Health Decisions

Women's health

Capabilities for Clinical Studies in Endometriosis

An Area of Significant Unmet Medical Need

Endometriosis affects approximately 10% of premenopausal women. Some patients are asymptomatic but many women experience symptoms that may include infertility, dysmenorrhea, dyspareunia and chronic pain. Up to 50% of women dealing with infertility and 70% of women experiencing pelvic pain may have endometriosis. There is also data suggesting an association between endometriosis and ovarian cancer and perhaps other cancers.

Treatment options include pain medications, hormone therapies and surgical procedures. However, currently available treatments are often ineffective for women experiencing moderate to severe pain from endometriosis. In addition, hormonal treatments have side effects that may include menopausal symptoms such as hot flashes, fatigue, headache, bone loss and vaginal dryness. Moreover, endometriosis tends to recur following discontinuation of hormonal treatments. Surgical treatments may involve excision or ablation of lesions. Surgical procedures provide short-term pain relief for women with moderate endometriosis. Unfortunately, as with hormonal treatments, endometriosis pain may recur. Thus, many endometriosis patients have significant unmet medical needs.

Fortunately, endometriosis research is an area of intense activity. Novel treatments that are currently the subject of clinical investigation include GnRH antagonists, selective progesterone receptor modulators and selective dopamine (D2) receptor agonists. Health Decisions understands the operational and methodological challenges in clinical development of investigational drugs and procedures for treatment of endometriosis.

Operational And Methodological Considerations In Endometriosis Trials

A Motivated Patient Population

One factor that promotes enrollment is that women who have not found an effective treatment for moderate to severe endometriosis pain are often highly motivated to participate in clinical studies of investigational treatments.

Key Considerations in Endometriosis Studies

- Ensuring subject completion of daily diaries
- Verifying subject adherence to study medications
- Addressing variability of subjective assessments
- Recruitment challenges based on patient acceptance of:
  - A long screening period
  - A "washout" period for existing medications
  - A commitment to six or more monthly visits
  - Risk of randomization to placebo
  - Required use of dual methods of nonhormonal contraception
  - Allowable rescue medications

A High Perceived Burden of Study Participation

On the other hand, endometriosis trials impose a long list of requirements on subjects:

- Studies typically include washout periods for hormonal treatments
- A screening period typically spans two menstrual cycles and includes asking women to change from their accustomed analgesics to those allowed as rescue medications during the study
- Risk of randomization to placebo is a serious consideration for patients with any painful condition
- There are generally monthly visits for six months or more
- There will likely be a requirement to use two methods of nonhormonal contraception to minimize risk of pregnancy for this premenopausal population
- Patients must commit to completing a daily diary about pain and medication use.

Not surprisingly, some patients in endometriosis trials are likely to find one or more of these requirements burdensome. Both recruitment and subject retention can present issues in endometriosis trials. As with any painful condition, risk of randomization to placebo may discourage some women from enrolling even when there is provision for rescue medication. For those who do enroll and especially those randomized to placebo or to a possibly less effective lower dose of an investigational drug, there is a risk of discontinuation. Studies may observe discontinuation rates on the order of 25%.

Issues with Assessments and Compliance

Important considerations in endometriosis trials include data quality and statistical analysis techniques. Pain assessments in endometriosis trials involve both dysmenorrhea and nonmenstrual pelvic pain. Furthermore, pain is assessed in multiple ways. Assessments involve both subjective scales and frequency and quantity of rescue medication used. Because the effects of endometriosis are multidimensional, trials may involve secondary endpoints for dyspareunia, quality of life, productivity and perhaps other areas. Subjective assessments always present the risk of high data variability.

Patient adherence is another important consideration in data quality and so it is important to ask patients to return unused medication and dispensed medication packages to verify adherence. For patients who discontinue a trial prematurely, there must be a decision about handling of missing pain-assessment data (e.g. the last-observation-carried-forward method). Still another consideration in studies involving a placebo control is the placebo effect itself.

For Assistance With Your Endometriosis Program

While successful execution of endometriosis studies involves many challenges, there is no question about the importance of ensuring that these studies are conducted with high quality and efficiency. Endometriosis is an area of significant unmet medical need affecting approximately 10% of women of reproductive age worldwide – some 176 million women.

As a CRO committed to improving healthcare outcomes for women, Health Decisions is determined to advance and accelerate clinical development of improved treatments for endometriosis. In addition, the Health Decisions clinical team understands how to address key operational issues and mitigate risks in endometriosis trials. Please contact us to explore how Health Decisions can assist with efficient, timely development of your investigative product for treatment of endometriosis.

If you are developing women's health products -

Make Your First Decision the Right Decision - Health Decisions

The Leading Full-Service Women's Health CRO from Pre-IND or -IDE to Regulatory Approval and Beyond

Health Decisions is a full-service CRO specializing in clinical development of drugs, diagnostics, medical devices and combination drug/devices in all areas of women's health as well development of diagnostics for all therapeutic areas. Our experience, expertise, site network and KOL and investigator relationships enable us to address the challenges of developing women's health products in areas including general gynecology indications, contraception, sexual health, infertility, obstetrics, menopause and osteoporosis and other indications that affect women disproportionately and profoundly, including autoimmune disorders and male-factor infertility. Health Decisions is headquartered in Durham, NC.

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