

*"During the first quarter of the year, CombiGene strengthened itself financially through a fully guaranteed rights issue of SEK 75 million in order to start the final and cost-intensive part of the preclinical phase of CG01 in accordance with plan. We have also strengthened the company's expertise and capacity regarding production, clinical studies, and gene therapy through three important new recruits."*

Jan Nilsson  
CEO

## Interim Report

January – March 2021 for CombiGene AB (publ)

# Interim report January – March 2021 for CombiGene AB (publ)

## Period January - March 2021

- Net sales: 0 (0) TSEK.
- Other operating revenues: 5 118 (4 058) TSEK.
- Profit from financial items: -10 856 (-7 583) TSEK.
- Earnings per share: -0,05 (-0,12) SEK.
- Liquidity as per the end of the reporting period: 34 091 (9 545) TSEK.
- Equity ratio as per the end of the reporting period: 87,87 (32,97)%.

## Events during the period

- CombiGene's Board of Directors resolves on a fully guaranteed rights issue of approximately SEK 75 million.
- Response from the Swedish and UK pharmaceutical authorities confirms CombiGene's plan for CG01's final preclinical studies.
- The material from the first large-scale production of CG01 is released for use in the final parts of the preclinical program.
- The CG01 project initiates preclinical biodistribution and toxicology studies.
- CombiGene's lipodystrophy project is awarded EUR 882,500 in development grants by the EU Eurostars program.

## Events after the end of the period

- CombiGene completes the fully guaranteed rights issue, and the company receives approximately SEK 75 million.
- Notice to attend the Annual General Meeting of CombiGene AB.



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



# Let's take the final steps towards the first human study

*During the first quarter of the year, CombiGene strengthened itself financially through a fully guaranteed rights issue of SEK 75 million in order to start the final and cost-intensive part of the preclinical phase of CGO1 in accordance with plan. We have also strengthened the company's expertise and capacity regarding production, clinical studies, and gene therapy through three important new recruits. CombiGene is thus a stronger company than ever before, and we will use this position to take the final decisive steps towards the first in human study in our epilepsy project CGO1 – a historic and value-creating milestone for CombiGene.*

## CGO1 continues to advance its positions

We are now pursuing the development of CGO1 along three tracks: final preclinical studies, GMP production of CGO1 and preparation of the first in human study. The work is proceeding according to plan in all areas.

**Preclinical studies.** During the quarter, the CGO1 material to be used in the final preclinical studies was released. After receiving confirmatory responses from the pharmaceutical authorities in Sweden and the UK, we were thus able to initiate toxicology and biodistribution studies in small animals. During the quarter, we also started work on selecting injection device for the administration of CGO1 and defining exactly how this administration should be carried out. During the year, we will start the final toxicology and biodistribution study in large animals.

**Production.** The GMP production of our drug candidate CGO1 will be carried out in the second half of 2021 and will be accompanied by customary analysis and quality assurance work before the drug candidate is released for use in early 2022.

### Preparation of the first study in humans.

The preparation of our first clinical study is also progressing according to plan. We are currently working on choosing which CRO company to work with and at which clinics the study will be conducted. The next steps include designing of the study together with our scientific committee, our CRO partner and clinicians, and thereafter presenting our study design to the relevant pharmaceutical authorities.

## Application for clinical trial permit 2022

Our plan is that all parts of the preclinical studies, GMP production and study preparations will be in place in 2022. We will then submit a clinical trial application (CTA) with the ambition to start the first human study in the second half of 2022.

## The EU continues to invest in CombiGene - allocating EUR 882 500 to the development of the lipodystrophy project CGT2

CombiGene's epilepsy project CGO1 has already been granted EUR 3.36 million by the EU program Horizon 2020. Now the EU is also choosing to invest in our lipodystrophy project CGT2 through the Eurostars program. This program is aimed at small and medium-sized development companies which intend to make use of the great benefits that international cooperation can bring. In other words, an excellent description of CombiGene.

