## INTERIM REPORT

January - March 2020

# Operations performing according to plan

#### **KEY EVENTS DURING THE FIRST QUARTER 2020**

As of today, BioArctic has not experienced any noteworthy disruptions to its operations owing to the COVID-19 pandemic.

#### FINANCIAL SUMMARY JANUARY - MARCH 2020

- Net revenues for the period amounted to MSEK 36.4 (63.4), a decrease of 27 MSEK
- Operating profit amounted to 3.8 MSEK (17.3) and the operating margin was 10.4 percent (27.3)
- Profit for the period amounted to MSEK 3.6 (13.6) and earnings per share were SEK 0.04 (0.15)
- Cash flow from operating activities amounted to MSEK -36.3 MSEK (333.6)
- Cash and cash equivalents at the end of the period amounted to MSEK 1,077.3 (1,255.6)

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

	Q		Jan-Dec	
MSEK	2020	2019	2019	
Net revenues	36.4	63.4	281.8	
Other operating income	3.4	6.9	14.8	
Operating profit/loss	3.8	17.3	112.5	
Operating margin, %	10.4	27.3	39.9	
Profit/loss for the period	3.6	13.6	88.5	
Earnings per share before dilution, SEK	0.04	0.15	1.00	
Earnings per share after dilution, SEK <sup>1</sup>	0.04	0.15	1.00	
Equity per share, SEK¹	11.11	11.71	11.07	
Cash flow from operating activities	-36.3	333.6	327.2	
Cash flow from operating activities per share, SEK	-0.41	3.79	3.72	
Equity/assets ratio, %	85.6	78.5	82.4	
Return on equity, %	0.36	1.33	8.88	
Share price at the end of the period	61.50	78.00	94.90	

<sup>&</sup>lt;sup>1</sup> There is no dilutive effect as the average market price for the period is lower than the exercise price as of March 31, 2020.

## Comments from the CEO



BioArctic's work towards providing patients suffering from neurodegenerative disorders access to effective treatments and improved diagnostics continues to make solid progress. The impact of COVID-19 on society at large, on the global economy and on people's daily lives is both alarming and difficult to process. Under the present circumstances, I am pleased that we have so far been able to continue our operations without noteworthy disruptions. Our success in building up cash reserves of more than SEK 1 billion is a great source of comfort in a situation where the financial markets are rife with uncertainty. BioArctic has a business model in which our partners conduct and finance the major clinical trials in Alzheimer's and Parkinson's diseases. This makes it possible for us to focus on our own innovative preclinical projects and on developing the medicines of the future for patients with disorders of the central nervous system.

Eisai, our partner in the field of Alzheimer's disease, continues to show a high degree of commitment to our most advanced drug candidate, BAN2401, and the program continues to expand. BAN2401 is being evaluated in a confirmatory Phase 3 study in patients with early Alzheimer's disease, and we note that the study has recently been expanded into several more countries. It is especially gratifying for us to note that Sweden is also part of the study, and that the first patients have now been enrolled. The aim with the Phase 3 study is to confirm the positive results of the Phase 2b study, as well as to form the basis for market registration. Eisai expects results from the study to become available in 2022.

At the same time, the open-label extension study continues with patients who took part in the previously completed Phase 2b study. The promising initial results were presented late last year and we look forward to the continued follow-ups. Furthermore, Eisai is preparing a broad Phase 3 program (AHEAD 3-45) in partnership with the Alzheimer's Clinical Trials Consortium (ACTC) aimed at preventing Alzheimer's disease by administering BAN2401 in the earliest stages of disease development. The program consists of two sub-studies with approximately 1,000 and 400 subjects, respectively.

In the Parkinson's disease area, our partner AbbVie has initiated recruitment for the second part of the ongoing Phase 1 program, in which our outlicensed drug candidate ABBV-0805 will be evaluated in patients for the first time. In parallel, we are focusing on delivering the subsequent projects that are included in this successful collaboration with AbbVie. The reputable periodical *Drug Discovery Today* recently published an article regarding the effective collaboration between BioArctic and AbbVie in Parkinson's disease. Being seen as an appreciated collaborating partner is a mark of quality for BioArctic and is important for our future projects and potential partners. It is our hope that ABBV-0805 will be one of the first disease-modifying treatments for Parkinson's disease, which is devastating to quality of life for patients and which incurs large costs to society.

