

## Press release

June 28, 2021

# CombiGene and Neurochase begin work to optimize the administration of CG01

**CombiGene's epilepsy project CG01 continues to advance towards the first clinical trial scheduled to begin in 2022. An important step on the way to clinical studies is optimizing the administration of CG01. CombiGene has now started this work with a preclinical study in large animals conducted in collaboration with the British company Neurochase.**

Neurochase and its founder neurosurgeon Professor Steven Gill specialize in administering drugs to different parts of the central nervous system and have extensive experience in administering the very same type of gene therapy vectors used in CG01.

In the study, the administration will be observed using so-called real-time imaging, i.e. a continuous monitoring how the injected substance spreads in the brain. In this way, the substance can be viewed in real time as the gene therapy vectors reach the intended part of the brain. The study also includes analyses of the protein expression in the brain to ensure that the expression of proteins follows the same pattern as the administered substance. The study will be conducted in two steps. In the first step, the administration technique itself is evaluated with a gene therapy vector that expresses a fluorescent protein together with a contrast fluid that makes it possible to follow the course of injection all the way to the target area and to see where the proteins are expressed. In the second step, the process is repeated with CG01 to confirm that the administration also gives the intended expression of the active substances NPY and Y2.

The results of the study are expected to be completed in the late summer of 2021 and a positive outcome will mean that long-term studies in toxicology and biodistribution in large animals can start as planned.

### About CG01

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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

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

### About CombiGene AB

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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](mailto:info@fnca.se).



#### **About Neurochase**

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*www.neurochase.com*

Neurochase, founded by Professor Steven Gill, aims to bring transformative targeted therapies to patients with neurological diseases using state of the art technology. The company's team specializes in creating bespoke therapeutic strategies for the direct delivery of therapies using Convection Enhanced Delivery (CED). Neurochase provides accurate, targeted and safe direct drug delivery to the CNS and develop globally scalable treatment strategies and solutions for the pharmaceutical and biotech industry. Neurochase Ltd. is a Registered Company Number 12428919

#### **For more information:**

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