



2ND WFH GENE THERAPY ROUND TABLE

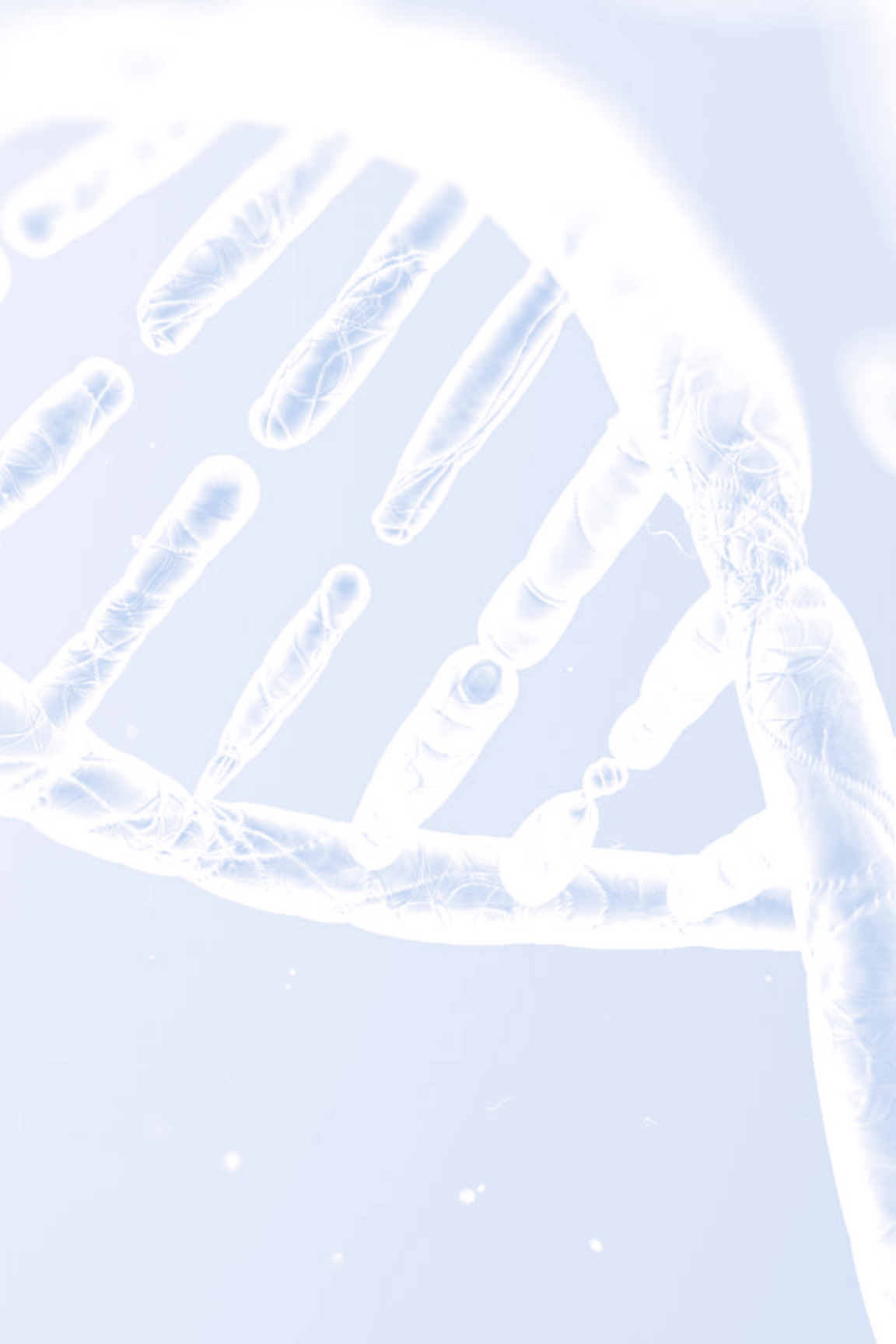
Understanding the Landscape and
Challenges of Gene Therapy for
Hemophilia around the World

APRIL 4–5, 2019 · MONTRÉAL, CANADA



WFH

WORLD FEDERATION OF HEMOPHILIA
FÉDÉRATION MONDIALE DE L'HÉMOFILIE
FEDERACIÓN MUNDIAL DE HEMOFILIA



The **2nd WFH Gene Therapy Round Table (GTRT)** continues the conversation on the expected challenges and opportunities that gene therapy for hemophilia will present. The focus is on models of access to gene therapy around the world, reimbursement, payment models and HTA considerations, regulatory issues and evidence generation on safety and durable efficacy of gene therapy products.

