

International Scientific Advisory Board (ISAB) Members

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| Chris Mason, M.D., Ph.D., FRSB, FRCS – Chair | Founder and CEO of the London Regenerative Medicine Network, Co-founder and Chief Scientific Officer at AvroBio Inc. |
| Andrew Brack | Associate Professor in Orthopaedic Surgery at the University of California, San Francisco |
| Budd A. Tucker, Ph.D. | Stephen A. Wynn Professor in Regenerative Ophthalmology, Associate Professor of Ophthalmology and Visual Sciences |
| Irwin Bernstein, M.D. | Chief, Division of Pediatric Hematology/Oncology at Seattle Children’s Hospital, Head of the Pediatric Oncology Program at the Fred Hutchinson Cancer Research Center. |
| Jeffery Molkentin, Ph.D. | Professor at the UC Department of Pediatrics at Cincinnati Children’s Hospital and a Howard Hughes Medical Institute investigator |
| Lee Rubin, Ph.D. | Director of Translational Medicine, Harvard Stem Cell Institute. Professor of stem cell and regenerative biology at Harvard University. |
| Melissa Carpenter, Ph.D. | President, Carpenter Group, Stem Cell Strategies. |
| Olivier Pourquie, Ph.D. | Professor of Genetics and of Pathology, Harvard Medical School and the Brigham and Woman’s Hospital. |
| Paul Simmons, Ph.D. | Head of Research & Product Development at Mesoblast Ltd. Adjunct Professor for the Stem Cell Research at the Brown Foundation Institute of Molecular Medicine. |
| Rod Dunbar, FRSNZ, Ph.D. | Director of the Maurice Wilkins Centre, Professor at the University of Auckland. |

Chris Mason MD, PhD, FRSB, FRCS – Chair



Chris Mason is a co-founder and Chief Scientific Officer at AvroBio Inc., an *ex vivo* gene therapy company based in Cambridge, Massachusetts. Since 2008, he has been Professor of Regenerative Medicine Bioprocessing in the Advanced Centre for Biochemical Engineering, University College London working on the clinical translation and commercialization of cell and gene therapies. He has a multidisciplinary track record, spanning R&D, clinical medicine, bioprocessing, regulation, healthcare economics, reimbursement and business. His other responsibilities include; Chair of the BioIndustry Association (BIA) Regenerative Medicine and Cell Therapy Industry Group,

Co-Chair of the Alliance for Regenerative Medicine (ARM) Cell Therapy Section, and Founder and CEO of the London Regenerative Medicine Network. Chris is on a number of national and international committees, working groups and initiatives related to the academic, clinical translation and commercialization of cell and gene therapies including; the UK-Israel Science Council, the Scientific Advisory Panel of the UK Cell and Gene Therapy Catapult, Ministerial Industry Taskforce on Attracting Advanced Therapy Manufacturing to the UK, and the Strategic Advisory Board of the Canadian Centre for the Commercialization of Regenerative Medicine. He is a general spokesperson for the cell and gene therapy sector including frequent newspaper, radio and TV interviews. Chris is Senior Editor of the journals; *'Cell and Gene Therapy Insight'* and *'Regenerative Medicine'*.

Andrew Brack, Ph.D.



Originally from Liverpool, England, Dr. Brack graduated with a PhD in Molecular Biology and Biophysics from King's College London. After a successful postdoctoral fellowship, with Tom Rando at Stanford University, where he produced important work on the circulatory factors associated with parabiosis- induced aged muscle rejuvenation, he started his own lab in 2008 at the Center for Regenerative Medicine, MGH, Harvard University, where he focused on the property of quiescence within muscle stem cells. In addition, he identified that the local environment (the niche) of the muscle stem cell became deregulated during aging, leading to a decline in stem cell number and function. In 2015 he moved his laboratory to

the Eli and Edythe Broad Center of Regeneration Medicine and Stem Cell Research at the University of California, San Francisco (UCSF). Dr. Brack is currently an Associate Prof. in Orthopaedic Surgery. At UCSF, Dr. Brack continues to investigate the regulation of skeletal muscle stem cells (MuSCs) during injury, disease and aging.

Budd A. Tucker, PhD



Dr. Tucker went on to complete his Ph.D. degree in neuroscience at Memorial University of Newfoundland's School of Medicine. He subsequently completed a 3-year post-doctoral fellowship at the Schepens Eye Research Institute, Harvard Medical School; where in 2009 under the Mentorship of Dr. Michael J. Young was promoted to the rank of faculty. In 2010 Dr. Tucker joined the Department of Ophthalmology and Visual Science at the University of Iowa where he is currently an Associate Professor of Ophthalmology and Visual Science. Dr. Tucker has a long-standing interest in the treatment of inherited retinal degenerative diseases such as retinitis pigmentosa (RP), Stargardt disease, Usher Syndrome and age-related macular degeneration (AMD). His lab is focused on combining state-of-the-art patient-specific stem cell, gene augmentation/genome editing and tissue engineering based technologies to develop treatments for inherited retinal degenerative blindness.

