CombiGene

*"Spark Therapeutics' decision to terminate the collaboration agreement for CG01 was very surprising and is of course a huge disappointment for all of us who in various capacities have worked for the success of the project. CombiGene's Board of Directors and management are now working to evaluate the situation that has arisen and investigate how the collaboration with Spark can be terminated and handed over in the best possible way, as well as its impact on the company going forward."*

*"Now we brush off the disappointment, see what lessons we can learn for the future, look ahead, and continue our work to develop CombiGene by focusing on our projects and our business development."*

Peter Ekolind
CEO for CombiGene

# Interim Report

January - September 2023

Q3

# Summary of the report

### Events during the period

- The Epilepsy Project progresses through optimization activities in preparation for in-human studies. (September 5)
- CombiGene chooses CDMO partner for the COZY01 pain project. (September 15)

### Events after the end of the period

- Spark Therapeutics terminates collaboration agreement for the epilepsy project CG01 with CombiGene. (October 14)
- CombiGene and Zyneyro choose initial indication in the COZY01 pain project (October 26)
- CombiGene chooses Charles River as preclinical toxicology partner for the COZY01 pain project. (October 31)

### Financial information

*July – September 2023*

- Net sales: TSEK 992 (5,213).
- Other operating revenues: TSEK 173 (8,285).
- Profit from financial items: TSEK -8,403 (3,953).
- Earnings per share: SEK -0.42 (0.20).

*January – September 2023*

- Net sales: TSEK 4,948 (21,354).
- Other operating revenues: TSEK 588 (20,888).
- Profit from financial items: TSEK -29,305 (5,785).
- Earnings per share: SEK -1.48 (0.29).
- Cash and cash equivalents: TSEK 107,187 (144,940).

**For further information:**
Peter Ekolind, CEO
Phone: +46(0)8 35 73 55
peter.ekolind@combigene.com

### Note to the reader

Amounts in brackets refer to the corresponding period of the previous year.

## CombiGene at a glance

### The company

CombiGene is the only listed gene therapy company in the Nordic Region. The company was listed on May 25, 2015 on SPOTLIGHT Stock Market (then AktieTorget). In December 2018, CombiGene was approved for listing on Nasdaq First North Stockholm.

### Our projects

CombiGene currently has four projects:

- The pain program COZY which consists of one peptide treatment and one gene therapy
- The lipodystrophy project CGT2
- The epilepsy project CG01 – evaluation ongoing

### Our team

CombiGene has 11 employess. Our team is made up of very knowledgeable and experienced professionals with longstanding experience from the international pharma industry and the biotech arena with thorough knowledge of different aspects of drug development and gene therapy.

### Cash position

At the end of the first quarter 2023, CombiGene had cash and cash equivalents amounting to TSEK 107,187 (144,940).



CombiGene's 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

# Disappointment in epilepsy collaboration with Spark Continued intensive development of the pain program

*On October 14, CombiGene announced that the Company will regain the global rights for the epilepsy project CG01 no later than January 12, 2024, following a strategic decision by Spark Therapeutics to terminate their further development of the project. In this CEO statement, I would like to give my view of what happened and tell you how I perceive the future of CombiGene.*

**Spark Therapeutics terminates CG01 collaboration**
Since our collaboration began in October 2021, Spark Therapeutics has worked intensively to complete the preclinical phase of the CG01 project and prepare the project for clinical studies. Their decision to now terminate the development of CG01 was therefore very unexpected and is of course a huge disappointment for all of us who in various capacities have worked for the success of the project.

In the midst of our great disappointment, however, we must realize that events like this are part of everyday life for any pharmaceutical company. The development of pharmaceuticals is associated with extensive risks of both a technical and strategic business nature, which is one of the reasons why CombiGene is working hard to broaden the project portfolio.

Somewhat simplified, you can say that the broader the project portfolio, the greater the chances of success. At the same time, it is of course important to economize with existing financial resources, and always prioritize activities where tangible value creation can be achieved at a reasonable cost.

There is also currently a harsh climate throughout the biotech industry, which is reflected, among other things, in a generally very low valuation of small and medium-sized drug development companies. The large pharmaceutical companies are also affected by the hard times, and we have recently seen several strategic decisions regarding reduced project portfolios and terminated collaborations. This does not reduce our disappointment, but I think it is important to put what happened in the right context – all drug development is associated with high risk and strategic decisions among the major pharmaceutical companies can affect both individual development projects and entire therapeutic areas.

CombiGene's Board of Directors and management are now working to evaluate the situation that has arisen and investigate how the collaboration with Spark can be terminated and handed over in the best possible way, as well as its impact on the company going forward. We will of course inform the market as soon as we have decided which path we have chosen.

It is important to know that CombiGene is not liable for any of the remuneration received by the company from Spark Therapeutics, a total of USD 8.5 million excluding development costs, but is also not entitled to any future milestone payments or royalties.

**So, what happens now?**
Now we brush off the disappointment, see what lessons we can learn for the future, look ahead, and continue our work to develop CombiGene by focusing on our projects and our business development. Despite the disappointment, we at CombiGene have shown that we are capable of bringing in an early project, creating value in the project that makes it interesting for a multinational pharmaceutical company to license it for continued development. This is a strength of our team that we are building on for future projects.

**High tempo in the COZY01 pain project**
At the same time, it is important to remember that CombiGene is a company with more than one project. Not only do we have CG01, but we are also continuing the work of collecting data for our second project, CGT2. In our new pain program COZY, we have two projects, a peptide project and a gene therapy project with a high level of activity and a very large market potential.

