

BIOARCTIC AB (PUBL)  
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# Interim Report January-March 2019

## Stockholm, May 9, 2019

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*Gunilla Osswald, PhD, CEO*

*Jan Mattsson, CFO*

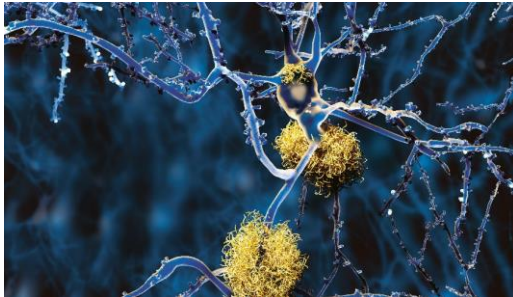


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# Great progress in the projects 2018 & 2019 year to date

## ALZHEIMER'S DISEASE



### BAN2401

- Phase 2b study demonstrated positive results in 856 patients with early Alzheimer's disease
- One Phase 3 confirmatory study in early Alzheimer's disease initiated by Eisai
- Presented at international congresses - AAIC, CTAD, AD/PD

### Discovery stage programs

- Progressed according to plan

## PARKINSON'S DISEASE



### ABBV-0805 (BAN0805)

- AbbVie licensed the portfolio and 50 MUSD milestone received
- IND produced by BioArctic approved by FDA
- Phase 1 study started by AbbVie

### Discovery stage projects

- Progressed according to plan

## COMPLETE SPINAL CORD INJURY



### SC0806

- Phase 1 safety evaluated and Phase 2 started

## DIAGNOSTICS AND TECHNOLOGY



### Blood Brain Barrier Technology

- Vinnova grant of 10 MSEK received together with Uppsala University (April)

# BioArctic – a unique Swedish biopharma company

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A **scientific breakthrough by our founder** enables development of disease modifying treatments for disorders in the central nervous system



**Successful strategic collaborations** with global pharmaceutical companies with a total **value of the agreements of 9.3 BSEK (ca 1 BUSD) plus royalties**



A **confirmatory Phase 3 trial initiated for BAN2401** – our most advanced drug candidate – a promising game changing treatment for early Alzheimer´s disease



**Positive financial results** during the last six years and **more than 1 BSEK in cash**





A **broad project portfolio** and a well-functioning research and development organization and fruitful collaborations with leading academic researchers generating **new projects with substantial marketing and out-licensing potential**

# Portfolio combines fully financed partner projects and cutting-edge proprietary projects

## PROJECT PORTFOLIO AS OF MARCH 31, 2019

Attractive combination of fully financed partner projects and cutting-edge, proprietary R&D pipeline with substantial market and out-licensing potential



	Product candidate	Indication	Partner	Discovery	Preclinical	Phase 1	Phase 2	Phase 3	
Neurodegenerative Diseases	<b>BAN2401:</b> anti-A $\beta$ antibody	Alzheimer's Disease		→					
	<b>BAN2401:</b> anti-A $\beta$ antibody	Down's Syndrome <sup>2</sup> Traumatic Brain Injury <sup>2</sup>	—	→					
	<b>BAN2401 BACK-UP:</b> anti-A $\beta$ antibody	Alzheimer's Disease		→					
	<b>AD1801:</b> Undisclosed information	Alzheimer's Disease	—	→					
	<b>AD1502:</b> Undisclosed information	Alzheimer's Disease	—	→					
	<b>AD1503:</b> Undisclosed information	Alzheimer's Disease	—	→					
	<b>ABBV-0805<sup>3</sup>:</b> anti- $\alpha$ -synuclein antibody	Parkinson's Disease	abbvie	→					
	<b>PD1601:</b> anti- $\alpha$ -synuclein antibody	Parkinson's Disease	abbvie	→					
	<b>PD1602:</b> anti- $\alpha$ -synuclein antibody	Parkinson's Disease	abbvie	→					
Diagnostics & Technology	<b>IMAGING AND BIOCHEMICAL BIOMARKERS:</b> A $\beta$	Alzheimer's Disease	—	→					
	<b>IMAGING AND BIOCHEMICAL BIOMARKERS:</b> $\alpha$ -synuclein	Parkinson's Disease	abbvie	→					
	<b>BBB-TECHNOLOGY:</b> blood-brain barrier	Multiple application areas	—	→					
Spine	<b>SC0806:</b> FGF1/medical device	Complete Spinal Cord Injury	—	→					

- 1) Partner with Eisai on BAN2401 for treatment of Alzheimer's disease. Eisai partnered with Biogen on BAN2401 in 2014
- 2) Dementia and cognitive impairment associated with Down's syndrome and Traumatic Brain Injury
- 3) AbbVie in-licensed BAN0805 in late 2018 and will continue to develop BAN0805, now with the designation ABBV-0805






