



## Interim report

January – March 2022

CombiGene AB (publ)



Continued preclinical development of CG01 in close collaboration with Spark

Intensified business development with the ambition of expanding our gene therapy portfolio

Bringing the CGT2 project to the stage where we can initiate the important proof-of-concept study



## Interim report January – March 2022 for CombiGene AB (publ)

### Period January – March 2022

- Net sales: 11,403 (0) TSEK.
- Other operating revenues: 3,102 (5,118) TSEK.
- Profit from financial items: -2,684 (-10,856) TSEK.
- Earnings per share: -0.14 (-0.95) SEK.
- Cash and bank: 121,665 (34,091) TSEK.

### Events during the period

- GMP production of CG01 made available for preclinical studies planned to enable First in Human study.
- CombiGene signs agreement with University of Michigan to evaluate the leading gene therapy candidate within the lipodystrophy project CGT2.
- CombiGene's and Neurochase's preclinical study provides valuable information for the upcoming long-term studies in toxicology and biodistribution.
- At the beginning of 2022, relations between Russia and Ukraine deteriorated and on February 24, 2022, Russia invaded Ukraine. The situation continues to be characterized by great uncertainty and the course of events is unpredictable. Market reactions to the development have been strongly negative, which can be seen in significant price falls in the stock markets in the countries concerned, but also in other markets, including the Swedish market. In addition, the United States and Europe have imposed economic sanctions on Russia. CombiGene has no operations in Russia or Ukraine and the implementation of the company's ongoing and planned preclinical/clinical studies and the results of these are not expected to be affected by the war in Ukraine. CombiGene will inform investors if such an impact on the business is expected to occur. Since the outbreak of war, the capital market has become turbulent and both the short-term and the long-term consequences for the world economy are difficult to predict. If this uncertain situation remains, it may pose greater challenges in raising new capital for the company.

## Events after the end of the period

- There have been no significant events after the end of the period.

### About CombiGene AB

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 preclinical/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 has an exclusive collaboration and licensing agreement for the CG01 project with Spark Therapeutics.

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](mailto: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



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

## Focus on business development with the ambition to in-license a third gene therapy project

*Through the collaboration and in-licensing agreement with Spark Therapeutics in 2021, CombiGene reached a new stage in the company's development. Through this agreement, CombiGene became an internationally recognized gene therapy company. Our ambition now is to build on this success by the in-licensing of a third gene therapy project, and our efforts to accomplish this were intensified in the first quarter of 2022.*

### In search of a third gene therapy project

We are primarily seeking AAV-based projects because it is within this technology platform that the company has established knowledge in key areas such as vector design (design of drug candidate), safety aspects, and production. Similarly, the areas of disease that are in focus are those where CombiGene has a solid knowledge and collaboration network, i.e., diseases of the central nervous system and metabolic diseases. Having said that, we will at the same time have an open attitude towards all potential projects and evaluate each opportunity on its own merits.

Finding the right gene therapy project to in-license and negotiating the right terms are however no easy tasks, and it goes without saying that it is not possible to predict exactly when we will have a third project to complement our very promising portfolio.

### Strengthening our collaboration with Spark

We have now been working together with Spark since mid-October 2021, and the collaboration is progressing well. I am particularly pleased with the decision to also initially expand CG01's clinical development program to include the U.S. as this will allow the project to find a natural foothold in the world's largest pharmaceutical market, at the same time as Spark can utilize their impressive resources, know-how and networks in an optimal way.

As previously communicated, the remaining preclinical program will be expanded and, in some parts, complemented with additional studies in order to prepare CG01 to meet the needs of an extended submission. In practice, this means that the preclinical part supporting initiation of clinical development of CG01 will take longer time to finalize.

### Future revenue stream from the agreement with Spark

Under the terms of agreement with Spark, CombiGene is eligible to receive up to USD 328.5 million excluding royalties, with USD 8.5 million upon signing, up to USD 50 million at preclinical and clinical milestones. This means that the revenue from the agreement with Spark will be effected in the form of large, but few milestone payments spread out over time.

