

ACTIVITY AGENDA & FACULTY

7:00–7:05 PM

Welcome and Introductions

Steven W. Pipe, MD

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

7:05–7:20 PM

Overview of Gene Therapy for Treatment of Hemophilia: Vector and Transgene Optimization

Steven W. Pipe, MD

7:20–7:35 PM

Update on Clinical Trials of Gene Therapy in Hemophilia A and Hemophilia B

Guy Young, MD

Director, Hemostasis and Thrombosis Program
Professor of Pediatrics (Clinical Scholar)
Keck School of Medicine of USC, Los Angeles, California

7:35–7:50 PM

What Does Success Look Like? Outcomes Beyond Clinical Trial Endpoints

Guy Young, MD

7:50–8:10 PM

What Does Readiness Look Like? Preparing HTC's for Gene Therapy

Steven W. Pipe, MD and Guy Young, MD

8:10–8:30 PM

Question and Answer Session

TARGET AUDIENCE

This activity is intended for physicians (hematologists), nurse practitioners, physician assistants, nurses managing patients with hemophilia, and scientists with an interest in basic, translational, and clinical research in hemophilia around the world.

PROGRAM OVERVIEW

With the expected approval of the first gene therapy for hemophilia in the next few months, it is important that clinicians are prepared for implementation in their respective hemophilia treatment centers (HTCs). During this 90-minute session, Drs. Steven Pipe and Guy Young will provide an overview of gene therapy for treatment of hemophilia along with an update on clinical trials in hemophilia A and B. They will also discuss what success looks like and how HTCs should be preparing for the advent of gene therapy. The presentation will conclude with a live question and answer session between attendees and Drs. Pipe and Young.

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ACCREDITATION STATEMENT

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CREDIT DESIGNATION

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