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

Julia Barrett, MD, MPH

Julia Barrett is a senior clinical consultant at Biologics Consulting Group, Inc. (BCG). She received her BA from Smith College, her MD from Northwestern University School of Medicine, and her MS in public health from George Washington University. Dr. Barrett completed an internship and residency in internal medicine at the University of Minnesota, a fellowship in general internal medicine at George Washington University, and was a staff physician at the National Cancer Institute (NCI) before joining the Food and Drug Administration (FDA) as a senior clinical reviewer. Since leaving the FDA, Dr. Barrett continued in the practice of internal medicine and has since begun her work in the regulatory consulting field.

Dr. Barrett was a clinical reviewer at the Center for Biologics Evaluation and Research (CBER), FDA from 1992 to 1997 in the Office of Vaccines Research and Review. While at FDA, she was responsible for providing comprehensive clinical review of Investigational New Drug (IND) applications and Biologics License Applications (BLAs). Dr. Barrett's regulatory expertise, coupled with her clinical experience, provides her with a unique perspective on FDA requirements for the design, preparation and implementation of Phase 1, 2, and 3 clinical protocols, as well as overall clinical development strategy. Dr. Barrett assists her clients with designing and conducting clinical programs for a variety of products (mostly biologics) and clinical indications. She has planned and participated in many FDA meetings and is involved in the preparation of FDA submissions, including pre-INDs, INDs, briefing packages, BLAs and New Drug Applications (NDAs).

Cosimo de Bari, MD, PhD

Cosimo de Bari is a professor translational medicine at the University of Aberdeen, where he heads the Regenerative Medicine Group in the Musculoskeletal Research Programme. Dr. De Bari received his medical degree from the University of Bari (Italy), where he also underwent specialist training in rheumatology. He then moved to Belgium, where he obtained his PhD from the Catholic University of Leuven, and was the recipient of the 2003 Rotary Young Investigator Award from the Royal Belgian Society for Rheumatology. After joining King's College London's Department of Rheumatology in 2003, Dr. de Bari was awarded a 2005 Clinician Scientist Fellowship from the Medical Research Council (MRC), and in December 2005 he was appointed clinical senior lecturer & consultant rheumatologist.

Dr. de Bari has expertise in translational stem cell research for musculoskeletal repair, regenerative medicine, and tissue engineering. His current research interests focus on the development of novel stem cell-based therapies for the musculoskeletal system, mainly articular cartilage and bone; they also include the study of the resident joint stem cells and their niches in health and diseases such as osteoarthritis and rheumatoid arthritis. The ultimate goal of Professor De Bari's

research programme is the development of novel cell-based therapeutic approaches to skeletal repair.

Dr. de Bari is a member of the research and training committee of the Osteoarthritis Research Society International (OARSI), and an editorial board member for several journals including *Regenerative Medicine* and *Arthritis & Rheumatism*. He serves on the scientific advisory panel of Action Medical Research, and on the Fellowships Implementation Committee of Arthritis Research UK.

Michel Sadelain, MD, PhD
Michael Sadelain is the Stephen and Barbara Friedman Chair at the Memorial Sloan-Kettering Cancer Center (MSKCC), and is the director of its Gene Transfer and Somatic Cell Engineering Facility. He is also a member of the Department of Medicine and Immunology Program, and the Molecular Pharmacology and Chemistry Program at MSKCC. He received his MD at the University of Paris and PhD at the University of Alberta, followed by a residency at Centre Hospitalier Universitaire Saint-Antoine (Paris) and a fellowship at Massachusetts Institute of Technology.

Dr. Sadelain and his team are actively conducting research at the Gene Transfer and Somatic Cell Engineering Facility, investigating how to control transgene expression *in vivo* in hematopoietic stem cells, and how to augment immune responses against tumor cells. Until 2007, Dr. Sadelain was co-chair of the program committee and on the board of directors of the American Society of Gene Therapy. He is also a member of the National Heart, Lung, and Blood Institute (NHLBI) Gene Therapy Resource Program review panel, and on the Curriculum Planning Committee at the Sloan-Kettering Institute.

