

HYDROCEPHALUS ASSOCIATION & CINCINNATI CHILDREN'S HOSPITAL RESEARCH WORKSHOP

Developing Non-Invasive Hydrocephalus Therapies:
Advancing Towards Clinical Trials

Friday, April 12, 2024

8:00 AM – 5:30 PM

Saturday, April 13, 2024

8:00 AM – 2:00 PM

Cincinnati Children's Hospital

T Building Conference Room

240 Albert Sabin Way

Cincinnati, OH 45229



*Attendees will stay at the Graduate Hotel, 151 Goodman Street,
Cincinnati, Ohio 45219 (Walking distance to workshop venue)*



WELCOME LETTER

From HA President and CEO, Diana Gray
and Cincinnati Children's host, Dr. Francesco Mangano



Welcome to the Hydrocephalus Association (HA) and Cincinnati Children's Hospital 2024 Research Workshop.

This workshop builds directly upon the momentum of the 2023 workshop, responding to the urgent need for non-invasive therapies for hydrocephalus. Your participation emphasizes our shared dedication to pushing the boundaries of hydrocephalus research. We aim to leverage this collective energy to foster innovation and address HA's critical patient-centered [Community Research Priorities](#) with a particular emphasis on:

- Developing new treatments that do not require brain surgery to manage hydrocephalus.
- Developing new one-time treatments to manage hydrocephalus (i.e. permanent treatments that do not require additional interventions).
- Developing ways to prevent the development of hydrocephalus.

Our focus is on advancing non-invasive treatments for hydrocephalus through consensus models, endpoint measurements, and external collaborations. The program was carefully created to contribute significantly to the advancement of preclinical and clinical trials and provide foresight for regulatory and funding processes. There will be direct opportunities to engage in conversations with governance agencies and industry liaisons, offering a valuable chance to gain insights into FDA expectations and leverage the expertise of companies.

We will integrate the priorities identified in this workshop with those from the 2023 workshop, resulting in the creation of a comprehensive white paper slated for publication in a peer-reviewed journal. The outcomes of this workshop will contribute significantly to our mission of finding a cure for hydrocephalus and improving the lives of those impacted by the condition.

Thank you for being an integral part of this critical initiative. Together, we are driving progress and innovation in hydrocephalus research.

Sincerely,

Diana Gray, MA
President and Chief Executive Officer
Hydrocephalus Association

Francesco Mangano, DO, FACS,
FAAP, FACOS
Division Chief of Pediatric Neurosurgery
Cincinnati Children's Hospital Medical Ctr

Planning Committee

James (Pat) McAllister, PhD
Washington University in St. Louis

Bonnie Blazer-Yost, PhD
Indiana University

Ramin Eskandari, MD
Medical University of South Carolina

Francesco Mangano, DO
Cincinnati Children's Hospital
Medical Center

June Goto, PhD
Cincinnati Children's Hospital
Medical Center

Lauren Jantzie, PhD
Johns Hopkins University

Maria Garcia Bonilla, PhD
Virginia Commonwealth University

Mark Johnson, MD, PhD
UMass Chan Medical School

HA & CCHMC Research Workshop Agenda

Friday, April 12, 2024

8:00 AM **Breakfast**

8:20 AM **Opening and welcoming remarks**

Francesco Mangano, DO
Cincinnati Children's Hospital Medical Center

Diana Gray, MA
Hydrocephalus Association

Monica Chau, PhD
Hydrocephalus Association

SESSION 1: CREATING SCIENTIFIC CONSENSUS

WHAT ARE THE IDEAL ANIMAL MODELS AND TECHNIQUES TO INDUCE HYDROCEPHALUS?

9:25 AM **MODERATOR: Pat McAllister, PhD**
Washington University in St. Louis
Introduction to the round table format

9:30 AM **Acquired hydrocephalus models overviews**
Marc del Bigio, MD, PhD
University of Manitoba
Animal models of hydrocephalus: what is their translational utility for development of therapies?

9:45 AM **SPEAKERS**
Pat McAllister, PhD
Washington University in St. Louis
Kaolin-induced hydrocephalus models

Jose Peiro, MD, PhD
Cincinnati Children's Hospital Medical Center
Fetal intracisternal BioGlue for a new model of obstructive hydrocephalus in large animal research

Lauren Jantzie, PhD
Johns Hopkins University
The essential role of inflammation in modeling PPHP secondary to IVH

Brandon Miller, MD, PhD
UTHealth Houston
Using blood vs. hemoglobin for IVH research

10:15 AM **Consensus building: Open discussion**

10:40 AM **Break**

10:55 AM **Genetic models overviews**

June Goto, PhD
Cincinnati Children's Hospital Medical Center
Genetic models of congenital hydrocephalus

Mark Johnson, MD, PhD
UMass Memorial Health;
Decoding iNPH: Mechanistic Insights from Genetically Engineered Mice

11:10 AM **Consensus building: Open discussion**

WHAT ENDPOINT MEASUREMENTS SHOULD WE BE USING?

