

### INTERIM REPORT January – March 2022

# Decision on marketing approval for lecanemab is approaching

#### **EVENTS DURING THE FIRST QUARTER 2022**

• Eisai commenced submission of lecanemab data in Japan for a prior assessment consultation, with the objective of obtaining faster regulatory marketing approval.

#### EVENTS AFTER THE FIRST QUARTER

- AbbVie took a strategic business decision to end its collaboration with BioArctic regarding its alpha-synuclein portfolio, including ABBV-0805. BioArctic will now, in accordance with the license agreement, take back the project and evaluate the best way forward.
- A recently published article in Neurology and Therapy based on disease modeling suggests that lecanemab could delay the progression to Alzheimer's dementia by several years.

#### FINANCIAL SUMMARY JANUARY – MARCH 2022

- Net revenues for the period amounted to MSEK 3.7 (7.2)
- Operating profit amounted to MSEK -44.1 (-29.1)
- Profit for the period amounted to MSEK -44.1 (-29.1) and earnings per share before and after dilution were SEK -0.50 (-0.33)
- Cash flow from operating activities amounted to MSEK -39.7 (-37.5)
- Cash and cash equivalents at the end of the period amounted to MSEK 801 (960)

#### **KEY FINANCIAL PERFORMANCE INDICATORS**

	Q	1	Jan-Dec	
MSEK	2022	2021	2021	
Net revenues	3.7	7.2	23.1	
Other operating income	0.7	1.7	3.5	
Operating profit/loss	-44.1	-29.1	-139.7	
Operating margin, %	neg	neg	neg	
Profit/loss for the period	-44.3	-29.1	-119.8	
Earnings per share before dilution, SEK	-0.50	-0.33	-1.36	
Earnings per share after dilution, SEK	-0.50	-0.33	-1.36	
Equity per share, SEK	8.46	9.98	8.96	
Cash flow from operating activities	-39.7	-37.5	-140.5	
Cash flow from operating activities per share, SEK	-0.45	-0.43	-1.60	
Equity/assets ratio, %	88.0	87.3	87.9	
Return on equity, %	-5.77	-3.26	-14.13	
Share price at the end of the period, SEK	103.20	91.00	119.20	

Unless otherwise stated, this Interim report refers to the Group. Figures in parentheses refer to the corresponding period last year. The amounts stated are rounded, which sometimes leads to some totals not being exact.

# Comments from the CEO

A new, disease-modifying treatment for Alzheimer's disease could give patients and their families better lives. We look forward with excitement to the Clarity AD Phase 3 results expected in September, while we can state that a decision on marketing approval of lecanemab for early Alzheimer's disease is approaching.

Our partner Eisai has already submitted two thirds of its rolling application to the US Food and Drug Administration (FDA) under the Accelerated Approval program. Submission of the final part is expected during the second quarter of the year, and a decision on accelerated approval in the US could thus come as early as this year. Both a completed application and potential approval would mean that BioArctic is entitled to milestone payments from Eisai.

At the same time, the regulatory processes in the rest of the world are progressing at a rapid pace. In early March, Eisai announced that, with the help of BioArctic's employees, it had commenced submitting data to the Japanese Pharmaceuticals and Medical Devices Agency (PMDA) under a process called prior assessment consultation. This allows the agency to preview available data before the application for marketing approval is submitted with the aim to shorten application review time.

Our partner Eisai plans to submit applications for full marketing approval to relevant authorities in the US, the EU and Japan at the latest in the first quarter of 2023, pending Phase 3 topline data. There is thus potential that lecanemab will be first to receive full marketing approvals in all these markets already next year. Full marketing approval is important for broader patient access and reimbursement in the US. In China as well, the developments are positive, with more than 100 patients included in the Clarity AD Phase 3 study, which will meet the requirements for a future application for marketing approval.

If lecanemab is approved in Europe, BioArctic will have the right to market and sell the product in the Nordic region. In preparation for potential marketing approval, we are building our commercial organization, and it is gratifying to see the broad experience represented in this new organization.

At this year's AD/PD<sup>™</sup> congress several presentations about lecanemab generated significant interest among participants and our co-founder, Professor Lars Lannfelt, held a highly appreciated presentation on lecanemab's unique binding profile that also promoted greater understanding of the drug candidate's mechanism of action. Eisai gave an update on the development of lecanemab including a new dosage form. The goal is to be able to offer patients a weekly subcutaneous treatment instead of a biweekly infusion. The new possibility for administering the treatment will be evaluated in the open-label extension study that follows the large Phase 3 study, Clarity AD. Eisai also plans to evaluate longer time intervals, up to three months, between maintenance doses of lecanemab.



"Our partner Eisai states that there is potential for full marketing approvals of lecanemab in the US, the EU and Japan as early as 2023."

Moreover, a recently published article in Neurology and Therapy based on disease modeling suggests that lecanemab could delay the progression to Alzheimer's dementia by several years. Analyses such as these are important to understand the potential and long-term effects for patients, families and society offered by lecanemab treatment.

It is also gratifying to see that both our blood-brain barrier technology and our drug candidate TDP-43 against amyotrophic lateral sclerosis (ALS) have developed so well that they can now be combined and together become a new project in our project portfolio.

We are disappointed about AbbVie's strategic decision to terminate its collaboration with BioArctic regarding our alpha-synuclein portfolio. Our preclinical data and the Phase 1 study supports progression to Phase 2. We are convinced that ABBV-0805 has the potential to become a disease-modifying treatment for patients with Parkinson's disease. We will now, in accordance with the license agreement, take back the project and evaluate the best way forward.

In conclusion, I look with great confidence forward to the results from the Clarity AD study in the autumn, which will be of great significance for BioArctic and patients with Alzheimer's disease. Furthermore, I am pleased with the positive developments in several of our projects, and I see how our advances can be combined to strengthen our projects. I am proud to lead a strong organization that can keep focus both on what is happening here and now and on the breakthroughs in disorders of the central nervous system that are just around the corner.

Gunilla Osswald CEO, BioArctic AB

### **BioArctic in short**

BioArctic AB (publ) is a Swedish biopharma company developing new drugs based on groundbreaking research for patients with central nervous system disorders. For a global market, the aim is to generate transformative medicines that can stop or slow down the progression of Alzheimer's disease, Parkinson's disease and other neurological diseases. BioArctic was founded in 2003 based on innovative research from Uppsala University, Sweden. BioArctic's B-share is listed on Nasdaq Stockholm Mid Cap (ticker: BIOA B).

# Strategy for sustainable growth

BioArctic's vision is to generate innovative medicines that improve life for patients with disorders in the central nervous system. Our work is based on groundbreaking scientific discoveries, and the company's researchers collaborate with strategic partners such as research groups at universities and major pharmaceutical companies.