# Long-standing and extensive partnerships

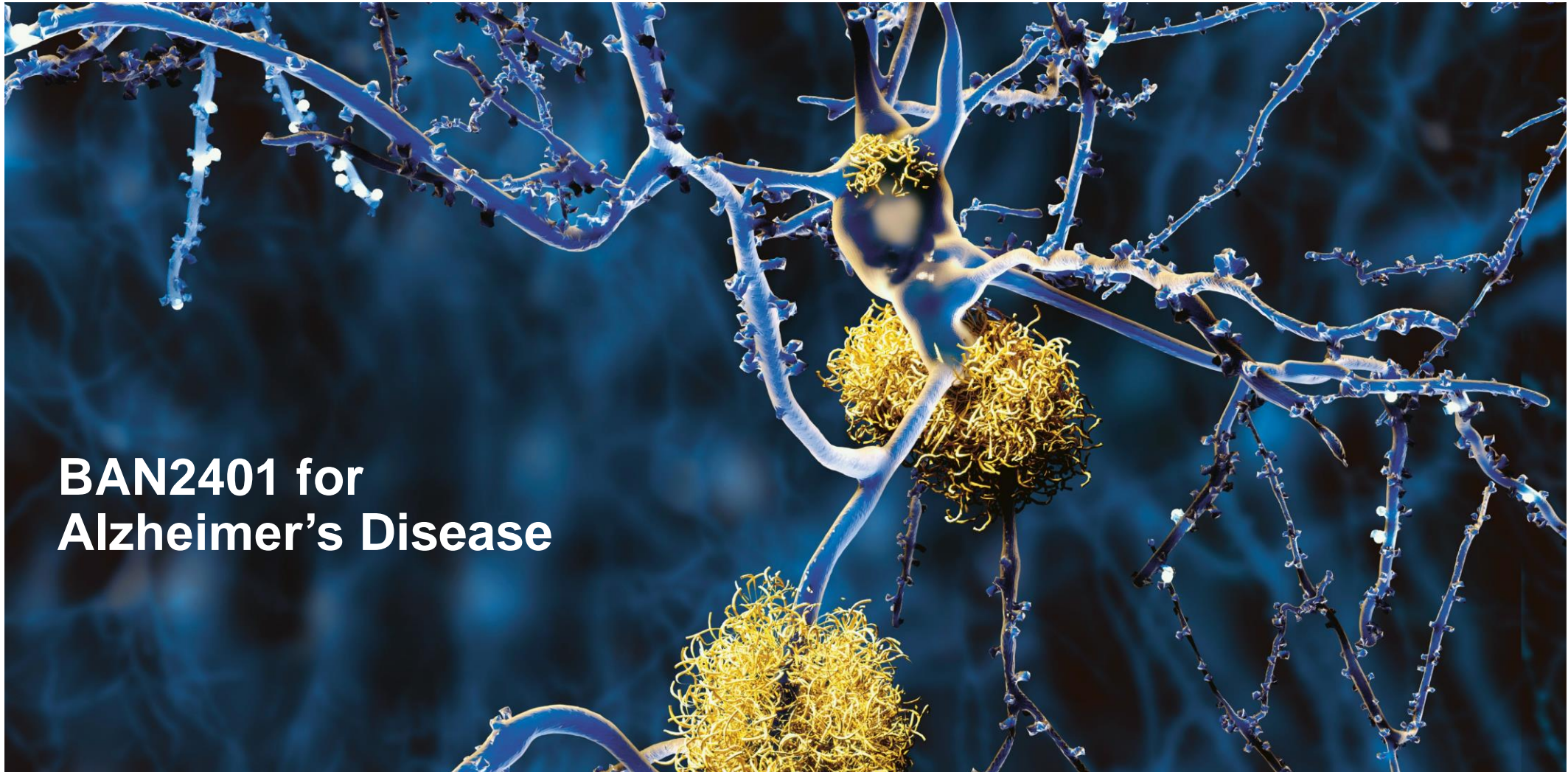
## Alzheimer's disease collaboration and license

Partner Track Record	Valuable Collaborations
 <p>Discovered and developed world's best selling medicine for symptoms in Alzheimer's</p>  <p>For confusion related to Alzheimer's</p> <p>10+ projects in dementia Currently in development</p> <p>High level of commitment to BAN2401</p>	<ul style="list-style-type: none"> <li>3 research collaborations and 2 licenses</li> <li>Abeta oligomer/protofibril antibodies BAN2401 and BAN2401 back-up as disease modifying treatments for Alzheimer's disease</li> </ul> <div style="display: flex; justify-content: space-around; align-items: center;"> <div data-bbox="644 768 963 1086"> <p>MEUR 218 Total value agreements</p> </div> <div data-bbox="950 768 1268 1232"> <p>MEUR 47 received</p> <p>Royalties High single digit %</p> </div> </div>

