Press release
March 18, 2021

CG01 project achieves important milestone

Initiating preclinical biodistribution and toxicology studies

CombiGene's epilepsy project CG01 is now advancing to the final phase of the preclinical program. Following the release of the material from the first large-scale production of CG01 on March 1, CombiGene is now initiating the important preclinical studies in biodistribution and toxicology, two cornerstones of the final preclinical phase, and is planning for the start of the first study in humans in 2022.

CombiGene is now initiating the important preclinical studies in biodistribution and toxicology (safety) together with *Northern Biomedical Research* (NBR). NBR is a contract research organization (CRO) that specializes in preclinical studies in the central nervous system (CNS) and has extensive experience in administering gene therapy vectors. The company is GLP compliant and routinely supports companies submitting data to the regulatory agencies. The studies will be carried out using the material produced by CombiGene's CDMO partner Viralgen. This material has been produced in accordance with the process that at a later stage will be used for GMP manufacturing.

In preparation for the biodistribution and toxicology studies, NBR has already conducted pilot studies with CG01 to ensure that it reaches the intended area of the brain and that the active substances encoded in CG01, NPY and Y2, are expressed.

The pilot studies have also generated material for *StageBio* and *Accelero*, the two companies that have developed the methods that will be used in the evaluation of the preclinical studies. *StageBio* will analyze the expression of NPY and Y2 in the brain as well as conduct so-called histopathology, i.e. see if there are any lesions in connection with CG01 treatment. Accelero will conduct biochemical analyses including analyses of antibodies against CG01, if developed.

The preclinical studies in biodistribution and toxicology are a very important step towards the first human study in the CG01 project, which is scheduled to start in 2022.

About CG01

Unlike many gene therapies, which are developed for the treatment of rare diseases, CG01 caters to a large population of patients. Epilepsy is a major global problem. Every year, approximately 47,000 drug-resistant patients with focal epilepsy are estimated to be added in the US, EU4, UK, Japan and China. CombiGene believes that it is realistic that 10-20% of these patients could be treated with the drug candidate CG01. Assuming, for example, that the therapy cost per patient is somewhere between $134,000 and $200,000 (which compared to approved gene therapy drugs is low), it provides sales between $750-$1,500 million annually.

Horizon 2020

CombiGene's lead project CG01 has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 823282

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.

About Northern Biomedical Research

Northern Biomedical Research has collaborated with customers in research, pharmaceutical industry and biotech for more than 25 years. The company specializes in pharmacology, toxicology, biodistribution and pharmacokinetics (the doctrine of drug turnover in the body). The company is based in Spring Lake, Michigan, USA.

About StageBio

StageBio is a leading provider of GLP-compliant necropsy, histology, pathology, and specimen archiving services for the biopharmaceutical, medical device, academic, and contract research industries. The company operates six GLP laboratories as well as two GLP specimen archiving facilities in the U.S., with substantial continued investment in facility and technology infrastructure to meet the growing demand for high-quality histopathology services globally. StageBio has a team of 25+ board-certified veterinary pathologists and more than 50 laboratory technicians on staff supporting a unified commitment to quality, scientific integrity and client satisfaction.

About Accelero

Accelero Bioanalytics is a GLP certified laboratory specialized in delivering bioanalytical services to the drug development industry since 2011. The company is located in Berlin, Germany.

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