### Payment and financing of external and internal R&D costs

During the remaining part of the preclinical program, all CG01-related R&D activities that CombiGene is running, internal as well as external, will be agreed upon and approved by Spark, who also assumes all agreed costs. In practice, this means that these activities will appear under operating expenses and net sales in CombiGene's income statement during the quarter in which they occur.

As CG01 enters the clinical phase, Spark will take over the responsibility for the continuation of the program and thus also bear all costs during this development phase.

### Focus in 2022

In 2022, CombiGene will focus on three areas: the continued preclinical development of CG01 in close collaboration with Spark; bringing the CGT2 project to the stage where we can initiate the important proof-of-concept study; and intensified business development with the ambition of expanding our gene therapy portfolio.

Jan Nilsson,  
CEO



*In October 2021, the epilepsy project CG01 achieved its most important milestone to date through the collaboration and licensing agreement with Spark Therapeutics. Since CombiGene and Spark entered the exclusive agreement, the two companies have jointly reviewed the future development of the CG01 project to establish the best path forward.*

**Expansion of the clinical development program**

In December 2021, the decision was taken to expand the clinical development program to include the U.S. in addition to Europe. Originally, the clinical development program for the CG01 project was planned to be performed in Europe, CombiGene’s home market. Establishing a clinical presence in the U.S. adds much further strength to the CG01 project and enables it to find a natural foothold on the world’s largest pharmaceutical market.

**Extended preclinical program**

In order to prepare CG01 to meet the needs of an extended submission, the remaining preclinical program will be expanded and, in some parts, complemented with additional studies. In practice, this means that the preclinical part of CG01 to support initiation of the First in Human study will take longer time to finalize than what was planned prior to the agreement with Spark.

**Spark will take full responsibility for the clinical development**

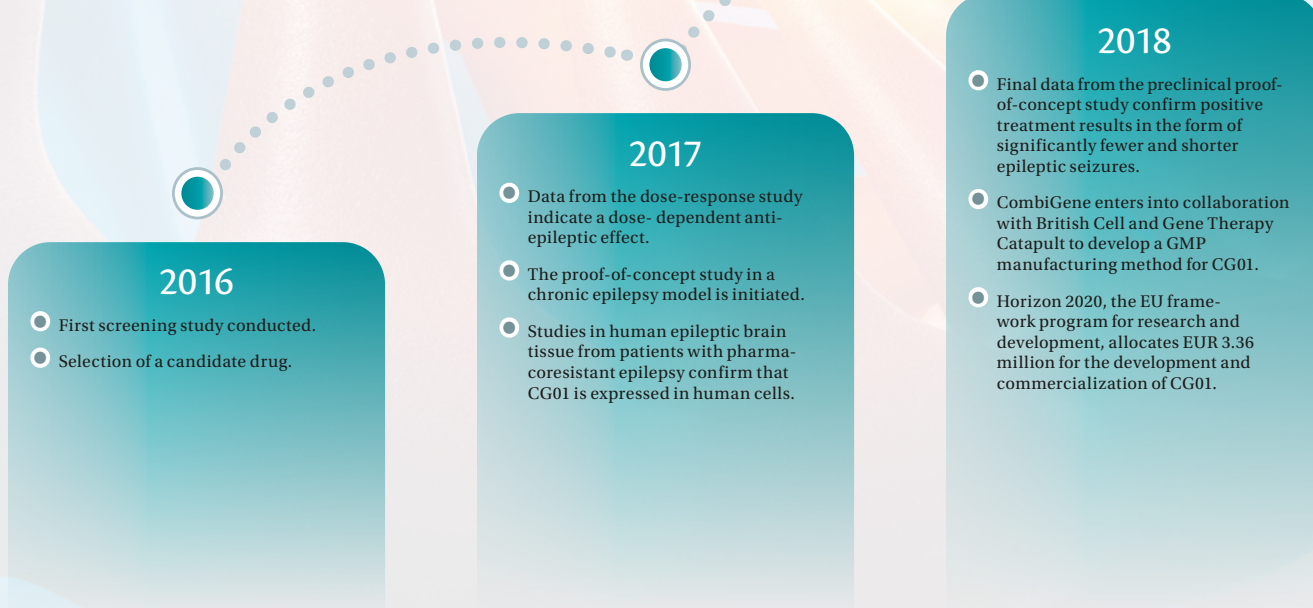
Once the preclinical program is completed, Spark will take full responsibility for the clinical development from the first human study onwards to global commercialization. The work done and know-how acquired by CombiGene regarding the planning of the clinical study has been transferred to Spark.