## Parkinson's disease collaboration and license

Partner Track Record	Valuable Collaborations
 <p>World's all time best selling medicine (BUSD 20)</p>  <p>10 different indications in immunology</p> <p>Approved product for symptoms associated with Parkinson's disease</p> 	<ul style="list-style-type: none"> <li>Research collaboration and license</li> <li>Alpha-synuclein antibodies as disease modifying therapies for Parkinson's (ABBV-0805, follow-up antibodies and diagnostics)</li> </ul> <div style="display: flex; justify-content: space-around; align-items: center;"> <div data-bbox="1842 758 2173 1086"> <p>MUSD 130 received</p> <p>MUSD 755 Total value milestones</p> </div> <div data-bbox="2147 908 2466 1232"> <p>Royalties Tiered %</p> </div> </div>

Sources: Eisai, AbbVie and BioArctic corporate information



# BAN2401 for Alzheimer's Disease

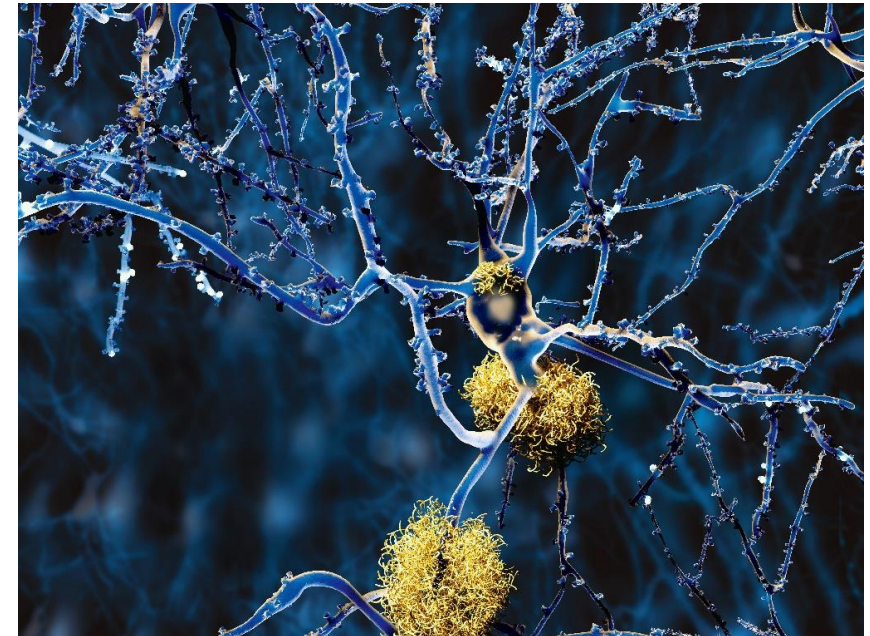


# Significant opportunity in Alzheimer's disease

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- 30 million people worldwide suffer from Alzheimer's disease today and the number is expected to double in 20 years
- High unmet medical need
- Patients treated at earlier stages of the disease
- Huge market with demand for several products and expected to be used in combination
- Alzheimer's field has lately suffered from drawback of several programs, BAN2401 is a unique antibody with the strongest clinical results now in Phase 3

## NEURONS WITH AMYLOID PLAQUES IN ALZHEIMER'S DISEASE





# BAN2401 is a unique antibody with robust clinical results

## UNIQUE ANTIBODY PROFILE

### Unique and targeted binding profile

- Highly selective for protofibrils of Abeta vs monomers and fibrils
- Protofibrils are the most toxic form of Abeta

### Unique clinical fingerprint

- Rapid onset of clinical effect
- Consistent effects on neurodegenerative biomarkers
- Low frequency of ARIA–E
- No titration required

