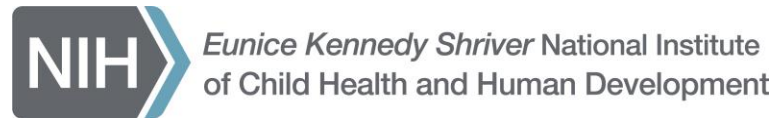


# NIH Clinical Center Pediatric Research Strategic Plan Working Group

Diana W. Bianchi and Brigitte Widemann

*Presentation to CC Governing Board (CCGB): November 16, 2023*  
*Presentation to CC Research Hospital Board (CCRHB): February 16, 2024*



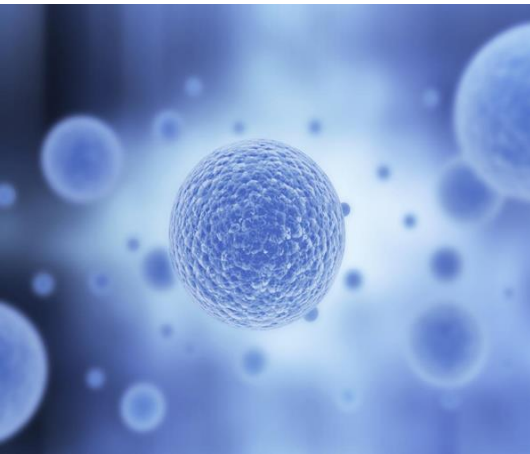
# CC Pediatric Research Strategic Plan Working Group

- **Diana Bianchi, NICHD (co-chair)**
- **Brigitte Widemann, NCI (co-chair)**
- **Deb Tucci, NIDCD**
- **Jim Gilman, CC**
- **Ben Solomon, NHGRI**
- **Beth Kozel, NHLBI**
- **Adam Hartman, NINDS**
- **Luigi Notarangelo, NIAID**
- **Michael Ombrello, NIAMS**
- **Maryland Pao, NIMH**
- **Rebecca Brown, NIDDK**
- **John Glod, NCI**
- **Lisa Rider, NIEHS**
- **PJ Brooks, NCATS**
- **Melissa Parisi, NICHD**



# CCGB Pediatric Strategic Plan Working Group Charge

To identify the most impactful scientific areas of pediatric research in which the NIH can play a major role to substantially improve child health. Using this horizon scanning, to perform long-term, strategic planning for intramural trans-NIH clinical pediatric research to occur over the next decade and beyond



# Scientific Priorities

- **Expanding the scope of natural history studies to support research on the continuum from diagnosis to treatment throughout disease trajectory**
- **Building a foundational clinical and scientific infrastructure to support gene therapy, CAR-T, and other cell and precision therapy studies in young children with inherited or acquired rare, serious, and refractory diseases of unmet need**
- **Evaluating the efficacy of precision medicine interventions in rare, non-malignant childhood diseases**
- **Increasing the number of pharmacokinetic and pharmacodynamic studies to improve rational medication use and proper dosing in children**



# Scientific Priorities (continued)

- **Performing metabolic phenotyping across a variety of pediatric conditions, as well as linking metabolic phenotyping on nutrition studies and assessing the impact of diet on immune phenotypes and metabolism**
- **Developing a cohort of all pediatric patients at the CC to measure physical and mental health and disease across disorders, along with a deeply phenotyped pediatric cohort of healthy volunteer children to establish a standard control group that can be used across studies**
- **Increasing support for research studies in pregnant and lactating people**



# Enhancement to Infrastructure and Resources

- **Workforce training (e.g., Grand Rounds, directory of pediatricians)**
- **Create a pediatric section of the IRB (potentially including pregnancy as well)**
- **Establish a core focused on protocol development for pediatric clinical research studies**
- **Pediatric natural history concierge**
- **Modular case report forms**
- **Common data elements**
- **Pediatric-friendly imaging resources and environment**
- **Data management and sharing**



# Relative Financial Investment Needed to Implement CC Pediatric Research Strategic Plan Priorities (1 of 3)

## **MINIMAL Financial Investment**

### **Strategic Plan Priority**

- **Add a specific pediatric section to the IRB**
- **Develop a directory of CC pediatric researchers**
- **Establish Grand Rounds focused on child health**





# Relative Financial Investment Needed to Implement CC Pediatric Research Strategic Plan Priorities (2 of 3)

## LOW/MODERATE Financial Investment

### Strategic Plan Priority

- Pediatric pharmacokinetic/pharmacodynamic (PK/PD) studies
- Lactation studies (including PK/PD)
- Infant/pediatric metabolic studies
- Phenotyping pediatric *All of Us* participants that are identified as having genomic variants
- Core for pediatric protocol development
- Unified clinical research form
- Developing pediatric common data elements
- Pediatric-friendly imaging/scanning protocols and facilities
- Establish a natural history study concierge





# Relative Financial Investment Needed to Implement CC Pediatric Research Strategic Plan Priorities (3 of 3)

## MAJOR Financial Investment

### Strategic Plan Priority

- **Clinical infrastructure to support gene therapy, CAR-T, and other cell and precision therapy studies in young children**
- **Deeply phenotyped pediatric-specific healthy control cohort**
- **Cohort study including all CC pediatric patients at the CC across multiple disorders**





**Thank You!**

**Questions?**