Objectives

- Explore possible models of access to gene therapy that advance the WFH's vision of Treatment for All
- Understand the challenges associated with the development of gene therapy for the global hemophilia community
- Develop a series of consensus statements on select challenges associated with the introduction of gene therapy for hemophilia
- Identify educational opportunities and advocacy initiatives regarding gene therapy

Program Committee

Co-Chairs

- Dr. John Pasi, *United Kingdom*
- Dr. Glenn Pierce, *United States*

Members

- Dr. Radoslaw Kaczmarek*, *Poland*
- Dr. David Lillicrap, *Canada*
- Dr. Johnny Mahlangu, *South Africa*
- Dawn Rotellini*, *United States*
- Thomas Sannié*, *France*
- Dr. Alok Srivastava, *India*
- Dr. Marijke van den Berg, *The Netherlands*
- Prof. Thierry VandenDriessche, *Belgium*

*People with a bleeding disorder / caregiver

Thursday, April 4

ROOM: ROYAL AB

OPENING: 8:15AM – 8:30AM

WELCOME AND OBJECTIVES

Alain Weill, *France*
Dr. Glenn Pierce, *USA*

8:30AM – 9:00AM

GENE THERAPY UPDATE

- The gene therapy pipeline *Dr. John Pasi, UK*
- The knowns and unknowns of gene therapy *Dr. Glenn Pierce, USA*

SESSION 1: 9:00AM – 10:00AM

CHANGING THE LIVES OF PEOPLE WITH HEMOPHILIA

Moderator: Dr. John Pasi

- Treatment landscape around the world *Dr. Glenn Pierce, USA*
- Gene therapy trial — patient experience *John (Jay) Konduros, Canada*
- Psychosocial issues / patient concerns *Marion Louise Taylor, UK*

PANEL DISCUSSION

BREAK 10:00AM – 10:30AM · ROYAL A FOYER

SESSION 2: 10:30AM – 12:00PM

HEALTH TECHNOLOGY ASSESSMENT, REIMBURSEMENT AND PAYMENT

Moderator: Jamie O'Hara

- Reimbursement and payment *Brian O'Mahony, Ireland*
- Reimbursement of curative therapies *Dr. Casey Quinn, USA*
- Effectiveness and value of novel therapies *Dr. Greg Guzauskas, USA*
- HTA and gene therapy *Dr. Sophie Söderholm Werkö, Sweden*
- NICE perspective of gene therapy *Sheela Upadhyaya, UK*

PANEL DISCUSSION

LUNCH: 12:00PM – 1:00PM · MÉCHANT BOEUF RESTAURANT

Thursday, April 4 (continued)

ROOM: ROYAL AB

SESSION 3: 1:00PM – 2:30PM

ACCESS TO GENE THERAPY GLOBALLY: THOUGHTS FROM THE FIELD

Moderator: Dr. Johnny Mahlangu

- Delivering humanitarian aid to LICs Phil Wood, *SOBI*
- St. Jude's clinical trial program Dr. Andrew Davidoff, *USA*
- Enhancing identification of patients through education David Kyne, *USA*
- Trials and tribulations of bringing gene therapy to a LIC Dr. Alok Srivastava, *India*

PANEL DISCUSSION

BREAK: 2:30PM – 3:00PM · ROYAL A FOYER

SESSION 4: 3:00PM – 4:00PM

SPECIFIC MANUFACTURING CHALLENGES

Moderator: Prof. Thierry VandenDriessche

- Large scale rAAV manufacturing in insect cells Dr. Jacek Lubelski, *uniQure*
- Manufacturing of AAV in mammalian cells Dr. Fraser Wright, *Wright Biologics Consulting*
- Identifying exact discrepancies in factor assay results from various assay methods Dr. Stephen Zoog, *BioMarin*

