



## PRESS RELEASE

January 25, 2022

# CombiGene signs agreement to evaluate the leading gene therapy candidate within the lipodystrophy project CGT2

**STOCKHOLM, January 25, 2022 — CombiGene AB (“CombiGene”, “the Company”) today announced that the Company has signed an important agreement with Professor Ormond MacDougald at the University of Michigan Medical School. The agreement comprises one pilot study and one main study in which CombiGene’s most promising gene therapy candidate within the lipodystrophy project CGT2 will be evaluated.**

Professor Ormond MacDougald’s new experimental model, which was published in the journal *Diabetes* in June 2021 has several characteristics that are similar to partial lipodystrophy in humans, the disorder that CombiGene is targeting with their CGT2 therapy.

“We observed a striking loss of white adipose tissue in adult lipodystrophic mice, along with increased fat deposition in the liver, elevated blood glucose levels, and increased insulin levels compared to the controls,” said Professor MacDougald.

Professor MacDougald and his clinical collaborator, Dr. Elif Oral, have an entire team working within the lipodystrophy field and their extensive knowledge and technical expertise will benefit CombiGene’s CGT2 project. The MacDougald Lab is a leading discovery lab for adipocyte biology. University of Michigan Metabolism, Diabetes and Endocrinology Division has emerged as a leader in the treatment of lipodystrophy syndromes due to Dr. Oral’s longstanding clinical interests in this condition.

“I’m very happy that CombiGene has signed this agreement with Professor MacDougald,” said Annika Ericsson, Preclinical Project Manager at CombiGene. “Professor MacDougald and his team have exactly the knowhow and experimental model that we need to evaluate our leading candidate in the lipodystrophy project.”

### Reference to the article

Adipocyte-Specific Deletion of Lamin A/C Largely Models Human Familial Partial Lipodystrophy Type 2  
Callie A.S. Corsa, Carolyn M. Walsh, Devika P. Bagchi, Maria C. Foss Freitas, Ziru Li, Julie Hardij, Katrina Granger, Hiroyuki Mori, Rebecca L. Schill, Kenneth T. Lewis, Jessica N. Maung, Ruth D. Azaria, Amy E. Rothberg, Elif A. Oral, Ormond A. MacDougald  
Journal: *Diabetes*  
*Diabetes* 2021;70(9):1970–1984  
DOI: <https://doi.org/10.2337/db20-1001>  
Published: 04 June 2021

### About CGT2

The goal of the CGT2 project is to develop a gene therapy treatment for partial lipodystrophy, a rare disease characterized by altered fat distribution on the body. In the absence of normal body fat, various organs begin to accumulate fat, leading on to serious metabolic complications, including extreme insulin resistance, hypertriglyceridemia (elevated values of blood fat triglyceride) and liver steatosis (fatty liver). There are currently a few symptom-relieving treatments for lipodystrophy, but no therapy that targets the root cause of the disease. For patients suffering from partial lipodystrophy, there are currently no treatments at all.



## **Eurostars Programme**



CombiGene's project CGT2 is supported by the Eurostars Programme.  
Project ID: 114714

## **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 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 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, +46 (0)852 80 03 99, [info@fnca.se](mailto:info@fnca.se).

### **For more information:**

*CombiGene AB (publ)*

Jan Nilsson, CEO

Tel: +46 (0)704 66 31 63

[jan.nilsson@combigene.com](mailto:jan.nilsson@combigene.com)

Bert Junno, Chairman of the Board

Tel: +46(0) 70 7 77 22 09

[bert.junno@combigene.com](mailto:bert.junno@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](http://www.combigene.com)