**Events in the quarter**

**GMP production.** In January 2022, CombiGene and its CDMO partner Viralgen announced that the GMP production of CG01 has been made available for the concluding preclinical studies planned to enable First in Human study. This is the first large scale GMP production of CG01, and both the production itself and the subsequent testing were performed according to original plans. The data from the analyses will form a central part of future regulatory applications to support proceeding to clinical studies.

**Pilot study to optimize administration.** In February 2022, CombiGene announced that the practical part of the pilot study, which was performed in collaboration with Neurochase, had been completed. In the study, the administration of CG01 was evaluated in terms of, among other things, injection volume and injection rate. The study provides valuable information about the Neurochase injection device for the upcoming long-term studies in toxicology and biodistribution in large animals.

**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.

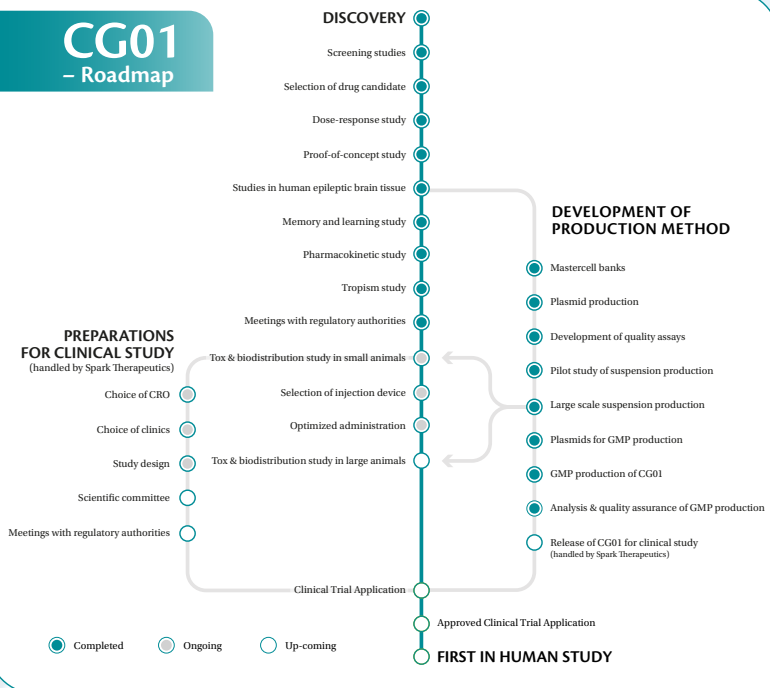
**2018**

- Final data from the preclinical proof-of-concept study confirm positive treatment results in the form of significantly fewer and shorter epileptic 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.



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 – Roadmap



### The agreement with Spark

The agreement provides Spark with the exclusive world-wide license to develop, manufacture and commercialize CG01. CombiGene will continue to execute certain aspects of the preclinical program in collaboration with Spark. Under the terms of agreement, CombiGene is eligible to receive up to USD 328.5 million excluding royalties, with USD 8.5 million upon signing and up to USD 50 million at preclinical and clinical milestones. CombiGene will also be reimbursed for certain authorized R&D expenses. Upon commercialization, CombiGene is eligible for tiered royalties ranging from the mid-single digits up to low double-digits based on net sales.

### 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 GMP 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.
- Delivery of 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.
- GMP-produced plasmids (starting material for the production of CG01) released for GMP production of CG01.
- First GMP production of CG01 initiated.
- CG01 patent is approved in the U.S. and Russia.
- Global and exclusive collaboration and licensing agreement with Spark Therapeutics.
- CombiGene's Horizon 2020 project successfully completed.

CGT2, CombiGene’s project to develop a gene therapy treatment for partial lipodystrophy, is in early preclinical development. The first step in designing gene therapy vectors and testing them in vitro (tests on different liver cells) has been carried out with good results. Since then, several in vivo studies have been performed to evaluate efficacy and narrow down the potential gene therapy candidates. The ambition for 2022 is to bring the CGT2 project to the stage where the important proof-of-concept study can be initiated.

