



Professor of Human Molecular Genetics
 UCL Institute of Ophthalmology
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Qualifications

1993 PhD Genetics, University College London
 1988 BSc Genetics, University College London

Current Appointments

2017 - President, European Society of Gene and Cell Therapy
 2015 - Chief Scientific Officer, MeiraGtx Ltd
 2015 - Director, Wolfson Gene Therapy Unit (UCL facility for GMP vector manufacture)
 2014 - Visiting Professor, Kellogg Eye Center, University of Michigan
 2011 - Theme leader (Gene Therapy), NIHR Biomedical Research Centre, Moorfields Eye Hospital
 2009 - Chief Scientific Advisor, Fighting Blindness, Ireland
 2008 - Head, Department of Genetics, UCL Institute of Ophthalmology
 2003 - **Professor of Human Molecular Genetics, UCL Institute of Ophthalmology**

Previous academic appointments

2001 - 2003 Reader in Physiology, UCL Institute of Ophthalmology
 1999 - 2001 Lecturer, UCL Institute of Ophthalmology
 1994 - 1998 Senior Research Fellow, UCL Institute of Ophthalmology
 1992 - 1994 Postdoctoral Scientist, MRC National Institute for Medical Research, London

Previous industrial appointments

2012 - 2013 Retina Institute, Japan - Scientific Advisory Board
 2011 Regeneron Inc., USA - Consultant
 2010 - 2015 ReGenX BioSciences, USA - Medical Advisory Board
 2010 - 2014 Alcon Pharmaceuticals, USA - Scientific Advisory Board
 2007 Third Rock Ventures, USA - Consultant
 2005 - 2007 Amsterdam Molecular Therapeutics, Netherlands - Scientific Advisory Board
 2002 - 2014 Oxford Biomedica PLC, UK - Consultant

Other Appointments and Affiliations

(i) Funding Boards: From 2015 Research to Prevent Blindness, New York, Scientific Advisory Board 2012-2015 Telethon Italy, Scientific Committee 2011 - L'Agence nationale de la recherche (ANR), France, (University Hospital Institutes Jury) 2010-2013 UK Medical Research Council, Neuroscience and Mental Health Board 2011-2014 UK Medical Research Council, Regenerative Medicine Research Committee 2009 Health Research Board, Ireland (Molecular and Cellular Medicines Committee) From 2008 Fighting Blindness, Ireland (Chair of Scientific and Medical Advisory Board) From 2006 UK Multiple Sclerosis Society (Advisory Board for Myelin Repair Centres, Edinburgh & Cambridge) **(ii) Editorial Boards:** From 2008 Gene Therapy (Associate Editor from 2011) From 2007 Expert Review of Ophthalmology From 2005 Regenerative Medicine 2002 – 2006 British Journal of Ophthalmology **(iii) International Societies:** From 2011 Board member European Society of Gene and Cell Therapy 2008 - 2011 UK Academy of Medical Sciences - Sectional Committee (Biochemistry, Molecular Biology, Immunology, Microbiology, Genetics and Developmental Biology) **(iv) Prize Committees:** 2012: Association for Research on Vision and Ophthalmology (ARVO) – selection committee for Merk Innovative Ophthalmology Research Awards From 2011: Foukes Foundation Medal Committee 2010: Sir Jules Thorn Charitable Trust Biomedical Research Award Committee From 2009: Worshipful Company of Spectacle Makers (Ruskell Medal) **(v) International Meeting Committees:** 2010- 2016 American Society of Gene and Cell Therapy – Neurologic and Ophthalmic Gene and Cell Therapy Committee From 2007 British Society for Gene and Cell Therapy – Program Organisation Committee From 2007 European Society of Gene and Cell Therapy – Scientific Committee on Genetic and Metabolic Diseases

