PRESS RELEASE
January 17, 2022

GMP production of CG01 made available for preclinical studies planned to enable First in Human study

STOCKHOLM — CombiGene AB ('CombiGene') and Spark Therapeutics ('Spark'), 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, together with their joint CDMO partner Viralgen – a leading AAV gene therapy manufacturer – today announced that the GMP production of CG01 has been made available for the concluding preclinical studies planned to enable First In Human study.

This is the first large scale GMP production of CG01, and both the production itself and the subsequent testing were performed according to original plans. The data from the analyses will form a central part of future regulatory applications to support proceeding to clinical studies.

"The availability of the GMP production of CG01 is yet another important and value-creating milestone for CombiGene. In collaboration with Spark, we are now performing the final parts of the preclinical program in which CombiGene will be reimbursed for agreed upon R&D expenses. Once this program is finalized, Spark will assume full control over the project's clinical program and future global commercialization. It is with a sense of great satisfaction that we have finalized the GMP production," said Jan Nilsson, CEO of CombiGene.

Viralgen's CEO, Javier García, said "Viralgen is excited to play an important role in the continued advancement of CG01. This material's successful and on-time testing is a key milestone in reaching patients and supporting both CombiGene and Spark in making a difference in epilepsy. We're pleased our manufacturing platform was able to effectively scale and produce this construct as expected."

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 Viralgen
https://viralgenvc.com
Viralgen is a CDMO born as a joint venture between AskBio and Columbus Venture Partners, combining decades of technology and drug development experience in multiple platforms to support best-in-class service offerings to the gene therapy market.

Viralgen was created in 2017 to respond to the unmet need for manufacturing of gene therapies, with the goal to help broaden access to these life-saving therapeutics and to contribute to the advancement of health and human welfare around the world. The company specializes in the production of rAAV viral vectors, and has built an optimized facility in San Sebastian, Spain that maximizes throughput and efficiency of our proprietary Pro10™ suspension manufacturing platform, enabling industry-leading scalability, reproducibility, and speed to market.

Through a superior technology platform, the company deliver industry-leading titers and cGMP-certified quality for all AAV serotypes to our client partners, optimize the cost-of-goods, and accelerate clinical development and commercialization of life-saving genetic medicines.

Viralgen new commercial facility was taken into production by the end of 2021 in San Sebastian (Spain).

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