BIOGRAPHICAL SKETCH

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NAME: **McCall, Maureen A**

eRA COMMONS USERNAME (credential, e.g., agency login): mamcca01

POSITION TITLE: Professor

EDUCATION/TRAINING *(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable. Add/delete rows as necessary.)*

INSTITUTION AND LOCATION	DEGREE *(if applicable)*	Completion Date MM/YYYY	FIELD OF STUDY
University of Maryland, College Park	B.S.	1974	Psychology
University of Maryland, College Park	M.S.	1977	Visual Psychophysics
SUNY, Albany, Albany , NY	Ph.D.	1983	Neurobiology

A. Personal Statement

My research goals are to characterize and compare elements of the normal retinal circuit and its output with those same elements in diseased retinas. To date, we study wild type animals and compare them to mutant animals that are models for human retinal disease including retinitis pigmentosa and congenital stationary night blindness. My expertise and strong history in the development and function of the visual system in general, and the retina in particular make me extremely qualified to carry out this research. I am a trained neurophysiologist with expertise in molecular biology and anatomical characterizations of the central nervous system. I trained as a graduate student/post-doc in electrophysiological assays of visual responses of cells in the visual pathway *in vivo* and I studied molecular biological techniques to manipulate the mouse genome in order to create selective changes in retinal receptor function. In the past 12 years, I have added to new electrophysiological techniques to enhance our assessment off changes in function at multiple levels: whole cell voltage clamp techniques, to assess changes in excitatory vs inhibitory currents in retinal cell function *in vitro;* multielectrode array recordings to assay ganglion cell function over a broad retinal area and multiple cell classes simultaneously; two photon microscopy to target particular cell classes for functional assessment and full field and multi-focal ERGs to non-invasively define the development and decline of retinal function in animal models of retinal disease. I have also incorporated the use of viral vectors for knockdown of gene expression and for gene therapy. I have directed a successful extramurally funded research lab for over 20 years. My publication record and my standing in the vision community are a testament to my past and future success and have prepared me to lead this project.

Ongoing and pending projects that I would like to highlight include:

R01EY029719-02
(McCall, MPI:Gregg)
3/1/19 – 2/28/2024
NIH/NEI
Glycine subunit specific inhibition and ganglion cell visual responses
The major goals of this project are to define the role of glycine receptor alpha subunits in mediating visual signaling in the retinal ganglion cells of the mouse.

R01EY12354-19
(Gregg, MPI)
2/1/2018-1/31/2023
GREGG, RONALD G (PD/PI)
Isolation of Congenital Stationary Night Blindness Genes

The goal of this project was to identify and characterize the function of genes required for the function of photoreceptor to ON bipolar cell synapse

Precision Biosciences, Inc.
(McCall)
2018 – 12/31/27
Gene Therapy using a Homing Endonuclease to restore visual function in rod photoreceptors in P23H rhodopsin autosomal dominant retinitis pigmentosa
The major goals of this project are to determine the ability of a homing endonuclease specific for the rhodopsin P23H mutation to alter rod photoreceptor degeneration in a pig model of human P23H rhodopsin, retinitis pigmentosa

Wave Life Sciences
(McCall)
7/2019 – 12/31/2021
Sterospecific oligonucleotides as a treatment for retinitis pigmentosa degeneration in a pig model of human P23H rhodopsin, retinitis pigmentosa
The major goals of this project are to determine the ability of sterospecific oligonucleotides specific for the rhodopsin P23H mutation to alter mutant rhodopsin expression and prevent rod photoreceptor degeneration in a pig model of human P23H rhodopsin, retinitis pigmentosa

Rznomics, Inc
(McCall)
2/2021 – 4/2023
Efficacy of a new RNA editing approach in retinitis pigmentosa
The major goals of this project are to determine the ability of a novel RNA editing approach specific for the rhodopsin P23H mutation to alter mutant rhodopsin expression and prevent rod photoreceptor degeneration in a pig model of human P23H rhodopsin, retinitis pigmentosa

Foundation for Fighting Blindness
(McCall)
7/1/2020 – 6/30/2022
Creation of new models of inherited retinal disease in swine
The goals of this project are to create a swine model of Stargardt's disease.

Foundation for Fighting Blindness
(coPIs McCall/Dinculescu)
7/1/2021 – 6/30/2023
Generation and Characterization of Novel Large Animal Model of Usher Syndrome Type 3
The goals of this project are to create a swine model of Stargardt's disease.