The company has scientific excellence and vast experience in developing drugs from idea to market. Under BioArctic's business model, the company at an early stage itself pursues project development and then, at an appropriate juncture, licenses commercial rights and late phase development to global pharmaceutical companies. In recent years, BioArctic has successfully developed high quality drug projects that have resulted in strategic license and partnership agreements in two major disease areas with high unmet medical need.

Three important cornerstones of BioArctic's strategy are:

- CONTINUE supporting the partnered projects with great market potential
- DEVELOP our own projects further, up to an appropriate time for partnership or exit
- EXPAND the portfolio with new projects and indications with high unmet medical need

## Operations

BioArctic conducts its research in four focus areas:

- Alzheimer's disease
- Parkinson's disease
- Other CNS disorders
- Blood-brain barrier technology

Neurodegenerative disorders are conditions in which cells in the brain degenerate and die. Normally the neurodegenerative processes begin long before any symptoms appear. Neurodegenerative disorders affect the lives of millions of people and constitute a growing global health care problem.

A key cause of Alzheimer's disease and Parkinson's disease is believed to be misfolding and aggregation of proteins. The spreading of aggregated soluble forms of proteins leads to neuronal dysfunction, cell death, brain damage and symptoms of disease. Each neurodegenerative disorder is characterized by different aggregated proteins. The protein amyloid beta  $(A\beta)$  is involved in Alzheimer's disease, while the protein alpha-synuclein ( $\alpha$ -synuclein) is involved in Parkinson's disease. BioArctic's aim with the antibodies currently in clinical phase, is to achieve a disease-modifying effect through the selective binding of antibodies, and elimination of the harmful soluble aggregated forms of the amyloid beta protein (oligomers/protofibrils) and the alpha-synuclein protein in the brain.

# Project portfolio

BioArctic has a balanced, competitive portfolio consisting of unique product candidates and technology platforms. All projects are focused on disorders of the central nervous system. The projects are a combination of fully funded projects run in partnership with global pharmaceutical companies and innovative in-house projects with significant market- and out-licensing potential. The projects are in various phases: from discovery to late clinical phase.

	Project	Partner	Research	Preclinical	Phase 1	Phase 2	Phase 3
ALZHEIMER'S DISEASE	Lecanemab (BAN2401) <i>Clarity AD</i>	Eisai <sup>1</sup>	Early Alzheime	er's disease3			
	Lecanemab (BAN2401) AHEAD 3-45	Eisai <sup>1</sup>	Preclinical (asy	ymptomatic) Alzh	eimer's disease4	4	
	BAN2401 back-up	Eisai					
	AD1801						
	AD1503						
	AD-BT2802						
	AD-BT2803						
	AD2603						
PARKINSON'S DISEASE	ABBV-0805 <sup>2</sup>	AbbVie					
	PD1601 <sup>2</sup>	AbbVie					
	PD1602 <sup>2</sup>	AbbVie					
OTHER CNS DISORDERS	Lecanemab (BAN2401)		Down's syndro Traumatic brain				
	ND3014		ALS				
	ND-BT3814		ALS				
BLOOD-BRAIN BARRIER	Brain Transporter (BT) technology platform						

As of March 31, 2022, the project portfolio consisted of:

1) Partnered with Eisai for lecanemab for treatment of Alzheimer's disease. Eisai entered partnership with Biogen regarding BAN2401 (lecanemab) in 2014

2) AbbVie in-licensed BAN0805 in late 2018 and develops the antibody with the designation ABBV-0805. On April 20, 2022, On April 20, 2022, AbbVie informed BioArctic that it had taken a strategic business decision to terminate the collaboration regarding BioArctic's alpha-synuclein portfolio

3) Mild cognitive impairment due to Alzheimer's disease and mild Alzheimer's disease

4) Normal cognitive function with intermediate or elevated levels of amyloid in the brain

5) Dementia and cognitive impairment associated with Down's syndrome and with traumatic brain injury

#### ALZHEIMER'S DISEASE

BioArctic has developed several unique and selective antibodies with the potential to slow the progression of Alzheimer's disease. The most advanced drug candidate, lecanemab (BAN2401) is currently being evaluated in two Phase 3 studies: Clarity AD for early Alzheimer's disease and AHEAD 3-45 for preclinical (asymptomatic) Alzheimer's disease. Lecanemab previously showed convincing results in a large Phase 2b study in patients with early Alzheimer's disease. The development of lecanemab against Alzheimer's disease is being financed and pursued by BioArctic's partner Eisai, which also co-owns the rights to the lecanemab back-up in Alzheimer's disease. BioArctic has five additional antibodies projects against Alzheimer's disease in its project portfolio. In addition, BioArctic conducts research in diagnostics to support its own projects in Alzheimer's disease.

#### Drug candidate lecanemab (collaboration with Eisai)

In Alzheimer's disease, the amyloid beta protein clumps together into increasingly larger aggregates in the brain – from the harmless form with a normal function (monomers) to larger forms such as oligomers, protofibrils, fibrils and finally amyloid plaques containing fibrils. Oligomers and protofibrils are considered the most harmful forms of amyloid beta that initiate the process of Alzheimer's disease. Lecanemab is a drug candidate which functions by eliminating these forms of amyloid from the brain and thereby has the potential to slow down the progression of disease. BioArctic's partner Eisai is responsible for the clinical development of lecanemab in Alzheimer's disease and the project is based on research from Uppsala University, Sweden.

Eisai is conducting two global Phase 3 studies with lecanemab, one in patients with early Alzheimer's disease (Clarity AD) and one in cognitively unimpaired individuals with intermediate or elevated amyloid levels in the brain who have not yet developed symptoms of Alzheimer's disease (AHEAD 3-45). Further DIAN-TU (Dominantly Inherited Alzheimer Network Trials Unit) has chosen to include lecanemab as the backbone anti-amyloid treatment in a clinical trial in combination with potential tau treatments in patients with dominant hereditary Alzheimer's disease.

Clarity AD is the pivotal and confirmatory Phase 3 study. It is based on the placebo-controlled Phase 2b study with lecanemab in 856 patients with early Alzheimer's disease which demonstrated dose dependent, clinically meaningful, and statistically significant effects of lecanemab on several clinical endpoints and on biomarkers and showed good tolerability. An open-label Phase 2b extension study, in which all participants receive the highest dose of lecanemab (10 mg / kg), is ongoing. The data reported so far from the extension study support and confirm the results seen in the Phase 2b study.

Clarity AD is a global placebo-controlled, double-blind, randomized, parallel-group study of lecanemab in 1,795 patients with early Alzheimer's disease i.e., mild cognitive impairment (MCI) due to Alzheimer's disease or mild Alzheimer's disease. According to Eisai, the aim is to obtain the topline data from the study by the end of September 2022 and thereafter apply for full marketing approval across geographies.

The participants in the Clarity AD study can, after 18 months of treatment, continue in an ongoing open-label extension study, without placebo control, where all eligible participants receive treatment with lecanemab.

