

THE GENE THERAPY EXPLORER

In many ways, it has been a very positive first half of the year for CombiGene. We have reported good results in the CG01 project with both a preclinical pharmacokinetic study and a preclinical learning and memory study. We have also made significant progress in the development of the production method for CG01. Although more significant milestones in the CGT2 project are a little further down the line, CGT2 continues to develop as planned.

Interim Report

January – June 2020 for CombiGene AB (publ)



Interim Report January-June 2020 for CombiGene AB (publ)

Period April - June 2020

- Net sales: 0 (0) TSEK.
- Other operating income: 2 161 (3 137) TSEK.
- Profit after financial items: -4 389 (-2 814) TSEK.
- Earnings per share: -0,03 (-0,05) SEK.

Period January - June 2020

- Net sales: 0 (0) TSEK.
- Other operating income: 6 219 (4 772) TSEK.
- Profit after financial items: -12 055 (-5 285) TSEK.
- Earnings per share: -0,10 (-0,10) SEK.
- Liquidity at the end of the period: 18 026 (21 839) TSEK.
- Equity ratio at the end of the period: 70,98 (59,35) %.

Significant events in the second quarter of 2020

- Cobra Biologics achieves an important milestone in CombiGene's epilepsy project CG01 through the delivery of the first DNA plasmid needed to produce CG01. The production method for plasmids is now fully developed and the first plasmid is produced for use in manufacturing of CombiGene's gene therapeutic drug candidate CG01.
- CombiGene completes the rights issue of shares and warrants, so-called units, which was decided by the Board of Directors on February 18, 2020. The subscription period ran from 20 March 2020 to 3 April 2020. Rights issue was subscribed for SEK 26.28 million before issue costs.
- CombiGene transacts a directed issue of units consisting of shares and warrants totaling SEK 4 million to Modelio Equity AB (publ) ("Modelio") and Oscar Molse essentially under the same conditions as the recently completed rights issue that contributed SEK 26.28 million to the Company.

- In connection with the rights issue and the directed issue in April, warrants of series 3 and series 4 were issued. Upon full exercise of all series 3 and series 4 warrants, the Company will receive a maximum of approximately SEK 39,37 million, before issue costs
- On June 29, 2020, CombiGene AB held its Annual General Meeting in Lund. The Annual General Meeting voted on all proposed matters in accordance with the Proposal of the Board of Directors or the Nomination Committee. Bert Junno, Jan Nilsson, Jonas Ekblom and Per Lundin were elected as new board members. Peter Nilsson was re-elected as a board member. Bert Junno was elected as the new Chairman of the Board. The AGM voted that fees for the period between the 2020 Annual General Meeting and the 2021 Annual General Meeting shall be paid SEK 125,000 to ordinary board members who do not receive salary from the company and SEK 200,000 to the Chairman of the Board. For further information, please see the company's website: combigene.com

Significant events after the end of the period

Epilepsy Project CG01

- At the beginning of the third quarter, CombiGene's production partner Cobra Biologics (Cobra) completed production and delivery of the two remaining DNA plasmids.
- Cobra has also produced three GMP master cell banks. This means that CombiGene now has a uniform starting material for all future production of the three plasmids with quality and characteristics that are exactly the same at each individual production batch. The master cell banks can thus be used every time CombiGene needs to manufacture new plasmids for the production of CG01 for future clinical studies and commercial production.
- CombiGene, together with the Spanish CDMO manufacturer Viralgen, has conducted a pilot study to evaluate the possibilities of using Viralgen's suspension method for the production of the drug candidate CG01.



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

- CombiGene has completed a tropism study showing that CG01 is only expressed by the nerve cells in the hippocampus and not the glial cells (a type of supporting cells in the brain).

Directed issue

- On August 4, the Board of Directors of CombiGene AB (publ) decided to carry out a directed share issue of 21,588,125 units, each consisting of one share and one warrant of series TO5, at a price of SEK 0.72 per unit, corresponding to an initial investment of EUR 1.5 million or approximately SEK 15.5 million, before issue costs, to the Dutch investment company NYIP ("Nyenburgh Holding BV") a leading Dutch life-science investor. The warrants of series TO5 are issued free of charge and have the same terms and conditions as the Company's series TO4 warrants, meaning that the warrants of series TO5 can be exercised for subscription of shares during the period 16 November 2020 – 30 November 2020 at a subscription price corresponding to 10 days volume-weighted average price for the period 2 November 2020 – 13 November 2020, with a discount of 30 percent, but not less than SEK 0.5 and not more than SEK 0.7 per new share. Upon full exercise of all warrants of series TO5, the Company will receive a maximum of approximately SEK 15 million, before issue costs.