PANEL DISCUSSION

SESSION 5: 4:00PM – 5:00PM

EVIDENCE GENERATION / LONG TERM FOLLOW-UP: SAFETY & DURABLE EFFICACY

Moderator: Dr. Glenn Pierce

- Collecting long term follow-up data on gene therapy Dr. Barbara Konkle, *USA*
- ATHN-14: gene therapy outcomes study Dr. Crystal Watson, *USA*

PANEL DISCUSSION

DINNER: 6:30PM · VERSES RESTAURANT

Friday, April 5

ROOM: ROYAL AB

SESSION 6: 8:00AM – 9:30AM

REGULATORY PERSPECTIVES

Moderator: Dr. Glenn Pierce

- Collecting safety data on gene therapy *Caroline Voltz-Girolt, The Netherlands*
- Regulatory perspectives from FDA *Dr. Peter Marks, USA*
- Regulatory perspectives from EMA *Dr. Sol Ruiz, Spain*
- Regulatory aspects unique to hemophilia *Dr. Anneliese Hilger, Germany*

PANEL DISCUSSION

SMALL GROUP BREAK-OUT: 9:30AM – 10:30AM

CONSENSUS STATEMENTS

Moderator: Donna Coffin

- Instructions *Donna Coffin, Canada*

SMALL GROUP DISCUSSIONS

BREAK: 10:30AM – 10:45AM · ROYAL A FOYER

SMALL GROUP REPORTING: 10:45AM – 12:00PM

CONSENSUS STATEMENT PRESENTATIONS AND DISCUSSION

Moderator: Dr. John Pasi

CLOSING: 12:00PM – 12:15PM

GTRT 2019 WRAP-UP & FEEDBACK

Dr. Glenn Pierce

LUNCH: 12:15PM · BOXED LUNCH



ANDREW DAVIDOFF

Dr. Andrew Davidoff is a general surgeon in Memphis, Tennessee and is affiliated with multiple hospitals in the area, including Methodist Hospitals of Memphis and St. Jude Children's Research Hospital. He received his medical degree from Perelman School of Medicine at the University of Pennsylvania and has been in practice for more than 20 years. He is one of 70 doctors at Methodist Hospitals of Memphis and one of 5 at St. Jude Children's Research Hospital who specialize in General Surgery.



GREG GUZAUSKAS

Greg Guzauskas, MSPH, PhD, is a health economist at the Comparative Health Outcomes, Policy, and Economics (CHOICE) Institute at the University of Washington in Seattle. He is the co-author of multiple health economic models and related publications, including reports by the Institute for Clinical and Economic Review (ICER) such as the recent one on emicizumab prophylaxis for severe hemophilia A.



ANNELIESE HILGER

Dr. Anneliese Hilger is the Acting head of division of the Haematology and Transfusion Medicine and head of the Blood Coagulation Products section at the Paul-Ehrlich-Institut, the Federal Agency for Vaccines and Biomedicines in Germany. She is in charge of marketing authorization, scientific evaluation of clinic trial applications, and national and European scientific advices of blood coagulation products and biotechnological analogues. In addition, she chairs the World Health Organization (WHO) Blood Regulators Network, she is a senior expert of the Blood Products Working Party (BPWP) of the EMA and is a lecturer at Bonn University.



JOHN "JAY" KONDUROS

Jay is a person with factor IX hemophilia who has recently undergone experimental gene therapy. Jay was born in Montreal in 1964, one of four children including an older brother with hemophilia. The family moved to the Toronto area when Jay was 9 years old and he has lived in that region ever since. Since childhood, Jay's life has been interspersed with a multitude of bleeding episodes and recovery periods, treating with blood plasma, factor concentrate, and recombinant products. Jay has lived through many eras of hemophilia care and considers himself extremely fortunate to take part in such a life changing hemophilia therapy.



