



Nominations for Appointment to the Grants Working Group (GWG)

Appointment of New Members

Francesca Cicchetti, PhD

Dr. Cicchetti is a professor at the department of Psychiatry & Neurosciences, Faculty of Medicine of Université Laval in Québec and a visiting associate professor at Harvard medical school in Boston. Dr. Cicchetti obtained her PhD in neurobiology at Université Laval in Québec (Canada) in 1998. In 2002, she completed a postdoctoral fellowship at Harvard Medical School in the field of cell replacement therapy for neurodegenerative disorders. Her work focuses on the development of therapeutic approaches for neurodegenerative disorders. Her research program is built on 3 distinct research themes: 1) to better understand the phenomenon that may contribute to the development or the propagation of pathological aspects found in neurodegenerative and mental disorders; 2) develop therapeutic strategies that would allow an early or late intervention in the evolution of the disease; 3) study the mechanisms of action underlying the beneficial and/or detrimental effects of current experimental therapies (cell transplantation and deep brain stimulation) in order to improve these methodologies for the clinic. Her research program is based on translational research, from the animal model to the clinic, with the goal to develop novel therapeutic targets for individuals suffering from Parkinson's disease, Huntington's disease and Schizophrenia.

Dr. Cicchetti has published over 80 manuscripts in various high impact journals including in PNAS, Annals of Neurology, Acta Neuropathologica, Trends in Pharmacology, Brain. She has received numerous awards and distinctions including the Canadian Institute of Health Research New Investigator Award (2007-2012), the Fonds de Recherche en Santé du Québec (FRSQ) Junior Research Award (2006-2007 and 2003-2006), Young Investigator Award from NARSAD (2006) and Parkinson Society Canada (2002) and more recently the prestigious National Researcher award from FRQS (2014). She is an active member of several scientific committees and editorial boards

Brian Schwartz, MD

Dr. Schwartz joined ArQule as Chief Medical Officer in July 2008 and in 2013 he took on the responsibility of head of research and development. Dr. Schwartz has more than a decade of experience in the pharmaceutical and biotechnology industries. Prior to joining ArQule, he was senior vice president, clinical and regulatory affairs, and chief medical officer at Ziopharm Oncology where he built and led the clinical, regulatory, and quality assurance departments with responsibilities for the development of new cancer drugs. Prior to Ziopharm, Dr. Schwartz held a number of positions at Bayer Healthcare. His experience in oncology encompassed the clinical development of novel cytostatic, cytotoxic and immunological agents. At Bayer, Dr. Schwartz was a key physician responsible for the global clinical development of sorafenib (Nexavar®) and led the clinical team through a successful Phase 3 trial in renal cell cancer, leading to U.S. Food and Drug Administration (FDA) approval. He has extensive regulatory experience working with the FDA's Oncology Division, the European Medicines Agency (EMA), and numerous other health authorities. Dr. Schwartz has also been responsible for U.S. clinical and regulatory activities,

including Phase 4 studies and interactions with the National Cancer Institute and other oncology cooperative groups. Dr. Schwartz received his medical degree from the University of Pretoria, South Africa, practiced medicine, and worked at the University of Toronto prior to his career in industry.

Reappointment of Scientific Members to the Grants Working Group

Grants Working Group Members originally appointed in 2008-10 have terms that are now expiring or just expired. We are seeking the reappointment of the individuals listed in the table below. Their updated biographies follow. In accordance with the rules set forth by Proposition 71, reappointments should be staggered into thirds, each with a 2, 4, or 6-year term.

