Prof. Maria Reig from BCLC, Hospital Clinic Barcelona. *"Preserved liver function is of vital importance in this patient population, why treatments not only need to be effective but also "liver" tolerable, meaning no negative impact on liver function. The data has shown that fostrox + Lenvima have encouraging results related to clinical outcome in patients with second-line advanced HCC without jeopardizing safety, including liver function. The planned phase 2b study, evaluating fostrox added to Lenvima in a randomized, controlled trial, is an important study, in our ongoing efforts to improve treatment options for patients in second-line*".

For additional information, please contact;
Magnus Christensen, CFO, Medivir AB
Telephone: +46 8 5468 3100
E-mail: magnus.christensen@medivir.com

About fostrox
Fostrox is a liver-targeted inhibitor of DNA replication that delivers the cell-killing compound selectively to the tumor while minimizing the harmful effect on normal cells. This is achieved by coupling an active chemotherapy (troxacitabine) with a prodrug tail. This design enables fostrox to be administered orally and travel directly to the liver where the active substance is released locally in the liver. With this unique mechanism, fostrox has the potential to become the first liver-targeted, orally administered drug that can help patients with various types of liver cancer. A phase 1b monotherapy study with fostrox has previously been conducted and a phase 1b/2a combination study in HCC was completed in November 2024, where it has shown encouraging anti-cancer efficacy with a good safety and tolerability profile [1].

About primary liver cancer
Primary liver cancer is the third leading cause of cancer-related deaths worldwide. Hepatocellular carcinoma (HCC) is the most common cancer that arises in the liver and it is the fastest growing cancer in the USA. Although existing therapies for advanced HCC can extend the lives of patients, treatment benefits are insufficient and death rates remain high. There are approximately 860,000 patients diagnosed with primary liver cancer per year globally and current five-year survival is less than 20 percent [2], [3], [4]. HCC is a heterogeneous disease with diverse etiologies, and lacks defining mutations observed in many other cancers. This has contributed to the lack of success of molecularly targeted agents in HCC. The limited overall benefit, taken together with the poor overall prognosis for patients with intermediate and advanced HCC, results in a large unmet medical need.

About Medivir
Medivir develops innovative drugs with a focus on cancer where the unmet medical needs are high. The drug candidates are directed toward indication areas where available therapies are limited or missing and there are great opportunities to offer significant improvements to patients. Medivir is focusing on the development of fostroxacitabine bralpamide (fostrox), a drug candidate designed to selectively treat cancer cells in the liver and to minimize side effects. Collaborations and partnerships are important parts of Medivir's business model, and the drug development is conducted either by Medivir or in partnership. Medivir's share (ticker: MVIR) is listed on Nasdaq Stockholm's Small Cap list. www.medivir.com.

1) Chon et al. ESMO 2024, Poster 986
2) Bray et al., CA Cancer J Clin. 2024;74:229-263
3) Rumgay et al.,European Journal of Cancer 2022 vol.161, 108-118.
*4) Yang, J.D., Hainaut, P., Gores, G.J. et al. A global view of hepatocellular carcinoma: trends, risk, prevention and management. Nat Rev Gastroenterol Hepatol **16**, 589–604 (2019).*