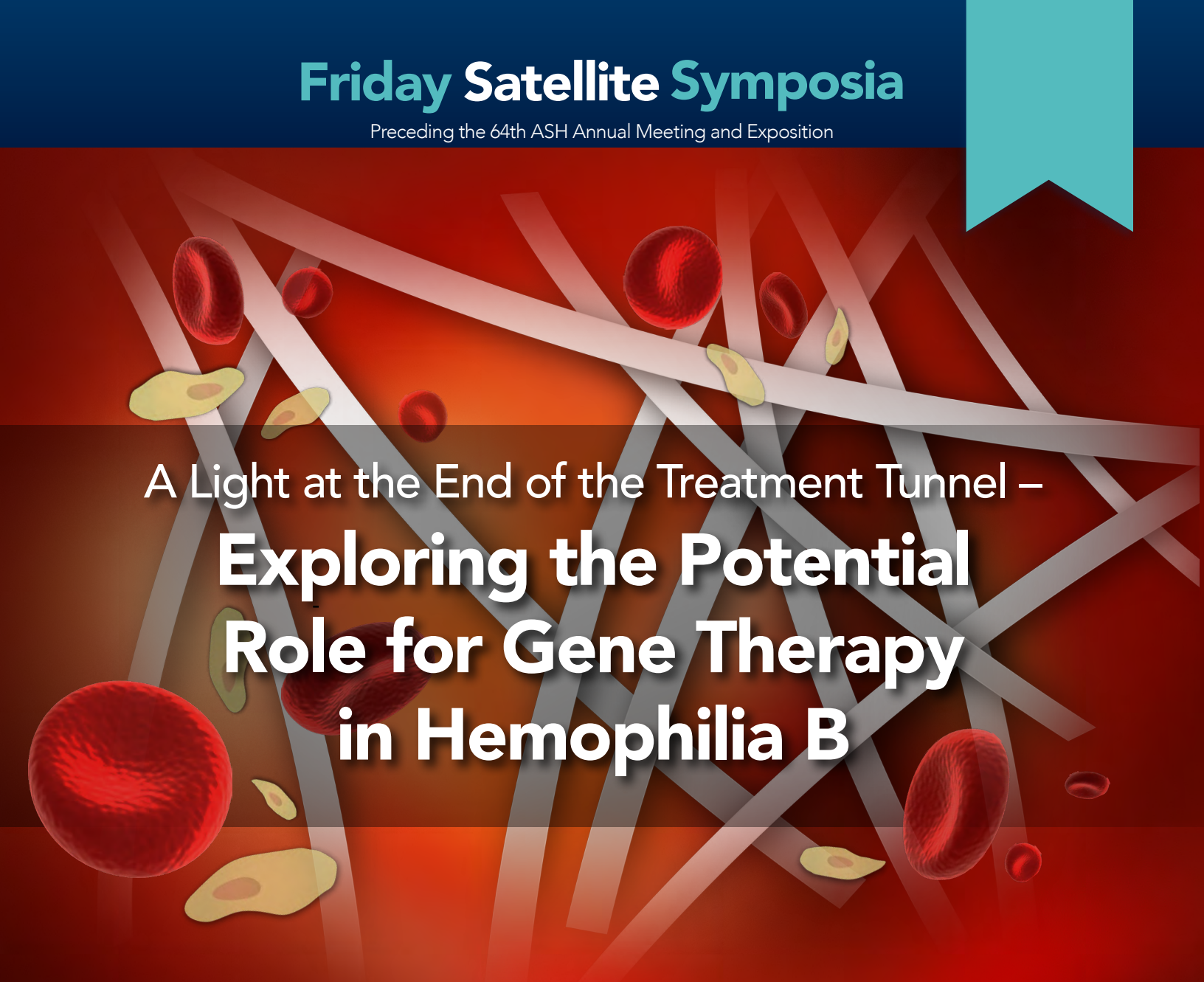


# Friday Satellite Symposia

Preceding the 64th ASH Annual Meeting and Exposition



## A Light at the End of the Treatment Tunnel – Exploring the Potential Role for Gene Therapy in Hemophilia B

**IN-PERSON + LIVE STREAMING SATELLITE SYMPOSIUM**

**FRIDAY, DECEMBER 9, 2022**

3:00 PM – 6:00 PM CST

**PROGRAM CHAIR**

Christine L. Kempton, MD, MSc

For more information, visit us at...

**gotoper.com**



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Dear Colleague:

On behalf of Physicians' Education Resource®, LLC (PER®), we would like to welcome you and thank you for choosing to attend **A Light at the End of the Treatment Tunnel – Exploring the Potential Role for Gene Therapy in Hemophilia B**.

