# From Then to Now and Beyond NICHD Research in Pediatric Therapeutics

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NIH *Eunice Kennedy Shriver* National Institute of Child Health and Human Development

# Disclaimer

The views expressed in this presentation are those of the presenter and do not necessarily reflect the policies of the National Institutes of Health or the Department of Health and Human Services.

# Obstetrics and Pediatrics Pharm Programs

OBSTETRICS

Research Project Grants (RPG)

Obstetric-Fetal Pharmacology Research Unit (OPRU) Network

Training

PEDIATRICS

Research Project Grants (RPG)

Specialized Centers in Research in Pediatric Developmental Pharmacology

Training*

Regulatory Activities*

Data Coordinating Center*

Pediatric Trials Network*

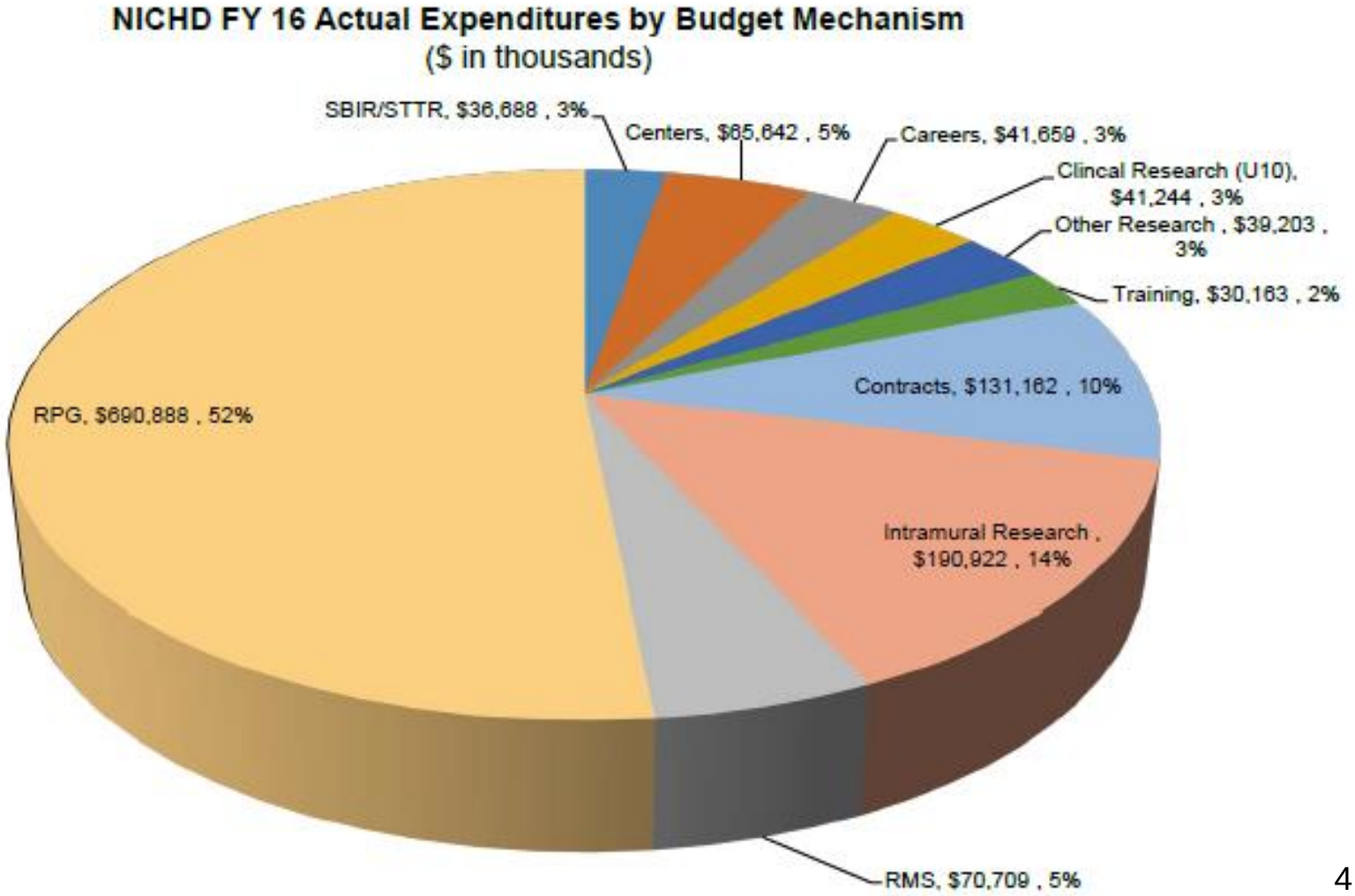
**Funding mechanisms**

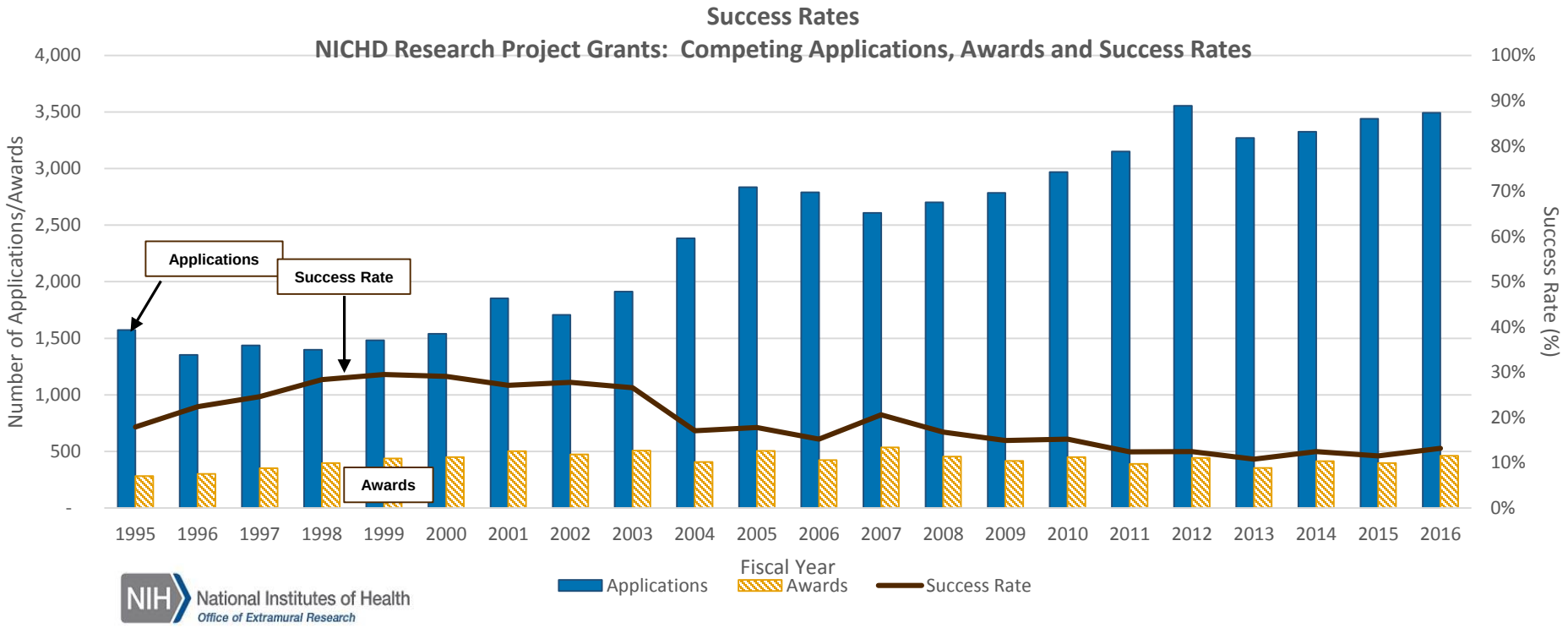
- RPG
- Cooperative agreement
- Contract

Grant Types
R03
R01
R21

* Best Pharmaceuticals for Children Act funds

# NICHD Budget At A Glance






Awards made under Reimbursable Agreements, Appropriations to NIH for Superfund-related activities, Gift Funds and Breast Cancer Research Stamp Funds are not included. Research Projects (RPG) defined as activities (R00,R01,R03,R15,R21,R22,R23,R29, R33,R34,R35,R36,R37,R55,R56,RL1,RL2,RL5,RL9,P01,P42, PN1,UC1,UC7,U01,U19,U34,UH2,UH3,UM1,DP1,DP2,DP3,DP4,DP5,RC1,RC2, RC3,RC4,UA5,UC1,UC2,UC3,UC4,RF1,UF1,PM1,RM1)
Source: NIH IMPAC, Success Rate File

# Branch priorities from public website

- **Developmental Pharmacology and Pathophysiology of Pregnancy**
  - **Gap:** lack of knowledge of mechanisms of drug action in obstetric and pediatric populations and in their pre-clinical models, to maximize drug efficacy and minimize toxicity
  - **Priority:** Support developmental pharmacology initiatives and initiatives that explore mechanisms of drug action in pregnant women. Critical areas include pain management in neonates and pregnant women, treatment of Type 2 and gestational diabetes, and preterm birth.