Eurostars is the largest international financing program for SMEs that want to collaborate on R&D. Competition is high and grants are only allocated to projects that score high up in the rankings.

Thanks to the Eurostars grant, we have been able to broaden the international cooperation in this important project to include The University Medical Center Hamburg-Eppendorf and the CRO company Accelero. The project allocation is allocated as follows: CombiGene is allocated EUR 481,000, Hamburg-Eppendorf EUR 265,000 and Accelero EUR 136,500.

Finally, I would like to thank all of you who follow and support CombiGene,

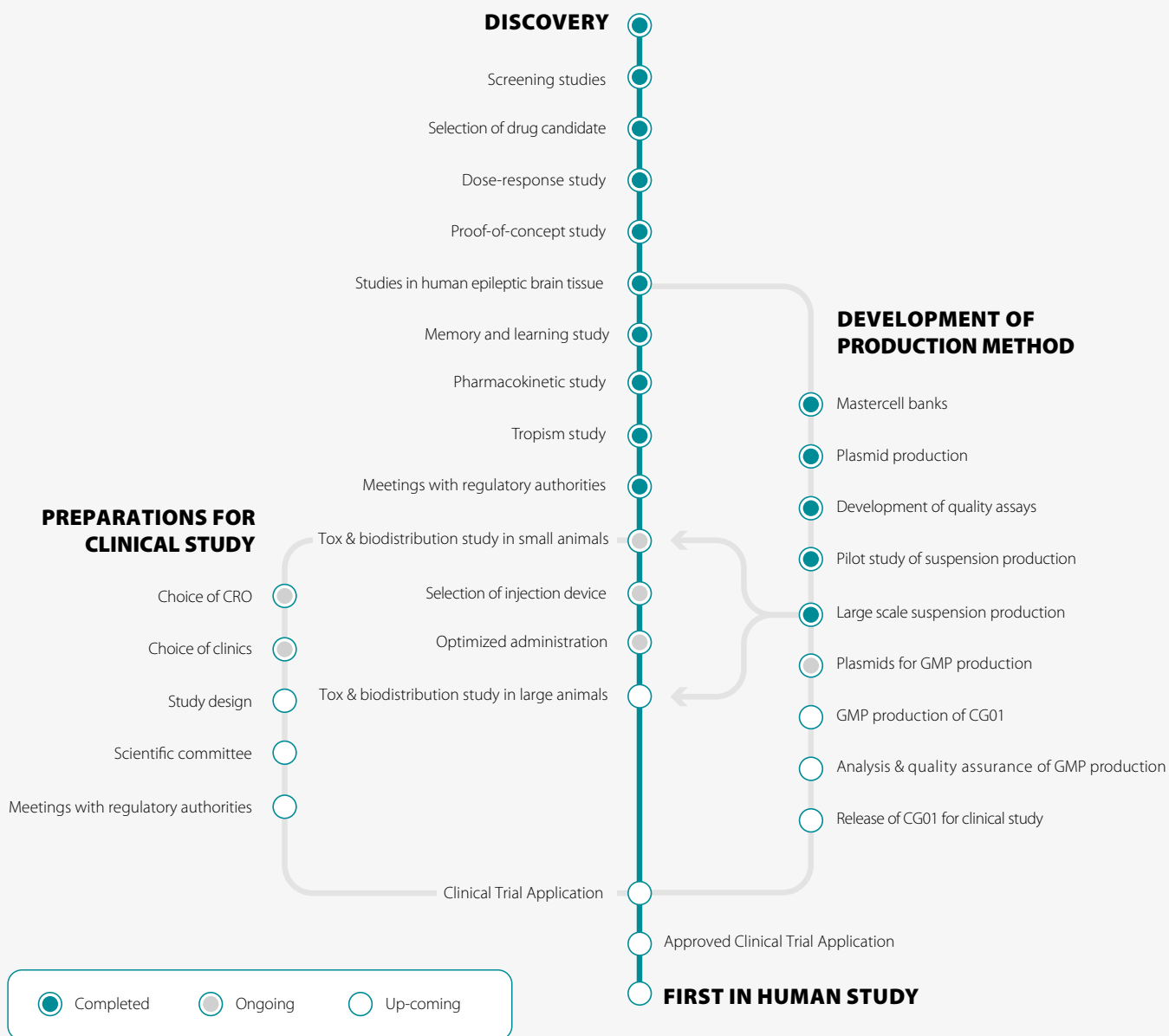
Jan Nilsson  
CEO



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

# CG01 roadmap

The illustration shows how CombiGene's epilepsy project CG01 is approaching the first IN human study, scheduled to begin in 2022.



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

# The global annual market for the candidate drug, CG01, is estimated to 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 had the diagnose 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, the five largest countries in Europe, 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 reach between USD 750 and 1,500 million.

## Comments from our Chief Research & Development Officer

### CG01 Update

CombiGene's epilepsy project CG01 continued to perform well in the quarter. The material we produced for our pre-clinical studies was released in March after customary analysis and quality assurance. Thus, we were able to initiate the first of two toxicology and biodistribution studies. During the quarter, we also started work on selecting the injection device and optimizing administration. The administration itself is extremely important because it is about reaching a very carefully defined area of the brain with a very precise dose of our drug. As regards the preparation of the first clinical study, we are currently evaluating which clinical research organisation to employ and at which clinics the study will be conducted.

*Karin Agerman*  
Chief Research & Development Officer



*Karin Agerman*  
Chief Research and Development Officer



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

## 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 study 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 EUR 3.36 million 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.
- CombiGene signs an agreement with the CDMO, Cobra Biologics, regarding production of plasmids for GMP manufacturing of CG01.

### 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 shows 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.
- Agreement on GMP production with Cobra for plasmids for CG01.
