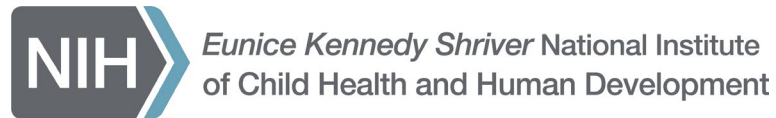


NICHD Director's Report ***NACHHD Meeting***

Diana W. Bianchi, M.D.

January 13, 2025



Talk Outline

- Congressional Updates and Transition
- NICHD Research Highlights
- NICHD Strategic Plan 2025
- Women's Health Research
- Pediatric Research
- NICHD Staff Updates



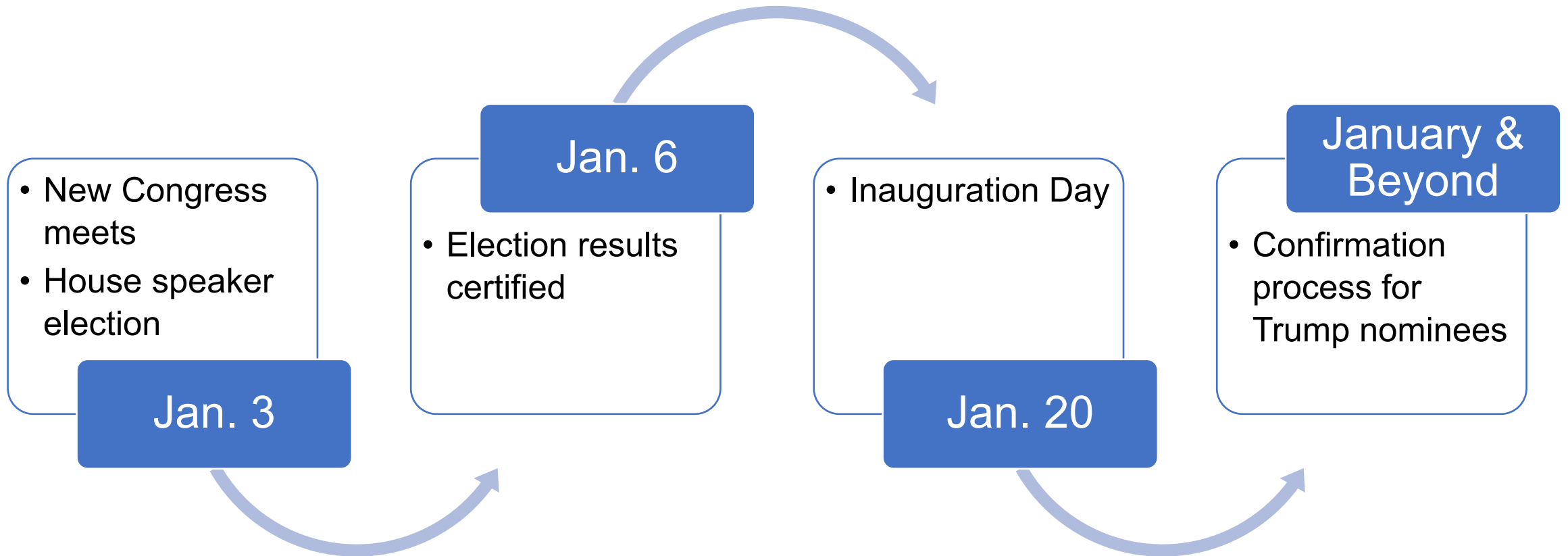
Congressional Updates and Transition

FY25 Appropriations and Administrative Transition

- **Continuing Resolution until March 14, 2025**
 - Nearly halfway through the fiscal year
- **Transition**
 - NIH working with transition teams to prepare for a smooth administrative transition