Proposed Reappointments to GWG

Last	First	Term	Expertise
Acsadi	Gyula	6	Pediatric Neurology; Gene Therapy for Motor Neuron Disease
Black	Lauren	6	Nonclinical Regulatory (Pharmacology/Toxicology)
Boyan	Barbara	2	Tissue Engineering; Bone/Cartilage Cell Biology
Bulte	Jeff	4	Cellular Imaging
Burger	Scott	2	Process Development; CMC Regulatory; Combination Products
Cavagnaro	Joy	6	Nonclinical Regulatory Affairs for Cell & Gene Therapy; Animal Modeling for Clinical Development
Ellis	James	4	Vector Development; Reprogramming; iPSC Banking; Rett Syndrome
Frey-Vasconcells	Joyce	2	CMC Regulatory
Gregory-Evans	Kevin	2	Ophthalmology
Kaplan	Henry	2	Ophthalmology
Korsgren	Olle	4	Islet allo- & xenotransplantation; Transplantation Immunology
Kwon	Brian	6	Spinal Cord Injury
Lorson	Christian	4	Alternative Splicing; Spinal Muscular Atrophy; Gene Therapy
Steer	Clifford	4	Liver & Neural Regeneration; microRNAs; Non-Viral Gene Therapy
Whittemore	Scott	6	Spinal Cord Injury & Cellular Therapeutics
Zimmermann	Wolfram	6	Cardiovascular Regenerative Medicine & Tissue Engineering

Gyula Acsadi, MD, PhD

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 positions as a Visiting Scientist in the Department of Pediatrics in the Waisman Center at the University of Wisconsin-Madison where he studied gene therapy and as a 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 where he rose through the ranks to his current position. 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 both granting agencies, including the NIH, and scientific peer-refereed journals. His research is supported by several funding agencies including the NIH, the Agency Muscular Dystrophy and Atrophy, and Families of SMA.

Lauren E. Black, PhD

Dr. Black is an employee of Charles River Laboratories, a global clinical and preclinical CRO, where she serves as Senior Scientific Advisor with Navigator Services. Navigators provide a center of expertise to assist drug sponsors with achieving regulatory and clinical objectives, through all stages of nonclinical and clinical development. Dr. Black provides expert toxicologic data analysis, clinical strategic development planning, tailored nonclinical program designs, and regulatory communications advice. Dr. Black's practice is focused on pharmacologic toxicity, biotechnology products, immunopharmacology, and expedited development for high risk indications. She also serves as the Program director at CRL for Immunology/Immunotoxicology related issues. Prior to joining the Charles River Laboratories, Dr. Black served at U.S. Food and Drug Administration (FDA) Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Research (CBER) for 11 years, and left in 2002 to consult to the pharmaceutical industry.

Dr. Black joined the FDA/CDER in 1991 as a Reviewing Pharmacologist with the Division of Antiviral Drug Products, assigned primarily to the INDs for immunosuppressants and

transplantation including Prograf, CellCept, and Rapamune. Additionally, Dr. Black reviewed ~30 other INDs and NDAs for antiviral drugs and antisense oligonucleotides. She performed key pharmacodynamic studies for early files for intravenous oligonucleotides, and published Agency guidance on the safety studies needed to initiate trials with these drugs. These efforts accelerated development of 2nd generation oligos with improved safety profiles.

In 1995, Dr. Black transferred to the FDA CBER, and reviewed ~400 INDs for biologic proteins, cell therapies, and immunomodulators. She served on the review committees for Remicade, Simulect, Amevive, Raptiva, and Tysabri. Dr. Black helped establish standards for toxicity programs for chronic biotherapies as use of these drugs approached broad clinical use. She co-led the committee to set standards for clinical dose estimates, and generated algorithms, terminology, and safety margin approaches for the Guidance to Industry on human starting doses.

Dr. Black also contributed to working groups on biotechnology safety, immunotoxicology, tissue engineering, xenotransplantation, as well as rheumatoid arthritis and osteoarthritis; she contributed to four *Guidance to Industry*. Dr. Black briefed FDA Advisory Committees on Dermatologic Products and Xenotransplantation, and received a number of FDA awards for policy and strategic research initiatives. She is invited to lecture on advanced medicines at academic meetings (ACT, STP, and SOT), universities, and pharma companies, and serves on safety advisory boards for a number of international venture and biotech companies.

