

PRESS RELEASE

October 12, 2021

CombiGene and Spark Therapeutics enter exclusive, global licensing agreement for gene therapy candidate CG01

STOCKHOLM, October 12, 2021 — CombiGene AB ('CombiGene' or the 'Company') and Spark Therapeutics, a member of the Roche Group (SIX: RO, ROG; OTCQX: RHHBY) and a fully integrated, commercial gene therapy company dedicated to challenging the inevitability of genetic disease, today announced the signing of an exclusive collaboration and licensing agreement for CombiGene's CG01 project, a gene therapy which aims to treat drug resistant focal epilepsy.

The agreement provides Spark with the exclusive world-wide license to develop, manufacture and commercialize CG01. CombiGene will continue to execute certain aspects of the preclinical program in collaboration with Spark. Under the terms of agreement, CombiGene is eligible to receive up to \$328.5 million excluding royalties, with \$8.5 million upon signing, up to \$50 million at preclinical and clinical milestones. CombiGene will also be reimbursed for certain authorized R&D expenses. Upon commercialization, CombiGene is eligible for tiered royalties ranging from the mid-single digits up to low double-digits based on net sales.

"This is a momentous day for CombiGene. Together with our partners and with the support of our shareholders and with an important contribution from the EU program Horizon 2020, we have worked long and hard to arrive at this deal with Spark, and I could not be more proud," stated Jan Nilsson, chief executive officer, CombiGene. "Our epilepsy project CG01 has continued to show strength throughout the preclinical phase, and in Spark we have now found a perfect partner to take CG01 through the clinical phase to full commercialization. CombiGene and Spark have had a productive ongoing dialogue during the latter parts of CG01's preclinical development, and the entire CombiGene team have come to know Spark as a visionary and patient-focused organization with the strength, know-how, and experience to exploit the full potential of CG01. We look forward to advancing this potentially transformative therapy together with Spark for the benefit of a patient group in need of better treatments."

"Spark is constantly evaluating new opportunities to challenge the status quo when it comes to the treatment of diseases with unmet needs, and collaboration is a critical part of our strategy," said Joseph La Barge, chief business officer, Spark Therapeutics. "We were impressed by CombiGene's gene therapy platform and look forward to harnessing our collective expertise to tackle diseases that affect signaling in the central nervous system."

"While many people with epilepsy respond well to current anti-seizure therapies, a significant portion are in need of new treatment options," said Federico Mingozzi, Ph.D., chief scientific officer, Spark Therapeutics. "Through our new collaboration and licensing agreement with CombiGene, we will work together leveraging our vast knowledge and experience in gene therapy to address unmet needs for people living with drug-resistant focal epilepsy."

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 in 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, satisfies all quality requirements and is scalable to meet all future needs, from limited volumes for preclinical and clinical trials to full commercial production.

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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This information is information that CombiGene is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact persons set out above, at October 12, 07:00

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