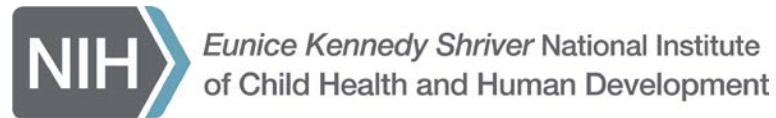


NICHD Director's Report

Diana W. Bianchi, M.D.

NICHD Director

September 18, 2019



Talk Outline

- Updates on Selected Trans-NIH and NICHD Initiatives
 - Helping to End Addiction Long-term (HEAL) Initiative
 - INvestigation of Co-occurring conditions across the Lifespan to Understand Down syndrome (INCLUDE)
 - Task Force on Research Specific to Pregnant Women and Lactating Women (PRGLAC)
- NICHD Staff Updates
- FY 2020 Appropriations



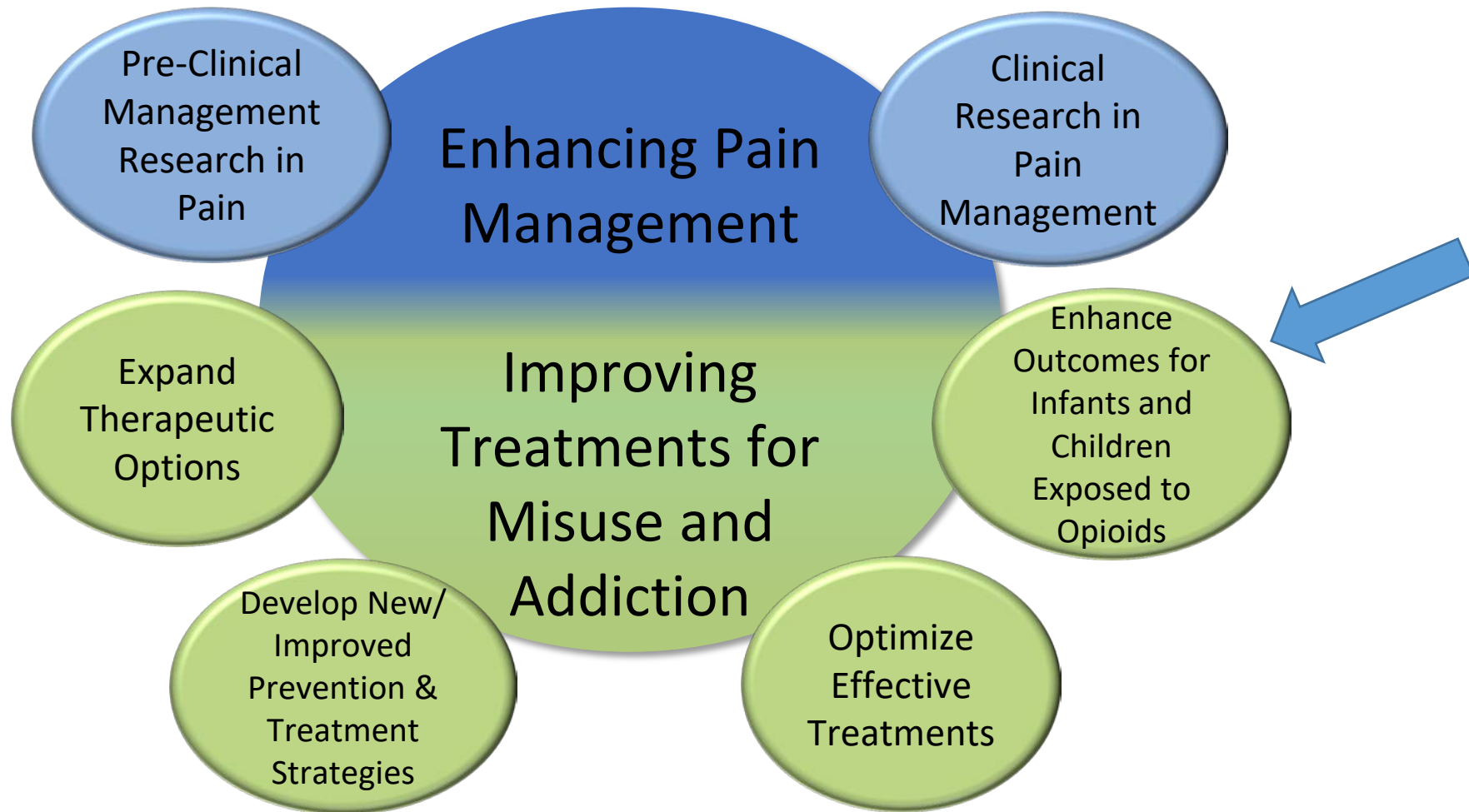
Updates on Selected Trans-NIH and NICHD Initiatives

