PRESS RELEASE
February 8, 2022

CombiGene's and Neurochase's preclinical study provides valuable information for the upcoming long-term studies in toxicology and biodistribution

STOCKHOLM — On June 28, 2021, CombiGene ("CombiGene", "the Company") announced that the Company, together with its partner Neurochase, had started the work to optimize the administration of CG01 in a preclinical pilot study in a large animal model. CombiGene can today announce that the practical part of the study has now been completed.

In the study, the administration of CG01 has been evaluated in terms of, among other things, injection volume and injection rate. The study provides valuable information of the Neurochase injection device for the upcoming long-term studies in toxicology and biodistribution in large animals.

"CombiGene continues to break new ground within the framework of the CG01 project," said Pernilla Fagergren, Project Manager at CombiGene. "No one has previously developed a gene therapy for the treatment of drug-resistant focal epilepsy. We have now gained new knowledge that is valuable for the upcoming long-term studies in toxicology and biodistribution in large animals."

Since CombiGene and Spark Therapeutics in October 2021 entered an exclusive collaboration and licensing agreement for CG01, the two companies have jointly reviewed the future development of the project with the ambition to establish the best path forward.

The most significant outcome of this review is the decision to expand the clinical development program to include clinics in the U.S. as well as in Europe. The U.S. is the world's largest pharmaceutical market and to establish a clinical presence there adds much further strength to the CG01 project.

In order to prepare CG01 to meet the needs of an expanded submission that also includes the U.S., the remaining preclinical program will be expanded and, in some parts, complemented with additional studies. In practice, this means that the preclinical part of CG01 will take longer to finalize.

During the remaining part of the preclinical program, all CG01-related R&D activities that CombiGene is running, internal as well as external, will be agreed upon and approved by Spark, who also assumes all agreed costs. As CG01 enters the clinical phase, Spark will take over the responsibility for the continuation of the project and thus also bear all costs during this development phase.

About CG01

CG01 is a unique gene therapy candidate aimed at a large patient population to solve a global need in epilepsy treatment. Epilepsy is a major global medical problem with approximately 47,000 drug-resistant patients with focal epilepsy estimated to be added each year across the US, EU4, UK, Japan, and China. CG01 is in a late preclinical stage. The production platform, jointly developed by CombiGene and its partners Cobra Biologics and Viralgen, is scalable and designed to provide material for preclinical and clinical, and future commercial production. CombiGene has signed an exclusive collaboration and licensing agreement for CombiGene's CG01 project with Spark Therapeutics.

Horizon 2020

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

CombiGene AB (publ), Agavägen 52A, SE-18155 Lidingö, Sweden info@combigene.com
www.combigene.com

About Spark Therapeutics
At Spark Therapeutics, a fully integrated, commercial company committed to discovering, developing and delivering gene therapies, we challenge the inevitability of genetic diseases, including blindness, hemophilia, lysosomal storage disorders and neurodegenerative diseases. We currently have four programs in clinical trials. At Spark, a member of the Roche Group, we see the path to a world where no life is limited by genetic disease. For more information, visit www.sparktx.com, and follow us on Twitter and LinkedIn.

About Neurochase
www.neurochase.com
Neurochase, founded by Professor Steven Gill, aims to bring transformative targeted therapies to patients with neurological diseases using state of the art technology. The company's team specializes in creating bespoke therapeutic strategies for the direct delivery of therapies using Convection Enhanced Delivery (CED). Neurochase provides accurate, targeted and safe direct drug delivery to the CNS and develop globally scalable treatment strategies and solutions for the pharmaceutical and biotech industry. Neurochase Ltd. is a Registered Company Number 12428919.

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 has signed an exclusive collaboration and licensing agreement for CombiGene's CG01 project with Spark Therapeutics.

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.

For more information:
CombiGene AB (publ)
Jan Nilsson, CEO
Tel: +46 (0)704 66 31 63
jan.nilsson@combigene.com

Bert Junno, Chairman of the Board
Tel: +46(0) 70 7 77 22 09
bert.junno@combigene.com

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