



Special Board of Governors Teleconference/Webinar

Eugene Washington, MD, MPH, MSc

Chair, Board of Governors

June 18, 2013

Patient-Centered Outcomes Research Institute

Teleconference/Webinar Agenda

What is being discussed today?

Uterine Fibroids PFA

- Status update and Board vote to move forward with Uterine Fibroids PFA as recommended by the PDC

Asthma PFA

- Public announcement of release of “Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma” PFA

Treatment Options for Uterine Fibroids

David Hickam, MD, MPH

Director, Assessment of Prevention, Diagnosis,
and Treatment Options Program

Uterine Fibroids

Why is this topic important?

- **Uterine Fibroids are of specific interest to patients and stakeholders**
 - As many as 3 out of 4 women have uterine fibroids sometime during their lives
 - There are large disparities within subpopulations, especially African American women
 - Fibroids are associated with a wide range of symptoms and represent a heavy disease burden for millions of women
 - System costs are on par with the combined costs of breast, colon and ovarian cancers in the United States
- **There are large knowledge and research gaps**
 - Lack of consensus on patient classification
 - New less-invasive procedures with limited outcomes data
 - New classes of medications
- **There is a shortage of research that includes patient-centered outcomes (long and short-term) and treatment comparisons**

Landscape Review Findings

Where is the greatest opportunity?

- Numerous clinical trials are currently being conducted or have been funded, with common issues prevailing
 - Recruitment and enrollment barriers
 - Startup time
 - Small sample sizes
- Funding the development of a **nationwide disease registry** that enables high quality observational studies provides an ideal opportunity for PCORI to make an impact
 - Quick implementation
 - Easier enrollment

Addressing Critical Gaps

What questions will registry data help answer?

- **What is the comparative effectiveness of all the available uterus-sparing procedures in terms of durability of symptom relief, fibroid recurrence, safety, fertility and related outcomes in women of childbearing age with symptomatic fibroids?**
- **Among women who do not desire future pregnancy, how to uterine-sparing procedures compare with hysterectomy in terms of durability of symptom relief and safety?**

Addressing Critical Gaps

What questions will registry data help answer?

- **What are the effect modifications of fibroid size and location, pre-procedural therapies to shrink fibroids, disease severity, prior treatments for symptomatic fibroids, prior reproductive history, family history, and demographics including race and age in the outcomes of interest with respect to hysterectomy and all the available procedures?**
- **What is the comparative effectiveness of available medical therapies (GnRH agonist, aromatase inhibitors, birth control pills and others) in terms of adherence, patient reported outcomes, durability of symptom relief, change in fibroid size, fibroid recurrence, safety, long-term fertility and other related outcomes?**

Addressing Critical Gaps

What would the scope of the project be?

- **Successful applicant will need to have compelling research plan (not just building a registry)**
- **Outcomes include both symptoms and fertility**
- **Potential for long-term follow-up with future funding**
- **Ancillary research questions will be highlighted and built into this project for:**
 - Methods for discerning disease heterogeneity and severity
 - Development of better PROs

Partnership with AHRQ

Why should PCORI work with AHRQ on this project?

- **Authorizing legislation directs PCORI to give preference to AHRQ when entering into contracts**
- **AHRQ has interest in this topic**
 - Future research needs initiative
- **AHRQ has experience and expertise in managing registry studies**
 - Current successful joint replacement registry

Uterine Fibroids PFA

What are we proposing?

Funding Announcement

- 1 award
- Development of registry with national scope
- Integrated observational studies that use registry data
- 5-year project commencing in early 2014
- Early results in 2017

Budget Target

- \$ 20 million in single cycle

Partnership with AHRQ

Board Vote: Uterine Fibroids PFA

Call for Motion to:

- **Approve the development of a PFA on “Treatment Options for Uterine Fibroids”** as recommended by the PDC

Once the Motion Is Seconded:

- Move to **discuss, amend, or take another action** on the Uterine Fibroids PFA

Vote:

- Majority vote to **approve, disapprove, or take another action** on the motion

Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma

Romana Hasnain-Wynia, MS, PhD
Program Director, Addressing Disparities

Asthma PFA – Recap

- **PFA Title:** Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma
- **Budget:** \$17 million
- **Number of Awards:** 4-8
- **Length:** 3-years
- **Brief Description:** CER that tests interventions to improve clinician and patient adherence to guidelines produced by the National Asthma Education and Prevention Program. Studies should also incorporate interventions at the community, home, and health system levels and assess combinations of patient-education tools, home-environment interventions, asthma medications, and team-based approaches.

Timeline & Next Steps

Action	Due Date
Release Date	Tuesday, June 18, 2013
PCORI Online System Opens	Monday, July 1, 2013
Pre-Letter of Intent Informational Webinar	Week of July 8, 2013
Letter of Intent Due	5:00 PM (EST) Thursday, August 1, 2013
Application Deadline	5:00 PM (EST) Wednesday, September 18, 2013
On-line Reviews	Tuesday, October 1 – Wednesday, November 13, 2013
Merit Review	Week of December 2, 2013
Awards Announced	December 2013

Questions & Discussion

Thank You!