Perspectives of stem cell therapy

The number of clinical trials evaluating ocular stem cell therapies is growing. Stem cell research has recently started its transition from bench to bedside, and several clinical trials are underway to investigate for the first time the therapeutic potential of pluripotent stem cells in humans. Strikingly, all current clinical trials worldwide are targeting diseases of the eye, thus emphasizing the unique position of ophthalmology at the forefront of this exciting new research field.

Pluripotent stem cells are defined by two characteristic features, namely the ability to continuously regenerate themselves during cell division (self-renewal) and the capability to differentiate into every specialized cell type of the human body (pluripotency), the latter have demonstrated that fully differentiated retinal cells such as retinal pigment epithelial (RPE) cells can be generated from both human ESC and iPSC in culture and that subretinal transplantation of these cells can prevent visual loss in animal models of retinal degenerative diseases. Meanwhile, several groups are conducting or preparing first clinical trials to assess safety and efficacy of stem cell-derived RPE cells in retinal diseases such as late-stage AMD and Stargardt disease. Therapeutic approaches under investigation include subretinal injection of suspensions of ESC-derived RPE cells, subretinal transplantation of sheets of ESC-derived RPE cells on artificial carrier membranes, and subretinal transplantation of sheets of iPSC-derived RPE cells without artificial carrier membranes (see figure). In contrast to ESC, iPSC can be generated from the patient’s own cells and thus avoid the ethically controversial use of embryonic tissue as well as the need for life-long immunosuppressive treatment to prevent graft rejection. Results from these clinical trials are eagerly awaited. Against the background of current scientific knowledge, the eye seems to be an organ particularly well-suited for the therapeutic application of stem cells. This results from the combination of several favourable factors, in particular the fact that in many retinal degenerative disorders only a single cell type such as the RPE is primarily affected by the disease and that highly differentiated RPE cell grafts for transplantation can be efficiently generated from pluripotent stem cells. Moreover, these grafts can be readily delivered to their correct subretinal position in the diseased eye using already well-established microsurgical techniques. The number of clinical trials evaluating ocular stem cell therapies is growing and the results will help to determine whether this exciting new technology is a future therapeutic option for currently untreatable retinal diseases.

References:

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