

# Research Funding provided by Choroideremia Research Foundation



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Funded	Researcher Name	Institution	Project Title	USD \$
2002	Miguel Seabra, MD, PhD, Professor, CEDOC, Chronic Diseases Research Center	Nova Medical School, University of Lisbon, Portugal	Choroideremia Research Lab Supplies	1,500
2003	Miguel Seabra, MD, PhD, Professor, CEDOC, Chronic Diseases Research Center	Nova Medical School, University of Lisbon, Portugal	Development of CHM Mouse Model	14,500
2004	Miguel Seabra, MD, PhD, Professor, CEDOC, Chronic Diseases Research Center	Nova Medical School, University of Lisbon, Portugal	Generation of CHM Viral Vector, pt. 1	20,550
2005	Kirill Alexandrov, PhD	Max Planck Institute, Germany	Forced Expression of REP2 to the Retina	13,000
2005	Miguel Seabra, MD, PhD, Professor, CEDOC, Chronic Diseases Research Center	Nova Medical School, University of Lisbon, Portugal	Generation of CHM Viral Vector, pt. 2	50,000
2006	Miguel Seabra, MD, PhD, Professor, CEDOC, Chronic Diseases Research Center	Nova Medical School, University of Lisbon, Portugal	Preclinical Gene Therapy Study Year 1	80,460
2007	Miguel Seabra, MD, PhD, Professor, CEDOC, Chronic Diseases Research Center	Nova Medical School, University of Lisbon, Portugal	Preclinical Gene Therapy Study Year 2	69,880
2010	Jean Bennett, MD, PhD, F.M. Kirby Professor of Ophthalmology	Scheie Eye Institute, Perelman School of Medicine, University of Pennsylvania, Philadelphia, PA	Mouse Study Testing for Three Viral Vector Candidates	100,000
2011	Jean Bennett, MD, PhD, F.M. Kirby Professor of Ophthalmology	Scheie Eye Institute, Perelman School of Medicine, University of Pennsylvania, Philadelphia, PA	Alternative In-Vitro Assay to Evaluate Three Viral Vector Candidates	75,000
2011	Miguel Seabra, MD, PhD, Professor, CEDOC, Chronic Diseases Research Center	Nova Medical School, University of Lisbon, Portugal	Pre-Clinical Gene Therapy Study Year 3	90,000
2012	Jean Bennett, MD, PhD, F.M. Kirby Professor of Ophthalmology	Scheie Eye Institute, Perelman School of Medicine, University of Pennsylvania, Philadelphia, PA	Purchase of MP-1 Nidek digital retinal microperimeter equipment	57,000
2012	Mariya Moosajee, MBBS, BSc (Hons), PhD, FRCOphth, Consultant Ophthalmic Surgeon and Clinical Academic Ophthalmologist	University College, London, UK	Ataluren to Treat Nonsense-Mediated Choroideremia; Evaluate 6 Readthrough Compounds on Zebrafish and iPS Derived CHM Cell Lines, Fibroblast, RPE with Dr. Kalatzis; Grant 1	88,864
2012	Jean Bennett, MD, PhD, F.M. Kirby Professor of Ophthalmology	Scheie Eye Institute, Perelman School of Medicine, University of Pennsylvania	First Generation Gene Therapy in Collaboration with Spark Therapeutics, pt. 1	75,000
2013	Miguel Seabra, MD, PhD, Professor, CEDOC, Chronic Diseases Research Center	Nova Medical School, University of Lisbon, Portugal	Pre-Clinical Gene Therapy Studies pt. 2	90,000
2013	Mariya Moosajee, MBBS, BSc (Hons), PhD, FRCOphth, Consultant Ophthalmic Surgeon and Clinical Academic Ophthalmologist	University College, London, UK	Ataluren to Treat Nonsense-Mediated Choroideremia; Evaluate 6 Readthrough Compounds on Zebrafish and iPS Derived CHM Cell Lines, Fibroblast, RPE with Dr. Kalatzis; Grant 2	88,864