Barbara D. Boyan, PhD

Dr. Barbara Boyan is Professor and the William H. and Alice T. Goodwin Chair in Biomedical Engineering and Dean, School of Engineering at the Virginia Commonwealth University in Richmond, VA. In addition, she is professor emerita in the Wallace H. Coulter Department of Biomedical Engineering at Georgia Tech and Emory University in Atlanta, Georgia. Dr. Boyan directs the Virginia branch of the FDA-sponsored Atlantic Pediatric Device Consortium. She is a Fellow in the American Association for the Advancement of Science (AAAS) and in the American Institute of Mechanical and Biomedical Engineering (AIMBE) and in 2012 she was elected to the National Academy of Engineering and was inducted into the Fellows of the World Congress of Biomaterials. She was appointed to the National Materials Advisory Board of the National Research Council of the National Academies and chaired their Roundtable on Biomedical Engineering Materials and Applications from 2008 to 2011. She has founded a number of biomedical technology companies and currently serves on the Boards of both public and private companies. The author of more than 400 peer-reviewed papers, reviews, and book chapters, Dr. Boyan holds 15 U.S. patents.

Jeff W.M. Bulte, PhD

Dr. Bulte is a Professor of Radiology, Oncology, Biomedical Engineering, and Chemical & Biomolecular Engineering at the Johns Hopkins University School of Medicine. He serves as the Director of Cellular Imaging in the JHU Institute for Cell Engineering. He received his PhD degree summa cum laude from the University of Groningen, The Netherlands in 1991. Dr. Bulte spent 10 years at the National Institutes of Health, first as a postdoctoral fellow and then as a Staff Scientist. He was recruited to Johns Hopkins as an Assistant Professor in 2001, became an Associate Professor in 2002, and a Full Professor in 2006. He is a Fellow and the 2014 Gold Medal Awardee of the International Society of Magnetic Resonance in Medicine (ISMRM), a Distinguished Investigator of The Academy of Radiology Research, and in 2015 received the Torsten Almen Award for Pioneering Research in Contrast Media. He has published over 225 peer-reviewed publications and 40 book chapters, which have been cited >22,500 times with an h-index of >75. His research focus is on translational & clinical molecular and cellular imaging as

related to stem cell assays, diagnostics, and therapy, with his lab having been part of the first-in-man MRI cell tracking studies using stem cells.

Scott Burger, MD

Dr. Burger is the principal of Advanced Cell & Gene Therapy. After completing his BS at Tulane University, he received his MD from the University of Pennsylvania School of Medicine. He completed postgraduate training in Laboratory Medicine as well as a clinical fellowship in Transfusion Medicine and a postdoctoral research fellowship, at Washington University in St. Louis.

As the principle of Advanced Cell & Gene Therapy, a consulting firm specializing in cell, gene, and tissue-based therapies, Dr. Burger works with clients in industry and academic centers worldwide. He provides assistance in process development and validation, GMP/GTP manufacturing, GMP facility design and operation, regulatory affairs, technology evaluation, and strategic analysis.

Dr. Burger is on the USP Cell, Gene and Tissue Therapies Expert Committee, and the advisory boards of several cell therapy biotechnology companies. He has served as editor of the International Society for Cellular Therapy Telegraft, and on the ISCT Executive Committee. He is a past medical director of the Cell Therapy Clinical Laboratory and Molecular and Cellular Therapeutics Facility at University of Minnesota, where he was responsible for process development, validation, and GMP production of a broad range of novel cell and gene therapies, in support of over 75 clinical trials. Dr. Burger also was Vice-President for Research and Development at Merix Bioscience, a biotechnology company focused on dendritic cell immunotherapy. He is a frequently invited speaker at industry and academic conferences and is the author of over 100 scientific publications and presentations, and recipient of numerous honors and awards.

Joy A. Cavagnaro, Ph.D., DABT, RAC

Dr. Cavagnaro is the President of Access BIO, Boyce, VA, a consultancy specializing in science-based regulatory strategies and preclinical product development services to facilitate biomedical research and emerging technologies. Specific product areas of expertise include vaccines, cellular and gene therapies, animal-based and plant-based biotherapeutics, biotechnology-derived and tissue engineered products.

