



**THE SAFE AND
EFFECTIVE PRACTICE
OF AAV GENE THERAPY**

**FRIDAY, SEPTEMBER 20, 2024
8:30 AM – 6:30 PM EDT**

EVENT REGISTRATION:
<https://cigt-gene-therapy.eventbrite.com>

OVERVIEW

Join the Clinical In Vivo Gene Therapy (CIGT) team at Children's Hospital of Philadelphia for an exciting one-day educational symposium, as we provide fundamental knowledge on the safe and effective practice of adeno-associated virus (AAV) gene therapy across medical specialties.

In this full-day, in-person seminar, attendees will gain an understanding of:

- In vivo gene therapy scientific foundations and approaches
- AAV commercial products
- AAV toxicities
- The practice of in vivo gene therapy

MEETING LOCATION

This is an in-person educational event at the Children's Hospital of Philadelphia, Hub for Clinical Collaboration, Auditorium, 3500 Civic Center Blvd, Philadelphia, PA 19104



REGISTRATION FEES

The in-person registration fee of \$40 includes continental breakfast, lunch, breaks, and the evening reception.

To register for the event, please visit: <https://cigt-gene-therapy.eventbrite.com>

OVERNIGHT ACCOMMODATIONS

For those wishing to stay overnight in Philadelphia, The Study is located just a few blocks from Children's Hospital. Participants are required to make their own hotel reservations by calling The Study directly at 215-387-1400. There is no room block or discounted rate available and rooms are subject to availability.

CANCELLATION POLICY

Children's Hospital of Philadelphia reserves the right to cancel or postpone any course due to any unforeseen circumstances. Cancellations must be received in writing by Friday, September 6, 2024.

CONTACT

Bernadette McGlynn, Clinical In Vivo Gene Therapy, mcglynnb@chop.edu

<https://www.chop.edu/centers-programs/clinical-vivo-gene-therapy>

AGENDA | Friday, September 20, 2024

8:00 a.m. **Registration and Continental Breakfast**

8:45 a.m. **SESSION 1: INTRODUCTION TO IN VIVO GENE THERAPY**

State of the Art and Science of In Vivo Gene Therapy
Lindsey George, MD

Intro to AAV Virology and Vectors
Beverly Davidson, PhD

Manufacturing
Johannes C. M. van der Loo, PhD

Immune Responses to AAV
Bhavya S. Doshi, MD

Discussion/Questions

Break

11:00 a.m. **SESSION 2: IN VIVO GENE THERAPY COMMERCIAL PRODUCTS**

Ophthalmology
Tomas S. Aleman, MD

Hematology
Benjamin J. Samelson-Jones, MD, PhD

Neuromuscular
Susan Matesanz, MD

Cardiology
Joseph Rossano, MD, MS, FAAP, FACC

Metabolism
Rebecca Ahrens-Nicklas, MD, PhD

Discussion/Questions

1:00 p.m. **Lunch**

2:00 p.m

SESSION 3: AAV TOXICITIES

Short-term Toxicities

Amy Waldman, MD

Cardiac Toxicity

Kimberly Y. Lin, MD

Hepatotoxicity

Benjamin J. Samelson-Jones, MD, PhD

Neurotoxicity

Juliette Hordeaux, DVM, PhD, DECVP

Long-term Concerns

Denise E. Sabatino, PhD

Discussion/Questions

Break

4:00 p.m.

SESSION 4: THE PRACTICE OF GENE THERAPY

Counseling and Informed Consent for Gene Therapy

Taylor Kaschak, RN

Do We Need to Worry About Vector Shedding?

Juliana Small, PhD

Considerations When Onboarding Licensed Vectors

Jennifer Harding, MPA

Strategies for Obtaining Insurance Approval

Ben Schimeneck, MBA

Pharmacy Perspectives

Richard Dyke

Long-term Follow Up and Registries

Lindsey A. George, MD

Discussion/Questions/Closing Remarks

5:30 p.m.