During the third quarter of the year, we chose a CDMO partner (production partner) in the COZY01 project and at the beginning of the fourth quarter, we chose a partner for the upcoming preclinical toxicology studies and indications for the clinical trial program.

In other words, there is no shortage of exciting tasks at CombiGene, and we will continue to work tirelessly to develop our projects.

Peter Ekolind
CEO

# The pain program COZY – a unique opportunity for a breakthrough in pain treatment

*The pain program COZY is being developed together with the Danish company Zyneyro with the goal of developing an effective treatment for severe chronic pain, a common and often difficult-to-treat condition. The program consists of two projects – a peptide treatment and a gene therapy treatment, both of which are based on a new biological mechanism of action that is expected to be without the side effects that current treatments often give rise to.*

## Pain is a major global problem

About 20-25 percent of the world's adult population suffers from some form of chronic pain and between six and eight percent of the population suffers from severe chronic pain. Conventional treatment consists mainly of anti-inflammatory drugs, antidepressants, anticonvulsant drugs and opioids (a group of substances with a morphine-like mechanism of action).[1]

The problem with these treatments is that they are not specifically developed to treat chronic pain. The pain relief that is achieved therefore often has a number of debilitating side effects such as substance abuse problems, depression, anxiety, fatigue, reduced physical and mental ability. In the United States, an estimated 700,000 people have died due to opioid abuse in the past 20 years.

## One program – two projects

The program consists of two projects: a peptide treatment (COZY01) and a gene therapy treatment (COZY02), which expresses the active part of the peptide from COZY01, with potential lifelong effect.

*In severe chronic pain*, the intention is to administer the peptide directly to the patient to achieve effective pain relief.

*In severe chronic pain where the possibilities for spontaneous reduction of the pain are considered excluded or unlikely* and which with conventional treatment requires daily medication, the intention is to achieve pain relief by treating the patient with an AAV vector that makes the body produce the pain-relieving peptide itself. In this way, long-term pain relief can be achieved without daily medication. Since the AAV vector encodes the peptide, the mechanism of action and thus the expected effect are the same as in direct administration of the peptide.

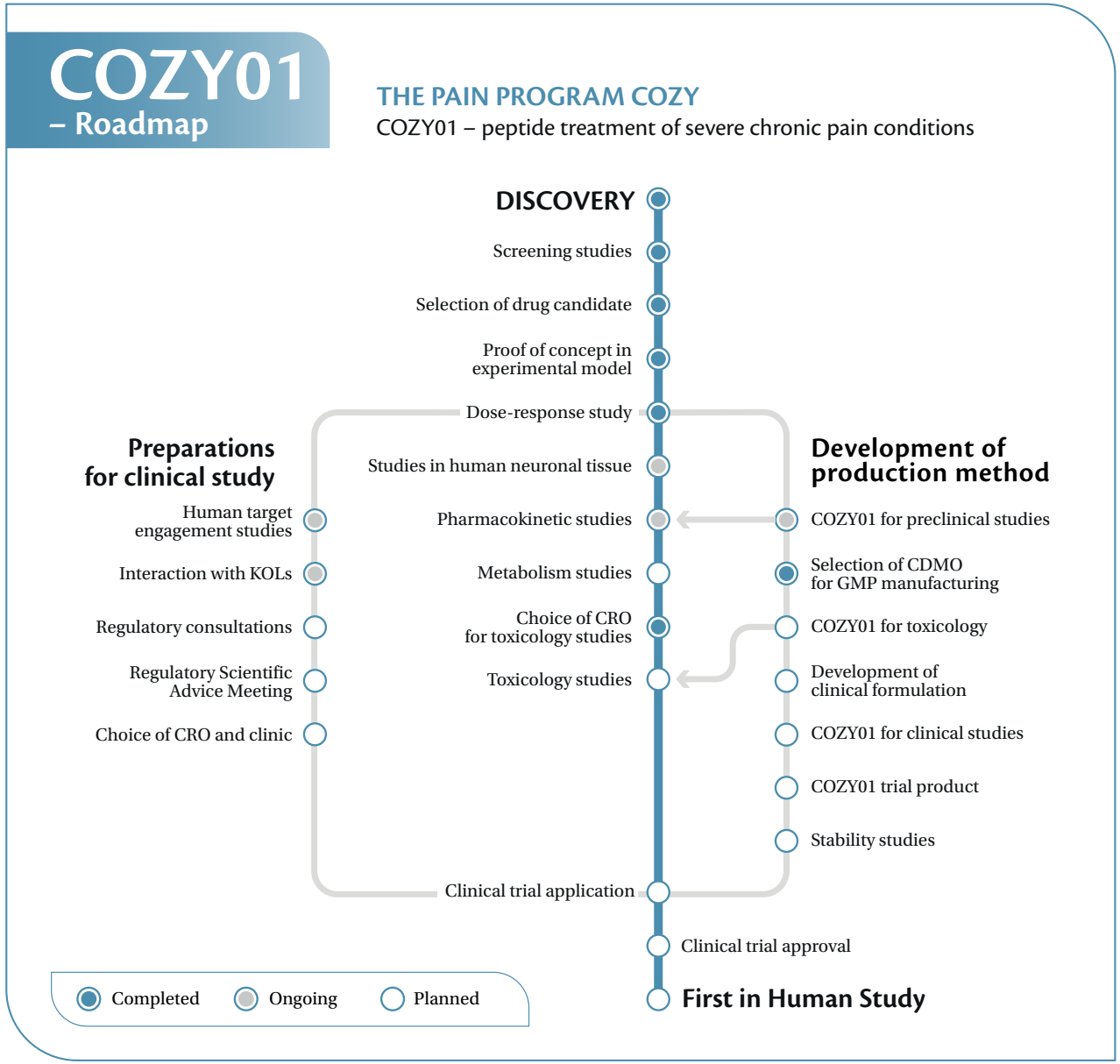
## Independent evaluation by the National Institutes of Health

An independent evaluation of the potential of COZY01 as a future pain treatment is underway at the National Institutes of Health (NIH) in the US, in a government-funded program (Preclinical Screening Platform for Pain, PSPP) aimed at finding pain management alternatives that are not opioid-based and that are not addictive or result in tolerance development. COZY01 has passed the first level of three and has been selected to move on to the next level where the substance will be tested in a behavioral model and in different pain models. During the first six months of the year, CombiGene and Zyneyro worked on preparatory activities for the next step.