- Analytical methods for quality control of the production of CG01 developed in collaboration with CGT Catapult.
- Manufacturing agreement with Viralgen for the production of CG01.
- Agreement with the British company Neurochase regarding the development of optimized administration of CG01.
- First large-scale production of CG01 at the Spanish gene therapy manufacturer Viralgen.

### 2021

- The material from the first large-scale production of CG01 released for use in the final parts of the preclinical program.
- The CG01 project initiates preclinical biodistribution and toxicology studies in small animals.



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



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

*With the lipodystrophy project, which was inlicensed 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 (CGT2) for partial lipodystrophy. The project is in an early development 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 just 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 USD 700 – 1,450 million.

Another group of patients with lipodystrophy lack a hormone called leptin. This group of patients will initially not be treated with CGT2. These patients are currently treated with a costing of USD 850,000 per patient per year in the USA, indicating what the healthcare system will pay for treating this type of disease.



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



# A few words from our Preclinical Project Manager

## CGT2 Update

CGT2, CombiGenes project to develop a gene therapy treatment of partial lipodystrophy is in early preclinical development. Since we licensed the project from Lipigon 2019, the pace of the project has accelerated, and we are now beginning to see that this work has begun to bear fruit.

The first step in designing gene therapy vectors and testing these in vitro (tests on different liver cells) has been completed with good results. Since then, in vivo studies have begun.

## Key events in the first quarter 2021

The most important news of the quarter is undoubtedly that our lipodystrophy project is awarded EUR 882,500 by the EU's Eurostars program to, together with the University Medical Center Hamburg-Eppendorf and the CRO company Accelerero, drive the project forward. The grant itself is important because it allows us to continue our work with full force. I also see our high ranking among the companies that have applied for funding from the Eurostars program as



Annika Ericsson  
Preclinical Project Manager

confirmation of the scientific strength of the project and CombiGene's ability to conduct effective and successful development work in collaboration with external partners. The project grant is allocated as follows: CombiGene receives EUR 481,000, Hamburg-Eppendorf EUR 265,000 and Accelerero EUR 136,500.

