

## Initial data from the long-term study on CombiGene's candidate drug for treating epilepsy indicate positive effects in the form of fewer seizures.

**Initial data from CombiGene's preclinical proof-of-concept-study (the long-term study) show that CombiGene's candidate drug, CGO1, has antiepileptic effects. The study has demonstrated that CGO1 reduces the frequency of epileptic seizures in animals. It is expected that final data from the study will be presented during the first quarter of 2018.**

The study, which began in April 2017, has been conducted at Lund University under the direction of Professor Merab Kokaia, one of CombiGene's scientific founders and head of the Epilepsy Centre at Lund University's Faculty of Medicine. The principal aim of the study is to provide additional evidence of the antiepileptic effect of CombiGene's candidate drug.

"It is very pleasing to report that our study has reached a positive outcome," says Professor Kokaia. "Much remains to be done before we will be able to present a functioning, approved therapy, but the long-term study is a very significant step towards the achievement of our objective. That we have now seen a positive response means that we can take the next step with greater confidence."

The outcome of the study is being analyzed in three different ways. MR scanning (magnetic resonance) has been used to investigate how much of the brain is damaged, video EEG (electroencephalography) is

used to observe electrical activity in the brain during a seizure, and histological analysis (tissue analysis) is used to find detailed answers with respect to deviations in brain tissue and to determine the levels of the candidate drug, CGO1.

CombiGene's CEO Jan Nilsson comments on the outcome of the long-term study:

"2017 has been an intensive year of development for CGO1. During the first quarter we completed a dose-response study and we have now completed the long-term study. Both studies have been exceptionally well conducted, which means that CombiGene is now ready, much earlier than I would have dared to hope, to take the next important step towards an approved anti-epilepsy gene therapy. During 2018 we will continue intensive work on our CGO1 epilepsy project with an aim to commencing process development towards production of CGO1 according to the requisite quality standards, so that we can eventually conduct the first clinical studies."

*This information is information that CombiGene AB (publ) is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted, by CEO Jan Nilsson, for publication on December 6 2017.*

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### About CombiGene AB

*By combining modern neuroscience with recent advances in gene delivery, CombiGene has developed a method shown to suppress epileptic seizures in preclinical studies. The current focus is on continuing to develop this method into an effective and safe therapy for epilepsy patients, but the method may also have development potential as a means of treating other neurological disorders. Founded on the basis of scientific discoveries made at Lund University and the University of Copenhagen, CombiGene has offices at Medicon Village in Lund, Sweden. The company is public and listed on the Swedish marketplace AktieTorget. [www.combigene.com](http://www.combigene.com)*

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