CombiGene AB – The Gene Therapy Explorer

CombiGene's vision is to provide patients affected by severe life-altering diseases with the prospect of a better life through novel 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 is public and listed on the Swedish marketplace Nasdaq First North Growth Market and the company's Certified Advisor is FNCA Sweden AB, +46 (0)852 80 03 99, info@fnca.se.

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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

A word from the CEO

In many ways, it has been a very positive first half of the year for CombiGene. We have reported good results in the CGO1 project with both a preclinical pharmacokinetic study and a preclinical learning and memory study. We have also made significant progress in the development of the production method for CGO1. Although more significant milestones in the CGT2 project are a little further down the line, CGT2 continues to develop as planned.

In addition, we have worked intensively with the financing of the company. In March and April, we conducted a rights issue and a directed issue that in total brought the company SEK 30 million before issue costs. Financing of the company is an issue that is always relevant to me as CEO and we continuously search for good long-term financing solutions for the company.

Evaluation of production method for CGO1

One of the most important areas right now is to finish the development of the production method for CGO1. The completion of this work is a prerequisite for us to be able to carry out the project's final preclinical studies and, subsequently, to start the first studies in humans.

Plasmids and master cell banks ready.

During the summer, Cobra Biologics (Cobra) has completed the production of the final two plasmids used as the starting material for CGO1. Cobra has also manufactured master cell banks for the three plasmids. The master cell banks guarantee that the quality and characteristics of the plasmids are exactly the same at each individual manufacturing run for all future production of the three plasmids.

The fact that the three plasmids and their master cell banks are now in place means that we can go ahead and make the final choice of production method.

Evaluation of suspension method for the production of CGO1.

Gene therapy is a very dynamic area, not only in terms of the development of new treatments, but also in terms of production methods. For CombiGene, these advancements have meant that we now have the opportunity to choose a suspension method for the production of CGO1.

In early August, we completed a successful pilot production of CGO1 together with the Spanish CDMO manufacturer Viralgen, which offers a suspension method for the production of AAV vectors. The development of suspension methods has moved very quickly in recent years and offers great advantages in scaling up production. Large-scale production of gene therapeutic drugs was for a long time a challenge for the entire industry. The most common production method to date, so-called adherent production, has been excellent for producing small volumes of gene therapeutic drugs, but is associated with significant difficulty in scaling up production volumes. For CombiGene's drug candidate CGO1, this posed particular difficulties as it is being developed for a large patient population compared to many other gene therapies. We estimate that nearly 10,000 patients will be able to be treated with CGO1 annually.

The initial results from Viralgen are very promising and we will now evaluate all aspects of the pilot study before making a final decision on the choice of production method.

Positive results from the tropism study in the CGO1 project

The brain is made up of neurons and non-neuronal cells called glial cells. A unique feature of viruses and thus virus vectors is that they have so-called tropism, which means that they specialize in selectively infecting certain types of cells. Our tropism study shows that CGO1 is only taken up by the nerve cells in the hippocampus and not the glial cells. This knowledge verifies our understanding of how CGO1 works and also provides positive answers to questions put to us by the authorities.



CombiGene's lead project CGO1 has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 823282

Nyenburgh Holding BV invests in CombiGene

CombiGene AB has completed a directed share issue of 21,588,125 units, each unit consisting of one share and one warrant of series TO5, at a price of SEK 0.72 per unit, corresponding to an initial investment of EUR 1.5 million or approximately SEK 15.5 million, before issue costs, to the investment company Nyenburgh Holding BV, a leading Dutch life-science investor.

The Board's decision to issue units, consisting of shares and warrants, is based on authorization from the Annual General Meeting on June 29, 2020. The subscription price corresponds to a discount of approximately 11 percent compared to the volume-weighted price over a period of ten trading days until 31 July 2020. The Board of Directors has assessed that the subscription price and other terms of the issue are market-based, especially in the light of the current market situation. The purpose of the directed issue and the reason for deviation from the shareholders' preferential rights is to cost-effectively bring capital from a strategic investor to the Company.

Through the directed issue to NYIP, CombiGene strengthens its financial position in a decisive way. The issue itself brings to the Company just over SEK 15 million. In addition, CombiGene may receive approximately SEK 54 million in the autumn 2020 through the warrants of series TO3, TO4 and TO5. With this capital, we are well equipped to continue the development of our epilepsy project CGO1 and our lipodystrophy project CGT2 entirely according to plan. I am also very pleased that NYIP has chosen to invest in CombiGene. NYIP has a great deal of experience investing in European life science companies and will bring CombiGene know-how and experience in addition to the capital they invest in the Company.