Annika Ericsson  
Preclinical 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.

- Priority-based patent application filed with the UK Patent Office.
- In vivo studies initiated for evaluation of the different gene therapy vectors.

## 2021

- The lipodystrophy projects receive EUR 882 500 in development grants from the EU Eurostars program.



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

# Gene therapy attracts great interest

Gene therapy is one of the most dynamic areas in today's drug development. At the end of 2020, 423 clinical studies were underway, of which 72 were in Phase III, the last clinical phase prior to market approval. The focus of the clinical studies is on oncological diseases, cardiovascular diseases and diseases related to the central nervous system. Investments in gene therapy increased by 70 percent in 2020. It is in this dynamic landscape that CombiGene operates.

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

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### Income and earnings

The Group has a total net sale of SEK 0 (0) thousand during the period January-March. Other operating revenues amounts to SEK 5,118 (4,058) thousand, of which SEK 4,586 (3,608) thousand refers to the revenue-earned portion of the grant received from Horizon 2020 and SEK 217 (0) thousand refers to the revenue-earned portion of the grant received from Eurostars. Operating profit for the period amounted to SEK -10,856 (-7,188) thousand. 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-March amounts to SEK -14 804 TSEK. Liquidity at the end of the period amounts to 34 091 TSEK. The equity ratio is 87,9%.

### Liquidity and financing

The total Horizon 2020 grant amounts to EUR 3,36 million of which EUR 2,9 million so far has been paid to the Company. The EU's Eurostars program, which is aimed at small and medium-sized enterprises wishing to collaborate on research and development projects, has allocated development grants to the CGT2 project. The total grant for CombiGene amounts to SEK 5 million, of which SEK 0.25 million has so far been paid. out.

In March / April 2021, a guaranteed rights issue of shares was carried out. The rights issue provided the company with approximately SEK 75 million before issue costs. The company's share capital increased by SEK 16,674,692.6, from SEK 22,927,702.4 to SEK 39,602,395. The number of shares increased by 166,746,926 shares, from 229,277,024 shares to 396,023,950 shares.

The board and company management continuously evaluate alternatives to ensure the company's financing in the short and medium term.

### The share

The number of shares at the end of the period was 229,277,024 with a quota value of SEK 0,10. The average number of shares for the period is 229,277,024. All shares are of the same type and have the same voting rights.

### Lund, 12 May 2021, CombiGene AB (publ)

Bert Junno  
Chairman

Jonas Ekblom  
Board member

Jan Nilsson  
Board member and CEO

Per Lundin  
Board member

Peter Nilsson  
Board member

### Employees

The number of employees in the Group at the end of the period was 7 (3), of whom 4 (2) are women. In addition, there was an administrative resource who was hired as 1 (2) consultants, of whom 1 (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.

### AGM and Annual Report

The Annual General Meeting of Shareholders for 2021 will be held in Lund on 25 May. The Annual Report will be available to the public at the company's office in Lund and will be published on Nasdaq's website no later than 3 weeks prior the the AGM.

### Review by auditors

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

### Future reporting date

Interim report January – June 2021, 20 August 2021.

### For further information, please contact:

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Tel: +46 (0) 46-275 60 10  
Email: jan.nilsson@combigene.com

