

# INTERIM REPORT

January – March 202<sup>o</sup>

New data continues to strengthen lecanemab

# **EVENTS DURING THE FIRST QUARTER 2021**

- BioArctic presented findings at the AD/PD conference suggesting that lecanemab could be developed into a disease modifying treatment for individuals with Down's syndrome with dementia.
- New preliminary results presented from the ongoing open-label extension of the Phase 2b study in early Alzheimer's disease continued to support the effect of the drug candidate lecanemab on brain amyloid levels.

## **EVENTS AFTER THE PERIOD**

• Lecanemab Phase 2b study results in early Alzheimer's disease published in peer-reviewed journal, Alzheimer's Research and Therapy, and lecanemab confirmatory Phase 3 Clarity AD clinical trial completed enrollment with 1,795 patients.

# FINANCIAL SUMMARY JANUARY - MARCH 2021

- Net revenues for the period amounted to MSEK 7.2 (36.4)
- Operating profit amounted to MSEK -29.1 (3.8)
- Profit for the period amounted to MSEK -29.1 (3.6) and earnings per share were SEK -0.33 (0.04)
- Cash flow from operating activities amounted to MSEK -37.5 (-36.3)
- Cash and cash equivalents at the end of the period amounted to MSEK 960.5 (1,077.3)

# **KEY FINANCIAL PERFORMANCE INDICATORS**

	Q	Q1		
MSEK	2021	2020	2020	
Net revenues	7.2	36.4	62.3	
Other operating income	1.7	3.4	3.6	
Operating profit/loss	-29.1	3.8	-85.0	
Operating margin, %	neg	10.4	neg	
Profit/loss for the period	-29.1	3.6	-68.5	
Earnings per share before dilution, SEK	-0.33	0.04	-0.78	
Earnings per share after dilution, SEK	-0.33	0.04	-0.78	
Equity per share, SEK	9.98	11.11	10.30	
Cash flow from operating activities	-37.5	-36.3	-92.3	
Cash flow from operating activities per share, SEK	-0.43	-0.41	-1.05	
Equity/assets ratio, %	87.3	85.6	86.4	
Return on equity, %	-3.26	0.36	-7.28	
Share price at the end of the period, SEK	91.00	61.50	95.40	

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



The 15th International Conference on Alzheimer's and Parkinson's diseases and related neurological disorders was held in March. The conference is one of the year's most important scientific forums in neurological diseases, gathering researchers and companies from around the globe. The new developments and analyses that were presented provided important information about developments in the field. In summarizing our impressions from this year's conference, we can confirm that scientific support has further increased for anti-amyloid beta antibodies that reduce harmful forms of amyloid in the brain, alleviate the symptoms, and slow the progress of the disease in patients with Alzheimer's disease. Today, there are four antibodies against amyloid-beta that are in the late clinical development phase. Of these, lecanemab and two others have already demonstrated clinical effect.

At the same time, understanding of how crucial the binding profiles of individual antibodies are for achieving a positive clinical effect and reducing the risk of side effects is increasing. Lecanemab, the drug candidate generated by BioArctic and developed by Eisai, stands out with its unique and highly specific binding profile. By binding selectively to the soluble aggregated forms of amyloid-beta — oligomers and protofibrils, the forms most toxic to nerve cells — lecanemab has been shown to remove amyloid-beta quickly and effectively from the brain.

During the conference, Eisai presented new data from the open-label extension study of the Phase 2b study in early Alzheimer's disease that further strengthens lecanemab. We can now state that more than 80 percent of the patients who received the highest dosage of lecanemab, both in the core study and in the open-label extension study, showed reduction of amyloid-beta to non-pathological levels when their brains were studied using diagnostic imaging. This effect can be seen after only 12 months of treatment. Lecanemab also has a continued low occurrence of ARIA-E compared to such side effects reported by competitors' antibody therapy under development. At the same time as the promising results from the open-label extension study are being presented, the large global Phase 3 study of lecanemab in 1,795 patients with early Alzheimer's disease is under way, and according to Eisai the results of the study are expected in September 2022.