Our pre-clinical portfolio also continued to develop well during the first quarter, with the added project in Alzheimer's disease and a project that is believed could impact several different neurodegenerative disorders. At the same time, we are working further on our diagnostics projects, the purpose of which is to identify people with neurodegenerative disorders at an early stage. Research is ongoing into our unique bloodbrain barrier technology, which improves the passage of antibody drugs into the brain. In pace with securing patent protection for our projects, we will become increasingly transparent in terms of the progress we are making.

Our innovative and broad portfolio, the extensive collaborations with international pharma companies, our support in the academic community and a strong cash position make BioArctic a unique Swedish biopharma company. Our projects, focused on disorders of the central nervous system, are tremendously important in our ambition to improve life for these patients.

Gunilla Osswald CEO, BioArctic AB

## BioArctic in short

BioArctic AB (publ) is a Swedish biopharma company that develops 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 diseases, principally Alzheimer's and Parkinson's 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 that our research generates 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 long experience in developing drugs from idea to market. Under BioArctic's business model, the company itself pursues project development at an early stage and then, at an appropriate juncture, licenses certain commercial rights to global pharmaceutical companies. In recent years, BioArctic has successfully delivered innovative drug projects that have resulted in advantageous partnership agreements in two major disease areas with significant 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 challenge.

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 antibodies currently in clinical phase bind selectively and eliminate the toxic soluble aggregated forms (oligomers/protofibrils) of these proteins in the brain with the aim of having a disease modifying effect.

## Project portfolio

BioArctic has a balanced, competitive portfolio consisting of unique product candidates, technological platforms and diagnostic tools. All our 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, 2020, the project portfolio consisted of:

- Two drug candidates in clinical phase: BAN2401 for early Alzheimer's disease (Phase 3) and ABBV-0805 for Parkinson's disease (Phase 1)
- Three projects in preclinical phase: BAN2401 for other indications such as Down's syndrome with dementia; BAN2401 back-up for Alzheimer's disease; and biomarkers and diagnostics for Alzheimer's disease
- Eight projects in research phase: four projects for Alzheimer's disease (AD1801, AD1502, AD1503, AD2603); two projects for Parkinson's disease (PD1601, PD1602); one project for other CNS-disorders (ND3014); and biomarkers and diagnostics for Parkinson's disease
- One blood-brain barrier technology for increased uptake into the brain of antibodies and other biologic drugs

		1					
	Project	Partner	Discovery	Preclinical	Phase 1	Phase 2	Phase 3
ALZHEIMER'S DISEASE	BAN2401	Eisai, Biogen <sup>1</sup>					
	BAN2401 back-up	Eisai					
	AD1801						
	AD1502			,			
	AD1503			,			
	AD2603						
PARKINSON'S DISEASE	ABBV-0805 <sup>2</sup>	AbbVie					
	PD1601	AbbVie					
	PD1602	AbbVie					
OTHER CNS DISORDERS	BAN2401 Downs syndrome <sup>3</sup> Traumatic brain injury						
	ND3014						
BLOOD-BRAIN BARRIER TECHNOLOGY	BBB technology platform						
DIAGNOSTICS	Imaging and biochemical biomarkers – Alzheimer's disease						
	Imaging and biochemical biomarkers – Parkinson's disease	AbbVie					

- 1) Partnered with Eisai for BAN2401 for treatment of Alzheimer's disease. Eisai entered partnership with Biogen regarding BAN2401 in 2014
- 2) AbbVie in-licensed BAN0805 in late 2018 and develops the antibody with the designation ABBV-0805
- 3) Dementia and cognitive impairment associated with Down's syndrome and 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, BAN2401, has shown positive results in a large Phase 2b study and is currently being evaluated in Phase 3. The development of BAN2401 in Alzheimer's disease is being financed and pursued by BioArctic's partner Eisai, which also owns the rights to the BAN2401 back-up. BioArctic has four additional antibodies against Alzheimer's disease in its project portfolio.

#### Drug candidate BAN2401 (collaboration with Eisai)

In Alzheimer's disease, the amyloid-beta protein clumps together into increasingly larger aggregates – from the harmless 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. Drug candidate BAN2401 is an antibody designed to bind most strongly to oligomers and protofibrils, thus helping the body's immune system to eliminate them from the brain.

