

# Professor Merab Kokaia's proof-of-concept study has been published in *Molecular Therapy: Methods & Clinical Development*

**The results of the study show that CGO1 has clinically relevant anti-seizure effects.**

On February 19th 2018 Professor Merab Kokaia, one of CombiGene's scientific founders, together with lead scientist Esbjörn Melin presented final data from the preclinical proof-of-concept study for CGO1 that was conducted in 2017. The study confirmed that CGO1 has clinically relevant anti-seizure effects in the form of fewer and shorter seizures. Following peer review, the study has now been presented in ***Molecular Therapy: Methods & Clinical Development***.

The study has been designed so that the results can be adapted for treatment of epilepsy in humans. With the aid of magnetic resonance imaging (MRI), the part of the hippocampus, i.e., the part of the brain in which the epileptic seizure arises and to which treatment is to be targeted, was localized. Subsequently, a unilateral focal treatment with gene therapy was administered to the epileptic focus. Results of the treatment have been analysed with video EEG (electroencephalography) to observe and measure electrical activity in the brain during a seizure, and histological analysis (tissue analysis) has been used to find detailed answers with respect to deviations in brain tissue and to determine how CGO1 is expressed in the brain.

The results of the study show that CGO1 has clinically relevant anti-seizure effects. Epileptic seizures were fewer and shorter in duration and some of the animals were completely free of seizures after being treated.



Lead scientist Esbjörn Melin in Professor Kokaia's lab

**The article is available here:**

[https://www.cell.com/molecular-therapy-family/methods/fulltext/S2329-0501\(19\)30102-0](https://www.cell.com/molecular-therapy-family/methods/fulltext/S2329-0501(19)30102-0)

## About CombiGene AB

*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 0399, info@fnca.se.*

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