Press Release
20 February 2025 09:00:00 CET

MEDIVIR

Medivir presents positive final phase 1b/2a fostrox + Lenvima data in advanced liver cancer at EASL Liver Cancer Summit

- *Fostrox, in combination with Lenvima®, final data confirm improved outcomes in second-line advanced liver cancer with a median overall survival (OS) of 13.7 months (95% CI 7.6 – NR), median time to progression of 10.9 months (95% CI 4.2 – 18.1) and an objective response rate (ORR) of 24%* [1].
- *Tumor reduction was seen in >75% of patients and the median duration of clinical benefit was 11.3 months.*
- *With a median follow-up of 10.5 months, fostrox + Lenvima was shown to be safe and well tolerated.*
- *Fostrox (fostroxacitabine bralpamide) delivers the cell-killing effect selectively to tumor cells locally in the liver, while minimizing harm to healthy cells.*
- *With currently no approved treatments in second-line after immunotherapy, Medivir aims to be ready within short to initiate its planned, randomized phase 2b study comparing fostrox + Lenvima with Lenvima + placebo.*

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 presented positive, final data from the phase 1b / 2a study of fostroxacitabine bralpamide (fostrox) + Lenvima in advanced liver cancer (hepatocellular carcinoma/HCC) at the EASL (European Association for the Study of the Liver) Liver Cancer Summit in Paris, France.

Today's EASL update, poster number P02-13, presented by Dr Jeff Evans on Thursday February 20th, confirmed encouraging efficacy with a median overall survival of 13.7 months in second- or third line advanced liver cancer patients. All patients in the study had tumor progression on previous treatment but subsequently showed tumor reduction in >75% of the patients treated with fostrox + Lenvima. The tumor reduction correlated with reduction in AFP levels from baseline to 6 weeks that were seen in 79% of patients. This is encouraging as AFP is a liver cancer biomarker where high AFP levels negatively impact outcome. The result from this study showed that tumor reduction was seen independent of AFP levels (≥400 ng/mL<). The treatment with fostrox + Lenvima continued to be safe and tolerable with no unexpected new adverse events. The most common adverse events were temporary low neutrophil and platelet count with limited clinical impact, leading to fostrox dose adjustment (delay, reduction or discontinuation) at only 11 occasions throughout the study.

The results from this study are encouraging considering the poor trajectory seen in second-line liver cancer where current available therapies have shown just 5 – 10% ORR, a typical TTP of only 3 - 4 months and median OS of 8-11 months.

Dr. Pia Baumann, Chief Medical Officer at Medivir, said:
- *"With these final results at a median follow-up of 10.5 months, fostrox + Lenvima have clearly shown promise of improved outcomes beyond current alternatives for second-line liver cancer patients. Fostrox is designed to only target tumor cells locally in the liver, without harming healthy cells, making it safe and tolerable also for longer term treatment. It is therefore encouraging to see that the second- and third line patients in our study have experienced substantially longer duration of benefit than previously seen in this patient population, as evidenced by the median overall survival of 13.7 months and median time to progression of 10.9 months. It is also reassuring to see that the response to treatment was seen independent of the patient's AFP level at baseline. These final results have further strengthened our belief in fostrox as a potential treatment for patients with advanced liver cancer and we continue the work to initiate the planned, randomized phase 2b study comparing fostrox + Lenvima with Lenvima + placebo."*

Dr Jeff Evans, Professor of Translational Cancer Research at University of Glasgow, and Honorary Consultant at the Beatson West of Scotland Cancer Centre, and investigator in the fostrox + Lenvima study, commented:
- *"With no treatments approved in second-line after immunotherapy, there is a significant unmet medical need for new treatments options for these patients. The final results from the phase 1b/2a study indicate potential for the fostrox + Lenvima combination to become a valuable treatment alternative for second-line advanced liver cancer patients. It is especially encouraging that target lesion reduction was seen in more than 75% of patients and that the reduction in lesion size was persistent over time. In this difficult-to-treat population, achieving and maintaining disease control for an extended period of time, as seen in this study, is important for optimal outcome. I look forward to evaluating the efficacy of fostrox plus Lenvima in a randomized, controlled trial."*

The data presented at EASL are the final results from Medivir's recently closed phase 1b/2a open-label, multi-center, dose-escalation and dose-expansion study, evaluating the safety and efficacy of fostrox in combination with Lenvima in patients for whom current first- or second-line treatment has proven ineffective or is not tolerable.

There are approximately 660,000 patients diagnosed with HCC per year globally and the current five-year survival is less than 20 percent[3].

The poster will be available on Medivir's website after the presentation at 2 pm CET.

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 [2].

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 [3], [4, [5]. 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) Evans et al., EASL Liver Cancer Summit 2025, Poster P02-13
2) Chon et al., ESMO 2024, Poster 986
3) Bray et al., CA Cancer J Clin. 2024;74:229-263
4) Rumgay et al.,European Journal of Cancer 2022 vol.161, 108-118.
*5) 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).*