**Annual medical and indirect costs of various diseases in the United States**


Source: Relieving Pain in America: A Blueprint for Transforming Prevention, Care, Education, and Research. Appendix C. The Economic Cost of Pain in the US. Institute of Medicine (US) Committee on Advancing Pain Research, Care, and Education. Washington (DC): National Academies Press (US); 2011

1 Prevalence of Chronic Pain and High-Impact Chronic Pain Among Adults — United States, 2016; CDC; Morbidity and Mortality Weekly Report Weekly / Vol. 67 / No. 36 September 14, 2018



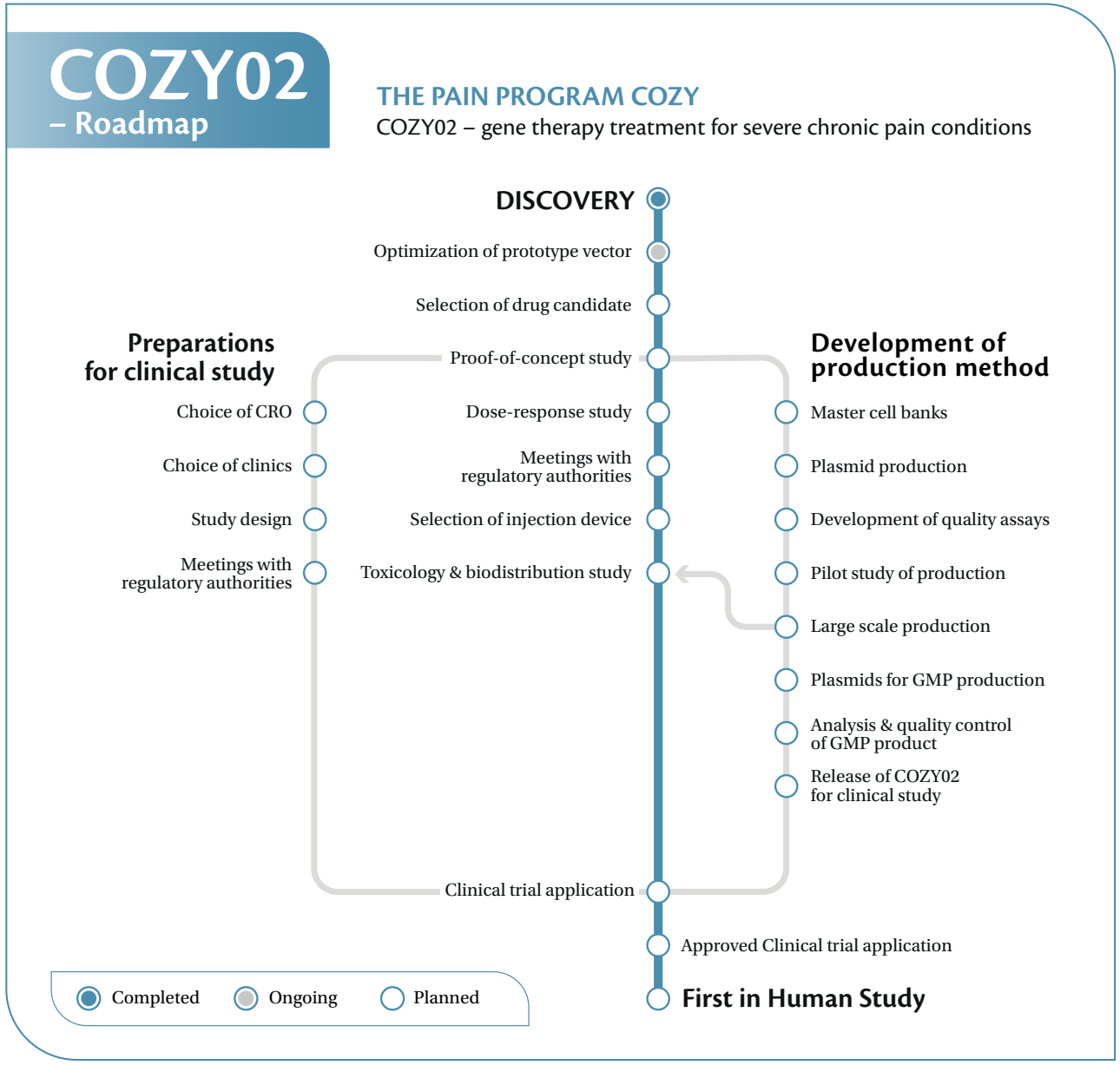

**COZY01 – peptide treatment of severe chronic pain**
The peptide treatment has shown positive effects in various preclinical models. The continued development will focus on conducting the necessary preclinical studies as quickly and efficiently as possible to evaluate safety and toxicology and to produce clinical trial material in order to obtain approval from regulatory authorities to conduct the first clinical trials in humans with COZY01.