During the AD/PD Conference, we were able to present results for the first time from our own research concerning Down's syndrome with dementia. Persons with Down's syndrome suffer from dementia to a much greater extent than others, and our research has now confirmed that these individuals have significantly elevated levels of the soluble aggregated forms of amyloid-beta (oligomers and protofibrils) compared with the control group. In other words, the pathology is similar to that seen in Alzheimer's disease. Treatment with lecanemab therefore has the potential to slow the development of dementia in these individuals.

The latest developments in Parkinson's disease were also in focus during the AD/PD Conference. New clinical data from the Parkinson's projects of other pharma companies provided important learnings concerning the design of the Phase 2 study that our partner, AbbVie, is now preparing for ABBV-0805, the drug candidate outlicensed by BioArctic. In Parkinson's disease as well, it is clear that the binding profile is crucial to how well an antibody will function.

Our research into the blood-brain barrier and the technology which we call Brain Transporter and is intended to facilitate the transport of antibodies into the brain, continues to perform well and we now have two Alzheimer's projects in progress linked to our technology.

When I hear about all the research being conducted into the central nervous system, it strengthens my conviction that BioArctic's research is on the global leading edge. It was therefore particularly gratifying recently to have the honor of presenting BioArctic and our vital research at a digital meeting, arranged by the Confederation of Swedish Enterprise, with the Swedish Royal Family. The royal family showed an impressive level of engagement in these crucial questions.

In summary, the research being conducted by our fantastic employees holds great promise and has the potential to make a big difference for patients around the world.

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 delivered high quality drug projects that have resulted in significant 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 five focus areas:

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

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 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 selectivity of antibodies, binding and elimination of the harmful soluble aggregated forms of the amyloid beta protein (oligomers/protofibrils) in the brain.

# Project portfolio

BioArctic has a balanced, competitive portfolio consisting of unique product candidates, technology platforms and methods for diagnostics. 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.

As of March 31, 2021, the project portfolio consisted of:

	Project	Partner	Discovery Preclinical Phase 1 Phase 2 Phase 3
ALZHEIMER'S DISEASE	Lecanemab (BAN2401) Clarity AD	Eisai <sup>1</sup>	Early Alzheimer's disease <sup>3</sup>
	Lecanemab (BAN2401) AHEAD 3-45	Eisai <sup>1</sup>	Preclinical (asymptomatic) Alzheimer's disease <sup>4</sup>
	BAN2401 back-up	Eisai	
	AD1801		
	AD1502		
	AD1503		
	AD-BT2802		
	AD-BT2803		
	AD2603		
PARKINSON'S DISEASE	ABBV-0805 <sup>2</sup>	AbbVie	
	PD1601	AbbVie	
	PD1602	AbbVie	
OTHER CNS DISORDERS	Lecanemab (BAN2401)		Down's syndrome <sup>5</sup> Traumatic brain injury <sup>5</sup>
	ND3014		
BLOOD-BRAIN BARRIER	Brain Transporter (BT) technology platform		
DIAGNOSTICS	Imaging and biochemical biomarkers – Alzheimer's disease		
	Imaging and biochemical biomarkers – Parkinson's disease	AbbVie	

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

<sup>2)</sup> AbbVie in-licensed BAN0805 in late 2018 and develops the antibody with the designation ABBV-0805

<sup>3)</sup> Mild cognitive impairment due to Alzheimer's disease and mild Alzheimer's disease

<sup>4)</sup> Normal cognitive function with intermediate or elevated levels of amyloid in the brain

<sup>5)</sup> 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 progress 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 owns the rights to the BAN2401 back-up in Alzheimer's disease. BioArctic has four additional antibodies against Alzheimer's disease in its project portfolio.

# 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).

Clarity AD is the pivotal and confirmatory Phase 3 study. It is based on the 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.