### Grants from the EU’s Eurostars international funding program

In February 2021, the Lipodystrophy project was awarded EUR 882,500 in project grants by the EU’s Eurostars international funding program. Thanks to this funding CombiGene has established a good collaboration with the University Medical Center Hamburg-Eppendorf, which has a research group with experts in lipid research. Through the Eurostars grant, CombiGene has also been able to strengthen its collaboration with Accelerio, a German CRO company that works on developing analytical methods to measure the efficacy of the CGT2 therapy.

### PCT application

In August 2021, CombiGene submitted a so- called PCT application to protect the vectors developed within the CGT2 project. The Patent Cooperation Treaty (PCT) is an international agreement that allows companies to seek patent protection internationally for their innovations in about 150 countries. Within the framework of the PCT, a preliminary assessment of patentability is made before the application can proceed to the national phase where national patent applications are submitted. PCT applications are handled by the World Intellectual Property Organization (WIPO), a self-funding body within the United Nations.

The now submitted PCT application builds on the UK patent application filed in 2020 and is a natural next step in ensuring adequate patent protection for the lipodystrophy project CGT2.

## CGT2 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.





## Events in the quarter

In January 2022, CombiGene signed an important agreement with Professor Ormond MacDougald at the University of Michigan Medical School in the U.S. The agreement comprises one pilot study and one main study in which CombiGene's most promising gene therapy candidate within the lipodystrophy project CGT2 will be tried and evaluated.

Professor Ormond MacDougald's new experimental model has large resemblances with partial lipodystrophy in humans, the disorder that CombiGene aims to treat. Professor MacDougald has an entire team working within the lipodystrophy field and the team has acquired extensive knowledge that will benefit CombiGene's CGT2 project.

### 2021

- The lipodystrophy project receives EUR 882,500 in development grants from the EU Eurostars program.
- PCT application submitted.

### 2022

- CombiGene signs agreement with University of Michigan to evaluate the leading gene therapy candidate within the lipodystrophy project CGT2.



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

## Value-creating business development through development of gene therapy assets and international partnering

*CombiGene has for several years worked long-term to establish the company as an interesting player in the international pharmaceutical market and has gradually built up an extensive network of partners with specific competences within gene therapy. Since CombiGene signed the collaboration and licensing agreement for CG01 with Spark Therapeutics in 2021, the company has intensified its efforts to find a new project to in-license.*

CombiGene's business development spans three areas:

- In-licensing of new projects with high potential for value creation within CombiGene.
- Alliances with partners and service companies that allow CombiGene to further develop licensed projects.
- Out-licensing of projects that target significant patient populations in late preclinical/early clinical phase. When it comes to drug candidates targeting limited patient populations, CombiGene may drive development and commercialization under its own management or seek strategic partnerships.

So far, CombiGene's business development has resulted in the in-licensing of the CGT2 lipodystrophy project from Lipigon, establishing collaboration with a number of CRO and CDMO companies within the CG01 and CGT2 projects, and out-licensing of the CG01 epilepsy project to Spark Therapeutics in an agreement with a potential value of USD 328.5 million excluding royalties.

### Focus on in-licensing new projects

With the agreement with Spark, CombiGene is well placed to take the next step in the company's development and the in-licensing of additional gene therapy projects will be in focus.

CombiGene is primarily seeking AAV-based projects because it is within this technology platform that the company has established knowledge in key areas such as vector design (design of drug candidate), safety aspects, and production. Similarly, the areas of disease that are in focus are those where CombiGene has a solid knowledge, i.e., diseases of the central nervous system and metabolic diseases.

Having said that, CombiGene will at the same time have an open attitude towards all potential projects and evaluate each opportunity on its own merits. CombiGene regularly participates in important partnering conferences and the company has continuous dialogues within academia as well as industry to identify interesting projects.

