



Lunch Break

Patient-Centered Outcomes Research Institute



Targeted PFA Ad Hoc Workgroup Treatment Options for Uterine Fibroids

Questions for consideration

March 5, 2013

Patient-Centered Outcomes Research Institute

Major Categories of Questions

- 🌱 **Alternatives to hysterectomy/ reproductive outcomes**
- 🌱 **Diagnostic Options**
- 🌱 **Fertility**
- 🌱 **Genetics/racial/ethnic considerations**
- 🌱 **Symptom Management**
- 🌱 **Prevention**
- 🌱 **Fibroid Causal/Correlated Relationships**
- 🌱 **Health care systems**

Alternatives to hysterectomy / Reproductive outcomes

- Do reproductive outcomes (ovarian function, fecundity, pregnancy complications) differ between different alternatives to hysterectomy for symptomatic fibroids?
- What is the comparative likelihood of specific reproductive outcomes (ability to get pregnant, live birth, preterm birth, cesarean section) following non-hysterectomy invasive treatments for symptomatic fibroids?
- How do uterine-conserving procedures impact fertility, pregnancy outcomes, and recurrence?
- Does removal or retention of the cervix affect sexual functioning? (dyspareunia or orgasm)
- Is hysterectomy the appropriate patient-centered benchmark of therapy?

Alternatives to hysterectomy / Reproductive outcomes (*continued*)

- How to choose the most appropriate treatment modalities (e.g., non-surgical options) that are suitable for my individual case? Situations? Risk factors?
- How safe and effective are the newest technologies for the treatment of uterine fibroids?
- Why do gynecologists mainly promote myomectomy? How do we promote shared decision making for other options? How do you train physicians in new technologies and treatment options?
- How does radiofrequency ablation of fibroids with this newly approved Acessa Procedure, compare with standard fibroid treatment of UAE, Myomectomy or hysterectomy in terms of quality of life and symptom improvement such as heavy bleeding?
- Does preoperative imaging with MRI affect surgical management and clinical outcome?

Alternatives to hysterectomy / Reproductive outcomes (*continued*)

- What is the comparative effectiveness of alternative to surgical therapies, current and novel comparisons?
- What are the best treatment option for patients approaching menopause?
- Is there any place for use of Aromatase inhibitors for the treatment of uterine fibroids?
- Is there a really benefits for use of GnRH agonists prior to surgery compared to cost?
- What is the success rate (QOL, re-intervention, etc) for medical therapy with progesterone receptor modulators compared to surgical treatment for symptomatic uterine fibroids in women in the peri-menopause?
- How do long-term (5-10 years) quality of life measurements differ between UFE and myomectomy?

Alternatives to hysterectomy / Reproductive outcomes (*continued*)

- Is there any randomized trial to support the superiority of one treatment over the other (i.e., surgical vs. embolization) using patient centered outcomes?
- For women with large uterine fibroids, what is the long-term comparative efficacy with respect to recurrence of symptoms requiring re-intervention if treated with laparoscopic myomectomy, robotic myomectomy, or abdominal myomectomy? Is there a racial disparity?
- Can fibroids be treated by medication to reduce blood pressure?
- What is the best tool to stage treatments, including the pharmacotherapeutic options as initial therapy, on durability of symptom relief and patient reported outcomes?
- What is the comparative effectiveness of appropriately timed and conceived noninvasive therapies?

Diagnostic Options

- How can effective family history of fibroids identify high-risk patients and help target early diagnosis?
- What interventions using genetic diagnostics can identify women who are more likely to develop uterine fibroids?
- What are the anatomic biomarkers that can risk stratify patients?
- What comparative studies using genome-wide association studies (GWAS) and meta-analyses of existing data could lead to tests of targeted therapies?
- Which comparative diagnostic interventions studies using genetic subtypes from cell-free DNA could predict tumor behavior?

Fertility

- What is the relationship between fibroids and fertility/infertility before and after treatment?
- Does early intervention affect future treatments/prognosis for symptoms and fertility?
- Pregnancy outcomes after uterine arterial embolization (UAE) for treatment of fibroids?
- For women who desire fertility and have uterine fibroids, how do pregnancy outcomes (fertility rates, live born infants, obstetrical complications, etc) compare between ablative technologies and excisional procedures – specifically myomectomy vs. MRgFUS vs. UF? Compare UAE and effect on fertility?

Fertility (*continued*)

- For women who wish to become pregnant and who have symptomatic fibroids requiring therapy, which fibroid therapy is best?
- Does this choice vary with patient age, extent of disease, prior surgical therapy or interval after treatment before pregnancy is desired? Esp for menopausal patients?
- What is the comparative re-treatment rate of fibroids after myomectomy (all approaches), uterine embolization and focused ultrasound and does this vary with patient age, extent of disease, prior surgical therapies and other patient specific factors?