Dr. Collins' Ten Years at NIH: Large Initiatives That Challenge the Status Quo

- 2013: Brain Research Through Advancing Innovative Neurotechnologies (BRAIN)
- 2015: Precision Medicine Initiative (now All of Us)
- 2016 Cancer Moonshot
- 2018: Helping to End Addiction Long-term (HEAL)



HEAL (Helping to End Addiction Long-term) Initiative



Advancing Clinical Trials in Neonatal Opioid Withdrawal (ACT NOW) Initiative

- Overarching goal: Optimize health for infants exposed to opioids *in utero*
- Overarching question: Among neonates with opioid withdrawal symptoms, to what extent can clinicians safely reduce or eliminate opioid treatment?
- Four Interlinked studies:
 - ACT NOW Current Experience Study (FY 2017 NIH Director Discretionary Funds)
 - **Status: Manuscripts in preparation**
 - ACT NOW Interventional Clinical Trials
 - Eat, Sleep, Console (ESC) Trial: non-pharmacologic
 - Weaning Trial: pharmacologic
 - **Status: Will launch in CY2019**
 - ACT NOW Longitudinal Cohort Observational Study
 - **Status: Application to be discussed at this Council meeting**

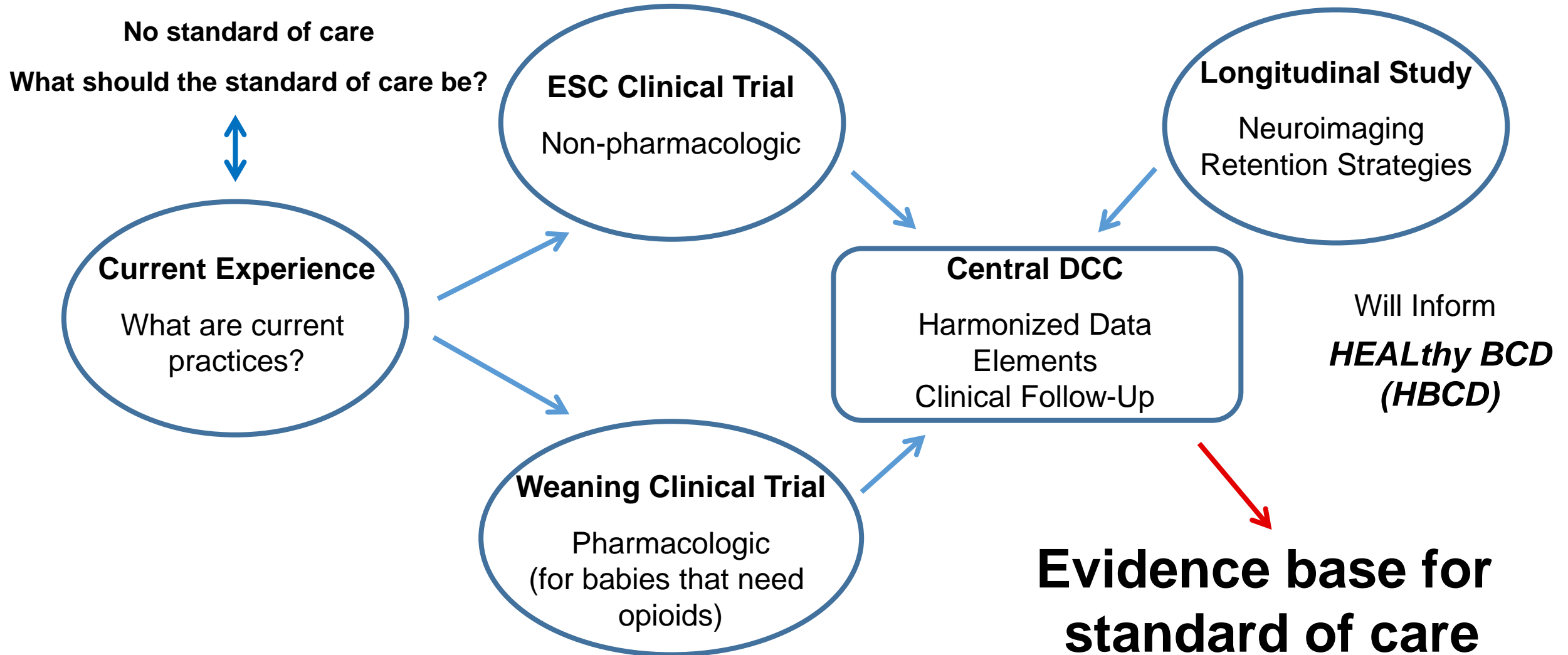


Specific Questions To Be Addressed by the ACT NOW Initiative

- How are clinicians treating babies now? How much variability exists?
 - ACT NOW Current Experience Study
- Do all babies need opioids for withdrawal symptoms? Can some babies be treated non-pharmacologically? Is it safe to do so? What are the long-term consequences?
 - ACT NOW Eat, Sleep, Console (ESC) Cluster Randomized Clinical Trial
- For those babies who do need opioids, can clinicians get them off the medications earlier? Is it safe to do so? What are the long-term consequences?
 - ACT NOW Weaning Randomized Clinical Trial
- What happens to these babies in the long term? To what extent is NOWS associated with atypical neurodevelopmental outcomes?
 - ACT NOW Longitudinal Cohort Study



ACT NOW Initiative Summary



Congressional Directive to Increase Research on DS

- In the FY 2018 budget legislation for NIH:
 - “Develop a new **trans-NIH** initiative ... to study trisomy 21, with the aim of yielding scientific discoveries to improve the **health and neurodevelopment of individuals with Down syndrome** and **typical individuals** at risk for:
 - Alzheimer's disease
 - cancer
 - cardiovascular disease
 - immune system dysregulation
 - and autism,
 - among others...”
- Unique double benefit: understanding both **Down syndrome** and **shared common conditions** (risks or resiliencies)

Protected from:

- Many solid tumors
- Atherosclerosis and heart attacks



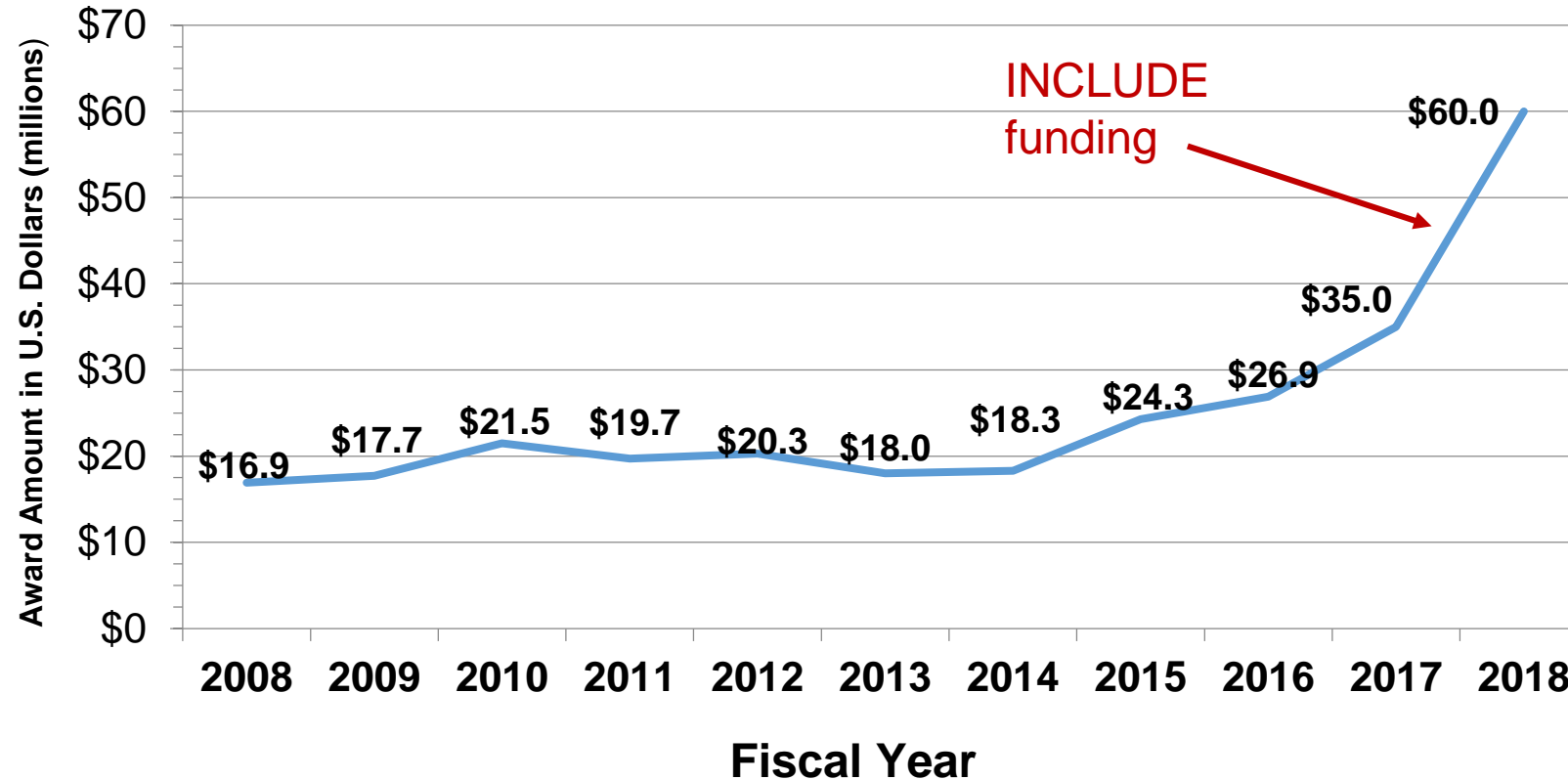
INCLUDE (INvestigation of Co-occurring conditions across the Lifespan to Understand Down syndromE)

3 components:

- 1. Conduct targeted, high-risk, high-reward basic science studies on chromosome 21.**
- 2. Assemble a large cohort of individuals with Down syndrome across the lifespan for comprehensive phenotyping and biomarker analysis.**
- 3. Include individuals with Down syndrome in existing and future clinical trials.**



NIH Funding for Research on Down Syndrome FY 2008 – FY 2018



FY2018 INCLUDE Funding

- ~\$23 M supported 49 supplements to existing NIH grants
- Funding went to NIH OD, distributed among 14 Institutes
- All 3 components addressed
- Data sharing expectation
- Leverage DS-Connect® when possible
- URL: <https://www.nih.gov/include-project>

INCLUDE Project Research Plan



The INCLUDE Project: Updates from FY 2019

- Congressional Directive in FY19 report language to NIH:
“continue to make investments in Down syndrome research that prioritize funding for both **research grants and early-stage investigators** that will **expand the current pipeline** of Down syndrome research, as well as implementation of the new trans-NIH initiative.”

Recent Fiscal Year 2019 Funding Opportunity Announcements (FOAs)