### Income and earnings

Net sales consist of milestone payments and compensation from license and cooperation agreements. Due to the nature of the business, there may be large fluctuations between revenues for different periods when revenue from milestone payments is recognized at the time when the performance obligations are met. The Group has a total net sale of SEK 11,403 (0) thousand during the period January-March. Other operating revenues amounts to SEK 3,102 (5,118) thousand, of which SEK 0 (4.586) thousand refers to the revenue-earned portion of the grant received from Horizon 2020 and SEK 1,053 (217) thousand refers to the revenue-earned portion of the grant received from Eurostars. Operating profit for the period amounted to SEK -2,684 (-10,856) 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 -15,079 TSEK. Liquidity at the end of the period amounts to 121,665 TSEK. The equity ratio is 95,7%.

### Liquidity and financing

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 1.5 million has so far been paid out.

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

### The share

The average number of shares for the period is 19,801,197. All shares are of the same type and have the same voting rights. At the Annual General Meeting of CombiGene on 25 May 2021 a reverse share split was resolved upon, whereby twenty (20) existing shares were consolidated into one (1) new share. Through the reverse share split, the number of shares in CombiGene decreased from 396,023,950 to 19,801,197. For comparability, a retroactive adjustment has been made to the number of shares.

### Employees

The number of employees in the Group at the end of the period was 9 (5), of whom 5 (4) are women.

### Risks and uncertainties

A drug development company of CombiGene's type is characterized by a high operational and financial risk. The Company is dependent on current and future licensing, collaboration, and other agreements with experienced partners for the development and successful commercialization of existing and future drug candidates. The most significant example of this is CombiGene's exclusive global collaboration and licensing agreement with Spark Therapeutics, which has a potential total value of USD 328.5 million excluding royalties. The agreement with Spark is thus of great importance for CombiGene's future operations, earnings, and financial position. Other factors that may negatively affect the likelihood of commercial success include, among other things, the risk that CombiGene's gene therapies are not deemed safe or not effective, and the risk that the business may not receive the necessary funding.

### 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 2022 will be held on 19 May. The Annual Report will be available to the public at the company's office in Lidingö and will be published on the Company's website no later than 3 weeks prior to 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 2022, 25 August 2022.

### For further information, please contact:

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### Stockholm, 12 May 2022, CombiGene AB (publ)

Bert Junno  
Chairman

Peter Nilsson  
Board member

Jonas Ekblom  
Board member

Per Lundin  
Board member

Gunilla Lundmark  
Board member

## Group income statement in summary

Figures in TSEK	2022 Jan-Mar	2021 Jan-Mar	2021 Jan-Dec	2020 Jan-Dec
Net sales	11,403	0	84,042	0
Other operating revenues	3,102	5,118	7,478	12,029
<b>Operating expenses</b>				
Other external expenses	-13,574	-12,631	-54,591	-29,640
Personnel expenses	-2,665	-2,463	-11,692	-7,185
Other operating expenses	-301	-231	-1,677	-869
<b>Profit/loss before depreciation</b>	<b>-2,035</b>	<b>-10,207</b>	<b>23,560</b>	<b>-25,665</b>
Depreciation	-649	-649	-2,595	-2,495
<b>Profit/loss after depreciation</b>	<b>-2,684</b>	<b>-10,856</b>	<b>20,965</b>	<b>-28,159</b>
Net financial income/expense	0	0	0	-1,392
<b>Income after net financial items</b>	<b>-2,684</b>	<b>-10,856</b>	<b>20,965</b>	<b>-29,551</b>
Tax	0	0	0	0
<b>Net profit/loss for the period</b>	<b>-2,684</b>	<b>-10,856</b>	<b>20,965</b>	<b>-29,551</b>
Attributable to				
Parent company shareholders	-2,684	-10,856	20,965	-29,383
Non-controlling interests	0	0	0	-169
Earnings per share before dilution	-0.14	-0.95	1.21	-3.31
Earnings per share after dilution	-0.14	-0.95	1.21	-3.31
Average number of shares before dilution	19,801,197	11,463,851	17,311,414	8,939,008
Average number of shares after dilution	19,801,197	11,463,851	17,311,414	8,939,008
<i>Total outstanding shares</i>	<i>19,801,197</i>	<i>11,463,851</i>	<i>19,801,197</i>	<i>11,463,851</i>