## Group income statement in summary

| Figures in TSEK                          | 2021<br>Jan-Mar | 2020<br>Jan-Mar | 2020<br>Jan-Dec | 2019<br>Jan-Dec |
|--|-----------------|-----------------|-----------------|-----------------|
| Net sales                                | 0               | 0               | 0               | 0               |
| Other operating revenues                 | 5 118           | 4 058           | 12 029          | 15 730          |
| <b>Operating expenses</b>                |                 |                 |                 |                 |
| Other external expenses                  | -12 631         | -8 561          | -29 640         | -25 263         |
| Personnel expenses                       | -2 463          | -1 462          | -7 185          | -6 165          |
| Other operating expenses                 | -231            | -612            | -869            | -827            |
| <b>Profit/loss before depreciation</b>   | <b>-10 207</b>  | <b>-6 576</b>   | <b>-25 665</b>  | <b>-16 525</b>  |
| Depreciation                             | -649            | -611            | -2 495          | -1 166          |
| <b>Profit/loss after depreciation</b>    | <b>-10 856</b>  | <b>-7 188</b>   | <b>-28 159</b>  | <b>-17 692</b>  |
| Net financial income/expense             | 0               | -478            | -1 392          | -237            |
| <b>Income after net financial items</b>  | <b>-10 856</b>  | <b>-7 665</b>   | <b>-29 551</b>  | <b>-17 929</b>  |
| Tax                                      | 0               | 0               | 0               | 0               |
| <b>Net profit/loss for the period</b>    | <b>-10 856</b>  | <b>-7 665</b>   | <b>-29 551</b>  | <b>-17 929</b>  |
| Attributable to                          |                 |                 |                 |                 |
| Parent company shareholders              | -10 856         | -7 583          | -29 551         | -17 602         |
| Non-controlling interests                | 0               | -82             | 0               | -326            |
| Earnings per share before dilution       | -0,05           | -0,12           | -0,13           | -0,28           |
| Earnings per share after dilution        | -0,05           | -0,12           | -0,13           | -0,28           |
| Average number of shares before dilution | 229 277 024     | 65 053 647      | 178 780 152     | 57 543 838      |
| Average number of shares after dilution  | 229 277 024     | 65 053 647      | 178 780 152     | 57 543 838      |
| Total outstanding shares                 | 229 277 024     | 65 053 647      | 229 277 024     | 65 053 647      |

## Group balance sheet in summary

| Figures in TSEK   | 2021<br>31 Mar | 2020<br>31 Mar | 2020<br>31 Dec | 2019<br>31 Dec |
|---|----------------|----------------|----------------|----------------|
| <b>ASSETS</b>   |                |                |                |                |
| Intangible assets   | 23 397         | 24 325         | 24 046         | 24 936         |
| <b>Total fixed assets</b>                                 | <b>23 397</b>  | <b>24 325</b>  | <b>24 046</b>  | <b>24 936</b>  |
| <b>Current assets</b>                                     |                |                |                |                |
| Inventories   | 824            | 0              | 824            | 0              |
| Other receivables   | 10 631         | 4 455          | 5 649          | 3 716          |
| Cash and bank balances                                    | 34 091         | 9 545          | 48 895         | 15 166         |
| <b>Total current assets</b>                               | <b>45 546</b>  | <b>14 000</b>  | <b>55 368</b>  | <b>18 882</b>  |
| <b>Total assets</b>                                       | <b>68 943</b>  | <b>38 325</b>  | <b>79 414</b>  | <b>43 818</b>  |
| <b>SHAREHOLDERS' EQUITY AND LIABILITIES</b>               |                |                |                |                |
| Share capital   | 22 928         | 6 505          | 22 928         | 6 505          |
| Other capital contribution                                | 136 305        | 69 348         | 136 305        | 69 348         |
| Other shareholders' equity                                | -87 800        | -57 393        | -58 248        | -39 787        |
| Profit/loss for the period                                | -10 856        | -7 583         | -29 551        | -17 602        |
| <b>Equity attributable to parent company shareholders</b> | <b>60 577</b>  | <b>10 877</b>  | <b>71 433</b>  | <b>18 464</b>  |
| <b>Minority interest</b>                                  | <b>0</b>       | <b>1 758</b>   | <b>0</b>       | <b>1 840</b>   |
| <b>Total shareholders' equity</b>                         | <b>60 577</b>  | <b>12 635</b>  | <b>71 433</b>  | <b>20 304</b>  |
| <b>Liabilities</b>  |                |                |                |                |
| Current liabilities                                       | 8 366          | 25 689         | 7 981          | 23 514         |
| <b>Total liabilities</b>                                  | <b>8 366</b>   | <b>25 689</b>  | <b>7 981</b>   | <b>23 514</b>  |
| <b>Total shareholders' equity and liabilities</b>         | <b>68 943</b>  | <b>38 325</b>  | <b>79 414</b>  | <b>43 818</b>  |

