



BioArctic AB

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Biotech & Money – Investival Showcase

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# Helping Patients with Disorders in the Central Nervous System by Developing Innovative Treatments

## Alzheimer's Disease



Three key areas with high unmet medical needs – all lacking effective treatments today

Disease modifying treatment in Alzheimer's and Parkinson's Disease – areas with huge and growing markets due to aging populations

BAN2401 Phase 2b study in early AD in collaboration with Eisai – first late stage study demonstrating potential disease modifying effect on both cognition and biomarkers

## Parkinson's Disease



BAN0805 for PD in collaboration with AbbVie – preparing for clinical development and IND in the U.S.

SC0806 a unique regenerative treatment for patients with Complete Spinal Cord Injuries in Phase 1/2

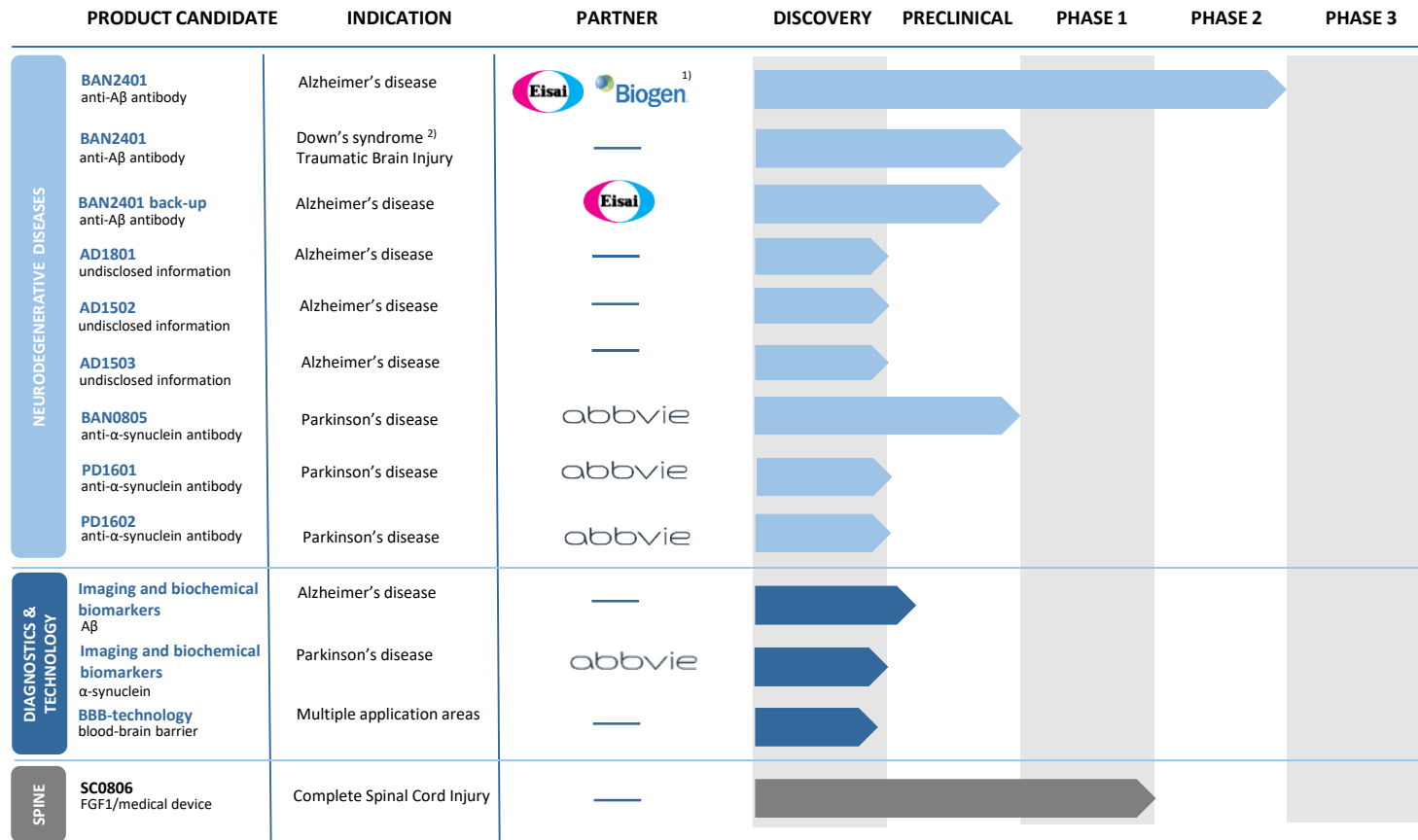
## Complete Spinal Cord Injury



Attractive combination of fully financed partner projects and innovative pipeline with substantial market and out-licensing potential