A clinical Phase 2b study with BAN2401 in 856 patients with early Alzheimer's disease demonstrates dose dependent, clinically meaningful and statistically significant effects of BAN2401 on several clinical endpoints and on biomarkers and was well tolerated.

Based on the results of the Phase 2b clinical study and after discussion with regulatory agencies, our partner Eisai has started and is now conducting the global confirmatory Phase 3 study with BAN2401 in early Alzheimer's disease patients to support a regulatory filing for BAN2401. The start of the study triggered a MEUR 15 milestone payment to BioArctic in May 2019.

The Phase 3 study (Clarity AD) is a global placebo-controlled, double-blind, parallel-group, randomized study in 1,566 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 either placebo or treatment. Patients receive placebo or BAN2401 10 mg/kg every other week through intravenous infusion. 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) and AD Assessment Scale-Cognitive Subscale (ADAS-Cog) will be key secondary endpoints together with brain amyloid levels as measured by amyloid-PET. According to Eisai, results from the study are targeted for 2022.

An open-label extension study, without placebo control, with continued BAN2401 treatment with the highest study dose for the participants in the Phase 2b study is in progress. A subset of patients from the core Phase 2b study enrolled in the open-label extension study. Eisai presented data from the extension study at the CTAD conference (Clinical Trials on Alzheimer's Disease) in San Diego in December 2019. Data showed that amyloid reduction in the brain that occurred as a

result of treatment with BAN2401 persisted after the conclusion of treatment. Differences in benefits on clinical outcomes were maintained after BAN2401 treatment in the two highest dose groups as compared with the placebo group.

BAN2401's unique binding profile has been confirmed in laboratory analyses, which are ongoing in parallel with the clinical development program. These results strengthen BioArctic's conviction that BAN2401's unique binding profile is important and distinguishes it from other amyloid beta antibodies. Professor Lars Lannfelt presented details on the binding profile at the AAIC® conference in July 2019 and at the CTAD conference in December 2019. In December 2019, BioArctic announced that the company had initiated a research collaboration with Eisai for deeper study of the unique binding profile of drug candidate BAN2401. Remuneration to BioArctic for implementing the research collaboration totals MEUR 3.25 (approximately MSEK 34) and is expected to be received over an 18-month period.

BAN2401 is one of the drug candidates that has been selected by the Alzheimer's Clinical Trials Consortium (ACTC) and Eisai to be evaluated in an upcoming clinical Phase 3 program focused on prevention of Alzheimer's disease (AHEAD 3-45). The planned clinical program will include individuals that are at risk for, or at a very early stages of, Alzheimer's disease. The program will be conducted with funding from various sources including the United States National Institute on Aging (NIA) and Eisai. According to ACTC and Eisai, the study will be starting during 2020.

Eisai is responsible for the clinical development in Alzheimer's disease and the project is based on research from Uppsala University, Sweden.

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

The antibody is a further developed version of BAN2401 for the treatment of Alzheimer's disease. The antibody was developed by BioArctic in collaboration with Eisai, which led to a new license agreement in 2015. The project is driven and financed by Eisai and is in preclinical phase.

## Drug candidates 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 than BAN2401 does, and each has the potential to be a disease-modifying treatment for Alzheimer's disease. All of them are being developed to treat early and mild Alzheimer's disease.

#### **PARKINSON'S DISEASE**

In the Parkinson's disease treatment area, BioArctic has been collaborating with AbbVie since 2016 when a research agreement was entered into. AbbVie was granted the option to license the right to develop and commercialize BioArctic's portfolio of antibodies against alpha-synuclein for Parkinson's disease and other potential indications. At the end of 2018, AbbVie exercised this option.

#### Drug candidate ABBV-0805 (collaboration with AbbVie)

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

AbbVie's option to license the portfolio was effective after clearance by the U.S. Antitrust legislation and triggered a milestone payment of MUSD 50 at the end of 2018. 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 already in March 2019 in healthy volunteers. In March 2020, the second portion of the study was initiated with patients with Parkinson's disease. AbbVie finances and progresses the 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.

## Drug candidates PD1601 and PD1602 (collaboration with AbbVie)

The antibodies projects PD1601 and PD1602 are targeting alpha-synuclein 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 conducted by BioArctic within the framework of the collaboration with AbbVie.

#### **OTHER CNS DISORDERS**

BioArctic targets 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 for other diseases in the central nervous system.

