SPHERAMINE – CELL THERAPY FOR PARKINSON´S DISEASE

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The presentation gives an overview of preclinical data, the first application in humans, and an ongoing Phase II trial investigating a novel cell-based therapy for Parkinson's disease.
Where surgical interventions are involved, the performance of controlled trials, ideally placebo-controlled studies constitutes a challenge in terms of balance of ethical issues and scientific requirements, equipoise of surgical risk and the need of unbiased clinical data. The presentation describes a clinical study in patients with Parkinson's Disease which was designed for the evaluation of a therapeutic approach aiming at the treatment of a neurological condition with a high unmet medical need.
The STEPS trial, a multi-center, double-blind, sham-surgery controlled study for the evaluation of safety, tolerability and efficacy of Spheramine® (cultured human retinal pigment epithelial cells on microcarriers) implanted bilaterally into the postcommissural putamen of Parkinson's disease patients, is described in this presentation. 68 patients in Hoehn and Yahr stages 3 and 4 were randomly assigned to receive intracranial injection of Spheramine®, or sham-surgery, in a ratio of 1:1.
Study procedures for selection of patients, selection of neurological and neurosurgical investigational centers, safety precautions, assessments, and methods for maintenance of the blind are discussed. The rationale for the choice of the control group and sample size, primary and secondary endpoints, statistical methods and duration of follow-up are introduced. An outlook on the expected scope and time requirements for development of an innovative biological therapy to market approval is given.