Foundation for Fighting Blindness
(McCall)
9/1/2021 – 8/31/2023
Defining the isoforms of CRBI in the pig retina
The goals of this project are to determine the isoforms of CRB1 present in the developing pig retina and compare them to human and mouse.

Pending

NIH
McCall, Gregg, Gamm; MPI
submitted 2/2022
Taking Rho1-2 to the clinic (received 2nd percentile)
The goals of this project are to determine if a meganuclease based gene-editing approach has efficacy, specificity and safety for treating the P23H hRHO mutation that causes adRP in a pig model.

FFB
(McCall, Schmidt; MPI)
submitted 2021
Awaiting, reviewed, awaiting notice of grant award (expected 8/2022)
Testing the efficacy of RT011, a deuteated form of DHA, as a mutation independent therapy in RP
The goals of this project are to determine if a dietary supplement has efficacy in delaying or arresting rod and cone photoreceptors from degeneration in mouse and pig models of adRP (P23H Rho mutation).

NIH
McCall, M.A.
submitted 6/2022 (R21)
Wave1, a novel stereopure ASO to treat P23H autosomal dominant retinitis pigmentosa
The goals of this project are to determine if an antisense oligonucleotide treatment can delay the progressive decline in retinal function an adRP pig model and evaluate the efficacy and durability of the rescue.

B. Positions and Honors

Professional Experience (Chronological order)

2021 –	present Member Scientific Advisory Board, Choroideremia Foundation
2019	present Vice Chair for Research, Department of Ophthalmology & Visual Sciences, UofL
2017 – 2022	Trustee, Association for Research in Vision and Ophthalmology
2015-present	Fellow Association for Research in Vision and Ophthalmology
2015 – 2017	Atlantic Coast Conference, Distinguished Lecturer
2014-present	Kentucky Lions Eye Research Endowed Chair
2014 – 2016	Chair, Neurotransmitters, Receptors & Calcium Signaling Study Section, NIH
2012 – 2013	Permanent Member, Neurotransmitters, Receptors & Calcium Signaling Study Section, NIH
2010/2012	Co-Organizer/Organizer FASEB Meeting on Retinal Neurobiology
2008	Short Course Organizer "Retinal ganglion cells in model organisms: development, function and disease" – Sponsored by the *Journal of Physiology (London)* ARVO Annual Meeting.
2007-present	Professor, Departments of: Ophthalmology & Visual Sciences, (Joint appt in: Anatomical Science & Neurobiology) Univ Louisville, Louisville, KY
2007 -	Member, Editorial Board Visual Neuroscience
2006 – 2009	Member/Chair (2009) - ARVO Program Committee (Visual Neuroscience Section)
2005 – 2009	Permanent Member, Biological Diseases of the Posterior Eye Study Section NIH
2004, 2006	Co-Organizer, The Laboratory Mouse in Vision Research, The Jackson Lab, Bar Harbor, ME
2004 – 2008	Member, Fight for Sight Review Panel
2004 – 2006	Ad Hoc Reviewer, Visual System SBIR Study Section NIH
1997-2007	Assist/Assoc Professor, Departments of: Psychological & Brain Sciences and Ophthalmology & Visua Sciences, Univ Louisville, Louisville, KY
1988-1996	Assist/Assoc Scientist, Waisman Center on Mental Retardation and Human Development, Univ Wisconsin, Madison, WI
1983-1988	Postdoctoral Trainee, Univ Wisconsin, Madison
1979–present	Member, Society for Neuroscience
1977–present	Member, Association Research for Vision & Ophthalmology
1977-1983	Research/Teaching Assist, SUNY at Albany, Center for Neurobiology, Albany, NY
1975-1977	Research/Teaching Assist, Univ Maryland, Visual Psychophysics Program, College Park, MD

C. Contributions to Science

1. Development and characterization of the natural history of swine models of retinal disease and therapeutic strategies to alter disease progression. There are many animal models of retinal disease. Those that recapitulate the human disease are of most use for examining therapeutic strategies to alter

disease progression. Large animal models are particularly useful for helping to define clinical outcomes. In particular the pig retina share many features in common with humans. As there were no pig models of adRP, we partnered with the National Swine Research Resource Center to create a transgenic pig that harbored the P23H hRHO mutation. We have studied the natural history of disease progression, which compares favorable to human and begun to use the model in the study of therapeutic structural and functional efficacy.