Strong science based research and highly educated engaged teams with vast experience in drug development and great track record of high quality deliverables

# Strategic Partnerships and Cutting-Edge Proprietary R&D per September 30, 2018



<sup>1)</sup> Partner with Eisai on BAN2401 for treatment of AD. Since 2014, Eisai partnered with Biogen in AD

<sup>2)</sup> Dementia and cognitive impairment associated with Down's syndrome and Traumatic Brain Injury

Source: company data

# Long-standing and Extensive Partnerships

## Eisai collaboration and license agreements Alzheimer's Disease



### Description of agreements

- Two research collaborations – disease modifying therapies for AD – resulted in two licenses for A $\beta$  oligomer/protofibril antibodies: BAN2401 and BAN2401 Back-up
- Third research collaboration – new target as a disease modifying therapy for AD

### Milestone / royalty potential

- Total aggregated value of the research collaborations and license agreements is approx. EUR 218m in signing fee and milestones, plus high single digit royalties
- BioArctic has received approx. EUR 47m for the research collaborations, signing fees and milestones

## AbbVie collaboration agreement Parkinson's Disease



### Description of agreements

- Research collaboration – alpha-synuclein antibodies as disease modifying therapies for PD incl. BAN0805 to IND, follow-up compounds and diagnostic
- Option for AbbVie for a license to develop and commercialize the antibodies

### Milestone / royalty potential

- Total potential value of the agreement is up to USD 755m incl. an up-front fee, option exercise fee, and success-based milestones plus tiered royalties
- BioArctic has received an USD 80m up-front payment for the research collaboration
- Payment of USD 50m to be received when exercising option to license, pending US antitrust legislation clearance

***Strategic collaborations with pharmaceutical industry validating potential value and commercialization potential for BioArctic with proven track record of delivering on research collaborations***

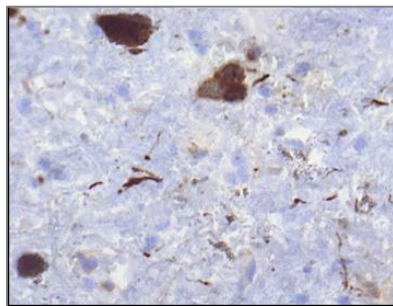
# BAN0805 – Groundbreaking Disease Modifying Drug in PD with Rationale for Selective Targeting of Alpha-synuclein Oligomers/Protofibrils

## Rationale for targeting alpha-synuclein

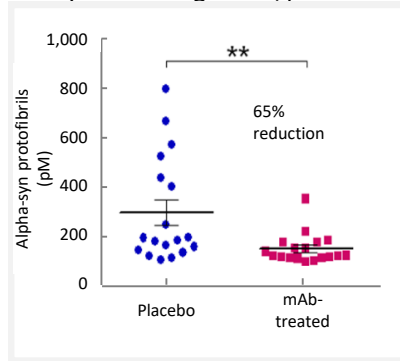
Human genetics

Pathology

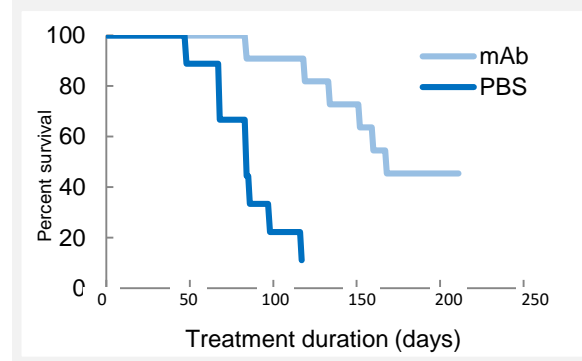
Pre-clinical proof of concept



Reduction of neurotoxic alpha-synuclein oligomers/protofibrils



Increases lifespan



Alpha-synuclein mutations lead to PD or Dementia with Lewy Bodies and are associated with increased oligomer/protofibril formation

Alpha-synuclein deposition is a hallmark of PD pathophysiology and alpha-synuclein oligomers/protofibrils are elevated in PD

