CombiGene

Q4

Year-end Report

January – December 2024

The company is public and listed on the Swedish marketplace Nasdaq First North Growth Market. The company's Certified Adviser is Västra Hamnen Corporate Finance 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 projects COZY01 and CGT2 have received funding from the Eurostars programme, co-financed by the European Union's research and innovation programme Horizon Europe. Projects ID: 4408 and 114714, respectively.

Summary of the Report

Events during the first quarter of 2024

- CombiGene has regained the global rights to the epilepsy project CG01 from Spark Therapeutics as the termination period for the collaboration and license agreement between the two companies has expired.
- CombiGene concludes the preclinical development of the lipodystrophy project CGT2.
- CombiGene's epilepsy project CG01 has been granted patents in two new countries, Australia and India.

Events during the second quarter of 2024

- CombiGene initiates a collaboration with Västra Hamnen Corporate Finance, which includes commissioned analysis, news flow monitoring, and web conferences in connection with CombiGene's quarterly reports.
- The 2024 Annual General Meeting decided, in accordance with the shareholder Orphazyme A/S's proposal, to re-elect Jonas Ekblom, Peter Nilsson, Per Lundin, Gunilla Lundmark, and Malin Almgren, and to elect Marcus Isaksson as a new member.
- Annika Ericsson, the current Director of Preclinical Development, takes over the role of Chief Scientific Officer after Karin Agerman, who has decided to leave the company.
- CombiGene switches to Västra Hamnen Corporate Finance as Certified Adviser from August 25, 2024.
- CombiGene enters into a license agreement with Spark Therapeutics for certain intellectual property developed or used by Spark in connection with the previous license agreement for CG01.
- CombiGene and the Royal Institute of Technology (KTH) receive a grant from Vinnova totaling 1 million SEK.

Events during the third quarter of 2024

- CombiGene announces new preclinical research results in the epilepsy project.

Events during the fourth quarter of 2024

- CombiGene initiates measures to extend the liquidity horizon by sharpening the strategic focus on gene therapy, prioritizing pipeline assets, and implementing a cost reduction program.
- CombiGene holds an extraordinary general meeting. The meeting decided, in accordance with the shareholder Strategic Partner A/S's proposal, that the board should consist of three ordinary board members with one deputy board member. Luca Di Stefano and Jakob Bendtsen were elected, and Marcus Isaksson was re-elected, as board members. Furthermore, Lars Thunberg was elected as a new deputy board member. The meeting also decided, in accordance with the shareholder Strategic Partner A/S's proposal, to amend the articles of association. The amendment was made to expand the business purpose, allowing the company to enter strategic partnerships with other companies in other industries.

Summary of the Report, continued

Events after the end of the period

- CombiGene AB initiates restructuring as part of a strategic review to maximize shareholder value and terminates all employment, including the CEO. The severance cost for the staff is estimated to amount to approximately SEK 6.8 million.
- CombiGene AB discontinues all activities and collaborations linked to research. The winding-up cost for operations and projects is estimated to amount to SEK 0.9 million.

From 2025, the company will no longer publish interim reports for the first and third quarters.

Financial information

	2024 Oct–Dec	2023 Oct–Dec	2024 Jan–Dec	2023 Jan–Dec
Net sales, TSEK	0	596	326	5,544
Other operating income, TSEK	1,962	875	3,274	1,464
Result after financial items, TSEK	-14,157	-6,360	-44,878	-35,665
Earnings per share, SEK	-0,71	-0.32	-2.27	-1.80
Cash and cash equivalents at the end of the period, TSEK	73,748	101,440	73,748	101,440

Focus on strategic transformation

The quarter has been marked by fundamental strategic decisions for CombiGene. After an analysis of both the capital market for biotech and the conditions for our ongoing research projects, the Board has decided to discontinue our research operations and lay off all employees.

The Board of CombiGene has concluded that the financial resources required to take the projects to the commercial phase are significant. In a risk averse market, the Board has therefore assessed that it is not possible to secure the necessary financing.

As a consequence of this decision, the Board has also chosen to terminate our agreement with Zyneyro and divest the company's ownership in CCRM Nordic AB. This means that CombiGene will no longer have any rights or obligations linked to the pain program, which gives the company flexibility and the opportunity to evaluate new strategic directions.