Honours/Prizes/Awards

2016	Election to Royal Society of Biology
2014	Prevention of Blindness Shield, Saudi Ophthalmology Society
2013	European Research Council – Advanced Award
2010	Association for Research into Vision and Ophthalmology/Pfizer Carl Camras Translational Research Award (\$10k unrestricted prize)
2009	Alcon Research Institute Award (\$200k unrestricted prize)
2009	Elected Senior Investigator, National Institute for Health Research
2009	Foundation Fighting Blindness (FFB) Board of Directors Award
2007	Foulkes Foundation Medal (Academy of Medical Sciences)
2007	Elected to UK Academy of Medical Sciences
2005	Jesse Mole Medal (British Retinitis Pigmentosa Society)
2004	Fincham Medal (The Worshipful Company of Spectacle Makers)
2001	GlaxoSmithKline Award for Medical Research
2001	The Sir Jules Thorn Award for Biomedical Research
1988	EMBO Molecular Biology Fellowship (Rome)

KeyNote and Named Lectures

2016 Brian Harcott Lecture – University of Leeds **2013** Steve Podos Colloquium Lecture, Alcon Research Institute, Seattle, JD Allen Lecture, University of Ottawa, Canada **2012** Pascol Rol Keynote Lecture, SPIE XXII Ophthalmic Technologies Conference, San Francisco **2011** Asia Association for Vision Research and Ophthalmology, Singapore; 18th International Chromosome Conference, Manchester, UK; COBRE Distinguished Vision Lecture, University of Oklahoma **2010** Elsemay Bjorn Lecture (Finnish Association for Eye Research), Tampere, Finland; Annual Congress of Japanese Ophthalmology, Kobe, Japan; Gordon Research Conference on Visual System Development, Barga Italy **2008** Cole Eye Distinguished Lecture, Cleveland Clinic, Ohio **2005** Jesse Mole Lecture, University of Oxford **2006** British Association of Optometry Annual Meeting **2004** Fincham Lecture, City University of London.

Engagement with policymakers

I provide gene and cell therapy input regularly for a wide range of policy forums, including UK Research Councils/Government Departments/Academy of Medical Sciences/ MHRA. Some recent examples of engagement with policymakers include: **2012** - Oral evidence to House of Lords Science and Technology Select Committee (enquiry on regenerative medicine); **2012** - Chair for Cross Council Workshop on New Approaches to Improving Hearing Devices (MRC and EPSRC). Will report 2013; **2012** - Organising Committee, Joint MHRA-MRC-Academy Medical Sciences - ABPI workshops to develop regulatory framework for regenerative medicine involving cell transplantation; **2012** - Participant, Joint Academies workshop on 'Human enhancement and the future of work' (Royal Society, Royal Academy of Engineering, Academy Medical Sciences, British Academy)- provided input on prospects for enhancing vision; **2012** – Participant 'New Strategy for UK Regenerative Medicine' On behalf of the 4 UK Research Council plus Technology Strategy Board - provided MRC input on gene and cell therapy; **2010** - Participant, 'Technology and Innovation Futures Project Workshop'. Commissioned by Government Office for Science. Participants from 4 UK Research Councils, industry, government - provided gene and cell therapy input. Report published 2011

Current grants

Biotechnology and Biological Sciences Research Council

* Regulation of epithelial apical membrane differentiation and function (Matter, Balda, Ali) 01/2014 -12/2017

European Research Council

* Generation of stem cell derived photoreceptors for the treatment and modelling of retinal degeneration (Ali) 08/2013 -07/2018

National Institute for Health Research

* NIHR Senior Investigator Award (Ali) 04/2014 - 03/2018
* NIHR Biomedical Research Centre at Moorfields Eye Hospital Support for Gene Therapy Theme (Ali) 04/2017 - 03/2022

RP Fighting Blindness

- * Maintaining effective antioxidant capacity in a degenerating retina: a genetic approach to treatment in (Ali, Wright, Murphy) 01/2014 - 07/2017
- * Centre for the Development of Gene Therapy for Inherited Retinal Dystrophies (Ali, Bainbridge, Michaelides) 08/2011 - 01/2017

Special Trustees Moorfields Eye Hospital

- * Refurbishment of human embryonic stem cell facility (Ali) 07/2016 - 12/2016
- * Retinal repair for Stargardt's macular degeneration (Ali, Bainbridge) 06/2016 - 04/2019