## ROBUST CLINICAL RESULTS DRIVING PHASE 3

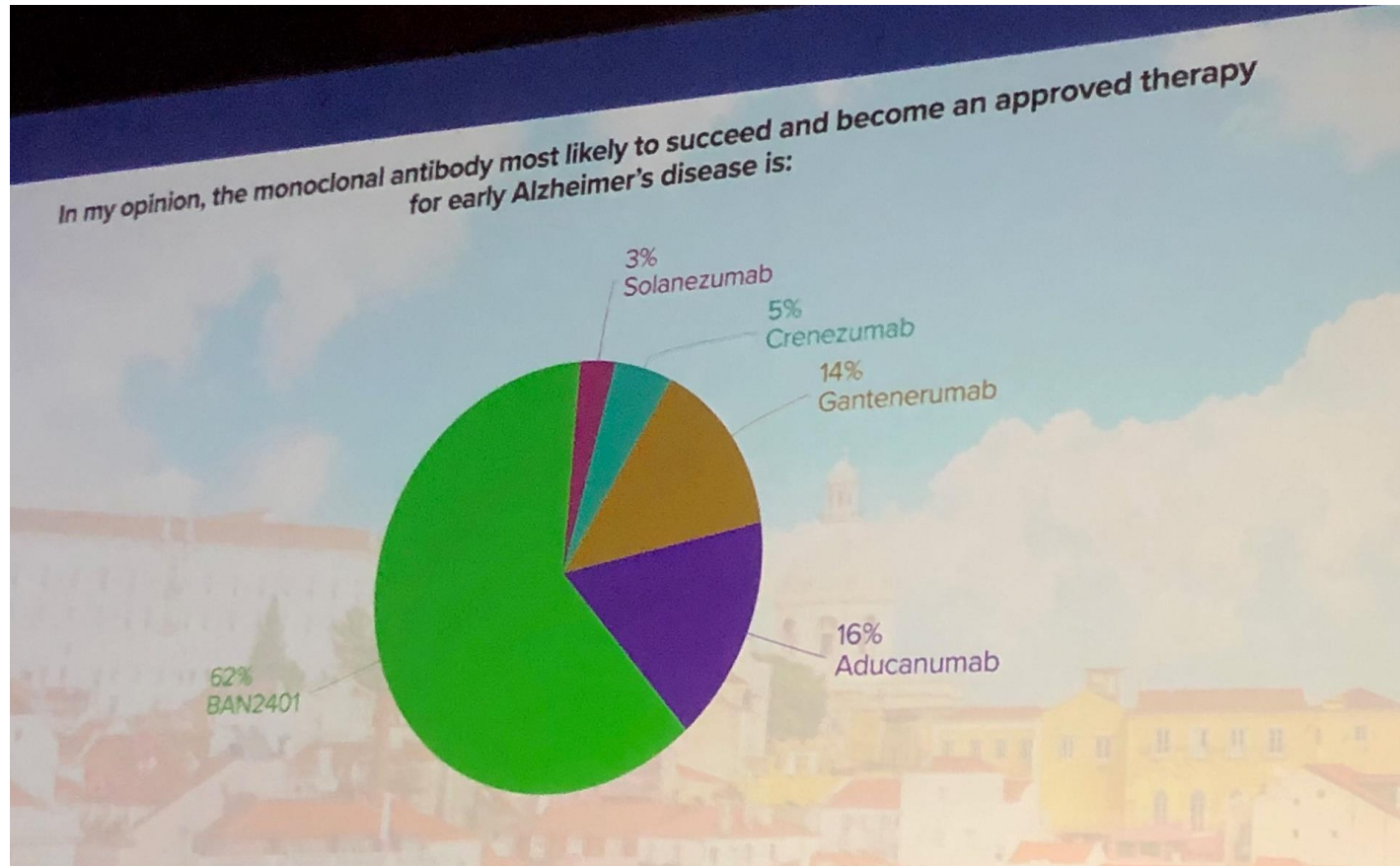
### Robust results in a large Phase 2b study

- 856 early Alzheimer’s disease patients
- Consistent effects on clinical endpoints, amyloid clearance and neurodegenerative biomarkers
- Most patients randomized to two top doses due to early effect – top dose selected as best dose
- Effect increase over time

### Single Phase 3 study (“Clarity AD”) designed to confirm the positive effects in Phase 2b

- 1566 subjects with early Alzheimer’s disease and confirmed amyloid pathology
- Primary and secondary endpoints including clinical outcomes, safety and imaging and other biomarkers
- Top dose from Phase 2b (10 mg/kg) or placebo twice a month

# At AD/PD in March 2019 BAN2401 was seen as the most promising monoclonal drug candidate as disease modifying treatment in Alzheimer's disease



Votes by an audience of approx. 1500 Key Opinion Leaders, researchers and physicians at AD/PD March 30, 2019

## BAN2401 – upcoming events

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- Eisai has initiated the confirmatory Phase 3 study in early Alzheimer’s disease patients
  - BioArctic to receive a milestone at start of Phase 3
  - Results expected as early as 2022
- Results from the open-label extension study with BAN2401, without placebo, for patients from the Phase 2b study
- Eisai is exploring the potential for a clinical study at even earlier (preclinical) stages of Alzheimer’s disease





**ABBV-0805 (BAN0805) for  
Parkinson's Disease**



# Significant opportunity in Parkinson's disease

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- Parkinson's disease is the second most common neurodegenerative disease
- In 2015, it was estimated that 6.2 million people suffered from Parkinson's disease worldwide
- Compared with Alzheimer's, Parkinson's disease affects a younger patient group, still at working age
- High unmet medical need
- There is currently no disease modifying treatment for Parkinson's