BARBARA A KONKLE

Dr. Konkle has built a career in clinical and laboratory-based research in disorders of hemostasis, including hemophilia. She is currently the Associate Chief Scientific Officer, Associate Director of the Washington Center for Bleeding Disorders, and Director of the Hemostasis, Platelet Immunology and Genomics Laboratory at Bloodworks Northwest and Professor of Medicine at the University of Washington in Seattle, WA in the U.S. Dr. Konkle is on the Board of Directors of the World Federation of Haemophilia and the Foundation for Women and Girls with Blood Disorders. In the past she has served as the Chair of the Board of Directors for the American Thrombosis and Haemostasis Network, a member of the Medical and Scientific Advisory Council of the National Hemophilia Foundation, a founding Board member of the North American Society on Thrombosis and Haemostasis, and on the Advisory Committee to the National Heart, Lung and Blood Institute of the U.S. National Institutes of Health.



DAVID KYNE

David started KYNE in 2009 to connect public and private organizations to help address unmet health needs, believing that targeted, strategic and creative communications can be a powerful health intervention. He has more than 20 years' experience leading creative communications teams, working across the global healthcare landscape. David leads a growing team that partners with biopharmaceutical companies; philanthropic foundations; public health bodies; UN agencies and NGOs to help improve and save lives. He also serves on the Board of Save-One-Life and advises the CDC Foundation Corporate Roundtable. Under David's leadership, KYNE has been named PRWeek's Boutique Agency of the Year, PR News Top Places to Work and PRWeek Global Campaign of the Year for United Against Malaria. David earned a Bachelor of Arts degree in history and politics from University College Dublin and a master's degree in public relations from the Dublin Institute of Technology.



JACEK LUBELSKI

Jacek Lubelski, Ph.D., joined uniQure as a scientist in 2008. As a Vice President, Global Pharmaceutical Development he is overseeing uniQure's vector, process, non-clinical and analytical development teams. Prior to leading uniQure's development organization he has led both our Vector Development and Process Development departments. Dr. Lubelski received a master's degree in biotechnology at the University of Maria Curie-Skłodowska, Lublin, Poland and a Doctorate in molecular biology from the University of Groningen, The Netherlands. Subsequently, in his postdoctoral work, he served as Postdoctoral Fellow at the Molecular Genetics Department of Groningen University in The Netherlands.



PETER MARKS

Peter Marks received his graduate degree in cell and molecular biology and his medical degree at New York University and completed Internal Medicine residency and Hematology/Medical Oncology training at Brigham and Women's Hospital in Boston. He has worked in academic settings teaching and caring for patients and in industry on drug development. He joined the FDA in 2012 as Deputy Center Director for CBER and became Center Director in January 2016.



BRIAN O'MAHONY

Brian is the Chief Executive of the Irish Haemophilia Society. He represents the Society on the statutory National Haemophilia Council and he is the Vice Chair of the Haemophilia Product Selection and Monitoring Advisory Board. He previously served as Chair of the Irish Haemophilia Society for 17 years from 1987 to 2003 and as President of the World Federation of Hemophilia (WFH) for 10 years from 1994 to 2004. He continues to work as a volunteer with WFH and he assists several national member organisations on an ongoing basis in areas such as strategic planning, and lobbying. In 2011 Brian was elected President of the European Haemophilia Consortium (EHC), and Brian currently is the President of the EHC. A medical scientist by profession, he is a Fellow of the Institute of Biomedical Sciences (UK) and a Fellow of the Academy of Clinical Science and Laboratory Medicine (Ireland). He also has post Graduate qualifications in Management and in Occupational Health and Safety. He is an assistant Professor in Health Service Management in Trinity College, Dublin. Brian has severe Haemophilia B.



JOHN PASI

John Pasi has been Professor of Haemostasis and Thrombosis at The Royal London Hospital, Barts and the London School of Medicine and Dentistry, since 2003. He was previously Professor of Haematology at the University of Leicester and Consultant Haematologist at the Royal Free Hospital and School of Medicine in London. He is closely involved in the design and development of clinical trials for new therapies and evolving phase 1-4 programmes. He currently chairs the London Haemophilia Clinical Advisory Group and Commissioning Forum.