This Phase 3 study is a global placebo-controlled, doubleblind, parallel-group, randomized study 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. Patients are allocated in a 1:1 ratio to receive intravenous infusion twice a month, either with placebo or with lecanemab 10 mg/kg. The primary endpoint is the change from baseline in the cognition and function scale Clinical Dementia Rating-Sum of Boxes (CDR-SB) at 18 months of treatment. Changes in the clinical scales AD composite score (ADCOMS), AD Assessment Scale-Cognitive Subscale (ADAS-Cog) and a clinical scale focusing of activities of daily living particularly relevant in mild cognitive impairment, (ADCS-ADL-MCI), will be key secondary endpoints together with brain amyloid levels as measured by amyloid-PET. According to Eisai, the goal is to

be able to present results from the study in September 2022 and thereafter submit an application for marketing approval.

An open-label extension study, without placebo control, with continued treatment with lecanemab with the highest study dose for all the participants in the Phase 2b study is in progress. During 2020 Eisai presented data from the study showing that the patients who had previously received placebo in the Phase 2b study had rapidly and continually decreasing amyloid levels in the brain after three, six and twelve months of treatment with lecanemab. Additionally, with treatment with lecanemab less than 10 percent of patients experienced ARIA-E side effects, consistent with previously reported data. This picture was further strengthened in March 2021 when Eisai at the 15th International Conference on Alzheimer's and Parkinson's Diseases and related neurological disorders, AD/PD, presented results showing that the effects of lecanemab on reducing amyloid in the brain on average persist for at least two years following discontinuation of treatment. At the same time, it was verified that when lecanemab is administered in a dose of 10 mg/kg every other week, amyloid levels in the brains of more than 80 percent of patients decreased to levels under those that define Alzheimer's disease. These results were achieved in both the primary study and in the open-label extension study, and the effect could be detected after only 12 months of treatment.

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.

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 clinical 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 that is being conducted with funding from the United States National Institute on Aging (NIA) and Eisai, 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 substudies 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.

# Back-up candidate to BAN2401 (collaboration with Eisai)

The antibody is a refined version of BAN2401 for the treatment of Alzheimer's disease. The antibody was developed by BioArctic 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, AD1502, AD1503 and AD2603 (owned by BioArctic)

BioArctic has four 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.

# 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 our 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. In 2018, AbbVie acquired a 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 (collaboration with AbbVie)

The drug candidate ABBV-0805 is a monoclonal antibody that selectively binds to and eliminates oligomers and protofibrils of alpha-synuclein. The goal is to develop a disease modifying treatment that stops or slows down disease progression.

In February 2019, the U.S. Food and Drug Administration, FDA, approved the application to conduct a clinical study with ABBV-0805 and the Phase 1 study started in March 2019. In July 2020, AbbVie decided to develop a detailed plan to accelerate ABBV-0805 into a Phase 2 study in Parkinson's disease patients. AbbVie drives and finances the continued clinical development of ABBV-0805.

The scope of the drug candidate ABBV-0805 may be expanded to include, for example, Lewy body dementia and multiple system atrophy.

The project is based on research from Uppsala University.

# Projects PD1601 and PD1602 (collaboration with AbbVie)

The antibody projects PD1601 and PD1602 target alphasynuclein for treatment of Parkinson's disease. The goal is to develop a disease modifying treatment that stops or slows down disease progression. The projects are included in the collaboration with AbbVie.

### 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, which is currently being clinically evaluated for Alzheimer's disease, can potentially also be used for other indications. BioArctic owns the rights for all such indications. The antibody lecanemab is in the preclinical phase as a potential treatment of cognitive disorders in conjunction with Down's syndrome and traumatic brain injury. BioArctic and Eisai presented at the AD/PD conference findings suggesting that lecanemab also could be developed into a disease modifying treatment benefiting individuals with Down's syndrome with dementia.

