

# NICHD Director's Report to Council

Diana W. Bianchi, M.D.

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## Outline for Today's Talk

- Inclusion
  - *JAMA* commentary
  - NIH policy change
  - *All of Us*-include our populations
- Congressional Updates
  - Continuing Resolution and Budget Update
  - Update on 21<sup>st</sup> Century Cures Activities
- Vision Update
  - Enhancing Partnerships (previously known as Building Bridges)
  - Communicating the Message
  - Shared Data and Resources Update
  - Strategic Planning



# The Crucial Importance of Inclusion



VIEWPOINT

# Improving Public Health Requires Inclusion of Underrepresented Populations in Research

Catherine Y. Spong, MD

Eunice Kennedy Shriver National Institute of Child Health and Human Development, National Institutes of Health, Bethesda, Maryland.

Diana W. Bianchi, MD

Eunice Kennedy Shriver National Institute of Child Health and Human Development, National Institutes of Health, Bethesda, Maryland.



Supplemental content

**Advances in genomics** have ushered in promising therapies tailored to the individual. Personalized medicine is promoted and has begun to positively influence care. For example, medications such as trastuzumab for the 30% of breast cancers that overexpress *ERBB2* and vemurafenib for patients with late-stage melanoma who carry the V600E variant have been beneficial.<sup>1</sup> Despite these advances, for many sectors of the population—children, older adults, pregnant and lactating women, and individuals with physical and intellectual disabilities—limited evidence-based therapies optimized to their specific medical needs exist. Combined, these groups comprise as much as 58% of the US population (eTable in the Supplement). Research focusing on or at the very least includes members of these groups is critically needed.

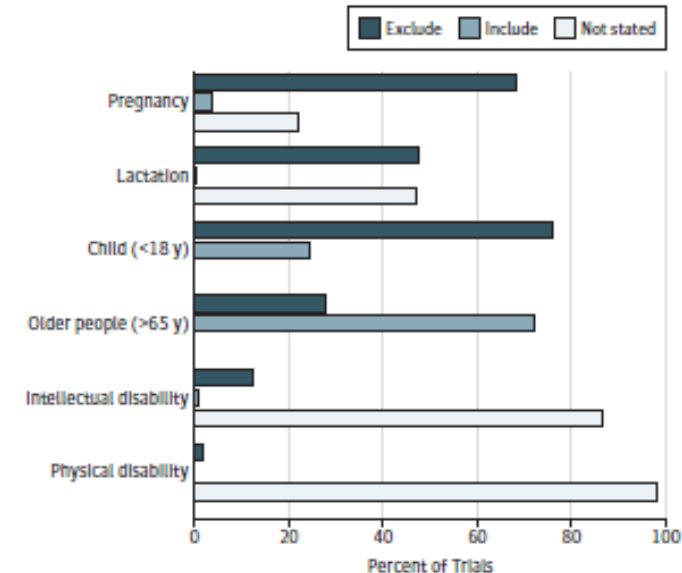
Until the initial passage of the Best Pharmaceuticals for Children Act in 2002, pediatric drug doses were based on extrapolation from adults. Importantly, body composition and metabolic processes change as children develop, resulting in different safety and efficacy

considerations are often prescribed with minimal evidence to support their use, especially psychotropic drugs with significant adverse effects.

Recently, discussions have arisen about the need for inclusion in research and elimination these gaps. In 2017, the National Institutes of Health (NIH) held a workshop, “Inclusion Across the Lifespan,” that highlighted current federal regulations that include protections for “vulnerable populations” (pregnant women, fetuses, neonates, prisoners, and children). Although these regulations were originally designed to protect these individuals, many investigators have called for reconsideration, opting to protect them *through* research, rather than *from* research. Inclusion will likely yield data that will benefit more people.

Many underrepresented populations encounter barriers to participation in research. In a review of 338 phase 3 and 4 NIH-funded actively recruiting studies in Clinicaltrials.gov, explicit exclusion was found in 68% for pregnant women, 47.3% for lactating women, 75.7%

Figure. Open NIH-Funded Phase 3 and 4 Studies as of October 19, 2017



Clinicaltrials.gov records (N=338) were reviewed. Exclusion for intellectual disabilities was based on IQ and defined intellectual disability or cognitive impairment; physical disabilities: exclusions for physical disabilities were inability to ambulate, extreme immobility, and paraplegia.