- **New Drug Development and Drug Repurposing**
  - **Gap**: lack of safe and efficient medications for children and pregnant women
  - **Priority**: Support identification of drug targets for children and pregnant women for conditions specifically relevant to these populations, including (but not limited to) neonatal/pediatric pain, rare diseases, Type 2 and gestational diabetes, preterm labor, and preeclampsia. Use these targets to develop new drugs or repurpose appropriate old drugs.
- **Novel Alternatives to Traditional Pediatric and Obstetric Clinical Trials**
  - **Gap:** Significant hardship in the design and execution of pediatric and obstetric clinical trials
  - **Priority**: Support innovative approaches and algorithms to predict drug safety and effectiveness in children and pregnant women. This includes modeling and simulation methods, and advanced methods of utilizing existing data, such as electronic medical records, opportunistic studies

# Branch priorities from public website

- **Outcome Measures for Pediatric and Obstetric Clinical Trials**
  - **Gap:** lack of outcome measures and biomarkers that reflect diseases/conditions and predict drug safety and effectiveness in children and pregnant women
  - **Priority:** Support initiatives for the identification and validation of outcome measures and biomarkers in this population. Outcome measures and biomarkers related to pain, sedation, Type 2 and gestational diabetes, and acute kidney injury would be highly relevant.
- **Pediatric Formulations**
  - **Gap:** Appropriate formulations for pediatric populations remain elusive or absent.
  - **Priority:** Support initiatives for the development of palatable and safe (i.e., without harmful excipients) formulations for children.
- **Therapeutic Devices**
  - **Gap:** Development of non-drug therapeutics, such as devices, is needed to improve therapeutic treatment for the fetus and for children.
  - **Priority:** Support development of non-invasive drug delivery systems and devices to measure drug safety or efficacy non-invasively.