## Group balance sheet in summary

Figures in TSEK	2022 31 Mar	2021 31 Mar	2021 31 Dec	2020 31 Dec
<b>ASSETS</b>				
Intangible assets	20,950	23,397	21,599	24,046
<b>Total fixed assets</b>	<b>20,950</b>	<b>23,397</b>	<b>21,599</b>	<b>24,046</b>
<b>Current assets</b>				
Accounts receivable	6,826	0	0	0
Inventories	0	824	0	824
Other receivables	13,114	10,631	7,879	5,649
Cash and bank balances	121,665	34,091	136,744	48,895
<b>Total current assets</b>	<b>141,604</b>	<b>45,546</b>	<b>144,622</b>	<b>55,368</b>
<b>Total assets</b>	<b>162,554</b>	<b>68,943</b>	<b>166,221</b>	<b>79,414</b>
<b>SHAREHOLDERS' EQUITY AND LIABILITIES</b>				
Share capital	990	22,928	990	22,928
Other capital contribution	224,124	136,305	224,124	136,305
Other shareholders' equity	-66,835	-87,800	-87,800	-58,417
Profit/loss for the year	-2,684	-10,856	20,965	-29,383
<b>Equity attributable to parent company shareholders</b>	<b>155,595</b>	<b>60,577</b>	<b>158,279</b>	<b>71,433</b>
<b>Total shareholders' equity</b>	<b>155,595</b>	<b>60,577</b>	<b>158,279</b>	<b>71,433</b>
<b>Liabilities</b>				
Current liabilities	6,959	8,366	7,942	7,981
<b>Total liabilities</b>	<b>6,959</b>	<b>8,366</b>	<b>7,942</b>	<b>7,981</b>
<b>Total shareholders' equity and liabilities</b>	<b>162,554</b>	<b>68,943</b>	<b>166,221</b>	<b>79,414</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>990</b>	<b>224,124</b>	<b>-66,835</b>	<b>158,279</b>
Net profit/loss for the period			-2,684	-2,684
<b>Amount as per the end of the reporting period</b>	<b>990</b>	<b>224,124</b>	<b>-69,519</b>	<b>155,595</b>

## Group cash flow statement in summary

Figures in TSEK	2022 Jan-Mar	2021 Jan-Mar	2021 Jan-Dec	2020 Jan-Dec
Cash flow from operating activities	-15,079	-14,804	22,115	-38,346
Cash flow from investing activities	0	0	-148	-3,211
Cash flow from financing activities	0	0	65,881	75,286
<b>Cash flow for the period</b>	<b>-15,079</b>	<b>-14,804</b>	<b>87,849</b>	<b>33,729</b>
Liquid assets at the beginning of the reporting period	136,744	48,895	48,895	15,166
<b>Liquid assets at the end of the reporting period</b>	<b>121,665</b>	<b>34,091</b>	<b>136,744</b>	<b>48,895</b>

## Parent Company income statement in summary

Figures in TSEK	2022 Jan-Mar	2021 Jan-Mar	2021 Jan-Dec	2020 Jan-Dec
Net sales	11,403	0	84,042	0
Other operating revenues	3,088	5,118	7,478	12,029
<b>Operating expenses</b>				
Other external expenses	-13,557	-12,625	-54,517	-29,136
Personnel expenses	-2,665	-2,463	-11,692	-7,185
Other operating expenses	-301	-231	-1,677	-869
<b>Profit/loss before depreciation</b>	<b>-2,032</b>	<b>-10,201</b>	<b>23,634</b>	<b>-25,160</b>
Depreciation	-75	-75	-300	-200
<b>Profit/loss after depreciation</b>	<b>-2,107</b>	<b>-10,276</b>	<b>23,334</b>	<b>-25,360</b>
Net financial income/expense	-574	0	-2,295	-4,352
<b>Income after net financial items</b>	<b>-2,681</b>	<b>-10,276</b>	<b>21,039</b>	<b>-29,712</b>
Tax	0	0	0	0
<b>Net profit/loss for the period</b>	<b>-2,681</b>	<b>-10,276</b>	<b>21,039</b>	<b>-29,712</b>