# Project ND3014 (owned by BioArctic)

Research to develop new antibodies for treating neurodegenerative disorders is ongoing at BioArctic. ND3014 is intended to be a disease modifying treatment with potential to address various neurodegenerative disorders. The project is in an early 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 and researchers at Uppsala University are collaborating on developing technology that facilitates the passage of antibodies across the blood-brain barrier. Together with Uppsala University, BioArctic received grants from Sweden's Innovation Agency, Vinnova, for continued research in the blood-brain barrier project. The technology, which is at an early stage, has shown highly encouraging results and has significant potential for many different treatments for various diseases of the brain.

# DIAGNOSTICS

# Alzheimer's disease diagnostics (owned by BioArctic) and Parkinson's disease diagnostics (in collaboration with AbbVie)

BioArctic is engaged in the development of new diagnostic methods that improve the ability to diagnose and monitor the treatment of Alzheimer's and Parkinson's disease. The company conducts a number of projects in collaboration with commercial and academic partners. Among other things, BioArctic is developing biochemical methods based on the company's antibodies to be applied to cerebral spinal fluid (CSF) testing. Beyond this, the company is exploring the possibilities to measure biomarkers with a simple blood test. BioArctic is also active in projects to improve the diagnostic imaging (PET) of the brain of patients. The goal is to create tools to better diagnose the disease, follow the disease progression and objectively measure the effect of drug treatment.

# 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 7.2 (36.4). The decrease in the quarter compared to last year relates to lower revenue from the Parkinson's program, which was according to plan together with the fact that during the same quarter last year a lump sum of MSEK 22.8 was recorded attributable to a remeasurement of the total costs of the Parkinson's program.

Other operating income relates to research grants and operating exchange rate gains. Other operating income amounted to MSEK 1.7 (3.4). The decrease is mainly attributable to exchange rate gains.

Total operating expenses for the first quarter amounted to MSEK -38.0 (-36.0). Project expenses increased due to a higher activity within own projects. The expenses for personnel in the first quarter increased as a result of an increase in the number of employees. Other external costs decreased to MSEK -6.0 (-6.8) as a result of reduced consultant costs and travel. 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 -29.1 (3.8) for the first quarter. The decrease in operating profit was primarily attributable to lower revenue from the Parkinson's program, which was according to plan.

Net financial items totaled MSEK 0.0 (0.8) 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 according to IFRS 16 Leases.

Profit (loss) amounted to MSEK -29.1 (3.6) for the first quarter.

Earnings per share before and after dilution amounted to SEK -0.33 (0.04) for the first quarter.



### LIQUIDITY AND FINANCIAL POSITION

Equity amounted to MSEK 878.6 as of March 31, 2021 compared with MSEK 907.3 as of December 31, 2020. This corresponds to equity per outstanding share of SEK 9.98 (10.30).

The equity/asset ratio increased from 86.4 percent as of December 31, 2020 to 87.3 percent as of March 31, 2021.

The Group's cash and cash equivalents consist of bank balances that at the end of the period amounted to MSEK 960.5 compared with MSEK 999.9 as of December 31, 2020. The decrease in right of use assets of MSEK 1.4 is attributable to depreciation in accordance with IFRS 16, which is mainly related to the head office's lease. The leasing liabilities of MSEK 19.4 (20.8) is related to the above described right of use assets. There were no loans as of March 31, 2021 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 -37.5 (-36.3).

Investments in the first quarter amounted to MSEK 0.9 (0.3). The investments are mainly related to laboratory equipment.

Cash flow from financing activities amounted to MSEK -1.8 (-2.8) for the first quarter and relates to the amortization of leasing liabilities.