GLENN PIERCE

Dr. Glenn Pierce currently serves on the WFH as VP Medical and WFH USA Board of Directors and NHF (US) Medical and Scientific Advisory Council. He is an Entrepreneur-in-residence at Third Rock Ventures, recently cofounded and is CMO at Ambys Medicines, and also is a biotech consultant in the gene therapy and hematological areas.



CASEY QUINN

Casey Quinn, PhD, is a Health Economics and Financing Research Affiliate in the Center for Biomedical Innovation/NEWDIGS Financing and Reimbursement of Cures in the US (FoCUS) program. Dr Quinn is leading a systematic review and primary research into patient and carer perspectives, as well as supporting several other workstreams. Dr Quinn's previous experience includes payer and commissioning roles with the UK NICE, NIHR, and NHS funding committees; he is also a member of the NY state drug utilization review board. In FoCUS, he is taking a role that spans general health economics and modeling methodology, as well as evidence generation from a payer perspective and how that feeds into measuring, valuing and financing innovation in new therapies. He earned his PhD in Health Economics from the University of York and has taught economics, health economics, and statistics at universities in Australia, the UK, and the US.



SOL RUIZ

Sol Ruiz is Head of Biologics, Biotechnology and Advanced Therapies at the Spanish Medicines Agency. She has a PhD in Biology. She is the chair of the BWP (Biologics Working Party) at the EMA (European Medicines Agency) since March 2014. She is also a member of the CAT (Committee for Advanced Therapies) and a co-opted member of the CHMP (Committee for Human Medicinal Products) since 2007. She also participates in several working groups related to biologics and advanced therapies both at the EMA and at the European Pharmacopoeia.



ALOK SRIVASTAVA

Dr. Alok Srivastava is Professor of Medicine at the Department of Haematology and Head, Center for Stem Cell Research, at Christian Medical College, in Vellore, India. He is also the Director of the IHTC of the WFH there. He was the VP (Medical) of the WFH and chair of the FVIII/IX subcommittee of the SSC of the ISTH. His interests are in developing innovative models of care and outcome assessments that are suited for resource constrained environments. His group is working on developing gene therapy for hemophilia. He chairs the ISTH SSC Task Force on gene therapy for hemophilia. He is the chair of the writing group for WFH guidelines for the management of hemophilia.



MARION LOUISE TAYLOR

Louise Taylor ITP Nurse Consultant did her general nurse and ICU Training in Johannesburg, South Africa. Before immigrating to the UK in 2000, she developed 3 ICU's in 2 major private hospitals in Johannesburg. Her interests changed to Haematology in 2010 and worked as an ITP Nurse Specialist and Research Co-ordinator. Louise is actively involved with ITP patients on a daily basis in a busy Haematology Day Ward, advising patients on their disease and treatment. Louise is one of the authors of the 'Immune Thrombocytopenia: A Practical Guide for Nurses and Other Allied Healthcare Professionals'. She is a member of the ITP Forum.



SHEELA UPADHYAYA

Sheela Upadhyaya is currently the Associate Director of the Highly Specialised Technology program at NICE and is responsible for running the program to evaluate medicines and technologies for rare and ultra-rare conditions for commissioning in the NHS. She has extensive experience in understanding the issues that face companies and other stakeholders when trying to secure access for medicines for the orphan and ultra-orphan conditions. These include developing innovative access arrangements in liaison with industry, clinicians, patients and the NHS. Sheela has co-authored several papers that discuss HTA methods for assessing value of orphan medicines and presented at many conference issue panels on the subject. Prior to joining NICE, she commissioned rare and ultra-orphan disease services in the NHS delivering improvements through collaborating with industry, clinicians and patient groups. During that time, she successfully decommissioned failing services and seamlessly transferred the care of patients to better quality services. Sheela has a passion for partnership working and believes that collaboration across the sector is the key to delivering high quality outcomes for all.



