Press release

Cantargia AB
556791-6019
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Cantargia receives positive EMA opinion for orphan designation of nadunolimab for treatment of pancreatic cancer

Cantargia AB today announced that the Committee for Orphan Medicinal Products (COMP) at the European Medicines Agency (EMA) has issued a positive opinion for orphan designation of nadunolimab (CAN04) for treatment of pancreatic cancer. The European Commission is now expected to grant the orphan designation. This provides a range of incentives in the continued clinical development of CAN04 in pancreatic cancer.

The interleukin-1 receptor accessory protein (IL1RAP)-binding antibody CAN04 is Cantargia's lead program and is investigated in multiple clinical trials evaluating combination with chemotherapy regimens in various forms of cancer, including pancreatic ductal adenocarcinoma (PDAC), the most common form of pancreatic cancer.

The COMP at the EMA has issued a positive opinion for orphan designation of CAN04 for treatment of pancreatic cancer. The opinion is forwarded to the European Commission which is expected to grant the orphan designation within 30 days. The positive opinion is based on data from the ongoing CANFOUR trial, a phase I/IIa study where CAN04 is investigated in patients with advanced PDAC as first line combination with gemcitabine and nab-paclitaxel. CAN04 was granted US Orphan Drug Designation for treatment of pancreatic cancer by the Food and Drug Administration (FDA) in September 2021.

The EMA orphan designation is designed to encourage the development of new treatments for life-threatening or chronically debilitating conditions that are rare and affect not more than five in 10,000 people in the EU. For a therapy to qualify for orphan designation in the EU, one of several requirements is that, unless no satisfactory method of treatment of the condition concerned has been authorized, the therapy must be of significant benefit to those affected by the condition. Therapies that meet the EMA's orphan designation criteria qualify for several incentives, including 10 years of market exclusivity, protocol assistance, and potentially reduced fees for regulatory activities. More information is available at www.ema.europa.eu/en/human-regulatory/research-development/orphan-designation-research-development.

CAN04 is currently evaluated in two ongoing clinical trials for treatment of PDAC. In parallel with the CANFOUR study, CAN04 is also investigated in combination with FOLFIRINOX as first line therapy in patients with metastatic PDAC in the phase Ib trial CAPAFOUR. Interim efficacy data from 33 PDAC patients in CANFOUR show that the CAN04 combination therapy results in durable responses or pseudoprogression, leading to both prolonged progression-free survival and median overall survival compared to historical control data. Preparations are ongoing for late-stage development in PDAC, to be initiated during 2022.

"*Orphan designation provides a large number of opportunities for nadunolimab. We intend to take advantage of those in our strong commitment to advance the development towards new treatment alternatives in pancreatic cancer.*", said Göran Forsberg, CEO of Cantargia.

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This is information that Cantargia AB is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact person set out above, at 08.30 CET on 14 October 2021.

About Cantargia
Cantargia AB (publ), reg. no. 556791-6019, is a biotechnology company that develops antibody-based treatments for life-threatening diseases and has established a platform based on the protein IL1RAP, involved in a number of cancer forms and inflammatory diseases. The main project, the antibody nadunolimab (CAN04), is being studied clinically in combination with chemotherapy or immune therapy with a primary focus on non-small cell lung cancer and pancreatic cancer. Positive interim data from the combination with chemotherapy indicate stronger efficacy than would be expected from chemotherapy alone. Cantargia's second project, the antibody CAN10, addresses treatment of serious autoimmune/inflammatory diseases, with initial focus on systemic sclerosis and myocarditis.

Cantargia is listed on Nasdaq Stockholm (ticker: CANTA). More information about Cantargia is available at www.cantargia.com.

About nadunolimab (CAN04)

The antibody CAN04 binds strongly to its target IL1RAP and functions by inducing ADCC and blocking IL-1α and IL-1β signaling. Thereby, CAN04 can counteract the contribution of the IL-1 system to the immune suppressive tumor microenvironment and development of resistance to chemotherapy. CAN04 is investigated in multiple ongoing clinical trials. In the phase I/IIa-study CANFOUR, first line combination therapy is investigated with standard chemotherapies in patients with PDAC (gemcitabine/nab-paclitaxel) and patients with NSCLC (cisplatin/gemcitabine) (NCT03267316). Positive interim data for the combination therapies show durable responses or pseudoprogression in patients with PDAC, resulting in median iPFS of 7.8 months and median survival of 12.6 months. Stronger efficacy was also observed in NSCLC patients with median PFS of 7.2 months. A response rate of 53% was observed in non-squamous NSCLC patients, with even higher responses in patients previously treated with pembrolizumab. These results show stronger efficacy than expected from chemotherapy alone. CAN04 is investigated with chemotherapy also in the phase I study CAPAFOUR, with the FOLFIRINOX regimen for first line treatment of metastatic PDAC (NCT04990037), and in two further clinical studies, CESTAFOUR and TRIFOUR, in additional forms of cancer, including biliary tact cancer, colorectal cancer and triple negative breast cancer. CAN04 is also evaluated with the immune checkpoint inhibitor pembrolizumab, with or without chemotherapy, in the phase I study CIRIFOUR (NCT04452214).