*Activities during the quarter*

- Selection of the American company AmbioPharm as contract manufacturer/CDMO partner (Contract Development and Manufacturing Organization). AmbioPharm is a world-leading company with many years of experience in the development and manufacture of peptides and has the full potential to be a long-term partner in this project.

*Activities after the end of the quarter*

- Decision to focus the first study in humans on patients with pain associated with herpes zoster (shingles) – a very painful complication. This patient group is relatively homogeneous, hence very well suited to study the effect of a COZY01 treatment.
- Selection of Charles River Laboratories as a partner in the peptide-based pain project, COZY01: Charles River is a highly respected, global provider of drug discovery and non-clinical development solutions and will perform the preclinical toxicology studies necessary to start the first in-human studies within the COZY01 project.




**COZY02 – gene therapy treatment of severe chronic pain where the possibility of spontaneous reduction of pain is considered excluded**

A prototype of the AAV vector that acts as a carrier of the genetic material in gene therapy has been developed by Zyneyro and tested in several preclinical models with very good and long-lasting effect. The upcoming work is focused on optimizing the genetic material that will be included in the vector so that we can administer this in future studies in humans. AAV is the vector type that CombiGene has extensive experience of from our other projects. When the vector is optimized, preclinical studies will follow to investigate and characterize distribution, protein expression, efficacy, dose-response and toxicology.

In parallel with the preclinical development, we will develop a process for manufacturing the selected vector for preclinical studies and for future clinical trials. Data from this work will form the basis for seeking permission to conduct a clinical trial on patients with severe chronic pain.

# CGT2 – scientific proof-of-concept study will be repeated to provide a basis for decisions on further development

*CGT2, CombiGene's project to develop a gene therapy 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 conducted to evaluate efficacy and gradually limit the number of potential gene therapy candidates.*

### Further studies initiated

After encountering challenges with the interpretation of the results of several studies conducted in 2022, CombiGene decided to conduct additional studies in 2023 for a thorough assessment of the CGT2 project. Unfortunately, these new studies have been delayed due to technical problems at the company's previous supplier of vectors. CombiGene has now switched to a new supplier and the new vectors will be used in collaboration with University Medical Center Hamburg-Eppendorf to conduct and replicate the scientific proof-of-concept study that forms the basis of the project. The results from this scientific proof-of-concept study are expected to be completed in the first half of 2024.

### Contribution from the EU's international funding program Eurostars

In February 2021, the lipodystrophy project was awarded EUR 882,500 in project grants by the EU's international funding program Eurostars. Through this grant, CombiGene collaborates with University Medical Center Hamburg-Eppendorf and its experts in lipid research. The CGT2 project will be funded by Eurostars until March 2024.

### PCT application has proceeded to national patent applications in the US and EU

In August 2021, CombiGene submitted a PCT application to protect the vectors developed within the CGT2 project. The application builds on the UK patent application filed in 2020 and is a natural next step to ensure adequate patent protection for the lipodystrophy project CGT2. During the first half of 2023, CombiGene filed national patent applications in the US and EU.

## Milestones

**2019**
- In-licensing of the project from Lipigon.

**2020**
- Design of expression plasmids, which are the starting material for gene-therapeutic vectors CombiGene intends to develop for treatment of partial lipodystrophy.
- In vitro studies (tests on liver cells) show intended 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 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. A number of in vivo studies are being conducted, but since some results are difficult to interpret, the company has chosen to repeat some studies in 2023.

**2023**
- National patent applications filed in the US and EU.

eurostars™ CombiGene´s project CGT2 is supported by the Eurostars Programme.Project ID: 114714

# Spark Therapeutics terminates collaboration agreement for the epilepsy project CG01 with CombiGene

*CombiGene will regain the global rights for the epilepsy project CG01 from Spark Therapeutics by January 12, 2024 at the latest. The collaboration agreement between CombiGene and Spark Therapeutics, which was signed in October 2021, shall be terminated because of a strategic decision by Spark Therapeutics to deprioritize the further development of CG01 in epilepsy as part of their current pipeline.*

CombiGene's Board of Directors and management are now working to evaluate the situation that has arisen and investigate how the collaboration with Spark can be terminated and handed over in the best possible way, as well as its impact on the company going forward. CombiGene will of course inform the market as soon as the company has decided which path it has chosen.

The collaboration agreement signed in October 2021 gave Spark the exclusive global license to develop, manufacture and commercialize CG01. Under the terms of the agreement, CombiGene was entitled to receive up to USD 328.5 million excluding royalties. During the collaboration, CombiGene has also been compensated for agreed development costs.

CombiGene is not liable for any of the payments received by the company from Spark Therapeutics, totaling USD 8,5 million excluding development costs, but is also not entitled to any future milestone payments or royalties.

# Strategy and business development

*CombiGene develops groundbreaking gene therapies with the ambition to offer patients affected by severe life-changing diseases opportunities for a better life. We source research assets from industry or academia and develop them through the preclinical phase up to preclinical/clinical proof-of-concept and then out-license them to a Big Pharma company for clinical development and commercialization.*

### Gene therapy has fantastic medical possibilities

There are a large number of diseases that today either require lifelong medical treatment or that completely lack effective therapies. It is precisely these diseases that are in focus for the development since gene therapy has the unique possibility of being able to replace defective/missing genes or change the expression of existing genes. This means that gene therapy in some cases can cure a disease instead of only alleviating the symptoms and that you can achieve long-term effects from one or a few treatments. There are currently about 300 gene therapy clinical studies conducted in the central nervous system, infectious and metabolic diseases among others.