The European Union

- * BATCURE – Developing new therapies for Batten disease (Ali, Mole) 01/2016 - 12/2018

The Medical Research Council

- * Using cell surface antigens for the isolation of human photoreceptor precursor cells for retinal stem cell therapy (Sowden, Ali) 10/2015 - 09/2018
- * A flow cytometry facility for ocular regenerative medicine (Ali, Bainbridge, Coffey, Daniels, Khaw, Limb, and Pearson) 04/2014 - 03/2019
- * Clinical trial of gene therapy for the treatment of Leber congenital amaurosis (Ali, Bainbridge, and Michaelides) 03/2015 - 02/2020
- * UKRMP Partnership Project Award – Microparticles for cell and drug delivery (Ali) 01/2014 - 11/2017
- * UKRMP Immunomodulation Hub – Overcoming immunological barriers to regenerative medicine (Ali) 09/2014 - 08/2018
- * Optimisation of human ESC-derived photoreceptor cell differentiation (Ali) 12/2014 - 11/2018
- * Clinical trial of gene therapy for the treatment of achromatopsia (Ali, Bainbridge, Michaelides) 10/2013 - 09/2018
- * Development of stem cell therapy to restore photopic vision (Ali, Sowden, Pearson) 01/2012 - 12/2016

The Wellcome Trust

- * Development of an AAV vector treatment of Batten disease caused by mutations in TPP1 (CLN2 Disease) (Mole and Ali) 10/2016 - 03/2018
- * Retina Patient Days – Engaging patients with gene and cell therapy research (Ali, Bainbridge, and Morrison) 05/2012 - 10/2016
- * Equipment Grant: High resolution retinal imaging (Rhurberg, Ali, Cheetham, Cordiero, Greenwood, Matter, Michaelides, Moss, Rees, and Webster) 10/2012 - 09/2017

Selected Publications from >170 peer reviewed publications since 1996; H-index of 60.

1) Donor and host photoreceptors engage in material transfer following transplantation of post-mitotic photoreceptor precursors. Pearson,R.A., Gonzalez-Cordero,A., West,E.L., Ribeiro,J.R., Aghaizu,N., Goh,D., Sampson,R.D., Georgiadis,A., Waldron,P.V., Duran,Y., Naeem,A., Kloc,M., Cristante,E., Kruczek,K., Warre-Cornish,K., Sowden,J.C., Smith,A.J., and Ali,R.R. **Nat. Commun.** 2016 **7**: 13029

2) Development of an optimized AAV2/5 gene therapy vector for Leber congenital amaurosis owing to defects in RPE65.Georgiadis A, Duran Y, Ribeiro J, Abelleira-Hervas L, Robbie SJ, Sunkel-Laing B, Fourali S, Gonzalez-Cordero A, Cristante E, Michaelides M, Bainbridge JW, Smith AJ, Ali RR. **Gene Ther.** 2016 **23**: 857-862

3) 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, Gardner PJ, Feathers KL, Luong VA, Yzer S, Balaggan K,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

4) Transplantation of Photoreceptor Precursors Isolated via a Cell Surface Biomarker Panel From Embryonic Stem Cell-Derived Self-Forming Retina. Lakowski J, Gonzalez-Cordero A, West EL, Han YT, Welby E, Naeem A, Blackford SJ, Bainbridge JW, Pearson RA, Ali RR, Sowden JC. **Stem Cells.** 2015 **33**(8):2469-82.