Lecanemab has a unique binding profile that distinguishes it from other amyloid beta antibodies and its unique binding profile has been confirmed in laboratory analyses, which are ongoing in parallel with the clinical development program. BioArctic has an ongoing research collaboration with Eisai in order to further deepen the knowledge about the drug candidate lecanemab's unique binding profile.

During the autumn 2021 Eisai conducted a Phase 1 study with the subcutaneous administration of lecanemab and it will now be evaluated in the Clarity AD extension study.

Lecanemab was selected by the Alzheimer's Clinical Trials Consortium (ACTC) and Eisai to be evaluated in a second clinical Phase 3 program which aims to evaluate the effects of lecanemab on preclinical asymptomatic Alzheimer's disease (AHEAD 3-45). The program, that was started 2020, include individuals that are at a very early stages of Alzheimer's disease with a high risk of developing the disease. The program consists of two clinical sub-studies: A3 and A45. After a joint screening process, the participants are included in one of the randomized, double-blind and placebo-controlled sub-studies based on amyloid levels in the brains of the specific individuals. AHEAD 3-45 is a global program that is expected to include approximately 1,400 individuals. DIAN-TU has chosen to include lecanemab as the backbone antiamyloid treatment in its NextGen-study in combination with potential tau treatments in patients with dominant hereditary Alzheimer's disease. The aim of the study is to assess the safety and tolerability of certain drug candidates as well as their effect on biomarkers and cognition in patients with hereditary Alzheimer's disease.

In June 2021 lecanemab was granted Breakthrough Therapy designation, an FDA program intended to facilitate and accelerate the development and review of drugs for serious or life-threatening conditions. This status includes more intensive guidance from the FDA on an efficient development program and eligibility for rolling review and review of the application for marketing approval, and potentially a priority review of the final application. In September 2021, Eisai announced that the company had initiated a rolling application to the FDA for approval of lecanemab in early Alzheimer's disease under the Accelerated Approval program.

In December 2021, lecanemab was granted Fast Track designation by the FDA, which supports expedited development of treatments for serious illnesses with significant medical need. At the same time, it was announced that two of the three parts in the rolling submission for accelerated approval of lecanemab had been submitted to the FDA. The application is based primarily on clinical, biomarker and safety data from the Phase 2b study of lecanemab in individuals with early Alzheimer's disease and confirmed A $\beta$  pathology. Data from the open-label Phase 2b extension study and blinded safety data from Clarity AD has been included to support the application for marketing approval. The third and final part of the application is planned to be submitted during the second quarter of 2022.

### Back-up candidate to lecanemab (collaboration with Eisai)

The antibody is a refined version of lecanemab for the treatment of Alzheimer's disease. The antibody was developed in collaboration with Eisai, which resulted in a new license agreement in 2015. The project is driven and financed by Eisai and is in the preclinical phase.

### Projects AD1801, AD1503 and AD2603 (owned by BioArctic)

BioArctic has three additional antibody projects against Alzheimer's disease in its project portfolio, all of which are in the research phase. These antibodies have different targets, and each has the potential to become a disease-modifying treatment for Alzheimer's disease. All of them are being developed to treat early Alzheimer's disease. AD1801 is an antibody project where the mechanism of action is linked to ApoE, which is the most common genetic risk factor for Alzheimer's disease. AD1503 is an antibody project against a shorter (truncated) form of amyloid beta, which has a pronounced ability to aggregate and create toxic forms that could cause Alzheimer's disease. The AD1502 project was stopped during the quarter as data does not support further development.

### Drug projects AD-BT2802 and AD-BT2803 (blood-brain barrier technology owned by BioArctic)

BioArctic has two antibody projects against Alzheimer's disease that are being combined with the blood-brain barrier technology — Brain Transporter, or BT — to facilitate uptake of antibodies in the brain.

#### PARKINSON'S DISEASE

In the Parkinson's disease treatment area, BioArctic has been collaborating with AbbVie since 2016 who acquired the license to develop and commercialize BioArctic's portfolio of antibodies against alpha-synuclein for Parkinson's disease and other potential indications.

#### Drug candidate ABBV-0805 and drug projects PD1601 and PD1602 (collaboration with AbbVie under termination)

ABBV-0805 is a monoclonal antibody that selectively binds to and eliminates oligomers and protofibrils of alphasynuclein. The goal is to develop a disease modifying treatment that stops or slows down disease progression. The project is based on research from Uppsala University.

At the International Congress of Parkinson's Disease and Movement Disorders® (MDS) in September 2021, BioArctic presented preclinical results and AbbVie presented results from the Phase 1 study that support continued development of the antibody in a Phase 2 study with dosing once a month. In November 2021, *Neurobiology of Disease* published an article from BioArctic that describes new preclinical data for the anti-alpha synuclein antibody ABBV-0805. The article contains data demonstrating the antibody's ability to selectively bind harmful soluble alpha-synuclein aggregates. The PD1601 and PD1602 antibody projects are also targeted against alpha-synuclein for treatment of Parkinson's disease. The objective of the project portfolio is to develop diseasemodifying treatments for Parkinson's disease, Lewy body dementia and multiple system atrophy.

On April 20, 2022, AbbVie informed BioArctic that it had taken a strategic business decision to terminate the collaboration regarding BioArctic's alpha-synuclein project portfolio.

#### **OTHER CNS DISORDERS**

BioArctic aims to improve the treatment of a number of central nervous system disorders. The company is evaluating the possibility of developing its existing as well as new antibodies against other diseases in the central nervous system.

### Drug candidate lecanemab (indications other than Alzheimer's disease, owned by BioArctic)

Lecanemab can potentially also be used for other indications which in that case would be owned by BioArctic. The antibody is in the preclinical phase as a potential treatment of cognitive disorders in conjunction with Down's syndrome and traumatic brain injury. BioArctic has presented findings supporting that lecanemab also could be developed into a disease modifying treatment benefiting individuals with Down's syndrome with dementia.

#### Project ND3014 and ND-BT3814 (owned by BioArctic)

The drug projects ND3014 and ND-BT3814 are focused on developing antibody drugs against TDP-43, a protein that is believed to play a key role in the development of the rare neurodegenerative disease ALS. The ND-BT3814 project is linked to BioArctic's blood-brain barrier technology. The projects are in research phase.

### BLOOD-BRAIN BARRIER TECHNOLOGY (BRAIN TRANSPORTER) (owned by BioArctic)

The blood-brain barrier controls the passage of substances between the blood and the brain. It protects the brain from harmful substances, but at the same time it can make the delivery of therapeutic agents to the brain more difficult. BioArctic is now developing the second generation of this technology, which has already demonstrated a profound increase in antibodies and improved exposure in the brain. The technology is now in three earlier projects against Alzheimer's disease, AD-BT2802, AD-BT2803 and ALS ND-BT3814. The technology has shown highly encouraging results and has significant potential for many different treatments for various diseases of the brain. Together with Uppsala University, BioArctic received grants from Sweden's Innovation Agency, Vinnova, for continued research in the blood-brain barrier project.