### The commercial possibilities of gene therapy

Gene therapy is not just an interesting field of research. With two gene therapies approved in the second quarter of 2023, there are currently nine gene therapies approved in the EU and/or in the US and, according to the Alliance for Regenerative Medicines, and another three gene therapies may be approved in 2023. The US Food and Drug Administration (FDA) has previously announced that they expect to approve 10 to 20 new cell and gene therapies annually from 2025 onwards. According to Precedence Research, the gene therapy market is expected to grow globally to USD 15.7 billion in 2030.

### Extensive work to find new projects

CombiGene is currently working intensely to find interesting new projects to complement the current project portfolio. The evaluation of potential projects is a structured and rigorous process based on several key criteria. The work includes review and analysis of intellectual property issues, preclinical data, intended contract structure, size of patient population and medical need, competitive situation, and the project's commercial conditions.

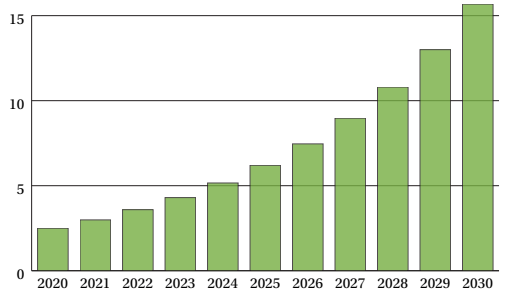
All criteria are important and a weakness in one of them, such as an unclear intellectual property situation, means that CombiGene chooses not to proceed with the project.

CombiGene has identified a number of projects that could be interesting to license. These include projects for diseases of the central nervous system, endocrine diseases, and genetic muscle diseases. CombiGene is currently conducting in-depth analyses of these projects.

### The importance of a broad portfolio

Thanks to the outlicensing of the epilepsy project CG01 to Spark Therapeutics in the autumn of 2021, CombiGene's financial position was strengthened, which enabled us to focus on the in-licensing of additional projects. The first concrete result of this is the cooperation agreement with Zyneyro that was signed in early 2023. We are now continuing to seek new projects to in-license with the ambition to build a broad portfolio that includes projects in several phases of drug development, ranging from projects in early preclinical evaluation to projects in clinical development. By having a broad portfolio of projects, we increase the chances of achieving commercial success.

**Expected gene therapy sales 2020–2030, MUSD**
Source: Precedence Research



# The CombiGene share

*CombiGene is a public company and is listed on Nasdaq First North Growth market. The share capital of the Company shall amount to no less than SEK 990,000 and no more than SEK 3,960,000 divided into no less than 19,800,000 shares and no more than 79,200,000 shares. CombiGene has one class of share. Each share carries equal rights to CombiGene's assets and profits and is entitled to one vote at the Annual General Meeting (AGM). The quota value is SEK 0.05. The CombiGene share register is maintained electronically by Euroclear. The share trades under the name CombiGene, the ticker is COMBI, and the ISIN-Code is SE0016101935.*

## 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.

## Authorization to issue new shares, warrants or convertibles

The AGM 2023 resolved, in accordance with the Board of Directors' proposal, to authorize the Board of Directors to, at one or several occasions and for the period up until the next AGM, resolve to increase the Company's share capital by issuing new shares, warrants and/or convertibles. Such issue resolution may be carried out with or without deviation from the shareholders' pre-emption rights and with or without provisions for contribution in kind, set-off or other conditions. The total number of shares that may be issued, or in the event of an issue of warrants or convertibles, any additional shares after conversion or exercise of any warrant, by virtue of the authorization, for issue resolutions made without deviation from the shareholders' pre-emption rights, shall not be limited in any other way than by the limits for the share capital and number of shares, as set forth from time to time in the registered Articles of Association.

For issue resolutions made by virtue of the authorization, with deviation from the shareholders' pre-emption rights, the total number of shares that may be issued, or in the event of an issue of warrants or convertibles, any additional shares after conversion or exercise of any warrant, shall be limited to 50 percent of the outstanding shares in the Company at any given time. Should the Board of Directors resolve on a share issue with deviation from the shareholders' pre-emption rights, the reason for this shall be to broaden the ownership structure, procure working capital, increase the liquidity of the share, or acquire businesses, or enable the acquiring of capital for acquisitions.

## LTI 2022

The AGM 2022 resolved, in accordance with the board of directors' proposal, on the implementation of a performance-based incentive program, named LTI 2022, directed issue of 900,000 warrants, transfer of the warrants to participants in LTI 2022 and transfer of warrants to cover costs for LTI 2022 and authorization to enter into swap agreement. A more detailed description of LTI 2022 can be found in the notice convening the Annual General Meeting 2022.

| Ten largest shareholders as of June 30, 2023 | Total holdings | Holding % |
|---|---|---|
| Pareto Securities AS | 1,618,372 | 8.17% |
| Nordqvist, Jan Ivar | 1,511,587 | 7.63% |
| Avanza Pension | 1,098,776 | 5.55% |
| Thoren Tillväxt AB | 494,894 | 2.50% |
| Nordnet Pensionsförsäkring AB | 464,316 | 2.34% |
| Försäkringsaktiebolaget Skandia | 272,777 | 1.38% |
| Thomassen Skaar, Christian | 261,825 | 1.32% |
| Olsson, Per Magnus | 249,669 | 1.26% |
| Ferstad, Arne | 214,072 | 1.08% |
| Darlista, Flamur | 191,934 | 0.97% |

# Financial information

### Income and earnings
Net sales consist of milestone payments and compensation from license and cooperation agreements. For January-September, the net sales consist of compensation from Spark regarding costs during the preclinical development of CG01, and compensation from Zyneyro for 50 percent of the costs for the COZY program incurred during the period. 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 TSEK 4,948 (21,354) during the period January-September. The decrease is explained by CombiGene, as planned, putting less resources into CG01 as Spark takes increasingly greater responsibility for the project. Other operating revenues amounts to TSEK 588 (20,888) and consist of TSEK 518 (1,440) which refers to the revenue-earned portion of the grant received from Eurostars. Other operating revenues also consist of realized and unrealized foreign exchange gains. Operating profit for the period amounted to TSEK -29,305 (5,785). The main costs during the period have been related to research & development, fees for consultants and personnel costs, and initial payment of DKK 5 million to Zyneyro.