2013	Jean Bennett, MD, PhD, F.M. Kirby Professor of Ophthalmology	Scheie Eye Institute, Perelman School of Medicine, University of Pennsylvania, Philadelphia, PA	First Generation Gene Therapy in Collaboration with Spark Therapeutics, pt.2	75,000

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2013	Vasiliki Kalatzis, PhD, Human Genetics, HDR Life Sciences	Institute for Neurosciences of Montpellier, INSERM, France	Pre-Clinical Gene Therapy Studies for Choroideremia Using a Human Cellular Model: Differentiation of Patient iPS Cells into Retinal Cells, pt. 2	49,000
2013	David Gamm, MD, PhD, Director, McPherson Eye Research Institute, Associate Professor, Ophthalmology and Visual Sciences	University of Wisconsin, Madison, WI	Microscope and Laboratory Equipment for Choroideremia Research	29,250
2013	Ian MacDonald, BsC, PhD, Professor of Metabolic Physiology, Faculty of Medicine & Health Sciences	University of Nottingham, UK	An Open Label Clinical Trial of Retinal Gene Therapy for Choroideremia	22,500
2014	Mariya Moosajee, MBBS, BsC (Hons), PhD, FRCOphth, Consultant Ophthalmic Surgeon and Clinical Academic Ophthalmologist	University College, London, UK	Ataluren to Treat Nonsense-Mediated Choroideremia; Evaluate 6 Readthrough Compounds on Zebrafish and iPS Derived CHM Cell Lines, Fibroblast, RPE with Dr. Kalatzis; Grant 3	81,314
2014	Mariya Moosajee, MBBS, BsC (Hons), PhD, FRCOphth, Consultant Ophthalmic Surgeon and Clinical Academic Ophthalmologist	University College, London, UK	Ataluren to Treat Nonsense-Mediated Choroideremia; Evaluate 6 Readthrough Compounds on Zebrafish and iPS Derived CHM Cell Lines, Fibroblast, RPE with Dr. Kalatzis; Grant 4	80,846
2014	Vasiliki Kalatzis, PhD, Human Genetics, HDR Life Sciences	Institute for Neurosciences of Montpellier, INSERM, France	Pre-Clinical Gene Therapy Studies for Choroideremia Using a Human Cellular Model: Differentiation of Patient iPS Cells into Retinal Cells, pt. 1	43,700
2014	David Gamm, MD, PhD, Director, McPherson Eye Research Institute, Associate Professor, Ophthalmology and Visual Sciences	Waisman Center, University of Wisconsin, Madison, WI	The Potential Role of hiPSCs in the Treatment of Choroideremia	55,122
2015	Jean Bennett, MD, PhD, F.M. Kirby Professor of Ophthalmology	Scheie Eye Institute, Perelman School of Medicine, University of Pennsylvania	Multi-Focal ERG/Visual Evoked Potentials Machine	34,212
2015	Mark Pennesi, MD, PhD Assistant Professor in Ophthalmic Genetics	Oregon Health and Science University, Portland, OR	Exploring the Potential of OCT Angiography to Monitor Progression in Choroideremia	64,350
2015	Miguel Seabra, MD, PhD, Professor, CEDOC, Chronic Diseases Research Center	Nova Medical School, University of Lisbon, Portugal	Direct Reprogramming of Fibroblasts into Functional RPE Cells by Specific Transcription Factors	20,000
2015	n/a	n/a	Fight for Sight Partnership - Post-Doctoral Award	23,750
2015	Mariya Moosajee, MBBS, BsC (Hons), PhD, FRCOphth, Consultant Ophthalmic Surgeon and Clinical Academic Ophthalmologist	University College, London, UK	Whole Organism Screening for Protective/Regenerative Drug Therapeutics in the CHM Zebrafish Model	66,471
2015	Jeffrey S. Mumm, PhD, Helen Larson & Charles Glenn Grover Professor in Ophthalmology, Associate Professor of Ophthalmology	Wilmer Eye Institute, Johns Hopkins Medicine, Baltimore, MD	Whole Organism Screening for Protective/Regenerative Drug Therapeutics in the CHM Zebrafish Model	83,527