Dr. Cavagnaro received her Ph.D. in Biochemistry at the UNC Chapel Hill followed by postgraduate work at Duke and Boston University Medical Centers. She has over 25 years experience in biotech spanning academia, the CRO and biotech industries and government.

Prior to establishing Access BIO, Dr. Cavagnaro was Vice President of Regulatory Affairs at Human Genome Sciences, Inc. Dr. Cavagnaro enjoyed a career in government at the FDA Center for Biologics Evaluation and Research before rejoining industry. During her tenure she was appointed to the Senior Biomedical Research Service, served as FDA's topic lead for safety for the ICH initiative for seven years and rapporteur for the ICH S6 Guidance on Preclinical Safety Evaluation of Biotechnology-derived Pharmaceuticals. She also chaired the working groups responsible for FDA's 1996 Comparability Guidance Document and 1995 Points to Consider in the Manufacture and Testing of Therapeutic Products for Human Use Derived from Transgenic Animals. Her last official FDA duty was to Chair the Annual FDA Science Forum on Regulatory Sciences, December 1997. Prior to joining FDA Dr. Cavagnaro was principal study director for biotechnology products at Covance (formerly Hazleton Labs).

Dr. Cavagnaro is Past Chair of the Regulatory Affairs Professional Society, National Capital Area Chapter of the Society of Toxicology and Immediate Past Chair and Founder of BioSafe, a focused expert science committee within BIO which identifies key scientific and regulatory issues and developments related to the preclinical safety evaluation of biopharmaceutical products. Dr. Cavagnaro is current North American Chair of DIA's Biotech SIAC and is co-chair of

Chesapeake Research Review's Independent IRB. She also serves on the Steering Committee for the NIH National Gene Vector Laboratory and is a member of the Clinical and Regulatory Affairs Committee of the American Society of Gene Therapy. Dr. Cavagnaro was the US BIO representative to the ABPI/BIA Early Stage Clinical Trials Taskforce.

James Ellis, PhD

Dr. Ellis is Professor at the University of Toronto, Senior Scientist at the Hospital for Sick Children in Toronto, and Scientific Co-Director of the Ontario Human iPS Cell Facility. He completed his Ph.D. on gene targeting using retrovirus vectors with Dr. Alan Bernstein at Mt. Sinai Hospital at the University of Toronto in 1990. A post-doctoral fellowship with Dr. Frank Grosveld at the National Institute for Medical Research in London, UK led to identification of Locus Control Region (LCR) elements that drive high-level expression from b-globin transgenes in mice for blood gene therapy. He has been a Scientist and Senior Scientist at the Hospital for Sick Children in Toronto since 1994 and has pursued viral gene therapy of stem cells and technology for reprogramming into induced pluripotent stem (iPS) cells.

The current research objective in the Ellis Lab is to generate the safest and most effective retrovirus and lentivirus vectors for manipulating stem cells during regenerative medicine. They discover epigenetic mechanisms that control transgene expression and that silence retrovirus vectors in stem cells, and apply this knowledge to improve vector design. The study of retrovirus silencing pathways in embryonic stem cells provides a window on the epigenetic control of endogenous genes and repeat sequences. The use of insulators and other powerful regulatory elements allows us to construct effective viral vectors for gene therapy of Rett syndrome in neural stem cells. They are now creating novel vectors that express highly in embryonic stem cells but extinguish during differentiation. These promising vectors may have exciting applications as pluripotency markers to facilitate the generation of iPS cells, for enhancing directed differentiation, or for ablating undifferentiated cells to prevent teratoma formation. The Ellis team is currently modeling Rett syndrome and other disorders using mouse and patient specific iPS cells.

