

Press release

July 28, 2021

Patent approved in the U.S. and Russia for CombiGene's gene therapy candidate CG01

CombiGene's gene therapy candidate CG01, which is being developed for the treatment of drug-resistant focal epilepsy, has received patent protection in the U.S., the world's largest pharmaceutical market as well as in Russia. The first application for patent protection for CG01 was filed in 2016, an international PCT application was filed in 2017 and the now granted U.S. and Russian patents last until 2037. The approvals are the first national approvals of the applications completed from the PCT application.

Every year, a large number of new patients with drug-resistant focal epilepsy are diagnosed and it is this group of patients that CG01 is intended for. CombiGene currently estimates that the annual global sales potential is somewhere between USD 750 million and USD 1500 million.

The epilepsy project CG01 is in late preclinical phase and CombiGene plans to start the clinical program in the second half of 2022.

"The fact that CG01 has now obtained patent protection in the U.S., the world's largest pharmaceutical market, is extremely important from a future commercial perspective", says **Jan Nilsson**, CEO of CombiGene. "The patent also verifies the level of innovation in CG01, which has the potential to become the first approved gene therapy treatment for epilepsy."

About CG01

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.

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 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 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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