Reception

Unless otherwise noted, faculty is from Children's Hospital of Philadelphia and/or the Perelman School of Medicine at the University of Pennsylvania.

FACULTY

Beverly L. Davidson, PhD

*Director, Raymond G. Perelman Center for Cellular and Molecular Therapeutics
Chief Scientific Strategy Officer
Children's Hospital of Philadelphia
Professor of Pathology and Laboratory Medicine
Perelman School of Medicine at the University of Pennsylvania*

Lindsey A. George, MD

*Assistant Professor of Pediatrics, University of Pennsylvania School of Medicine
Director, Clinical In Vivo Gene Therapy, Children's Hospital of Philadelphia
Attending Physician, Division of Hematology, Children's Hospital of Philadelphia*

Johannes C.M. van der Loo, PhD

Director, Viral Vector Core, Children's Hospital of Philadelphia

Bhavya S. Doshi, MD

*Attending Physician, Division of Hematology, Children's Hospital of Philadelphia
Instructor of Pediatrics, Perelman School of Medicine, University of Pennsylvania*

Tomas S. Aleman, MD

*Associate Professor of Ophthalmology, Perelman School of Medicine, University of Pennsylvania
Director, Retinal Structure and Function Laboratory
Attending Physician, Division of Pediatric Ophthalmology, Children's Hospital of Philadelphia*

Benjamin J. Samelson-Jones, MD, PhD

*Associate Director, Clinical In Vivo Gene Therapy
Assistant Professor of Pediatrics, Perelman School of Medicine, University of Pennsylvania
Attending Physician, Division of Hematology, Children's Hospital of Philadelphia*

Susan Matesanz, MD

*Medical Director, Clinical In Vivo Gene Therapy
Attending Physician, Division of Neurology, Children's Hospital of Philadelphia*

Joseph Rossano, MD, MS, MHCM

*Chief, Division of Cardiology, Children's Hospital of Philadelphia
Co-Executive Director, Cardiac Center
Jennifer Terker Endowed Chair in Pediatric Cardiology
Professor of Pediatrics, Perelman School of Medicine, University of Pennsylvania*

Rebecca Ahrens-Nicklas, MD, PhD

*Assistant Professor of Pediatrics, Perelman School of Medicine, University of Pennsylvania
Attending Physician, Human Genetics and Metabolism, Children's Hospital of Philadelphia*



Amy Waldman, MD

Medical Director, Leukodystrophy Center Associate Director, Clinical In Vivo Gene Therapy Associate Professor of Neurology, Perelman School of Medicine, University of Pennsylvania Attending Physician, Division of Neurology, Children's Hospital of Philadelphia

Kimberly Y. Lin, MD

Medical Director, Pediatric Cardiomyopathy Program, Children's Hospital of Philadelphia Cardiac Director, Penn-CHOP Friedreich Ataxia Center of Excellence Co-Director, Penn-CHOP Joint Familial Cardiomyopathy Program Associate Professor of Pediatrics, Perelman School of Medicine, University of Pennsylvania Attending Physician, Division of Cardiology

Juliette Hordeaux, DVM, PhD, DECVP

Executive Director, Translational Research, University of Pennsylvania Gene Therapy Program Doctor of Veterinary Medicine DiplECVP Pathologist

Denise E. Sabatino, PhD

Investigator, Division of Hematology, Children's Hospital of Philadelphia Research Professor of Pediatrics, Perelman School of Medicine, University of Pennsylvania

Taylor Kaschak, RN

Nurse Navigator, Children's Hospital of Philadelphia Registered Nurse, Children's Hospital of Philadelphia

Juliana Small, PhD

Senior Regulatory Affairs Translational Specialist, Clinical In Vivo Gene Therapy, Children's Hospital of Philadelphia

Jennifer Harding, MPA

Administrative Director, Clinical In Vivo Gene Therapy, Children's Hospital of Philadelphia

Ben Schimeneck, MBA

Administrative Director, Physician Practice Administration, Children's Hospital of Philadelphia

Richard Dyke

Director, Pharmacy Business Operations, Children's Hospital of Philadelphia

