PRESS RELEASE
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The first in vivo study – a new milestone in CombiGene lipodystrophy project CGT2

CombiGene's lipodystrophy project CGT2 is in an exciting phase where different drug candidates are evaluated with the goal of selecting the final candidate for further preclinical studies in 2021. A first selection of the drug candidates has been made in in vitro trials with liver cells and CombiGene is now starting the next stage of the selection process with in vivo studies to evaluate which protein expression can be achieved in experimental models.

The first of two in vivo studies now being conducted aims to measure the level of protein expression from the different drug candidates and in which organs it is expressed. The second in vivo study is performed to measure the different candidates' effect on fatty liver disease, which is the condition that the CGT2 project primarily intends to treat in partial lipodystrophy.

"The fact that we are now initiating in vivo studies to identify our final drug candidate is a very important step in the preclinical development. The aim of the studies is to identify one or two drug candidates with high potential in order to initiate proof-of-concept studies during the first half of 2021," says Annika Ericsson Senior Project Manager at CombiGene.

About the lipodystrophyproject CGT2

The CGT2 project aims to develop a gene therapeutic treatment for partial lipodystrophy. The project was inlicensed from Lipigon Pharmaceuticals AB 2019 and is in early preclinical development with a focus on design and testing of gene therapy vectors.

Lipodystrophy is a rare disorder that is characterized by abnormal distribution of fat in the body. Patients suffer from lipoatrophy, which means that body fat is lost. In the absence of normal body fat different organs begin to accumulate fat, which subsequently leads to serious metabolic complications, among them, extreme insulin resistance, hypertriglyceridemia (elevated levels of the blood fat triglyceride) and hepatic steatosis (fatty liver disease).

There are currently a few treatments that can alleviate the symptoms of lipodystrophy, but no form of therapy that is targeted directly at the fundamental cause of the disorder. For patients suffering from partial lipodystrophy there are currently no treatments whatsoever.

About CombiGene AB

CombiGene's vision is to offer patients affected by severe life-changing diseases opportunities for a better life through innovative gene therapies. CombiGene's business concept is to develop effective gene therapies for serious diseases that today lack adequate treatment methods. Research assets are taken in from a network of external researchers and developed further up to clinical concept verification. Drug candidates for common diseases will be co-developed and commercialized through strategic partnerships, while CombiGene may drive the development and commercialization in-house for medicines aimed at limited patient populations. The company is public and listed on the Nasdaq First North Growth Market and the company's Certified Advisor is FNCA Sweden AB, +46 (0)852 80 03 99, info@fnca.se.

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