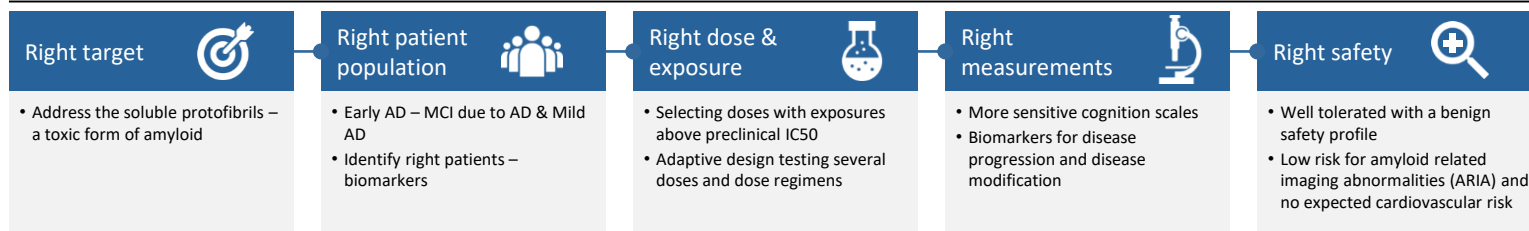
**Oligomer/protofibril selective antibody** reduces neurotoxic alpha-synuclein oligomer/protofibril levels, delays disease progression and increases life-span in a PD mice model

**BAN0805 in preparation for IND to start clinical trials in the US 2019**

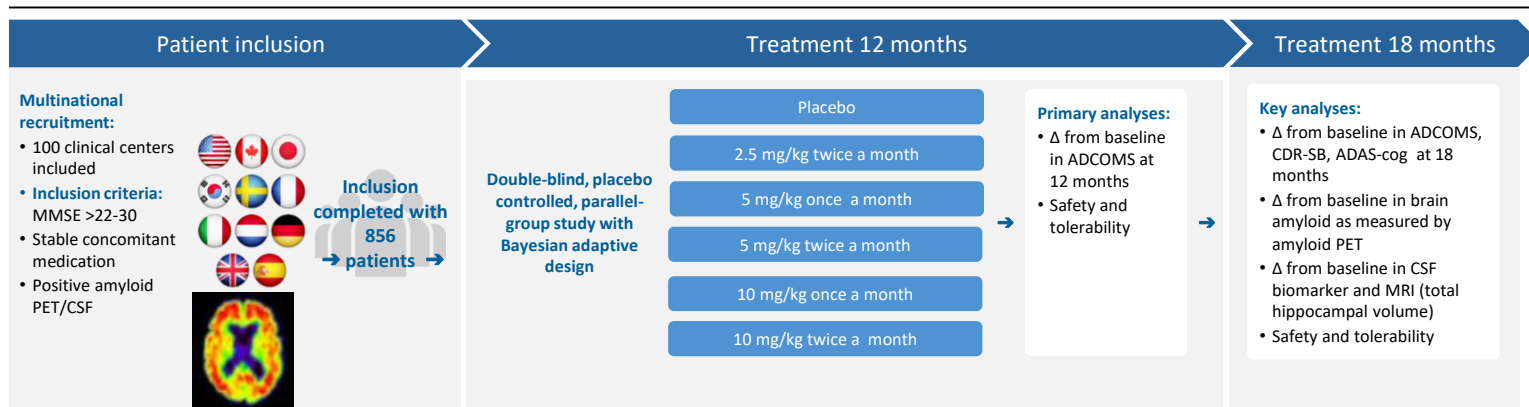
# BAN2401 – Innovative Phase 2b Study Design

## Positive 18 Month Results Reported

### Important parameters



### Phase 2b study design



**BAN2401 18 months treatment demonstrated an effect on both cognition and biomarkers with a good tolerability profile**

**Completion of study after 18 months treatment and 3 months follow-up - Q4 2018**

Source: Company information

Note: ADCOMS = Alzheimer's Disease Composite Score, an evaluation tool developed by Eisai

# Positive Phase 2b Study Results Support BAN2401 as a Potential Treatment for a Broad Population of Early Alzheimer Patients

## BAN2401 Treatment Effect in Early AD

### Clinical Outcome Measures

- Slowing of disease progression observed across clinical outcome measures at the highest dose, including 30 % on ADCOMS
- Slowing of disease progression observed across sub-groups

### Brain Amyloid PET

- Pronounced dose-dependent amyloid clearance across the dose range
- 81% of subjects converted to amyloid negative state
- Consistent and pronounced amyloid clearance across all sub-groups

