

INGENEIOUS

NEWS FROM COMBIGENE AB

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CombiGene – World-leading Swedish gene-therapy company

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CombiGene has on previous occasions held several meetings with attending physicians, in Sweden and Denmark, to gain an understanding of their everyday reality and look at the possibilities for treating epilepsy with CG01.

 **combiGene**
The gene therapy explorer

Continued high level of activity

A lot of positive things continue to happen at CombiGene. Our new colleagues have brought great expertise and incredible energy to the company. Starting with this issue of Ingenious we will present these people and the fantastic ways in which they are contributing to the development of CombiGene.

Our important CG01 epilepsy project is progressing at a good pace. Among other things, this means that we are now approaching selection of the CMO (Contract Manufacturing Organisation) which will produce the GMP-classed (Good Manufacturing Practice) material for coming studies, and the CRO (Contract Research Organisation), which on our behalf will conduct the experimental safety studies which are necessary before clinical trials can begin.

On April 18th CombiGene submitted a public takeover bid for Panion Animal Health, formerly a subsidiary of CombiGene. The main reason for acquiring Panion is that, by gathering all of our assets under one roof, we will improve our negotiating position with strategic partners. Acquisition of Panion reduces the complexity for a potential future acquirer. Quite simply, it will be easier to do business with CombiGene. For further information, see the article about the Panion bid in this issue of *Ingenious*.

Jan Nilsson
CEO



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Please read our press releases and newsletters, available on our website.

www.combigene.com



CombiGene submits bid for Panion

In 2016 CombiGene signed an agreement for out-licensing of its technology for treating epilepsy to Panion Animal Health AB for application in veterinary care. On April 18th CombiGene submitted a bid for Panion. *INGENIOUS* contacted CombiGene's CEO, Jan Nilsson, for the story behind the bid for Panion.



Panion was spun off from Combigene in 2016. Why does CombiGene now wish to buy back the company?

There are several reasons, but the main reason for acquiring Panion is that, by gathering all of our assets under one roof, we will improve our negotiating position with strategic partners. In my experience, and I'm sure many would agree, when you're on the buying side, you want to have full control over the projects you intend to acquire. As a buyer, you want to avoid a situation where there are several different negotiating partners. At CombiGene we see the acquisition of Panion as a logical means of reducing the complexity for a potential future acquirer. Quite simply, it will be easier to do business with CombiGene.

The other reason has to do with synergies. When Panion was distributed to CombiGene's shareholders in 2016 the motive was that a separate and dedicated veterinary medical company would be able to take the product to the veterinary market faster. Since then, both CombiGene and Panion have gained considerable experience in the gene therapy field via their respective development programmes. This means that we foresee synergies in important areas such as quality and regulatory issues. Acquisition of Panion will also give us coherent control over our intellectual property rights, which means a very great deal with respect to potential partners.

CombiGene is offering 16.7 million kronor for Panion (0.67 kronor per share), corresponding to a premium of 25.2 percent. What more can you say about the way the takeover bid is being made?

We have chosen to offer CombiGene shares in payment, instead of cash. Since Panion's shareholders are offered shares in CombiGene in exchange, the shareholders will benefit from all synergies resulting from the transaction. The bid will potentially result in greater long-term value for all shareholders.

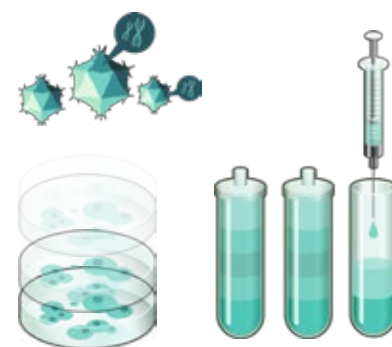
See all the information about the bid for Panion on CombiGene's website combigene.com.

COMMENTS FROM OUR CHIEF RESEARCH AND DEVELOPMENT OFFICER

CG01 – update

The level of activity in the CG01 project remains high. Here are a few of the most important activities during the first quarter:

- Work at CGT Catapult was ramped up during the quarter. In addition to work with the ongoing development of analysis methods, CGT Catapult has also begun process development for manufacturing of CG01.
- Major focus has also been placed on assessing several of the CMOs (Contract Manufacturing Organisations), one of which will produce the GMP-classed (Good Manufacturing Practice) material for coming experimental safety studies and the first clinical study.
- Our new colleague Annika Ericsson has assumed responsibility from day one and done a fantastic job with detailed planning of the coming experimental safety study, as well as assessing an appropriate CRO (Contract Research Organisation) for conducting the study. This work is vitally important, since this study is mandatory before we can begin our first clinical study.
- Tentative preparations for the initial clinical study have begun, since we hired a British consultant who to arrange a meeting with clinics in the UK in order to gain an understanding of the situation for therapy-resistant epilepsy patients in the UK. The consultant has considerable experience of



clinical studies and gene therapy projects, having worked with both large pharmaceutical companies and biotech firms. Together with our consultant, we planned and held the first meeting with epilepsy specialist physicians and neurosurgeons in the UK in early April. (See separate article in this issue of *Ingenious*.)

