CIRM Scientific and Medical Research Funding Working Group
Biographical information of candidates nominated to serve as
Alternate Scientific Members of the Working Group

Gyula Acsadi, M.D., Ph.D.
Dr. Acsadi is the Clinical Division Chief of Pediatric Neurology and Co-Director of the Muscular Dystrophy Association Clinic at The Children's Hospital of Michigan and Associate Professor in the Department of Pediatrics and Neurology at Wayne State University. He received his M.D. and completed a Neurophysiology Fellowship and a Residency in Pediatrics at the Medical University of Pecs, Hungary. He received his Ph.D. in Molecular Genetics from the Hungarian Academy of Sciences. He also completed a Residency in Pediatric Neurology at The Children's Hospital of Michigan. Dr. Acsadi has held the position of Visiting Scientist in the Department of Pediatrics in the Waisman Center at the University of Wisconsin-Madison where he studied gene therapy, and the position of Visiting Assistant Professor in the Neuromuscular Research program at the Montreal Neurological Institute at McGill University before moving to The Children's Hospital of Michigan. He is Board certified by the American Board of Psychiatry & Neurology as a neurologist with special qualifications in child neurology.

Throughout Dr. Acsadi's professional career, his clinical interest has been in pediatric neurological diseases in general but with main emphasis in researching and treating neuromuscular disease including muscular dystrophies and motor neuron diseases. This interest has led to his involvement in clinical trials for spinal muscular atrophy (SMA). He has participated in designing and conducting these trials and served as site PI in multicenter trials. Recently, he was responsible for putting together the "Pediatric Pilot Project" for the National Institutes of Health (NIH) Rare Diseases Clinical Research Consortia (RDCRC) sponsored "Inherited neuropathy consortium" grant.

During and shortly after completing medical school, Dr. Acsadi was interested in behavioral and electrophysiological studies of the limbic system. With the advancements of the molecular genetic era, he was involved in early gene therapy work for muscular dystrophy that led to a publication as first author in Nature. Subsequently, Dr. Acsadi's research interests have focused on viral and non-viral gene transfer in animal models for muscular dystrophy and amyotrophic lateral sclerosis (ALS). During the past 5-6 years, he has been working on the molecular mechanisms of disease process of spinal muscular atrophy (SMA) using cell culture model systems.

Dr. Acsadi is a member of the Child Neurology Society of USA and the American Academy of Neurology. He has been listed in Best Doctors in America and Top Doctors. He has published over 40 articles in peer-referred journals. Dr. Acsadi is active in scholarly review for granting agencies, including the NIH, and for scientific peer-refereed journals. His research has been supported by several funding

agencies including the NIH, the Muscular Dystrophy Association, Project Cure SMA, and Families of SMA.

Richard Sanford Finkel, M.D.
Dr. Finkel is Clinical Professor in Neurology and Pediatrics at the University of Pennsylvania School of Medicine. He holds several appointments at The Children's Hospital of Philadelphia (CHOP) including Attending Staff Physician in Neurology; Director of the Neuromuscular Program; Co-director of the Neurology Resident Training Program; and is a member of the Clinical Affairs and Compliance Committees in the Department of Pediatrics and of the IVIG Pharmacy Advisory and Non-USP Formulary Medication Use Committees. Additionally, he is Attending Staff Physician in Neurology and Electromyographer at the Hospital of the University of Pennsylvania. Dr. Finkel earned his B.A. in Chemistry at Washington and Jefferson College in Washington, Pennsylvania; his M.D. at Washington University in St. Louis; and completed an internship and residency in pediatrics at The Children's Hospital Boston and a neurology fellowship in the Harvard Longwood Program. He then completed neuromuscular training under Dr. Michael J. Bresnan at The Children's Hospital Boston and electromyography training under Dr. H. Royden Jones, Jr. (The Children's Hospital Boston and the Lahey Clinic) and Dr. Mark Hallett (Brigham and Women's Hospital).