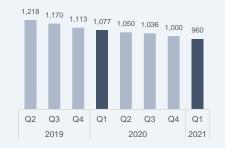
#### **PARENT COMPANY**

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

# **EVENTS DURING THE PERIOD JANUARY - MARCH 2021**

- BioArctic presented findings at the AD/PD conference suggesting that lecanemab
  also could be developed into a disease modifying treatment benefiting individuals
  with Down's syndrome with dementia.
- New preliminary results that Eisai presented at the AD/PD conference from the
  ongoing open-label extension of the Phase 2b study in early Alzheimer's disease
  continued to support the effect of lecanemab on brain amyloid levels.
- Eisai increased the number of participants in Clarity AD to ensure a robust dataset with 200 patients. Eisai still expects readout by September 2021.
- BioArctic received patent approval from the European Patent Office for antibodies against truncated amyloid beta, which are linked to the AD1503 project.

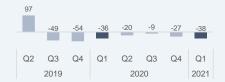
# Cash and cash equivalents (MSEK)



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

31 Mar 2021	31 dec 2020
12.2	13.6
7.2	7.1
960.5	999.9
941.1	979.2
	7.2 960.5

# Cash flow from operating activities (MSEK)





# Other information

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

Lecanemab Phase 2b study results in early Alzheimer's Disease published in peer-reviewed journal, Alzheimer's Research and Therapy, and lecanemab confirmatory Phase 3 Clarity AD clinical trial completed enrollment with 1,795 patients.

# **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 the quarter, BioArctic's patent portfolio consisted of 13 patent families with more than 230 granted patents and more than 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. To date, approximately MEUR 65 has been received and recognized.

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 is responsible for the clinical development. The total value of the agreement could amount to MUSD 755 in addition to royalty payments. To date, MUSD 130 has been received. For more information regarding BioArctic's two large collaboration partners, please see the Annual Report 2020 on pages 22, 29, 43 and 44.

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 the risks of the business. 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 2020 on pages 50 - 53.

### **FLUCTUATIONS IN REVENUE GENERATION**

Currently, BioArctic does not have any drugs on the market. The company develops a number of drug candidates and diagnostics 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, diagnostics, 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. 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, innovative blood-brain barrier technology and diagnostics, 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 180 – 220 for the fiscal year January – December 2021.

During 2020 operating expenses were MSEK 151 and during the last three years the average annual level of the operating expenses has been approximately MSEK 190.

### **EMPLOYEES**

At the end of the period, the number of employees was 47 (43) of which 18 (16) are men and 29 (27) women. Just over 80 percent work in R&D and approximately 70 percent are PhDs. In the organization there is one Associate Professor, two Professors and three medical doctors.

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, 2021, these corresponded to 10 (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, 20211

EAROLOT ONARCHOEDERO AO OF MARON 31, 2021						
	Num	ber	Share	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		
Gladiator	-	3,448,563	3.9	1.6		
Third AP-Fund	-	2,903,492	3.3	1.3		
Unionen	-	2,391,835	2.7	1.1		
Swedbank Robur Funds	-	1,843,058	2.1	0.8		
Investment AB Öresund	-	1,430,000	1.6	0.7		
Handelsbanken Funds	-	1,399,045	1.6	0.6		
Wellington Management	-	1,320,133	1.5	0.6		
Tot. 10 largest shareholde	14,399,996	56,750,479	80.8	92.2		
Other	-	16,909,510	19.2	7.8		
Total	14,399,996	73,659,989	100.0	100.0		

### **ANNUAL GENERAL MEETING 2021**

The Annual General Meeting (AGM) for BioArctic will be held on 6 May. As a measure to reduce the risk of spreading the coronavirus and in the light of the uncertainty about what restrictions and guidelines will apply to meetings at the time of the meeting, the Board of Directors of BioArctic has

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

decided that the AGM will be held solely through advance voting using the postal voting method. There will thus be no possibility of physically attending the AGM, either in person or by proxy. The full notice to attend, and the bases for decisions in the matters arising at the Annual General Meeting, are available on the company's website, www.bioarctic.com under the Governance tab.