2015	Gerald Luttj, PhD, Director, Ocular Vasculogenesis and Angiogenesis Laboratory; Professor of Ophthalmology	Wilmer Eye Institute, Johns Hopkins Medicine, Baltimore, MD	Production and Testing of CHM hiPSC-Derived Retinal and Vascular Cells (part 1)	100,000

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2015	David Gamm, MD, PhD, Director, McPherson Eye Research Institute; Associate Professor, Ophthalmology and Visual Sciences	Waisman Center, University of Wisconsin, Madison, WI	Establishment of CHM Biobank	10,050
2015	David Gamm, MD, PhD, Director, McPherson Eye Research Institute; Associate Professor, Ophthalmology and Visual Sciences	Waisman Center, University of Wisconsin, Madison, WI	Production and Testing of CHM hiPSC-Derived Retinal and Vascular Cells (part 2)	100,000
2015	n/a	4D Molecular Therapeutics, Emeryville, CA	Development of AAV Capsid Variants with Enhanced Panretinal Gene Delivery of the REP-1 Transgene for the Treatment of Choroideremia	500,000
2016	Edwin Stone, MD, PhD, Seamans-Hauser Chair in Molecular Ophthalmology; Director, Molecular Ophthalmology Laboratory; Director, Carver Family Center for Macular Degeneration; Director, Carver Nonprofit Genetic Testing Laboratory; Director, Institute for Vision	University of Iowa Foundation, Iowa City, IA	Project CHM Genotyping Program (part 2) - Funded in Conjunction with PTC Therapeutics	55,000
2016	Robert MacLaren, MB, ChB, Dphi, FRCOphth, FRCS, FACS, FMedSci, Professor of Ophthalmology	University of Oxford, UK	OPI Lumera OCT Microscope Equipment Purchase	47,000
2016	Mariya Moosajee, MBBS, BSc (Hons), PhD, FRCOphth, Consultant Ophthalmic Surgeon and Clinical Academic Ophthalmologist	University College, London, UK	Whole Organism Screening for Protective/Regenerative Drug Therapeutics in the CHM Zebrafish Model; grant 2	28,606
2016	Mariya Moosajee, MBBS, BSc (Hons), PhD, FRCOphth, Consultant Ophthalmic Surgeon and Clinical Academic Ophthalmologist	University College, London, UK	Freezer for CHM Research Samples	5,788
2016	Mariya Moosajee, MBBS, BSc (Hons), PhD, FRCOphth, Consultant Ophthalmic Surgeon and Clinical Academic Ophthalmologist	University College, London, UK	Investigating the Degenerating Choroid in Choroideremia	100,934
2017	Michael Young, PhD, FARVO, Co-Director, Ocular Regenerative Medicine Institute and Director, Minda de Gunzburg Center for Retinal Regeneration	Schepens Eye Institute, Mass General Boston; Department of Ophthalmology, Harvard Medical School, Boston, MA	Localized Gene Delivery Through Suprachoroidal Space Using a Novel Autostop Needle	25,000
2018	David Gamm, MD, PhD, Director, McPherson Eye Research Institute; Associate Professor, Ophthalmology and Visual Sciences	Waisman Center, University of Wisconsin, Madison, WI	Year 1: Elucidating the Function of REP1 in Human Pluripotent Stem Cell-Derived RPE and Photoreceptor cells	50,000 co-funded with CRF Canada
2018	David Gamm, MD, PhD, Director, McPherson Eye Research Institute; Associate Professor, Ophthalmology and Visual Sciences	Waisman Center, University of Wisconsin, Madison, WI	Determining the Downstream Consequences of Endogenous REP1 Activity in Human RPE and Photoreceptor cells	40,000