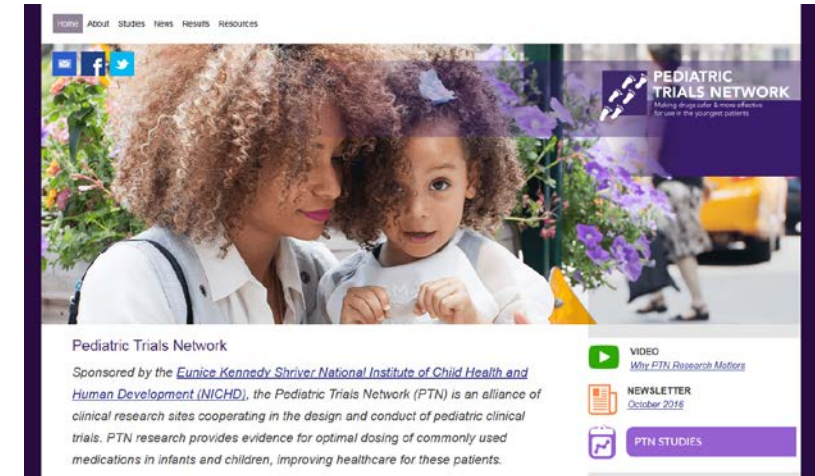
- R21 Clinical Trial Readiness Research Award (RFA-OD-19-015)
- R61/R33 Phased Awards for Clinical Trials for Co-Occurring Conditions in Individuals with Down syndrome (RFA-OD-19-018)
- **R01 Transformative Research Award (RFA-OD-19-016) for basic science studies**
- Notice of Availability of Competitive Supplements/Revisions (NOT-OD-19-071)
- Notice of Availability of Administrative Supplements (Clinical Trial Optional) (NOT-OD-19-087)

In addition, NIH institutes and centers were encouraged to increase overall funding for research on DS using non-INCLUDE funds from their own budgets



NICHD's Pediatric Trials Network (PTN)

- Studies the formulation, dosing, efficacy, and safety of drugs and medical devices used in children
- Conducts phase 1-4 clinical trials for many diverse conditions
- Supported by NICHD through the Best Pharmaceuticals for Children Act (BPCA), legislation that mandates drug studies in children to enable FDA labeling
- PTN clinical research sites are located at over 100 universities and children's hospitals across the U.S.
 - A Coordinating Center/Operations Core is located at Duke
- The PTN will partner with INCLUDE to support drug studies in individuals with Down syndrome
 - Additional objective is to train investigators to perform research on people with IDD



See www.Pediatrictrials.org



Future Activities in DS Research: 2019 and Beyond

- Issue FY19 awards and supplements by September 2019
- Plan FY2020 initiatives
- Develop a clinical trial infrastructure for adult and pediatric studies in DS
- Outreach activities
- Workshops:
 - Virtual Down Syndrome Cohort Across the Lifespan, Sept 23-24, 2019. Webcast option will be available. [URL will be available very soon!]
 - State of the Science for Clinical Trials in Down syndrome, May 2020.
 - Planning clinical trials for Alzheimer's disease



Task Force on Research Specific to Pregnant Women and Lactating Women (PRGLAC)

- Report submitted to HHS Secretary and Congress in September 2018
- Key recommendations include:
 - Change existing culture that has limited scientific knowledge of therapeutic product safety, effectiveness, and dosing for pregnant and lactating women
 - Protect *through* research instead of *from* research
 - Remove pregnant women as a vulnerable population through Common Rule
 - Expand workforce of clinicians and researchers with expertise in obstetric and lactation pharmacology and therapeutics
- Remove regulatory barriers
- All 15 recommendations and full Task Force report are available online:
<https://www.nichd.nih.gov/About/Advisory/PRGLAC>



Early Results: PregSource® Medication Tracker



Medication and Supplement Tracker

[Add](#)
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Select "Add" to list a prescription or over-the-counter medicine, vitamin, or herbal supplement. If you stopped taking an item or need to change information about it, select "Edit" next to that item.

Current Medications and Supplements

Add Medication or Supplement

Type the name of a medication, vitamin, or herbal supplement in the search field to add that item to your list.

What are you taking?

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Search

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Systemic Formulas Bio Command 3 [Bactrex](#)

Systemic Formulas T3 [Bactrex](#) Tincture

pregsource.nih.gov



Implementation of PRGLAC Recommendations

- Charter extended until March 2021
- Two meetings held (May and August 2019)
- Key Activities:
 - Established four working groups to address subsets of the recommendations and develop plans for implementation
 - Research and Training
 - Regulatory
 - Communications
 - Discovery
 - Existing members and additional *ad hoc* members included in the four working groups
 - Working groups are meeting to draft an implementation plan for 13 of 15 initial recommendations