Javier San Martin, MD
Javier San Martin is senior vice president of clinical development at Alder Biopharmaceuticals, a Seattle-based biotech company that provides strategic and technical advice to small and mid-size biotechnology firms on translation science, drug development, regulatory and commercialization strategies. He received his MD from the Universidad de Buenos Aires School of Medicine in 1989, and completed his residency in internal medicine at the Center of Medical Education and Clinical Investigation in Buenos Aires, where he became chief resident and, later, head of the inpatient care in the internal medical ward.

Prior to joining Alder Biopharmaceuticals, Dr. San Martin was executive director of drug development and global development leader of Prolia (denosumab) and the Sclerostin Antibody Program at Amgen. For ten years he was with Eli Lilly, as a medical advisor and clinical research physician. He specializes in Phase 2 to Phase 4 drug development, clinical operations, regulatory affairs, medical affairs, and patient care. He is a member of the American Society of Bone and Mineral Research, and has

published widely in peer-reviewed journals, including the *New England Journal of Medicine*, and the *Journal of Clinical Endocrinology & Metabolism*.

Barbara Matthews, MD, MPH
Dr. Matthews is Regulatory/Clinical Consultant and President at Biodirect, Inc. Regulatory & Clinical Consultants. As a consultant to the pharmaceutical industry, she provides guidance on regulatory issues that arise during all stages of drug development and the approval process. In addition to regulatory guidance, Dr. Matthews provides assistance in the clinical development strategy for biopharmaceutical therapeutics. Indications for these therapeutics include diseases responsive to immunomodulation, e.g. rheumatoid arthritis, inflammatory bowel disease, sepsis, psoriasis. Other indications for which she has experience include oncology, neurology, dermatology, angiogenesis, and ophthalmology. The types of products include monoclonal antibodies, proteins, antisense, and cell & gene therapies. In order to achieve these tasks, Dr. Matthews critically reviews clinical data, identifies potential regulatory concerns in clinical development, and assists in the preparation of safety and/or efficacy sections for regulatory review. In addition, she assists sponsors in preparation for advisory meetings and meetings with FDA.

Prior to becoming a consultant, Dr. Matthews was a Medical Officer and Team Leader at the Food and Drug Administration (FDA) Center for Biologics Evaluations and Research (CBER) in the Division of Clinical Trial Design and Analysis in the Immunology & Infectious Disease Branch. Following her tenure at the FDA, she was Associate Director of Medical Affairs at GloboMax, LLC, where she worked with clients in development of their pharmaceutical products and served as a medical monitor for clinical studies.

Mahendra S. Rao, MD, PhD
Mahendra Rao is director of the National Institutes of Health Center for Regenerative Medicine (NIH CRM). He received his MD from Bombay University in India and his PhD in developmental neurobiology from the California Institute of Technology, Pasadena. Following postdoctoral training at Case Western Reserve University, Cleveland, he established his research laboratory in neural development at the University of Utah, Salt Lake City. He next joined the National Institute on Aging as chief of the Neurosciences Section, where he studied neural progenitor cells and continued to explore his longstanding interest in their clinical potential. He then spent six years as the vice president of Regenerative Medicine at Life Technologies in Carlsbad, California. Dr. Rao is also a co-founder of Q Therapeutics, a neural stem cell company based in Salt Lake City. He returned in August 2011 to the National Institutes of Health (NIH), as director of the new NIH Center for Regenerative Medicine (NIH CRM).

Dr. Rao is internationally renowned for his research involving human embryonic stem cells (hESCs) and other somatic stem cells. He has worked in the stem cell field for more than 20 years, with stints in academia, government and regulatory affairs, and industry, and has served internationally on advisory boards for companies

involved in stem cell processing and therapy, and on committees including the U.S. Food and Drug Administration's Cellular Tissue and Gene Therapies Advisory Committee. Dr. Rao also served as the California Institute of Regenerative Medicine and International Society for Stem Cell Research liaison to the International Society for Cellular Therapy.