The mainstay of therapy in hemophilia B is supplementation of the missing Factor IX (FIX) proteins. Several improved therapeutic agents have recently arrived on the market and hemophilia management is undergoing significant changes. Novel approaches to extend agent half-life are also in development, changing the treatment landscape for hemophilia. Despite recent improvements in treatment options, hemophilia B continues to have a significant negative impact on patients' functionality and health-related quality of life. Gene therapy for hemophilia B has the potential to reduce the treatment burden for patients and their care providers by eliminating the need for regular prophylaxis through long term expression of endogenous FIX at levels sufficient to provide protection from bleeding. Gene therapy for hemophilia presents a challenge to the existing structure of hemophilia treatment centers and requires enhanced collaboration and information sharing to maximize benefits and minimize risks.

This educational activity addresses the unmet needs in hemophilia B, including improving knowledge of the current standard of care and novel gene therapy strategies that may change the treatment landscape. In addition, it addresses the evolving treatment paradigm for hemophilia B, including the role of gene therapy and how this may change the way in which hemophilia care is delivered.

In working together with PER®, we strive to make this activity, along with all PER®'s accredited continuing education (CE) activities, useful and impactful for health care professionals involved with the care of patients with hemophilia B.

If there is anything additional that you need, if you experience an issue, or if you have an idea for a future activity that would help you in the care of your patients, please let us know.

We could not have our accredited CE activities without you, and we appreciate your commitment to learning about the latest advancements in the treatment of hemophilia B. Let's work together to make this an educational experience that will make a positive difference for our patients!

We hope you enjoy your experience at this first-class PER® satellite and that you can attend another PER® accredited CE activity soon.

Regards,

**Christine L. Kempton, MD, MSc**

Professor, Department of Hematology and Medical Oncology and Department of Pediatrics  
HoG Inc. Director's Chair in Hemostasis  
Director, Benign Hematology Program  
Director, Hemophilia of Georgia Center for Bleeding & Clotting Disorders of Emory  
Vice Chair for Faculty Development, Department of Hematology and Medical Oncology  
Emory University School of Medicine  
Atlanta, GA

**Jim Palatine, RPh, MBA**

President  
Physicians' Education Resource®, LLC  
Cranbury, NJ  
jpalatine@mjhlifesciences.com

## AGENDA

|         |                                                                |                                   |
|---------|----------------------------------------------------------------|-----------------------------------|
| 3:00 PM | Welcome and Introductions                                      | Christine L. Kempton, MD, MSc     |
| 3:05 PM | Current Approaches for the Management of Hemophilia B          | Annette Von Drygalski, MD, PharmD |
| 3:30 PM | Audience Q/A                                                   | All Faculty                       |
| 3:35 PM | A Closer Look at the Impact of Hemophilia B on Quality of Life | Steven Pipe, MD                   |
| 3:55 PM | Audience Q/A                                                   | All Faculty                       |
| 4:05 PM | New Treatment Paradigms: Gene Therapy in Hemophilia B          | Andrew D. Leavitt, MD             |
| 4:30 PM | Audience Q/A                                                   | All Faculty                       |
| 4:40 PM | Implementing Gene Therapy for Hemophilia B Into Practice       | Steven Pipe, MD                   |
| 5:05 PM | Audience Q/A                                                   | All Faculty                       |
| 5:15 PM | Patient-Centered Multidisciplinary Approaches to Optimal Care  | Christine L. Kempton, MD, MSc     |
| 5:40 PM | Audience Q/A                                                   | All Faculty                       |
| 5:50 PM | Closing Remarks                                                | All Faculty                       |
| 6:00 PM | Adjourn                                                        |                                   |