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

| Figures in TSEK                                      | Share capital | Other capital contribution | Accumulated profit/loss | Total shareholders' equity |
|--|---------------|----------------------------|-------------------------|----------------------------|
| <b>Balance brought forward</b>                       | <b>22 928</b> | <b>136 305</b>             | <b>-87 800</b>          | <b>71 433</b>              |
| Net profit/loss for the period                       |               |                            | -10 856                 | -10 856                    |
| <b>Amount as per the end of the reporting period</b> | <b>22 928</b> | <b>136 305</b>             | <b>-98 656</b>          | <b>60 577</b>              |

## Group cash flow statement in summary

| Figures in TSEK   | 2021           | 2020          | 2020          | 2019           |
|---|----------------|---------------|---------------|----------------|
|   | Jan-Mar        | Jan-Mar       | Jan-Dec       | Jan-Dec        |
| Cash flow from operating activities                     | -14 804        | -10 616       | -38 346       | -21 605        |
| Cash flow from investing activities                     | 0              | -4            | -104          | -1 521         |
| Cash flow from financing activities                     | 0              | 5 000         | 72179         | 6487           |
| <b>Cash flow for the period</b>                         | <b>-14 804</b> | <b>-5 620</b> | <b>33 729</b> | <b>-16 639</b> |
| Liquid assets at the beginning of the reporting period  | 48 895         | 15 165        | 15 166        | 31 805         |
| <b>Liquid assets at the end of the reporting period</b> | <b>34 091</b>  | <b>9 545</b>  | <b>48 895</b> | <b>15 166</b>  |

## Parent Company income statement in summary

| Figures in TSEK                         | 2021           | 2020          | 2020           | 2019           |
|---|----------------|---------------|----------------|----------------|
|   | Jan-Mar        | Jan-Mar       | Jan-Dec        | Jan-Dec        |
| Net sales                               | 0              | 0             | 0              | 0              |
| Other operating revenues                | 5 118          | 4 058         | 12 029         | 15 730         |
| <b>Operating expenses</b>               |                |               |                |                |
| Other external expenses                 | -12 625        | -8 134        | -29 136        | -23 732        |
| Personnel expenses                      | -2 463         | -1 462        | -7 185         | -6 064         |
| Other operating expenses                | -231           | -612          | -869           | -825           |
| <b>Profit/loss before depreciation</b>  | <b>-10 201</b> | <b>-6 149</b> | <b>-25 160</b> | <b>-14 891</b> |
| Depreciation                            | -75            | -38           | -200           | -19            |
| <b>Profit/loss after depreciation</b>   | <b>-10 276</b> | <b>-6 187</b> | <b>-25 360</b> | <b>-14 910</b> |
| Net financial income/expense            | 0              | -478          | -4 352         | -181           |
| <b>Income after net financial items</b> | <b>-10 276</b> | <b>-6 665</b> | <b>-29 712</b> | <b>-15 091</b> |
| Tax                                     | 0              | 0             | 0              | 0              |
| <b>Net profit/loss for the period</b>   | <b>-10 276</b> | <b>-6 665</b> | <b>-29 712</b> | <b>-15 091</b> |