Anticipated Congressional Timeline



Congressional Confirmations

NIH Director Process





NICHD Research Highlights

Reflecting on a Productive 2024



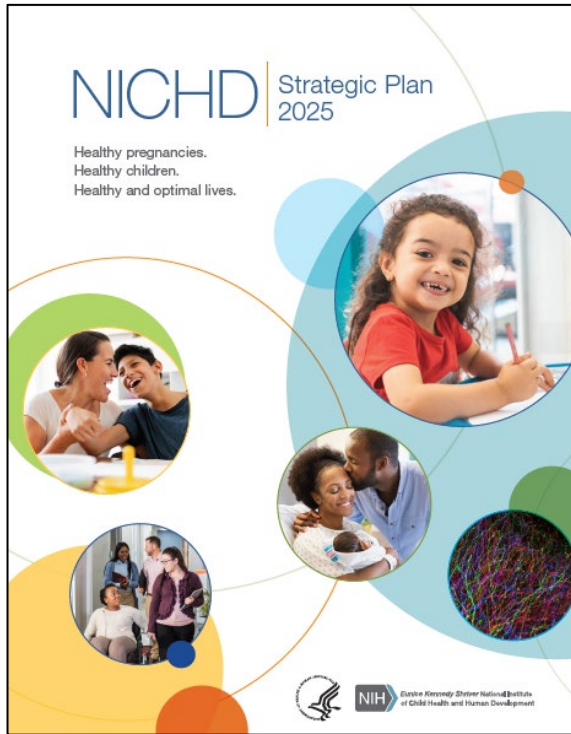
- Visit NICHD's [2024 Selected Research Advances showcase](#):
 - Supporting Women's Health
 - Improving Postpartum Health Outcomes
 - Advancing Fetal and Neonatal Health
 - Improving Care for Pediatric Injury
 - Advancing Child and Adolescent Health
 - Treating and Understanding Pediatric Infections
 - Addressing Health Disparities for People with Disabilities
 - Expanding Research on Intellectual and Developmental Disabilities
 - Advancing Medical Rehabilitation Research
 - Discovering How Cells are Programmed





NICHD Strategic Plan 2025

“Refreshed” NICHD Strategic Plan 2025 – Almost finalized!

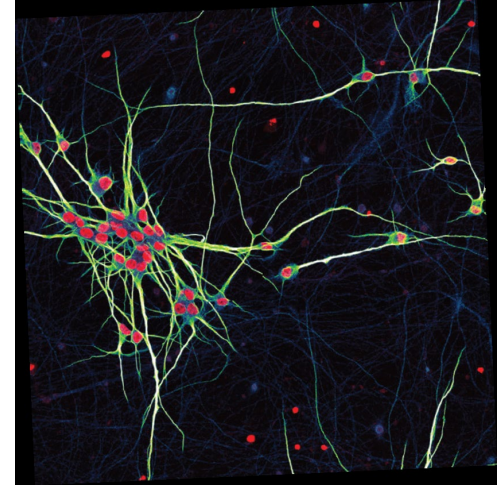


- Overarching scientific goals remain the same:
 - Understanding the Molecular, Cellular, and Structural Basis of Development
 - Advancing Gynecologic, Andrologic, and Reproductive Health
 - Setting the Foundation for Healthy Pregnancies and Lifelong Wellness
 - Improving Child and Adolescent Health and the Transition to Adulthood
 - Fostering Safe and Effective Therapeutics and Devices for Pregnant Women, Lactating Women, Children, and People with Disabilities
- Objectives updated within these goals
- Scientific Stewardship and Management & Accountability goals
- Appreciate Council’s feedback throughout the process



Overview of Updates to Strategic Plan Objectives (1)

- **Understanding the Molecular, Cellular, and Structural Basis of Development**
 - New objectives take advantage of new technologies in this research area, including AI
- **Advancing Gynecologic, Andrologic, and Reproductive Health**
 - Stronger emphasis on gynecologic conditions and contraception
 - Prior Strategic Plan focused on developing fundamental knowledge in reproductive health, and we have made great strides.
 - 2025 plan shifts focus to translation and interventions to improve gynecologic health