### Upcoming PER® Conferences

2022

Inaugural International Congress Pediatric Oncology

December 16

2023

|                                                                                                                                  |                      |                |
|----------------------------------------------------------------------------------------------------------------------------------|----------------------|----------------|
| <i>The 1st Annual Hawaii Lung: A Multidisciplinary Case-Based Conference™</i>                                                    | Honolulu, HI         | January 21     |
| <i>20th Annual Winter Lung Conference®</i>                                                                                       | Hollywood, FL        | February 3-5   |
| <i>19th Annual International Symposium on Melanoma and Other Cutaneous Malignancies®</i>                                         | Dallas, TX           | February 4     |
| <i>27th Annual International Congress on Hematologic Malignancies®</i>                                                           | Miami Beach, FL      | February 23-26 |
| <i>40th Annual Miami Breast Cancer Conference®</i>                                                                               | Miami Beach, FL      | March 2-5      |
| <i>NYGU™ 16th Annual Interdisciplinary Prostate Cancer Congress® and Other Genitourinary Malignancies</i>                        | New York, NY         | March 10-11    |
| <i>8th Annual School of Gastrointestinal Oncology®</i>                                                                           | Orlando, FL          | May 6          |
| <i>7th Annual International Congress on Oncology &amp; Pathology™: Towards Harmonization of Pathology and Oncology Standards</i> |                      | May 6          |
| <i>14th Annual International Symposium on Ovarian Cancer and Other Gynecologic Malignancies</i>                                  |                      | May 13         |
| <i>22nd Annual International Congress on the Future of Breast Cancer® East</i>                                                   | New York, NY         | July 15-16     |
| <i>24th Annual International Lung Cancer Congress®</i>                                                                           | Huntington Beach, CA | July 27-29     |
| <i>22nd Annual International Congress on the Future of Breast Cancer® West</i>                                                   | San Diego, CA        | July 28-29     |
| <i>20th Annual Meeting of the International Society of Gastrointestinal Oncology®</i>                                            | Tempe, AZ            | October 13-14  |
| <i>21st Annual School of Breast Oncology®</i>                                                                                    | Atlanta, GA          | November 2-4   |
| <i>41st Annual CFS®: Innovative Cancer Therapy for Tomorrow®</i>                                                                 | New York, NY         | November 8-10  |
| <i>18th Annual New York Lung Cancers Symposium®</i>                                                                              | New York, NY         | November 11    |



Virtual Conference



In-Person + Virtual Conference



In-Person Conference

### Target Audience

This educational activity is directed toward hematologists and hematologist-oncologists. Pediatricians, nurses, nurse practitioners, physician assistants, and other clinicians involved in the management of patients with hemophilia B also are invited to attend.

### Learning Objectives

At the conclusion of this activity, you should be better prepared to:

- Discuss the burden of disease for patients with hemophilia B
- Outline the current standard of care for patients with hemophilia B
- Assess clinical trial data for gene therapy for hemophilia B
- Describe models for implementation of gene therapy for hemophilia B into clinical practice
- Apply strategies to select appropriate patients for gene therapy
- Review the role of the multidisciplinary team in the management of patients receiving gene therapy for hemophilia B

### Slides/e-Syllabus

Final slides will be uploaded to the e-syllabus on the PER<sup>®</sup> website by 12 PM EST the next business day after the presentation. To access these materials, please visit <https://www.gotoper.com/hemb1222iss-esyl>.

### How to Request Credit

To complete the online evaluation and receive a certificate of credit or participation, please visit

<https://www.gotoper.com/hemb1222iss-eval>.

The online evaluation will be available at the end of the day and the request for credit will be available beginning December 9, 2022 and will remain available until January 23, 2023.

### Accreditation/Designation of Credit

Physicians' Education Resource<sup>®</sup>, LLC is accredited by the Accreditation Council for Continuing Medical Education (ACCME) to provide continuing medical education for physicians.

Physicians' Education Resource<sup>®</sup>, LLC designates this live activity for a maximum of 3.0 *AMA PRA Category 1 Credits™*.

Physicians should claim only the credit commensurate with the extent of their participation in the activity.

### Acknowledgement of Educational Grant Support

This activity is supported by an educational grant from CSL Behring LLC.

Thank you for attending **A Light at the End of the Treatment Tunnel – Exploring the Potential Role for Gene Therapy in Hemophilia B!**

To register for upcoming accredited CE live activity or to view our online accredited CE activities, please visit [www.gotoper.com](http://www.gotoper.com).

We appreciate your attendance and look forward to seeing you at future PER<sup>®</sup> accredited CE activities.

### Faculty, Staff, and Planners' Disclosures

The following individuals have no relevant financial relationships with ineligible companies to disclose: the staff of Physicians' Education Resource®, LLC.