Dr. Finkel serves as a neurology consultant to the Comprehensive Lysosomal Storage Disorders Center at CHOP. In this capacity he sees several patients per year with mucopolysaccharidoses: MPS-1, MPS-2, MPS-5 and MPS-6. He has participated in clinical trials in the lysosomal storage diseases, Fabry and Pompe disease, and in the ataluren studies for Duchenne muscular dystrophy. He has experience in clinical trial design for rare pediatric disorders. Dr. Finkel's research and clinical activities on muscular dystrophies, dystrophinopathies, neuropathies, Duchenne muscular dystrophy, and SMA have received support from the National Institutes of Health (NIH), private funding agencies, and the biotechnology and pharmaceutical industries.

Dr. Finkel is a member of numerous professional societies, has received awards and recognition for his accomplishments both as a physician and as an educator, and has published 10 editorials, reviews, or book chapters and published or contributed to over 50 articles in peer-refereed journals. One of his papers was recognized as the 2006 World Muscle Society Best Paper on SMA. He serves on the editorial board at *Neuromuscular Disorders* and as an *ad hoc* reviewer for journals that among others include *The New England Journal of Medicine*, *Genetics in Medicine, Journal of Pediatrics Neurology,* and *Annals Neurology*.

Kevin Gregory-Evans, M.D., Ph.D., FRCS, FRCOphth
Kevin Gregory-Evans is Professor of Ophthalmology in the Faculty of Medicine at the University of British Columbia and holder of the Julia Levy BC Leadership Chair in Macular Research. He achieved an M.B.B.S. at St. Bartholomew's Medical School,

University of London; was awarded an FRCS from the Royal College of Surgeons and Physicians, Glasgow; was awarded an FRCOphth from the Royal College of Ophthalmologists, London; and was awarded an M.D. in molecular biology and a Ph.D. in cell biology at the University of London. Before 2009, Dr. Gregory-Evans was Reader in Molecular Ophthalmology at the Imperial College London. He underwent clinical ophthalmology training at Moorfields Eye Hospital, London and basic science research training with Professors Alan Bird and Shomi Bhattacharya (Institute of Ophthalmology, London) and Professor Richard Weleber (Oregon Health Sciences University, Portland, Oregon).

Dr. Gregory-Evans' research interests include novel approaches in the diagnosis and treatment of retinal disease. In total, Dr Gregory-Evans has published 65 peer-reviewed, original research articles, 12 research review articles, and 9 book chapters. This has included basic science studies in molecular genetics, molecular therapeutics in model systems and stem cell therapeutics. In 1994 he reported one of the first localizations for a retinal disease gene and since then has reported on another 15 retinal disease genes. Dr Gregory-Evans has recently led two pre-clinical molecular therapeutic studies and two pre-clinical stem cell interventional studies. Clinical studies have included investigator-led descriptive studies in patients with retinal degeneration, corneal disease, cataract, and glaucoma and a number of investigator-led interventional studies in retinal degeneration, cataract, and glaucoma. He has acted as a lead investigator for two pharma-led interventional studies: SUSTAIN (Study of ranibizumab in patients with subfoveal choroidal neovascularization secondary to age-related macular degeneration, Novartis Pharamceuticals) and VIEW2 (VEGF trap-eye: investigation of efficiency and safety in wet AMD, Bayer Schering Pharma).

In 2008, Dr. Gregory-Evans was elected to the Macular Disease Advisory Group at the Royal College of Ophthalmologists, UK, to advise on setting up retinal disease treatment clinics throughout the UK. In 2006 he was elected an Examiner for post-graduate examinations of the Royal College of Ophthalmologists and between 2004 and 2009 was Theme Lead for undergraduate ophthalmology, Imperial College London. He is an active reviewer for numerous vision science and molecular genetics journals. He is an active speaker for lay audiences including: the Foundation Fighting Blindness Canada; the Canadian National Institute for the Blind; the British Retinitis Pigmentosa Society; and the Royal National Institute for the Blind (UK).

