

INGENEIOUS

NEWS FROM COMBIGENE AB

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CombiGene – World-leading Swedish gene-therapy company

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Big thanks to all our shareholders!

Let me begin this editorial by expressing my sincere thanks to all of our shareholders who participated in CombiGene's recently concluded preferential rights issue. Your support and the confidence you have shown in CombiGene means a lot to us and really inspires us to continue working hard.

I would also like to congratulate our collaboration partner CGT Catapult on the recent opening of their large-scale manufacturing centre in Stevenage, which was approved by the MHRA (Medicines and Healthcare Products Regulatory Agency). It is very gratifying to note that the partner we have chosen to assist us in the development of a manufacturing method for our epilepsy treatment, CGO1, continues to grow and thrive. Don't miss the interview with Professor Johan Hyllner, who helped to establish CGT Catapult!

Strong financial resources give us the means to continue developing our CGO1 project with full force and without delay. As this issue of Ingenious goes to press, it is difficult to say exactly when we will be able to proceed with clinical studies. Developing a manufacturing method for a gene therapy is very advanced and, in many respects, groundbreaking work. Our next task will be to continue process development for our candidate drug, CGO1, and to subsequently reach the next important milestone, the start of obligatory biodistribution and toxicity studies during 2019.

Concurrently with the development of CGO1 we will continue to strengthen our ties with potential partners in the major pharma companies.

During 2018/2019 we also actively seek new and interesting projects to broaden our portfolio. CombiGene is Sweden's only listed gene-therapy company and we have in recent years amassed considerable valuable experience and built expertise which, I believe, is unique to CombiGene. All in all, this makes us a very attractive partner for future development projects.

Jan Nilsson, CEO,
CombiGene

EDITORIAL STAFF

Contact:

redaktionen@combigene.com

Legally responsible publisher:

Jan Nilsson

Production: Wiberg & Co Reklambyrå.

Text: Michael Vallinder

English translation: Mark Wilcox

CombiGene AB (publ) Medicon Village,
SE-223 81 Lund, Sweden
info@combigene.com

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newsletters, available on our website.

www.combigene.com

Successful preference issue for CombiGene

CombiGene's preference issue, which concluded on September 18th 2018, was a huge success. The issue of units was oversubscribed to 46 million kronor, which corresponds to a subscription rate of about 148 percent. The issue raised about 31 million kronor before deductions for issuing expenses. Owing to the very great interest in the offer, the issue guarantees have not been utilized.



As soon as the results of the issue were announced, Ingenious contacted CombiGene's CEO Jan Nilsson for a comment.

What is your first reaction to the outcome of this very successful issue?

"I am delighted that our shareholders have shown such great confidence in our project. It is truly inspiring to be able to continue on the chosen path and develop CombiGene as we have

done in recent years: to deliver on the goals we have set, methodically and step-by-step."

In concrete terms, what does the successful issue mean for CombiGene?

"Overall, the issue means two things. First of all, we now meet the terms for EU funding. Horizon 2020, the EU framework programme for research and development, notified us in May that they will grant CombiGene

3.36 million euros in the ongoing development and commercialization of our epilepsy project, CGO1. This is conditional upon CombiGene securing part-funding amounting to 30 percent of the cost of the project. This condition has been met and we have received an initial payment of 1.5 million euros from Horizon 2020.

Secondly, together with the EU grant, the issue gives us the financial resources to continue developing our candidate drug, CGO1, with full force and without delay. In concrete terms, this means that during 2018 and 2019, together with our partner CGT Catapult, we will develop a manufacturing method for CGO1 and, subsequently, initiate biodistribution and toxicity studies."

What will happen now?

"Now, we'll do what we always do at CombiGene. We'll roll up our sleeves and continue working. And we are more inspired than ever!"

Major interest in CombiGene at NLS Days in Stockholm



■ **NLS Days (Nordic Life Science Days) is the biggest partnering conference for the life science industry in the Nordic region. Since the start in 2013, NLS Days has been a forum for interesting meetings and valuable business contacts.**

The most recent edition of NLS Days, held 11-12 September 2018 at Stockholm Waterfront, attracted some 1,300 delegates from 40 countries. Delegates included CombiGene's CEO Jan Nilsson and CombiGene's Chief Research and Development Officer Karin Agerman.

During the conference Karin and Jan held about 20 meetings with various companies and organizations to establish important new contacts and bring existing contacts among the big pharma companies up to date on the latest developments for CombiGene. In addition to meetings with drugs companies, Karin and Jan met with potential investors, as well as representatives for CROs and CMOs (clinical research organizations and contract manufacturing organizations) that may be interesting partners in future clinical studies.

This was the first time Karin Agerman represented CombiGene at NLS Days. Ingenious contacted her for a comment.

"Participation in NLS Days was a very valuable experience for me, personally. CombiGene may be a small company, but we're part of something that is huge. When you take part in an event like NLS Days, you realize how quickly things are moving in the life science sector and just how much interest CombiGene has attracted among a great many players. Taking part in a conference such as this gives us a fantastic opportunity to maintain existing contacts and make new ones. And it gives you an enormous will and energy to continue developing CombiGene."



Interview with Professor Johan Hyllner, former Chief Scientific Officer at CGT Catapult

■ **British CGT Catapult is CombiGene's manufacturing partner for development of a complete and finalized manufacturing method that will enable CombiGene to take its candidate drug, CGO1, to commercial GMP manufacturing and clinical trials. A collaboration agreement was reached in early 2018.**

CombiGene and CGT Catapult first met at Nordic Life Science Days in Malmö, in autumn 2017, when CombiGene's CEO Jan Nilsson heard a presentation by Professor Johan Hyllner, who was at that time CGT Catapult's Chief Scientific Officer. "I realized immediately that CGT Catapult was a potential partner for CombiGene," says Jan Nilsson. "We had already been in contact with several possible partners, none of which had the breadth and scope of CGT Catapult, and I first spoke with Johan Hyllner just after his presentation to discuss a possible collaboration. For a small company like CombiGene, choosing the right partners is crucial and we feel we've definitely made the right choice with CGT Catapult."

