InDex Pharmaceuticals announces successful interactions with the PMDA for the clinical development of cobitolimod in Japan

16 August, 2022 – InDex Pharmaceuticals Holding AB (publ) today announced that the company has received positive feedback from the Japanese regulatory authority, the Pharmaceuticals and Medical Devices Agency (PMDA), regarding the clinical development plan for a future marketing authorization application for the company's TLR9 agonist cobitolimod, for the treatment of moderate to severe left-sided ulcerative colitis. The PMDA has accepted enrolment of Japanese ulcerative colitis patients in the second global phase III induction study, without performing specific Japanese studies prior to study start.

The phase III program for cobitolimod in moderate to severe left-sided ulcerative colitis consists of two sequential global induction studies and a year long maintenance study, with patients that have responded to cobitolimod as induction therapy. The first phase III study CONCLUDE is ongoing and results are expected during H2 2023. The purpose of the consultation meeting with the PMDA was to obtain input on cobitolimod's development plan for Japan, with the aim to include Japanese patients in the second induction study, which is planned to start upon a positive read-out of the first induction study. According to the PMDA feedback, no additional studies are required prior to including Japanese patients in the global phase III study. In addition, the PMDA stated overall acceptance for the non-clinical package as well as the study design of the phase III program. Pharmacokinetic data for cobitolimod in Japanese patients will have to be collected prior to applying for market approval, but can be conducted during the remaining phase III program.

"We are very pleased that the PMDA supports our development plan for cobitolimod in Japan, and that they agreed to include Japanese patients in the second phase III induction study with cobitolimod, without conducting specific studies in Japanese subjects," said Johan Giléus, acting CEO of InDex Pharmaceuticals. "This is a unique decision by PMDA, indicating great potential for cobitolimod and a need for new treatment options that can help more patients with moderate to severe ulcerative colitis return to a normal life. In addition, the positive feedback from PMDA is advantageous for discussions with potential candidates for strategic collaborations in Japan."

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The information was submitted for publication through the agency of the contact person set out above at 10:30 CET on 16 August 2022.

About ulcerative colitis
Ulcerative colitis is a chronic disease caused by inflammation of the large intestine. Today approximately 2 million people in Europe and the US suffer from ulcerative colitis. The symptoms are characterised by blood- and mucus-mixed diarrhea, frequent stools, abdominal pain, fever, weight loss and anemia. Moreover, patients have a significantly elevated risk of developing colon cancer. Despite the currently available drugs, many patients with ulcerative colitis still suffer from severe symptoms.

About the CONCLUDE study
CONCLUDE is a global randomised, double-blind, placebo-controlled, clinical phase III study to evaluate cobitolimod as a novel treatment for patients with moderate to severe left-sided ulcerative colitis with an inadequate response or failure to tolerate conventional therapy, biological therapy or JAK inhibitors. The study has been designed in consultation with both the US and European regulatory authorities, the FDA and EMA respectively. The induction study will include approximately 440 patients, and the primary endpoint will be clinical remission at week 6, which is the same primary endpoint as used in the successful phase IIb study CONDUCT. Apart from the dosing 250 mg given at baseline and week 3, which was the highest dose and the one that showed the best efficacy in the phase IIb study, the phase III study also evaluates a higher dose, 500 mg, in an adaptive study design.

Upon a positive read-out of the first induction study, InDex plans to initiate a second induction study with the best dose. Patients responding to cobitolimod in the induction studies will be eligible to continue in a one-year maintenance study, where they will be treated with either cobitolimod or placebo once every three weeks.

Cobitolimod in brief
Cobitolimod is a first-in-class Toll-like receptor 9 (TLR9) agonist that can provide an anti-inflammatory effect locally in the large intestine, which may induce mucosal healing and relief of the clinical symptoms in ulcerative colitis. Cobitolimod met the primary endpoint in the phase IIb study CONDUCT and demonstrated an outstanding combination of efficacy and safety. The results have been published in the reputable medical journal, The Lancet Gastroenterology & Hepatology. Data from four previous completed placebo-controlled clinical trials support the efficacy and safety demonstrated in the CONDUCT study.

InDex Pharmaceuticals in brief
InDex is a pharmaceutical development company focusing on immunological diseases where there is a high unmet medical need for new treatment options. The company's lead asset is the drug candidate cobitolimod, which is being evaluated in the phase III study CONCLUDE as a novel treatment of moderate to severe ulcerative colitis – a debilitating, chronic inflammation of the large intestine. InDex has also developed a platform of patent protected discovery stage substances, so called DNA based ImmunoModulatory Sequences (DIMS), with the potential to be used in the treatment of various immunological diseases.

InDex is based in Stockholm, Sweden. The company's shares (ticker INDEX) are traded on Nasdaq First North Growth Market Stockholm. Redeye AB is the company's Certified Adviser. For more information, please visit www.indexpharma.com.