

UPDATED RETINAL BULLETIN

Attached is the latest list of Gene Therapy Clinical Trials. The potential of gene therapy for treatment of inherited retinal degeneration is growing!

**For additional information on this subject, please
visit our website at:**

www.rvrc.com

TABLE 1
Current Gene Therapy Clinical Trials²⁸

| Disease (Gene)/(Vector) | Sponsor | Phase | Status | Clinical Trial Sites | NCT# |
|-----------------------------------|--------------------------------------|-------|------------------------|---|----------|
| Leber Congenital Amaurosis | | | | | |
| <i>RPE65</i> / (AAV2) | Samuel G. Jacobson (Scheie, U. Penn) | I | Active, not recruiting | Shands Children's Hospital, University of Florida; Scheie Eye Institute, U. Penn | 00481546 |
| <i>RPE65</i> / (AAV2) | Spark Therapeutics (CHOP, U. Penn) | I | Active, not recruiting | CHOP, U. Penn | 00516477 |
| <i>RPE65</i> / (AAV4) | Nantes | I | Completed | Nantes University Hospital | 01496040 |
| <i>RPE65</i> / (AAV2) | AGTC | I/II | Active, not recruiting | OHSU; U. Mass, Worcester | 00749957 |
| <i>RPE65</i> / (AAV2) | Hadassah | I/II | Recruiting | Hadassah Medical Organization | 00821340 |
| <i>RPE65</i> / (AAV2) | Spark Therapeutics | III | Active, not recruiting | CHOP; U. Iowa | 00999609 |
| <i>MERTK</i> / (AAV2) | Fowzan Alkuraya | I | Recruiting | King Khaled Eye Specialist Hospital | 01482195 |
| Stargardt Disease | | | | | |
| <i>ABCA4</i> / (EIAV) | Sanofi | I/II | Recruiting | OHSU; Centre Hospitalier Nationale d'Ophthalmologie des Quinze-Vingts | 01367444 |
| Usher Syndrome | | | | | |
| <i>MYO7A</i> / (EIAV) | Sanofi | I/II | Recruiting | OHSU; Centre Hospitalier Nationale d'Ophthalmologie des Quinze-Vingts | 01505062 |
| Choroideremia | | | | | |
| <i>CHM</i> / (AAV2) | University of Oxford | I/II | Recruiting | Moorfields Eye Hospital; St Mary's Hospital, Central Manchester University; Oxford Radcliffe Hospitals Eye Unit, Southampton University Hospitals | 01461213 |
| <i>CHM</i> / (AAV2) | University of Oxford | II | Not yet recruiting | U. Oxford; Moorfields Eye Hospital UCL | 02407678 |
| <i>CHM</i> / (AAV2) | Ian M. MacDonald | I/II | Active, not recruiting | U. Alberta | 02077361 |
| <i>CHM</i> / (AAV2) | Byron Lam | II | Recruiting | Bascom Palmer Eye Institute | 02553135 |
| <i>CHM</i> / (AAV2) | Spark Therapeutics | I/II | Recruiting | CHOP, U. Penn | 02341807 |
| X-Linked Retinoschisis | | | | | |
| <i>RS1</i> / (AAV8) | NEI | I/II | Recruiting | NIH Clinical Center | 02317887 |
| <i>RS1</i> / (AAV2tYF) | AGTC | I/II | Recruiting | Massachusetts Eye and Ear Infirmary; Kellogg Eye Center, U. Mich; OHSU; Retina Foundation of the Southwest | 02416622 |

Continued on next page

Table 1 continued from previous page

| Retinitis Pigmentosa – Optogenetics | | | | | |
|--|--------------------------------------|------|---------------------------|--|----------|
| <i>Channelrhodopsin2</i> (RST-001) / (AAV) | RetroSense Therapeutics | I/II | Recruiting | Retina Foundation of the Southwest | 02556736 |
| Age-Related Macular Degeneration | | | | | |
| <i>sFLT01</i> / (AAV2) | Genzyme/Sanofi | I | Active, not recruiting | Retina Consultants of Arizona; Johns Hopkins University; Ophthalmic Consultants of Boston; U. Mass, Worcester; Retina Consultants of Houston | 01024998 |
| <i>sFLT01</i> / (AAV2) | Lions Eye Institute, Perth Australia | I/II | Active, not recruiting | Lions Eye Institute, Nedlands Australia | 01494805 |
| Endostatin, Angiostatin / (EIAV) | Oxford BioMedica | I | Active, not recruiting | U. Iowa; Johns Hopkins University; OHSU | 01301443 |
| Endostatin, Angiostatin (EIAV) | Oxford BioMedica | I | Recruiting, by invitation | Johns Hopkins University; OHSU | 01678872 |

RPE65 = retinal pigment epithelium-specific protein 65kDa; *MERTK* = *MER* proto-oncogene tyrosine kinase; *ABCA4* = ATP-binding cassette, sub-family A, member 4; *MYO7A* = myosin VIIA; *CHM* = choroideremia, rab escort protein 1; *RS1* = retinoschisin 1; *sFLT01* = soluble fms-related tyrosine kinase 1; AAV = adeno-associated virus; EIAV = equine infectious anemia virus; U. = University of; CHOP = Children's Hospital of Philadelphia; OHSU = Oregon Health & Science University; UCL = University College London; NEI = National Eye Institute; NIH = National Institutes of Health