Irwin D. Bernstein, MD



Irwin D. Bernstein, MD, is chief of the Division of Pediatric Hematology/Oncology at Seattle Children's Hospital, professor of pediatrics at the University of Washington School of Medicine, and head of the Pediatric Oncology Program at the Fred Hutchinson Cancer Research Center. Dr. Bernstein holds the John R. Hartmann Endowed Chair in Pediatric Oncology/Hematology at Seattle Children's and is an American Cancer Society professor. Bernstein's research interests include hematopoietic stem cells, antibody targeted therapies for lymphoma and leukemia, and the biology of acute myeloid leukemia. His research has led to the development of Mylotarg, a drug widely used

for treating acute myelogenous leukemia – and his current studies of hematopoietic stem cells – which have led to a novel approach based on the Notch pathway for expanding stem cell numbers in cord blood that is being tested in clinical trials to improve cord blood transplantation.

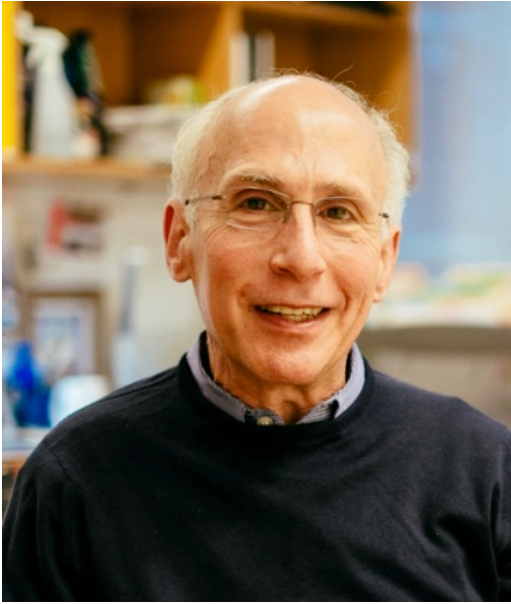
Jeffery Molkentin, Ph.D.



Jeffery Molkentin, Ph.D., is a professor at the UC Department of Pediatrics at Cincinnati Children's Hospital and a Howard Hughes Medical Institute investigator. Dr. Molkentin's laboratory focuses on understanding the molecular mechanisms behind heart and skeletal muscle disease, and in particular his research studies the processes that underlie skeletal muscle degeneration and cell death. His work has helped advance the understanding of molecular events behind muscular dystrophy. Dr. Molkentin's research aims to understand the intracellular signaling pathways and transcriptional regulatory circuits that control mammalian cell growth

and differentiation. His work has advanced the understanding of molecular events behind heart disease and muscular dystrophy

Lee Rubin, Ph.D.



Dr. Rubin is currently a Professor in the field of stem cell and regenerative biology at Harvard University as well as the Director of Translational Medicine at the Harvard Stem Cell Institute. Dr. Rubin has a broad experience in both academia and industry, particularly in the realms of cell-based assays and drug discovery. Prior to coming to Harvard, he was Chief Scientific Officer of Curis, Inc., a Cambridge-based biotechnology company, where his group identified the first small molecule antagonist of the hedgehog-signalling pathway that was developed by Genentech and is now approved as oral treatment for basal cell carcinoma.

At Harvard, much of his work is focused on finding key molecular mediators of different

neurodegenerative diseases. For example, his group has identified new targets for the treatment of the motor neuron disorders Spinal Muscular Atrophy and Amyotrophic Lateral Sclerosis. Recently, his group discovered that a circulating protein, GDF11, has the ability to reverse some of the changes in the CNS associated with aging. They are actively exploring the therapeutic implications of these observations as well.

Melissa Carpenter, Ph.D.



Dr. Carpenter received a Ph.D. in the Laboratory of Cellular and Molecular Neurobiology at the University of California, Irvine. For the last 15 years, she has worked on the development of cell therapies using human adult and embryonic stem cells, in academia and industry, in the United States and Canada. This work involved discovery research and the translation of this research into therapies for regenerative medicine, including developing strategies for preclinical development and navigating the regulatory issues surrounding stem cell therapies. This diversity provides a unique perspective concerning the successful development of cell therapies. At CytoTherapeutics (now StemCells, Inc), Dr.