# Topics

- From Then to Now
- Beyond

**Then**

Creation PPRU Network

A false assumption: FDA rule 1994

Realization of profound knowledge gaps

**Now**

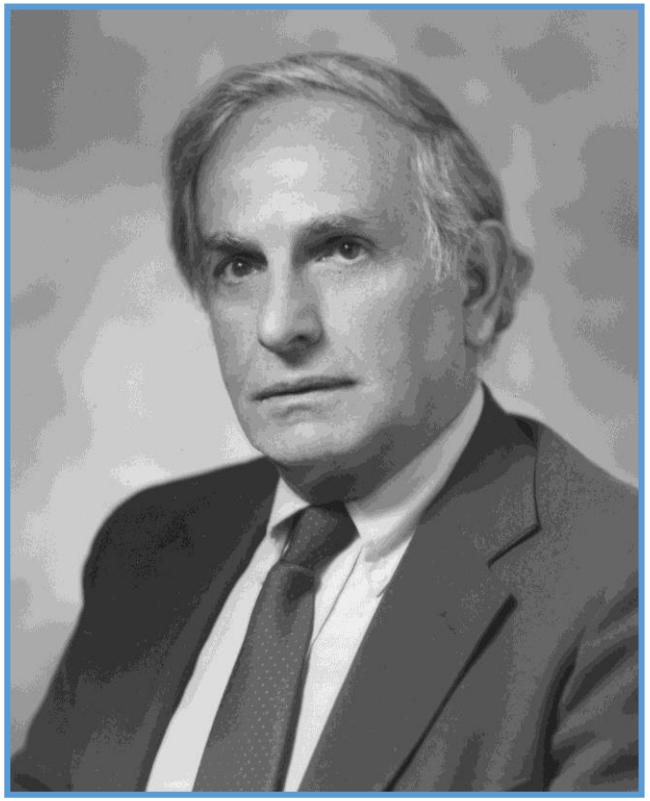
Need to address knowledge gaps

High number of failed/negative drug studies

Profound changes in NIH/FDA clinical trials requirements

**Beyond**

**??**



Sumner J. Yaffe, MD
The Father of Pediatric Clinical Pharmacology

DEVELOPMENTAL PHARMACOLOGY Release Date: January 5, 2000 RFA: HD-00-001

# Developmental Pharmacology/Mechanisms of ADRs

## Program Officer: George P. Giacoia, M.D.

- **RFA HD-00-001 DEVELOPMENTAL PHARMACOLOGY** (R01,R03 R21)
  Release Date: January 5, 2000
  National Institute of Child Health and Human Development Primary)
  Secondary. NCI,NIES,NIGMS,NIMH,NINDS

- **Developmental Pharmacology PAR 07-416(R01)PAR 07-417(R03)PAR-07-418 Expired 2010 F**
- **Developmental Pharmacology PAR 11-057 (R01) PAR 11-58(R03)PAR 11-59 Expired 2013 F**
- **Developmental Pharmacology and Toxicology Role of Ontogeny PAR 13-306 (R01) PAR 13-307 (R03) PAR 13308(R21 2001) Expired 2016 F**

- **Mechanisms of Adverse Drug Effects in Children PAR-08-248(R01) PAR Expired 2010F**
- **Mechanisms of Adverse Drug Effects in Children PAR-11-051; PAR-052 (R03) Expired 2014 F**

*(F) = Foreign Institutions are eligible to apply.*

# Biomarkers

Program Officer: George P. Giacoia, M.D.

- PA-01-043 Biomarkers and Clinical Endpoints in Pediatric Clinical Trials
  January 18, 2001 - February 1, 2004
- PAR-13-296 (F) Biomarkers: Bridging Pediatric and Adult Therapeutics (R01)
  September 5, 2011-September 8, 2016 (Previous FOA - PAR-11-322)
- PAR-13-299 (F)Biomarkers:  Bridging Pediatric and Adult Therapeutics (R03)
  September 16, 2011-September 8, 2016 (Previous FOA - PAR-11-322)
- PAR-17-169 (F) Biomarkers: Bridging Pediatric and Adult Therapeutics (R21)
  September 16, 2011-May 8, 2020 (Previous FOAs - PAR-13-295, PAR-11-324)

*(F) = Foreign Institutions are eligible to apply.*

*Red text indicates active opportunities.*

# OPP Branch FOA
# Translational Research in Pediatric and Obstetric Pharmacology and Therapeutics

## Program Officer: Zhaoxia Ren

- PAR-17-189 (F)- Translational Research in Pediatric and Obstetric Pharmacology and Therapeutics (R01)
  August 16, 2009- April 4, 2020 (Previous FOAs - PAR-13-309, PAR-11-246, PAR-09-155)
- PAR-17-188 (F) - Translational Research in Pediatric and Obstetric Pharmacology and Therapeutics (R03)
  August 16, 2009-April 4, 2020 (Previous FOAs -PAR-13-310, PAR-11-247, PAR-09-154)
- PAR-17-187 (F) - Translational Research in Pediatric and Obstetric Pharmacology and Therapeutics (R21)
  August 16, 2009-April 4, 2020 (Previous FOAs - PAR-13-311, PAR-11-248, PAR-09-156)

*(F) = Foreign Institutions are eligible to apply.*
*Red text indicates active opportunities.*

# FOAs Development of Adequate pediatric Formulations

Program Officer: George P. Giacoia, M.D.

Funding Opportunities

- PAR-17-192 (F) - Development of Appropriate Pediatric Formulations and Pediatric Drug Delivery Systems (R21)
  September 16, 2011-May 8, 2020 (Previous FOAs -PAR-13-326, PAR-11-303)
- .PAR-17-193 (F) - Research Project Grant (R01)
  September 5, 2011-May 8, 2020 (Previous FOAs - PAR-13-325, PAR-11-301)
- PAR-17-191 (F) - Small Grant Program (R03)
  September 16, 2011-May 8, 2020 (Previous FOAs - PAR-17-191, PAR-13-344, PAR-11-302)
- PAR-17-200 Small Business Technology Transfer (STTR) Grant (R41) - Phase I only
  November 5, 2011-January 6, 2020
- PAR-17-199 Small Business Innovation Research (SBIR) Grant (R43) - Phase I only
  November 5, 2011-January 6, 2020 (Previous FOAs - PAR-13-345, PAR-11-304)