## eTable. Approximately 58% of the Total US Population Are Not Typically Included in Research Studies (Pregnant Women, Children, Older People, Those With Intellectual and Physical Disabilities)<sup>a</sup>

	Approximate No. of People	Source
Total US population	325 000 000	<a href="http://www.worldometers.info/world-population/us-population">http://www.worldometers.info/world-population/us-population</a>
Pregnant women	6 300 000	<a href="http://www.statisticbrain.com/pregnancy-statistics">http://www.statisticbrain.com/pregnancy-statistics</a>
Children	73 000 000	<a href="https://www.childstats.gov/americaschildren/tables/pop1.asp">https://www.childstats.gov/americaschildren/tables/pop1.asp</a>
Age >65 y	46 200 000	<a href="https://www.census.gov/newsroom/facts-for-features/2016/cb16-ff08.html">https://www.census.gov/newsroom/facts-for-features/2016/cb16-ff08.html</a>
Intellectually and developmentally disabled	6 500 000	<a href="http://www.specialolympics.org/Sections/Who We Are/What Is Intellectual Disability.aspx">http://www.specialolympics.org/Sections/Who We Are/What Is Intellectual Disability.aspx</a>
Physically disabled	56 700 000	<a href="https://www.census.gov/newsroom/releases/archives/miscellaneous/cb12-134.html">https://www.census.gov/newsroom/releases/archives/miscellaneous/cb12-134.html</a>

<sup>a</sup>These numbers are approximate to provide a general impact; the numbers do not account for overlap between categories.



# Policy Change: Inclusion Across the Lifespan

- The new NIH policy requires that applicants and grantees include **individuals of all ages** when conducting clinical research, unless there is a strong justification for their exclusion.
- Applies to all exempt and non-exempt human subjects research (see NOT-OD-18-116)
- ***Inclusion Across the Lifespan*** must be addressed in all grant applications submitted ***on/after January 25, 2019***, and all responses to solicitations issued on/after this date.



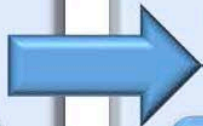


# Policy Change: Inclusion Across the Lifespan

**In applications or proposals:**  
Include an Inclusion plan

Submit a plan for including individuals across the lifespan

If excluding based on age, provide rationale and justification for the specific age range\*



**In progress reports:**  
Report age at enrollment

The policy requires the age of participants at enrollment to be included in reports

Age at enrollment may be reported to NIH in units ranging from hours to years.

Remember: Scientific Review Groups (SRGs) will assess each application/proposal as being "acceptable" or "unacceptable" with regard to the age-appropriate inclusion or exclusion of individuals in the research project.

\***Exclusion based on age must be due to *ethical* or *scientific* reasons.** Acceptable justifications ***may*** include:

- The disease to be studied does not occur in the excluded age group
- The research topic is not relevant to the excluded age group
- Knowledge sought is already available for the excluded group
- Separate study for the excluded age group is warranted or preferable
- Research involves data from pre-enrolled participants
- Laws/regulations bar inclusion of individuals in a specific age group in research
- The study poses unacceptable risk to the excluded age group



## Help Us to Change the Culture

Protect through research rather  
than from research





## Help Us Include NICHD Populations in Use Case Studies

- Visit the All of Us Community Forum (<https://allofusresearchpriorities.ideascale.com/a/ideas/recent/campaigns/21251>) to view currently proposed ideas.
  - Register with the website to comment and vote on proposed ideas
  - Submit your own use cases for the data.
  - To find research ideas related to NICHD mission areas, search for NICHD using the Search Ideas box.
- Deadline is Friday, February 9, 2018.
- Ideas captured by the program will be considered at a Research Priorities Workshop in March.
- All of Us plans to launch nationwide in Spring 2018.



# Congressional Updates

## Congressional Updates



- US government is operating through January 19<sup>th</sup> with a Continuing Resolution
- NICHD has been involved in implementing several parts of the 21<sup>st</sup> Century Cures Act
  - Inclusion Across the Lifespan
  - PRGLAC
  - Medical Rehabilitation Research
    - To be discussed later this morning
  - Next Generation of Researchers Initiative



# 21<sup>st</sup> Century Cures Act: Next Generation of Researchers Initiative

- 21<sup>st</sup> Century Cures Act – Section 2021. Investing in the Next Generation of Researchers
- Twombly et al. Association of National Institute of Child Health and Human Development Career Development Awards With Subsequent Research Project Grant Funding. *JAMA Peds* Jan 16 2018
- In FY2017, NICHD funded 29 **early stage investigators (ESI)**
  - Program Director / Principal Investigator (PD/PI) who has completed their terminal research degree or end of post-graduate clinical training, whichever date is later, within the past 10 years and who has not previously competed successfully as PD/PI for a substantial NIH independent research award.