CAROLINE VOLTZ-GIROLT

Caroline has been working for the EMA for the past 11 years where she held different positions. She started in regulatory affairs where she was deeply involved in the implementation of the ATMP Regulation. She moved in the biologics office where she became a PTL for ATMP and the scientific secretary of the gene therapy working party. She was also involved in the pandemic activities. In 2013, she moved to the CNS office where she was involved in the revision of the GVP module V and RMP template and then moved in the oncology, haematology and diagnostics office where she is currently working as an EMA product lead and is the scientific secretary of the blood products working party. She is also a member of the innovation task force and PRIME. Before working at EMA, Caroline was working in industry.



CRYSTAL WATSON

Crystal Watson, ATHN's President and CEO has over 25 years of experience working in the blood disorders community in both government and nonprofit organizations. Since joining ATHN in 2008, Crystal has successfully championed the evolution of the technology and infrastructure underpinning the national database now being used to support research and surveillance. Prior to joining ATHN, Crystal was with the CDC in the Division of Blood Disorders and the National Center for Public Health Informatics and spent 10-years with Hemophilia of Georgia. She has a Bachelor of Science degree in Social Work from the University of North Carolina.



SOPHIE SÖDERHOLM WERKÖ

Dr. Sophie Werkö has an MSc in Business Administration and a PhD from the University of Stockholm. She has a longstanding engagement with HTA and joined the Swedish Agency for Health Technology Assessment and Assessment of Social Services (SBU) in 2007 as a Project Director. In 2012 she was appointed Manager of International Relations and in 2015 she also became Manager of Patient Engagement. Sophie also serves as the Board Chair for the International Network of Agencies for Health Technology Assessment (INAHTA) and represents Sweden in the EU HTA Network (HTAN). She has participated in the work of the European network for Health Technology Assessment (EUnetHTA) since 2009 and serves, since 2016, as Associated Editor of the International Journal of Technology Assessment in Health Care and since 2014 of Research Involvement and Engagement.



PHILIP WOOD

Mr. Philip Wood has been Head of Haemophilia at Swedish Orphan Biovitrum AB since October 26, 2017. Mr. Wood served as Vice President and Commercial Therapeutic Area Head of Haemophilia at Swedish Orphan Biovitrum AB since November 12, 2013 until October 26, 2017. Mr. Wood joined Sobi in March 2012 as Global Strategic Lead for Haemophilia A team, focusing on the pre-launch platform for the potential Factor VIII product. He worked for both Wyeth and Pfizer in various positions of increasing responsibility from sales representative to European Director and European Specialty Asset Team Leader where he was responsible for the UK and then European re-launch of ReFacto® and Benefix®.