As a Full Professor at the University of Toronto, Dr. Ellis has mentored many trainees at all levels. He is a member the American Society of Gene & Cell Therapy (ASGCT) Embryonic Stem Cell Committee and a member of the International Society for Stem Cell Research (ISSCR). He has reviewed grants for CIRM, NIH, CIHR and other agencies, is a member of the editorial panel for *Stem Cells* and a *F1000* Member contributor, and is a frequent reviewer for the *Nature* and *Cell* family of journals among others. In 2010, he has given international invited talks in the US, Canada, Japan, Brazil, and Italy.

Joyce Frey-Vasconcells. PhD

Joyce L. Frey-Vasconcells is considered one of the foremost regulatory experts regarding cell therapies, combination products, gene therapies, tumor vaccines, and tissues and brings extensive regulatory expertise and experience for this unique group of products. Prior to starting Frey-Vasconcells Consulting, Dr. Frey-Vasconcells served 6 years as a regulatory consultant for Pharmanet working with industry whose mission is to foster product development in the areas of cell therapy, tissues, gene therapy, and tissue engineered products. Prior to joining Pharmanet, she served more than 12 years at the FDA. At FDA, Dr. Frey-Vasconcells was the Deputy Director, Office of Cellular, Tissue, and Gene Therapies (OCTGT) with the Center for Biologics Evaluation and Research (CBER). She was instrumental in developing many of CBER's science and public health policies regarding the regulation of cells, tissues, gene therapies, tumor vaccines, and combination products (tissue engineered products). In 2001, Dr. Frey-Vasconcells was named the Regulatory Expert for Cell Therapies at FDA. She also served on various committees related to combination products, tissue reference group, and HHS committee on tissue engineering to name a few.

Since starting Frey-Vasconcells Consulting, Dr. Frey-Vasconcells has continued working with industry on an individual basis and with organizations whose mission is to foster product

development in the areas of cell therapy, tissues, gene therapy, and tissue engineered products. She is clearly considered one of the foremost regulatory experts regarding tissues, cell therapies, combination products, gene therapies, and tumor vaccines and is recognized by various state and country stem cell networks as an expert in the regulation of stem cell therapies. She brings extensive regulatory expertise and experience for this unique group of products

Kevin Gregory-Evans

Kevin Gregory-Evans is currently Professor of Ophthalmology in the Faculty of Medicine, University of British Columbia, and holder of the Julia Levy BC Leadership Chair in Macular Research, Vancouver, Canada. Before 2009 he was Reader in Molecular Ophthalmology, Imperial College London, UK. He underwent clinical ophthalmology training at Moorfields Eye Hospital, London, UK and basic science research training with Professors Alan Bird and Shomi Bhattacharya (Institute of Ophthalmology, London, UK) and Professor Richard Weleber (Oregon Health Sciences University, Portland, Oregon, USA). In 1985 he achieved an MBBS, at St. Bartholomew's Medical School, University of London; in 1989 was awarded FRCS, Royal College of Surgeons and Physicians, Glasgow, UK; was awarded FRCOphth, Royal College of Ophthalmologists, London, in 1990; in 1994 was awarded an MD in molecular biology, University of London; and in 2009 was awarded a PhD in cell biology, University of London, UK.

His research interests include novel approaches in the diagnosis and treatment of retinal disease. In total, Dr Gregory-Evans has published 84 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 localisations 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 Pharmaceuticals); and VIEW2 (VEGF trap-eye: investigation of efficiency and safety in wet AMD, Bayer Schering Pharma). Most recently he has been a Lead Investigator for STAR: A Phase 2, Multicenter, Randomized, Double Masked, Placebo Controlled Study of the Safety and Efficacy of Ataluren (PTC124) for the Treatment of Nonsense Mutation Aniridia (sponsored by PTC Therapeutics Inc).

Henry J. Kaplan, MD, FACS

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.

Olle Korsgren, MD, PhD

Dr. Korsgren is Professor of Transplantation Immunology and Professor of Cell Transplantation at Uppsala University and is a senior staff member in the Department of Clinical Immunology at Uppsala Hospital. He received his Bachelor of Medicine, M.D. and Ph.D. from Uppsala University and is licensed by the National Board of Health and Welfare to practice medicine and as a Specialist in Clinical Immunology.