## NETWORK OF NEURONS IN PARKINSON'S DISEASE



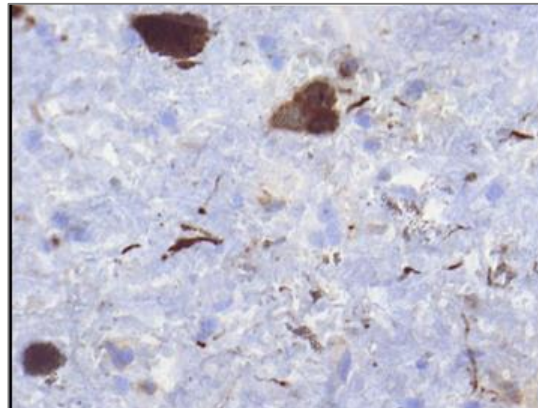
# BAN0805 – Unique potential disease modifying antibody for Parkinson’s disease with strong preclinical results

## RATIONALE FOR TARGETING ALPHA-SYNUCLEIN

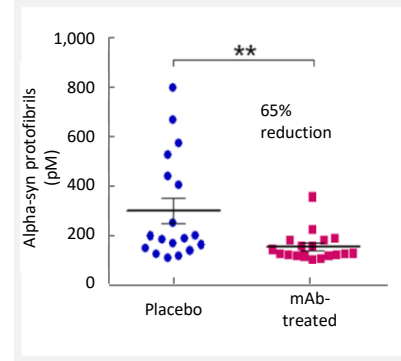
Human genetics

Pathology

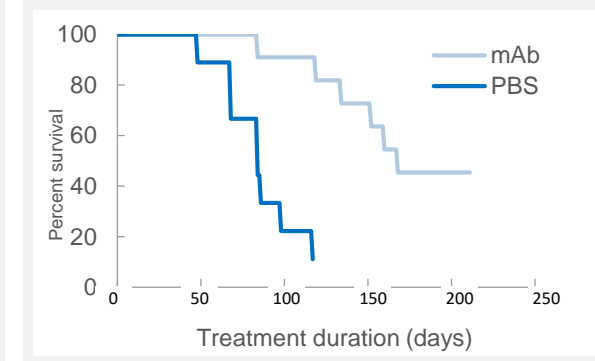
Pre-clinical proof of concept



Reduction of neurotoxic alpha-synuclein oligomers/protofibrils



Increases lifespan



**Alpha-synuclein mutations** lead to Parkinson’s disease or Dementia with Lewy Bodies and are associated with increased oligomer/ protofibril formation

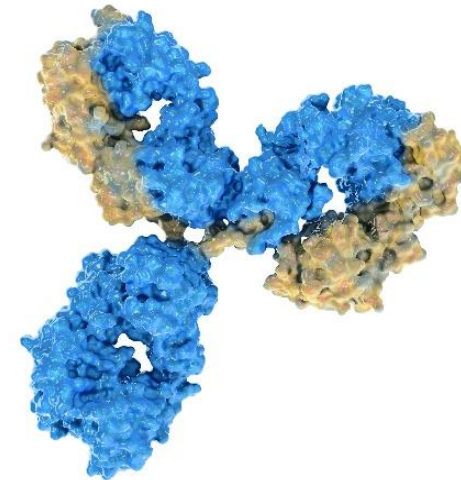
**Alpha-synuclein deposition** is a hallmark of Parkinson’s disease pathophysiology and alpha-synuclein oligomers/protofibrils are elevated in Parkinson’s disease

**Oligomer/protofibril selective antibody** reduces neurotoxic alpha-synuclein oligomer/protofibril levels, delays disease progression and increases lifespan in a mouse model of Parkinson’s disease

# Updates on BAN0805/ABBV-0805 and follow-up antibodies

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- Alpha-synuclein antibody portfolio licensed by AbbVie December 14, 2018
- Received a milestone payment of 50 MUSD for the license
- BAN0805/ABBV-0805 IND-application was approved by FDA in February 2019
- AbbVie started Phase 1 with ABBV-0805 in March 2019
- AbbVie is responsible for the clinical development in Parkinson´s disease
- BioArctic will deliver follow-up antibodies in the continued collaboration with AbbVie





**SC0806 for Complete  
Spinal Cord Injury**



# High unmet patient need in Complete Spinal Cord Injury

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- Patients suffer from other serious symptoms, incl. neuropathic pain, bowel and bladder incontinence, sensory loss, pressure sores, infertility and sexual dysfunction
- 2.5 million people live with paralysis, 40% complete spinal cord injury
- More common among younger men, injured in accidents
- No treatment available for these patients
- Orphan drug designation in the US and EU for SC0806