Effects of the covid-19 pandemic

The Covid-19 pandemic will affect all parts of our society for a long time. Exactly how great the effects of this unique situation will be, no one yet knows. The immediate effects on CombiGene's operations remain limited. We continue our work as before but use digital technology to minimize the number of social contacts. We do not currently expect any substantial delays in any of our projects.

Jan Nilsson
CEO

The global annual market for the candidate drug, CG01, is estimated at USD 750 – 1,500 million

Epilepsy is a global problem. It is estimated that the disease affects 0.6 to 0.8 percent of the world's population. In 2016, 5.7 million people were diagnosed with epilepsy in the USA, the EU5 and Japan. About one-third of these patients do not respond to conventional medical treatment. Of these, some 60 percent suffer from focal epilepsy, i.e., a form of epilepsy in which seizures arise in a well-defined area of the brain. It is these epilepsy patients CombiGene intends to help with its candidate drug, CG01.

Enormous potential for CombiGene

It is estimated that some 47,000 patients are diagnosed with drug-resistant focal epilepsy each year in the USA, EU5, Japan and China. CombiGene estimates that, realistically, 10–20 percent of these patients could be treated with the company's candidate drug, CG01. Assuming that the treatment cost per patient could amount to somewhere between 134,000 and USD 200,000 (which is low in comparison with approved gene-therapy drugs), annual sales could be as much as between USD 750 and 1,500 million.

Comments from our Chief Research and Development Officer

CG01 Update

During the first half of 2020 and the beginning of the third quarter further milestones were achieved in the CG01 project in terms of both knowledge acquisition via two concluded preclinical studies and with respect to development of our production method. The following is a summary of the most significant advances.

Key events second quarter 2020

- During the second quarter, our production partner Cobra achieved an important milestone in the CG01 project through the delivery of the first DNA plasmid.

Important events at the start of the third quarter of the year

- **Final delivery of plasmids.** At the beginning of the third quarter of the year, our production partner Cobra completed production and delivery of the two remaining DNA plasmids.
- **Master cell banks.** Cobra has also developed three GMP master cell banks. The master cell banks guarantee that the quality and characteristics of the plasmids are exactly the same at each individual manufacturing run for all future productions of the three plasmids.



*Karin Agerman
Chief Research and Development Officer*

The master cell banks can thus be used every time CombiGene needs to be manufacturing new plasmids for the production of CG01 for future clinical studies and for commercial production.



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

- **Pilot study of suspension method for the manufacture of CG01.** At the beginning of August, CombiGene completed a successful pilot production of CG01 together with Spanish CDMO manufacturer Viralgen, which offers a suspension method for the production of AAV vectors. The development of suspension methods has improved rapidly in recent years and offers great advantages in scaling up production.
- **Tropism study.** The brain is made up of neurons and glial cells. A unique feature of viruses is that they have so-called tropism, which means that they specialize in selectively infecting certain types of cells. Our tropism study shows that CG01 is only taken up by the nerve cells in the hippocampus and not the glial cells. This knowledge verifies our understanding of how CG01 works and it also provides positive answers to questions put to us by the authorities.

CG01 – milestones

2016

- First screening study conducted.
- Selection of a candidate drug.

2017

- Data from the dose-response study indicate a dose-dependent anti-epileptic effect.
- The proof-of-concept in a chronic epilepsy model is initiated.
- Studies in human epileptic brain tissue from patients with pharmaco-resistant epilepsy confirm that CG01 is expressed in human cells.

2018

- Final data from the preclinical proof-of-concept study confirm positive treatment results in the form of significantly fewer and shorter seizures.
- CombiGene enters into collaboration with British Cell and Gene Therapy Catapult to develop a GMP manufacturing method for CG01.
- Horizon 2020, the EU framework program for research and development, allocates 3.36 million euros for the development and commercialization of CG01.

2019

- Acquisition of Panion Animal Health gives CombiGene full control over the company's intangible assets in the CG01 project.
- Agreement with CRO Northern Biomedical Research (NBR), which specializes in preclinical studies in the central nervous system (CNS). The agreement covers assessment of the candidate drug, CG01, in a smaller pilot study, a biodistribution study and a safety study, a so-called toxicity study.
- Agreement with the CDMO, Cobra Biologics, for GMP manufacturing of CG01 for clinical studies and future commercial production.