## Drug candidate BAN2401 (indications other than Alzheimer's disease, owned by BioArctic)

BAN2401, which is currently being clinically evaluated for Alzheimer's disease, can potentially also be used for other indications which are owned by BioArctic. The antibody BAN2401 is in the preclinical phase as a potential treatment of cognitive disorders in conjunction with Down's syndrome and traumatic brain injuries.

#### Drug candidate 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 new project is in an early research phase.

## BLOOD-BRAIN BARRIER TECHNOLOGY (owned 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 research groups 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 research grants from Sweden's Innovation Agency, Vinnova, for continued research in the blood-brain barrier project. The research, which is at an early stage, has shown highly encouraging results and the technology has significant potential in the treatment of several different diseases of the brain. During the year, BioArctic increased activities in this area and added further expertise.

#### **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 a project 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.

#### **OTHER**

# Product candidate SC0806 (traumatic complete spinal cord injury) (operations being phased out, owned by BioArctic)

In late 2019, it was decided to close the project for BioArctic's treatment concept SC0806, a biodegradable medical device with growth factor FGF1 that is surgically implanted into an injured spinal cord with the intent to restore function.

An interim analysis was conducted in the Phase 1/2 study with SC0806 for treatment of patients with complete spinal cord injuries. Unfortunately, the treatment did not result in the passage of any electrical impulses across the injured area, which is considered a prerequisite for patients to regain motor function. Based on the results, BioArctic decided to stop recruitment to the study and to not pursue the project further after the final patient has completed the study. This will not impact BioArctic's research and development of drugs for Alzheimer's, Parkinson's and other disorders of the central nervous system.

The Phase 1/2 study with SC0806 received partial financing from the EU Horizon 2020 research and development program (Grant Agreement No. 643853).

## 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 a point in time when performance obligations are fulfilled.

Net revenues in the first quarter amounted to MSEK 36.4 (63.4), which was a decrease of 27.0 MSEK. The decrease is attributable to the lower revenue from the Parkinson's program, which is according to plan. During the quarter, however, a lump sum of MSEK 22.8 in revenue was recorded, which is attributable to a remeasurement of the total costs of the Parkinson's program since performance was better than originally planned.

Other operating income relates to research grants, operating exchange rate gains and expenses incurred but onward invoiced. Other operating income amounted to MSEK 3.4 (6.9) for the first quarter.

Total operating expenses for the first quarter amounted to MSEK 36.0 (53.0) compared to the same period previous year. Project expenses for the first quarter decreased due to lower activity in the Parkinson's program as planned, offset by increased expenses for own projects. The personnel expenses for the first quarter increased compared with the same period the previous year. The increase is primarily attributable to an increase in the number of employees. Other operating expenses 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.

Operating profit before financial items (EBIT) amounted to MSEK 3.8 (17.3) for the first quarter. The decrease in operating profit year-on-year is due primarily to lower revenue from the Parkinson's program, which is according to plan.

Net financial items totaled MSEK 0.8 (0.1) 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 debt according to IFRS 16 Leases.

Profit (loss) for the period amounted to MSEK 3.6 (13.6) for the first quarter. Earnings per share before and after dilution amounted to SEK 0.04 (0.15) for the first quarter.



#### **FINANCIAL POSITION**

Equity amounted to MSEK 978.4 (1 031.4) as of March 31, 2020. This corresponds to equity per outstanding share of SEK 11.11 (11.71).

The equity/asset ratio has increased from 82.4 percent as of December 31, 2019 to 85.6 percent as of March 31, 2020. Compared with the first quarter last year, the equity/asset ratio increased from 78.5 percent to 85.6 percent.

The Group's cash and cash equivalents consist of bank balances that at the end of the period amounted to MSEK 1,077.3 (1,255.6). The leasing liabilities as of March 31, 2020 of MSEK 24.6 relate to financial leasing and is an effect from the application of IFRS 16 Leases as of January 1, 2019. There were no loans as of March 31, 2020 and no loans have been taken since this date. The Group has no other credit facility or loan commitments.

In order to reduce 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 -36.3 (333.6). The cash flow for the period from the preceding year included a milestone payment of MUSD 50 received from AbbVie.

Investments in the first quarter amounted to MSEK 0.3 (0.5). The investments are mainly related to laboratory equipment.

