



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

# Endometriosis Research Progress

## *Advancing Women's Health Through Better Understanding, Diagnosis, and Treatment*

### What is endometriosis?

Endometriosis is one of the most common gynecological diseases, affecting an estimated 10% of U.S. women. It occurs when tissue similar to the uterine lining grows outside the uterus, which can cause pain, sometimes severe, and infertility. Many women endure years of symptoms before getting a diagnosis, which can only be confirmed through surgery. Available endometriosis treatments, ranging from hormone pills to hysterectomy (surgical removal of the uterus), are not effective for everyone. Some treatment options leave a chance for recurrence, and many have challenging side effects.

### How does NICHD support endometriosis research?

Endometriosis has been a high priority for NICHD for decades. The institute has funded research to improve health outcomes for women living with or at risk for endometriosis, including the development of elagolix (Orilissa®), the first treatment for endometriosis-related pain approved in the United States. NICHD currently supports more than 50 projects focused on understanding the causes and mechanisms of the disease, and on developing effective ways to prevent, diagnose, and treat it. The institute also leads the new initiative, *Advancing Cures and Therapies and Ending Endometriosis Diagnostic Delays*, or ACT ENDO, to develop and improve endometriosis diagnostics and treatments through cross-disciplinary projects. NICHD is working to improve the quality of life for women with endometriosis and their families.

## Success Snapshots

### **Treating Endometriosis-Related Pain (Neurocrine Biosciences, Inc.)**

Pain from endometriosis can be debilitating. More than 20 years ago, small business support from NICHD enabled Neurocrine Biosciences, Inc., to investigate treating endometriosis pain with experimental drugs that block the action of gonadotropin-releasing hormone (GnRH), a key hormone in the menstrual cycle. NICHD-funded work on these drugs, called non-peptide GnRH antagonists, paved the way for clinical studies on elagolix (Orilissa®). In 2018, it became the first pill specifically approved for endometriosis pain relief in the United States.

### **Building a Menstrual-Based Diagnostic for Endometriosis (NextGen Jane)**

People with endometriosis often wait up to 10 years between symptom onset and surgical diagnosis. A non-invasive diagnostic would enable earlier detection and treatments that could prevent disease progression. With small business support from NICHD, NextGen Jane's "smart tampon" kits collect menstrual blood from study participants with endometriosis. After completing more than 2,000 kits, the company is working to validate an assay to diagnose endometriosis from these samples.

## Selected NICHD-Funded Endometriosis Projects

### Diagnostics & Treatments

#### Modifying the Disease Course

Current endometriosis treatments do not cure or reverse the disease course, meaning new therapies that delay or interrupt progression are urgently needed. With small business support from NICHD, researchers at a biopharmaceutical company developed and are evaluating a new non-hormonal endometriosis treatment. Early studies suggest that the compound eliminates the hallmark lesions of the disease, meaning it could be a first-of-its-kind curative therapy.

#### Reducing Treatment Side Effects

Hormonal drugs that treat endometriosis by suppressing estrogen levels can cause adverse effects ranging from bone density loss to hot flashes; identifying non-hormonal treatment options is a priority. Oleuropein, a compound found in olive oil and olive leaves, blocks a certain type of estrogen receptor, making it less likely to cause these side effects. NICHD-funded studies in mice suggest that it may reduce endometriosis lesions and improve the chances of pregnancy. Additional work will determine whether the treatment also benefits humans.

#### Removing Tissue Without Surgery

NICHD-supported research in mice is examining magnetic hyperthermia, which uses heat to remove disease-causing tissues, as a non-surgical endometriosis treatment. The procedure, adapted from cancer treatments, delivers magnetic nanoparticles that accumulate specifically in endometriosis tissue. Applying an alternating magnetic field causes the particles generate heat, effectively “burning away” the lesions. This non-surgical technique appears safe and effective, although more work is needed before it can be evaluated in humans.



### Knowledge Advances

#### Confirming an Increased Risk for Stroke

Previous studies have linked endometriosis to increased risk for cancers, autoimmune diseases, and cardiovascular problems, including stroke. But findings on the nature of the link to stroke were inconclusive. An NIH-funded analysis of more than 110,000 women found that those diagnosed with endometriosis had a 34% higher risk for stroke than women without endometriosis. Women with endometriosis should consider discussing potential risk for stroke and other cardiovascular conditions with their health care team.

#### Identifying Differences in Gene Expression

Existing endometriosis knowledge does not explain why only some women get the disease. Recent findings from NICHD-funded work identified several DNA modifications that contribute to differences in gene expression (the degree to which certain genes are turned on or off) in tissue samples from women with endometriosis and those without the disease. Specifically, they measured variations in DNA methylation, a chemical modification that influences gene activity. These results will help improve understanding of why the effects of endometriosis are so varied and may inform new treatment approaches.

**Learn More About NICHD  
Endometriosis Projects**



**NICHD Endometriosis Website:**  
<https://go.nih.gov/M0gpYZy>



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