# Comments to the financial development

#### **REVENUES AND RESULT**

Revenues consist of milestone payments, payments from research agreements and research grants. Because of the nature of the business operations, there may be large fluctuations in revenues for different periods, as revenues from milestone payments are recognized at the point in time when performance obligations are fulfilled.

Net revenues in the first quarter amounted to MSEK 3.7 (7.2). The decrease in the quarter is explained by the fact that the scope of the current research collaboration agreement with Eisai is smaller than before.

Other operating income relates to research grants and operating exchange rate gains. Other operating income amounted to MSEK 0.7 (1.7) in the first quarter. The increase is mainly attributable to exchange rate gains.

Total operating expenses for the first quarter amounted to MSEK -48.5 (-38.0). Project expenses for projects fully owned by BioArctic increased due to a higher activity within those projects. The expenses for personnel for the first quarter increased somewhat mainly as a result of an increase in the number of employees. Other external costs increased somewhat during the quarter. Other operating expenses mainly consist of realized operating exchange rate losses.

Since BioArctic's own projects are in an early research phase they did not meet all the conditions for R&D costs to be capitalized and thus, all such costs have been charged to the income statement. The external projects are owned by our partners and BioArctic has no costs for the clinical programs.

Operating profit before financial items (EBIT) amounted to MSEK -44.1 (-29.1) for the first quarter. The decrease in operating profit was primarily attributable to lower revenue from the research collaboration with Eisai and increased external costs and costs of personnel.

Net financial items totaled MSEK -0.2 (0.0) for the first quarter. Financial income consists of financial exchange rate gains and financial expenses consists of negative interest on cash and cash equivalents and interest on leasing liabilities.

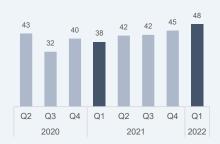
Profit (loss) amounted to MSEK -44.3 (-29.1) for the first quarter.

Earnings per share before and after dilution amounted to SEK -0.50 (-0.33) for the first quarter.

#### Net revenues (MSEK)



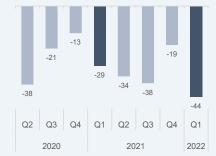
#### **Operating expenses (MSEK)**



#### Operating profit/loss (MSEK)



#### Profit/loss for the period (MSEK)



#### LIQUIDITY AND FINANCIAL POSITION

Equity amounted to MSEK 745.1 as of March 31, 2022 compared with MSEK 788.7 as of December 31, 2021. This corresponds to equity per outstanding share of SEK 8.46 (9.98). The equity/asset ratio was 88.0 percent as of March 31, 2022compared with 87.9 percent as of December 31, 2021.

The Group's cash and cash equivalents consist of bank balances that at the end of the quarter amounted to MSEK 800.8 compared with MSEK 848.4 as of December 31, 2021. There were no loans as of March 31, 2022 and no loans have been taken since this date. The Group has no other credit facility or loan commitments.

In order to neutralize foreign exchange rate exposure some liquid funds are held in foreign currency. This has reporting effects in connection with the recalculation of currency to the current rate. These effects are recognized in the operating profit and in financial income and expenses.

#### **CASH FLOW AND INVESTMENTS**

Cash flow from operating activities for the first quarter amounted to MSEK -39.7 (-37.5).

For the first quarter cash flow from investing activities amounted to MSEK -6.0 (-0.9). The investments were mainly related to laboratory equipment. Cash flow from financing activities amounted to MSEK -2.0 (-1.8) for the first quarter and relates to the amortization of leasing liabilities.

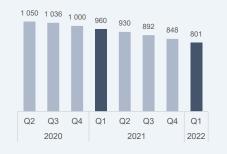
#### **PARENT COMPANY**

All of the Group's business operations are conducted in the Parent Company.

#### **EVENTS DURING THE FIRST QUARTER 2022**

 Eisai commenced submission of lecanemab data in Japan for preliminary review, with the objective of obtaining fast regulatory marketing approval.

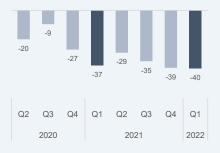
Cash and cash equivalents (MSEK)



#### **Financial position (MSEK)**

	31 Mar 2022	31 dec 2021
Non-current lease liabilities	5.8	7.8
Current lease liabilities	8.3	8.1
Cash and cash equivalents	800.8	848.4
Net cash position	786.7	832.5

### Cash flow from operating activities (MSEK)





# Other information

#### **EVENTS AFTER THE REPORTING PERIOD**

- AbbVie took a strategic business decision to end its collaboration with BioArctic regarding its alpha-synuclein portfolio, including ABBV-0805. BioArctic will now, in accordance with the license agreement, take back the project and evaluate the best way forward.
- A recently published article in Neurology and Therapy based on disease modeling suggests that lecanemab could delay the progression to Alzheimer's dementia by several years.

#### PATENTS

Patents are crucial to the company's future commercial opportunities. BioArctic has therefore an active patent strategy covering all major pharmaceutical markets including the US, EU, Japan and China. At the end of March 2022, BioArctic's patent portfolio consisted of 13 patent families with more than 230 granted patents and approximately 60 ongoing patent applications.

### PARTNERSHIPS, COLLABORATIONS AND MAJOR AGREEMENTS

Collaborations and license agreements with leading pharma and biopharma companies are an important part of BioArctic's strategy. In addition to financial compensation, BioArctic benefits from the expertise the company's partners contribute in drug development, manufacturing and commercialization. BioArctic has entered into a number of such agreements with the Japanese global pharma company Eisai and the American global biopharma company AbbVie. These strategic partnerships with leading global companies confirm that BioArctic's research is of very high quality. In the future BioArctic may enter into additional agreements that can contribute further funding and research and development competence for BioArctic's product candidates in preclinical and clinical phase, manufacturing and marketing competence, geographic coverage and other resources.

BioArctic has been collaborating with Eisai in the field of Alzheimer's disease since 2005. The company has signed research and licensing agreements concerning the lecanemab and BAN2401 back-up antibodies. The total value of these agreements may amount to MEUR 222 in addition to royalties. As of 31 March 2022, up to 151 MEUR remains.

BioArctic has been collaborating with AbbVie in the field of Parkinson's disease since 2016, when a research agreement was signed that included products such as the antibody BAN0805, now designated ABBV-0805. At the end of 2018, AbbVie exercised its option to license BioArctic's alphasynuclein antibody portfolio for Parkinson's disease and other potential indications. BioArctic has had primary responsibility for the preclinical development work and AbbVie has been responsible for the clinical development. Over the course of the contract, BioArctic received MUSD 130. In light of the collaboration being terminated, no further milestone payments or royalties will be paid to BioArctic from AbbVie. BioArctic Collaborating with universities is also of great importance to BioArctic. The company has ongoing collaborations with academic research groups at a number of universities.