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

#### PARENT COMPANY

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

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

• The spread of COVID-19 has increased in intensity and scope, both in Sweden and around the world, in the first quarter of 2020. BioArctic is carefully monitoring the course of events in our business environment and is complying with guidelines from government authorities. At present it is difficult to assess, and too early to estimate, how the virus will impact BioArctic's operations over the long term. To date, BioArctic has not experienced any noteworthy disruptions to its operations owing to the COVID-19 pandemic.

# Cash and cash equivalents (MSEK) 1,256 1,218 1,170 1,113 1,077

Q2 Q3

2019

Ω1

2020

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

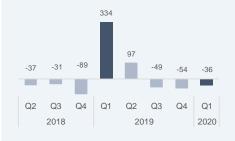
Ω1

02 03 04

2018

31 Mar	2020	2019
Non-current lease liabilities	18.2	25.4
Current lease liabilities	6.4	6.1
Cash and cash equivalents	1,077.3	1,255.6
Net cash position	1,052.7	1,224.1

## Cash flow from operating activities (MSEK)





## Other information

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

There are no key events to report after the period.

#### **PATENT**

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 period, BioArctic's patent portfolio consisted of 12 patent families with more than 150 granted patents and approximately 70 patent applications.

## COLLABORATIONS, PARTNERSHIPS AND MAJOR AGREEMENTS

Collaborating with universities is of great importance to BioArctic. The company has ongoing collaborations with academic research groups at a number of universities. Collaborations and license agreements with leading pharma and biopharma companies are also an important part of BioArctic's strategy. In addition to financial compensation we get access to our partners' skills in drug development, manufacturing and commercialization. BioArctic has entered into a number of such agreements with the Japanese international 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. BioArctic's status as a valued collaborating partner was recently confirmed in an article in Drug Discovery Today describing the collaboration between AbbVie and BioArctic. In the future BioArctic may enter into additional agreements that can contribute further funding and research and development competence for 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 BAN2401 and BAN2401 back-up antibodies. The total value of these agreements may amount to MEUR 221 in addition to royalties. To date, approximately MEUR 62 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. 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 2019 on pages 18, 25 and 40.

#### **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, 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 2019 on pages 46-49. The Board of Directors notes that to date, COVID-19 has not had any major impact on operations. The company routinely monitors the development of the pandemic and is working proactively to manage any risks over the longer-term.

#### **FLUCTUATIONS IN REVENUE GENERATION**

Currently, BioArctic does not have any drugs that have been commercialized and are being sold on the market. The company develops certain 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, diagnostic tools, as well as the bloodbrain 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 and are difficult to predict.

#### **FUTURE PROSPECTS**

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

stage projects and the continued positive development of BioArctic.

## EXPECTED DEVELOPMENT OF OPERATING EXPENSES

Operating expenses are expected to be in the range of MSEK 180 - 230 for the fiscal year January – December 2020. During 2019 operating expenses were MSEK 184.

#### **EMPLOYEES**

At the end of the period, the number of employees was 43 (36) of which 16 (13) are men and 27 (23) women. Approximately 85 percent are active 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 for tasks in competence areas that the company lacks or only has a need for periodically. As of March 31, 2020, these corresponded to 10 (11) full-time positions.

In 2020, BioArctic strengthens its management team with two strategic recruitments. As of January 1, Tomas Odergren assumed the role of Chief Medical Officer. The previous Chief Medical Officer Hans Basun has transitioned into the role of Senior Director Clinical Development. In May 2020 Oskar Bosson will assume the role of VP Communications & IR.

#### **ANNUAL GENERAL MEETING 2020**

The Annual General Meeting 2020 will be held on May 7, at 5:00 p.m. CET at Lindhagens' Conference Center in Stockholm, Sweden.

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, <a href="www.bioarctic.com">www.bioarctic.com</a> under the Governance tab.

#### 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, 20201

	Number		Share	of (%)
•	A-shares	B-shares	capital	votes,
Demban AB (Lars Lannfelt)	8,639,998	22,723,707	35.6	50.1
Ackelsta AB (Pär Gellerfors)	5,759,998	15,150,036	23.7	33.4
Fourth AP-Fund	-	4,000,000	4.5	1.8
Third AP-Fund	-	3,810,032	4.3	1.8
Unionen	-	2,562,723	2.9	1.2
Norron Funds	-	2,007,704	2.3	0.9
Investment AB Öresund	-	1,684,645	1.9	0.8
Handelsbanken Funds	-	1,540,000	1.7	0.7
Swedbank Robur Funds	-	1,446,328	1.6	0.7
Second AP-Fund	-	1,441,666	1.6	0.7
Tot. 10 largest shareholde	14,399,996	56,366,841	80.1	92.1
Other	-	17,293,148	19.9	7.9
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).