Dr. Korsgren's research activity has been focused on making islet transplantation a possible treatment for patients with type I diabetes. This has led him into several different areas from the ontogeny of the fetal pancreas and the development of techniques to make human islet isolation possible to the immunological problems involved in islet allo- and xenotransplantation. He is the Principal Investigator of the Nordic Network for clinical islet transplantation.

Dr. Korsgren has received several honors and awards, and he is frequently invited to give seminars and lectures at international meetings and workshops. Dr. Korsgren's present and past commitments include serving on the editorial boards of several scientific journals, such as *Xenotransplantation* and *Transplantation*, and participating as a frequent reviewer for numerous international funding agencies. Dr. Korsgren has authored more than 200 scientific publications. An inventor, he has been awarded five patents.

Brian K. Kwon, MD

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, PhD

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 project 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*.

Clifford J. Steer, MD

Dr. Steer is Professor of Medicine and Genetics, Cell Biology and Development; Member, Graduate School Faculty in Molecular, Cellular, Developmental Biology, and Genetics; Member, Cancer Center; and Director of the Molecular Gastroenterology Program at the University of Minnesota. He is also the Director of the Physician-Scientist Training Program; and is the Associate Dean for Faculty Affairs in the Medical School. Dr. Steer completed his B.S. degree, his M.D., and his residency training in internal medicine at the University of Minnesota. He moved to the National Institutes of Health (NIH) where he was a staff member of the Laboratory of Biochemistry and Metabolism in the National Institute of Arthritis, Diabetes, Digestive and Kidney Diseases (NIADDK). In his 14 years at NIH, he trained as a hepatologist and developed skills in basic sciences. His major interests at NIH included intracellular trafficking of proteins, carbohydrate receptors, and the clathrin/coated pit endocytic pathway.

Dr. Steer's laboratory is involved in three major areas of research. First, he is applying the *Sleeping Beauty* transposon gene vector to a variety of different animal disease models, including liver, bone marrow and brain disorders, and several projects involving *ex vivo* gene therapy of somatic cells. Secondly, his laboratory discovered that ursodeoxycholic acid (UDCA), a hydrophilic bile acid, is a potent antiapoptotic agent and has characterized the effects and mechanism by which UDCA, and its conjugates, exert their effects. Dr. Steer has used UDCA as a therapeutic agent to treat transgenic models of Huntington's disease, head trauma, acute stroke, spinal cord injury, Parkinson's disease, myocardial infarction, retinitis pigmentosa, and acute renal failure. Lastly, Dr. Steer is characterizing the role of microRNAs (miRNAs) in gene regulation for several target organs and stem cell populations. He has identified miRNAs that may be involved in the progression of colon tissue from polyp to cancer and studies are ongoing to identify specific miRNAs as biomarkers for colon cancer in blood. The laboratory is also elucidating the role of genomic methylation in the control of miRNA biogenesis, has identified a subset of miRNAs in mitochondria that may act as a rheostat for the control of apoptosis, cell proliferation and differentiation, and is studying mechanisms involved in the uncoupling of protein and transcript in regenerating liver after partial hepatectomy. Most recently, his work has focused on the development of humanized livers in pigs using a combination of CRISPR gene editing and blastocyst complementation.

Dr. Steer's honors and awards include: Phi Beta Kappa, Magna Cum Laude, University of Minnesota; Holloman Award in Biotechnology; American Society for Clinical Investigation; Associate Editor, Hepatology (2001-06); Thorne Stroke Award; Department of Medicine Faculty Award for Outstanding Research; Senior Investigator Award; and he was recently made a Fellow of the American Association for the Study of Liver Diseases. Dr. Steer has participated in more than 65 NIH Study Sections, many of which he Chaired, including Gene and Drug Delivery Systems; GI Cell and Molecular Biology; and the Hepatobiliary Pathophysiology Study Section. He is on the Editorial Boards of *Gene Vaccines and Therapy*; *Hepatic Medicine: Evidence and Research*; and *Genes*. He has published more than 260 articles, and his laboratory has been featured globally in newspapers, including Time magazine, for Dr. Steer's work in gene therapy and the use of bile acids in the treatment of neurodegenerative disorders. At the University of Minnesota, Dr. Steer serves on the President's Distinguished Faculty Mentor Program; Gastroenterology Executive Committee; Department of Medicine Research Advisory Committee; Senior Member, Graduate Faculty, Integrative Biology and Physiology; Senior Member, Graduate Faculty, Comparative and Molecular Biosciences, College of Veterinary Medicine; and Biomedical Engineering Institute.