## Parent Company balance sheet in summary

| Figures in TSEK                                       | 2021<br>31 Mar | 2020<br>31 Mar | 2020<br>31 Dec | 2019<br>31 Dec |
|---|----------------|----------------|----------------|----------------|
| <b>ASSETS</b>   |                |                |                |                |
| Intangible assets                                     | 4 465          | 3 098          | 4 540          | 3 135          |
| Financial assets                                      | 22 601         | 23 467         | 23 175         | 23 463         |
| <b>Total fixed assets</b>                             | <b>27 066</b>  | <b>26 565</b>  | <b>27 714</b>  | <b>26 598</b>  |
| <b>Current assets</b>                                 |                |                |                |                |
| Inventories   | 824            | 0              | 824            | 0              |
| Other receivables                                     | 11 231         | 4 455          | 6 233          | 3 684          |
| Cash and bank balances                                | 33 909         | 9 266          | 48 703         | 14 959         |
| <b>Total current assets</b>                           | <b>45 964</b>  | <b>13 721</b>  | <b>55 759</b>  | <b>18 643</b>  |
| <b>Total assets</b>                                   | <b>73 030</b>  | <b>40 286</b>  | <b>83 474</b>  | <b>45 241</b>  |
| <b>SHAREHOLDERS' EQUITY AND LIABILITIES</b>           |                |                |                |                |
| <b>Restricted equity</b>                              |                |                |                |                |
| Share capital   | 22 928         | 6 505          | 22 928         | 6 505          |
| Statutory reserve                                     | 4              | 4              | 4              | 4              |
| Reserve for development expenses                      | 612            | 508            | 612            | 508            |
| <b>Non-restricted equity</b>                          |                |                |                |                |
| Share premium reserve                                 | 116 619        | 49 255         | 116 619        | 49 255         |
| Accumulated loss including profit/loss for the period | -75 454        | -41 452        | -64 604        | -34 787        |
| <b>Total shareholders' equity</b>                     | <b>64 708</b>  | <b>14 820</b>  | <b>75 558</b>  | <b>21 484</b>  |
| <b>Liabilities</b>                                    |                |                |                |                |
| Current liabilities                                   | 8 322          | 25 466         | 7 916          | 23 757         |
| <b>Total liabilities</b>                              | <b>8 322</b>   | <b>25 466</b>  | <b>7 916</b>   | <b>23 757</b>  |
| <b>Total shareholders' equity and liabilities</b>     | <b>73 030</b>  | <b>40 286</b>  | <b>83 474</b>  | <b>45 241</b>  |



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

| Figures in TSEK                                      | Share capital | Statutory reserve | Reserve for development expenses | Share premium reserve | Accumulated profit/loss | Total shareholders' equity |
|--|---------------|-------------------|----------------------------------|-----------------------|-------------------------|----------------------------|
| <b>Balance brought forward</b>                       | <b>22 928</b> | <b>4</b>          | <b>612</b>                       | <b>116 619</b>        | <b>-64 604</b>          | <b>75 558</b>              |
| Net profit/loss for the period                       |               |                   |                                  |                       | -10 850                 | -10 850                    |
| <b>Amount as per the end of the reporting period</b> | <b>22 928</b> | <b>4</b>          | <b>612</b>                       | <b>116 619</b>        | <b>-75 454</b>          | <b>64 708</b>              |

## Parent Company cash flow statement in summary

| Figures in TSEK   | 2021           | 2020          | 2020          | 2019           |
|---|----------------|---------------|---------------|----------------|
|   | Jan-Mar        | Jan-Mar       | Jan-Dec       | Jan-Dec        |
| Cash flow from operating activities                     | -14 794        | -10 689       | -38 284       | -14 971        |
| Cash flow from investing activities                     | 0              | -4            | -3 259        | -8 706         |
| Cash flow from financing activities                     | 0              | 5 000         | 75 286        | 7 011          |
| <b>Cash flow for the period</b>                         | <b>-14 794</b> | <b>-5 693</b> | <b>33 743</b> | <b>-16 666</b> |
| Liquid assets at the beginning of the reporting period  | 48 703         | 14 959        | 14 959        | 31 625         |
| <b>Liquid assets at the end of the reporting period</b> | <b>33 909</b>  | <b>9 266</b>  | <b>48 703</b> | <b>14 959</b>  |