### CSF Biomarkers

- Elevated Abeta demonstrates target engagement
- Impact on AD pathophysiology with benefits on neuro-degeneration markers: t-tau, p-tau, neurogranin and NfL

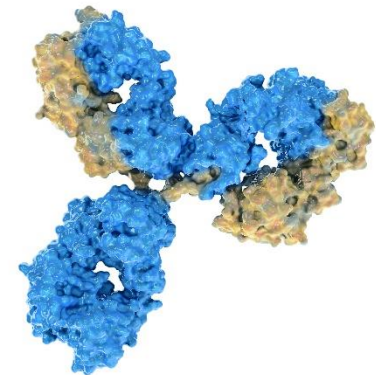
BAN2401 was well tolerated with < 10% ARIA-E at any dose

Selectively targeting Abeta protofibrils with low affinity to monomers confer an advantageous benefit risk profile



## BAN2401 – Next Steps

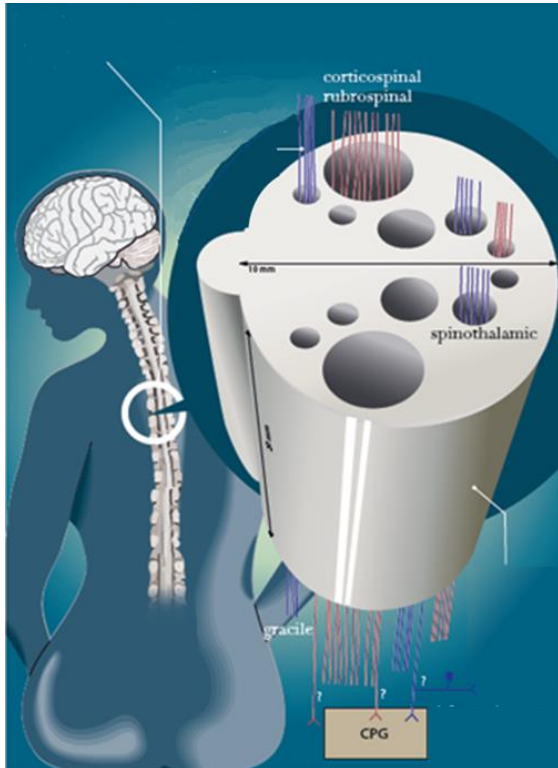
- Eisai is currently conducting interactions with regulatory agencies regarding the future BAN2401 program
- The study will be completed in Q4 2018 and includes a further 3 months follow-up after completion of 18 months of treatment (at 21 months)
- Open-label extension study with BAN2401, without placebo, for patients from the Phase 2b study will be initiated Q4 2018



# SC0806 – Unique Regenerative Treatment of Complete SCI

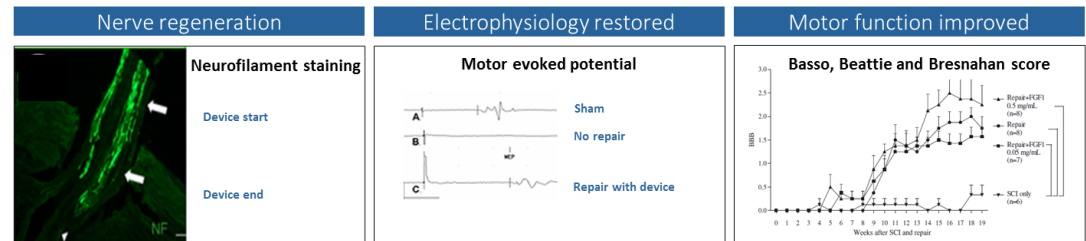
## SC0806 – Regenerative Treatment of Complete SCI

## 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> |



### Preclinical Proof of Concept shown in rats

- Rat experiments demonstrate nerve regeneration, restored electrophysiology and motor function
- The motor evoked potential (MEP) has been restored in rats with resected spinal cords

