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ADENO-ASSOCIATED VIRAL VECTORS FOR AMD TREATMENT

THERAPEUTICS

Viral vectors that activate Rap1a to treat AMD.

TECHNOLOGY TYPE

Gene Therapy, Silencing, & Editing
Ophthalmology

STAGE OF DEVELOPMENT

Studies in mice underway.

IP PROTECTION

Provisional patent filed.

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TECHNOLOGY SUMMARY

Contrary to advances in treating wet AMD, dry AMD continues to elude modern therapeutics and there are no effective or established treatments despite dry AMD comprising 90% of all AMD incidences.

Researchers at the University of Utah have recently developed a potential gene therapy for the treatment of wet and dry AMD using adeno-associated viral vectors that inhibit the generation of reactive oxygen species (ROS). ROS have been linked to the disease progression of AMD. The inventors have shown that the activation of an enzyme, Rap1a, in retinal pigment epithelium (RPE) can reduce ROS generation. Sub-retinal injections of the viral vector into mice result in a modest improvement in the expression of Rap1a after immunostaining of mouse retinal tissue. Further validation of the vector could enable the creation of the first effective therapeutic for dry AMD and an alternative treatment option for wet AMD.

FEATURES AND BENEFITS

- Better alternative to Lucentis.
- Reduces inflammatory pathway signaling and generation of reactive oxygen species.
- Potential to reduce vision-loss from macular diseases associated with the death of RPE, such as dry AMD.

RECENT PUBLICATIONS

Wang, H., Han, X., Bretz, C. A., Becker, S., Gambhir, D., Smith, G. W., … Hartnett, M. E. (2016). Retinal pigment epithelial cell expression of active Rap 1a by scAAV2 inhibits choroidal neovascularization. Molecular Therapy - Methods & Clinical Development, 3, 16056. doi: 10.1038/mtm.2016.56

INVENTOR PROFILE

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