I would like to extend a big thank you to everyone who has been a part of CombiGene's journey – our employees, former Board, partners and shareholders. CombiGene is entering a new phase and the Board's top priority now is to re-orient the company and identify new future opportunities for the company's shareholders. I wish the Board every success in this mission.

Peter Ekolind
CEO

CombiGene enters a new phase

CombiGene is facing a completely new phase. After a strategic review, the company has adjusted its focus and cost structure to ensure value creation in a different direction for our shareholders. With a cash position of SEK 58–63 million (SEK 2.93–3.18 per share) after liquidation costs and subsequently a continuous positive cash flow from interest income, we have a strong financial foundation and the ability to act with both flexibility and power.

Our top priority now is to continuously evaluate investment opportunities. The Board has an open attitude to investments, collaborations and potential acquisitions, but we will only act if the right conditions are met. Thanks to our financial stability, we can conduct dialogues without external time pressure, which gives us room to maneuver to make decisions that create value on our terms. If no sufficiently attractive alternatives are identified, a dividend to shareholders can be considered.

Finally, I would like to extend a big thank you to former employees, CEO and previous Board for your efforts in developing CombiGene. Your work has been completely in line with what the shareholders expected. The canceled agreement with Spark, in combination with a capital need in COZY that far exceeds existing cash were simply deemed too overwhelming for current shareholders.

Luca Di Stefano
Chairman of the Board, CombiGene AB

The CombiGene share

The number of shares at the end of the period amounts to 19,801,197. The average number of shares for the period is 19,801,197. The quota value is SEK 0.05. All shares are of the same type and have the same voting rights.

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 December 31, 2024

	Total holdings	Holding %
Strategic Partner A/S	2,327,058	11.75%
Nordqvist, Ivar	1,960,994	9.90%
Molse, Oliver	1,655,372	8.36%
Avanza Pension	1,029,447	5.20%
M&L Industriförvaltning AB	650,000	3.28%
Thoren Tillväxt AB	494,894	2.50%
Nordnet Pensionsförsäkring AB	392,141	1.98%
Ferstad, Arne	302,000	1.53%
Olsson, Per Magnus	262,491	1.33%
Darlista, Flamur	200,842	1.01%
Övriga aktieägare	10,525,958	53.16%
Total number of shares	**19,801,197**	**100%**

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 CombiGene share register is maintained electronically by Euroclear.

Share name: CombiGene
Ticker symbol: COMBI
ISIN-Code: SE0016101935

Financial Information

Revenue and Results

Net sales consist of milestone payments and compensation from license and collaboration agreements. During January-December 2024, it consists of compensation from Spark Therapeutics for ongoing costs for the preclinical development of CG01. Due to the nature of the business, there can be significant fluctuations between revenues for different periods as milestone payments are recognized when performance obligations are met. The Group has generated SEK 326 (5,544) thousand during the period January-December. The decrease is explained by the termination of the collaboration agreement with Spark. Other operating income amounts to SEK 3,274 (1,464) thousand, of which SEK 1,255 (0) thousand relates to recognized income from the Eurostars grant for COZY01, SEK 212 (0) thousand relates to recognized income for COZY02, and SEK 200 (518) thousand relates to recognized income from the grant for CGT2. Other operating income also consists of realized and unrealized exchange rate differences. The operating result for the period amounts to SEK -47,218 (-38,600) thousand. The main costs during the period have been related to research & development, consultant fees, and personnel costs, as well as a contractual payment of DKK 5 million, equivalent to SEK 7.5 million, to Zyneyro. In connection with the return of the rights for the CGT2 project to Lipigon Pharmaceuticals AB, the assets related to the project were written down to zero, resulting in an impact of SEK -2,040 thousand. In connection with the completion of the preclinical development of the epilepsy program CG01, the assets related to the project were written down to zero, resulting in an impact of SEK -12,554 thousand.

Cash Flow and Financial Position

Cash flow for the period January-December amounts to SEK -30,030 (-31,551) thousand. Cash and cash equivalents at the end of the period amount to SEK 73,748 (101,440) thousand. The equity ratio is 94.3 (96.6) percent.

