Effective Health Care

Recombinant Human Growth Hormone (rhGH) in Patients with Cystic Fibrosis (CF) Nomination Summary Document

Results of Topic Selection Process & Next Steps

- Recombinant human growth hormone in patients with cystic fibrosis will go forward for refinement as a comparative effectiveness or effectiveness 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 Program topics are posted for public comment, please go to http://effectivehealthcare.ahrq.gov/index.cfm/join-the-email-list1/.

Topic Description

Nominator: Public payer

Nomination Summary: The nominator is interested in the comparative effectiveness of adding recombinant human growth hormone (rhGH) to usual care for patients with cystic fibrosis, including the potential benefits (growth outcomes, decreased hospitalizations, etc.) and harms (glucose intolerance, hypoglycemia, etc.).

Key Questions from Nominator:

1. What is the effectiveness of rhGH for CF patients for improving clinical outcomes including: height and weight, clinical course, quality of life?
2. What is the evidence for harms of rhGH in CF patients?

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/.)

- Cystic Fibrosis affects both children and adults, and its clinical course varies from patient to patient. For patients whose pancreas and/ or intestines are affected, their growth can be (possibly greatly) reduced due to continual malnutrition and digestive problems. Additionally, many CF patients suffer from repeated lung infections and most die from lung issues. With both the respiratory and digestive systems injuriously impacted, the life expectancy for people with CF has been much lower than the national average life expectancy.

- The safety and efficacy of rhGH as a treatment for cystic fibrosis is still under investigation. There are a few recent trials that examine the efficacy of rhGH in children with CF. However, no comparative effectiveness reviews comparing usual care to usual care in combination with rhGH were identified. The harms of rhGH will be an important aspect of the review conducted within the EHC Program.