The following individuals have relevant financial relationships with ineligible companies to disclose\*:

| Faculty                           | Grant/Research Support                                         | Consultant                                                                                                                                                                                                                                                                                          | Speakers' Bureau | Other |
|-----------------------------------|----------------------------------------------------------------|-----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|------------------|-------|
| Christine L. Kempton, MD, MSc     |                                                                | BioMarin Pharmaceutical Inc; Genentech; Pfizer Inc; Sanofi US; Spark Therapeutics; Takeda                                                                                                                                                                                                           |                  |       |
| Andrew D. Leavitt, MD             | BioMarin Pharmaceutical Inc; Pfizer Inc; Sangamo Therapeutics; | BioMarin Pharmaceutical Inc; Pfizer, Inc                                                                                                                                                                                                                                                            |                  |       |
| Steven Pipe, MD                   | Siemens Corporation; YewSavin, Inc                             | Apotex Pharmaceutical Holdings Inc; ASC Therapeutics; Bayer; BioMarin Pharmaceutical Inc; CSL Behring; Freeline Therapeutics Holdings; Genentech; GeneVentiv Therapeutics; HEMA Biologics; LFB; Novo Nordisk; Pfizer Inc; Regeneron Pharmaceuticals, Inc; Roche; Sanofi; Spark Therapeutics; Takeda |                  |       |
| Annette Von Drygalski, MD, PharmD | Pfizer, Sanofi                                                 | CSL-Behring, Sanofi, Takeda, Uniqure, Genentech, Regeneron, ASC, Biomarin, Novo Nordisk                                                                                                                                                                                                             |                  |       |

*\*Information accurate at time of printing*

PER® has identified and mitigated all COI prior to the start of this activity by using a multistep process.

### Off-Label Disclosure and Disclaimer

This accredited CE activity may or may not discuss investigational, unapproved, or off-label uses of drugs. Participants are advised to consult prescribing information for any products discussed. The information provided in this accredited activity is for continuing medical and/or nursing education purposes only and is not meant to substitute for the independent clinical judgment of a physician or nurse relative to the diagnostic, treatment, and management options for a specific patient's medical condition.

The opinions expressed in the content are solely those of the individual faculty members and do not reflect those of PER® or any of the companies that provided commercial support for this activity.

### Physician Payments Sunshine Act

PER® complies with the Physician Payments Sunshine Act as a part of the Affordable Care Act (ACA). Accordingly, we may be required to collect information on transfers of value provided to any covered recipient under the ACA.

## Faculty

**Christine L. Kempton, MD, MSc**

Professor, Department of Hematology and Medical Oncology and Department of Pediatrics  
 HoG Inc. Director's Chair in Hemostasis  
 Director, Benign Hematology Program  
 Director, Hemophilia of Georgia Center for Bleeding & Clotting Disorders of Emory  
 Vice Chair for Faculty Development, Department of Hematology and Medical Oncology  
 Emory University School of Medicine  
 Atlanta, GA

Board certified in hematology and internal medicine, Christine L. Kempton, MD, MSc, specializes in the treatment of hemostatic and thrombotic disorders as well as platelet disorders. Dr Kempton leads the clinical care team at the Hemophilia of Georgia Center for Bleeding and Clotting Disorders of Emory, located at Emory University Hospital Midtown. She began practicing with Emory Healthcare as a network physician in 2005.

Dr Kempton completed her medical degree at Mayo Clinic Alix School of Medicine. She completed her training in internal medicine at Oregon Health and Science University where she was chief resident. Her subspecialty training in hematology was completed at University of North Carolina at Chapel Hill. She has also earned a master of science in clinical research from Emory University.

Dr Kempton's research interests center on complications of hemophilia and novel treatments of congenital and acquired bleeding disorders.

**Andrew D. Leavitt, MD**

Director, Program for Noncancerous Blood Disorders  
 Co-Director, Hemophilia Treatment Center  
 University of California, San Francisco  
 San Francisco, CA

Andrew D. Leavitt, MD, is director of University of California, San Francisco's (UCSF) program for noncancerous blood disorders and co-director of the UCSF Hemophilia Treatment Center. He cares for patients with nonmalignant blood diseases, including bleeding disorders, such as hemophilia and von Willebrand disease, platelet function disorders, disorders of insufficient platelets, venous thromboembolic disorders (which involve blood clots), neutropenia (reduced numbers of the white blood cells called neutrophils), and anemia.

Leavitt is medical director of UCSF's laboratory for blood and marrow transplant research. He is internationally known for his studies of the development of megakaryocytes, large cells in the bone marrow that divide to produce blood platelets. His research focuses on improving treatment and outcomes for patients with venous thromboembolic diseases.

Leavitt obtained his medical degree at Harvard Medical School. He completed a residency in internal medicine and served as chief resident at the University of Michigan. At UCSF, he completed a fellowship in hematology and oncology, followed by a fellowship in transfusion medicine. He joined the UCSF faculty in 1992.

Leavitt has served on the editorial boards of *Blood* and *The Journal of Clinical Investigation*. He currently serves on the editorial board of the *Journal of Clinical and Translational Science*.