Henry J. Kaplan, M.D., F.A.C.S.
Dr. Kaplan is the Evans Professor of Ophthalmology; Professor in the Department of Microbiology and Immunology; and Chairman of the Department of Ophthalmology & Visual Sciences at the University of Louisville School of Medicine and Director of the Kentucky Lions Eye Center, Louisville. He received his A.B. from Columbia University and his M.D. from Cornell Medical School and is Fellow, American College of Surgeons (F.A.C.S.). Dr. Kaplan completed an Internship in Medicine at Lakeside

Hospital, The University Hospitals of Cleveland, Case-Western Reserve University in Cleveland; a Surgical Residency at Bellevue Hospital, New York University Medical Center; a National Institutes of Health (NIH) Research Fellowship in Immunology in the Department of Cell Biology at the University of Texas Southwestern Medical School in Dallas under the mentorship of J. Wayne Streilein and Ruppert Billingham; an Ophthalmology Residency at the University of Iowa Hospitals and Clinics in Iowa City; and a Retina-Vitreous Fellowship at the Medical College of Wisconsin in Milwaukee under the tutelage of Thomas M. Aaberg. Dr. Kaplan has served as an Assistant Professor in the Department of Cell Biology at the University of Texas Southwestern Medical School; an Associate Professor then Professor and Director of Research in the Department of Ophthalmology at Emory University School of Medicine; and Professor and Chairman of the Department of Ophthalmology and Visual Sciences at the Washington University School of Medicine in St. Louis.

Dr. Kaplan has a novel background as a clinician scientist with scientific expertise in immunology and clinical expertise in vitreo-retinal diseases. His postdoctoral training launched his interest in autoimmune diseases of the eye and led to the discovery of anterior chamber-associated immune deviation (ACAID). His clinical interest in uveitis has led to his research in ocular immunology and autoimmune diseases, which has been funded by the National Eye Institute (NEI) for the past 30 years. His interest in retinal diseases has involved the study of the pathogenesis and treatment of both age-related macular degeneration and hereditary retinal diseases. Many novel observations and insights were made in collaboration with different colleagues, including the first successful submacular surgery to recover central vision in the presumed ocular histoplasmosis syndrome (POHS); the development of techniques to harvest and transplant sheets of RPE cells into the subretinal space of man and other species; the first clinical trial of human allogenic RPE cell transplantation in patients with exudative age-related macular degeneration (AMD) in the United States; the role of senescent Bruch's membrane in RPE attachment and differentiation; and the study of RPE cell differentiation and dedifferentiation *in vitro*. Dr. Kaplan is also involved in research focused on characterizing the Pro23His rhodopsin mutation model of retinitis pigmentosa in the miniature swine. This model will then be used to study the ability of swine induced pluripotent stem cells (iPSCs) to regenerate photoreceptors destroyed in this disease.

Dr. Kaplan's research has been supported by the NIH, and he has participated in peer review at the NIH including serving as a member and then Chairman of the Visual Disorders Study Section at the NEI. He has previously served as a co-editor and then Editor-in-Chief of *Ocular Immunology and Inflammation* and as member of the American Uveitis Society of which he was President from 1997 – 1999.

Brian K. Kwon, M.D.
Dr. Kwon is Associate Professor in the Department of Orthopaedics at the University of British Columbia (UBC); Associate Scientific Director of the Rick Hansen Institute; and Research Scientist at the International Collaboration on Repair Discoveries

(ICORD). He completed his M.D. at Queen's University and his residency in orthopaedic surgery at UBC. He then completed a Ph.D. in neuroscience at UBC, studying spinal cord regeneration. He also completed a fellowship in spine surgery at Thomas Jefferson University, one of the nation's busiest spine trauma centers. Currently, he is an attending spine surgeon at Vancouver General Hospital, British Columbia's sole regional referral center for all spinal cord injuries. Additionally, he runs a research laboratory at ICORD.

