

Speaker Biographies

John Maraganore, Ph.D.

Chief Executive Officer, Alnylam

Since 2002, John Maraganore has served as the CEO and a Director of Alnylam. Prior to Alnylam he served as an officer and a member of the management team for Millennium Pharmaceuticals, Inc. As Senior Vice President, Strategic Product Development for Millennium, he was responsible for the company's product franchises in oncology, cardiovascular, inflammatory, and metabolic diseases. He was previously Vice President, Strategic Planning and M&A and prior to that he was General Manager of Millennium BioTherapeutics, Inc., a former subsidiary of Millennium. Before Millennium he served as Director of Molecular Biology and Director of Market and Business Development at Biogen, Inc. At Biogen, Dr. Maraganore invented and led the discovery and development of Angiomax™ (bivalirudin for injection, formerly Hirulog™) currently marketed by The Medicines Company. Prior to Biogen, Dr. Maraganore was a scientist at ZymoGenetics, Inc., and the Upjohn Company. Dr. Maraganore received his M.S. and Ph.D. in biochemistry and molecular biology at the University of Chicago. Dr. Maraganore is a Director for Agios Pharmaceuticals and bluebird bio. He also serves as a Venture Partner with Third Rock Ventures. Dr. Maraganore is a member of the Biotechnology Industry Organization (BIO) Board and the BIO Executive Committee, and serves as the chair of the Emerging Company Section and as co-chair of the Regulatory Environment Committee.

Ariel Lager

Patient Living with Acute Intermittent Porphyria

Ariel Lager lives with her husband and two young daughters in New Jersey. She was diagnosed with Acute Intermittent Porphyria in 2010 and has suffered frequent and severe recurrent attacks since, often requiring extended hospitalizations for excruciating neuropathic pain, intractable vomiting, fatigue, and psychological symptoms. She works full-time managing the renewable energy practice group of a Philadelphia based consulting company.

Bill Querbes, Ph.D.

Associate Director, Research, Alnylam

As Associate Director of Research, Dr. Querbes is responsible for project leadership related to Alnylam's discovery and early clinical stage programs, with a current focus on ALN-AS1, Alnylam's investigational RNAi therapeutic for acute hepatic porphyrias. Bill joined Alnylam as a scientist in 2006 and since that time has played key roles in the advancement of RNAi delivery technologies and preclinical programs in the genetic medicine and cardiometabolic stars. He received his Ph.D. in Medical Science from Brown University and his BS in Biology from SUNY Genesco.

Herbert L. Bonkovsky, MD, FACP, FACG, FAGA, FAASLD--Brief Biosketch

Herbert L. Bonkovsky, M.D., is a tenured Professor of Medicine and Molecular Medicine & Translational Research and Chief of Hepatology and The Liver and Metabolic Disorders Laboratory at Wake Forest University/NC Baptist Medical Center, Winston-Salem, NC. Dr. Bonkovsky also has academic appointments as Visiting Professor at Winston-Salem State University, Professor of Medicine, at The University of CT Health Center, and Professor of Biology and Medicine at the University of North Carolina. Dr. Bonkovsky is known nationally and internationally as a clinical hepatologist, teacher, mentor, and clinical investigator. He is a valedictory graduate of Earlham College [Richmond, IN] and Case Western Reserve University School of Medicine [Cleveland, OH]. His Post-graduate training was at Duke University, Case Western Reserve University School of Medicine, The U.S. National Institutes of Health, Dartmouth Medical School, and Yale University.

Dr. Bonkovsky formerly served as Director of the Division of Digestive Disease and Nutrition at the University of Massachusetts Medical School and as Director of the Office of Clinical Research and the General Clinical Research Center and Director of the Liver-Biliary-Pancreatic Center at the University of Connecticut Health Center. He was recruited to Carolinas HealthCare System [CHS], Charlotte, NC, from U Conn in 2007 and, for 5 years, served as VP for Research at CHS. Dr. Bonkovsky has continued to maintain an active clinical practice, focused on liver disorders and metabolic disorders, especially disorders of iron, porphyrin, and heme metabolism. His currently funded research is in porphyrin and heme metabolism, effects of heme and iron on gene expression and intermediary metabolism, and drug- and herbal supplement-induced liver injury.

Among Dr. Bonkovsky's seminal contributions to the study of disorders of porphyrin and heme metabolism were the first preparation and therapeutic use of heme [in the form of

the hydroxide, hematin] for therapy of acute porphyrias and demonstration of defective activity of ferrochelatase as the underlying cause of erythropoietic protoporphyria. Hemin for human use was the first Orphan Drug ever developed, and its use for therapy of acute porphyric syndromes has stood the test of time. Still today, it is the treatment of choice for treatment of attacks of acute porphyria.

Dr. Bonkovsky also played a key role in establishing the programs in liver transplantation both at Emory University and at U Mass Medical Center. He also was a founding member of the Liver Center at Carolinas HealthCare System, Charlotte, NC, where he served for several years as VP and Director of Research. He was recruited to Wake Forest to continue with his funded research and to help build up subspecialty expertise and services in hepatology, with emphasis upon diseases of metal metabolism, such as hemochromatosis, hypoceruloplasminemia, Wilson's disease, and the porphyrias. The Liver Clinics at Wake Forest Baptist Medical Center also evaluate and manage patients with the more commonly occurring liver diseases, such as viral hepatitis, alcoholic and non-alcoholic fatty liver disease, and auto-immune liver disorders [such as autoimmune hepatitis, primary biliary cirrhosis, primary sclerosing cholangitis]. Dr. Bonkovsky also serves as chief of the Liver, Digestive, and Metabolic Disorders Laboratory of Wake Forest, which has competed successfully for external funding from NIH [NIDDK, NHLBI] and from Foundations and Societies. In the past 6 years, Dr. Bonkovsky has filed five patent applications for new therapies and diagnostic tests for viral hepatitis and neuro-muscular diseases.

Dr. Bonkovsky is a member of many learned and professional societies: he is a Fellow of the American College of Gastroenterology, the American College of Physicians, the American Gastroenterological Association, and the American Association for the Study of Liver Diseases. He is also an elected member of the American Society of Clinical Investigation [The Young Turks] and the Association of American Physicians [The Old Turks]. Continually since 1996, Dr. Bonkovsky has been selected by his peers as one of America's Top Doctors and Top Gastroenterologists and Hepatologists. Dr. Bonkovsky is author or co-author of more than 345 presented abstracts, 47 brief reports, 43 books or book chapters, and 394 original papers.