Researchers at the University of South Florida have performed preclinical trials that may lead to a possible treatment for endometriosis through the use of the autophagy inhibitor hydroxychloroquine.

Endometriosis is a gynecological disease which afflicts women of childbearing age. Although itself benign, it is a very painful condition characterized by endometriotic lesions at ectopic sites leading to infertility and an increased risk of specific subtypes of ovarian cancer. The causes of endometriosis are unclear. Although formation of an endometriotic lesion is dependent on a combination of cellular events, the first step is cell survival, which, if it occurs, will allow endometrial cells to implant and increase its potential of developing into a lesion.

Autophagy, a “self-eating” process whereby damaged cellular material and organelles are sequestered in autophagosomes and degraded, is a survival mechanism activated in response to multiple stresses. If autophagy is activated, endometrial cell survival, implantation at ectopic sites, and generation of endometriotic lesions would ensue. Thus, inhibition of autophagy will trigger anoikis providing a unique molecular basis for novel therapeutic targeting of the endometrial cells to hinder lesion formation.

Our inventors have discovered a potential new treatment, through preclinical studies, for endometriosis using the autophagy inhibitor hydroxychloroquine. The inventors successfully inhibited autophagic flux via the use of hydroxychloroquine, which altered the number of lesions formed in the mouse model. The use of hydroxychloroquine could potentially promote an anoikis response, thus leading to a decrease in the likelihood of implantation and lesions formation of endometrial cells. This has the potential to become an important and previously uninvestigated treatment strategy for patients with endometriosis.

**ADVANTAGES:**
- Decrease development of lesions
- Decrease risk of ovarian cancer
- Decrease pain

**Potential New Treatment Avenues for Endometriosis**

![Figure: Lesion count in endometriosis mouse model treated with hydroxychloroquine (HCQ) versus PBS](image)

**Stage of Technology: Preclinical**