- 5) 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.
- 6) Effective transplantation of photoreceptors isolated from three-dimensional cultures of embryonic stem cell-derived retina. Gonzalez-Cordero A, West EL, Pearson RA, Duran Y, Carvalho 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
- 7) Repair of the degenerate retina by photoreceptor transplantation. Barber AC, Hippert C, Duran Y, West EL, Bainbridge JWB, Warre-Cornish K, Luhmann UF, Lakowski J, Sowden JC, Ali RR and Pearson RA. **Proc Natl Acad Sci** 2013 **110** (1):354-359.
- 8) 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
- 9) Defining the integration capacity of embryonic stem cell-derived photoreceptor precursors. EL West, A Gonzalez-Cordero, RA Pearson, JC Sowden, M Takahashi, RR Ali **Stem Cells** 2012; **30** (7):1424-35
- 10) Effective transplantation of photoreceptor precursor cells selected via cell surface antigen expression. J Lakowski, YT Han, RA Pearson, A Gonzalez-Cordero, EL West, S Gualdoni, AC Barber, M Hubank, RR Ali and JC Sowden **Stem Cells**. 2011; **29** (9): 1391-404
- 11) Long-term and age-dependent restoration of visual function in a mouse model of CNGB3-associated achromatopsia following gene therapy. LS Carvalho, J Xu, RA Pearson, AJ Smith, JW Bainbridge, LM Morries, SJ Fliesler, XQ Ding and RR Ali. **Human Molecular Genetics** 2011; **20** (16): 3161-75
- 12) Long-term preservation of cones and improvement in visual function following gene therapy in a mouse model of Leber congenital amaurosis (LCA) caused by GC1 deficiency. M Mihelec, RA Pearson, SJ Robbie, PK Buch, WB Bainbridge, AJ Smith and RR Ali **Human Gene Therapy** 2011; **22** (10): 1179-90
- 13) Long term survival of photoreceptors transplanted into the adult murine neural retina requires immune modulation. EL West, RA Pearson, SE Barker, UF Luhmann, RE MacLaren, AC Barber, Y Duran, AJ Smith, JC Sowden and RR Ali. **Stem Cells** 2010; **28** (11): 1997-2007
- 14) Assessment of ocular transduction using single stranded and self-complementary recombinant adeno-associated virus serotype 2/8. M Natkunaraja, P Trittibach, J McIntosh, Y Duran, SE Barker, AJ Smith, AC Nathwani and RR Ali **Gene Ther.** 2008; **15** (6): 463-7
- 15) Gene therapy for retinitis pigmentosa and Leber congenital amaurosis caused by defects in AIPL1: effective rescue of mouse models of partial and complete Aipl1 deficiency using AAV2/8 vectors. MH Tan, AJ Smith, B Pawlyk, X Xu, X Liu, JW Bainbridge, M Basche, J McIntosh, HV Tran, A Nathanwi, T Li and RR Ali **Human Molecular Genetics** 2009; **18** (12): 2099-114
- 16) Effect of gene therapy on visual function in Leber Congenital Amaurosis. JWB Bainbridge, AJ Smith, SE Barker, S Robbie, R Henderson, KS Balaggan, A Viswanathan, GE Holder, A Stockman, N Tyler, S Petersen-Jones, SS Bhattacharya, AJ Thrasher, FW Fitzke, GS Rubin, AT Moore and RR Ali **New England Journal of Medicine** 2008; **358** (21): 2231-9
- 17) Effective gene therapy with non-integrating lentiviral vectors. RJ Yáñez-Muñoz, KS Balaggan, A MacNeil, S Howe, M Schmidt, AJ Smith, P Buch, RE MacLaren, PN Anderson, SE Barker, Y Durán, C Bartholomae, C Von Kalle, JR Heckenlively, C Kinnon, RR Ali and AJ Thrasher **Nature Medicine** 2006; **12** (3) :348-353
- 18) Retinal repair by transplantation of photoreceptor precursors. RE MacLaren, RA Pearson, A MacNeil, RH Douglas, TE Salt, M Akimoto, A Swaroop, JC Sowden and RR Ali **Nature** 2006; **444** (7116): 203-207
- 19) AAV-mediated gene transfer slows photoreceptor loss in the RCS rat model of retinitis pigmentosa. AJ Smith, FC Schlichtenbrede, JW Bainbridge, AJ Thrasher and RR Ali **Molecular Therapy** 2003; **8** (2): 118-195
- 20) Restoration of photoreceptor ultrastructure and function in retinal degeneration slow mice by gene therapy. RR Ali, G Sarra, C Stephens, M de Alwis, JWB Bainbridge, PM Munro, S Fauser, MB Reichel, C Kinnon, DM Hunt, SS Bhattacharya and AJ Thrasher **Nature Genetics** 2000; **25** (3): 306-310