## Investing in the Future: NICHD's Annual Young Investigators Conference

- Offered for 30 years
- Previously held in Aspen, Colorado, and more recently near Chicago
- Annual meeting to facilitate the training of physician scientists
- Traditionally, the program was open to fellows and junior faculty in neonatology, maternal fetal medicine and reproductive endocrinology who planned to become principal investigators
- Attendees were nominated by their program directors
- NICHD plans to update the program to reflect current needs



# The New (2018) NICHD's Young Investigators Conference

- Will focus on skills needed by **any and all young clinician investigators** who are working in areas that we fund
- Dr. Rosemary Higgins will lead the effort
- Will be held in Potomac, Maryland at The Bolger Center so more NICHD “faculty” can participate
- Activities will include study design, mock study section, setting up a wet lab, work-life balance, networking with program staff
- We will be introducing lesser known grant opportunities and career opportunities
- Chairs of Pediatric, Ob-Gyn and Rehabilitative Medicine Depts will be asked to nominate attendees



# Vision Update

## Vision Update

- Enhancing Partnerships (previously known as Building Bridges)
  - ECHO/NIDA (Opioids)
  - NIA/Foundations (Down syndrome)
- Communicate the Message
  - Voice of the ~~Patient~~ Participant
  - New web site
- Shared Data and Resources
- Strategic Planning Process





# Neonatal Opioid Exposure and Withdrawal

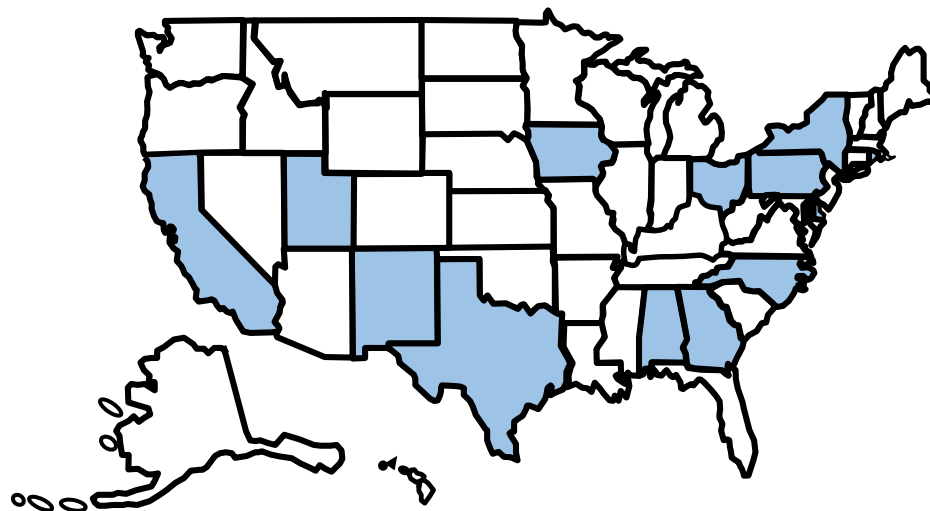
- Need to think about babies differently from adults
  - They generally do not die as a result of exposure
  - Most babies are born in the hospital and are resuscitated if they do not breathe
  - Not all newborns exposed to opioids develop significant signs of withdrawal right away
- Neonates in various stages of withdrawal occupy a significant number of NICU beds, social service resources, local government expenses for foster family placement
- No consistent approaches to care for mothers and babies



# The ACT NOW Partnership

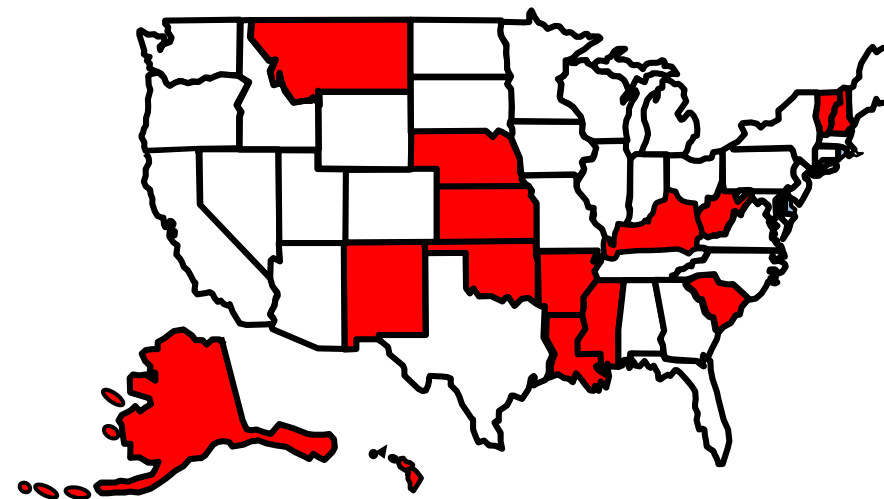


## Neonatal Research Network Centers (2016-2021)



- Started in 1986
- 15 sites, mainly urban
- Sites do not necessarily have high prevalence of NOWs