Overview of Updates to Strategic Plan Objectives (2)

- **Setting the Foundation for Healthy Pregnancies and Lifelong Wellness**

- IMPROVE and other programs have brought increased focus on maternal interventions aligned with community needs and preferences
- 2025 plan continues focus on maternal and infant health, building on these successes
- Sharpened focus in areas of stillbirth and preterm birth



- **Improving Child and Adolescent Health and the Transition to Adulthood**

- Added a focus on pediatric primary care and interventions to improve the health of children of all ages;
- Retained focus on adolescent transition to adulthood, building on what was started in the 2020 plan



Overview of Updates to Strategic Plan Objectives (3)

- **Fostering Safe and Effective Therapeutics and Devices for Pregnant Women, Lactating Women, Children, and People with Disabilities**
 - Building on the PRGLAC implementation plan and the early years of the MPRINT (Maternal and Pediatric Precision in Therapeutics) program
 - Emphasizes collaborations with other NIH ICs and other organizations, which historically have been successful



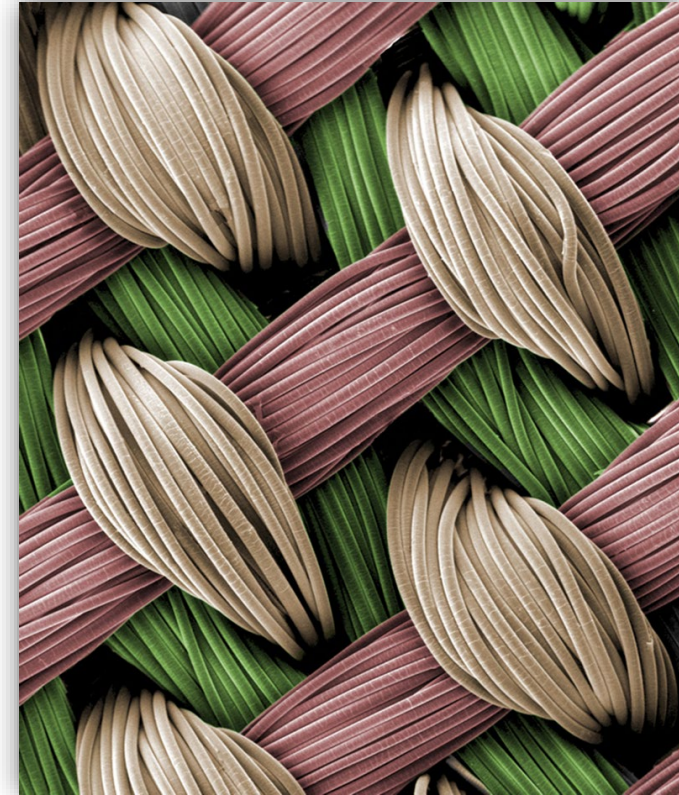
Aspirational Goals

- Some aspirational goals are retained (e.g., diagnose, prevent, and treat endometriosis; limb regeneration)
- Added exciting new goals related to:
 - Improving function for people with neurological impairment
 - Using menstrual effluent and semen to diagnose disorders and conditions
 - Preeclampsia and postpartum hemorrhage
 - Human milk
 - Stillbirth
 - Health of preterm infants
 - Pediatric well care
 - Gene therapy



NICHD Strategic Plan Cross-Cutting Themes

- Health Disparities and Health Equity
- Prevention
- Infectious Disease
- Nutrition
- Global Health
- **Advanced Technologies and Artificial Intelligence**
- **Research Training and Career Development**



Credit: Guilak Lab, Washington University



Women's Health “Below the Belt”



Gynecologic Health and Disease

Contraception Research

Fertility and Infertility

Pregnancy and Perinatology

Maternal and Pediatric Infectious Disease

Obstetric and Pediatric Pharmacology and Therapeutics

Intellectual and Developmental Disabilities

Population Dynamics

Recent JAMA Article Highlights Women's Health Research at NIH

JAMA | Special Communication | **WOMEN'S HEALTH**

Recent Developments in Women's Health Research at the US National Institutes of Health