#### **RISKS AND UNCERTAINTY FACTORS**

The management makes assumptions, judgments and estimates that affect the content of the financial statements. Actual results may differ from these assumptions and estimates, as is also stated in the accounting principles. The objective of the Group's risk management is to identify, mitigate, measure, control and limit business risks. Significant risks are the same for the Parent Company and the Group.

BioArctic's operational and external risks mainly consist of risks related to research and development, clinical trials and dependence on key employees.

A detailed description of exposure and risk management is presented in the Annual Report 2021 on pages 56-59.

Russia's invasion of Ukraine is a tragedy, above all for the people in the war zone or who have been forced to flee. There is a great deal of uncertainty regarding how the situation will develop and how it will impact the global economy over both the short and long term. BioArctic is closely following the course of events in the world around us and is presently of the opinion that the invasion does not have any direct impact on the company's operations.

#### FLUCTUATIONS IN REVENUE GENERATION

Currently, BioArctic does not have any drugs on the market. The company develops a number of drug candidates for Alzheimer's and Parkinson's diseases in collaboration with global pharmaceutical companies. The company also conducts research for wholly owned projects including new potential antibody treatments as well as a blood-brain barrier technology platform. The company signs research and licensing agreements with partners and then receives remuneration for research as well as milestone payments and royalties, which the company uses to finance current and new projects. Milestone payments are normally received when the project reaches predetermined development targets - the start of clinical trials, for example - or when clinical trials move from one phase to a later phase. Milestone payments may also be paid upon submissions of applications to regulatory authorities, approvals and sales milestones. Thus, these payments arise unevenly over time.

#### **FUTURE PROSPECTS**

The company enjoys a strong financial position and has a business model in which its revenue and earnings are currently primarily based on non-recurring revenue from research and licensing agreements the company signed. The company's liquidity facilitates continued development of the projects covered by strategic partnership agreements as well as financing of the company's own projects in early phase and therefore are less costly. BioArctic's focus areas comprise unique drug candidates and an innovative blood-brain barrier technology, areas with high unmet medical need. All projects are focused on disorders of the central nervous system and have great market potential. BioArctic's ambition is to generate the medicines of the future for patients with central nervous system disorders.

### EXPECTED DEVELOPMENT OF OPERATING EXPENSES

Operating expenses are expected to be in the range of MSEK 220 – 260 for the fiscal year January – December 2022. During 2021 operating expenses were MSEK 166. During the last three years the average annual level of the operating expenses has been approximately MSEK 170. The build-up of the commercial organization prior to the potential launch of lecanemab, and costs for the expanded in-house project portfolio, explain the expected higher level of costs for 2022.

#### **EMPLOYEES**

At the end of the first quarter, the number of employees was 55 (47) of which 21 (18) are men and 34 (29) women. Around 80 percent work in R&D and around 70 percent are PhDs.

A cost-efficient organization at BioArctic is achieved by hiring consultants for specific assignments and tasks in competence areas that the company lacks or only has need for periodically. As of March 31, 2022, these corresponded to 11 (10) full-time positions.

#### THE SHARE AND SHAREHOLDINGS

The share capital in BioArctic amounts to SEK 1,761,200 divided by 88,059,985 shares which is split between 14,399,996 A-shares and 73,659,989 B-shares. The quotient value for both A- and B-shares is SEK 0.02. The A-share has 10 votes per share and the B-share has 1 vote per share.

#### LARGEST SHAREHOLDERS AS OF MARCH 31, 2022<sup>1</sup>

	Nun	ıber	Share o	of (%)
	A-shares	B-shares	capital, %	votes, %
Demban AB (Lars Lannfelt)	8,639,998	22,628,052	35.5	50.1
Ackelsta AB (Pär Gellerfors)	5,759,998	15,086,301	23.7	33.4
Fourth AP-Fund	-	4,300,000	4.9	2.0
Swedbank Robur Funds	-	3,980,307	4.5	1.8
Third AP-Fund	-	3,094,097	3.5	1.4
Unionen	-	2,391,835	2.7	1.1
Investment AB Öresund	-	1,330,000	1.5	0.6
Hans Edvin Öhman	-	1,159,607	1.3	0.5
OM Holding AS	-	985,955	1.1	0.5
Handelsbanken Funds	-	955,263	1.1	0.4
Tot. 10 largest shareholders	14,399,996	55,911,417	79.8	91.8
Other	-	17,748,572	20.2	8.2
Total	14,399,996	73,659,989	100.0	100.0

1) Source: Monitor by Modular Finance AB. Compiled and processed data from various sources, including Euroclear, Morningstar and Swedish Financial Supervisory Authority (Finansinspektionen).

#### **ANNUAL GENERAL MEETING 2022**

BioArctic's Annual General Meeting will take place on May 5 and will be held only by advance voting, so-called postal voting method. The notice to the AGM was distributed on the 6th of April 2022.

#### LONG-TERM INCENTIVE PROGRAMS

The Annual General Meeting 2019 approved the Board of Directors' proposal for resolution concerning an employee warrant program for the company's management, researchers and other staff, a directed issue of warrants and the transfer of warrants or shares in the company to the participants in the employee warrant program.

The employee warrant program 2019/2028 include not more than 1,000,000 warrants. To enable the company's delivery of shares under the employee warrant program 2019/2028, the Annual General Meeting approved a directed issue of a maximum of 1,000,000 warrants.

The dilutive effect of the employee warrant program 2019/2028 is estimated to be a maximum of 1.1 percent of the share capital and 0.5 percent of the votes in the company (calculated on the number of existing shares in the company), assuming full exercise of all employee warrants. The employee warrants can be exercised three years after allocation at the earliest. As of the end of the period, 745,000 employee warrants were allocated, of which 170,000 were allocated during the first quarter 2022. The allocation of employee warrants had a dilutive effect corresponding to 540,000 shares, or 0.6 percent, at the end of the period. However, these options are not included in the calculation of earnings per share after dilution since the company is reporting negative earnings. More information is available on www.bioarctic.com

The information was submitted for publication, though the agency of the named contact persons, at 8:00 a.m. CET on April 28, 2022.

This interim report has not been subject to review by BioArctic's auditors.

Stockholm, Sweden, April 28, 2022

Gunilla Osswald CEO, BioArctic AB (publ)

### INVITATION TO PRESENTATION OF THE REPORT FOR JANUARY – MARCH 2022

BioArctic invites investors, analysts, and media to an audiocast with teleconference (in English) today, April 28, at 9:30–10:30 a.m. CET. CEO Gunilla Osswald and CFO Jan Mattsson will present BioArctic, comment on the interim report and answer questions.