*(F) = Foreign Institutions are eligible to apply.*

*Red text indicates active opportunities.*

# Funding Opportunities Related to Pregnancy, Devices, Pain Control

- RFA-HD-10-010 - Molecular Mechanism of Adverse Metabolic Drug Effects in Children and Adolescents (R01) F
  Application Date: November 1, 2010 Program Officer: Perdita Taylor-Zapata, M.D.
- PAR-13-389 - Discovery of Molecular Targets for Pregnancy-Related/Induced Diseases and Development of Therapeutics to Prevent/Treat These Diseases (R01)
  5-Jan-14 - May 8, 2017 Program Officer: Katerina Tsilou, M.D. To be renewed
- **FOA Devices –**
- PAR-13-090 - Safe and Effective Instruments and Devices for Use in Neonatal and Pediatric Care Settings (R43/R44) – NICHD Not Primary
  June 30, 2009-April 6, 2016 Program Officer: Tonse Raju, M.D. (Previous FOAs - RFA-HD-12-193, RFA-HD-10-013, RFA-HD-09-018)
- PAR-13-091- Safe and Effective Instruments and Devices for Use in Neonatal and Pediatric Care Settings (R41/R42) - NICHD Not Primary
  June 30, 2009 - April 6, 2016 Program Officer: Tonse Raju, M.D. (Previous FOAs - RFA-HD-12-192, RFA-HD-10-012, RFA-HD-09-017)

**FOA Pain control – Program Officer:**

- PA-16-312 (F) - Safety and Outcome Measures of Pain Medications Used in Children and Pregnant Women Program Officer: Zhaoxia Ren, M.D., Ph.D.
  September 16, 2016 - January 8, 2020

*(F) = Foreign Institutions are eligible to apply.*

*Red text indicates active opportunities.*

# Precision Medicine

**Funding**

- PAR-14-274 - Pharmacogenetics, Pharmacoepigenetics and Personalized Medicine in Children (R01)
  September 5, 2014-September 8, 2017 Not Renewed PO Katherina Tsilou

# Newborn Initiative

- **Newborn Drug Development Initiative Meeting (**March 29–30, 2004) Working Groups – Cardiology, Neurology, Pain Control, Pulmonary, Ethics and Drug Prioritization
- Publications White papers
  Clancy RR; Neurology Group on Neonatal Seizures. seizures. Clin Ther. 2006 Sep;28(9):1342-52. PubMed PMID: 17062308.
- -Perlman JM. Intervention strategies for neonatal hypoxic-ischemic cerebral injury. Clin Ther. 2006 Sep;28(9):1353-65.
- -Giacoia GP, Mattison DR. Selected Proceedings of the NICHD/FDA newborn drug development initiative: Part II. Clin Ther. 2006 Sep;28(9):1337-41. Review.
- .-Evans JR, Lou Short B, Van Meurs K, Cheryl Sachs H. Cardiovascular support in preterm infants. Clin Ther. 2006 Sep;28(9):1366-84. Review.
- .-Ward RM, Benitz WE, Benjamin DK Jr, Blackmon L, Giacoia GP, Hudak M, Lasky T, Rodriguez W, Selen A. Criteria supporting the study of drugs in the newborn. ClinTher. 2006 Sep;28(9):1385-98. Review. .
- -Baer GR, Nelson RM; Ethics Group ethical challenges in neonatal research.

  ClinTh2008Sep;28(9):1399-407.

## Direct Funding BPCA

**BPCA Funds ic NHBLI Bronchopulmonary Dysplasia;**

- **NICHDfun Newborn Network Study of Shock in preterm infants;pharmacology of retinol**
- **FOAs in Developmental pharmacolo<Treatment of retrolental fibroplasiagy:role of ontogeny; Mechanisms of ADRs**
  **U54 Developmental Pharmacology**
- **Pediatrc Trials Network Studies of pk of antibiotics and antifungal**

# US Pediatric Formulations Initiative

## Program Officer: George Giacoia, M.D.

Working Groups

- Scientific, technical, and regulatory barriers for the development of pediatric formulations
- Taste, smell, and flavor research in infants and children
- Economic issues and partnerships
- Use and application in pediatrics of new drug delivery systems

Funding Opportunities

- PAR-17-192 (F) - Development of Appropriate Pediatric Formulations and Pediatric Drug Delivery Systems (R21)
  September 16, 2011-May 8, 2020 (Previous FOAs- PAR-13-326, PAR-11-303)
- PAR-17-193 (F) - Research Project Grant (R01)
  September 5, 2011-May 8, 2020 (Previous FOAs - PAR-13-325, PAR-11-301)
- PAR-17-191 (F) - Small Grant Program (R03)
  September 16, 2011-May 8, 2020 (Previous FOAs - PAR-13-344, PAR-11-302)

*(F) = Foreign Institutions are eligible to apply.*

*Red text indicates active opportunities.*

# US Pediatric Formulation Initiative (Continued)

Collaboration between EU and US PFI

- Salunke, S., Giacoia, G., Tuleu, C. (2012). The STEP (Safety and Toxicity of Excipients for Paediatrics) Database. Part 1 – A Need Assessment Study. *Int J Pharm*, 435(2), 101-111. https://www.ncbi.nlm.nih.gov/pubmed/22583848
- Salunke, S., Brandys, B., Giacoia, G., Tuleu, C. (2013). The STEP (Safety and Toxicity of Excipients for Paediatrics) Database: Part 2 – The Pilot Version. *Int J Pharm,* 457(1), 310-322. https://www.ncbi.nlm.nih.gov/pubmed/24070789

Workshops

- December 6–7, 2005
- November 1–2, 2011

Abdel-Rahman, S.M., Amidon, G.L., Kau, A., Lukacova, V., Vinks, A.A., Knipp, G.T., and Members of the BCS Task Force. (2012). Summary of the National Institute of Child Health and Human Development–Best Pharmaceuticals for Children Act Pediatric Formulation Initiatives Workshop–Pediatric Biopharmaceutics Classification System Working Group. *Clin Ther*, 34(11), S11-S24 https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3534959/

# Ontogeny of Transporters in Children Working Group

**Chair: Kim Brouwer, Pharm.D., Ph.D, University of North Carolina at Chapel Hill**

Funding Opportunities Program Officer: George Giacoia, M.D.