Liquidity and Financing

The EU's Eurostars program, which targets small and medium-sized enterprises that want to collaborate on research and development projects, has awarded the CGT2 project development grants. The total grant for CombiGene amounts to SEK 5 million, and in August, CombiGene received the final payment of SEK 200 thousand. The Eurostars program has also awarded the COZY01 project development grants. The total grant for CombiGene amounts to SEK 5 million, of which SEK 1,875 thousand has been paid out so far. Within the COZY02 project, CombiGene, together with the Royal Institute of Technology (KTH), has received a grant from Vinnova of SEK 1 million, of which SEK 450 thousand has been paid out. The board and management continuously evaluate options to ensure the company's financing in the short and medium term.

Incentive Programs and Warrants

The 2022 Annual General Meeting decided on a performance-based incentive program (LTI 2022). The program's duration is three years and is offered to certain employees and consultants, or newly hired persons, in the company. A maximum of 617,220 Performance Share Rights can be allocated to participants, corresponding to approximately 3 percent of outstanding shares and votes in the company, and 282,780 warrants can

Financial information, continued

be issued to secure the company's cost under the program, corresponding to approximately 1.4 percent of outstanding shares and votes in the company. In accordance with the board's proposal, the meeting decided 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.

Personnel

The number of employees in the Group at the end of the period was 9 (11), of which 5 (6) are women.

Significant Events After the End of the Period

CombiGene AB initiates restructuring as part of a strategic review to maximize shareholder value and terminates all employment, including the CEO. The severance cost for the staff is estimated to amount to approximately SEK 6.8 million. CombiGene discontinues all operations and all collaborations related to research. The winding-up cost for operations and projects is estimated to amount to SEK 0.9 million.

Principles for the Preparation of the Interim Report

CombiGene applies the Annual Accounts Act and the Swedish Accounting Standards Board's general advice BFNAR 2012:1 (K3) in the preparation of its financial reports. The same accounting principles have been used in this interim report as in the latest annual report.

Annual General Meeting and Annual Report

The 2025 Annual General Meeting will be held on June 17. More information about the implementation of the Annual General Meeting will be presented at a later date. The annual report will be available to the public at the company's office in Stockholm and published on the company's website no later than three weeks before the meeting.

Review by Auditor

This report has not been reviewed by the company's auditor.

Upcoming Financial Reports

Interim report January-June 2025, August 22, 2025.
Year-end report 2025, February 13, 2026.

For further information:

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Group income statement in summary

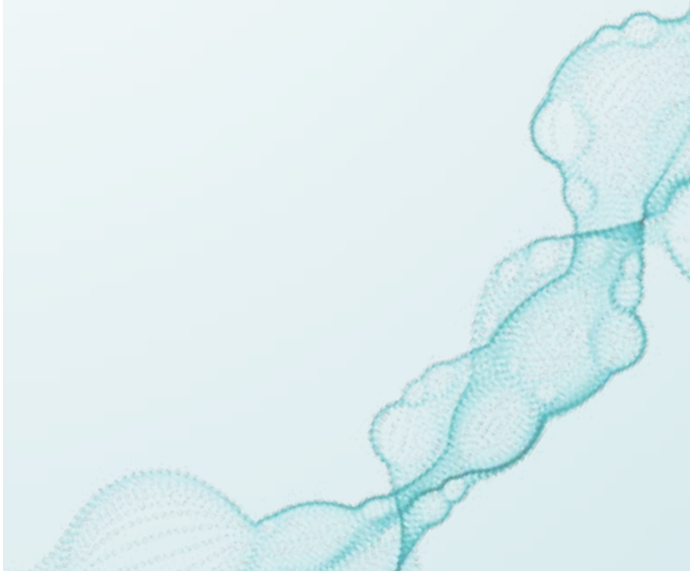
Figures in TSEK	2024 Oct–Dec	2023 Oct–Dec	2024 Jan–Dec	2023 Jan–Dec
Operating income				
Net sales	0	596	326	5,544
Other operating revenues	1,962	875	3,274	1,464
Operating expenses				
Other external expenses	-2,230	-4,307	-19,567	-26,835
Personnel expenses	-3,630	-4,504	-14,541	-14,868
Other operating expenses	-1,654	-1,281	-3,712	-1,281
Profit/loss before depreciation	**-5,552**	**-8,620**	**-34,220**	**-35,976**
Depreciation and amortisation of tangible and intangible assets	-10,944	-678	-12,998	-2,624
Profit/loss after depreciation	**-16,496**	**-9,298**	**-47,218**	**-38,600**
Net financial income/expense	2,339	2,938	2,340	2,935
Income after net financial items	**-14,157**	**-6,360**	**-44,878**	**-35,665**
Tax	0	0	0	0
Net profit/loss for the period	**-14,157**	**-6,360**	**-44,878**	**-35,665**
Attributable to				
Parent company shareholders	-14,157	-6,360	-44,878	-35,665
Earnings per share before dilution	-0.71	-0.32	-2.27	-1.80
Earnings per share after dilution	-0.71	-0.32	-2.27	-1.80
Average number of shares before dilution	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
Total outstanding shares	*19,801,197*	*19,801,197*	*19,801,197*	*19,801,197*