Dr. Kwon's primary research interests are in spine trauma and spinal cord injury (SCI). He has led local clinical trials in acute human spinal cord injury and is currently leading a multicenter Canadian clinical trial for acutely injured patients. He is particularly interested in the bi-directional process of translational research for spinal cord injury – both "bench to bedside" and "bedside back to bench". He has worked extensively on establishing biomarkers of human SCI to facilitate human trials and on the development of preclinical models that can serve as testing grounds for novel therapeutic strategies. He has also led initiatives to provide a framework for how promising therapies for SCI should be evaluated in the laboratory setting prior to translation into human patients.

Dr. Kwon has been awarded a New Investigator Award from the Canadian Institutes for Health Research, a Scholar Award from the Michael Smith Foundation for Health Research, and a Mentored Clinician Scientist Award from the Vancouver Coastal Health Research Institute. In 2009 he was selected by Business in Vancouver as one of British Columbia's Top 40 under 40 for his leadership, vision, and achievement. In 2010 he received the Kappa Delta Young Investigator Award from the American Academy of Orthopaedic Surgeons for his research in spinal cord injury. He was the sole Canadian representative on the bi-annual AOA-COA North American Travelling Fellowship and will represent Canada again in 2011 on the bi-annual America-British-Canadian Travelling Fellowship.

Christian L. Lorson, Ph.D.

Dr. Lorson is Professor in the Departments of Molecular Microbiology and Immunology and Veterinary Pathobiology at the University of Missouri Medical School in Columbia. He received his B.A. in Biology from Colorado College in Colorado Springs and his Ph.D. in Molecular Microbiology and Immunology at the University of Missouri Medical School. Following completion of his doctoral degree, Dr. Lorson completed a Research Fellowship in Molecular Biology and Microbiology at the New England Medical Center at Tufts University School of Medicine. He was subsequently named Assistant Research Professor before moving to Arizona State University where he was Assistant Professor in Biology and then Assistant Professor in Veterinary Pathobiology. He then moved to the University of Missouri as an Assistant Professor where he moved up through the ranks to his current position. Dr. Lorson is a Member of the Genetics Area Program, the Molecular Biology Program, and the Life Sciences Center at the University of Missouri-Columbia and also serves as the Scientific Director of FightSMA.

Dr. Lorson's research focus is Spinal muscular atrophy (SMA), an autosomal recessive motor neuron disease that is the leading genetic cause of infantile death. The gene responsible for SMA is called *survival motor neuron-1* (*SMN1*). Interestingly, a human-specific copy gene is present called *SMN2, which* is nearly identical to *SMN1*. SMA is an extremely intriguing target for therapeutic intervention for a number of reasons: 1) While SMA presents in a broad clinical spectrum, a single gene is responsible for all clinical forms of the disease; 2) Loss of *SMN1* and *SMN2* is lethal, therefore essentially all SMA patients retain one or more copies of *SMN2*; and 3) *SMN2* encodes a fully functional SMN protein. Therefore, by identifying molecules that stimulate full-length SMN expression from the *SMN2* gene, these molecules could lead to the development of effective therapies for a broad range of SMA patient populations. Several ongoing projects in the lab include: 1) Development of bi-functional RNAs delivered via a gene therapy vector that modulate *SMN2* pre-mRNA splicing such that full length *SMN2* is expressed that can potentially lessen the SMA phenotype**;** 2) Development of a trans-splicing therapeutic approach where the endogenous target RNA and therapeutic RNA delivered via a gene therapy vector are trans-spliced to create the correct RNA sequence; and 3) Identification of SMN-inducing compounds, more specifically novel aminoglycosides, that can induce SMN protein levels in patient fibroblasts.

Dr. Lorson is a member of the numerous professional societies, serves as an *ad hoc* reviewer for several scientific journals and funding agencies including the National Institutes of Health (NIH), has served as an industry consultant for SMA-related therapy development, and is on the scientific advisory boards of FightSMA and the Muscular Dystrophy Association. Dr. Lorson is active at FightSMA as its Scientific Director and organizes its annual meeting and serves as its representative at the American Society of Gene Therapy. His work has been supported by the NIH, the Muscular Dystrophy Association, SMA Europe, the Huntington Society of Canada, and Families of SMA. He has been a Muscular Dystrophy Association-highlighted researcher in *Quest Magazine*.

