

Dimension Therapeutics Announces Appointments to Scientific Advisory Committee and Clinical Advisory Boards

CAMBRIDGE, Mass.--([BUSINESS WIRE](#))--**Dimension Therapeutics, Inc.** a leading rare disease company advancing novel, liver-directed treatments for diverse genetic disorders, today announced that it has expanded its Scientific Advisory Committee with the appointments of Ian Alexander, M.B.B.S., Ph.D., of the Sydney Children's Hospitals Network, and Nicola Longo, M.D., Ph.D., of the University of Utah. Drs. Alexander and Longo join existing members of Dimension's SAC, including Chair James M. Wilson, M.D., Ph.D., of the University of Pennsylvania, Terence R. Flotte, M.D., of the University of Massachusetts Medical School, and Emil D. Kakkis, M.D., Ph.D., CEO of Ultragenyx Pharmaceuticals. In addition, Mark L. Batshaw, M.D., of Children's National Medical Center, will head the company's Clinical Advisory Board (CAB) for urea cycle disorders, and David A. Weinstein, M.D., M.M.Sc., of the University of Florida, will head Dimension's CAB for glycogen storage disease type Ia (GSDIa).

"Dimension has attained significant momentum, and we are able to access a leading group of advisors as we prepare to commence our first clinical trial in the second half of this year and advance additional programs to meet compelling unaddressed needs for patients with rare diseases," said **Annalisa Jenkins**, MBBS, MRCP, Dimension's Chief Executive Officer. "We are thrilled to attract the leading researchers and clinical experts to our science advisory group, as well as to our newly formed advisory boards for our programs in OTC deficiency, the most common urea cycle disorder, and GSDIa."

Dimension's New SAC Members

Ian Alexander, BMedSci, MBBS (Hons), Ph.D., FRACP (Paeds), HGSACG, FAHMS, is Director Laboratory Research and Senior Staff Specialist, The Children's Hospital at Westmead (CHW), and Professor in Pediatrics and Molecular Medicine, University of Sydney. He is also head of the Gene Therapy Research Unit, a joint initiative of Children's Medical Research Institute and CHW. During his career, Dr. Alexander headed the first group in Australia to treat a genetic disease (SCID-X1) by gene therapy. After completing specialty training in pediatrics at Prince of Wales Children's Hospital, Dr. Alexander obtained a Ph.D. in Molecular Biology from the Garvan Institute in Sydney before completing clinical genetics training at the Murdoch Institute in Melbourne. He then undertook postdoctoral studies at the Fred

Hutchinson Cancer Research Center in Seattle before returning to Australia to take up his current position. In 2015 he was made a Fellow of the Australian Academy of Health and Medical Sciences.

Nicola Longo, M.D., Ph.D., serves as the Professor and Chief of the Division of Medical Genetics at the University of Utah. He is also Director of the Metabolic Service, Co-Director of the Biochemical Genetics Laboratory, and Director of the Fellowship Training Program in Biochemical Genetics at the University of Utah. He is an expert in inherited metabolic diseases and is intimately involved in the treatment of patients with these diseases at Primary Children's Hospital. Dr. Longo earned his M.D. and Ph.D. in Molecular Biology at the University of Parma School of Medicine. He received residency and fellowship training in pediatrics, medical genetics, and clinical biochemical genetics at Emory University in Atlanta, Georgia.

Dimension's CAB members

Mark Batshaw, M.D., is Chief Academic Officer and Physician-in-Chief of the Children's National Health System, where he also serves as Director of the Children's Research Institute. He is also Chairman of Pediatrics and Associate Dean for Academic Affairs at the George Washington University School of Medicine and Health Sciences. Dr. Batshaw's research focus is on inborn errors of urea synthesis, in which he is considered an international authority in the development and testing of innovative therapies. He completed his undergraduate degree at the University of Pennsylvania and attended medical school at the University of Chicago. He also completed his residency in pediatrics at the Hospital for Sick Children/University of Toronto in Toronto, Ontario, and his post-doctoral fellowship in neurodevelopmental pediatrics at the Kennedy Institute of Johns Hopkins University School of Medicine.

David A. Weinstein, M.D., M.M.Sc., is Director of the Glycogen Storage Disease Program and Professor of Pediatric Endocrinology at Shands Hospital at the University of Florida. Previously, he was Director of the Glycogen Storage Disease Program at Children's Hospital Boston. Dr. Weinstein has been inducted in the Rare Disease Research Hall of Fame and was awarded a United Nations recognized international humanitarian award for his efforts to help children with glycogen storage disease from around the world. Following his graduation from Trinity and Harvard Medical School, Dr. Weinstein completed a residency, chief residency, and fellowship in pediatric endocrinology at Children's Hospital Boston. He subsequently obtained a Masters in clinical investigation from Harvard and MIT.

About Dimension Therapeutics

Dimension Therapeutics, Inc., is a leading rare disease company focused on developing novel treatments for devastating disorders associated with the liver and based on an industry-leading, extensively validated adeno-associated virus (AAV) platform. The company is advancing multiple programs toward clinical development, including: programs addressing unmet needs for patients suffering from OTC deficiency and GSDIa; a collaboration with Bayer HealthCare in hemophilia A, and a wholly owned program in hemophilia B, which is expected to enter clinical testing in the second half of 2015. Dimension has preferred access to multiple best-in-class AAV vectors from REGENXBIO, which founded Dimension with Fidelity Biosciences in October 2013. The Dimension team and senior advisors include biotech industry veterans and renowned thought leaders in gene therapy and rare diseases. For more information, please visit www.dimensiontx.com.