Racial/ethnic considerations

- Which treatment strategies work best for women of different races and ethnicities, age groups, and other risk factors?
- Is uterine fibroid treatment less effective for African American women than other women who are getting fertility services?
- Are there racial differences in accessing treatment options?

Symptom Management

- Does the comparative effectiveness of available treatments for symptomatic uterine fibroids differ based on a patient's specific symptoms?
- What quality of life measures for fibroid symptoms could be studied for comparative assessment of treatment options?
- How do we promote shared decision making with patients and providers as they weigh treatment options (medical therapy, interventional options)?
- What are the best treatment options for women with severe symptoms or disease, who want to bear children?
- How does Acessa (radiofrequency ablation of fibroids) compare with high-intensity focused ultrasound in symptomatic reduction of pelvic pain and bleeding?
- What is the proper efficacy endpoint for noninvasive fibroid therapies based on patient and disease factors?
- What is the appropriate time frame for intervention?

Fibroid Causal/Correlated Relationships

- What is the evidence demonstrating a relationship between symptoms and development of uterine fibroids
 - i.e.—is successful treatment incorrectly equated with causality?
 - Controlled prospective cohort studies of women with and without disease
- Molecular aspects of abnormal fibroid growth and relationship to hormonal milieu
 - Signal pathways regulating growth using biomarkers for risk stratification
 - Developmental issues (intrauterine or early environment)
 - Dietary relationship

Health care systems

- Are there patient, provider, and/or health system characteristics that contribute to more “advanced” fibroids at time of treatment in African American women (using staging guidelines)?
- Are women more satisfied with the treatment outcome when they receive counseling as part of an interdisciplinary team?
- Do differences in the availability of coverage for treatment of symptomatic fibroids affect the risk of complications in women undergoing invasive treatments (hysterectomy, myomectomy, UAE)?
- Does preoperative imaging with MRI affect surgical management and clinical outcomes?

Health care systems (*continued*)

- How does access to multiple specialists affect patient outcomes and choices, especially for rural populations? What telehealth options could promote treatment options for patients?
- How do we modify communication and engagement to be culturally sensitive and at the right level? Can we improve on patient education tools to ensure they are meeting the needs of today's women? Use health fairs? Waiting rooms?
- Can we compare the performances of hospitals across the U.S. to understand where and why many hysterectomies are being performed?



Discussion of Critical Gaps in Fibroids Research and Key Research Questions

Patient-Centered Outcomes Research Institute

Criteria for Knowledge and Research Gaps

Knowledge gaps should:

- **Be patient-centered:** Is the proposed knowledge gap of specific interest to patients, their caregivers, and clinicians?
- **Assess current options:** What current guidance is available on the topic and is there ongoing research? How does this help determine whether further research is valuable?
- **Have potential to improve care and patient-centered outcomes:** Would new knowledge generated by research be likely to have an impact in practice?
- **Provide knowledge that is durable:** Would new knowledge on this topic remain current for several years, or would it be rendered obsolete quickly by subsequent studies?
- **Compare among options:** Which of two or more options lead to better outcomes for particular groups of patients?



15 Minute Break

Patient-Centered Outcomes Research Institute



Continued Discussion of Critical Gaps in Fibroids Research and Key Research Questions

Patient-Centered Outcomes Research Institute

Criteria for Knowledge and Research Gaps

Knowledge gaps should:

- **Be patient-centered:** Is the proposed knowledge gap of specific interest to patients, their caregivers, and clinicians?
- **Assess current options:** What current guidance is available on the topic and is there ongoing research? How does this help determine whether further research is valuable?
- **Have potential to improve care and patient-centered outcomes:** Would new knowledge generated by research be likely to have an impact in practice?
- **Provide knowledge that is durable:** Would new knowledge on this topic remain current for several years, or would it be rendered obsolete quickly by subsequent studies?
- **Compare among options:** Which of two or more options lead to better outcomes for particular groups of patients?



Recap and Next Steps

Patient-Centered Outcomes Research Institute

We Still Want to Hear From You

- We welcome your input on today's discussions or our process in general
- We're accepting comments and questions for consideration on this topic through 5 pm ET March 19th via:
 - Email (fibroids@pcori.org)
 - Our "Submit a Question on our Targeted Topics for Research Funding" web page
- We'll take all feedback into consideration

Connect with PCORI



www.pcori.org



@PCORI



"PCORINews"



Patient-Centered Outcomes Research Institute



**Thank You for Your
Participation**

Patient-Centered Outcomes Research Institute