Things went quickly after that initial contact. During the autumn representatives for CombiGene and CGT Catapult met on several occasions and, as early as January 2018, an agreement was reached.

Ingeneious contacted Johan Hyllner to hear what he had to say about CombiGene. Some time after the agreement between CombiGene and CGT Catapult was signed Johan Hyllner resigned as

Chief Scientific Officer at CGT Catapult and is now acting on behalf of the EU as an adviser to five companies within Horizon 2020's SME instrument (Small and Medium-sized Enterprises).

Tell us a little about CGT Catapult's selection process.

"CGT Catapult has a very structured process for selecting collaborating companies. First, they gauge the level of scientific excellence and integrity of potential collaborating companies. Other parameters are benefit to society that may result from the project and how CGT Catapult and the collaborating company can benefit from each other."

In CombiGene's case, the time from initial contact to finalized agreement was relatively short. Does this mean collaboration with CombiGene was an easy decision?

"Absolutely. CombiGene delivered on all counts. Not least, we were very impressed by the science done by CombiGene's founders, Professor Merab Kokaia and Associate Professor David Woldbye."

CGT Catapult is not well known in Sweden. Can you tell us a little about the organization?



"Of course. CGT Catapult is a UK government initiative created to advance the development of cell and gene therapy; thus, the name CGT. The focus is on UK enterprises, but CGT Catapult also assists international companies, as in the case of CombiGene. Above all, CGT provides expertise in process development, upscaling and quality assurance of manufacturing, regulatory and clinical work, and health economics and remuneration systems.

Is CGT Catapult a large organization?

It's growing. Myself included, we were originally nine employees when we started five years ago. CGT Catapult has grown over the years, both in term of research and development facilities and personnel. Staff now number about 170."

You are now an adviser to five companies within Horizon 2020's SME instrument. Not long ago Horizon 2020, the EU framework programme for research and development, decided to invest 3.36 million euros in CombiGene's ongoing development and commercialization of the company's gene therapy project focussing on difficult-to-treat epilepsy. Can you comment on this?

"First of all, I am very pleased. For a company of CombiGene's size, securing long-term financing of operations is very positive. At the same time, the funding from Horizon 2020 naturally implies a mark of quality. As mentioned, I am no longer active in CGT Catapult, but I wish CombiGene every success in their very exciting journey."



CGT Catapult gets the green light in Stevenage

■ **CGT Catapult's large-scale GMP manufacturing centre in Stevenage, UK, was officially opened on April 23rd this year. Among prominent guests attending the inauguration were CombiGene's CEO Jan Nilsson and board chairman Arne Ferstad, which we noted in Ingeneious #2.**

On September 11th CGT Catapult announced that the centre had been officially approved by the MHRA (Medicines and Healthcare products Regulatory Agency). The new Stevenage centre will

provide collaborating companies with the resources for large-volume manufacturing of their medicines according to GMP standard. Although this is not currently in question for CombiGene,

we at Ingeneious would like to take this opportunity to congratulate our colleagues at CGT Catapult on the newly approved facility.

Read all of our news in one place

We would like to keep you well informed about what is happening in the company. Therefore, we are now launching CombiGene's digital newsletter to allow you to read all of our news first-hand.

Subscribe via our website, where you can register the e-mail address to which you wish the newsletter to be sent. Use the link "Subscribe to our newsletter" at the top-right corner of the page. The subscription is free of charge and you can cancel it whenever you please.

Read "More from the company" and our Press Releases at: www.combigene.com



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<http://combigene.com/anmalan-nyhetsbrev/>

For many sufferers, there is no help – yet.

We hope to change this. We are in the process of developing a world-leading method for treating epilepsy. The potential is enormous, the outlook is good.

For very many people.

This autumn CombiGene will take part in the following events:

22–24 October 2018

Conference: 2nd Annual Gene therapy for Rare Disorders Europe

Venue: Novotel London West, London, UK

CombiGene's CEO Jan Nilsson, Chief Research and Development Officer Karin Agerman and board chairman Arne Ferstad will attend. Jan Nilsson will also take part in the panel discussion "The Next Step – Pioneering Gene Therapies in More Common Diseases", 24 October at 11.30 a.m.

Read more: <http://genetherapy-europe.com/>

5-6 November 2018

Conference: BIO Europe

Venue: Bella Center Copenhagen, Copenhagen, Denmark

BIO Europe, Europe's biggest life science partnering conference, is an important forum where CombiGene can meet potential partners in a range of disciplines. CombiGene will be represented by the company's Chief Research and Development Officer Karin Agerman, who will take part in a panel discussion entitled "Partnering landscape in advanced therapies", on 6 November at 11.00 a.m.

Read more: <https://ebdgroup.knect365.com/bioeurope/>

20 November 2018

Presentation: Gene therapy: the future treatment for epilepsy?

Venue: Epilepsy Association in Greater Stockholm.

Merab Kokaia, Professor in Neurophysiology and head of the Epilepsy Centre, Lund University Faculty of Medicine, discusses the challenges and opportunities implicit in turning an idea into an approved drug.



The gene therapy explorer

CombiGene's objective is to develop treatment methods that can improve the quality of life for millions of people throughout the world.

www.combigene.com