



**Health Tuesday 8.1.2019  
Funding in Health and Wellbeing**

**H2020 funding for clinical development  
– case Herantis Pharma  
Päivi Vuorio**

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# TreatER – European Union funding for 2017-2019

**What is TreatER?**  
**Who are we in TreatER?**  
**How TreatER was set up?**  
**Advice, comments...**

This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 732386



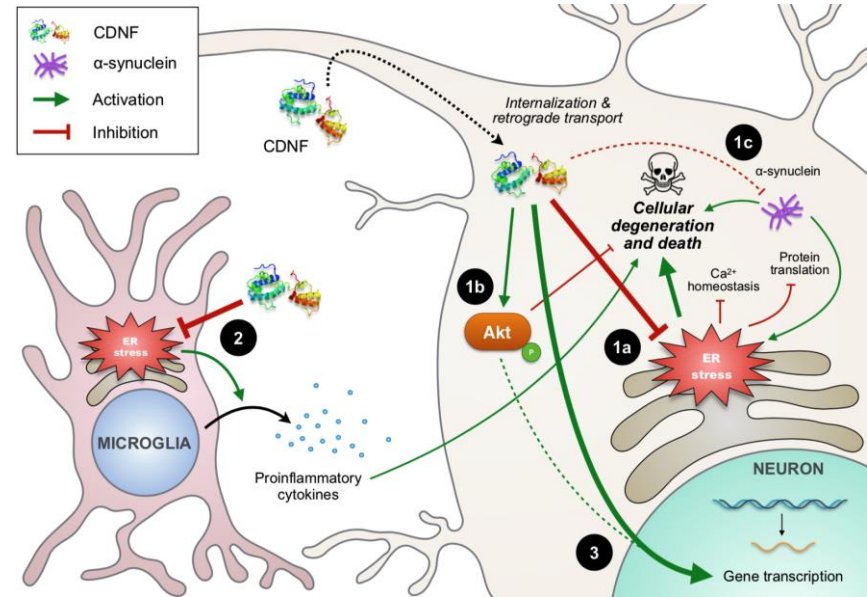
# What is TreatER ?

- **Clinical study in Parkinson's disease with two unique goals:**
  - 1) Proof-of-concept of CDNF protein for disease modification
  - 2) Validation of clinically tested device for intracerebral drug delivery
- **Clinical study divided in two parts - primary and extension study:**
  - A Phase I-II, Randomised, Double-Blind, Placebo Controlled, Safety and Tolerability Study of Intermittent Bilateral Intraputamenal Cerebral Dopamine Neurotrophic Factor (CDNF) Infusions Administered via an Investigational Drug Delivery System to Patients with Idiopathic Parkinson's Disease (PD) of Moderate Severity.
  - A Randomised, Double-Blind, Multi-centre, Active Treatment, Extension and Safety Study for Patients with Idiopathic Parkinson's Disease (PD) Who Previously Completed the CDNF/DDS Main Study HP-CD-CL-2002.
- **Basic CDNF research to uncover further potential in treatment of neurodegenerative diseases**
- **Business development: partnering, commercialisation**
- **EU funding ~6 million euro**

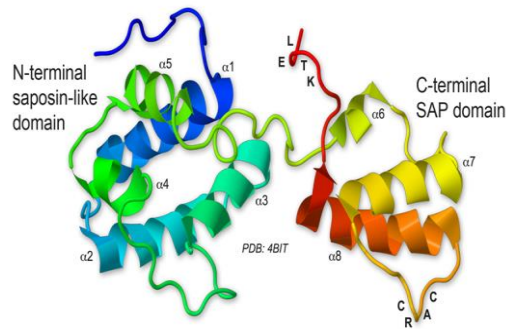
# What is TreatER?