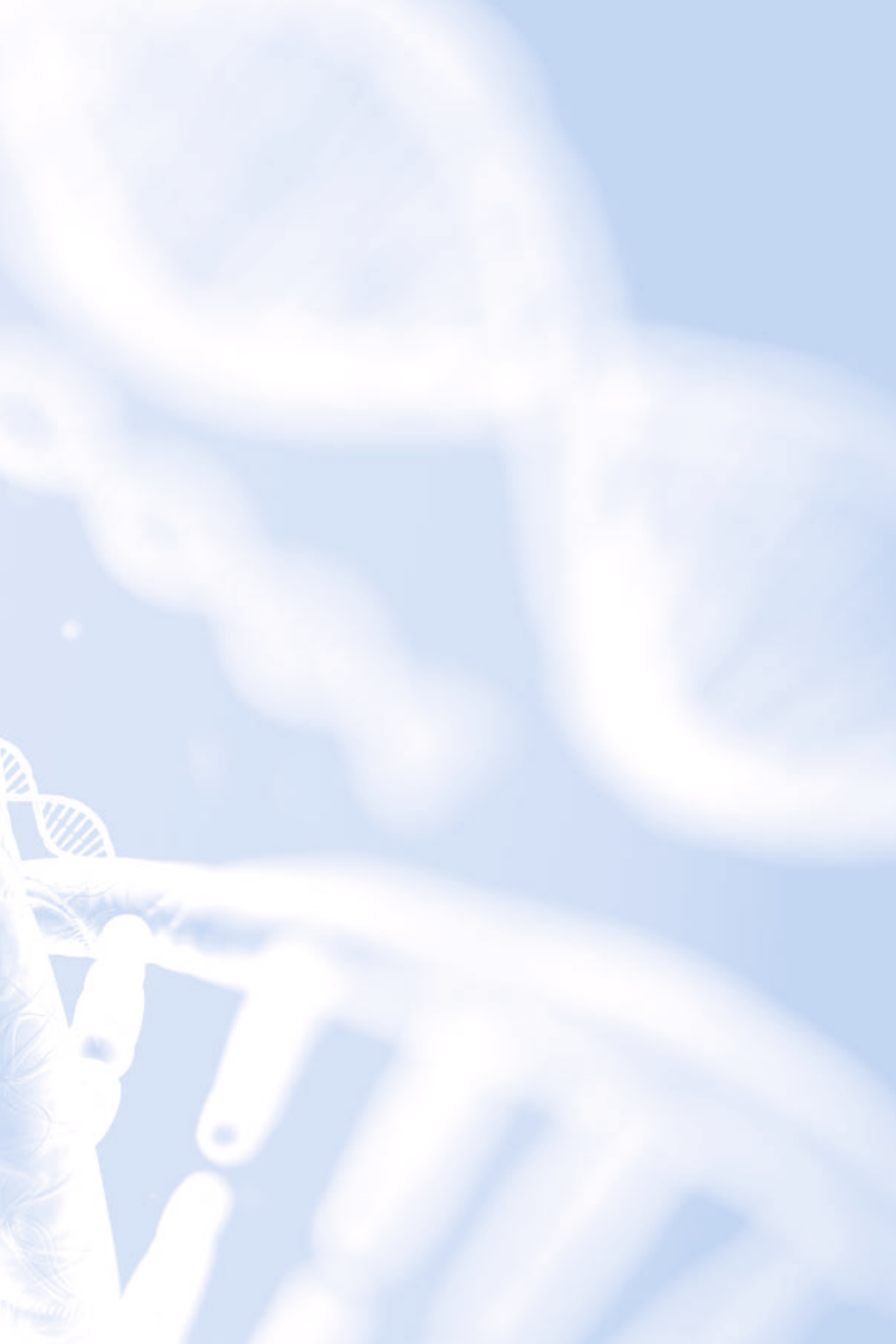
FRASER WRIGHT

Dr. Wright received his PhD in 1989 from the University of Toronto, Department of Biochemistry for work characterizing the interaction of complement with IgM, and completed post-doctoral studies at INSERM / CENG Grenoble, France in molecular immunology focused on antigen processing and presentation. He was awarded an CRCS/ MRC Scholarship gaining faculty appointment at the University of Toronto. In 1996 he moved to industry, first as a Scientist at Pasteur Sanofi working on the development of cancer immunotherapeutics, and then as Director of Development and Clinical Manufacturing at Avigen, a pioneering gene therapy company developing rAAV based investigational products for hemophilia and Parkinson's Diseases. In 2004 he returned to academia, establishing the Clinical Vector Core at the Center for Cellular and Molecular Therapeutics at Children's Hospital of Philadelphia, gaining faculty appointment at the University of Pennsylvania Perelman School of Medicine as professor of Pathology and Laboratory Medicine. Dr. Wright has contributed to several clinical development programs in gene therapy, including those for Luxturna and Kymriah, gene therapy products that were licensed in 2017, the first gene therapies for a genetic (RPE65 deficiency) and nongenetic (CAR-T immunotherapy) disease, respectively, approved in the United States. He is a Co-founder of Spark Therapeutics, served as Chief Technology Officer at Spark and subsequently at Axovant Gene Therapies, and is Founder and Principal at Wright Biologics Consulting.



STEPHEN ZOOG

Stephen Zoog, PhD is Executive Director and Head of BioAnalytical Sciences at BioMarin Pharmaceuticals, Inc. With >15 years drug development experience, Steve oversees the laboratories focused on assay development and sample testing for pharmacokinetics, immunogenicity, and biomarkers in both clinical trials and animal studies. He is an active participant in global health authority interactions regarding methods used in evaluation and development of small molecules, enzyme replacement therapies, gene therapies, and companion diagnostics.



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