

CDNF: Finnish academia-industry collaboration for unique clinical study in Parkinson's disease

Brain Diseases Symposium, Helsinki 14 Oct 2016 Pekka Simula, CEO, Herantis Pharma Plc

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Significant unmet clinical needs in Parkinson's disease

Parkinson's disease (PD) is a progressive neurodegenerative disease, which

cannot be cured

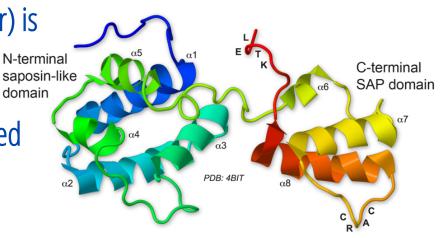
Estimated 7.000.000 patients worldwide

- Europe's financial burden caused by PD in 2010: €13,9 billion
- Available PD treatments are symptomatic and only address the motor symptoms of the disease
- Two huge unmet clinical needs remain:
 - Addressing non-motor symptoms, which based on recent research have a much greater impact on quality-of-life than motor symptoms
 - Disease modification i.e. slowing down or stopping disease progression



Promising therapeutic identified at University of Helsinki

 CDNF (Cerebral Dopamine Neurotrophic Factor) is a novel neurotrophic and neuroprotective factor identified by professor Mart Saarma's group at the University of Helsinki and published in Nature



- ✓ CDNF protects dopaminergic neurons
- ✓ Promising data in several Parkinson's disease and other neurodegenerative disease models
- ✓ Disease model data suggest CDNF as a potentially disease-modifying treatment, which alleviates both motor and non-motor symptoms
- Great target for drug development which is however slow, risky, and very expensive



CDNF development started as industry-academia collaboration





- Manufacturing
- Toxicology
- Regulatory planning

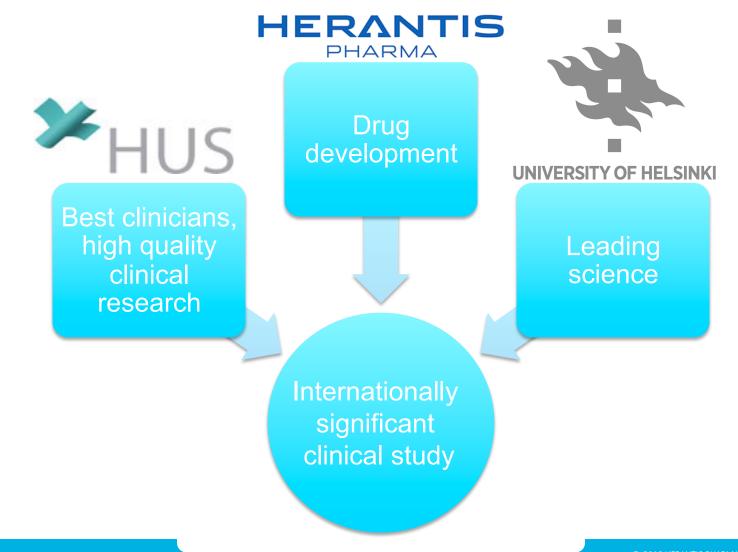


Scientific research

- Mechanism of action
- Disease models
- Peer-reviewed publications

Supporting
each other also
in funding processes,
symposiums, 3rd parties, etc!

Joining forces with clinical experts when approaching the clinic...



...for the international attention you need



- Years of hard work and collaboration lead to Horizon 2020 application, which recently passed the evaluation for a €6 million EU grant for the first-in-human CDNF study in PD
 - Main beneficiaries include HUS, University of Helsinki, and Herantis
 - Turku PET centre also involved with functional imaging expertise
 - Lund and Karolinska University Hospitals and other valued partners joined the consortium
- Tekes support has been instrumental on the long development path toward the clinic and the international attention



We hope to continue collaboration in other indications — case ALS

ALS is an aggressive motoneuron disease with no cure. Thanks to promising preclinical data CDNF was granted Orphan Designation for the treatment of ALS in 2016 in EU and USA



- ALS is a death sentence given to estimated 140,000 patients a year
 - Financial burden of motoneuron diseases in Europe: €7.7 billion
- Finland has great expertise in ALS how to continue development of CDNF for the treatment of ALS in Finland?
 - ALS patients of today have no hope. Would we like to give the first opportunity of trying CDNF to Finnish patients, clinicians, and researchers?
 - Herantis is a small company with limited resources and hands already full commercializing two Finnish inventions: CDNF for Parkinson's disease, and Lymfactin® for secondary lymphedema. We cannot expand to ALS alone!





Thank you

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