

# Gene Therapy

## NAVIGATING UNFAMILIAR TERRITORY IN HEMOPHILIA

**Sunday, June 25, 2023**

1:15 pm – 2:30 pm local time

Room 510 - Level 5

## Program

### Welcome & Introduction

Prof. David Lillicrap, *Kingston, ON, Canada*

### Treating With Gene Therapy & Shared Decision-making: A Collaborative Expedition Through Uncertainties

Prof. Margareth Ozelo, *Campinas, SP, Brazil*

### Liver Health and Corticosteroid Use

Prof. Paolo Angeli, *Padua, Italy*

### Long-term Safety & Follow-up in Gene Therapy: What Do We Know?

Prof. David Lillicrap, *Kingston, ON, Canada*

### More Than Just Numbers: What Does Gene Therapy Really Mean for the Patient?

Prof. Barbara A. Konkle, *Seattle, WA, USA*

### Closing Remarks

Prof. David Lillicrap, *Kingston, ON, Canada*

### Q&A

Faculty & audience

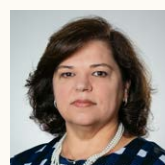
## Faculty



### Prof. David Lillicrap

*Queen's University, Kingston, ON, Canada*

David Lillicrap is Professor in the Department of Pathology and Molecular Medicine at Queen's University in Kingston, ON, Canada. His main research interests are focused on the immune response to factor VIII in Hemophilia A, the development and evaluation of novel therapies for Hemophilia A and the pathobiology of von Willebrand factor. He is a past-Chair of the WFH Research Committee and member of the Medical Advisory Board of the WFH. He is a past-member of Council of ISTH, and is currently one of the two Editors-in-Chief of the Journal of Thrombosis and Haemostasis.



### Prof. Margareth Ozelo

*University of Campinas, Campinas, SP, Brazil*

Margareth Ozelo is Professor at the Internal Medicine Department and Director of the Hematology Division at the University of Campinas (UNICAMP) in Campinas, São Paulo, Brazil. She is involved in several research projects including gene therapy for Hemophilia, risk factors for inhibitor development, immune tolerance induction and the management of musculoskeletal complications of Hemophilia and inherited platelet disorders. Prof. Ozelo is also Director of the WFH International Hemophilia Training Center from Hemocentro UNICAMP.



### Prof. Paolo Angeli

*University of Padua, Padua, Italy*

Paolo Angeli is Professor of Internal Medicine and Head of the Unit of Internal Medicine and Hepatology of the University of Padua, Italy. He is also Chair of the EASL-Clif Consortium and Editor-in-Chief of the Journal of Hepatology. Prof. Angeli has an interest in the pathophysiology and treatment of liver failure and liver transplantation. He has also contributed to guidelines/papers on the management of patients with decompensated cirrhosis.



### Prof. Barbara A. Konkle

*University of Washington School of Medicine, Seattle, WA, USA*

Barbara Konkle is Professor of Medicine in Hematology at the University of Washington in Seattle, WA, USA. She currently serves as the Chair of the Steering Committee for the WFH Gene Therapy Registry. Prof. Konkle has a long-standing interest in the genetics of bleeding disorders and recently led the 'My Life Our Future' project which genotyped over 11,000 patients and potential carriers of Hemophilia. Since 2001, she has also been participating in clinical trials of gene therapy for Hemophilia.