## Eight Work Packages

1. Primary study
2. Extension study
3. Clinical data analysis
4. Basic research
5. Partnering and commercialization
6. Dissemination and communication
7. Project management
8. Ethics



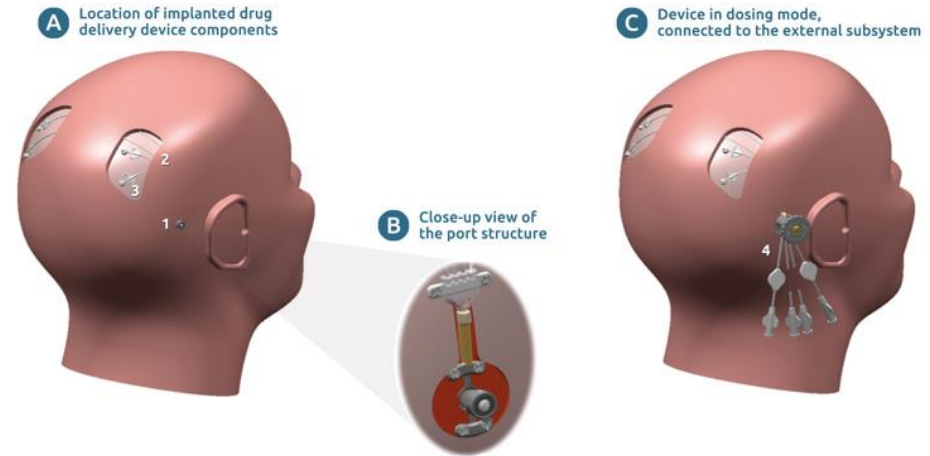
# What is TreatER ? - CDNF



- CDNF = Cerebral Dopamine Neurotrophic Factor, 18 kDa protein, an innovation of Prof. Mart Saarma and his group, University of Helsinki, Institute of Biotechnology
- Endogenous secreted protein; found in normal plasma and CSF, declines with aging
- Effectively protects and restores the function of degenerating and dying neurons in many *in vivo* models of neurodegenerative diseases, incl. Parkinson's disease
- Patented worldwide by Herantis, full commercial rights at Herantis
- Administered in brain using implanted drug delivery system, monthly dosing

# What is TreatER ? – Drug Delivery System, DDS

- CDNF does not permeate blood brain barrier
- Intraputamenal administration via DDS
- DDS implanted in robot-assisted neurosurgery: 4 catheters to putamen area
- Catheters connected via a port to external system connected to infusion pumps
- Developed and technology owned by Renishaw plc (UK)
- Clinically tested in studies in UK



# What is TreatER ? – Clinical study

- 18 patients in 3 study sites (Stockholm, Lund, Helsinki):
- Screening for eligibility, 15 inclusion and 18 exclusion criteria to be met before randomization
- Implantation of DDS in neurosurgical procedure
- 6 monthly infusions of CDNF or placebo in primary study and 6 monthly CDNF infusions in extension study
- 25 study visits, a number of assessments, e.g. neuropsychiatric evaluations, laboratory safety assessments, brain MRI, CT and PET scans and home diary based evaluations





# Who are we in TreatER?

**HERANTIS**  
PHARMA

**RENISHAW**   
apply innovation™

## Herantis Pharma Plc

- 12-people Finnish public drug development company
- Focused on regenerative medicines for breakthrough in unmet clinical need: neurodegenerative diseases, lymphedema
- Both programs originate from University of Helsinki
- Merger of Hermo Pharma and Laurantis Pharma 2014
- TreatER: CDNF for clinical study, clinical study sponsor

## Renishaw plc

- Multi-industry, UK based company: e.g. precision measurement, position and motion control, metal 3D printing, healthcare (neurological products)
- ~4800 employees, 70 offices in 35 countries
- TreatER: Provider of stereotactic robot and accessories, software for surgical planning, support for neurosurgery, and DDS