#### **EMPLOYEE WARRANT PROGRAM**

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. 485,000 employee warrants were allocated by the end of the quarter of which 5,000 were allocated in the quarter. There is no dilutive effect as of December 31, 2019, according to IAS 33.47, as the average market price for the period is lower than the exercise price. More information is available at www.bioarctic.com

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This information is information that BioArctic AB (publ) is obligated to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, though the agency of the named contact persons, at 08:00 a.m. CET on April 22, 2020.

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

Stockholm, Sweden, April 22, 2020

Cjale Cerd

Gunilla Osswald

Gunilla Osswald CEO, BioArctic AB (publ)

## INVITATION TO PRESENTATION OF INTERIM REPORT FOR THE PERIOD JANUARY – MARCH 2020

BioArctic invites to an audiocast with teleconference (in English) for investors, analysts and media today, April 22, at 09:30 – 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-q1-2020

To participate in the conference, please call: +46 8 505 583 59 (Sweden), +45 781 501 09 (Denmark), +31 107 129 163 (Netherlands), +47 235 002 36 (Norway), +41 225 675 632 (Switzerland), +44 333 300 9261 (UK), +49 692 222 0377 (Germany) or +1 833 526 8380 (USA)

#### **CALENDAR 2020/2021**

Annual General Meeting 2020 Interim report Jan-Jun 2020 Interim report Jan-Sep 2020 Full Year Report Jan-Dec 2020 May 7, 2020, at 5:00 p.m. CET July 10, 2020, at 8:00 a.m. CET October 14, 2020, at 8:00 a.m. CET February 4, 2021, at 8:00 a.m. CET



#### FOR FURTHER INFORMATION, PLEASE CONTACT

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## BioArctic AB (publ)

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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 should have precedence.

# Financial statements, Group

#### **CONSOLIDATED INCOME STATEMENT**

			Jan-Dec	
kSEK	2020	2019	2019	
Net revenues (note 4)	36,431	63,388	281,772	
Other operating income	3,385	6,931	14,826	
Operating revenues	39,816	70,319	296,598	
Operating expenses				
Project related expenses	-10,486	-29,938	-72,422	
Other external expenses	-6,755	-7,973	-31,169	
Personnel expenses	-15,967	-12,018	-59,715	
Depreciations of tangible assets	-2,522	-2,371	-9,199	
Other operating expenses	-291	-684	-11,554	
Operating profit/loss	3,794	17,335	112,538	
Financial income	1,097	330	1,630	
Financial expenses	-289	-257	-1,192	
Profit/loss before tax	4,602	17,408	112,976	
Tax	-1,048	-3,778	-24,507	
Profit/loss for the period	3,554	13,629	88,468	
Earnings per share				
Earnings per share before dilution, SEK	0.04	0.15	1.00	
Earnings per share after dilution, SEK¹	0.04	0.15	1.00	

<sup>&</sup>lt;sup>1</sup> There is no dilutive effect as the average market price for the period is lower than the exercise price as of March 31, 2020.

#### CONSOLIDATED STATEMENT OF COMPREHENSIVE INCOME

		Q1	
kSEK	2020	2019	2019
Profit/loss for the period	3,554	13,629	88,468
Other comprehensive income	-	-	-
Comprehensive income for the period	3,554	13,629	88,468

#### **CONSOLIDATED BALANCE SHEET**

kSEK	31 Mar 2020	31 Mar 2019	31 dec 2019
ASSETS			
Tangible fixed assets	9,046	9,148	9,590
Right-to-use assets	25,792	31,669	27,544
Deferred tax assets	337	277	298
Other financial assets	1,511	1,500	1,511
Current assets excluding cash and cash equivalents	28,612	16,274	31,619
Cash and cash equivalents	1,077,255	1,255,567	1,112,770
TOTAL ASSETS	1,142,552	1,314,435	1,183,332
EQUITY AND LIABILITIES			
Equity	978,366	1,031,365	974,497
Deferred tax liabilities	38,685	32,520	38,685
Non-current lease liabilities	18,200	25,357	20,927
Current lease liabilities	6,380	6,149	6,439
Other current liabilities	11,574	20,398	24,030
Accrued expenses and deferred income	89,347	198,645	118,753
EQUITY AND LIABILITIES	1,142,552	1,314,435	1,183,332