In light of that the Annual General Meeting will be held without physical presence, BioArctic invites to a digital information meeting on April 27, 2021 at 15.00 CET. The chairman of the board and the CEO will participate at the information meeting. The chairman of the board will present the proposals to the Annual General Meeting and answer questions. The Company's CEO Gunilla Osswald will also present the Company's operations and development in 2020. Shareholders who wish to participate in the information meeting are asked to register no later than April 22 at 17.00 by e-mail to Else-Britt Lundgren, at the address arsstamma@bioarctic.se.

### 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 shall 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, 550,000 employee warrants were allocated, of which 10,000 were allocated in the first quarter 2021. The allocation of employee warrants had a dilutive effect corresponding to 500,000 shares, or 0.57 percent, at the end of the period. More information is available on <a href="https://www.bioarctic.com">www.bioarctic.com</a>

The information was submitted for publication, though the agency of the named contact persons, at 8:00 a.m. CET on April 21, 2021.

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

Stockholm, Sweden, April 21, 2021

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Gunilla Osswald CEO, BioArctic AB (publ)

# INVITATION TO PRESENTATION OF INTERIM REPORT FOR THE PERIOD JANUARY – MARCH 2021

BioArctic invites investors, analysts and media to an audiocast with teleconference (in English) today, April 21, 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-q1-2021

To participate in the conference, please call: +46 8 505 583 51 (Sweden), +45 781 501 08 (Denmark), +31 107 129 162 (Netherlands), +47 239 639 38 (Norway), +41 225 675 632 (Switzerland), +44 333 300 90 32 (UK), +49 692 222 203 77 (Germany) or +1 833 249 8405 (USA)

### **CALENDAR 2021**

Annual General Meeting 2021 May 6, 2021

Half-Year report Jan-Jun 2021 July 9, 2021, at 08:00 a.m. CET

Interim report Jan-Sep 2021 Oct 21, 2021, at 08:00 a.m. CET

Full Year Report Jan-Dec 2021 Feb 3, 2022, 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**

	Q1	Q1		
kSEK	2021	2020	2020	
Net revenues (note 4)	7,177	36,431	62,347	
Other operating income	1,687	3,385	3,597	
Operating revenues	8,863	39,816	65,943	
Operating expenses				
Project related expenses	-11,299	-10,486	-50,242	
Other external expenses	-6,008	-6,755	-23,370	
Personnel expenses	-17,264	-15,967	-62,977	
Depreciations of tangible assets	-3,275	-2,522	-11,013	
Other operating expenses	-161	-291	-3,353	
Operating profit/loss	-29,144	3,794	-85,012	
Financial income	269	1,097	7	
Financial expenses	-231	-289	-1,686	
Profit/loss before tax	-29,106	4,602	-86,691	
Tax	20	-1,048	18,174	
Profit/loss for the period	-29,086	3,554	-68,517	
Earnings per share				
Earnings per share before dilution, SEK	-0.33	0.04	-0.78	
Earnings per share after dilution, SEK	-0.33	0.04	-0.78	

# CONSOLIDATED STATEMENT OF COMPREHENSIVE INCOME

	Q1		Jan-Dec	
kSEK	2021	2020	2020	
Profit/loss for the period	-29,086	3,554	-68,517	
Other comprehensive income	-	=	<u>-</u>	
Comprehensive income for the period	-29,086	3,554	-68,517	

# **CONSOLIDATED BALANCE SHEET**

kSEK	31 Mar 2021	31 Mar 2020	31 dec 2020
ASSETS			
Tangible fixed assets	17,660	9,046	18,120
Right-to-use assets	20,418	25,792	21,820
Deferred tax assets	457	337	452
Other financial assets	1,571	1,511	1,562
Current assets excluding cash and cash equivalents	5,455	28,612	8,420
Cash and cash equivalents	960,466	1,077,255	999,940
TOTAL ASSETS	1,006,027	1,142,552	1,050,313
EQUITY AND LIABILITIES			
Equity	878,602	978,366	907,299
Deferred tax liabilities	20,666	38,685	20,666
Non-current lease liabilities	12,207	18,200	13,627
Current lease liabilities	7,223	6,380	7,141
Other current liabilities	7,710	11,574	17,887
Accrued expenses and deferred income	79,618	89,347	83,692
EQUITY AND LIABILITIES	1,006,027	1,142,552	1,050,313