Charge to PRGLAC Working Groups

- Written Implementation Plan should include:
 - Steps needed to address each recommendation
 - Whether any of the recommendations have already been implemented
 - A plan for implementing them, including potential costs and timelines
 - What agencies and stakeholders should be involved
 - If any existing programs should be established or expanded, those should be noted
 - Metrics to evaluate success of implementation
- Draft plan to be discussed at February 3-4, 2020 meeting at NIH





NICHD Staff Updates

NICHD Executive Officer

- Rodney Rivera, M.S., named NICHD's Associate Director for Administration and Executive Officer
- Rodney has served NICHD in multiple roles, including as our Acting Executive Officer since October 2017 and NICHD's Deputy Executive Officer since August 2015
- Rodney has provided strategic direction, implementation, oversight, and evaluation of core administrative functions



NICHD Deputy Director

- Alison Cernich, Ph.D named NICHD's Deputy Director
- Director of NICHD's National Center for Medical Rehabilitation Research (NCMRR) since 2015. One of her major accomplishments as NCMRR Director was leading the development of the congressionally mandated NIH Rehabilitation Research Plan in 2018, an effort that included coordination with 17 ICs and several external stakeholders
- Alison has been instrumental in the planning, development and implementation of NICHD's 2020 Strategic Plan



Acting Director of NCMRR

- Theresa Cruz, Ph.D., a health scientist administrator in National Center for Medical Rehabilitation Research (NCMRR), will serve as Acting Director
- We will launch a national search to find a new NCMRR Director

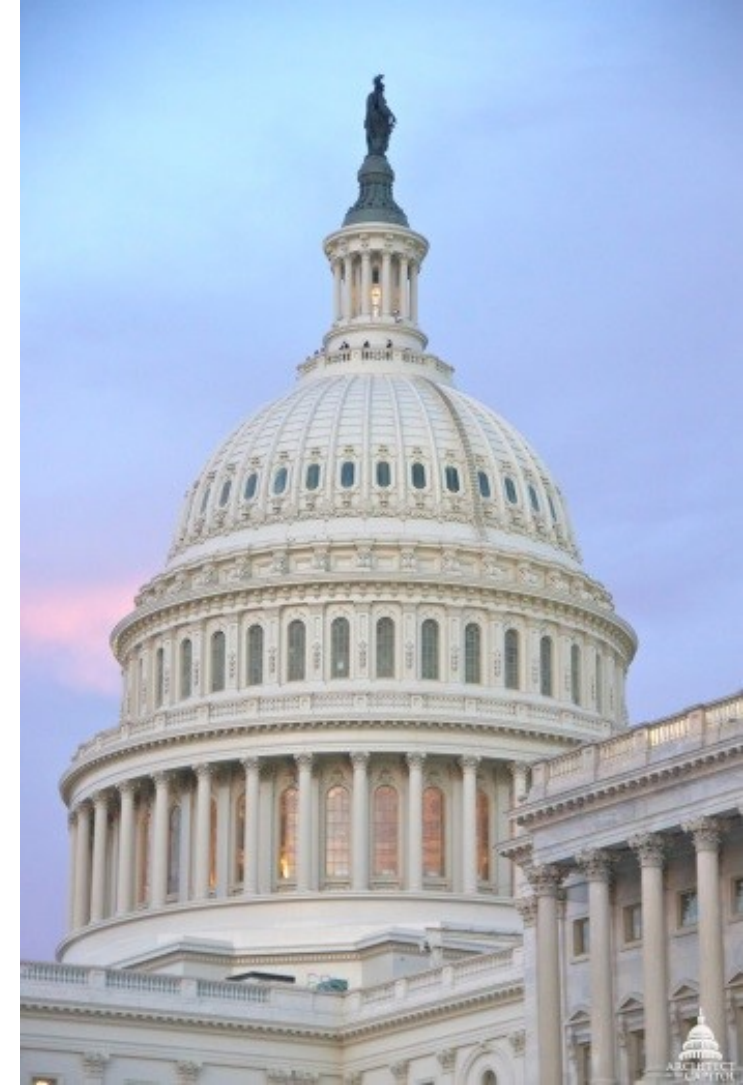




FY 2020 Appropriations

FY 2020 Appropriations

- Federal government is currently funded through September 30, 2019





Questions?