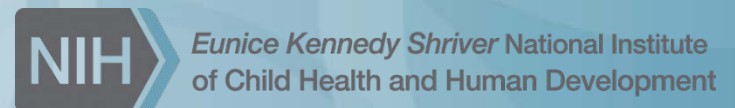
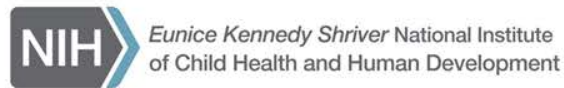


# 21<sup>st</sup> Century Cures Act: Implications for NICHD

Lisa Kaeser, J.D.  
June 8, 2017





# 21<sup>st</sup> Century Cures Act

- Passed the House on November 30, 2016, by vote of 392-26
- Passed the Senate on December 5 by a vote of 94-5
- President signed the bill on December 13



President Obama Delivers Remarks and Signs the 21st Century Cures Act



## An Act

To accelerate the discovery, development, and delivery of 21st century cures, and for other purposes.

Public Law 114-255

### **Purpose:**

- Provide additional funding for the NIH and FDA, relieves administrative burdens, and increases access





# New/Revised Programs

- Precision Medicine Initiative (now “All of Us”)
- Next Generation Researchers Initiative
- Raises NIH’s Loan Repayment Program cap from \$35,000 to \$50,000 – includes clinical and pediatric programs
- EUREKA Prize Competitions and encourages high-risk, high-reward research





# Relieves Burdens and Increases Access

- Exempts NIH from **conference and travel** requirements
- HHS must examine financial **conflicts of interest** & financial expenditure reporting
- NIH must consider ways to reduce burden relating to **sub-recipient monitoring**
- **Clinical Trials** – requires reporting on status in CT.gov
- **Data Access and Privacy**
  - Requires HHS to issue Certificates of Confidentiality and enhances protections for certain types of research
  - Authorizes the NIH Director to require funding recipients to share data



# Cures Act Provisions – NICHD

NICHD has role to play in each of the following:

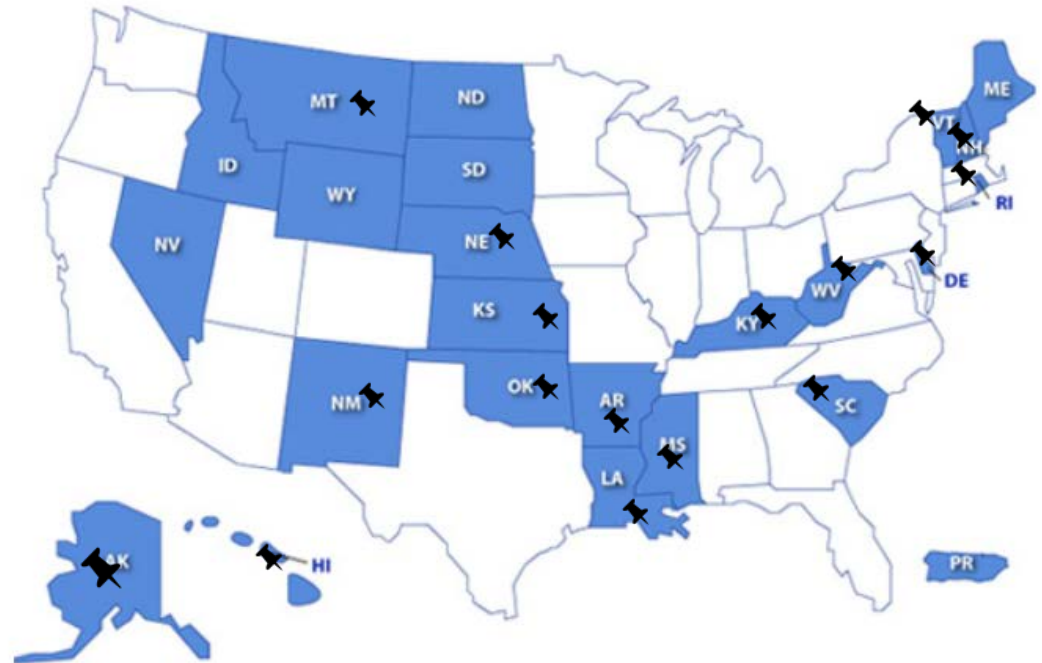
**National Pediatric Research Network – IDeA States Pediatric Clinical Trials Network underway**

**Global Pediatric Network – led by FDA/private industry**

**Inclusion of children in clinical research**

**Task Force on Research Specific to Pregnant Women and Lactating Women**

**Medical Rehabilitation Research**



Shaded states are eligible for IDeA.