### Cash flow and financial position
Cash flow for the period January-September amounts to TSEK -23,673 (-10,412). Cash and cash equivalents at the end of the period amounts to TSEK 107,187 (144,940). The equity ratio is 95.8% (96.3).

### 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 4.1 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.

### Incentive programs and warrants
The 2022 Annual General Meeting resolved on a performance-based incentive program (LTI 2022). The duration of the program is approximately three years and will be offered to certain employees and consultants, or newly hired persons, in the company. A maximum of 617,220 Performance Share Rights may be allocated to the participants, corresponding to approximately 3 percent of the out-standing shares and votes in the Company, as well as 282,780 warrants that can be issued to hedge the Company's cost under the Program, which corresponds to approximately 1.4 percent of the outstanding shares and votes in the Company. In accordance with the Board's proposal, the AGM resolved on a directed issue of 900,000 warrants with the right to subscribe for new shares in the company for the implementation of LTI 2022.

### Employees
The number of employees in the Group at the end of the period was 11 (11), of whom 6 (6) 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. 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.

### Deviation from Interim report January-September 2022
The comparative figures for the periods July-September and January-September last year differ from the interim report for January-September 2022. In the Group's and the Parent Company's reports on profit for the period, exchange rate differences in Other operating income and Other operating expenses have been reported net for both 2023 and 2022.

### Review by auditors
This report has not been subject to review by the Company's auditors.

### Future reporting dates
Year-end report 2023, 16 February 2024.

### For further information, please contact:
CombiGene AB (publ), Peter Ekolind, CEO
Phone: +46 (0)8 35 73 55
Email: peter.ekolind@combigene.com

# Group income statement in summary

| Figures in TSEK | 2023 Jul-Sep | 2022 Jul-Sep | 2023 Jan-Sep | 2022 Jan-Sep | 2022 Jan-Dec |
|---|---|---|---|---|---|
| **Operating income** | | | | | |
| Net sales | 992 | 5,213 | 4,948 | 21,354 | 26,699 |
| Other operating revenues | 173 | 8,285 | 588 | 20,888 | 15,044 |
| | | | | | |
| **Operating expenses** | | | | | |
| Other external expenses | -5,985 | -5,912 | -22,527 | -25,698 | -32,567 |
| Personnel expenses | -2,804 | -2,985 | -10,364 | -8,811 | -13,032 |
| Other operating expenses | -127 | 0 | 0 | 0 | -496 |
| **Profit/loss before depreciation** | **-7,750** | **4,602** | **-27,356** | **7,731** | **-4,352** |
| | | | | | |
| Depreciation | -649 | -649 | -1,946 | -1,946 | -2,595 |
| **Profit/loss after depreciation** | **-8,399** | **3,953** | **-29,302** | **5,785** | **-6,947** |
| | | | | | |
| Net financial income/expense | -4 | 0 | -4 | 0 | 790 |
| **Income after net financial items** | **-8,403** | **3,953** | **-29,305** | **5,785** | **-6,157** |
| | | | | | |
| Tax | 0 | 0 | 0 | 0 | 0 |
| **Net profit/loss for the period** | **-8,403** | **3,953** | **-29,305** | **5,785** | **-6,157** |
| | | | | | |
| Attributable to | | | | | |
| Parent company shareholders | -8,403 | 3,953 | -29,305 | 5,785 | -6,157 |
| | | | | | |
| Earnings per share before dilution | -0.42 | 0.20 | -1.48 | 0.29 | -0.31 |
| Earnings per share after dilution | -0.42 | 0.20 | -1.48 | 0.29 | -0.31 |
| | | | | | |
| Average number of shares before dilution | 19,801,197 | 19,801,197 | 19,801,197 | 19,801,197 | 19,801,197 |
| Average number of shares after dilution | 19,801,197 | 19,801,197 | 19,801,197 | 19,801,197 | 19,801,197 |
| *Total outstanding shares* | *19,801,197* | *19,801,197* | *19,801,197* | *19,801,197* | *19,801,197* |