#### Webcast: https://tv.streamfabriken.com/bioarctic-q1-2022

To participate in the conference, please call: +46 8 505 583 56 (Sweden), +45 823 331 94 (Denmark), +31 107 129 162 (Netherlands), +47 235 002 36 (Norway), +41 225 805 976 (Switzerland), +44 333 300 08 04 (UK), +49 692 222 391 66 (Germany) or +1 631 913 1422 (USA)

CALENDAR 2022 Annual General Meeting 2022 Interim report Jan-Mar 2022 Interim report Jan-Sep 2022 Full Year Report Jan-Dec 2022

May 5, 2022 July 12, 2022, at 8:00 a.m. CET October 20, 2022, at 8:00 a.m. CET February 3, 2023, at 08:00 a.m. CET

#### FOR FURTHER INFORMATION, PLEASE CONTACT

Gunilla Osswald, CEO, <u>gunilla.osswald@bioarctic.se</u>, phone +46 8 695 69 30 Jan Mattsson, CFO, <u>jan.mattsson@bioarctic.se</u>, phone +46 70 352 27 72 Oskar Bosson, VP Communications & Investor Relations, <u>oskar.bosson@bioarctic.se</u>, phone +46 70 410 71 80

# **BioArctic AB** (publ)

Swedish Corporate Identity Number 556601-2679 Warfvinges väg 35, SE-112 51, Stockholm, Sweden Telephone +46 (0)8 695 69 30 www.bioarctic.com

This report has been prepared in a Swedish original version and translated into English. In the event of any inconsistency between the two versions, the Swedish language version applies.







# Financial statements, Group

#### CONSOLIDATED INCOME STATEMENT

			Jan-Dec	
kSEK	2022	2021	2021	
Net revenues (note 4)	3,737	7,177	23,146	
Other operating income	663	1,687	3,542	
Operating revenues	4,400	8,863	26,688	
Operating expenses				
Project related expenses	-14,927	-11,299	-55,067	
Other external expenses	-7,452	-6,008	-24,851	
Personnel expenses	-22,169	-17,264	-72,499	
Depreciations of tangible assets	-3,474	-3,275	-13,108	
Other operating expenses	-449	-161	-886	
Operating expenses	-48,471	-38,007	-166,411	
Operating profit/loss	-44,071	-29,144	-139,723	
Financial income	88	269	194	
Financial expenses	-273	-231	-984	
Profit/loss before tax	-44,256	-29,106	-140,512	
Тах	-0	20	20,722	
Profit/loss for the period	-44,256	-29,086	-119,789	
Earnings per share				
Earnings per share before dilution, SEK	-0.50	-0.33	-1.36	
Earnings per share after dilution, SEK	-0.50	-0.33	-1.36	

#### CONSOLIDATED STATEMENT OF COMPREHENSIVE INCOME

	C	Q1	
kSEK	2022	2021	2021
Profit/loss for the period	-44,256	-29,086	-119,789
Other comprehensive income	-	-	-
Comprehensive income for the period	-44,256	-29,086	-119,789

#### CONSOLIDATED BALANCE SHEET

kSEK	31 Mar 2022	31 Mar 2021	31 dec 2021
ASSETS			
Tangible fixed assets	21,472	17,660	16,963
Right-to-use assets	15,176	20,418	16,785
Deferred tax assets	607	457	608
Other financial assets	1,588	1,571	1,588
Current assets excluding cash and cash equivalents	7,111	5,455	13,380
Cash and cash equivalents	800,846	960,466	848,405
TOTAL ASSETS	846,800	1,006,027	897,730
EQUITY AND LIABILITIES			
Equity	745,130	878,602	788,676
Deferred tax liabilities	-	20,666	-
Non-current lease liabilities	5,850	12,207	7,785
Current lease liabilities	8,316	7,223	8,092
Other current liabilities	11,318	7,710	15,737
Accrued expenses and deferred income	76,186	79,618	77,438
EQUITY AND LIABILITIES	846,800	1,006,027	897,730

#### CONSOLIDATED STATEMENT OF CHANGE IN EQUITY (CONDENSED)<sup>1</sup>

kSEK	31 Mar 2022	31 Mar 2021	31 dec 2021
Opening balance at 1 January	788,676	907,299	907,299
Correction of opening balance	-	-	-402
Comprehensive income for the period	-44,256	-29,086	-119,789
Share-based payments	709	388	1,567
Closing balance	745,130	878,602	788,676

#### CONSOLIDATED STATEMENT OF CASH FLOW (CONDENSED)

	Q1		Jan-Dec	
kSEK	2022	2021	2021	
Operating profit	-44,071	-29,144	-139,723	
Adjustment for non-cash items	2,205	511	5,230	
Interest received/paid	-97	39	-597	
Income tax paid	1,656	16	-309	
Cash flow from operating activities before changes in working capital	-40,307	-28,579	-135,398	
Change in working capital	591	-8,921	-5,059	
Cash flow from operating activities after changes in working capital	-39,716	-37,500	-140,457	
Cash flow from investing activities	-6,020	-949	-4,412	
Cash flow from financing activities	-2,036	-1,813	-7,388	
Cash flow for the period	-47,773	-40,261	-152,257	
Cash and cash equivalents at beginning of period	848,405	999,940	999,940	
Exchange rate differences in cash and cash equivalents	213	788	723	
Cash and cash equivalents at end of period	800,846	960,466	848,405	

1) A minor error was discovered during the transition to a new system for translation in accordance with IFRS 16, which affects the opening balance for equity 2021 by MSEK 0.4, corresponding to 0.05%.