2020

- Preclinical pharmacokinetic study completed with positive results. The study confirms that CG01 creates long-term expression of the active substances NPY and Y2.
- The preclinical learning and memory study show that NPY and Y2 have no significant negative effect on cognitive functions.
- Delivery of all three plasmids needed to produce CG01.
- Delivery of master cell banks for the three plasmids
- Successful pilot study performed with suspension production method
- Positive results in tropism study



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The global market for the candidate drug, CGT2, is estimated at USD 700 – 1,450 million



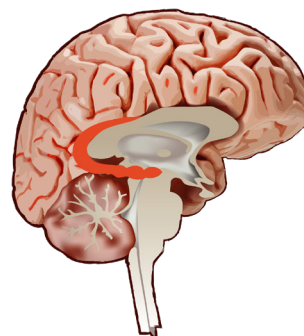
With the lipodystrophy project, which was incensed from Lipigon Pharmaceuticals AB (Lipigon) in autumn 2019, CombiGene has expanded its operations to include metabolic disorders. The initial aim of the project is to develop a gene-therapy treatment for partial lipodystrophy, a very rare condition for which there is currently no adequate treatment. The project is in an early stage.

Partial lipodystrophy is a very rare disorder for which there is currently no effective treatment . It is estimated that there are about 500 patients in the USA and 300 patients in the EU, and that the patient population is expected to grow by under four percent per year. Assuming that CGT2 will be used to treat between 25 and 50 percent of the patients and that the treatment per patient costs USD 1.5 million in the USA and USD 1.3 million in Europe, the total sales potential is 700 – USD 1,450 million.

A group of patients with lipodystrophy lack a hormone called leptin. This group of patients cannot be treated with CGT2, but there is a medical treatment which costs USD 850,000 per patient per year in the USA, indicating that there is a high willingness to pay for treating this type of disease.

Collaboration partner

Lipigon was founded 2010 by leading lipid experts from Umeå University, Sweden, primarily acting as an IP holding company. From 2016 the company expanded by employing in-house scientists and establishing its own laboratories. Today Lipigon has offices and laboratories in Umeå, Sweden and at the BioVenture Hub in Gothenburg.



A few words from our Senior Project Manager

CGT2 Update

CGT2, CombiGene's project to develop a treatment of partial lipodystrophy, is now in an early preclinical discovery phase. Since we inlicensed the project from Lipigon in 2019 the pace in the project has accelerated, and we are now seeing the first results of this work.

The first step in designing gene therapy vectors and testing these in vitro (tests on different liver cells) has been carried out with good results. Planning for the project's next stage of in vivo studies has been ongoing during the summer.



*Annika Ericsson
Senior Project Manager*

Lipodystrophy project – milestones

2019

- In-licensing of the project from Lipigon.

2020

- Design of expression plasmids, which are a starting material for gene-therapeutic vectors CombiGene intends to develop for treatment of partial lipodystrophy.
- In vitro studies (tests on liver cells) show proper protein expression.



Gene therapy attracts great interest

Gene therapy is currently one of the most exciting areas of pharmaceuticals development. At the close of 2019, 352 clinical studies were under way, of which 32 were phase III, the final clinical phase before market approval. The focus of the clinical studies is in oncological diseases, cardiovascular diseases and diseases related to the central nervous system. Investment in gene therapy amounted during 2019 to USD 7.6 billion.

The great interest in gene therapy, from both researchers and investors, is explained by the unique advantages that gene therapy offers. First of all, gene therapy can potentially treat diseases for which adequate treatment methods are lacking currently. Secondly, after only one or a few treatments, gene therapy can have a long-term and, possibly, lifelong effect, as compared to conventional drugs, which often must be taken several times daily for the rest of the patient's life.

That gene therapy is one of the most interesting areas of pharmaceutical development is also confirmed by successes in recent years in the USA. On 30 August 2017 the US Food and Drug Administration (FDA) approved the first gene-therapeutic drug for the US market. By the close of 2019 four gene therapies had been approved in both the USA and the EU. In addition, three other products have been approved in the EU and one in the USA. The FDA has also demonstrated great confidence in gene therapy by simplifying the regulatory framework for this type of drug. CombiGene is of the view that the number of approved gene therapies will grow quickly in the coming years and become established treatment alternatives in a range of disease areas.



Financial information

Income and earnings

The Group has a total of SEK 0 (0) thousand during the period January-June. Other operating income amounts to SEK 6,219 thousand (4,772), of which 5,241 (4,410) KSEK refers to the revenue-earned portion of the grant received from Horizon 2020. Operating profit for the period amounted to -12,055 (-5,285) KSEK. The main costs during the period have been related to research & development, fees for consultants and personnel costs.

Cash flow and financial position

Cash flow for the period January-June amounts to SEK 2,861 thousand. Liquidity at the end of the period amounts to SEK 18,026 thousand. The equity ratio is 70.98%.