- PAR-13-306 (F) -Developmental Pharmacology (R01)
  August 21, 2007-September, 8, 2016 (Previous FOAs - PAR-11-057, PAR-07-416)
- PAR-13-308 (F) – Developmental Pharmacology and Toxicology: Role of Ontogeny (R21)
  August 21, 2007-September 8, 2016 (Previous FOAs - PAR-11-059, PAR-11-058, PAR-08-215)
- PAR-13-307 (F) – Developmental Pharmacology and Toxicology: Role of Ontogeny (R03)
  August 21, 2007-September 8, 2016 (Previous FOAs - PAR-11-058, PAR-08-215)

## Publications


- Brouwer, K. L., Aleksunes, L.M., Brandys, B., Giacoia, G.P., Knipp, G., Lukacova, V.,…de Wildt, S.N., Pediatric Transporter Working Group. (2015). Human ontogeny of drug transporters: review and recommendations of the Pediatric Transporter Working Group. *Clin Pharmacol Ther, 98*, 266-87. PMID: 26088472. https://www.ncbi.nlm.nih.gov/pubmed/26088472


*(F) = Foreign Institutions are eligible to apply.*

# Initiative to Advance Pediatric Therapeutics – Diabetes Working Group

**Chair: William Tamborlane, M.D., Yale University**

**Subgroups:**

- **Type 1 – Therapeutics -** Chair: Linda DiMeglio, M.D., Riley Hospital for Children
- **Type 2 – Therapeutics -** Chair: William Tamborlane, M.D., Yale University
- **Type 1 – Natural History and Biomarkers -** Chair: Mark Rigby, M.D., Ph.D., Janssen
- **Type 2 – Natural History and Biomarkers -** Chair: Philip Zeitler, M.D., Ph.D., Children's Hospital Colorado
- **Pharmacology -** Chair: Michael Spigarelli, M.D., Ph.D., University of Utah

**The T2D subgroup Publication**

Tamborlane, W.V., Haymond, M.W., Dunger, D., Shankar, R., Gubitosi-Klug, R., Bethin, o Tomassi PK…Giacoia, G. (2016). Expanding Treatment Options for Youth with Type 2 Diabetes: Current Problems and Proposed Solutions. *Diabetes Care, 39*(3), 323-329. doi: 10.2337/dc15-1649.
https://www.ncbi.nlm.nih.gov/pubmed/26908928

# Main Concerns about NIH Clinical Trials IOM Report 2010 and GAO Report 2016

- Large investment, $3 billion/year
- Variable quality of trial design
- Incomplete registration and reporting of trial results
- Inconsistent oversight and monitoring
- Inability to assess across IC's

*Slide from Mike Lauer*

# NIH Reforms
# Goals

- Enhance application and award process
- Increase NIH's ability to assess the merits and feasibility of clinical trial applications
- Improve oversight and transparency
- Increase sharing of results
- Ensure rigor and efficiency
- Improve stewardship
- Maintain public trust

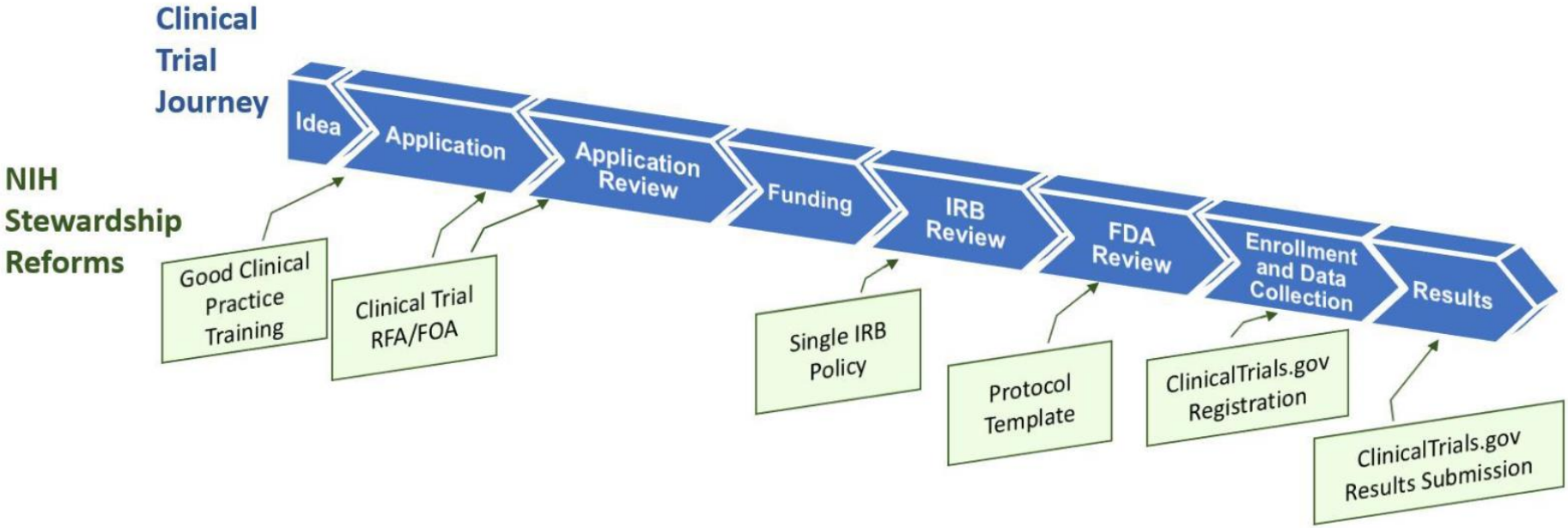
Hudson, et al., JAMA 2016; 316:1353-4

# Revised NIH Definition of Clinical Trial

A **research study** in which one or more **human subjects** are **prospectively assigned** to one or more **interventions** (which may include placebo or other control) to evaluate the effects of those interventions on **health-related biomedical or behavioral outcomes**.