**HERANTIS**  
PHARMA

# Who are we in TreatER?



## 3 clinical study sites

- Karolinska University Hospital, Stockholm
- Skåne University Hospital, Lund
- Helsinki University Hospital
  - Neurology
  - Neurosurgery
  - Radiology
  - Hospital pharmacies
  - Laboratories

## PET imaging

- Karolinska Institutet, Stockholm
- In Finland PET studies done in Turku PET Centre under separate agreement

# Who are we in TreatER?



HELSINGIN YLIOPISTO  
HELSINGFORS UNIVERSITET  
UNIVERSITY OF HELSINKI



UNIVERSITY OF  
OXFORD

## Basic research

- University of Helsinki, Centre for Biotechnology, Prof. Mart Saarma's research group
- University of Oxford, UK, Nuffield Department of Clinical Neurosciences, Assoc. Prof. Laura Parkkinen's research group
- Nonclinical research on CDFN, MANF and biomarkers

# Who are we in TreatER?



- EPDA = European Parkinson's Disease Association (120000 members in nearly 30 countries)
- Suomen Parkinson-liitto ry (~8000 members) and Parkinson Förbundet (Sweden, ~9000 members) as EPDA member organisations
- TreatER: Main responsibility of WP6 on dissemination and communication

# Who are we in TreatER?



## 2 mid-sized pharmaceutical companies

- Orion Corporation (Finland)
- H. Lundbeck AS (Denmark)
  - Nordic pharmaceutical companies, CNS disorders as a core therapy area in both companies
- TreatER: No active role in practical work in clinical or nonclinical studies. Advisory support will increase along the project progress.

# How TreatER was set up?

- Consortium core structure: Herantis (CDNF) and Renishaw (DDS) + clinical study sites + basic research + big/mid size pharma + patient organisation
- The first effort to apply for EU funding from H2020 in 2014. Most of the present partners involved already at that time. Application passed the 1<sup>st</sup> round but not the 2<sup>nd</sup>.
- Due to conflicts of interest some eager partner candidates had to withdraw from the process and were replaced by other organisations
- The second, and this time successful try in 2016. Per Commission demand, last-minute change in project coordinator from Herantis to University of Helsinki. The Commission regarded Herantis as a too small player to administer 6 m€ grant.
- Project start on 1 January 2017

# TreatER - Comments, advice

- Review of earlier approved EU projects, e.g. the structure of consortium, beneficial when compiling your application
- Know your partners. Focus on including the relevant partners, the ones that you want to collaborate with or without support from EU. Do not concentrate on finding partners e.g. based on geographical location.
- Conflicts of interest should be checked with the administration of candidate partner organization early enough. Research people might not always have knowledge on all commitments.
- As the coordinator, be flexible and adaptive throughout the application process – last-minute surprises will come
- Follow the call information.
- Thorough review of application by experienced reviewer(s) (whether consultant(s) or inhouse expert(s))

# TreatER Comments, advice

- Commission portal not very user friendly but manageable anyway – regards both the project application and implementation
- Know your Project Officer in the Commission. Meet her/him personally if possible.
- Ask for advice – free of charge - from Business Finland/EUTI, whether it is about the application or managing the project. Highly recommended!
- We do not know yet about the results, but my overall experience of collaboration within the TreatER consortium and with the Commission is now very positive



# TreatER - Further information

- <https://treater.eu/>
- <https://clinicaltrials.gov/ct2/show/NCT03295786>
- <http://herantis.com/pipeline/cdnf-in-parkinsons-disease/>
- <https://www.renishaw.com/en/neurosurgical-products-and-systems--6332>
- <https://www.epda.eu.com/>
- <https://yle.fi/uutiset/3-10135949>
- <https://www.parkinson.fi/ajankohtaista/etsitaan-potilaita-laaketutkimukseen>
- <https://www.horisontti2020.fi/>

# TreatER

## Developing a novel treatment for Parkinson's disease

A European consortium bringing together experts from neurology and neurosurgery together with developers of novel drugs and drug delivery systems