A novel pill to treat bleeding from uterine fibroids aims for FDA approval

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For millions of premenopausal women, uterine fibroids turn their monthly periods into virtual hemorrhages.

A new drug called elagolix cut blood loss by half over six months in the overwhelming majority of women who participated in two clinical trials published Wednesday in the *New England Journal of Medicine*.

Elagolix, being developed by AbbVie, was approved by the U.S. Food and Drug Administration in 2018 under the brand name Orilissa to reduce the pain of endometriosis—another common, debilitating female disorder.

The fibroid studies, conducted at 77 sites in the United States and Canada, were led by William D. Schlaff, the chair of obstetrics and gynecology at Thomas Jefferson University in Philadelphia. The results have been submitted to the FDA, which is expected to issue its decision in the first half of 2020.

Elagolix works by suppressing the gonadotropin hormone and the ovarian sex hormones, estrogen and progesterone. In effect, it throws women into a temporary menopause.

Because this suppression can cause bone loss, the researchers gave a subset of women low doses of sex hormones along with elagolix. Of those 395 women, about 70% cut their blood loss by half without suffering more bone thinning than women taking a placebo. The "add-back" hormones also reduced menopausal side effects such as hot flashes and night sweats, although these remained common.

"As a clinician working with patients like this for 40 years, I think this is a valuable clinical tool," Schlaff said of elagolix and add-back hormones. "It's oral, the effect is fast onset, and the side-effect profile is tolerable."

The brand name and pricing that would be used if the FDA approves this use of elagolix have not yet been established for the drug, an AbbVie spokesperson said. The list price for a four-week supply of Orilissa is $907.39.

An estimated 80% of women approaching menopause have fibroids—muscular growths in the uterine wall. Half of those women will develop symptoms, primarily heavy menstrual bleeding, which can lead to a blood iron deficiency. Charlotte Owens, medical director at AbbVie, noted that African American women have a higher risk for uterine fibroids and often develop more severe symptoms than Caucasian women.

After menopause, when the ovaries shut down, menstrual bleeding stops, but many women find the transitional bleeding so troublesome that they seek treatment.

Current options—including drugs that target hormones, procedures that destroy the uterine lining or surgical removal of the fibroids or entire uterus—all have drawbacks. A device called an electric morcellator, which minces fibroids and removes the tissue through tiny incisions, has been largely abandoned because in rare cases it can disseminate an undetected uterine cancer. Philadelphia cardiac surgeon Hooman Noorchashm and his late wife, anesthesiologist Amy Reed, campaigned for a ban on morcellators after her cancer was spread during a hysterectomy.

Existing gonadotropin hormone-suppressing drugs, including one approved in 1989 that Schlaff helped to test in patients, have to be given

as injections and take up to two weeks to begin working.

Other companies besides AbbVie have been working to tap the potentially huge market of women with fibroid-related bleeding. A few years ago, Allergan seemed to be in the lead with ulipristal acetate, brand name Esmya, which was already approved in Europe. But after European regulators initiated an investigation into whether the drug led to liver damage in some patients, the FDA declined to approve it.

Myovant Sciences' relugolix, which reduced blood loss in nearly three-quarters of patients in the second of two late-stage clinical trials, is also seeking approval for the drug in combination with add-back hormone medications.