Fernandez de Castro,JP; Scott, PA; Fransen, JW; Demas, J; DeMarco, P.J; Kaplan, HJ; **McCall, MA** Cone Photoreceptors Develop Normally in the Absence of Functional Rod Photoreceptors in a Transgenic Swine Model of Retinitis Pigmentosa Investigative Ophthalmology & Visual Sciences 2014 Apr 17;55(4):2460

Huckenpahler, A.L., Carroll,J., Salmon, A.E., Sajdak, B.S., Mastey, R.R., Allen, K.P., Kaplan, H.J., **McCall, M.A.** Non-invasive imaging and correlative histology of cone photoreceptor structure in the pig retina. TVST, (2019) 8(6) article 36.

Chan YK, Wang SK, Chu CJ, Copland DA, Letizia AJ, Costa Verdera H, Chiang JJ, Sethi M, Wang MK, Neidermyer WJ Jr, Chan Y, Lim ET, Graveline AR, Sanchez M, Boyd RF, Vihtelic TS, Inciong RGCO, Slain JM, Alphonse PJ, Xue Y, Robinson-McCarthy LR, Tam JM, Jabbar MH, Sahu B, Adeniran JF, Muhuri M, Tai PWL, Xie J, Krause TB, Vernet A, Pezone M, Xiao R, Liu T, Wang W, Kaplan HJ, Gao G, Dick AD, Mingozzi F, **McCall MA**, Cepko CL, Church GM. Engineering adeno-associated viral vectors to evade innate immune and inflammatory responses 2021 Science, Translational Medicine (2021) Feb 10;13(580).

2. Electophysiological evaluation of changes in retinal visual processing in several animal models of retinal disease. In these collaborative experiment, we studied the impact of altered bipolar cell function on the retinal output, the retinal ganglion cell spiking response. Having established the deficits resulting from these mutations, we have begun to explore the application of gene therapy to restore normal function in mouse models of retinal disease.

Scalabrino ML, Boye SL, Fransen KM, Noel JM, Dyka FM, Min SH, Ruan Q, De Leeuw CN, Simpson EM, Gregg RG, **McCall MA**, Peachey NS, Boye SE. (2015) Intravitreal delivery of a novel AAV vector targets ON bipolar cells and restores visual function in a mouse model of complete congenital stationary night blindness. Human Molecular Genetics 24(2): 6229-39.

Peachey NS, Hasan N, FitzMaurice B, Burrill S, Pangeni G, Karst SY, Reinholdt L, Berry ML, Strobel M, Gregg RG, McCall MA, Chang B. (2017). A missense mutation in Grm6 reduces but does not eliminate mGluR6 expression or rod depolarizing bipolar cell function. J Neurophysiol. 118:845-854. PMID:28490646

Hasan, N., Pangeni, G., Ray, Fransen, K.H., Noel JM, Borghuis, B., McCall**, M.A**., Gregg, R.G. LRIT3 is required for Nyctalopin expression and normal ON and OFF pathway signaling in the retina eNeuro (2020).

3. **The work in my lab also has been instrumental in understanding whether retinal transplants or prosthetic devices can establish visual function in adRP rodent models**

Sagdullaev, B.T., Aramant, R.B., Seiler, M.J., Woch, G. & **McCall, M.A.** Retinal transplantation induces recovery of retino-tectal visual function in a rodent model of retinitis pigmentosa. Investigative Ophthalmology & Visual Science, 44(4):1686-95 (2003).

Fransen, JW; Pangeni,G; Pardue, MT; **McCall, MA** Local signaling from a retinal prosthetic device in a rodent *RP* model *in vivo* Journal of Neural Engineering, 2014 Aug;11(4):046012. doi: 10.1088/1741-2560/11/4/046012. Epub 2014 Jun 18.

Light, JG; Fransen JW; Adekunle, AN; Adkins, A; Pangeni, G; Loudin, J; Kathieson, K; Palanker, DV; **McCall, MA**; Pardue, MT. Inner retinal preservation in rat models of retinal degeneration implanted with subretinal photovoltaic arrays, Experimental Eye Research 2014 Nov;128:34-42.

Complete List of Published Work in My Bibliography:
https://www.ncbi.nlm.nih.gov/sites/myncbi/maureen.mccall.1/bibliography/45370720/public/?sort=date&direction=descending