Scott R. Whittemore, PhD

Dr. Whittemore is the Henry D. and Marianna Garretson Endowed Professor of Neurological Surgery, Vice Chair for Research, and the 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 a combination of *in vitro*, *in vivo*, and *ex vivo* approaches to spinal cord repair using a variety of primary cells, ES cells, recombinant viral vectors, and transgenic mice strains – both constitutive and conditional over expressers/dominant negative null mice. We also study molecular mechanism(s) and pharmacological approaches to specifically facilitate oligodendrocyte, microvascular, and neuronal survival after SCI, focusing specifically on the role of the integrated stress response (ISR) in these two cell types. We also investigate spinal cord circuitry with respect to motor control, using conditional virus expression to silence neurotransmission. Most recently, we have begun to explore the role of the gut microbiome on functional recovery after SCI. I have mentored 17 postdoctoral fellows (2 current), and 10 Ph.D. or M.D./Ph.D. students (2 current). Nearly all published multiple manuscripts and have moved on to excellent positions in science and/or medicine.

Dr. Whittemore has been continually funded by the National Institutes of Health (NIH) since 1988 and serves on numerous editorial boards, study sections (including for the NIH), and advisory boards. He has published over 143 scientific papers in peer-refereed journals and 26 books and/or book chapters. He is an active member of several professional societies.

Wolfram-Hubertus Zimmermann, MD

Dr. Zimmermann is Professor of Pharmacology and Toxicology and Director of the Department of Pharmacology at the University Medical Center of the Georg-August-University in Goettingen (Germany). He studied Medicine at the University of Hamburg (Germany), DUKE University Medical School in Durham (US), Harvard Medical School in Boston (US), and the University of Cape Town (South Africa). He graduated from Medical School in 1998 and earned his doctorate from the University of Hamburg in 2000. In parallel, Dr. Zimmermann completed a second academic degree in Molecular Biology at the University of Hamburg in 2001. He trained at the Institutes of Pharmacology and Toxicology at the Friedrich-Alexander University (Germany) and the University Medical Center in Hamburg, where he was promoted to the rank of Assistant Professor in 2003. Dr. Zimmermann completed his training in Pharmacology and Toxicology in 2006 (board examination) and was awarded the *Venia Legendi* (and Habilitation) in Pharmacology and Toxicology in 2007. He was appointed to his current position at the Georg-August-University in Göttingen in 2008.

Dr. Zimmermann's research interests include novel pharmacological and cell-based approaches to repair diseased myocardium with a special emphasis on myocardial tissue engineering. He also serves as a clinical consultant in pharmacology and toxicology.

Dr. Zimmermann has published 100+ articles and book chapters, presented his research at numerous national and international conferences, and holds 7 patents on different aspects of myocardial tissue engineering. He is the recipient of several awards including the Oskar-Lapp-

Award and the Albert-Fraenkel Award from the German Society of Cardiology and the Young Investigator Award of the International Society for Heart Research. Dr. Zimmermann serves as *ad hoc* reviewer for several international scientific journals and granting agencies. He is member of different editorial boards (e.g. *Circulation Research*, *American Journal of Physiology*, *Journal of Molecular and Cellular Cardiology*). Dr. Zimmermann holds grants from the German Research Council, the Federal Ministry of Science and Education as well as the European Union. Dr. Zimmermann is the speaker of the German Center for Cardiovascular Diseases at its partner site in Göttingen.