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Stargardt macular dystrophy and evolving therapies

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Authors

Hussain, Rehan M.
Ciulla, Thomas A.
Berrocal, Audina M.
Gregori, Ninel Z.
Flynn, Harry W.
Lam, Byron L.

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Department

Ophthalmology, School of Medicine

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Abstract

Introduction: Stargardt macular dystrophy (STGD1) is a hereditary retinal degeneration that lacks effective treatment options. Gene therapy, stem cell therapy, and pharmacotherapy with visual cycle modulators (VCMs) and complement inhibitors are discussed as potential treatments.

Areas covered: Investigational therapies for STGD1 aim to reduce toxic bisretinoids and lipofuscin in the retina and retinal pigment epithelium (RPE). These agents include C20-D3-vitamin A (ALK-001), isotretinoin, VM200, emixustat, and A1120. Avacincaptad pegol is a C5 complement inhibitor that may reduce inflammation-related RPE damage. Animal models of STGD1 show promising data for these treatments, though proof of efficacy in humans is lacking. Fenretinide and emixustat are VCMs for dry AMD and STGD1 that failed to halt geographic atrophy progression or improve vision in trials for AMD. A1120 prevents retinol transport into RPE and may spare side effects typically seen with VCMs (nyctalopia and chromatopsia). Stem cell transplantation suggests potential biologic plausibility in a phase I/II trial. Gene therapy aims to augment the mutated ABCA4 gene, though results of a phase I/II trial are pending.

Expert opinion: Stem cell transplantation, ABCA4 gene therapy, VCMs, and complement inhibitors offer biologically plausible treatment mechanisms for treatment of STGD1. Further trials are warranted to assess efficacy and safety in humans.

Keywords

C20-D3-vitamin A, Stargardt macular dystrophy, visual cycle

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