Liquidity and financing

The total horizon 2020 allocation amounts to EUR 3.36 million of which EUR 2.7 million so far has been paid to the Company. The Board of Directors and management are continuously evaluating options to ensure the company's financing in the short and medium term. On November 11, 2019, CombiGene took out a loan of SEK 7 million and on 27 March 2020 CombiGene increased the loan of an additional SEK 5 million. On May 7, 2020, CombiGene repaid SEK 9 million of the loan. Left is a loan of SEK 3 million and lenders are Modelio Equity AB and Munkekullen 5 förvaltning AB. As of January 1, 2020, lenders have the right to call for directed, on part or all of the loan, set-off issues of shares in the company, continuously at a 10% discount in relation to the reference price. The reference price is the average volume-weighted price (VWAP) over the last 20 (20) trading days. The reference price can be at least SEK 1.10. If the lender calls for directed set-off issues of shares in the Company, the Board of Directors is obliged to exercise its authorization to issue shares accordingly.

On February 18, 2020, the Board of Directors decided to issue a rights issue of units consisting of shares and warrants. The final result of the preferential issue showed that units for a total of SEK 13.44 million were subscribed for preferential rights, corresponding to 41.3 percent of the rights issue. Additional expressions of interest for subscription of units without the support of unit rights were added of SEK 12.84 million. As a result, units were subscribed for a total of SEK 26.28 million, corresponding to a subscription rate of 80.81 percent. The company was paid SEK 26.28 million before issue costs through the rights issue. Issue costs amounted to SEK 4.7 million, of which approximately SEK 3 million was offset against newly issued shares to the guarantors. The subscription price per share was SEK 0.50 and the warrants were issued free of charge. A total of 52,568,360 shares, 26,284,180 series TO3 warrants and 26,284,180 series TO4 warrants were issued in the rights issue.

On April 24, 2020, the Board of Directors decided to carry out a directed issue of units consisting of shares and warrants totaling SEK 4 million to Modelio Equity AB (publ) and Oscar Molse. The directed issue was conducted on essentially the same terms as the recently completed rights issue, meaning that Modelio and Oscar Molse, with half each, subscribed for a total of 4,000,000 units consisting of a total of 8,000,000 shares, 4,000,000 series TO3 subscription and 4,000,000 series TO4 warrants. The subscription price per share was SEK 0.50 and the warrants were issued free of charge. CombiGene was initially paid EUR 4 million through the issue of the shares, before issue costs of approximately SEK 0.1 million.

A total of 30,284,180 series TO3 warrants and 30,284,180 30 warrants by TO4 have been issued. Series TO3 warrants may be exercised for subscription of shares during the period 17 August 2020 – 31 August 2020 at a subscription price corresponding to 10 days volume-weighted average price (VWAP) for the period 3 August 2020 – 14 August 2020 with a discount of 30 percent, but not less than SEK 0.4 per cent and a maximum of SEK 0.4 SEK 0.6 per new share. Series TO4 warrants may be exercised for subscription of shares during the period 16 November 2020 to 30 November 2020 at a subscription price corresponding to 10 days volume-weighted average price for

the period 2 November 2020 – 13 November 2020, with a discount of 30 percent, but not less than SEK 0.5 and a maximum of SEK 0.7 per new share. CombiGene may therefore be paid a maximum of SEK 39.4 million upon full exercise of all issued series TO3 and series TO4 warrants. The warrants are subject to customary conversion terms.

On August 4, the Board of Directors of CombiGene AB (publ) decided to carry out a directed share issue of 21,588,125 units, each consisting of one share and one warrant of series TO5, at a price of 72 cents per unit, corresponding to an initial investment of EUR 1.5 million or approximately SEK 15.5 million, kronor, before issue costs, to the Dutch investment company NYIP ("Nyenburgh Holding BV") a leading Dutch life-science investor. The series TO5 warrants are issued free of charge and have the same terms and conditions as the Company's series TO4 warrants, meaning that the series TO5 warrants may be exercised for subscription of shares during the period 16 November 2020 – 30 November 2020 at a subscription price corresponding to 10 days volume-weighted average price for the period November 2, 2020 – 13 November 2020, with a discount of 30 percent, but not less than SEK 0.5 and a maximum of SEK 0.7 per new share. Upon full exercise of all series TO 5 warrants, the Company will be charged a maximum of approximately SEK 15 million, before issue costs.

The Board of Directors is of the opinion that the above issues together with the warrants that may be exercised in August and November respectively constitute sufficient capital to operate the business in 2020. Should the warrants not provide sufficient capital, the business plan can be adjusted, which will affect the projects.