# Group balance sheet in summary

| Figures in TSEK | 2023 30 Sep | 2022 30 Sep | 2022 31 Dec |
|---|---|---|---|
| **ASSETS** | | | |
| **Fixed assets** | | | |
| Intangible fixed assets | 17,167 | 19,652 | 19,004 |
| Financial fixed assets | 5 | 0 | 0 |
| **Total fixed assets** | **17,171** | **19,652** | **19,004** |
| | | | |
| **Current assets** | | | |
| Accounts receivable | 183 | 0 | 4,216 |
| Other receivables | 3,706 | 5,608 | 3,223 |
| Cash and cash equivalents | 107,187 | 144,940 | 131,777 |
| **Total current assets** | **111,076** | **150,548** | **139,217** |
| | | | |
| **TOTAL ASSETS** | **128,248** | **170,200** | **158,221** |
| | | | |
| | | | |
| **SHAREHOLDERS' EQUITY AND LIABILITIES** | | | |
| Share capital | 990 | 990 | 990 |
| Other capital contribution | 224,124 | 224,124 | 224,124 |
| Other shareholders' equity | -72,992 | -66,835 | -66,835 |
| Profit/loss for the period | -29,305 | 5,785 | -6,157 |
| **Equity attributable to parent company shareholders** | **122,817** | **164,064** | **152,122** |
| | | | |
| **Total equity** | **122,817** | **164,064** | **152,122** |
| | | | |
| **LIABILITIES** | | | |
| Current liabilities | 5,431 | 6,136 | 6,099 |
| **Total liabilities** | **5,431** | **6,136** | **6,099** |
| | | | |
| **TOTAL SHAREHOLDERS' EQUITY AND LIABILITIES** | **128,248** | **170,200** | **158,221** |

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

| Figures in TSEK | Share capital | Other capital contribution | Accumulated profit/loss | Total shareholders' equity |
|---|---|---|---|---|
| **Balance brought forward** | **990** | **224,124** | **-72,992** | **152,122** |
| Net profit/loss for the period | | | -29,305 | -29,305 |
| **Amount as per the end of the reporting period** | **990** | **224,124** | **-102,297** | **122,817** |

# Group cash flow statement in summary

| Figures in TSEK | 2023 Jan-Sep | 2022 Jan-Sep | 2022 Jan-Dec |
|---|---|---|---|
| Cash flow from operating activities | -23,559 | -10,412 | -16,666 |
| Cash flow from investing activites | -114 | 0 | 0 |
| Cash flow from financing activities | 0 | 0 | 0 |
| | | | |
| **Cash flow for the period** | **-23,673** | **-10,412** | **-16,666** |
| | | | |
| Liquid assets at the beginning of the reporting period | 131,777 | 136,744 | 136,744 |
| Exchange rate difference cash and cash equivalents | -917 | 18,609 | 11,699 |
| **Liquid assets at the end of the reporting period** | **107,187** | **144,940** | **131,777** |

# Group financial key ratios

| | 2023 Jan-Sep | 2022 Jan-Sep | 2022 Jan-Dec |
|---|---|---|---|
| Earnings per share before dilution, SEK | -1.48 | 0.29 | -0.31 |
| Earnings per share after dilution, SEK | -1.48 | 0.29 | -0.31 |
| Shareholders' equity per share, SEK | 6.20 | 8.29 | 7.68 |
| Equity ratio, % | 95.77 | 96.39 | 96.15 |
| Average number of shares before dilution | 19,801,197 | 19,801,197 | 19,801,197 |
| Average number of shares after dilution | 19,801,197 | 19,801,197 | 19,801,197 |
| Total outstanding shares | 19,801,197 | 19,801,197 | 19,801,197 |

# Parent Company income statement in summary

| Figures in TSEK | 2023 Jul-Sep | 2022 Jul-Sep | 2023 Jan-Sep | 2022 Jan-Sep | 2022 Jan-Dec |
|---|---|---|---|---|---|
| **Operating income** | | | | | |
| Net sales | 992 | 5,213 | 4,948 | 21,354 | 26,699 |
| Other operating revenues | 173 | 8,315 | 594 | 20,890 | 15,044 |
| | | | | | |
| **Operating expenses** | | | | | |
| Other external expenses | -5,967 | -5,905 | -22,492 | -25,640 | -32,494 |
| Personnel expenses | -2,804 | -2,985 | -10,364 | -8,811 | -13,032 |
| Other operating expenses | -136 | 0 | 0 | 0 | -492 |
| **Profit/loss before depreciation** | **-7,741** | **4,638** | **-27,315** | **7,792** | **-4,275** |
| | | | | | |
| Depreciation | -75 | -75 | -225 | -225 | -300 |
| **Profit/loss after depreciation** | **-7,816** | **4,563** | **-27,540** | **7,567** | **-4,575** |
| | | | | | |
| Net financial income/expense | -578 | -574 | -1,725 | -1,721 | -1,505 |
| **Income after net financial items** | **-8,393** | **3,989** | **-29,265** | **5,845** | **-6,080** |
| | | | | | |
| Tax | 0 | 0 | 0 | 0 | 0 |
| **Net profit/loss for the period** | **-8,393** | **3,989** | **-29,265** | **5,845** | **-6,080** |

# Parent Company balance sheet in summary

| Figures in TSEK | 2023 30 Sep | 2022 30 Sep | 2022 31 Dec |
|---|---|---|---|
| **ASSETS** | | | |
| Intangible fixed assets | 3,971 | 4,162 | 4,087 |
| Financial fixed assets | 17,482 | 19,159 | 18,585 |
| **Total fixed assets** | **21,453** | **23,321** | **22,673** |
| | | | |
| **Current assets** | | | |
| Accounts receivable | 183 | 0 | 4,216 |
| Other receivables | 3,907 | 6,354 | 3,980 |
| Cash and cash equivalents | 106,983 | 144,747 | 131,583 |
| **Total current assets** | **111,074** | **151,101** | **139,779** |
| | | | |
| **TOTAL ASSETS** | **132,527** | **174,422** | **162,452** |
| | | | |
| | | | |
| **SHAREHOLDERS' EQUITY AND LIABILITIES** | | | |
| **Restricted equity** | | | |
| Share capital | 990 | 990 | 990 |
| Statutory reserve | 4 | 4 | 4 |
| Reserve for development expenses | 868 | 760 | 760 |
| | | | |
| **Non-restricted equity** | | | |
| Share premium reserve | 165,826 | 165,826 | 165,826 |
| Accumulated loss including profit/loss for the period | -40,554 | 745 | -11,181 |
| **Total shareholders' equity** | **127,133** | **168,323** | **156,398** |
| | | | |
| **LIABILITIES** | | | |
| Current liabilities | 5,393 | 6,099 | 6,054 |
| **Total liabilities** | **5,393** | **6,099** | **6,054** |
| | | | |
| **TOTAL SHAREHOLDERS' EQUITY AND LIABILITIES** | **132,527** | **174,422** | **162,452** |