Carpenter derived human neural stem cells and successfully developed the media formulation used for scale-up of these cells. Dr. Carpenter has been involved with human embryonic stem cell (hESC) research since the field was established. She was Director of Stem Cell Biology at Geron Corporation where she was responsible for managing discovery research and preclinical programs aimed at developing therapies for Parkinson's Disease, Spinal Cord Injury, Cardiac Disease, Liver Disease and Diabetes. After leaving Geron, Dr. Carpenter held the position of Principal Investigator and Scientist at the Robarts Research Institute and Associate Professor, Faculty of Medicine at the University of Western Ontario.

Olivier Pourquie, Ph.D.



Olivier Pourquie is Professor in the Department of Genetics at Harvard Medical School and Professor of Pathology at the Brigham and Women's Hospital. He was the director of the Institute for Genetics and Molecular and Cellular Biology (IGBMC) in France and before that a Howard Hughes Medical Institute Investigator at the Stowers Institute for Medical Institute in Kansas City. The Pourquie laboratory is a world leader in vertebrate musculo-skeletal axis development. Using chicken and mouse embryos as model systems, they combine developmental biology and genomic approaches to study patterning and differentiation of the precursors of muscles and vertebrae. They also develop

quantitative approaches at the interface with physics to study morphogenesis of the vertebral column. While most of this work is being carried out *in vivo*, they also develop protocols to recapitulate these early developmental processes *in vitro* using mouse and human embryonic or reprogrammed stem cells.

Dr. Pourquie authored more than 100 peer-reviewed publications. He is an elected member of the European Molecular Biology Organization and of the Academia Europea. Nature Magazine recognized his work on the segmentation clock that controls the periodicity of vertebrae as one of the milestones in developmental biology of the 20th century. He is the Editor in Chief of the journal *Development*. Pourquie graduated as an engineer in France and trained with Nicole Le Douarin in Paris.

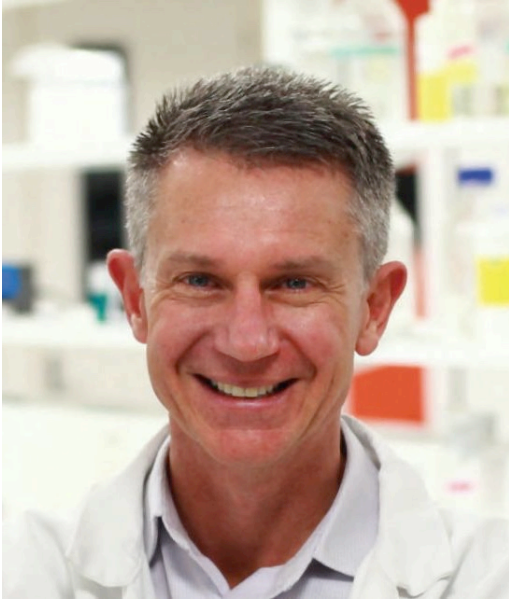
Paul Simmons, Ph.D.



Prof. Paul J. Simmons is currently Head of Research & Product Development at Mesoblast Ltd., a regenerative medicine company based in Melbourne, Australia founded in part on patents covering Dr Simmons' pioneering work on prospective isolation of adult mesenchymal precursor cells (MPC). His move to Mesoblast follows a long and distinguished career in stem cell research spanning nearly 30 years and his contributions to the field were recently recognized by his Presidency of the ISSCR from 2006 to 2007. Dr. Simmons established his first lab at the Hanson Centre in Adelaide, Australia and was subsequently recruited to the Peter MacCallum Cancer Centre (Peter Mac) in Melbourne as Program Head in Stem

Cell Biology. Prior to Mesoblast, he was Director of the Centre for Stem Cell Research at the Brown Foundation Institute of Molecular Medicine (IMM), University of Texas Health Science Center at Houston and held the C. Harold and Lorine G. Wallace Distinguished University Chair.

Rod Dunbar, FRSNZ, Ph.D.



Professor Rod Dunbar holds both a medical degree (MBChB) and a PhD from the University of Otago in New Zealand. He spent 6 years as a post-doctoral research fellow at the University of Oxford, before returning to New Zealand in 2002 under a Wellcome Trust International Senior Research Fellowship. His lab at the University of Auckland's School of Biological Sciences focuses on human cellular immunology and stem cell biology, and applications to human health. Since 2009, Professor Dunbar has also served as the Director of the Maurice Wilkins Centre, the national Centre of Research Excellence for the discovery of new medicines, vaccines, and diagnostics. In

2016 he was elected a Fellow of the Royal Society of New Zealand.