#### CONSOLIDATED QUARTERLY DATA

	2022	2021	2021	2021	2021	2020	2020	2020
MSEK	Q1	Q4	Q3	Q2	Q1	Q4	Q3	Q2
Income statement								
Net revenues	4	5	4	7	7	8	11	7
Other operating income	1	1	1	1	2	1	1	-2
Operating expenses	-48	-45	-42	-42	-38	-40	-32	-43
Operating profit/loss	-44	-39	-37	-34	-29	-30	-21	-38
Operating margin, %	neg							
Profit/loss for the period	-44	-19	-38	-34	-29	-13	-21	-38
Balance sheet								
Fixed assets	39	36	37	39	40	42	36	35
Current assets	7	13	6	5	5	8	4	14
Cash and cash equivalents	801	848	892	930	960	1,000	1,036	1,050
Equity	745	789	807	844	879	907	920	940
Deferred tax liabilities	-	-	21	21	21	21	39	39
Lease liabilities	14	16	18	19	19	21	22	23
Current liabilities	88	93	90	89	87	102	95	98
Cash flow								
From operating activities	-40	-39	-35	-29	-37	-27	-9	-20
From investing activities	-6	-2	-2	-0	-1	-7	-3	-1
From financing activities	-2	-2	-2	-2	-2	-1	-1	-2
Cash flow for the period	-48	-43	-38	-31	-40	-35	-14	-23
Key ratios								
Equity/asset ratio, %	88.0	87.9	86.3	86.7	87.3	86.4	85.5	85.5
Return on equity, %	-5.8	-2.4	-4.5	-4.0	-3.3	-1.4	-2.2	-4.0
Data per share								
Earnings per share before dilution, SEK	-0.50	-0.22	-0.43	-0.39	-0.33	-0.15	-0.23	-0.43
Earnings per share after dilution, SEK	-0.50	-0.22	-0.43	-0.39	-0.33	-0.15	-0.23	-0.43
Equity per share, SEK	8.46	8.96	9.17	9.59	9.98	10.30	10.45	10.68
Cash flow operating activities per share, SEK	-0.45	-0.45	-0.39	-0.33	-0.43	-0.30	-0.11	-0.22
Share price at the end of the period, SEK	103.20	119.20	162.60	137.80	91.00	95.40	88.95	73.35
Number of shares outstanding at the end of the period, thousands	88,060	88,060	88,060	88,060	88,060	88,060	88,060	88,060
Average number of shares outstanding before dilution, thousands	88,060	88,060	88,060	88,060	88,060	88,060	88,060	88,060
Average number of shares outstanding after dilution, thousands	88,605	88,610	88,585	88,560	88,560	88,332	88,105	88,092
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# Financial statements, Parent company

#### PARENT COMPANY INCOME STATEMENT

	Q1		Jan-Dec	
kSEK	2022	2021	2021	
Net revenues	3,737	7,177	23,146	
Other operating income	663	1,687	3,542	
Operating revenues	4,400	8,863	26,688	
Operating expenses				
Project related expenses	-14,927	-11,299	-55,067	
Other external expenses	-9,614	-8,064	-33,224	
Personnel expenses	-22,169	-17,264	-72,499	
Depreciations of tangible assets	-1,540	-1,399	-5,604	
Other operating expenses	-449	-161	-885	
Operating expenses	-48,699	-38,187	-167,279	
Operating profit/loss	-44,299	-29,323	-140,591	
Financial income	88	269	194	
Financial expenses	-105	-14	-145	
Profit/loss after financial items	-44,316	-29,068	-140,542	
Change in tax allocation reserves		-	94,809	
Profit/loss before tax	-44,316	-29,068	-45,733	
Tax	12	12	63	
Profit/loss for the period	-44,304	-29,056	-45,670	

There are no items recognized as other comprehensive income in the Parent Company. Accordingly, total comprehensive income matches profit for the year.

PARENT COMPANY BALANCE SHEET (CONDENSED)

kSEK	31 Mar 2022	31 Mar 2021	31 dec 2021
ASSETS			
Tangible fixed assets	21,472	17,660	16,963
Deferred tax assets	401	337	388
Other financial assets	1,638	1,621	1,638
Current assets excluding cash and cash equivalents	9,126	6,870	15,353
Cash and cash equivalents	800,800	960,419	848,359
TOTAL ASSETS	833,436	986,908	882,702
EQUITY AND LIABILITIES			
Equity	745,932	804,960	789,526
Tax allocation reserve	-	94,809	-
Other current liabilities	11,318	7,521	15,737
Accrued expenses and deferred income	76,186	79,618	77,438
EQUITY AND LIABILITIES	833,436	986,908	882,702

### Notes

#### NOTE 1 GENERAL INFORMATION

This interim report for the period January – March 2022 covers the Swedish Parent Company BioArctic AB (publ), Swedish Corporate Identity Number 556601-2679, and the fully owned subsidiary LPB Sweden AB, Swedish Corporate Identity Number 559035-9112. All the Group's business operations are conducted in the Parent Company. BioArctic is a Swedish limited liability company registered in and with its registered office in Stockholm. The head office is located at Warfvinges väg 35, SE-112 51, Stockholm, Sweden.

#### **NOTE 2 ACCOUNTING PRINCIPLES**

The consolidated financial statements for BioArctic AB (publ) have been prepared in accordance with IFRS (International Financial Reporting Standards) as adopted by the EU, the Annual Accounts Act and the Swedish Financial Reporting Board's RFR 1 Supplementary Accounting Rules for Groups. The Parent Company's financial statements are presented in accordance with the Swedish Annual Accounts Act and RFR 2 Accounting for Legal Entities.

The interim report for the period January – March 2022 is presented in accordance with IAS 34 Interim Financial Reporting and the Swedish Annual Accounts Act. Disclosures in accordance with IAS 34 are presented both in notes and elsewhere in interim report. The accounting principles and calculation methods applied are in accordance with those described in the Annual Report 2021. New and amended IFRS standards and interpretations applied from 2022 have not had a material impact on the financial statements.

The guidelines of the European Securities and Markets Authority (ESMA) on alternative performance measures have been applied. This involves disclosure requirements for financial measures that are not defined by IFRS. For performance measures not defined by IFRS, see the Calculations of key figures section.

#### **NOTE 3 SEGMENT INFORMATION**

An operating segment is a part of the Group that conducts operations from which it can generate income and incur costs and for which independent financial information is available. The highest executive decision-maker in the Group follows up the operations on aggregated level, which means that the operations constitute one and the same segment and thus no separate segment information is presented. The Board of Directors is identified as the highest executive decision maker in the Group.

#### **NOTE 4 NET REVENUES**

	Q1	Q1	
kSEK	2022	2021	2021
Geographic breakdown of net revnues			
Europe	1,649	2,365	8,466
Asia	2,088	4,812	14,681
Total net revenues	3,737	7,177	23,147
Net revenues per revenue type			
Milestone payments, recognized at a given point in time	-	-	-
Income from research collaborations, recognized over time	3,737	7,177	23,147
Total net revenues	3,737	7,177	23,147

BioArctic's net revenues essentially consist of income from the research collaborations concerning Parkinson's disease with AbbVie and Alzheimer's disease with Eisai. Under the collaboration agreement with AbbVie, BioArctic received an initial payment of MSEK 701.6, or MUSD 80, during the third quarter 2016. This payment is related to compensation for the preclinical development work that BioArctic will carry out under the agreement. Of the initial payment, MSEK 70.4 was reported as a one-time payment in 2016. The rest of the payment will be accrued based on the costs incurred up until the completion of the project. The project is continuously evaluated with the regard to status and remaining costs. As of March 31, 2022, MSEK 644.8 has been recognized as revenue and the remaining amount to be recognized as a revenue up until the completion of the project is MSEK 56.8. MSEK 1.6 (2.4) was recognized as revenue in first quarter 2022.

Current research collaboration agreement with Eisai refers to the period July 2021 to June 2022. The revenue for the research collaboration is recognized over time based on the fulfillment of the performance obligation. In the first quarter MSEK 2.1 (4.8) was recognized as revenue.

