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Stem cells for retinal disease: a perspective on the promise and perils

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ABSTRACT

PURPOSE: To summarize key concepts, and early safety and efficacy signals from clinical trials for stem/progenitor cell-based interventions for retinal disease.

DESIGN: Interpretive essay.

METHODS: Review and synthesis of selected recent reports of stem/progenitor cell-based approaches for retinal disease, with interpretation and perspective.

RESULTS: Stem/progenitor cell-based interventions represent a novel class of potential therapies for retinal diseases, such as age-related macular degeneration, inherited retinal dystrophies, and others. Sources include pluripotent stem cells, fetal and postnatal tissues. Two mechanisms of “rescue” have been proposed: regenerative or trophic. While pluripotent and fetal sourced-cell types have been tested in preclinical animal models of retinal disease, many postnatal stem/progenitor cell populations currently in trial do not have preclinical safety or efficacy data. Some early phase trials of cell therapies suggest acceptable safety profiles. Other reports, involving some types of autologous, non-ocular cell sources, have been linked to severe, blinding complications. Larger trials will be needed to determine short and long-term safety and efficacy of these cell-based interventions.

CONCLUSIONS: Stem/progenitor cell-based interventions have the potential to address blinding retinal diseases that affect hundreds of millions worldwide. Yet no FDA-approved stem cell therapies for retinal disease exist. While some early phase trial data are promising, reports of blinding complications from cell interventions remain troubling. It is paramount to apply a strong level of scientific rigor toward a well-planned, step-wise sequence of preclinical and clinical studies, to determine whether this class of potential therapies will be safe and effective for individuals with retinal diseases.