Group balance sheet in summary

Figures in TSEK	2024 31 Dec	2023 31 Dec
ASSETS		
Fixed assets		
Intangible fixed assets	0	16,518
Tangible fixed assets	675	851
Financial fixed assets	86	5
Total fixed assets	**761**	**17,373**
Current assets		
Other receivables	1,370	1,799
Cash and cash equivalents	73,748	101,440
Total current assets	**75,118**	**103,239**
TOTAL ASSETS	**75,879**	**120,612**
SHAREHOLDERS' EQUITY AND LIABILITIES		
Share capital	990	990
Other capital contribution	224,124	224,124
Other shareholders' equity	-108,657	-72,992
Profit/loss for the period	-44,878	-35,665
Equity attributable to parent company shareholders	**71,579**	**116,457**
Total equity	**71,579**	**116,457**
LIABILITIES		
Current liabilities	4,300	4,156
Total liabilities	**4,300**	**4,156**
TOTAL SHAREHOLDERS' EQUITY AND LIABILITIES	**75,879**	**120,612**

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

Figures in TSEK	Share capital	Other capital contribution	Other shareholders' equity	Accumulated profit/loss	Total shareholders' equity
Balance brought forward	**990**	**224 124**	**-72 992**	**-35 665**	**116 457**
Allocation of profit/loss			-35,665	35,665	0
Net profit/loss for the period				-44,878	-44,878
Amount as per the end of the reporting period	**990**	**224,124**	**-108,657**	**-44,878**	**71,579**



Group cash flow statement in summary

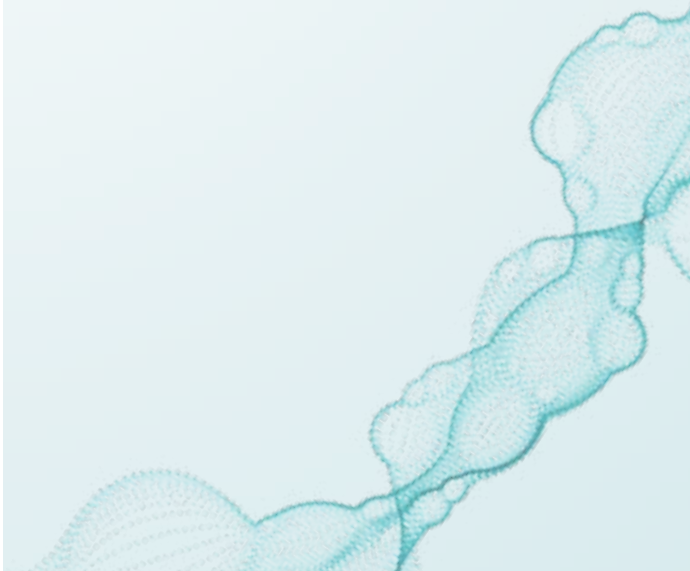
Figures in TSEK	2024 Oct–Dec	2023 Oct–Dec	2024 Jan–Dec	2023 Jan–Dec
Cash flow from operating activities	-6,231	-6,998	-29,949	-30,557
Cash flow from investing activites	0	-880	-81	-994
Cash flow from financing activities	0	0	0	0
Cash flow for the period	**-6,231**	**-7,878**	**-30,030**	**-31,551**
Liquid assets at the beginning of the reporting period	76,685	107,187	101,440	131,777
Exchange rate difference cash and cash equivalents	3,294	2,130	2,338	1,213
Liquid assets at the end of the reporting period	**73,748**	**101,440**	**73,748**	**101,440**