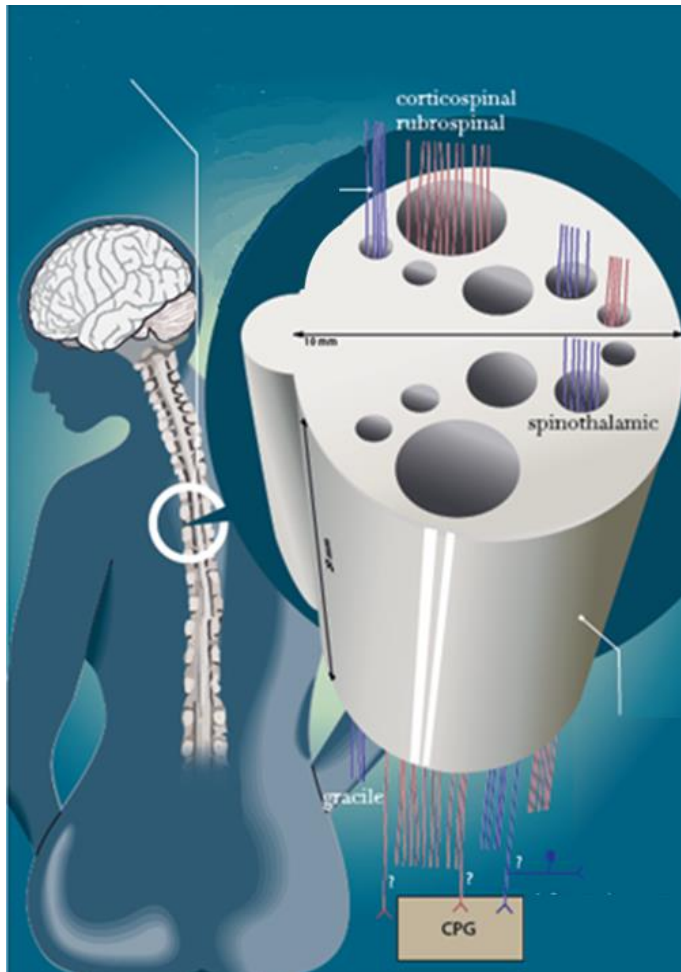


The patients require life-long treatment and care, which means high costs for healthcare systems and societies

# SC0806 – Unique regenerative treatment of Complete Spinal Cord Injury

## SC0806 – Regenerative Treatment of CSCI

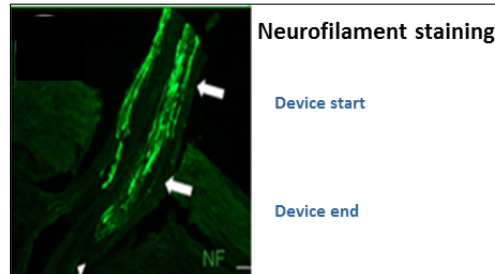
## Treatment Rationale



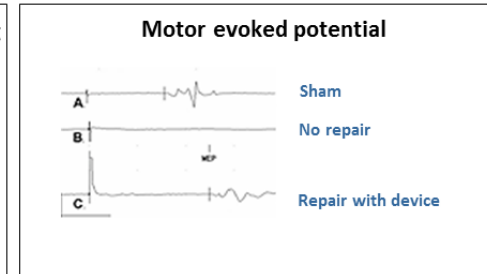
### SC0806 makes nerve regeneration possible

FGF1 activated by heparin	<ul style="list-style-type: none"> <li>Stimulation of central axon outgrowth</li> <li>Decreases gliosis</li> </ul>
Peripheral nerve autografts	<ul style="list-style-type: none"> <li>Optimal regeneration environment</li> </ul>
Biodegradable device	<ul style="list-style-type: none"> <li>Provides sustained release of FGF1</li> <li>Positioning of nerve grafts from white to gray matter</li> </ul>

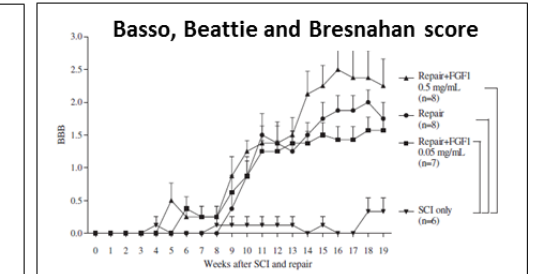
#### Nerve regeneration



#### Electrophysiology restored



#### Motor function improved



### Preclinical Proof of Concept shown in rats with resected spinal cords

- Rat experiments demonstrate nerve regeneration, restored electrophysiology and motor function after SC0806 treatment

