Press Release
28 November 2024 08:30:00 CET

MEDIVIR

Medivir announces finalisation of the phase 1b/2a study with fostrox + Lenvima in advanced liver cancer, remaining patients transferred to compassionate use

- *Study closed on November 26th, 2024, end-of-treatment data to be presented at an upcoming scientific congress.*
- *Patients have stayed on treatment longer than anticipated and the 3 remaining patients have each been on treatment for at least 15 months.*
- *The patient with the longest treatment time in the study shows sustained partial response after more than 27 months.*

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 that the phase 1b/2a study of fostroxacitabine bralpamide (fostrox) + Lenvima® in 2nd or 3rd line advanced liver cancer (HCC), closed on November 26th. The 3 remaining patients in the study have been transferred to compassionate use, allowing continued benefit from the treatment.

At the ESMO congress in Barcelona in September, the company presented positive, mature data showing an encouraging tolerability profile and improved outcomes beyond what can be expected from current alternatives in second-line advanced HCC. Patients in the study showed extended duration of benefit, resulting in longer than expected treatment time, as evidenced by a median Time to Progression of 10.9 months. This is substantially longer than previously seen in second-line HCC. With the study now closed, the company plans to present end-of-treatment data at an upcoming scientific congress.

- "*The objectives of the phase 1b/2a study, evaluating safety and preliminary efficacy of fostrox in combination with Lenvima, were met. Mature data presented at ESMO this year showed that the combination is safe and tolerable with encouraging anti-tumor activity. These results indicate improved outcomes beyond current second-line alternatives in advanced HCC and with a compassionate use program in place, the remaining patients can continue benefitting from the treatment. We can now close the study and focus all our efforts on the planned randomized phase 2b study of fostrox in combination with Lenvima as we strive to make the combination the first, approved treatment option in second-line advanced HCC"*, says Dr. Pia Baumann, CMO at Medivir.

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 been completed and a phase 1b/2a combination study in HCC is ongoing where it has shown encouraging anti-cancer efficacy with a good safety and tolerability profile (ref Chon et al., ESMO 2024, Poster 986).

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 660,000 patients diagnosed with primary liver cancer per year globally and current five-year survival is less than 20 percent [1], [2]. 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) Rumgay et al.,European Journal of Cancer 2022 vol.161, 108-118.
*2) 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).*