Janine Austin Clayton, MD; Diana W. Bianchi, MD; Richard Hodes, MD;
Tara A. Schwetz, PhD; Monica Bertagnolli, MD

IMPORTANCE This article highlights key National Institutes of Health (NIH) programs, policies, and scientific advances that have informed and improved the health of women and describe the promise and potential of harnessing cutting-edge science and integrative approaches to advance women's health research. Policy updates combined with recent scientific and programmatic initiatives are intended to expand understanding of women's health, deliver diagnostics, and develop preventive approaches and novel therapies to meet critical health needs of contemporary women.

JAMA.doi:10.1001/jama.2024.25878
Published online January 6, 2025.



National Academies' Study: Assessment of NIH Research on Women's Health

- Study from the National Academies of Science, Engineering, and Medicine (NASEM) released in December: <https://www.nih.gov/about-nih/who-we-are/nih-director/statements/statement-nasem-report-womens-health-research>
- NASEM report states: “female specific conditions, such as fibroids, endometriosis, PCOS, and aspects of the menopause transition, are not within the purview of any specific IC.”
- 42 USC 285g (Sec. 448): Purpose of the Institute. **Provided legislative authority for NICHD to conduct and support "research**, training, health information dissemination, and other programs with respect to **gynecologic health**, maternal health, child health, intellectual disabilities, human growth and development, including prenatal development, population research, and special health problems and requirements of mothers and children.“



NICHD's Research Purview and Portfolio Overlooked in NASEM Report

- [NIH Director Statement](#): *“While the report provides thoughtful recommendations on how NIH can expand its research efforts, it does not acknowledge the full breadth of NIH’s extensive work on women’s health research. It omits the key congressional language that establishes the study of gynecological and pregnancy-related conditions at NIH’s Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) and it understates the significance of ongoing women’s health initiatives supported by NICHD and other NIH institutes.”*
- NICHD’s investment in gynecologic health research is active and growing
- Gynecologic Health and Disease Branch in DER maintains a research portfolio in endometriosis, uterine fibroids, pelvic floor disorders, PCOS, gynecologic pain



RADx® Tech ACT ENDO Challenge

Advancing Cures and Therapies and ending ENDOmetriosis diagnostic delays (ACT ENDO)



- NICHD, partnering with NIBIB, is leveraging Rapid Acceleration of Diagnostics Technology (RADx® Tech) "innovation funnel" program to accelerate time to diagnosis of endometriosis, eliminate invasive techniques and/or improve accessibility
- Robust response to Phase 1 submission
 - Diagnostic approaches include:
 - Imaging, stool-based biomarkers, GI motility, menstrual effluent DNA/RNA/biomarkers
- Next steps: Pitch event, then technology development sprint
- Final winners expected to be announced in March 2026



Pediatric Research



Developmental Biology and Congenital Anomalies

Child Development and Behavior

Intellectual and Developmental Disabilities

Pediatric Growth and Nutrition

Pediatric Trauma and Critical Illness

Maternal and Pediatric Infectious Disease

Obstetric and Pediatric Pharmacology and Therapeutics

Pregnancy and Perinatology

Population Dynamics

NASEM study: Strategies to Enhance Pediatric Health Research Funded by NIH

- Required by FY24 Appropriations Report language
- First meeting held Dec. 5-6, 2024
 - NICHD staff provided the sponsor briefing
 - Presentation slides and video of the meeting are available
- NASEM will hold 3-5 public meetings
- Final report with recommendations anticipated in early 2026
- More information: <https://www.nationalacademies.org/our-work/strategies-to-enhance-pediatric-health-research-funded-by-nih>



Strategies to Enhance Pediatric Health Research Funded by NIH – Statement of Task