The share

The number of shares at the end of the period was 125,622,007 with a quota value of SEK 0.10. The average number of the aktier period is 109,696,581. All shares are of the same type and have the same voting rights.

Staff

The number of employees in the Group at the end of the period was 3 (3), of whom 2 (2) are women. In addition, there was an administrative resource and a Chief Financial Officer who was hired as 2 (2) consultants, of whom 2 (2) were women.

Risks and uncertainties

A drug development company of CombiGenes type is characterized by a high operational and financial risk. There are many factors that can negatively affect the likelihood of commercial success. The risks, which at the company's current stage is considered most important to consider, is the risk that CombiGene's method is not safe or not effective, and the risk that the business may not receive the necessary financing. During the current period, significant changes in these risk or uncertainty factors have occurred.

Principles for preparation of the interim report

CombiGene prepares its financial reports in accordance with the Swedish Annual Accounts Act and BFNAR 2012:1 (K3) Annual Accounts and Consolidated Accounts. The same accounting principles have been applied in this interim report as were applied in the most recent annual report.

Review by auditors

This report has not been subject to review by the company's auditors.

Future financial report

Interim report January - September 2020, 13 November 2020.

For further information, please contact:

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Lund, 21 August 2020, CombiGene AB (publ)

Bert Junno
Chairman of the board

Jonas Ekblom
Board member

Jan Nilsson
Board member and CEO

Per Lundin
Board member

Peter Nilsson
Board member

Group income statement in summary

Figures in TSEK	2020 Apr-Jun	2019 Apr-Jun	2020 Jan-Jun	2019 Jan-Jun	2019 Jan-Dec
Nets sales	0	0	0	0	0
Other operating revenues	2 161	3 137	6 219	4 772	15 730
Operating expenses					
Other external expenses	-3 451	-4 515	-12 012	-7 155	-25 263
Personnel expenses	-1 571	-1 436	-3 033	-2 902	-6 165
Other operating expenses	-119		-731		-825
Profit/loss before depreciation	-2 980	-2 814	-9 556	-5 285	-16 524
Depreciation	-611	0	-1 222	0	-1 166
Profit/loss after depreciation	-3 591	-2 814	-10 779	-5 285	-17 690
Net financial income/expense	-798	0	-1 276	0	-238
Income after net financial items	-4 389	-2 814	-12 055	-5 285	-17 929
Tax	0	0	0	0	0
Net profit/loss for the period	-4 389	-2 814	-12 055	-5 285	-17 929
Attributable to					
Parent company shareholders	-4 303	-2 814	-11 886	-5 285	-17 602
Non-controlling interests	-86	0	-169	0	-326
Earnings per share before dilution	-0,03	-0,05	-0,10	-0,10	-0,28
Earnings per share after dilution	-0,03	-0,05	-0,10	-0,10	-0,28
Average number of shares before dilution	100 748 665	51 593 476	109 696 581	51 593 476	57 543 838
Average number of shares after dilution	100 748 665	51 593 476	109 696 581	51 593 476	57 543 838
Total outstanding shares	125 622 007	51 593 476	125 622 007	51 593 476	65 053 647

Group balance sheet in summary

Figures in TSEK	2020 30 Jun	2019 30 Jun	2019 31 Dec
ASSETS			
Intangible assets	23 714	1 842	24 936
Total fixed assets	23 714	1 842	24 936
Current assets			
Other receivables	4 822	1 939	3 716
Cash and bank balances	18 026	21 839	15 166
Total current assets	22 847	23 778	18 882
Total assets	46 561	25 619	43 818
SHAREHOLDERS' EQUITY AND LIABILITIES			
Share capital	12 562	5 159	6 505
Other capital contribution	88 094	54 590	69 348
Other shareholders' equity	-57 393	-39 295	-39 787
Profit/loss for the year	-11 886	-5 285	-17 602
Equity attributable to parent company shareholders	31 377	15 170	18 464
Minority interest	1 672	0	1 840
Total shareholders' equity	33 048	15 170	20 304
Liabilities			
Current liabilities	13 513	10 450	23 514
Total liabilities	13 513	10 450	23 514
Total shareholders' equity and liabilities	46 561	25 619	43 818

Summary report of changes in the Group's shareholders' equity

Figures in TSEK	Share capital	Other capital contribution	Accumulated profit/loss	Non-controlling interests	Total shareholders
Balance brought forward	6 505	69 348	-57 389	1 840	20 304
Issue	6 057	24 227			30 284
Issue costs		-5 482			-5 482
Minority acquisition with non-controlling interests			-4		-4
Net profit/loss for the period			-11 886	-169	-12 055
Amount as per the end of the reporting period	12 562	88 094	-69 279	1 671	33 048