# SC0806 – Phase 2 ongoing with interim analysis expected 1H 2020

## PROJECT STATUS

- Clinical Phase 1/2 trial ongoing in patients with Complete Spinal Cord Injury
  - Surgery at Karolinska University Hospital, Stockholm, Sweden
  - Rehabilitation 18 months with Lokomat™ in Sweden, Estonia, Finland and Norway
  - 9 patients included in Panel A (6 treated with SC0806 and 3 control patients)
  - Safety evaluation of patients in Panel A performed and support progression into Panel B i.e. Phase 2
  - First patient included in Phase 2 during Q1 2019
- Orphan Drug designation in US and EU – may grant 7 and 10 years exclusivity, respectively
- EU Horizon 2020 research and innovative program Grant Agreement No. 643853 of MEUR 6.4

## THE LOKOMAT™ USED IN THE REHABILITATION





## Diagnostics and Technology Platforms



# Advancing diagnostics and technology platforms to fuel pipeline

## ANTIBODY BASED IMAGING (PET) IN ALZHEIMER'S PATIENTS

Research collaboration with Uppsala University to develop new technologies to image the brain

## ALZHEIMER'S DISEASE DIAGNOSTICS

Research collaboration with Brain Biomarker Solutions in Gothenburg AB

## PARKINSON'S DISEASE DIAGNOSTICS

Research for better diagnostic tools and biomarkers for Parkinson's disease (participation in a European research consortium with a grant from the EU's Horizon 2020)

## BLOOD BRAIN BARRIER TECHNOLOGY

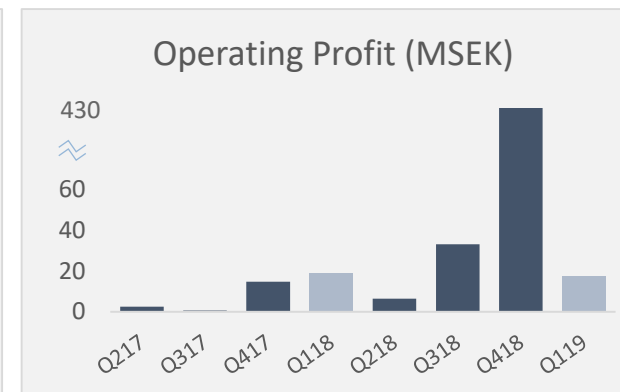
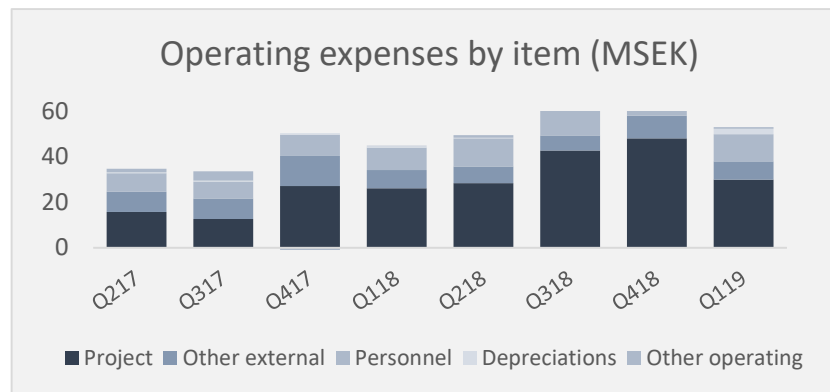
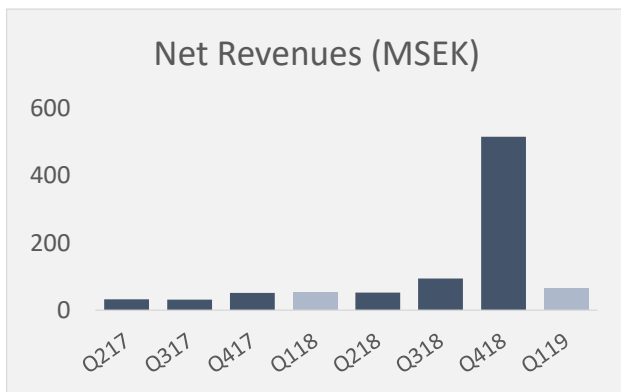
Development of multi-specific antibodies with a transporter to facilitate passage across the blood brain barrier (in collaboration with Uppsala University with a grant from Sweden's Innovation Agency, Vinnova)