- The committee is tasked with examining the current NIH pediatric research portfolio and structure including:
 - The methods and rationale involved in categorizing projects as “Pediatrics” research using the Research, Condition, and Disease Categorization Process;
 - How pediatric components have been included or excluded from larger NIH initiatives;
 - Structural or process impediments to pediatric research applicants;
 - How pediatric research priorities are established within and across ICs; and,
 - How pediatric research activity is coordinated across NIH ICs.
 - Ways in which the NIH Clinical Center (CC) could be used to advance innovative pediatric research



Gabriella Miller Kids First Research Act 2.0 Signed into Law



- New law extends program through 2028
- Fosters collaborative research across NIH to uncover the causes of childhood cancers and structural congenital anomalies
- Supports data sharing within the pediatric research community
- 2024 stats: 12,759 samples sequenced, 11,204 genomes delivered to investigators, and 6,101 genomes released on the Kids First Data Resource Center portal
- Kids First Portal: over 6,500 users from 50+ countries can access clinical data, phenotypes, and genetic variants
- Next steps: enhance KF interoperability efforts; release the most data to the KF Portal in a single year; continue multimodal data integration in the KF portal





NICHD Staff Updates

Lindy Thaker, MD

Chief, Pediatric Growth and Nutrition Branch

- Pediatrician and global nutrition expert
- Prior work with USAID supporting food and nutrition programs
- MD from Indiana University School of Medicine; MPH from Johns Hopkins Bloomberg School of Public Health
- Pediatric nutrition fellowship at The Children's Hospital of Philadelphia; credentialed as a physician nutrition specialist



Maranke Koster, PhD

Chief, Developmental Biology and Congenital Anomalies Branch

- PhD in Developmental Biology from Baylor College of Medicine
- Research has focused on investigating mechanisms that control normal development of ectodermal tissues as well as pathological mechanisms that lead to ectodermal dysplasias
- Faculty positions at the University of Colorado Anschutz Medical Campus and (most recently) Brody School of Medicine at East Carolina University



Check Out Our New Women's Health Research Fact Sheets!

- Assisted Reproductive Technology
- Femtech Small Businesses
- Uterine Fibroids
- Endometriosis
- Infertility
- Polycystic Ovary Syndrome
- Pelvic Floor Disorders
- Covid-19



Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)

Uterine Fibroids and Women's Health

Advancing Diagnostics & Treatments to Improve Patient Care

What are uterine fibroids?

Uterine fibroids, or leiomyomas, are benign tumors that grow in or on the wall of the uterus. Typically made of muscle and connective tissue cells, uterine fibroids are the most common non-cancerous tumors in women of childbearing age. Some women experience debilitating pain, heavy menstrual bleeding, and problems with fertility from fibroids, while others have minor or no symptoms. Research suggests that fibroids occur disproportionately among Black and African American women, who also tend to develop fibroids at younger ages and have more severe symptoms than other groups. Removing the uterus (hysterectomy) is currently the only "cure" for fibroids, although symptom management can also include medication and minimally invasive surgeries.

How does NICHD support research on uterine fibroids?

NICHD funds research to understand the causes and risk factors of fibroids and to identify better ways of diagnosing and treating them. In 2020, the U.S. Food and Drug Administration approved the drug Oriahnn® for treating heavy menstrual bleeding from uterine fibroids. A drug called elagolix, which was initially developed through an NICHD small business grant. In 2024, NICHD launched the Specialized Centers for Research on Health Disparities in Uterine Leiomyoma to learn more about fibroids and explore the disparities related to fibroid risk occurrence, and symptom severity among Black and African American women. The overall goal is to improve gynecological health for all women.

Success Snapshots

Developing a Prototype to Predict Risk

Knowing who is at risk for developing uterine fibroids could facilitate earlier treatment and prevention. With NICHD support, researchers developed a gene-based score to help predict aspects of fibroid disease. Created by analyzing genome-wide scans and electronic health records, these polygenic risk scores estimate the likelihood that a person will have a particular pattern of fibroid disease characteristics, as well as their severity of symptoms. Additional evaluation of the prototype is underway.