Karin Agerman

Chief Research and
Development Officer



EUROPEISKA UNIONEN
Europeiska regionala utvecklingsfonden

The CG01 project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 823282. The content of this report reflects only the Company's view. The Commission are not responsible for any use that may be made of the information.

Amid financial reports, horses and gardens

Anna Jönsson joined us in January 2019 as CombiGene's Chief Financial Officer (CFO). Ingeinous contacted Anna to hear what she had to say about her first months at CombiGene.

"Tremendously fun and incredibly intensive," replied Anna succinctly. "That's just how I like it. In my position one is in one way or another involved in most of what's going on in the company. Spring is also a very intensive period, with the year-end statement, the annual report, the Q1 report and, not least, the annual general meeting."

It sounds as though her employment at CombiGene, which is half-time, together with her duties in other companies, would demand most of her energy, but it is soon apparent that such is not the case. After a day at the office, Anna takes care of her three horses (a halfblood and two ponies), does crossfit training, works in the garden and spends time with her family (husband and two children).



The crucial reality

There are many decisive elements behind the creation of a gene therapy: discovery of a potential mechanism of action, fundamental research, preclinical studies, development of analysis methods, process development, biodistribution studies, regulatory approval, choice of collaborative partners and much more.

For those involved in the project, the days are filled with meetings, analyses, reports, planning, feedback, unforeseen problems, elegant solutions, new findings, new opportunities, late nights and early mornings, passion and energy. At the same time, it is vital to intermittently lift our gaze from the details of the project and reconnect with everyday reality, i.e., the work of the doctors and nurses who will deliver the new therapy in clinical practice. CombiGene has on previous occasions held several meetings with attending physicians, in Sweden and Denmark, to gain an understanding of their everyday reality and look at the possibilities for treating epilepsy with CGO1.

Clinical Advisory Board in the UK

As CGO1 comes ever closer to the clinical studies stage, it is increasingly important to know in detail how CGO1

can be practically applied in care and how procedures, decision-making processes and approaches differ from country to country. On April 5th CombiGene, together with a very experienced British consultant, took part in a Clinical Advisory Board meeting in London, UK. Attending the meeting were two neurosurgeons, a paediatric epilepsy specialist physician, a neurologist and a nurse who specializes in clinical studies. CombiGene was represented by CEO Jan Nilsson, Chief Research and Development Officer, Karin Agerman, project manager Maria Klockare and the company's scientific founder, Professor Merab Kokaia.

"The meeting in London was very productive," says Karin Agerman. "We began with a rather detailed presentation of the CGO1 project with respect to the mechanism of action and



administration, and received positive feedback. It was apparent that the physicians at the meeting are positively disposed towards gene therapy as a form of treatment for epilepsy. At the same time, we gained a good insight into the provision of treatment for epilepsy in the UK."

CombiGene now plans to intensify dialogue with healthcare representatives and to hold similar

Clinical Advisory Boards in other European countries and, possibly, in the USA.

"Understanding the day-to-day reality of epilepsy treatment and how it varies from one country to the next is a decisive success factor for CombiGene," concludes Karin Agerman.

NOTED



Big Pharma continues to show big interest in gene therapy

Great interest in gene therapy on the part of the international pharmaceutical industry continues to manifest itself through the acquisition of gene-therapy companies. A recent example is Johnson & Johnson's (J&J) acquisition of MeiraGTx. J&J is paying USD 100 million upfront, plus an additional USD 340 million in milestone payments for commercial rights to MeiraGTx's clinical-stage development programme for retinal diseases.

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

Sign up here!
<http://combigene.com/annalan-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.

Events

June 3-6, 2019

BIO International Convention, Philadelphia USA

<https://convention.bio.org/2019/>

September 10-12, 2019

NLSDays, Malmö

<https://www.nlsdays.com/>

September 10, 2019

Aktiedagen, Stockholm

October 22-25, 2019

European Society of Gene and Cell Therapy 27th annual congress, Barcelona

<https://www.esgct.eu/congress/barcelona-2019.aspx>

November 4, 2019

Stora Aktiedagen, Göteborg



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.

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