Keith H. Wells, PhD
Keith Wells is a senior consultant and head of the New England Office of Biologics Consulting Group, Inc. (BCG). BCG provides national and international regulatory and product development advice on the development and commercial production of biological, drug, and device products. He was formerly senior director of vaccine manufacturing and process development for OraVax, where he was responsible for directing the process development, validation, and manufacturing of products including novel live-virus vaccines, recombinant vaccines, a bacterial toxoid, and an immune-globulin product. He holds a PhD in microbiology and immunology from the State University of New York Health Science Center at Syracuse.

Dr. Wells has over 22 years of broad-based experience in biological product development, including process development, validation, manufacturing operations, and regulatory strategy. Current and prior clients include governments, non-governmental organizations, and companies ranging from virtual organizations to multi-national biopharmaceutical companies. He has played crucial roles in the clinical and commercial development of vaccines and other biological products with sales in excess of $2 billion.

Dr. Wells' product expertise includes vaccines, monoclonal antibodies, therapeutic proteins, viral vectors, and blood products, including medical countermeasures for biological defense. His areas of development expertise include biological process scale-up, cell culture development, downstream processing, validation, and assay development. He has additional expertise in developing and implementing quality systems, and in compliance audits and inspections. He also has experience in the design, validation, and operation of biological manufacturing facilities. Dr. Wells has substantial experience in the preparation of regulatory submissions including Investigational New Drug (IND) applications, Biologic License Applications (BLAs), and Common Technical Documents (CTDs). He has served on numerous special emphasis and source-selection panels for the National Institute for Allergy and Infectious Disease.

Grant Williams, MD
Dr. Williams is a consultant in oncology clinical trial design and regulatory strategy. He received his medical degree and subsequent training in internal medicine, pathology, and medical oncology at the University of Alabama in Birmingham from 1977-1988. For 16 years, from 1989 to 2005, he worked in the Division of Oncology Drug Products at FDA evaluating cancer drug applications, 8 years as a medical reviewer, 5 years as a medical team leader and 3 years as Deputy Director. During his tenure at FDA he contributed to 3 guidance documents, including the Cancer

Endpoints Guidance. He subsequently spent three years as Executive Director in oncology clinical development at Novartis and GSK. He has authored numerous papers and book chapters on cancer drug endpoints and oncology drug regulation. In 2008 Dr. Williams formed a consulting company, Williams Cancer Drug Consulting LLC, which provides advice on oncology clinical trial design and regulatory strategy.

Marco Zarbin, MD, PhD, FACS
Marco Zarbin is professor and chair at the Institute of Ophthalmology and Visual Science, and Professor of Neurosciences at University of Medicine and Dentistry (UMDNJ) Medical School. He is also chief of the Department of Ophthalmology at University Hospital, Newark. He received his MD and PhD from The Johns Hopkins University School of Medicine, and completed residency and fellowships in vitreoretinal surgery and retinal vascular disease at The Wilmer Ophthalmological Institute at Johns Hopkins. He is certified with the American Board of Ophthalmology.

Dr. Zarbin specializes in medical and surgical diseases of the retina, vitreous, and macula, with research interests in retinal cell transplantation and growth factors. His research is focused on developing new surgical treatments for age-related macular degeneration, the leading cause of blindness in persons above age 55. His clinical interests are limited to medical and surgical diseases of the retina and vitreous, with special expertise in age-related macular degeneration, trauma management, complex retinal detachment (including retinopathy of prematurity), and surgery of the macula (including macular holes, subretinal hemorrhage, and subretinal neovascularization).

Dr. Zarbin is an associate editor of the journal *Investigative Ophthalmology & Visual Science,* and is a member of the editorial board of *Survey of Ophthalmology*. He is vice chair of the scientific advisory board of the Foundation Fighting Blindness, a member of the board of Governors (ex officio) of the New Jersey Academy of Ophthalmology, and member of the National Advisory Council of the National Institutes of Health. In addition, he is a member of the American Ophthalmological Society, the Retina Society, the Macula Society, and the Vitreous Society, and Dr. Zarbin past president of the board of trustees of the Association of University Professors of Ophthalmology. Dr. Zarbin has co-authored 112 peer-reviewed scientific publications and 33 book chapters, and has co-edited one book on age-related macular degeneration.