PRESS RELEASE
Lund, September 15 2020

CombiGene and CGT Catapult collaboration completes development of quality control analytical assays for clinical production of CombiGene's AAV-based gene therapy for the treatment of epilepsy

The extensive and advanced work to develop the critical analytical assays needed for quality control in the manufacture of the AAV1 based gene therapy product CG01 has now been completed in a collaboration between CombiGene and Cell and Gene Therapy Catapult (CGT Catapult). CombiGene will now integrate the analytical assays into its production process, taking it another step closer to its final large scale production design for clinical trials and commercial distribution.

Ensuring that pharmaceuticals are of the same quality during every individual production run is an essential regulatory requirement. To meet this, CombiGene and CGT Catapult have collaborated to develop a tailor-made package of analytical assays to ensure that the production of the AAV1 based gene therapy CG01 meets these regulatory requirements.

CG01 represents an innovative approach for the treatment of drug-resistant focal epilepsy. CGT Catapult experts developed seven unique assays in order to characterise the various viral vector components and their performance. The methods to conduct these tests will now be transferred to the CDMO selected by CombiGene to manufacture their AAV drug material for clinical use. These assays are mandated by regulatory requirements so completion of their development to suitable standards of reproducibility, accuracy and precision is a significant milestone.

"It is very gratifying that we now have concluded the work on the analytical methods together with CGT Catapult. It has been challenging work both technically and scientifically. This is another important step in securing the production of CG01", says Karin Agerman, Chief Research & Development Officer at CombiGene.

Matthew Durdy, CEO at CGT Catapult commented:

"CombiGene is an innovative gene therapy company working in an important area of unmet medical need. The availability of reliable and meaningful assays for manufacturing control is an issue for the industry and an area in which we set out to increase innovation and capability. This successful collaboration with CombiGene has done that, and will further the development of this therapy for the benefit of patients with epilepsy."

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

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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.

About The Cell and Gene Therapy Catapult

The Cell and Gene Therapy Catapult was established as an independent centre of excellence to advance the growth of the UK cell and gene therapy industry, by bridging the gap between scientific research and full-scale commercialisation.

With more than 230 employees focusing on cell and gene therapy technologies, it works with partners in academia and industry to ensure these life-changing therapies can be developed for use in health services throughout the world. It offers leading-edge capability, technology and innovation to enable companies to take products into clinical trials and provide clinical, process development, manufacturing, regulatory, health economics and market access expertise.

Its aim is to make the UK the most compelling and logical choice for UK and international partners to develop and commercialise these advanced therapies. The Cell and Gene Therapy Catapult works with Innovate UK.

For more information please visit ct.catapult.org.uk or visit http://www.gov.uk/innovate-uk.

For further information:

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