InDex Pharmaceuticals' in-depth analysis of the CONDUCT study confirms the successful top line results and supports the strategy going forward

February 19, 2020 – InDex Pharmaceuticals Holding AB (publ) today announced the conclusions from in-depth analysis of the complete data set from the phase IIb dose optimisation study CONDUCT, evaluating the first-in-class TLR9 agonist cobitolimod for the treatment of moderate to severe ulcerative colitis. The analysis confirms that the highest dose tested, which met the primary endpoint of the study, demonstrates an outstanding combination of efficacy and safety.

In-depth analysis of the complete data set has now been concluded and hence the Clinical Study Report can be finalised. As previously reported, the CONDUCT study met the primary endpoint with 21.4% of the patients in clinical remission at week 6 in the group receiving 250mg x 2 of cobitolimod, compared to 6.8% of the patients in the placebo group (p=0.0247*). The results in secondary endpoints, ranging across clinical measures like patient reported symptoms and endoscopic evaluation as well as biochemical and quality of life measures, show clear supportive evidence for the efficacy of this dose. Several secondary endpoints demonstrated statistically significant efficacy of the 250mg x 2 dose compared to placebo (meeting the predefined exploratory type I error rate of p<0.10). The analysis of the complete data set also confirms the excellent safety profile seen across all dose groups.

"A significant number of patients with moderate to severe ulcerative colitis do not respond to or cannot tolerate available medical therapies, resulting in a high unmet medical need. With the convincing results seen in patients with left-sided ulcerative colitis in the CONDUCT study, together with the novel and unique mechanism of action, I believe that cobitolimod has great potential as a future treatment alternative," said Professor Walter Reinisch from the Medical University of Vienna and Medical Advisor in the CONDUCT study. "I look forward to the further development of cobitolimod."

"Following in-depth analysis of the complete data set with the help of key opinion leaders, we can conclude that the CONDUCT study fulfilled its objectives in both the primary and a number of clinically relevant secondary endpoints. The robustness and consistency of the CONDUCT results support InDex's strategy to move cobitolimod forward in development and our phase III preparations are continuing according to plan," said Peter Zerhouni, CEO of InDex Pharmaceuticals. "Once ongoing regulatory discussions have been completed, we will be able to finalise the phase III design. Our plan is to be ready to enrol patients in the second half of this year. With its outstanding combination of efficacy and safety, we see great potential for cobitolimod to take a significant share of the large and growing market for moderate to severe ulcerative colitis."

A scientific manuscript with the complete study results is under preparation and will be submitted to a medical journal for publication. These data are also planned to be presented at upcoming international medical conferences.

**Predefined one-sided test where the significance limit was set to <0.10. Two-sided test gives p=0.0495.*

Conference call for investors, analysts and media
A conference call on the company's year-end report for 2019 and the conclusions from the CONDUCT study will be held with CEO Peter Zerhouni on Thursday, February 20, 2020 at 11:00 (CET). During the conference call, which will be held in Swedish, it will be possible to ask questions to the company.

The conference call can be followed at https://tv.streamfabriken.com/index-pharmaceuticals-q4-2019

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About the CONDUCT study
CONDUCT was a randomised, double blind, placebo-controlled, exploratory study for evaluating cobitolimod's efficacy and safety in inducing clinical remission, compared to placebo, in patients with left-sided moderate to severe active ulcerative colitis. The study objective was to identify the most efficacious dose and dose regimen for further development. The 213 patients were divided into four treatment arms receiving cobitolimod and one arm receiving placebo. Three different dose strengths of cobitolimod were investigated: 30mg, 125mg and 250mg given twice, at baseline and at week 3. Also, 125mg given four times, at baseline and each week until week 3, was investigated. In addition to cobitolimod or placebo, all patients continued with their standard of care treatment.

The primary endpoint of the study was induction of clinical remission at week 6 defined by modified Mayo sub scores, with a rectal bleeding score of 0, a stool frequency score of 0 or 1 and an endoscopy score of 0 or 1. The study was conducted at 91 sites in 12 different European countries: the Czech Republic, France, Germany, Hungary, Italy, Poland, Romania, Russia, Serbia, Spain, Sweden and the Ukraine respectively. For more details on the study please visit www.clinicaltrials.gov/show/NCT03178669.

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. Biological drugs represent the largest market segment in ulcerative colitis in terms of value with annual sales estimated to more than USD 5 billion. The total pharmaceutical market for ulcerative colitis is expected to grow to about USD 8 billion in 2023.

Cobitolimod in brief
Cobitolimod is a 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 has previously achieved clinical proof-of-concept in moderate to severe active ulcerative colitis, with a very favourable safety profile. Data from four previous 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. 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 lead asset is the drug candidate cobitolimod, which is in late stage clinical development for the 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 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 with e-mail certifiedadviser@redeye.se and phone number +46 8 121 576 90 is the company's Certified Adviser. For more information, please visit www.indexpharma.com

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