NOT-OD-15-015 : Effective January 25, 2015

# Reforms over the clinical trial lifecycle




https://nexus.od.nih.gov/all/2016/09/16/clinical-trials-stewardship-and-transparency/

# Policy on Good Clinical Practice Training for NIH Awardees Involved in NIH-funded Clinical Trials

- NOT-OD-16-148
- Issued September 16, 2016
- Effective January 1, 2017
- Complement to other required training on human subjects protections
- Required of all NIH-funded investigators and clinical trial site staff responsible for the conduct, management, and oversight of NIH-funded clinical trials
- Acceptable courses include those offered by NIAID, the National Drug Abuse Treatment Clinical Trials Network , and the CITI Program

# Policy on Funding Opportunity Announcements (FOA) for Clinical Trials

- NOT-OD-16-147;
- Issued September 16, 2016
- Target effective date: January , 2018
- Applications will require specific information about protocols, specific review criteria, terms and conditions in Notices of Grant Awards
- Mechanisms will differ by IC
- Responding to a specific clinical trial FOA is the only way to propose an investigator-initiated clinical trial

# Final NIH Policy on the Use of a Single Institutional Review Board for Multi-Site Research

- NOT-OD-16-094
- Issued June 21, 2016
- Effective for applications received on or after November 25, 2017
- Applies to all domestic sites in multisite clinical research, not just clinical trials
- Improve efficiency
- Minimize duplicative reviews
- NOT-OD-16-109 Direct and indirect cost scenarios

# NOT-OD-16-043

## Protocol Template: NIH and FDA Request for Public Comment on Draft Clinical Trial Protocol Template for Phase 2 and 3 IND/IDE Studies

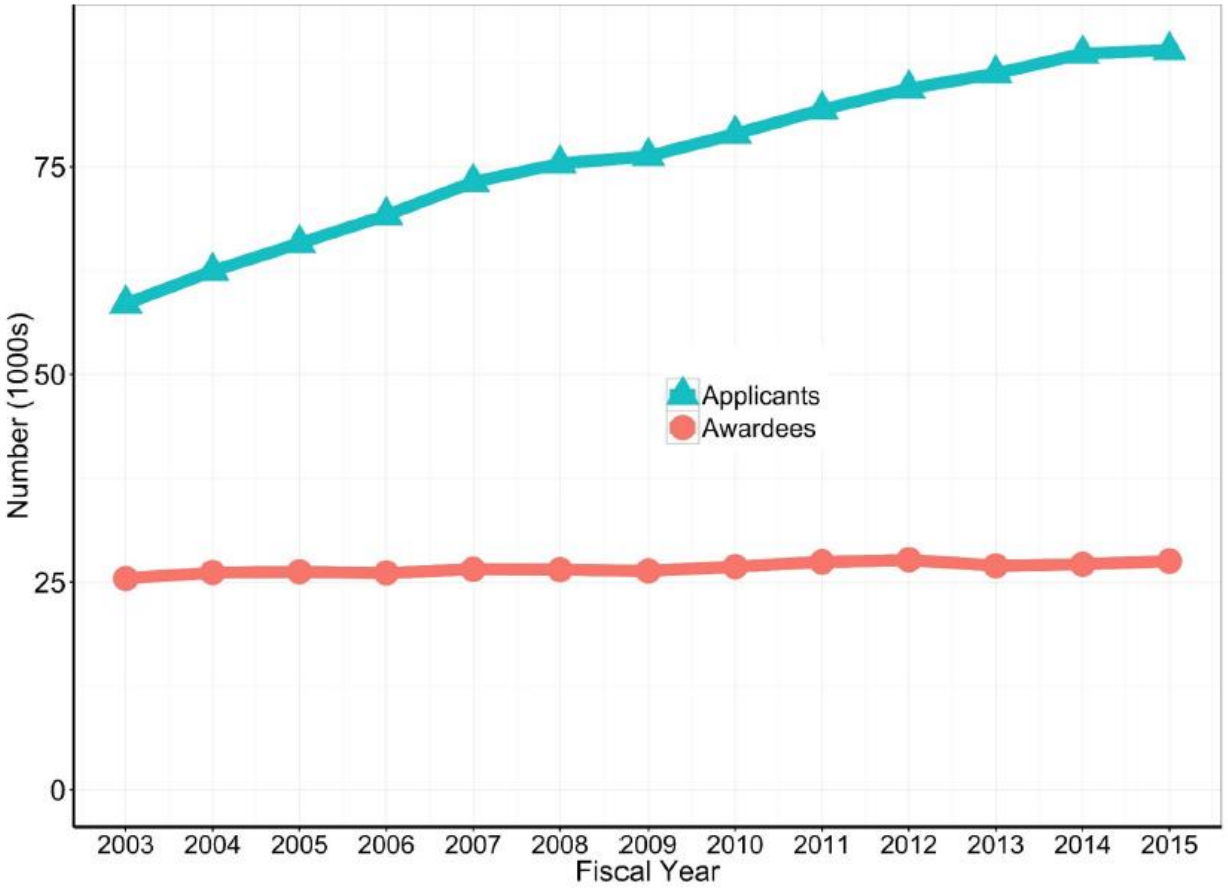
- NOT-OD-16-043
- Issued March 17, 2016
- Developed by FDA and NIH
- Corresponds to GCP
- Review of comments underway
- May develop an online tool
- Plan to adapt template for Phase 1 trials as well as social/behavioral intervention trials

# **NIH Policy** on the Dissemination of NIH-Funded Clinical Trial Information

- NOT-OD-16-149
- NIH Policy, Federal Register
- Issued September 21, 2016
- Effective January 18, 2017
- Applies to **all NIH-funded clinical trials** (not just FDA-regulated trials) regardless of study phase, type of intervention, or whether they are subject to the regulation
- Up to $10,000/day fine