# Definition of key ratios

In this financial report BioArctic reports key financial ratios, some of which are not defined by IFRS. The Company's assesses that these key ratios are important additional information, since they enable investors, securities analysts, management of the company and other stakeholders to better analyze and evaluate the company's business and financial trends. These key ratios should not be analyzed separately or replace key ratios that have been calculated in accordance with IFRS. Neither should they be compared to other key ratios with similar names applied by other companies, as key ratios cannot always be defined in the same way. Other companies may calculate them in a different way than BioArctic.

The key ratios "Net revenues", "Result for the period", "Earnings per share" and "Cash flow from operating activities" are defined according to IFRS.

Key ratios	Definition	
Other income	Other income than net revenue	
Operating profit	Result before financial items	
Operating margin, %	Operating profit divided by net revenues	
Cash flow from operating activities per share, SEK	The cash flow from operating activities for the period divided by the weighted number of shares	
Equity/asset ratio, %	Adjusted equity divided by total assets	
Return on equity, %	Net income divided by equity expressed as a percentage	
Equity per share	Adjusted equity divided by the number of shares at the end of the period	

# Glossary

#### Accelerated approval

An application process which gives an opportunity for an early approval of a drug candidate, where the company at a later stage is required to present additional data to verify clinical effect in order to receive full marketing approval.

#### ADAS-Cog

ADAS-Cog (Alzheimer's Disease Assessment Scale cognitive subscale) is a well-established cognition scale whereof parts are included in ADCOMS.

#### ADCOMS

Alzheimer's Disease Composite Score – A cognition scale consisting of parts from three different scales (CDR-SB, ADAS-cog and MMSE) developed by Eisai. The cognition scale enables a sensitive detection of changes in clinical functions of symptoms in early Alzheimer's disease.

#### ADCS-ADL-MCI

ADCS-ADL-MCI (Alzheimer's Disease Cooperative Study -Activities of Daily Living - Mild cognitive impairment) is a clinical scale focusing of activities of daily living particularly relevant in mild cognitive impairment.

#### Alfa-synuclein (α-synuclein)

A naturally occurring protein in the body that, in conjunction with Parkinson's disease, misfolds and forms harmful structures in brain cells.

#### Amyloid beta (Aβ)

A naturally occurring protein in the brain that, in conjunction with Alzheimer's disease, misfolds into harmful structures in brain cells. Amyloid beta form the plaque around brain cells visible in patients with Alzheimer's disease.

#### Antibody

A biological molecule originating in the immune system that binds to a target molecule with a high degree of accuracy.

#### ApoE (Apolipoprotein E)

ApoE transports fats in the blood. ApoE comes in three forms. Individuals expressing the ApoE4 form are at greater risk of developing Alzheimer's disease.

#### ARIA-E

A form of cerebral edema that occurs in some patients treated with anti-amyloid monoclonal antibodies for Alzheimer's disease.

#### **Binding profile**

A binding profile specifies in which way and to which forms of a protein (such as amyloid beta or alpha-synuclein) an antibody binds.

#### Biomarker

A measurable molecule, the levels of which can indicate a change in the body and enable diagnosis of a patient or measurement of the effect of a drug.

#### **Blood-brain barrier**

A structure of tightly bound cells that surround blood vessels in the brain. This barrier regulates the exchange of nutrients and waste and protects against bacteria and viruses.

#### Breakthrough therapy designation

The breakthrough therapy designation is an FDA program intended to facilitate and accelerate the development and review of drugs for serious or life-threatening conditions.

#### CDR-SB

CDR-SB (Clinical Dementia Rating Sum of Boxes) is a cognition and function scale which is part of ADCOMS.

#### Central nervous system (CNS)

The part of the body's nervous system comprising the brain and spinal cord.

#### **Clinical studies**

Drug trials performed in human subjects.

#### **Disease modifying treatment**

A treatment that interferes with the processes of the disease and changes it in a positive way.

#### **Dose dependent**

Increased effect at higher dose.

#### **Drug candidate**

A drug under development that has not yet gained marketing approval.

#### Early Alzheimer's disease

Mild cognitive impairment due to Alzheimer's disease and mild Alzheimer's disease.

#### **Fast Track Designation**

Fast Track designation is an FDA program intended to facilitate and expedite the development and review of drugs for serious or life-threatening conditions.

#### FDA

The US Food and Drug Administration.

#### Interim analysis

A statistical analysis conducted during an ongoing clinical trial to evaluate preliminary findings.

#### Licensing

Agreement where a company that has invented a drug gives another company the right to further develop and sell the drug for certain payments.

#### **Milestone payment**

Financial remuneration received as part of a project or collaboration agreement once a specified goal has been achieved.

#### Monomer

An individual molecule with the ability to bind to other similar molecules to form larger structures such as oligomers and protofibrils.

#### Neurodegenerative disease

A disease that entails a gradual breakdown and degeneration in brain and nervous system function.

#### Oligomer

Molecules consisting of a number of monomers.

#### **Open-label extension study**

Clinical study conducted after a completed randomized and placebo-controlled study in which all patients receive active substance.

#### Pathology

The study of diseases and how they are diagnosed, through analysis of molecules, cells, tissues and organs.

#### PET

Positron emission tomography, an imaging method used to perform medical examinations.

#### **Phase 1 studies**

Studies the safety and tolerability of a drug. Performed in a limited number of healthy human volunteers or patients.

#### **Phase 2 studies**

Studies the safety and efficacy of a drug. Performed in a limited number of patients. Later stages of phase 2 studies can be called phase 2b and evaluate the optimal dose of the studied drug.

#### Phase 3 studies

Confirms the efficacy and safety of a drug. Performed in a large number of patients.

#### **Placebo-controlled**

A study design in research which means that some of the patients receive inactive compound to obtain a relevant control group.

#### Preclinical (asymptomatic) Alzheimer's disease

Normal cognitive function but with intermediate or elevated levels of amyloid in the brain.

#### **Preclinical phase**

Stage of development where preclinical studies of drug candidates are conducted to prepare for clinical studies.

#### **Preclinical studies**

Studies conducted in model systems in laboratories prior to conducting clinical trials in humans.

#### **Product candidate**

A product under development that has not yet gained marketing approval.

#### **Protofibril**

A harmful aggregation of amyloid beta formed in the brain, which gives rise to Alzheimer's disease, or a harmful aggregation of alpha-synuclein formed in the brain and gives rise to Parkinson's disease.

#### **Research phase**

Early research focused on studying and elucidating the underlying molecular disease mechanisms and generation of potential drug candidates.

#### **Selective binding**

The affinity of a molecule for binding to a specific receptor.

#### Subcutaneous treatment

That the drug is given to the patient through an injection under the skin.

#### **Tolerability**

The degree of side effects from a drug that can be tolerated by a patient.

#### Truncated amyloid beta

Shortened (truncated) forms of the amyloid beta protein.