# CONSOLIDATED STATEMENT OF CHANGE IN EQUITY (CONDENSED)

	31 Mar 2021	31 Mar 2020	31 dec 2020
Opening balance at 1 January	907,299	974,497	974,497
Comprehensive income for the period	-29,086	3,554	-68,517
Share-based payments	388	315	1,319
Paid dividend	-	-	-
Closing balance	878,602	978,366	907,299

# CONSOLIDATED STATEMENT OF CASH FLOW (CONDENSED)1

	Q	1	Jan-Dec
kSEK	2021	2020	2020
Operating profit	-29,144	3,794	-85,012
Adjustment for non-cash items	511	-27,887	-19,991
Interest received/paid	39	-289	-1,679
Income tax paid	16	-11,116	-12,217
Cash flow from operating activities before changes in working capital	-28,579	-35,498	-118,899
Change in working capital	-8,921	-852	26,558
Cash flow from operating activities after changes in working capital	-37,500	-36,350	-92,341
Cash flow from investing activities	-949	-257	-12,524
Cash flow from financing activities	-1,813	-2,755	-6,598
Cash flow for the period	-40,261	-39,361	-111,463
Cash and cash equivalents at beginning of period	999,940	1,112,770	1,112,770
Exchange rate differences in cash and cash equivalents	788	3,846	-1,367
Cash and cash equivalents at end of period	960,466	1,077,255	999,940

<sup>1)</sup> For the comparison period Jan-Dec 2020, an adjustment of kSEK 1,460 has been made between the lines Change in working capital and Cash flow from investing activities compared with the Full Year Report 2020.

# **CONSOLIDATED QUARTERLY DATA**

	2021	2020	2020	2020	2020	2019	2019	2019
MSEK	Q1	Q4	Q3	Q2	Q1	Q4	Q3	Q2
Income statement								
Net revenues	7	8	11	7	36	26	21	171
Other operating income	2	1	1	-2	3	0	9	-1
Operating expenses	-38	-40	-32	-43	-36	-48	-40	-44
Operating profit/loss	-29	-30	-21	-38	4	-21	-11	127
Operating margin, %	neg	neg	neg	neg	10.4	neg	neg	74.0
Profit/loss for the period	-29	-13	-21	-38	4	-17	-8	100
Balance sheet								
Fixed assets	40	42	36	35	37	39	40	41
Current assets	5	8	4	14	29	32	29	16
Cash and cash equivalents	960	1,000	1,036	1,050	1,077	1,113	1,170	1,218
Equity	879	907	920	940	978	974	991	1,000
Deferred tax liabilities	21	21	39	39	39	39	33	33
Lease liabilities	19	21	22	23	25	27	28	30
Current liabilities	87	102	95	98	101	143	187	213
Cash flow								
From operating activities	-38	-27	-9	-20	-36	-54	-49	97
From investing activities	-1	-7	-3	-1	-0	-0	-2	-1
From financing activities	-2	-1	-1	-2	-3	-2	-2	-134
Cash flow for the period	-40	-35	-14	-23	-39	-56	-53	-37
Key ratios								
Equity/asset ratio, %	87.3	86.4	85.5	85.5	85.6	82.4	80.0	78.4
Return on equity, %	-3.3	-1.4	-2.2	-4.0	0.4	-1.7	-0.8	9.9
Data per share								
Earnings per share before dilution, SEK	-0.33	-0.15	-0.23	-0.43	0.04	-0.19	-0.09	1.14
Earnings per share after dilution, SEK	-0.33	-0.15	-0.23	-0.43	0.04	-0.19	-0.09	1.14
Equity per share, SEK	9.98	10.30	10.45	10.68	11.11	11.07	11.26	11.35
Cash flow operating activities per share, SEK	-0.43	-0.30	-0.11	-0.22	-0.41	-0.62	-0.56	1.10
Share price at the end of the period, SEK	91.00	95.40	88.95	73.35	61.50	94.90	61.75	74.40
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	88,855	88,355	88,082	88,060	88,060	88,060	88,060	88,060