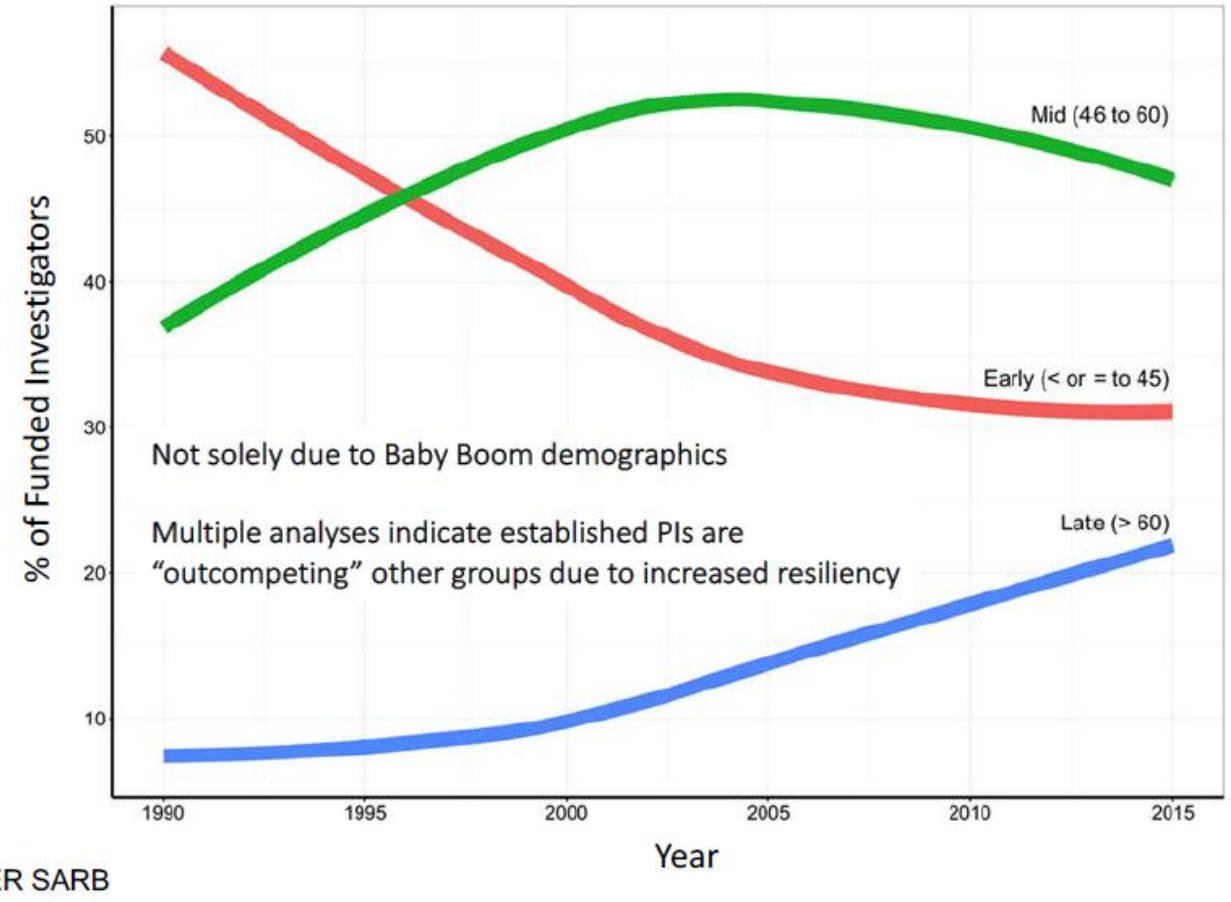
- Withholding of future funding for the grant and any future grant to the grantee institution

# Hypercompetition: Applicants and Awardees for NIH RPGs




OER SARB

# Age of Investigators Funded by NIH




OER SARB

Lawrence A. Tabak, DDS, PhD - Principal Deputy Director, NIH
Enhancing Stewardship: The Next Generation of Researchers Initiative - 114th Meeting of the Advisory Committee to the Director (ACD)

# NIH Next Generation Researchers Initiative proposal

- NIH will take a multi-pronged approach:
- Further extending the payline for early stage investigators, with an aim of funding most applications that score in the top 25 percent
- Providing additional support for mid-career investigators with ≤ 10 years as a principal investigator
  - Extending the payline for those about to lose all NIH funding
  - Identifying “rising stars” who are seeking support for their second RPG, but just missed the payline
- The total cost of these measures, to be derived in each IC by rearranging priorities in other categories, is estimated (pending availability of funds), at:
  - ~$210 million the first year
  - Ramping up over 5 years to reach approximately $1.1 billion per year

# Interdisciplinary partnerships

An interdisciplinary *project* draws on methods and understandings from several disciplines, but creates something new that is greater than the sum of its parts. Interdisciplinary projects are necessarily focused on solving a particular problem, but interdisciplinary partnerships created for one project can continue through an entire career. Ideally, interdisciplinary projects both contribute to new approaches that span disciplines and provide insights for each participating discipline.