Group financial key ratios

	2024 Oct–Dec	2023 Oct–Dec	2024 Jan–Dec	2023 Jan–Dec
Earnings per share before dilution, SEK	-0.71	-0.32	-2.27	-1.80
Earnings per share after dilution, SEK	-0.71	-0.32	-2.27	-1.80
Shareholders' equity per share, SEK	3.61	5.88	3.61	5.88
Equity ratio, %	94.33	96.55	94.33	96.55
Average number of shares before dilution	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
Total outstanding shares	*19,801,197*	*19,801,197*	*19,801,197*	*19,801,197*

Parent Company income statement in summary

Figures in TSEK	2024 Oct–Dec	2023 Oct–Dec	2024 Jan–Dec	2023 Jan–Dec
Operating income				
Net sales	0	596	326	5,544
Other operating revenues	1,967	870	3,274	1,464
Operating expenses				
Other external expenses	-2,249	-4,290	-19,557	-26,782
Personnel expenses	-3,630	-4,504	-14,541	-14,868
Other operating expenses	-2,604	-1,280	-4,646	-1,280
Profit/loss before depreciation	**-6,515**	**-8,607**	**-35,144**	**-35,922**
Depreciation	-44	-104	-376	-329
Profit/loss after depreciation	**-6,559**	**-8,712**	**-35,520**	**-36,252**
Net financial income/expense	-11,717	2,364	-13,438	639
Income after net financial items	**-18,276**	**-6,348**	**-48,958**	**-35,613**
Tax	0	0	0	0
Net profit/loss for the period	**-18,276**	**-6,348**	**-48,958**	**-35,613**



Parent Company balance sheet in summary

Figures in TSEK	2024 31 Dec	2023 31 Dec
ASSETS		
Intangible fixed assets		
Tangible fixed assets	0	3,896
Financial fixed assets	675	851
Total fixed assets	1,213	16,908
Summa anläggningstillgångar	**1,887**	**21,655**
Current assets		
Other receivables	1,627	2,006
Cash and cash equivalents	72,361	101,235
Total current assets	**73,988**	**103,241**
TOTAL ASSETS	**75,875**	**124,896**
SHAREHOLDERS' EQUITY AND LIABILITIES		
Restricted equity		
Share capital	990	990
Statutory reserve	4	4
Reserve for development expenses	0	868
Non-restricted equity		
Share premium reserve	165,826	165,826
Accumulated loss including profit/loss for the period	-94,991	-46,902
Total shareholders' equity	**71,828**	**120,786**
LIABILITIES		
Current liabilities	4,047	4,111
Total liabilities	**4,047**	**4,111**
TOTAL SHAREHOLDERS' EQUITY AND LIABILITIES	**75,875**	**124,896**

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	Accumu-lated profit/loss	Accumu-lated profit/loss for the year	Total share-holders' equity
Balance brought forward							
Allocation of profit/loss					-35,613	35,613	0
Provisions for reserve for development expenses			-868		868		0
Net profit/loss for the period						-48,958	-48,958
Amount as per the end of the reporting period	**990**	**4**	**0**	**165,826**	**-46,033**	**-48,958**	**71,828**

Parent Company cash flow statement in summary

Figures in TSEK	2024 Oct–Dec	2023 Oct–Dec	2024 Jan–Dec	2023 Jan–Dec
Cash flow from operating activities	-7,419	-6,998	-31,132	-30,568
Cash flow from investing activites	0	-880	-81	-994
Cash flow from financing activities	0	0	0	0
Cash flow for the period	**-7,419**	**-7,878**	**-31,213**	**-31,561**
Liquid assets at the beginning of the reporting period	76,486	106,983	101,235	131,583
Exchange rate difference cash and cash equivalents	3,294	2,130	2,338	1,213
Liquid assets at the end of the reporting period	**72,361**	**101,235**	**72,361**	**101,235**

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

The Board of Directors and the Chief Executive Officer certify that the year-end 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 year-end report has not been reviewed by the company's auditors.

Stockholm, February 14, 2025


Luca Di Stefano


Jakob Bendtsen


Marcus Isaksson


Lars Thunberg

Luca Di Stefano
Chairman

Peter Ekolind
CEO

Jakob Bendtsen
Board member

Marcus Isaksson
Board member

Lars Thunberg
Deputy member

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.

Neuronal tissue is the type of tissue that consists of nerve cells, also called neurons, and their supporting cells. This tissue is mainly found in the brain, spinal cord and nervous system.

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), retro virus 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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