MODERATOR: Bonnie Blazer Yost, PhD
Indiana University – Purdue University Indianapolis (IUPUI)

11:25 AM **Harriet Kamendi, PhD**
Kandih BioScience
FDA toxicology expectations

Charles V. Vorhees, PhD
University of Cincinnati
Behavioral testing paradigms as hydrocephalus endpoint measures

Diana Lindquist, PhD
Cincinnati Children's Hospital Medical Center
Translating MRI to the preclinical realm: advanced rodent neuroimaging for the evaluation of hydrocephalus

11:45 AM **Consensus building: Open discussion**

12:10 PM **Lunch**

SESSION 2: PATIENT PERSPECTIVE

WHAT MOTIVATES PATIENTS TO PARTICIPATE IN CLINICAL STUDIES?

MODERATOR: Samantha Lanjewar, PhD
Hydrocephalus Association

1:10 PM **Denise Berman, MS**
Alejandro Rojas, PhD
Ananke Therapeutics

Lynn Beirl, BA
Flourishing in Mid-Life Women's Hydrocephalus Group

HA & CCHMC Research Workshop Agenda (cont.)

SESSION 3: LESSONS FROM CURRENT & PAST CLINICAL TRIALS

- MODERATOR: David Limbrick, MD, PhD**
Virginia Commonwealth University
- 1:50 PM **David Limbrick, MD, PhD**
Virginia Commonwealth University
ESTHI trial and lessons learned
- 2:15 PM **Francesco Mangano, DO**
Cincinnati Children's Hospital Medical Center
Governance in clinical trials: DSMB
- 2:30 PM **Robin Ohls, MD, PhD**
University of Utah
Phase I-III trials in preterm infants using Darbepoetin
- 2:55 PM **Bonnie Blazer Yost, PhD**
Indiana University – Purdue University Indianapolis (IUPUI)
Perspectives from kidney disease clinical trials and navigating the patent process for hydrocephalus treatment
- 3:20 PM **Break**
- 3:35 PM **Shenandoah (Dody) Robinson, MD**
Johns Hopkins University
Overcoming regulatory barriers on the way to Phase I: A clinical trial of EPO+MLT in preterm infants with severe IVH
- 4:00 PM **Mark Luciano, MD**
Johns Hopkins University
Minority access to Neurosurgery: iNPH as a case study

SESSION 4: INDUSTRY PERSPECTIVE

- MODERATOR: Francesco Mangano, DO**
- 4:25 PM **Mike McBreen**
Integra LifeSciences
How we partner to help patients: an industry perspective

SESSION 5: TAKEAWAYS ON HOW TO MOVE TO CLINICAL TRIALS AND BEYOND

- 5:00 PM **MODERATOR: Lauren Jantzie, PhD**
Johns Hopkins University
How can we better design preclinical trials with a focus towards clinical translation?
- 5:20 PM **End of program day**
- 6:30 PM **Group dinner (invitation only)**
hosted by Integra LifeSciences
Via Vite Ristorante
520 Vine Street
Cincinnati, OH 45202
(513) 721-8483

HA & CCHMC Research Workshop Agenda (cont.)

Day 2, April 13

8:00 AM **Breakfast**

9:00 AM **Opening and welcoming remarks**

SESSION 6: ROADMAP TO ADVANCING TO CLINICAL TRIALS AND BEYOND

Regulatory Aspect

MODERATOR: Ramin Eskandari, MD
Medical University of South Carolina (MUSC)

8:30 AM **FDA speaker**
Food and Drug Administration (FDA)
Center for Drug Evaluation and Research (CDER)
Advancing to clinical trials

9:00 AM **Bruce Mackler, PhD, JD**
The drug discovery process and the role of the academic researcher

9:25 AM **Harriet Kamendi, PhD**
Kandih BioScience
FDA toxicology: planning preclinical studies

University IP offices

9:50 AM **Carole Burns, PhD**
UMass Chan Medical School BRIDGE Office of Innovation and Business Development
Bridging the Gap: A new paradigm for advancing academic innovation

10:15 AM **Justin Levy, JD & Jon Brophy, MSE**
Cincinnati Children's Innovation Ventures
Technology licensing basics for medical devices and therapeutics in hydrocephalus

10:40 AM **Break**

SESSION 7: FUNDERS' SUPPORT

Government Funding

MODERATOR: Carolyn Harris, PhD
Wayne State University

11:00 AM **Jill Morris, PhD**
National Institutes of Health (NIH), National Institute of Neurological Disorders and Stroke (NINDS)
NINDS funding opportunities supporting translational research

11:25 AM **Cecilia Dupecher, PhD**
Department of Defense (DoD), Congressionally Directed Medical Research Programs (CDMRP)
Department of Defense, Congressionally Directed Medical Research Programs and support for clinical trials

Private Funding

11:50 AM **Scott Davis, PhD**
The Sontag Foundation
The role of investment groups to advance therapies

12:15 PM **Monica Chau, PhD**
Hydrocephalus Association
Hydrocephalus Association's role in supporting scientists

12:35 PM **Lunch and networking**

2:00 PM **End of workshop program**



HYDROCEPHALUS ASSOCIATION

(888) 598-3789 | www.hydroassoc.org | info@hydroassoc.org

[f](#) [t](#) [i](#) [i](#) [v](#) @hydroassoc