# Maximizing Investigators Research Award (MIRA) (R35)NIGMS

- In comparison to R01 funding of NIGMS investigators, MIRA benefits include:
- A longer grant period - five year awards rather than the current NIGMS median of 4 years;
- More flexibility to pursue new ideas and opportunities as they arise during the course of research because the award is not tied to specific aims or predicated on completing specific, pre-defined projects;
- Increased stability of funding through longer-term commitments of support, improved success rates, and more graduated, rather than all-or-none funding decisions of R35 renewals;
- A reduction in administrative burden associated with managing multiple grants;
- A reduction in required application writing

# The future :Pediatric Pharmacology Investigators related issues

- Need for creation of investigators multidisciplinary teams
- Use of multiple rather than single PI/PD applicants
- Avoid duplication of efforts
- Translate recommendations of various working groups to appropriate research studies
- Increase critical mass of pediatric therapeutics researchers
- promote policies that will result  earlier independence and increased funding for new investigators

Back up

# Trans-NIH Involvement in BPCA

- NHLBI: Pediatric Respiratory Outcomes Program (PROP), neonatal pulmonary hypertension, asthma outcomes
- NINDS: Maternal Outcomes and Neurodevelopmental Effects of Anti-Epileptic Drugs (MONEAD), Fragile X Drug Development, cyanide assay
- NCI: collaborations with Children's Oncology Group
- NIDDK: CKiD (Chronic Kidney Disease in children) project
- NIA: Formulations
- NINR: Formulations
- NIGMS and NIMH: T32 clinical pharmacology training
- NIMH: mental health clinical trials
- NIAID: numerous anti-infective trials, assay sharing

# Research in Pediatric Developmental Pharmacology (RPDP) Centers (U54


- Cooperative agreement
- Program Scientist Katerina Tsilou

- Multidisciplinary interactions between basic and clinical scientists, translational research
- Investigate the fundamental mechanisms of changes in drug disposition and response over the course of human development, from birth through adolescence
- Attempts to provide answers to the question '**WHY** are children different than adults in drug metabolism and response?'
- Current sites Children's Mercy , Kansas City ;Children's National Medical Center Washington DC; Indiana University-Purdue University at Indianapolis; University of California, San Diego.

# Pediatric Trials Network (PTN) Overview

https://pediatrictrials.org/

- >6000  children enrolled in over 160 pediatric sites in 5 countries Israel, Canada, United Kingdom, Singapore, Australia
- 45 Task Orders
- 21 clinical trials – Phase I-IV studies
- 74 drugs studied, 15 active INDs
- >40 publications, many poster and symposium presentations
- 10 completed Clinical Study Reports submitted to the FDA
- CONTRACT MECHANISM

# Goals of the Trans NIH Special Interest Group on biomarkers in pediatric therapeutics

- To pursue opportunities for strengthening cross-disciplinary pediatrics biomarker research at the NIH while innovating beyond existing investments.
- provide leadership, vision, and support to promote a strong body of pediatric biomarker research funded by the NIH,
- Collect evaluate and disseminate scientific information and funding opportunities for biomarker research in pediatric therapeutics at NIH
-

# Membership

- NIH Program Staff (intramural and extramural)
- FDA regulators
- Investigators from pediatric networks involved in the study of diseases and drug studies in pediatrics
- Pharmaceutical companies scientists involved in pediatric studies

# Deliverables

- Generation of Trans NIH biomarkers initiatives
- Analysis of currently available information
- Convening panels and mini symposiums
- Development of a road map by disease specific working groups

# Trans-NIH Special Interest Group in Pediatric Biomarkers

## Presentations/Webinar Presentations

| Date | Lecture Title | Lecturer(s) and Affiliation(s) |
|---|---|---|
| **January 12, 2016** | Application of Metabolomics to Provide Pediatric Biomarkers | Susan Sumner - RTI International, University of North Carolina at Chapel Hill, North Carolina State University |
| **February 23, 2016** | Harmonization of Terminology for Biomarkers and Endpoints to Strengthen Quality and Improve Efficiency of Translational Science | Lisa McShane - National Cancer Institute |
| **March 17, 2016** | Pediatric Biomarkers and the Convergence of Academic and Regulatory Sciences | Lynne Yao - U.S. Food and Drug Administration |
| **April 14, 2016** | Biomarkers in Pediatrics: Children as Biomarker Orphans | Allen Everett - Johns Hopkins University |
| **May 12, 2016** | Pharmacometabolomics Informs Pharmacogenomics | Richard Weinshilboum - Mayo Clinic |
| **June 16, 2016** | Imaging Biomarkers in Pediatric Therapeutics: Risks Versus Benefits<br>Pediatric Quantitative MRI | Diane Chugani - AI DuPont Hospital for Children, University of Delaware<br>Carlo Pierpaoli - Eunice Kennedy Shriver National Institute of Child Health and Human Development |
| **July 14, 2016** | Pharmacodynamic Biomarkers | Alexander Vinks- Cincinnati Children's Hospital Medical Center<br>Edmund Capparelli - University of California, San Diego |
| **October 20, 2016** | State of the Art in the Preservation of Biospecimens for Pediatric Biomarker Research | Allison Hubel - University of Minnesota |
| **November 17, 2016** | Nutritional Biomarkers | Patrick Stover - Cornell University |

# Trans-NIH Special Interest Group in Pediatric Biomarkers

## Presentations/Webinar Presentations (Continued)

| Date | Lecture Title | Lecturer(s) and Affiliation(s) |
|---|---|---|
| **January 26, 2017 (Webinar)** | Microbiome Impact on Pediatric Diseases and Clinical Pharmacology | Rob Knight - University of California, San Diego |
| **February 16, 2017** | Biomarker Qualification Program: Implications for Pediatrics | Susan McCune - U.S. Food and Drug Administration |
| **March 23, 2017** | Study Designs, Biomarkers, and Endpoints in Pediatric Asthma Medication Evaluation | Stanley Szefler - Children's Hospital Colorado |
| **April 20, 2017** | Sepsis Biomarkers for Prognostic and Predictive Enrichment | Hector Wong - Cincinnati Children's Hospital Medical Center |
| **May 18, 2017** | Use of Biomarkers in a Drug Development Program for a First-In-Class Drug in Children with Duchenne Muscular Dystrophy | Eric Hoffman - Binghamton University – SUNY/ReveraGen Biopharma |
| **June 22, 2017** | Biomarkers in Type 1 Diabetes in Pediatrics | Kevan Herold - Yale Center for Clinical Research/Yale Diabetes Center |