## Financial summary

# Revenues and operating profit Q1 2019



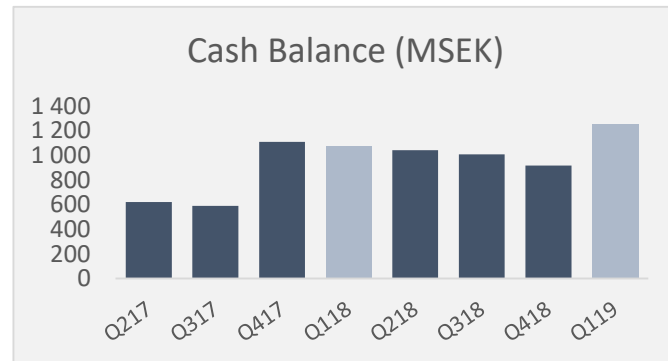
- Net revenues increased to 63.4 MSEK (52.3)
- Mainly related to the AbbVie research collaboration regarding the Parkinson's project

- About 85% of the costs are related to R&D
- Project expenses increased slightly to 29.9 MSEK (26.1)
- Other operating expenses were 22.4 MSEK (18.5), which increase is related to BioArctic being a larger organization

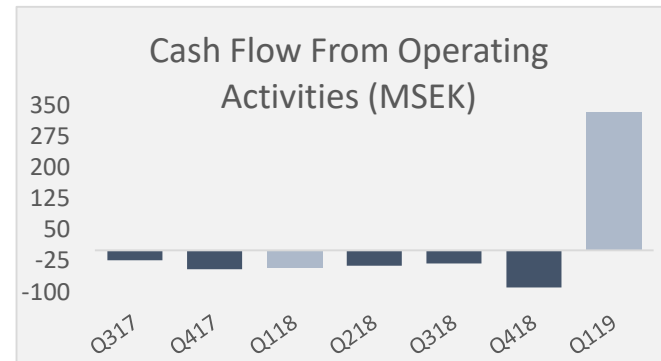
- Operating profit decreased to 17.3 MSEK (18.9)

Operating expenses are expected to be in the range of 190 - 250 MSEK for the fiscal year January - December 2019

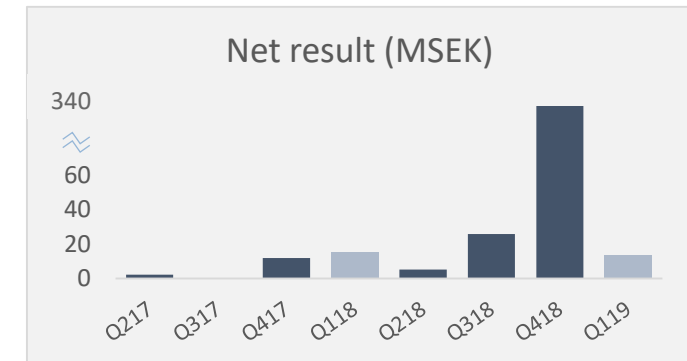
# Cash related and net profit Q1 2019



- Cash balance amounted to 1,255.6 MSEK (1,078.7) at the end of the quarter



- Operating cash flow amounted to 333.6 MSEK (-42.0) during Q1
- The reason for the positive number in Q1 is related to the MUSD 50 payment from AbbVie for the in-licensing of ABBV-0805



- Profit for the period decreased to 13.6 MSEK (15.4) during Q1

To sum up, BioArctic showed another quarter with positive net results and a strong cash balance





**Upcoming news and  
closing remarks**

# Upcoming news flow

ALZHEIMER'S DISEASE	PARKINSON'S DISEASE	COMPLETE SPINAL CORD INJURY	DIAGNOSTICS AND TECHNOLOGY
			
<p><b>BAN2401</b></p> <ul style="list-style-type: none"><li>• Phase 3 confirmatory study in early Alzheimer's disease start by Eisai and milestone payment</li><li>• International congresses incl. AAIC in July</li><li>• Phase 2b open label extension study results</li><li>• Clinical study in earlier stages of Alzheimer's disease decision</li></ul>	<p><b>ABBV-0805 (BAN0805)</b></p> <ul style="list-style-type: none"><li>• Phase 1 study completion by AbbVie</li></ul> <p><b>Discovery stage projects</b></p> <ul style="list-style-type: none"><li>• Advancement into preclinical stage</li></ul>	<p><b>SC0806</b></p> <ul style="list-style-type: none"><li>• Interim analyses of safety and efficacy of the first part of the Phase1/2 study</li></ul>	<p><b>Blood Brain Barrier Technology</b></p> <ul style="list-style-type: none"><li>• Expansion and continued development of the Blood Brain Barrier Technology platform in collaboration with Uppsala University</li></ul>
<p><b>Discovery stage programs</b></p> <ul style="list-style-type: none"><li>• Advancement into preclinical stage</li></ul>			

**GUNILLA OSSWALD, CEO**



**JAN MATTSSON, CFO**



**NEXT REPORT & IR CONTACT**

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