## Parent Company balance sheet in summary

Figures in TSEK	2022 31 Mar	2021 31 Mar	2021 31 Dec	2020 31 Dec
<b>ASSETS</b>				
Intangible assets	4,312	4,465	4,387	4,540
Financial assets	20,306	22,601	20,880	23,175
<b>Total fixed assets</b>	<b>24,619</b>	<b>27,066</b>	<b>25,267</b>	<b>27,714</b>
<b>Current assets</b>				
Accounts receivable	6,826	0	0	0
Inventories	0	824	0	824
Other receivables	13,788	11,231	8,563	6,233
Cash and bank balances	121,487	33,909	136,545	48,703
<b>Total current assets</b>	<b>142,101</b>	<b>45,964</b>	<b>145,108</b>	<b>55,759</b>
<b>Total assets</b>	<b>166,719</b>	<b>73,030</b>	<b>170,376</b>	<b>83,474</b>
<b>SHAREHOLDERS' EQUITY AND LIABILITIES</b>				
<i>Restricted equity</i>				
Share capital	990	22,928	990	22,928
Statutory reserve	4	4	4	4
Reserve for development expenses	760	612	760	612
<i>Non-restricted equity</i>				
Share premium reserve	165,826	116,619	165,826	116,619
Accumulated loss including profit/loss for the year	-7,781	-75,454	-5,101	-64,604
<b>Total shareholders' equity</b>	<b>159,797</b>	<b>64,708</b>	<b>162,478</b>	<b>75,558</b>
<b>Liabilities</b>				
Current liabilities	6,922	8,322	7,898	7,916
<b>Total liabilities</b>	<b>6,922</b>	<b>8,322</b>	<b>7,898</b>	<b>7,916</b>
<b>Total shareholders' equity and liabilities</b>	<b>166,719</b>	<b>73,030</b>	<b>170,376</b>	<b>83,474</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>990</b>	<b>4</b>	<b>760</b>	<b>165,826</b>	<b>-5,101</b>	<b>162,478</b>
Net profit/loss for the period					-2,681	-2,681
<b>Amount as per the end of the reporting period</b>	<b>990</b>	<b>4</b>	<b>760</b>	<b>165,826</b>	<b>-7,781</b>	<b>159,797</b>

## Parent Company cash flow statement in summary

Figures in TSEK	2022 Jan-Mar	2021 Jan-Mar	2021 Jan-Dec	2020 Jan-Dec
Cash flow from operating activities	-15,058	-14,794	22,109	-38,284
Cash flow from investing activities	0	0	-148	-3,259
Cash flow from financing activities	0	0	65,881	75,286
<b>Cash flow for the period</b>	<b>-15,058</b>	<b>-14,794</b>	<b>87,843</b>	<b>33,743</b>
Liquid assets at the beginning of the reporting period	136,545	48,703	48,703	14,959
<b>Liquid assets at the end of the reporting period</b>	<b>121,487</b>	<b>33,909</b>	<b>136,545</b>	<b>48,703</b>

## Group financial key ratios

Figures in TSEK	2022 Jan-Mar	2021 Jan-Mar	2021 Jan-Dec	2020 Jan-Dec
Earnings per share before dilution, SEK	-0.14	-0.95	1.21	-3.31
Earnings per share after dilution, SEK	-0.14	-0.95	1.21	-3.31
Shareholders' equity per share, SEK	7.86	5.28	7.99	6.23
Equity ratio, %	95.72	87.87	95.22	89.95
Average number of shares before dilution	19,801,197	11,463,851	17,311,414	8,939,008
Average number of shares after dilution	19,801,197	11,463,851	17,311,414	8,939,008
<i>Total outstanding shares</i>	<i>19,801,197</i>	<i>11,463,851</i>	<i>19,801,197</i>	<i>11,463,851</i>

## 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
2021	New share issue	39,602,395	16,674,693	396,023,950	166,746,926	0.10
2021	Reverse share split (1:20)	39,602,395	0	19,801,197	-376,222,753	2.00
2021	Reduction of share capital	990,060	-38,612,335	19,801,197	0	0.05
<b>At the end of the period</b>		<b>990,060</b>		<b>19,801,197</b>		<b>0.05</b>

**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

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 has an exclusive collaboration and licensing agreement for the CG01 project with Spark Therapeutics.

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](mailto:info@fnca.se).



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