**Section 2038. COLLABORATION AND  
COORDINATION TO ENHANCE RESEARCH.**

**(i) APPROPRIATE AGE GROUPINGS IN  
CLINICAL RESEARCH.—**

**POLICY UPDATES.—**Not later than 180 days after the date of enactment of this Act, the Director of the National Institutes of Health shall convene a workshop of experts on pediatric and older populations to provide input on—

- (A) appropriate age groups to be included in research studies involving human subjects; and**
- (B) acceptable justifications for excluding participants from a range of age groups from human subjects research studies.**





# Inclusion in Research

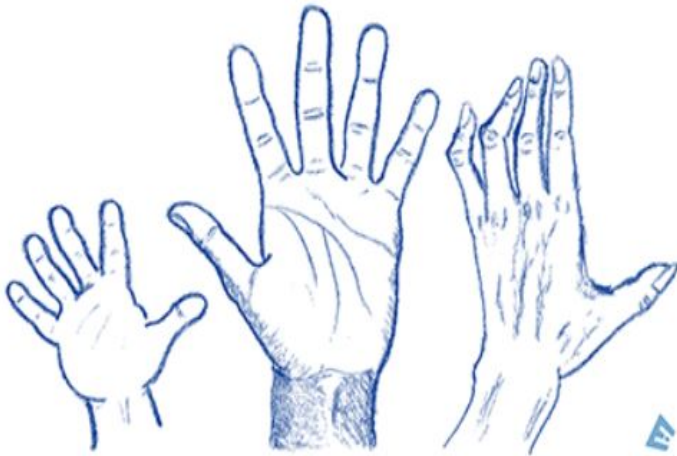
- Ensure women, children, and racial/ethnic minorities are appropriately represented in clinical research
- Assemble clinical research data on women, minorities, and “relevant” age categories including pediatric and older populations
- Improve research related to sexual and gender minority populations
- Requires the NIH Director to hold a workshop regarding appropriate age groups in research and update policies, as appropriate



June 1 – 2, 2017



## Inclusion Across the Lifespan



Natcher

- Pediatric Inclusion

- Requires the NIH Director to include in clinical research, and collect data on, children and older populations
- Requires workshop to obtain input from stakeholders
- Request for Information to gain additional input – ***open until June 30***
- Report findings and update policies as appropriate



# NIH Office of Extramural Research:

## *Collection of Age Data at Individual Level*

- **Goals:** have right people represented in studies to accomplish the science, avoid arbitrary groupings, minimize administrative burdens
- **Proposed Plan:** allow submission of individual participant age information in grant applications and progress reports, using age at enrollment
- **Benefits:** leverages data many investigators collecting, allows monitoring and flexibility



# Workgroup Topics

- Study population: Inclusion/Exclusion Criteria, Age Restrictions
- Study designs and metrics
- Ethical challenges and enrollment of vulnerable populations
- Data collection and reporting for subgroup analyses



# Preliminary Take-Aways

- Change the culture – assume inclusion, justify exclusion
- Too many inclusion/exclusion criteria
- Use study design, targeted recruitment strategies to ensure representation across age groups
- Require investigators to report by age of participants
- No arbitrary age limits in clinical trial design
- Involve stakeholders in study design
- Instead of age, use assessment tool for capacity to consent/participate
- Use meta-analyses to capture subgroup effects



## Next Steps

- RFI open through June 30:  
<https://grants.nih.gov/grants/guide/notice-files/NOT-OD-17-059.html>
- Within 180 days, NIH Director shall make findings and conclusions available to the public
- NIH Director shall determine whether policies on inclusion need to be updated

## **SEC. 2041. TASK FORCE ON RESEARCH SPECIFIC TO PREGNANT WOMEN AND LACTATING WOMEN.**

ESTABLISHMENT.—Not later than 90 days after the date of enactment of this Act, the Secretary of Health and Human Services (referred to in this section as the “Secretary”) shall establish a task force, in accordance with the Federal Advisory Committee Act...

(2) DUTIES.—The Task Force shall provide advice and guidance to the Secretary regarding Federal activities related to identifying and addressing gaps in knowledge and research regarding safe and effective therapies for pregnant women and lactating women, including the development of such therapies and the collaboration on and coordination of such activities.



# Task Force – Medications Used in Pregnancy and Postpartum

- Purpose: “to identify and make recommendations to address gaps in knowledge and research about safe and effective therapies used during pregnancy and for lactating women”
- Report to HHS Secretary and Congress by September 2018
- Sunsets after two years unless extended





# Task Force – Specific Topics Required

- Existing Federal efforts and programs to understand the health effects on pregnant and lactating women, and related birth and pediatric outcomes
- Research collaboration potential
- Ethical issues surrounding inclusion of pregnant and lactating women in clinical research
- Effective communication strategies with health care providers and the public



Fall 2013 The idea

Coalition Birth

June 2014 Meeting with Federal Agencies

Fall 2014 Legislation Writing

2015-16 21<sup>st</sup> Century Cures/Innovations

•Slide courtesy of Coalition to Advance Maternal Therapeutics



# What Happens After the Bill Passes?

## *Task Force Implementation*

- Authority delegated to NIH Director January 19, 2017
- Director asks NICHD to lead – Cathy Spong spearheading the effort
- Charter establishing Task Force filed March 13 (FACA Committee)
- Slate of non-Federal nominees prepared for Secretary's approval – required Federal members designated
- Analysis of currently supported research underway



