InDex Pharmaceuticals develops oral formulation of cobitolimod

May 4, 2018 – InDex Pharmaceuticals Holding AB (publ) today announced that it has developed a novel formulation of its lead drug candidate cobitolimod for oral administration, with targeted delivery to the lower part of the gastrointestinal tract. The oral formulation of cobitolimod is a potential follow-on product to the topical formulation, which is currently being investigated in the CONDUCT study - a phase IIb dose optimisation study in moderate to severe active ulcerative colitis.

Topical application of cobitolimod can provide rapid onset of action, while minimizing the risk of systemic side effects, when administered rectally to the site of inflammation in left-sided ulcerative colitis. However, oral therapy could enable delivery of cobitolimod to parts of the gastrointestinal tract which are inaccessible to the topical delivery system.

The developed oral product consists of a core matrix in a capsule with a pH sensitive coating, designed to initiate release of cobitolimod in the terminal ileum for controlled delivery to the colon. This oral formulation development provides opportunities for securing additional intellectual property. The GMP ready capsule has demonstrated stability with a documented high probability of extended release in the colon. The *in vitro* release profile is similar to those of marketed oral mesalazine products, with controlled release technologies. Additionally, the release profile can be adjusted to target other parts of the gastrointestinal tract, both by modifying the composition of the core matrix and the coating of the capsule.

"We have seen competitive efficacy and an excellent safety profile with the current formulation of cobitolimod. Patient and physician market research supports its future acceptance and commercial potential in both the US and Europe," said Peter Zerhouni, CEO of InDex Pharmaceuticals. "Nevertheless, an oral version would provide added patient convenience and broaden the potential therapeutic use of cobitolimod to extensive colitis and even Crohn's disease, thus significantly increasing the asset's value."

InDex's life cycle management strategy for cobitolimod is to launch first the topical formulation, for which top line results from the CONDUCT study are expected in the fourth quarter of 2018. The oral formulation will be a follow-on product and the next stage of its development is contingent on the results of CONDUCT.

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Cobitolimod in brief
Cobitolimod is a new type of drug that can help patients with moderate to severe ulcerative colitis back to a normal life. It is a so-called 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 active ulcerative colitis. Cobitolimod has achieved clinical proof-of-concept in moderate to severe active ulcerative colitis, with a very favorable safety profile. Data from four placebo-controlled clinical trials indicate that cobitolimod has statistically significant effects on those endpoints that are most relevant in this disease, both from a regulatory and clinical perspective. These endpoints include the key clinical symptoms such as blood in stool, number of stools, and mucosal healing, respectively. Based on the encouraging results from earlier studies InDex is now performing the phase IIb study CONDUCT to evaluate higher doses and dose frequencies than investigated in previous studies with cobitolimod. The goal of the study is to optimise the treatment and achieve substantially higher efficacy, while maintaining the

compound's excellent safety profile. The CONDUCT study will include 215 patients with left-sided moderate to severe active ulcerative colitis at 90 sites in 12 countries. It is a randomised, double blind, placebo-controlled study for evaluating cobitolimod's efficacy and safety in inducing clinical remission compared to placebo. The dose optimisation study investigates three different dose strengths of cobitolimod and two different dose frequencies. The objective is to have top line results from the study in the fourth quarter of 2018. Cobitolimod is also known as Kappaproct® and DIMS0150.

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 foremost asset is the drug candidate cobitolimod, which is in late stage clinical development for the treatment of moderate to severe active 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 treatment of various immunological diseases.

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

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