Identifying New Uses for Existing Drugs

The oral medication tranilast decreases production of collagen (a building block of muscle, skin, and connective tissues) and inflammation-causing elements. In other countries, it is approved for treating asthma, and issues linked to collagen in inflammatory substance overproduction. Testing in a uterine fibroid mouse model, NICHD-funded researchers found that treatment with the drug for 8 weeks led to notably smaller tumors compared to untreated mice. This work suggests new uses of existing drugs for treating fibroids.

Selected NICHD-Funded Projects on Uterine Fibroids

Fibroid Origins & Health Disparities

Understanding the Role of Genes

To explore fibroid origins, a NICHD-funded study examined gene expression or activity in uterine muscle tissue. The team noted higher expression of the von Willebrand factor gene (VWF) in uterine muscle samples from Black women, compared to White women. VWF includes instructions for a protein that regulates blood vessel formation, a key aspect of fibroid growth.

Another NICHD-supported team used new genetic sequencing technology to identify 21 genes that are expressed differently in fibroid tissue samples from Black and Hispanic women, compared to samples from White women. Understanding how genetics may contribute to racial disparities can help inform care.

Linking Ancestry to Fibroid Characteristics

NICHD-funded researchers linked fibroid risk and features among U.S. Black and White women to their ancestry. For Black women: West African ancestry correlated with risk for a single fibroid, East African ancestry correlated with risk for multiple fibroids, and Northern European ancestry protected against multiple fibroids. For White women: Northern European ancestry protected against fibroids; and West African ancestry was linked to risk for larger fibroids. These insights may help quantify risk and define treatments.

Identifying the Effects of Stress on Fibroids

Exposure to stress affects health in many ways. NICHD-supported researchers found that women with fibroids who scored high on questionnaires about stress had high levels of microRNAs linked with tumor growth in their uterine muscle. The researchers suggest that stress from structural racism and other situations may explain some disparities in fibroid rates.



Fibroid Factors, Effects, & Treatments

Identifying Environmental Effects on Risk

Exposure to phthalates, chemicals in personal care products, cosmetics, and medical items, has wide-ranging health effects. One NICHD-funded study found that phthalate exposure may trigger the growth of fibroid cells and delay the rate at which they die. This finding explains higher fibroid rates in phthalate-exposed women and suggests that reducing such exposures may prevent fibroids.

Other NICHD-funded research discovered that the green-tea compound epigallocatechin gallate (EGCG) reduced levels of substances that promote cell division and proteins that cause the tumors' fibrous contents. This team now leads a NICHD-funded clinical trial to test if EGCG reduces fibroid symptoms, including improving fertility.

Evaluating Fibroids' Role in Fetal Growth

NICHD researchers found that uterine fibroids do not seem to restrict fetal growth in pregnancy. Earlier studies suggested that fibroids could cause smaller full-term infants. The study also confirmed a higher risk of preterm birth for pregnant women with fibroids. These findings can help optimize outcomes for those with fibroids.

Developing Non-Surgical Treatments

Current non-invasive fibroid treatments reduce symptoms, but do not affect tumor size or growth. NICHD-funded researchers created a new method that shrinks fibroids non-invasively in mice by encasing a tumor-killing drug in nano-sized spheres, and using the bloodstream to deliver the spheres directly to the fibroid. They are now exploring whether the technique is safe and effective for treating fibroids in humans.

Learn More About NICHD Uterine Fibroid Projects



NICHD's Fibroids Website:
<https://go.nih.gov/9vCKUes>





Thank You!
Questions?

RADx[®] Tech ACT ENDO Phase 1 Winners



- 3CPM Company, Inc.
- EndoCyclic Therapeutics
- Endometrics Ltd
- Feinstein Institutes - Northwell Health
- Sur180 Therapeutics, INC
- NextGen Jane
- Rudra Therapeutics
- Washington University in St. Louis

Diagnostic approaches include:

Imaging, stool-based biomarkers, GI motility, menstrual effluent DNA/RNA/biomarkers

