PRESS RELEASE
Lund, Mars 1 2021

The material from the first large-scale production of CG01 is now released for use in the final parts of the preclinical program

In the autumn of 2020, CombiGene, together with the Spanish gene therapy manufacturer Viralgen, successfully completed the first large-scale production of the gene therapeutic drug candidate CG01, which is developed for the treatment of drug-resistant focal epilepsy. The produced CG01 material has since undergone a regular quality assurance with a large number of different analyses to ensure that the material meets all the requirements necessary for it to be used in the final parts of the preclinical program. All analyses have now been completed and the produced material has been released at Viralgen and is ready to be used in the important biodistribution and safety studies that will be conducted, as planned, by CombiGene's CRO partners, the US based NBR and the UK based Neurochase.

Clinical studies 2022

Once the final preclinical studies have been conducted and analysed with documented positive results, CombiGene will be ready to apply for permission to start the first study in humans, a so-called clinical study. CombiGene is currently working with interested clinics and clinicians to design the protocol of the first clinical study.

The CG01 project has high commercial potential

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.

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

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About CombiGene AB

www.combigene.com

CombiGene's vision is to provide patients affected by severe life-altering diseases with the prospect of a better life through novel gene therapies.

CombiGene's business concept is to develop effective gene therapies for severe life-altering diseases where adequate treatment is currently lacking. Development assets are sourced from an external research network and developed to achieve clinical proof of concept.

Drug candidates for common diseases will be co-developed and commercialized through strategic partnerships, while the company may manage this process on its own for drugs targeting niched patient populations.

The company is public and listed on the Swedish marketplace 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 Viralgen

www.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 the proprietary Pro10™ suspension manufacturing platform, enabling industry-leading scalability, reproducibility, and speed to market.

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

Viralgen's new commercial facility will be in production by the end of 2021 in San Sebastian (Spain).

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