## Group financial key ratios

| Figures in TSEK                          | 2021        | 2020       | 2020        | 2019       |
|--|-------------|------------|-------------|------------|
|  | Jan-Mar     | Jan-Mar    | Jan-Dec     | Jan-Dec    |
| Earnings per share before dilution, SEK  | -0,05       | -0,12      | -0,17       | -0,31      |
| Earnings per share after dilution, SEK   | -0,05       | -0,12      | -0,17       | -0,31      |
| Shareholders' equity per share, SEK      | 0,26        | 0,19       | 0,31        | 0,31       |
| Equity ratio, %                          | 87,87       | 32,97      | 89,95       | 46,34      |
| Average number of shares before dilution | 229 277 024 | 65 053 647 | 178 780 152 | 57 543 838 |
| Average number of shares after dilution  | 229 277 024 | 65 053 647 | 178 780 152 | 57 543 838 |
| Total outstanding shares                 | 229 277 024 | 65 053 647 | 229 277 024 | 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           |
| 2020                            | New share issue      | 14 721 013                | 2 158 813    | 147 210 132        | 21 588 125    | 0,10           |
| 2020                            | New share issue      | 17 666 081                | 2 945 068    | 176 660 811        | 29 450 679    | 0,10           |
| 2020                            | New share issue      | 17 822 218                | 156 137      | 178 222 176        | 1 561 365     | 0,10           |
| 2020                            | New share issue      | 20 768 890                | 2 946 672    | 207 688 899        | 29 466 723    | 0,10           |
| 2020                            | New share issue      | 22 927 702                | 2 158 813    | 229 277 024        | 21 588 125    | 0,10           |
| <b>At the end of the period</b> |                      | <b>22 927 702,4</b>       |              | <b>229 277 024</b> |               | <b>0,10</b>    |

# Glossary

## AED

Anti-Epileptic Drug.

## Clinical phase I

Phase I refers to the first instance of testing of a candidate drug on humans. Phase I trials are often conducted with a small group of healthy volunteer trial subjects to determine the safety and dosage of an as yet non-approved treatment method.

## Clinical phase II

Phase II trials refer to a pharmaceutical product under development that is administered to a small group of patients to study the safety, dosage and efficacy.

## Clinical phase III

Phase III studies include a sufficient number of patients to meet regulatory prerequisites for approval. The aim is to determine the statistical significance with respect to the effect of a new candidate drug, without major side effects and under carefully controlled real-world conditions. The new drug is sometimes compared with an established treatment, such as an approved drug.

## Clinical studies

Research studies that explore whether a new, as yet non-approved, drug, medical strategy, treatment, or device is safe and effective for humans.

## GMP

A Good Manufacturing Practice (GMP) is a system for ensuring that pharmaceutical products are consistently produced and controlled according to quality standards. Permits for GMP are granted by the Food and Drug Administration in the country in question and the process is characterized by extremely rigid and high demands on quality in all respects.

## In vitro

A term used in biomedical science to describe a biological process made to occur in a laboratory vessel or other controlled experimental environment rather than within a living organism.

## In vivo

A term used in biomedical science to describe an experimental biological process, and observations thereof, made to occur within a living organism.

## Neuropeptide

Neuropeptides are small, protein-like molecules (peptides) that are used by neurons to communicate with each other.

## NPY

Neuropeptide Y, a neurotransmitter that is widely distributed in the central nervous systems of animals and humans.

## Proof of concept

Documented evidence that a potential product or method has the intended effect.

## Viral vector

Viral vectors are tools that are used to deliver genetic material to cells. Examples of viral vectors are lentivirus, adeno-associated virus (AAV), retrovirus and adenovirus. AAV vectors are non-hazardous viruses that can infect human cells without causing disease and can be used to deliver genetic material into human cells.



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# 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 CGO1 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.*



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