

Herantis Pharma Plc company presentation Nordic Life Sciences days

Stockholm 14 Sep 2016 Pekka Simula, CEO

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Herantis Pharma Plc

- Drug development company developing novel pharmaceutical products based on leading science
- Focus in regenerative medicine, developing growth factor based therapies and anti-inflammatory compounds in indications with significant unmet clinical needs
- Target indications include
 - Secondary lymphedema
 - Parkinson's disease
 - ALS
 - Dry Eye





Herantis portfolio (drug candidates)

Drug candidate	Indication	Preclin	Phase 1	Phase 2	Phase 3
CDNF neuro- protective factor	Parkinson's disease*	Λ	Λ		
CDNF neuro- protective factor	Amyotrophic lateral sclerosis (ALS)	Λ			
CDNF neuro- protective factor	Other neuro- degenerative diseases	**			
Lymfactin®	Secondary lymphedema	Λ	Λ		
Cis-UCA Eye Drops	Dry Eye	Λ	Λ	Λ	
Cis-UCA Emulsion Cream	Atopic dermatitis	Λ	Λ	Λ	

^{*}Phase 1 started by submissions of Clinical Trial Applications

^{**}Promising unpublished preclinical data exist. Formal development program not started



Lymfactin® for secondary lymphedema

Lymfactin® is the world's first clinical stage gene therapy aiming to repair the lymphatic system

- Secondary lymphedema is a chronic progressing disease with no approved medicines
 - Lymphedema causes chronic and severe swelling of the affected limb,
 significantly impacting quality of life
 - Over 30,000 new breast cancer associated cases diagnosed every year
 - Estimated market size € hundreds of million
- In preclinical models Lymfactin® repaired the cause of lymphedema
 - World's first gene therapy aiming to repair the lymphatic system
 - Potential also in primary lymphedemas with our patented technology



- Phase 1 clinical study ongoing in patients with breast cancer associated lymphedema
 - Database lock and top-line data expected in 2017



CDNF for Parkinson's disease (PD)

Based on preclinical data we believe CDNF is the most promising disease-modifying treatment for PD

- Parkinson's disease is a progressive, incurable neurodegenerative disease
 - Symptoms include tremors, slowness of movement, sleep disturbance, depression
 - Estimated seven million patients worldwide
 - Current market over \$3 billion with treatments that only address motor symptoms of the disease
 - Disease modification and non-motor symptoms remain huge unmet clinical needs
- Based on broad preclinical data CDNF is expected to treat the disease, not just its motor symptoms
 - Clinical Trial Applications submitted for phase 1-2 clinical study to start enrolling patients in 2016: N=18, randomized, placebo-controlled
 - Planned study sites include Karolinska University Hospital and Lund University Hospital





CDNF for ALS (Amyotrophic lateral sclerosis)

ALS is an aggressive motor neuron disease leading to death typically in 2-5 years. CDNF may improve survival and quality of life of patients



- Estimated 140,000 new ALS cases are diagnosed annually
- Symptoms include muscle weakness, difficulty in speaking, swallowing, and eventually breathing
- CDNF was granted orphan designation in 2016 for treatment of ALS by both EMA and FDA, based on scientific data to justify the assumption that CDNF will be of significant benefit to ALS patients
- Preclinical development continues in close collaboration with leading scientists; a clinical development program is under consideration



Herantis continues collaboration with scientific fathers

CDNF is based on long term research by professor Mart Saarma

- Vice President of European Research Council (ERC)
- Numerous international science prizes including Karl Schlossmann
 Science Prize, Runeberg Medical Science Prize, Finnish Innovation Prize,
 Estonian State Prize for Science and Technology
- Among leading and most appreciated researchers in the world in neurotrophic factors; found CDNF, published in Nature





Lymfactin is based on long term research by academy professor Kari Alitalo

- Director of the Translational Cancer Biology research program, nominated several times a national centre of excellence; over 500 scientific publications
- Numerous international science prizes including InBev-Baillet Latour International Health Prize, Louis Jeantet Prize for Medicine, Anders Jahre Prize, Dr. A.H.Heineken Prize
- Among leading and most appreciated researchers in the world in vascular endothelial growth factors (VEGFs); invented Lymfactin[®]



Herantis Pharma summary

- Ongoing clinical development: Two fully funded clinical studies in regenerative medicine
 - CDNF for a disease-modifying treatment of Parkinson's disease
 - Clinical study passed evaluation toward a €6 million Horizon 2020 grant
 - Lymfactin® for the treatment of secondary lymphedema caused by breast cancer treatments
- Looking for development partners to also expand in new indications
 - Orphan designation granted for CDNF for the treatment of ALS
 - Promising preclinical efficacy data in yet another neurodegenerative disease
 - Lymfactin[®] has potential also in primary lymphedema





Thank you

For additional information please visit www.herantis.com