PRESS RELEASE
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CombiGene and Spark Therapeutics plan to expand the clinical development program to include the U.S. as well as Europe

STOCKHOLM, 16 December 2021 — The clinical development program for CombiGene's CG01 project, which is being developed to treat drug-resistant focal epilepsy, was originally planned to be performed in Europe, CombiGenes home market. Since CombiGene and Spark Therapeutics in October this year 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 a global submission 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.

"I am extremely pleased to expand CG01's clinical development program to include the U.S. With this decision, the project will find a natural foothold on the world's largest pharmaceutical market, at the same time as Spark can utilize the company's impressive resources, know-how and networks in an optimal way," said Jan Nilsson, CombiGene's CEO. "CombiGene and Spark will now jointly run the remaining part of the preclinical program before Spark, as per our agreement, takes over the full responsibility for the clinical development program and future global commercialization."

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, and 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 trials to full commercial production.

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.

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 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.

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