Group cash flow statement in summary

Figures in TSEK	2020	2019	2019
	Jan-Jun	Jan-Jun	Jan-Dec
Cash flow from operating activities	-17 938	-9 778	-21 605
Cash flow from investing activities	-4	-188	-1 521
Cash flow from financing activities	20 803	0	6 487
Cash flow for the period	2 861	-9 966	-16 639
Liquid assets at the beginning of the reporting period	15 165	31 805	31 805
Liquid assets at the end of the reporting period	18 026	21 839	15 165

Parent company income statement in summary

Figures in TSEK	2020	2019	2020	2019	2019
	Apr-Jun	Apr-Jun	Jan-Jun	Jan-Jun	Jan-Dec
Nets sales	0	0	0	0	0
Other operating revenues	2 161	3 137	6 219	4 772	15 730
Operating expenses					
Other external expenses	-3 462	-4 508	-11 595	-7 139	-23 732
Personnel expenses	-1 571	-1 435	-3 033	-2 902	-6 064
Other operating expenses	-119	0	-731	0	-825
Profit/loss before depreciation	-2 991	-2 806	-9 140	-5 269	-14 891
Depreciation	-38	0	-75	0	-19
Profit/loss after depreciation	-3 028	-2 806	-9 215	-5 269	-14 910
Net financial income/expense	-316	0	-794	0	-181
Income after net financial items	-3 344	-2 806	-10 009	-5 269	-15 091
Tax	0	0	0	0	0
Net profit/loss for the period	-3 344	-2 806	-10 009	-5 269	-15 091

Parent Company balance sheet in summary

Figures in TSEK	2020 30 Jun	2019 30 Jun	2019 31 Dec
ASSETS			
Intangible assets	3 060	1 842	3 135
Financial assets	23 467	167	23 463
Total fixed assets	26 527	2 009	26 598
Current assets			
Other receivables	5 376	1 970	3 684
Cash and bank balances	17 838	21 660	14 959
Total current assets	23 214	23 630	18 643
Total assets	49 741	25 639	45 241
SHAREHOLDERS' EQUITY AND LIABILITIES			
Restricted equity			
Share capital	12 562	5 159	6 505
Statutory reserve	4	4	4
Reserve for development expenses	508	695	508
Non- restricted equity			
Share premium reserve	68 001	34 501	49 255
Accumulated loss including profit/loss for the year	-44 796	-25 154	-34 787
Total shareholders' equity	36 278	15 205	21 484
Liabilities			
Current liabilities	13 463	10 435	23 757
Total liabilities	13 463	10 435	23 757
Total shareholders' equity and liabilities	49 741	25 639	45 241

Summary report of changes in the Parent Company's shareholders' equity

Amounts in TSEK	Share capital	Statutory reserve	Reserve for development expenses	Share premium reserve	Accumulated profit/loss	Total shareholders' equity
Balance brought forward	6 505	4	507	49 255	-34 788	21 484
Issue	6 057			24 227		30 284
Issue costs				-5 482		-5 482
Net profit/loss for the period					-10 009	-10 009
Amount as per the end of the reporting period	12 562	4	507	68 001	-44 796	36 278

Parent Company cash flow statement in summary

Figures in TSEK	2020	2019	2019
	Jan-Jun	Jan-Jun	Jan-Dec
Cash flow from operating activities	-17 920	-9 777	-14 971
Cash flow from investing activities	-4	-188	-8 706
Cash flow from financing activities	20 803	0	7 011
Cash flow for the period	2 878	-9 965	-16 665
Liquid assets at the beginning of the reporting period	14 959	31 625	31 625
Liquid assets at the end of the reporting period	17 838	21 660	14 959

Group financial key ratios

Figures in TSEK	2020	2019	2019
	Jan-Jun	Jan-Jun	Jan-Dec
Earnings per share before dilution SEK	-0,10	-0,10	-0,28
Earnings per share after dilution SEK	-0,10	-0,10	-0,28
Shareholders' equity per share, SEK	0,26	0,29	0,31
Equity/assets ratio, %	70,98	59,35	46,34
Average number of shares before dilution	109 696 581	51 593 476	57 543 838
Average number of shares after dilution	109 696 581	51 593 476	57 543 838
Total outstanding shares	125 622 007	51 593 476	65 053 647

