Effective Health Care

Pulmonary Arterial Hypertension Nomination Summary Document

Results of Topic Selection Process & Next Steps

- Pulmonary arterial hypertension (PAH) will go forward for refinement as a systematic review. The scope of this topic, including populations, interventions, comparators, and outcomes, will be further developed in the refinement phase.

- When key questions have been drafted, they will be posted on the AHRQ Web site and open for public comment. To sign up for notification when this and other Effective Health Care (EHC) Program topics are posted for public comment, please go to http://effectivehealthcare.ahrq.gov/index.cfm/join-the-email-list1/.

Topic Description

Nominator: Health care professional association

Nomination Summary: The nominator is interested in the effectiveness and comparative effectiveness of medical treatments for pulmonary arterial hypertension.

Staff-Generated PICO
Population(s): Patients with pulmonary arterial hypertension; no exclusions for age, gender, ethnicity, or comorbidities; treatment might be severity dependent and should be defined by functional class
Intervention(s): Newer drugs (inhaled treprostinil and ambrisentan); combination therapies; use of testing (vasoreactivity, biomarkers) for diagnosis, management, and evaluation of PAH treatment
Comparator(s): Older therapies (calcium channel antagonists, prostanoids, endothelin antagonists, phosphodiesterase inhibitors); monotherapy; no use of testing
Outcome(s): Decreased mortality; decreased morbidity (including but not limited to control of pulmonary artery pressure, prevention of right ventricular dysfunction and/or right heart failure, improved dyspnea, improved functional class, improved quality of life); side effects of various drugs (including but not limited to liver function abnormalities, headache, flushing, epistaxis, dyspepsia, diarrhea, peripheral edema, nausea, nasal congestion, dizziness, increased INR or prothrombin time

Key Questions from Nominator:

1. What are the long-term (> 90-day) mortality, morbidity, and functional outcomes associated with various drug therapies for pulmonary arterial hypertension?
   - **a.** Compare two newer drug therapies, both FDA approved, for the treatment of PAH (inhaled treprostinil and ambrisentan) to older treatments
   - **b.** Compare combination drug therapies to monotherapy drug treatment

2. What are the optimal testing strategies and modalities (vasoreactivity, biomarker) for guiding the diagnosis and management of PAH as well as for use in evaluating short- and long-term treatment effectiveness?

Considerations

- The topic meets all EHC Program selection criteria. (For more information, see http://effectivehealthcare.ahrq.gov/index.cfm/submit-a-suggestion-for-research/how-are-research-topics-chosen/.)

- PAH is a progressive and incurable disease with a variety of etiologies and a pathology that is not fully understood. There has been rapid development and approval of vasodilator medications for PAH over the past 3 decades; however, there continues to be a lack of data supporting the long-term effectiveness of these therapies. Based on disease etiology and severity of the disease, the timing and optimal combinations of drug therapy are all important areas of clinical uncertainty for caregivers. In order to optimize PAH care, newer information regarding the latest drug therapies and combination therapies should be systematically reviewed to help inform clinical practice.