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Are stem-cell therapies for Parkinson's disease ready for clinical trials?

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As stem cell-based therapies are moving rapidly towards clinical trials, treatments for Parkinson's Disease (PD), an incurable condition, may be on the horizon. A recent announcement of a Phase I/IIa clinical trial involving transplantation of stem cells into the first human subjects has raised hope among patients and sparked discussions in the research community. In a commentary published in the *Journal of Parkinson's Disease*, authors propose five key questions that should be addressed as this trial begins.

News of California-based biotechnology company, International Stem Cell Corporation's (ISCO) clinical trial spread rapidly through online and print news and social media. Many PD patients and their families have questioned whether they should try to sign up for such a study. "As with many such exiting news items, however, one should also react with caution, especially since the outcome of this trial can affect the development of other stem cell programs moving towards clinical trials," explained lead author Roger A. Barker, PhD, of the John van Geest Centre for Brain Repair, Department of Clinical Neurosciences, University of Cambridge, UK.

In the wake of clinical trial announcements from ISCO it is timely to provide insights into how the opportunities provided to PD patients in this and similar trials should be evaluated. Without this, the patient community is left trying to interpret complex scientific issues on its own, and individual patients cannot make informed decisions on whether they should seek to participate in the planned trials or not.

The authors review the clinical transplantation trial in PD planned by ISCO this year in light of the criteria defined by the GForce-PD. This global collaborative initiative aims to define criteria to gauge progress from experimental results towards clinical trials, while ensuring that all steps are conducted to the highest standard and that the trials are not initiated prematurely.

Before any stem-cell-based trial in PD is done, the authors call for discussion of these five key questions:

- What is being transplanted, and what is the proposed mechanism of action?
- What are the pre-clinical safety and efficacy data supporting the use of the proposed stem cell product?
- Can arguments concerning ethics, risk mitigation, or trial logistics outweigh concerns regarding the expected efficacy of the cell and constitute a primary justification for choosing one cell type over another in a clinical trial?
- What is being claimed regarding the potential therapeutic value of the stem cell-based therapy – better control of symptoms or a cure?
- What is the regulatory oversight of the trial and is it guided by input from experts in the field?

In this commentary, the authors briefly review how cell-based therapies for PD have evolved and discuss some of the early results, as well as some of the ethical issues concerning fetal stem-cell use. They then elaborate on the five key questions and express some concern that there is missing or incomplete information available from ISCO. In particular, there are concerns that the particular cell types being transplanted may not function as desired, and that supporting safety and efficacy data have not been made public. The consortium also suggests that the length of follow-up in the proposed trial may not be sufficient.

While early claims suggesting the possibility of a "cure" had been made, ISCO has now taken a more measured position regarding potential benefits. Nevertheless, the authors caution that exaggerated claims are all too common, given the regulatory hurdles, commercial interests, and personal ambitions of the participants in early-stage clinical trials.

With the reality of the first human clinical trial in patients with PD upon us, co-author and Editor-in-Chief of the *Journal of Parkinson's Disease* Patrik Brundin, MD, PhD, Director of the Center for Neurodegenerative Science at Van Andel Research Institute in Grand Rapids, MI, commented, "This is an exciting prospect but should only be undertaken when all the necessary pre-clinical data and regulatory approvals have been obtained and verified and the criteria for moving those cells to trials fully resolved and met. Acting prematurely has the potential not only to tarnish many years of scientific work, but can threaten to derail and damage this exciting field of regenerative medicine. Hopefully, in 2016, we are ready to take a more careful approach as we strive to repair the PD brain with stem cell-based therapies, avoiding many of the mistakes that have dogged this field over the last three decades."

More information: "Are Stem Cell-Based Therapies for Parkinson's Disease Ready for the Clinic in 2016?" by Roger A. Barker, Malin Parmar, Agnete Kirkeby, Anders Bjőrklund, Lachlan Thompson, and Patrik Brundin (DOI: 10.3233/JPD-160798), published online in advance of *Journal of Parkinson's Disease*, Volume 6, Issue 1

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