Share capital development

Year	Event	Total share-capital (SEK)	Change (SEK)	Total shares	Change shares	Quotient (SEK)
1990	Company registration	50 000	50 000	500	500	100,00
1997	Bonus issue	100 000	50 000	1 000	500	100,00
2010	New share issue	102 600	2 600	1 026	26	100,00
2013	New share issue	143 600	41 000	1 436	410	100,00
2014	Bonus issue	574 400	430 800	5 744	4 308	100,00
2014	New share issue	604 400	30 000	6 044	300	100,00
2014	Split 1 000:1	604 400	0	6 044 000	6 037 956	0,10
2014	New share issue	884 400	280 000	8 844 000	2 800 000	0,10
2015	New share issue	1 134 400	250 000	11 344 000	2 500 000	0,10
2015	New share issue	1 138 197	3 797	11 381 970	37 970	0,10
2016	New share issue	1 180 159	41 962	11 801 590	419 620	0,10
2017	New share issue	1 652 223	472 064	16 522 230	4 720 637	0,10
2018	New share issue	1 719 783	67 560	17 197 836	675 596	0,10
2018	New share issue	5 159 348	3 439 565	51 593 476	34 395 650	0,10
2019	New share issue	6 372 384	1 213 036	63 723 836	12 130 360	0,10
2019	New share issue	6 373 090	706	63 730 896	7 060	0,10
2019	New share issue	6 505 365	132 275	65 053 647	1 322 751	0,10
2020	New share issue	11 762 201	5 256 836	117 622 007	52 568 360	0,10
2020	New share issue	12 562 201	800 000	125 622 007	8 000 000	0,10
At the end of the period		12 562 200,7		125 622 007		0,10

Glossary

Biodistribution study. In vivo test-article distribution or localization studies performed in selected nonclinical species to support early biotherapeutic drug development.

Candidate drug. An unapproved drug that is under development.

Cardiovascular diseases. A group of disorders of the heart and blood vessels.

CDMO. Contract Development and Manufacturing Organisation.

CG01. CombiGene's epilepsy project.

CGT2. CombiGene's project for treatment of partial lipodystrophy.

Preclinical study. Studies or trials in healthy subjects or participants with an illness to study the effects of a drug or treatment method. Clinical studies are divided into different phases. These are called Phase I, Phase II, Phase III and Phase IV.

CNS. Central nervous system.

CRO. A company that specializes in preclinical and/or clinical studies.

Dose-response study. A study describing how the body responds to a specific concentration of a drug.

Drug resistance. Reduction in effectiveness or non-effectiveness of a medication.

Engineering Run. Process demonstration of some or all of the manufacturing process steps.

EU5. France, Italy, Spain, UK and Germany.

FDA. US Food and Drug Administration. The agency of the USA that regulates and supervises food, tobacco, drugs, cosmetics, medical devices, vaccines, biopharmaceuticals, radiation emitting devices, and veterinary products.

Gene therapy. A form of treatment of certain conditions whereby one or several new genes are introduced into the cells of an organism.

GMP. (Good Manufacturing Practice) Regulatory framework controlling the manufacture of pharmaceuticals.

Horizon 2020. The EU's framework programme for research and development.

Lipodystrophy. A disorder characterized by abnormal fat distribution in the body.

Metabolic disorders. Diseases or disorders that disrupt normal metabolism.

Oncological diseases. Cancer diseases.

Preclinical studies. Studies of a drug that are conducted before studies in humans can begin.

Proof-of-concept study. A study that covers all important functions with an aim to demonstrating that the concept functions as intended.

Screening study. Medical studies in a population to look for as-yet-unrecognised conditions or risk markers in individuals without signs or symptoms.

Toxicity study. Study of possible toxicity in humans.

Tropism. Describes the ability of viruses to selectively infect certain types of cells.



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

CombiGene – The gene therapy explorer

With one project nearing the clinical-study phase and one project in an early preclinical phase, CombiGene is the leading Nordic gene-therapy company. Gene therapy has seen rapid development in recent years, with a number of approved therapies and several major corporate deals. During this period we've built up a unique position with respect to knowledge within this field in the Nordic region. The company's expertise covers all central areas of the gene therapy field: viral vectors, preclinical studies including biodistribution and toxicity studies, development of GMP-classed manufacturing methods, upscaling of production volumes and regulatory strategy.

Few areas of pharmaceutical development are as exciting and promising as gene therapy and, in many respects, CombiGene is at the very forefront of development. During our work with the CG01 epilepsy project, on a nearly daily basis, we have won new ground, gained new insights and expanded our knowledge. You might say that we are on an expedition, exploring the fantastic possibilities of gene therapy. We are now continuing our voyage of discovery with another exciting project – the lipodystrophy project. Even here, we expect to create new and valuable knowledge as we carry this project forward.

And that's why we've chosen to call ourselves the gene therapy explorer.



 **combiGene**

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