Source: Nordblom et al. Restorative Neurology and Neuroscience 30 (2012) 91–102

# SC0806 – Unique Regenerative Treatment of Complete SCI

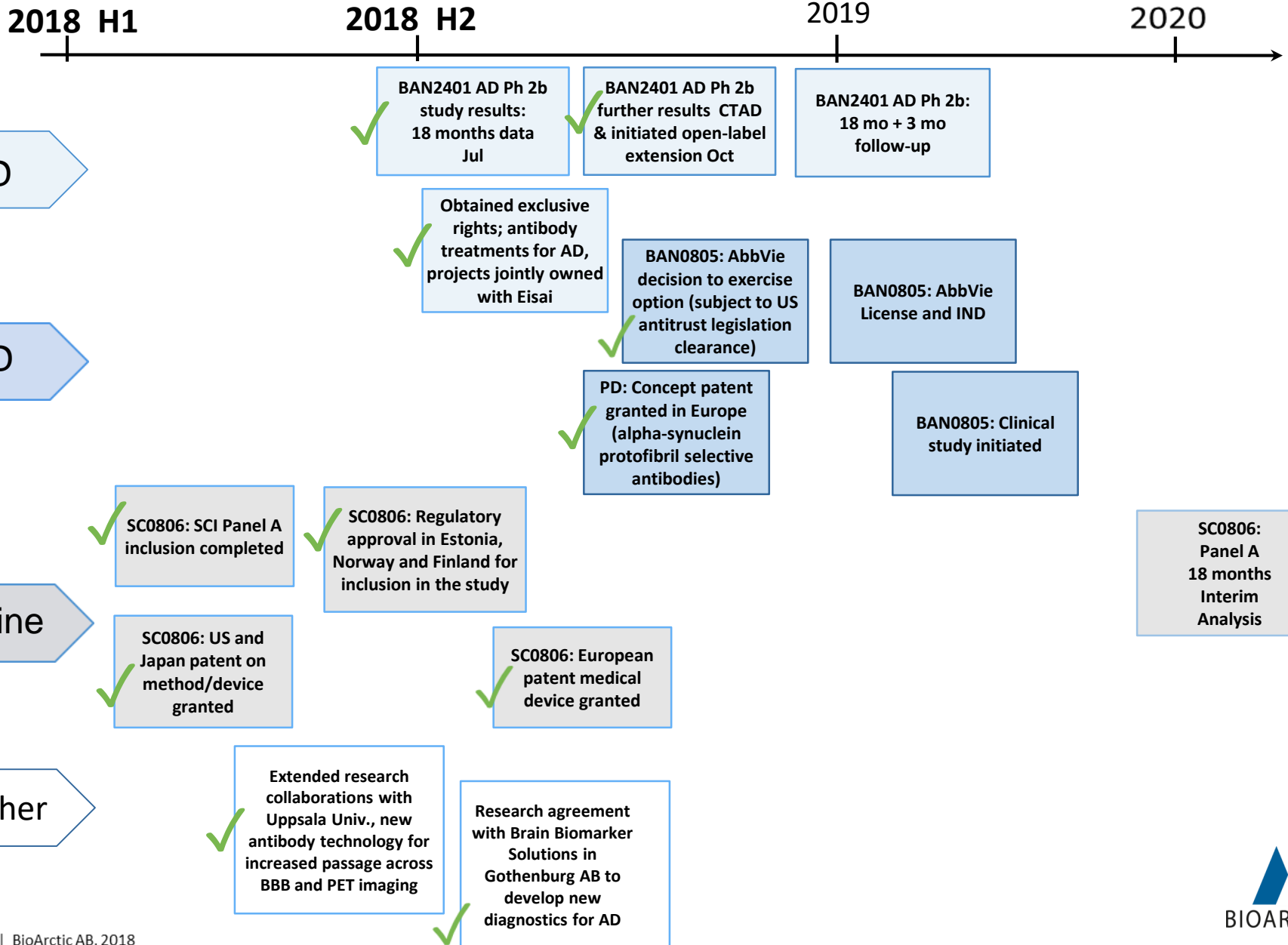
## The Lokomat™ used in the Rehabilitation



## Project Status

- Clinical Phase 1/2 trial ongoing with SC0806 in patients with Complete Spinal Cord Injury
  - Surgery in Sweden
  - Rehabilitation 18 months with Lokomat™ in Sweden, Estonia, Finland and Norway
  - Patients receiving SC0806 treatment are given the option of 12 months additional participation in an extension study
  - 9 patients included in Panel A (6 treated with SC0806 and 3 control patients)
  - Screening of patients for Panel B on-going
  - Interim analysis planned Q4 2019/Q1 2020
- Orphan Drug designation in US and EU – granting 7 and 10 years exclusivity, respectively
- EU Horizon 2020 research and innovative program Grant Agreement No. 643853 of MEUR 6.4

# Recent & Anticipated News Flow



# Thank you for your attention

## Q&A

Gunilla Osswald, CEO, PhD



### Next report & IR Contact

- ▶ **Next report:**  
Full Year Report 2018  
Feb 14, 2019
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