# 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 |
|---|---|---|---|---|---|---|
| **Balance brought forward** | **990** | **4** | **760** | **165,826** | **-11,181** | **156,398** |
| Provisions for reserve for development expenses | | | 109 | | -109 | 0 |
| Net profit/loss for the period | | | | | -29,265 | -29,265 |
| **Amount as per the end of the reporting period** | **990** | **4** | **868** | **165,826** | **-40,554** | **127,133** |

# Parent Company cash flow statement in summary

| Figures in TSEK | 2023 Jan-Sep | 2022 Jan-Sep | 2022 Jan-Dec |
|---|---|---|---|
| Cash flow from operating activities | -23,569 | -10,407 | -16,661 |
| Cash flow from investing activites | -114 | 0 | 0 |
| Cash flow from financing activities | 0 | 0 | 0 |
| | | | |
| **Cash flow for the period** | **-23,683** | **-10,407** | **-16,661** |
| | | | |
| Liquid assets at the beginning of the reporting period | 131,583 | 136,545 | 136,545 |
| Exchange rate difference cash and cash equivalents | -917 | 18,609 | 11,699 |
| **Liquid assets at the end of the reporting period** | **106,983** | **144,747** | **131,583** |

# 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 |
| **At the end of the period** | | **990,060** | | **19,801,197** | | **0.05** |

# Declaration by the Board of Directors and the CEO


Jonas Ekblom


Malin Almgren


Gunilla Lundmark


Per Lundin


Peter Nilsson


Peter Ekolind

The Board of Directors and the Chief Executive Officer certify that the interim report provides a true and fair view of the company's business, financial position, performance and describes material risks and uncertainties, to which the company is exposed.

The interim report has not been reviewed by the company's auditors.

Stockholm, November 10, 2023

**Jonas Ekblom**
Chairman

**Malin Almgren**
Board member

**Gunilla Lundmark**
Board member

**Per Lundin**
Board member

**Peter Nilsson**
Board member

**Peter Ekolind**
CEO

# Glossary

**AAV** Adeno-associated virus.

**AMPA receptor** A transmembrane receptor subtype for glutamate that acts as an ion channel and mediates fast synaptic signal transmission in the central nervous system (CNS). AMPA receptors are also present in peripheral nerves and may play a role in pain signaling.

**C-kinase** A family of protein kinase enzymes that are involved in controlling the function of other proteins through the phosphorylation of hydroxyl groups of serine and threonine amino acid residues on these proteins, or a member of this family.

**CDMO** Contract development and manufacturing organization is a company that serves other companies in the pharmaceutical industry on a contract basis to provide comprehensive services from drug development through drug manufacturing.

**Chronic pain** Pain that has lasted longer than three to six months. In some cases, the chronic pain may disappear at a later stage. Thus, chronic pain is not necessarily permanent.

**Clinical development** Comprises three phases, see clinical phase I, II, III below.

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

**CRO** Contract Research Organization is a company that provides support to the pharmaceutical, biotechnology, and medical device industries in the form of research services outsourced on a contract basis.

**Eurostars** A funding instrument that supports innovative SMEs (Small and Medium-sized Enterprises), and project partners (large companies, universities, research organizations and other types of organizations) by funding international collaborative R&D and innovation projects.

**Gene therapy** A medical field which focuses on the genetic modification of cells to produce a therapeutic effect or the treatment of disease by repairing or reconstructing defective genetic material.

**GMP** Good Manufacturing Practice 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, for example cultivated cells, 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.

**Lipodystrophy** A rare disease characterized by altered fat distribution on the body. In the absence of normal body fat, various organs, primarily the liver, begin to accumulate fat, leading on to serious metabolic complications, including extreme insulin resistance, hypertriglyceridemia (elevated values of blood fat triglyceride) and liver steatosis (fatty liver).

**Neuropathic pain** Nerve pain can occur after diseases and injuries of the somatosensory nervous system and spread within a neuroanatomical innervation area. The term neuropathic pain is usually associated with pain that persists after healing of the initial insult.

**PCT** Patent Cooperation Treaty, an international patent law treaty, concluded in 1970. It provides a unified procedure for filing patent applications to protect inventions in each of its contracting states.

**Peptide** Short chains of amino acids linked by peptide bonds.

**PICK1** A protein that interacts with C-kinase 1.

**Plasmid** Small, extrachromosomal DNA molecule within a cell that is physically separated from chromosomal DNA.

**Preclinical study** In vitro and in vivo studies carried out before the clinical development (see above) with the objective to make sure that the new therapy is safe and has the intended effect.

**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.

CombiGene

CombiGene's vision is to provide patients affected by severe diseases with the prospect of a better life through gene therapy and other forms of advanced treatments. Our business has three focus areas: sourcing of new and promising assets, development of these assets to proof of concept under our management and expertise, and outlicensing of the assets to a strategic partner for continued development and commercialization. Revenue is achieved through milestone payments and royalties. The company is public and listed on the Swedish marketplace Nasdaq First North Growth Market. The company's Certified Advisor is FNCA Sweden AB.

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