#### **CONSOLIDATED STATEMENT OF CHANGE IN EQUITY (CONDENSED)**

	31 Mar 2020	31 Mar 2019	31 dec 2019
Opening balance at 1 January	974,497	1,017,736	1,017,736
Comprehensive income for the period	3,554	13,629	88,468
Share-based payments	315	-	383
Paid dividend	-	-	-132,090
Closing balance	978,366	1,031,365	974,497

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

	Q	Q1		
kSEK	2020	2019	2019	
Operating profit	3,794	17,335	112,538	
Adjustment for non-cash items	-27,887	-56,280	-107,485	
Interest received/paid	-289	-47	-757	
Income tax paid	-11,116	-74,717	-80,919	
Cash flow from operating activities before changes in working capital	-35,498	-113,710	-76,622	
Change in working capital	-852	447,338	403,787	
Cash flow from operating activities after changes in working capital	-36,350	333,629	327,165	
Cash flow from investing activities	-257	-563	-3,273	
Cash flow from financing activities	-2,755	-1,829	-138,506	
Cash flow for the period	-39,361	331,236	185,385	
Cash and cash equivalents at beginning of period	1,112,770	917,307	917,307	
Exchange rate differences in cash and cash equivalents	3,846	7,024	10,077	
Cash and cash equivalents at end of period	1,077,255	1,255,567	1,112,770	

#### **CONSOLIDATED QUARTERLY DATA**

	2020	2019	2019	2019	2019	2018	2018	2018
MSEK	Q1	Q4	Q3	Q2	Q1	Q4	Q3	Q2
Income statement								
Net revenues	36.4	26.4	20.6	171.3	63.4	515.3	94.0	52.3
Other operating income	3.4	0.0	8.6	-0.7	6.9	0.7	0.6	3.6
Operating expenses	-36.0	-47.5	-39.7	-43.8	-53.0	-85.7	-61.5	-49.4
Operating profit/loss	3.8	-21.1	-10.5	126.8	17.3	430.3	33.1	6.4
Operating margin, %	10.4	-79.8	-50.9	74.0	27.3	83.5	35.2	12.3
Profit/loss for the period	3.6	-17.1	-8.3	100.3	13.6	335.2	25.9	5.1
Balance sheet								
Fixed assets	36.7	38.9	40.2	41.0	42.6	11.0	9.9	10.0
Current assets	28.6	31.6	29.2	15.9	16.3	464.8	13.8	12.0
Cash and cash equivalents	1,077.3	1,112.8	1,170.2	1,218.4	1,255.6	917.3	1,008.5	1,041.7
Equity	978.4	974.5	991.3	999.5	1,031.4	1,017.7	682.5	656.7
Deferred tax liabilities	38.7	38.7	32.5	32.5	32.5	32.5	5.5	5.5
Lease liabilities	24.6	27.4	28.5	30.0	31.5	-	-	_
Current liabilities	100.9	142.8	187.3	213.2	219.0	342.8	344.2	401.6
Cash flow								
From operating activities	-36.3	-54.2	-49.4	97.2	333.6	-89.3	-31.5	-37.3
From investing activities	-0.3	-0.4	-1.6	-0.7	-0.6	-1.7	-0.5	-0.7
From financing activities	-2.8	-1.5	-1.5	-133.6	-1.8	-	-	_
Cash flow for the period	-39.4	-56.2	-52.5	-37.1	331.2	-91.0	-32.0	-38.0
Data per share								
Earnings per share before dilution, SEK	0.04	-0.19	-0.09	1.14	0.15	3.81	0.29	0.06
Earnings per share after dilution, SEK <sup>1</sup>	0.04	-0.19	-0.09	1.14	0.15	3.81	0.29	0.06
Equity per share, SEK	11.11	11.07	11.26	11.35	11.71	11.56	7.75	7.46
Cash flow operating activities per share, SEK	-0.41	-0.62	-0.56	1.10	3.79	-1.01	-0.36	-0.42
Share price at the end of the period, SEK	61.50	94.90	61.75	74.40	78.00	82.00	118.90	21.80
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 <sup>1</sup>	88,060	88,060	88,060	88,060	88,060	88,060	88,060	88,060

<sup>&</sup>lt;sup>1</sup> There is no dilutive effect as the average market price for the period is lower than the exercise price as of March 31, 2020.