2018	Miguel Seabra, MD, PhD, Professor, CEDOC, Chronic Diseases Research Center	Nova Medical School, University of Lisbon, Portugal	How CHM Defect Affects Cross Talk Between Organelles and Cellular Functions such as Mitochondria, Lysosome, Autophagy, and Proteostasis	107,823
2018	Keirnan Willett, MD, Department of Ophthalmology	University of Pennsylvania, Philadelphia, PA	Vascular Biomarkers in Retinal Gene Therapy for Leber Congenital Amaurosis and Choroideremia	22,500 co-funded with Fight for Sight

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2018	Jason A. Mills, PhD, Research Investigator and Kathleen Boesze-Battaglia, PhD, Professor of Biochemistry and Biophysics	MDBR, Orphan Disease Center, University of Pennsylvania, Philadelphia, PA	Targeting Phagosome Maturation to Restore Dysfunctional Retinal Pigmented Epithelium in CHM	100,000 co-funded with UPENN MDBR
2019	David Gamm, MD, PhD, Director, McPherson Eye Research Institute; Associate Professor, Ophthalmology and Visual Sciences	Waisman Center, University of Wisconsin, Madison, WI	Year 2: Elucidating the Function of REP1 in Human Pluripotent Stem Cell-Derived RPE and Photoreceptor Cells	75,000
2019	Miguel Seabra, MD, PhD, Professor, CEDOC, Chronic Diseases Research Center	Nova Medical School, University of Lisbon, Portugal	Mechanisms in Cell Death in Choroideremia	101,983
2020	Katrina Stingl, MD, Ophthalmologist, Clinical Scientist	University Eye Hospital, Tübingen, Germany	Adaptive Optics Imaging in Follow-Ups of Choroideremia Patients after Gene Therapy	61,079 co-funded with UPENN ODC MDBR
2020	Richard Harbottle, PhD, Group Leader, DNA Vector Group Leader	German Cancer Research Centre, DKFZ, Heidelberg, Germany	Autonomously Replicating DNA Nanovectors for Gene and Cell Therapy of Choroideremia	45,238
2020	David Williams, PhD, Professor in Residence, Ophthalmology	University of California, Los Angeles, CA	Understanding Mitochondrial Defects in Choroideremia	61,079
2020	Kim Edwards, Graduate Student	University of Wisconsin, McPherson Eye Research Institute, Madison, WI	RANDY WHEELLOCK RESEARCH AWARD WINNER: Identifying the Function of REP-1 Protein in Retina (RPE/Photoreceptors) and Non-Retina Tissues	50,000
2020	David Gamm, MD, PhD, Director, McPherson Eye Research Institute; Associate Professor, Ophthalmology and Visual Sciences	University of Wisconsin, McPherson Eye Research Institute, Madison, WI	Randy Wheelock Research Award Budget Supplement	30,000
2020	Abigail Fahim, MD, PhD, Clinical Assistant Professor, Ophthalmology and Visual Sciences	Kellogg Eye Center, University of Michigan, Ann Arbor, MI	Investigating Choroideremia Pathophysiology using iPSC-derived Retinal Pigment Epithelium	59,459 co-funded with CRF Canada
2020	Stacey Hume, PhD, FCCMG, Associate Professor, Department of Medical Genetics	University of Alberta, Canada	BOREN FAMILY RESEARCH AWARD: Identifying the Cause of a Discordant Phenotype in Two Brothers with the Identical CHM Mutation	45,422 co-funded with CRF Canada
2020	Yi (Fay) Zhai, MD, PhD, Clinical Research Fellow, Department of Ophthalmology	University of Alberta, Canada	OSTER FAMILY RESEARCH AWARD: Measuring the En Face Ellipsoid Zone (EZ) Area as a Biomarker of Photoreceptor Structure/Function in Choroideremia	28,145 co-funded with CRF Canada