## IDeA States Pediatric Clinical Trials Network



- Started in 2016
- 17 sites, many are rural
- Sites overlap with areas of high prevalence of NOWs

**Goal: inform the design of a clinical trial to improve the care and outcomes for infants with NOWS through the collection of demographic information and clinical characteristics for infants with NOWS**



# Down Syndrome Research: NICHD/ NIA/ Private Foundations



## Communicating the Message:

## Voice of the Participant

- “Voice of the Patient” changed to “Voice of the Participant”
- David and Kathleen Egan will speak later this morning



Image from DS-Connect® promotional video

# New website launched! [www.nichd.nih.gov](http://www.nichd.nih.gov)



Browser address bar: <https://www.nichd.nih.gov/> | Homepage | NICHD - Eunice...

US Department of Health and Human Services | National Institutes of Health | Directory | Follow [Facebook] [Twitter] [YouTube] [LinkedIn]

**NIH** Eunice Kennedy Shriver National Institute of Child Health and Human Development  
Health research throughout the lifespan

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**1 in every 33 infants born in the U.S. has a birth defect.**  
[This month, learn more about common types, risks, and treatments.](#)

[News Release: NIH launches PregSource, a crowdsourcing project to better understand pregnancy](#)

[News Release: NIH researchers report first 3D structure of DHHC enzymes](#)

[Science Update: Folic acid, multivitamins before and during pregnancy may reduce autism risk, suggests NIH-funded study](#)

### Featured

**[NICHD Needs Your Input for the All of Us Research Program](#)**  
The deadline for submitting ideas and comments is Friday, February 9, 2018.

**[Human Placenta Project](#)**  
The Human Placenta Project is a collaborative research effort launched by NICHD to understand the role of the placenta in health and disease.

### Director's Corner

[Improving Odds of Success for Young Researchers](#)

Diana W. Bianchi, M.D., Director

[Biosketch](#)

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### Divisions, Centers & Offices

Office of the NICHD Director (OD)

Division of Extramural Research (DER)

Division of Intramural Population Health Research (DIPHR)

Division of Intramural Research (DIR)

National Center for Medical Rehabilitation Research (NCMRR)

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## Shared Data and Resources Update: Biosamples Task Force



- Goals: Make as many samples in the NICHD Biorepository and associated data publicly available (DASH)
  - Reduce expense of storing samples with no possibility of future meaningful use
- Two parts to the effort:
  1. Assess historical samples in the NICHD Biorepository
  2. Identify strengths, needs, and potential difficulties in creating policies to facilitate future sharing



# Create Consent Categories for Historical Samples

Group	Description of Consent (color coding indicates whether to use or not)
<b>1</b>	<b>Specimen collection not addressed in consent</b>
	The consent form makes no mention of biospecimen collection associated with the study [Do not use]
<b>2</b>	<b>Consent is silent on secondary use</b>
	The consent form informs participants that specimen(s) will be collected, but makes no mention of possible use of the specimens after the study concludes [Possible use]
<b>3</b>	<b>Consent is vague on secondary use</b>
	The consent form informs that specimen(s) will be collected, and seems to suggest broader use after the parent study [Possible use]
<b>4</b>	<b>Consent is specific on secondary use</b>
<b>4a</b>	Unrestricted future use [Use]
<b>4b</b>	Restricted future use, e.g. in terms of future study topics and/or investigators (pending verification of sample labeling) [Use]
<b>4c</b>	No future use [Do not use]

Color	Meaning	Samples (n)
Red	Do not use	12K
Yellow	Possible use	415K
Green	Use	831K



# Moving Forward

- NICHD's DASH is the “home” for the data and corresponding availability of biorepository samples
- Need to develop guidance on:
  - Clear language in consent form
  - Basic expectations about data and resource sharing for staff and investigators
  - Use of standard data elements for resources to optimize sharing
- Continue to strengthen FOIA language on data and resource sharing
- Disseminate NICHD policy to access samples in the Biorepository



## Where Are We With the Strategic Planning Process?

- End goals: Determine scientific priorities for NICHD moving forward, align resources with priorities
- Proceeding ahead with details of the strategic planning process
  - Developed a set of focus questions
  - Meeting with potential facilitator Jan 2018
- Develop a proposed work plan
  - NICHD planning subcommittee
  - Strategic Plan Work Group will include ~50 people, including some Advisory Council members
  - Stay tuned: 2018 timeline



**Questions?**