

## Robin Ali – Biography

Robin Ali is Professor of Human Molecular Genetics at UCL Institute of Ophthalmology, London where he is Head of Department of Genetics. He is also the Theme Leader for Gene Therapy at NIHR Biomedical Research Centre for Ophthalmology, Moorfields Eye Hospital and Director of the UCL GMP gene therapy vector manufacturing facility. He is a Visiting Professor at The Kellogg Eye Center, University of Michigan and Founder and Chief Scientific Officer of MeiraGTx Ltd, a gene therapy company with offices in New York and London.

The main focus of Robin Ali's research is the development of gene and cell therapy for the treatment of retinal disorders. As chief investigator, he established the world's first clinical trial of gene therapy for retinopathy. The results from this trial reporting an improvement in vision (NEJM, 2008; NEJM 2015) along with results from two other trials, established proof-of-principle of gene therapy for inherited retinal degeneration. His group has also provided the first proof-of-concept for effective transplantation of photoreceptors (Nature 2006; Nature, 2012) which has provide the basis for ES cell-derived photoreceptor transplantation, now a major programme in his laboratory (Nature Biotech 2013). Robin Ali and members of his team have received numerous prizes and awards for their work on developing new treatments for retinal degeneration including the Pfizer /ARVO Karl Camras Translational Award in 2010 and Alcon Research Institute Award in 2009. In 2007 he was elected to the UK Academy of Medical Sciences and in 2009 appointed Senior Investigator of The UK National Institute of Health Research. He is President of the European Society of Gene and Cell Therapy and has served on the advisory boards of a number of funding bodies including the UK Medical Research Council (Neurosciences and Mental Health Board and the Regenerative Medicine Research Committee), Research to Prevent Blindness USA and Fighting Blindness Ireland, as well as the advisory boards of a number of pharmaceutical and biotech companies.

Robin Ali obtained his BSc (1988) and PhD in Genetics (1993) at University College London, continued by postdoctoral training at the MRC National Institute for Medical Research and UCL Institute of Ophthalmology and established his laboratory at UCL Institute of Ophthalmology in 1999.

### Recent selected publications

1. Long-term effect of gene therapy on Leber's congenital amaurosis. Bainbridge JW, Mehat MS, Sundaram V, Robbie SJ, Barker SE, Ripamonti C, Georgiadis A, Mowat FM, Beattie SG, Gardner PJ, Feathers KL, Luong VA, Yzer S, Balaggan K, Viswanathan A, de Ravel TJ, Casteels I, Holder GE, Tyler N, Fitzke FW, Weleber RG, Nardini M, Moore AT, Thompson DA, Petersen-Jones SM, Michaelides M, van den Born LI, Stockman A, Smith AJ, Rubin G, Ali RR. **N Engl J Med.** 2015 **372** (20) :1887-97
2. Gene therapy restores vision in rd1 mice after removal of a confounding mutation in Gpr179. Nishiguchi KM, Carvalho LS, Rizzi M, Powell K, Holthaus SM, Azam SA, Duran Y, Ribeiro J, Luhmann UF, Bainbridge JW, Smith AJ, Ali RR. **Nat Commun.** 2015 **6**:6006
3. Effective transplantation of photoreceptors isolated from three-dimensional cultures of embryonic stem cell-derived retina. Gonzalez-Cordero A, West EL, Pearson RA, Duran YCarvalho LS, Chu CJ, Naeem A, Blackford SJI, Georgiadis A, Lakowski J, Hubank M, Bainbridge JWB, Smith AJ, Sowden JC, Ali RR. **Nature Biotechnology** 2013; 31 (8) 741-747
4. Repair of the degenerate retina by photoreceptor transplantation. AC Barber, C Hippert, Y Duran, EL West, JW Bainbridge, K Warre-Cornish, UF Luhmann, J Lakowski, JC Sowden, RR Ali and RA Pearson. **Proc Natl Acad Sci U S A.** 2013; 110 (1): 354-9
5. Restoration of vision following transplantation of photoreceptors. RA Pearson, AC Barber, M Rizzi, C Hippert, T Xue, EL West, Y Duran, AJ Smith, JZ Chuang, SA Azam, UFO Luhmann, A Benucci, CH Sung, JW Bainbridge, M Carandini, KW Yau, JC Sowden, RR Ali **Nature** 2012, 485 (7396) 99-103.