Gustav Steinhoff, M.D.

Dr. Steinhoff is Director of the Department of Cardiac Surgery and Medical Faculty Head at the Reference and Translation Center of Cardiac Stem Cell Therapy (RTC) at the University of Rostock, Germany. Dr. Steinhoff completed medical study at the Erasmus University Rotterdam and at Baylor College in Houston before serving as flight physician in the military. He completed his M.D. thesis, Research Fellowship in Transplantation Immunology, a General Surgery Specialization, and Habilitation in General Surgery at the Medical School Hannover in Germany before becoming a Staff Surgeon in Cardiothoracic and Vascular Surgery at the University of Kiel, Germany. He then took a position as Staff Surgeon and Associate Professor of Cardiothoracic Surgery at the Medical School Hannover before moving to the University of Rostock and becoming Full Professor and Director of the Department of Cardiac Surgery.

Dr. Steinhoff is interested in regenerative medicine, tissue engineering, and stem cell therapies. His basic research focuses are in stem cell therapy for myocardial regeneration, non-viral gene therapy with magnetic nanoparticles, and heart valve tissue engineering. His clinical research interests are in bone marrow stem cell therapy for myocardial regeneration and new extracorporal oxygenator systems and minimized heart lung machines. Dr. Steinhoff's research includes studies in animal models for cardiac dysfunction, ischemia-reperfusion, extracorporal circulation, heart valve surgery, and intravital microscopy of mirocirculation.

Dr. Steinhoff's research is supported by the Federal Ministry of Education and Research (BMBF), the German Research Foundation (DFG), and the State of Mecklenburg-Vorpommern. He is a central reviewer for cardiovascular research for the DFG and a member of the German Central Ethical Commission (ZES) for stem cell research. He has received the Rudolf Schoen Preis award from the Medical School Hannover and the German Transplantation Research Prize (DTG – Rudolf Pichlmayr Preis). Dr. Steinhoff has published over 187 scientific articles and holds 1 patent.

Scott R. Whittemore, Ph.D.
Dr. Whittemore is the Henry D. and Marianna Garretson Endowed Professor of Neurological Surgery, Vice Chair for Research, and the Scientific Director of the Kentucky Spinal Cord Injury Research Center at the University of Louisville School of Medicine. He received his B.S. in Biology from Middlebury College in Vermont and his Ph.D. in Physiology & Biophysics from the University of Vermont in Burlington. He then performed postdoctoral training in the Department of Psychobiology at the University of California, Irvine with Dr. Carl W. Cotman and in the Department of Medical Genetics at Uppsala University in Sweden with Dr. Hakan Persson. In 1986, Dr. Whittemore joined the faculty at the University of Miami in the Departments of Neurological Surgery and Physiology & Biophysics where he rose to full professor. In 1998, Dr. Whittemore moved to the University of Louisville.

The general research focus of Dr. Whittemore's laboratory is to utilize molecular and cellular biological techniques to address repair in spinal cord injury (SCI). These studies involve the use of various types of stem cells, gene therapy with multiple viral vectors, and more recently combining rehabilitative strategies. These studies are usually initiated *in vitro* and successful approaches then taken into whole animal experiments. Specific projects involve remyelination by genetically modified, engrafted stem cells, altering angiogenic responses after SCI, and targeting the endoplasmic reticulum stress response for therapeutic intervention after SCI.

Dr. Whittemore has been continually funded by the National Institutes of Health (NIH) since 1988, and serves on numerous editorial boards, study sections (including the NIH), and advisory boards. He has published over 116 scientific papers in peer-refereed journals and 25 books and/or book chapters. He is an active member of several professional societies.