# What Happens After a Bill Passes?

## *Collect the Data*

New reporting categories being developed for NIH-funded research:

- Pregnancy
- Breastfeeding, Lactation, and Breastmilk

[https://report.nih.gov/categorical\\_spending.aspx](https://report.nih.gov/categorical_spending.aspx)



# Task Force – Next Steps

- Announcements in Federal Register
- All meetings open to the public
  - August 21-22, 2017
  - November 6-7, 2017
  - February 26-27, 2018 (tentative)
  - May 14-15, 2018 (tentative)
- Public presentations at each meeting
- Web page created:  
<https://www.nichd.nih.gov/about/advisory/PRGLAC/Pages/index.aspx>

**SEC. 2040. IMPROVING MEDICAL REHABILITATION RESEARCH AT THE NATIONAL INSTITUTES OF HEALTH.**

(a) IN GENERAL.—Section 452 of the Public Health Service Act (42 U.S.C. 285g–4) is amended—

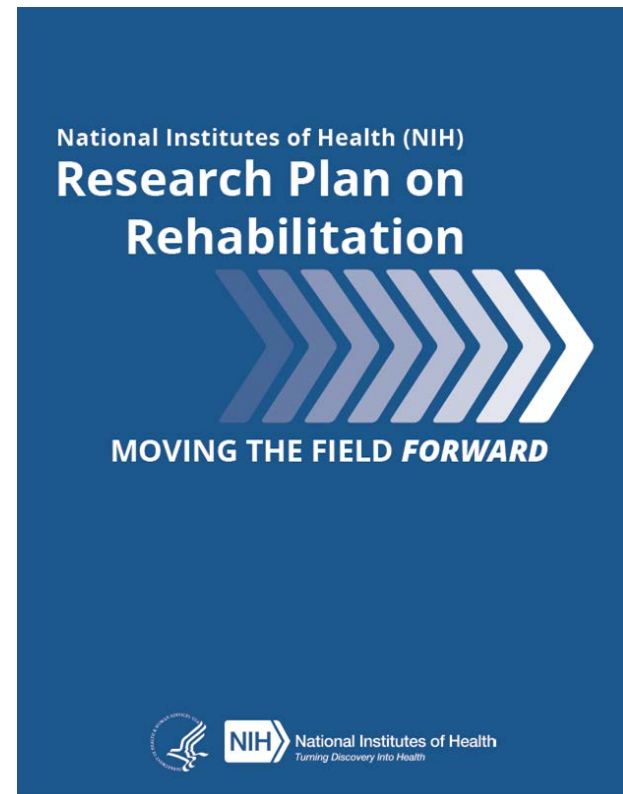
(d)(1) The Director of the Center, in consultation with the Director of the Institute, the coordinating committee established under subsection (e), and the advisory board established under subsection (f), shall develop a comprehensive plan (referred to in this section as the ‘Research Plan’) for the conduct, support, and coordination of medical rehabilitation research...



# Improving Medical Rehabilitation Research at the NIH

## New Provisions:

- Augments requirements for Research Plan
- Requires development of objectives and benchmarks
- Requires scientific workshop every five years
- Enhances coordination within NIH and across the Federal government
- Defines “medical rehabilitation research”





## **1990 Law**

- NCMRR established within NICHD
- Supports peer-reviewed research
- Research plan required within 18 months, with updates as appropriate

## **Cures Act - 2016**

- NCMRR established within NICHD
- Supports peer-reviewed research
- Revised research plan not less than every five years
- NCMRR Director annually reports to Coordinating Committee and Advisory Board, identifying resources for research





## 1990 Law

- Coordinating Committee makes recommendations for research priorities

## Cures Act - 2016

- Coordinating Committee makes recommendations for research priorities
- Committee periodically hosts scientific workshop





## **1990 Law**

- Establishes Advisory Board with specified membership

## **Cures Act - 2016**

- Reauthorizes Advisory Board with updated, specified membership
- Adds DPCPSI Director to involve the NIH OD
- Review and coordinate/prevent duplication
- Secretary may enter into Interagency Agreements
- New definition of “medical rehabilitation research”



## Ahead of the Game

- ✓ Scientific Conference – **Spring 2016**
- ✓ Research Plan – **Fall 2016**
- ✓ Increased Coordination - **Ongoing**
- ✓ Annual Report - **annually**





# 21<sup>st</sup> Century Cures – Next Steps

- Legislative Implementation Work Group
  - Formal process with designated members from across NIH
  - Assigns implementation plans to the appropriate IC/OD Office
  - Final plans submitted to NIH Director
  - Task Force and Rehab plans submitted by NICHD



Thank you!

Questions?

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