## Faculty

**Steven Pipe, MD**

Professor of Pediatrics and Pathology  
 Director, Special Coagulation Laboratory  
 Laurence A. Boxer, MD Research Professor of Pediatrics and Communicable Diseases  
 Medical Director, Pediatric Hemophilia and Coagulation Disorders Program  
 University of Michigan  
 Ann Arbor, MI

Steven Pipe, MD, is a professor of pediatrics and pathology. He is also the Laurence A. Boxer, MD, Research Professor of Pediatrics and Communicable Diseases at the University of Michigan, in Ann Arbor. He is the medical director of the Pediatric Hemophilia and Coagulation Disorders Program and the director of the special coagulation laboratory. His clinical interests include bleeding and thrombotic disorders and congenital vascular anomalies. Dr Pipe also directs a basic research lab investigating coagulation factor VIII and the molecular mechanisms of hemophilia A. He has been actively involved in clinical trials with novel therapeutics for hemophilia including gene therapy. He was the 2015 recipient of the Leadership in Research Award from the National Hemophilia Foundation. He has served on the board of directors for the Hemostasis and Thrombosis Research Society, as chair of the board of directors for the American Thrombosis and Hemostasis Network, and currently serves as chair of the Medical and Scientific Advisory Committee to the National Hemophilia Foundation.

**Annette Von Drygalski, MD, PharmD**

Director, Hemophilia and Thrombosis Treatment Center  
 Professor of Medicine  
 UC San Diego Health  
 San Diego, CA

Annette Von Drygalski, MD, PharmD, is a board-certified hematologist who cares for people with diseases of the blood, spleen, and lymph. She directs the Hemophilia and Thrombosis Treatment Center at UC San Diego Health. In this role, Dr Von Drygalski developed translational and clinical research interests addressing the evolving needs of aging patients with hemophilia, such as cardiovascular and joint disease.

As an associate professor in the department of medicine, Dr Von Drygalski instructs medical students, residents, and fellows at UC San Diego School of Medicine. She has a strong basic and translational research interest in hemophilia, and is an adjunct assistant professor at the Scripps Research Institute. She is the institutional leading investigator of several international clinical trials exploring long-acting or novel clotting factor products, and studies hypertension, bone, and joint disease in patients with hemophilia.

Dr Von Drygalski completed fellowship training in hematology/oncology at UC San Diego School of Medicine and a residency in internal medicine at the Medical College of Wisconsin. She earned her medical and pharmaceutical doctorates from the Ludwig Maximilian University in Munich, Germany. She holds a registered in musculoskeletal (RMSK) sonography certification and is board certified in hematology and medical oncology.

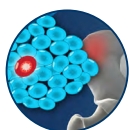
Dr Von Drygalski is the recipient of a Bayer Hemophilia Awards Program early career investigator award. She has also received a career development award from the National Hemophilia Foundation for studies of novel bypassing strategies in hemophilia and the role of fibrinolysis in hemophilic joint disease.

# Did you miss one of our sessions?

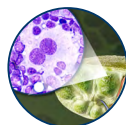
Check out the On-Demand Video Updates  
from the Annual Hematology Meeting

## —Rapid Archive from→ **NEW ORLEANS**

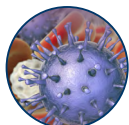
Archives will be available on December 10th



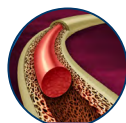
**Optimized use of MRD in  
Hematologic Malignancies**



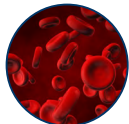
**Maximizing Clinical Practice  
for Hodgkin Lymphoma**



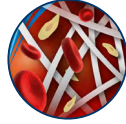
**State-of-the-Art CMV Care**



**State-of-the-Art Care for  
MF and PV**



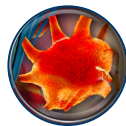
**Emerging Treatments for  
Anemia in CKD**



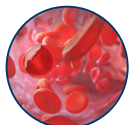
**Exploring Gene Therapy in  
Hemophilia B**



**Holistic Approaches in  
Sickle Cell Disease Treatment**



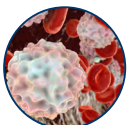
**Diagnosing Uncommon  
Hematologic Disorders**



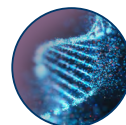
**Diagnosis and Treatment of  
Hemolytic Anemias**



**Novel Therapies to Prevent  
and Treat GvHD**



**Optimized Treatments for  
ALL in Adolescents and Adults**



**Novel Treatments for Indolent  
and Aggressive B-Cell Lymphomas**



**Multidisciplinary Management  
of BPDCN**



**Treating Transfusion-Related  
Iron Overload**



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