# Financial statements, Parent company

#### PARENT COMPANY INCOME STATEMENT

		1	Jan-Dec	
kSEK	2020	2019	2019	
Net revenues	36,431	51,957	281,772	
Other operating income	3,385	18,362	14,826	
Operating revenues	39,816	70,319	296,598	
Operating expenses				
Project related expenses	-10,486	-29,938	-72,422	
Other external expenses	-8,632	-9,504	-38,265	
Personnel expenses	-15,967	-12,018	-59,715	
Depreciations of tangible assets	-802	-704	-2,961	
Other operating expenses	-291	-684	-11,554	
Operating profit/loss	3,638	17,471	111,681	
Financial income	1,097	330	1,630	
Financial expenses	-27	-47	-110	
Profit/loss after financial items	4,709	17,753	113,200	
Change in tax allocation reserves	-	-	-28,857	
Profit/loss before tax	4,709	17,753	84,344	
Tax	-1,070	-3,852	-18,390	
Profit/loss for the period	3,638	13,901	65,954	

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 2020	31 Mar 2019	31 dec 2019
ASSETS			
Tangible fixed assets	9,046	9,148	9,590
Deferred tax assets	267	203	250
Other financial assets	1,611	1,600	1,611
Current assets excluding cash and cash equivalents	29,945	16,274	31,619
Cash and cash equivalents	1,077,159	1,255,469	1,112,672
TOTAL ASSETS	1,118,027	1,282,694	1,155,742
EQUITY AND LIABILITIES			
Equity	840,641	916,342	836,687
Tax allocation reserve	176,674	147,817	176,674
Other current liabilities	11,365	20,100	23,810
Accrued expenses and deferred income	89,347	198,435	118,571
EQUITY AND LIABILITIES	1,118,027	1,282,694	1,155,742

### **Notes**

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

This Interim Report for the period January – March 2020 covers the Swedish Parent Company BioArctic AB, Swedish Corporate Identity Number 556601-2679, and the two fully owned subsidiaries SpineMedical AB, Swedish Corporate Identity Number 559003-7080, and 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.

The BioArctic Group's Interim Report for the period January – March 2020 was approved by the Company's Board of Directors Board on April 21, 2020.

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

The consolidated financial statements for BioArctic AB have been prepared in accordance with IFRS (International Financial Reporting Standards) as adopted by the EU, the Swedish 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 2020 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 the Annual Report 2019. New and amended IFRS standards and interpretations applied from 2020 are deemed not to have 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	2020 2	2019	2019
Geographic breakdown of net revnues			
Europe	27,975	63,388	119,796
Asia	8,456	-	161,976
Total net revenues	36,431	63,388	281,772
Net revenues per revenue type			
Milestone payments	-	11,431	173,407
Income from research collaborations	36,431	51,957	108,366
Total net revenues	36,431	63,388	281,772

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 (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. As of March 31, 2020, MSEK 628.9 has been recognized as revenue and the remaining amount to be recognized as a revenue up until the completion of the project is MSEK 72.

## 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 period's cash flow from operating activities 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

#### **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.

#### Alfa-synuclein (α-synuclein)

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

#### Amyloid beta (Aß)

A naturally occurring protein in the brain that, in conjunction with Alzheimer's disease, misfolds into harmful structures in brain cells. They 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.

#### **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.

#### **Complete Spinal Cord Injury**

A complete injury means that the spinal cord is complete severed. In an incomplete injury there are still a few nerve contacts left.

#### 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.

#### Interim analysis

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

#### Milestone payment

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

#### Monoclonal antibody

An antibody that can be produced so that all copies are exactly alike.

#### Monomer

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

#### **Neurodegenerative disorders**

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

#### Oligomer

Molecules consisting of a number of monomers.

#### **PET**

Positron emission tomography, an investigation imaging method

#### Phase 1 studies

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

#### Phase 2 studies

Studies of the safety and efficacy of a drug and dose finding. Performed on a limited number of patients.

#### Phase 3 studies

Confirmatory studies of the safety and efficacy of a drug in a clinical setting. Performed on a large number of patients.

#### **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.

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#### **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 that gives rise to Parkinson's disease.

#### Research phase

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

#### **Tolerability**

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

