

PRESS RELEASE

April 11, 2023

One of *Nature's* journals publishes article about CombiGene's epilepsy project

STOCKHOLM — On April 7, 2023, Esbjörn Melin, Industrial Post doc at CombiGene, together with researchers from Lund and the University of Copenhagen, published the scientific article "*Combinatorial gene therapy for epilepsy: Gene sequence positioning and AAV serotype influence expression and inhibitory effect on seizures*" in the journal *Gene Therapy*, which is part of Nature's Publishing Group.

The scientific article is a compilation of the research results produced within the framework of the preclinical development of CombiGene's epilepsy project CG01. This data is now peer-reviewed, which means that an anonymous academic panel of experts has assessed the research quality, methods, and scientific novelty value of the article before it has been approved for publication.

The publication confirms that CG01 is a suitable drug candidate that provides a clear overexpression of Neuropeptide Y (NPY) and functional activity of the Y2 receptors resulting in anti-seizure effects and reduced release of glutamate in experimental models. The article also demonstrates CG01-induced overexpression of both NPY and Y2 transgenes in drug-resistant epileptic brain tissue donated from patients who have undergone surgical procedures to limit epileptic seizures. The results validate the anti-epileptic properties of the NPY peptide and Y2 receptor and show that this gene therapy can be expressed in human brain tissue, which is a prerequisite for clinical treatment effect.

"At CombiGene, we are very pleased with the publication in *Gene Therapy* and I would like to take this opportunity to congratulate Esbjörn Melin on a successful scientific work. This is also the second time that *Nature Publishing Group* has drawn attention to CombiGene's work. In December 2018 (*Nature* | Vol 564 | December 13, 2018), CombiGene's activities were highlighted in a large article describing the possibilities of using gene therapy for the treatment of epilepsy", says Karin Agerman, CombiGene's Chief Scientific Officer in a comment.

The epilepsy project CG01 was outlicensed to Spark Therapeutics in October 2021 in an agreement where CombiGene is eligible to receive up to USD 328.5 million in various milestone payments.

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 preclinical/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.



For more information:

CombiGene AB (publ)

Jan Nilsson, CEO

Phone: +46 704 66 31 63

jan.nilsson@combigene.com

Please read "Ingeneious", a newsletter from CombiGene which contains general news and information that is judged not to have a significant effect on the share price. INGENEIOUS and press releases are available at www.combigene.com