# Financial statements, Parent company

# PARENT COMPANY INCOME STATEMENT

		1	Jan-Dec	
kSEK	2021	2020	2020	
Net revenues	7,177	36,431	62,347	
Other operating income	1,687	3,385	3,597	
Operating revenues	8,863	39,816	65,943	
Operating expenses				
Project related expenses	-11,299	-10,486	-50,242	
Other external expenses	-8,064	-8,632	-31,161	
Personnel expenses	-17,264	-15,967	-62,977	
Depreciations of tangible assets	-1,399	-802	-3,829	
Other operating expenses	-161	-291	-3,353	
Operating profit/loss	-29,323	3,638	-85,618	
Financial income	269	1,097	7	
Financial expenses	-14	-27	-707	
Profit/loss after financial items	-29,068	4,709	-86,318	
Change in tax allocation reserves	_	-	81,865	
Profit/loss before tax	-29,068	4,709	-4,453	
Тах	12	-1,070	75	
Profit/loss for the period	-29,056	3,638	-4,378	

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)

	31 Mar 2021	31 Mar 2020	31 dec 2020
ASSETS			
Tangible fixed assets	17,660	9,046	18,120
Deferred tax assets	337	267	325
Other financial assets	1,621	1,611	1,612
Current assets excluding cash and cash equivalents	6,870	29,945	9,882
Cash and cash equivalents	960,419	1,077,159	999,892
TOTAL ASSETS	986,908	1,118,027	1,029,831
EQUITY AND LIABILITIES			
Equity	804,960	840,641	833,628
Tax allocation reserve	94,809	176,674	94,809
Other current liabilities	7,521	11,365	17,702
Accrued expenses and deferred income	79,618	89,347	83,692
EQUITY AND LIABILITIES	986,908	1,118,027	1,029,831

# **Notes**

#### **NOTE 1 GENERAL INFORMATION**

This Interim Report for the period January – March 2021 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. The Parent Company 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 2021 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 2020. New and amended IFRS standards and interpretations applied from 2021 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		Jan-Dec	
kSEK	2021	2020	2020	
Geographic breakdown of net revnues				
Europe	2,365	27,975	33,805	
Asia	4,812	8,456	28,541	
Total net revenues	7,177	36,431	62,347	
Net revenues per revenue type				
Milestone payments, recognized at a given point in time	-	-	=	
Income from research collaborations, recognized over time	7,177	36,431	62,347	
Total net revenues	7,177	36,431	62,347	

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. In conjunction with a restatement of the total costs of the Parkinson's program in light of better performance than originally planned, a positive lump sum of MSEK 22.8 in revenue has been recorded during the first quarter 2020. As of March 31,

2021, MSEK 637.0 has been recognized as revenue and the remaining amount to be recognized as a revenue up until the completion of the project is MSEK 64.6. MSEK 2.4 was recognized as revenue in the period January–March 2021. A research collaboration agreement with Eisai began in January 2020. After supplementary orders, the agreement covers approximately MEUR 3.67, or approximately MSEK 38.5. The revenue for this research collaboration is recognized over time based on the fulfillment of the performance obligation. At March 31, 2021, MSEK 33.3 had been recognized as revenue, of which MSEK 4.8 during the period.

# 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.

Definition
Other income than net revenue
Result before financial items
Operating profit divided by net revenues
The cash flow from operating activities for the period divided by the weighted number of shares
Adjusted equity divided by total assets
Net income divided by equity expressed as a percentage
Adjusted equity divided by the number of shares at the end of the period

# Glossary

### **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 (Aß)

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.

# 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.

### 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.

### PFT

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

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#### 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.

### **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.

