Suggesting a Topic for Effective Health Care Research

The Effective Health Care Program (EHC) researches available health care tests and treatments to determine whether there are significant advantages or disadvantages with different approaches. The results of this comparative effectiveness research can help people make better decisions about what health care they want to have, and can help clinicians and health care purchasers to focus on the best tests and treatments.

SEE A LIST OF EXISTING EHC PRODUCTS

The process of identifying, selecting, and developing important research topics for research reviews or new research is a key aspect of the EHC program. The process is enhanced by strong involvement of stakeholders, maintaining transparency and public accountability, and striving for continuous self-evaluation and improvement. The program uses an established set of criteria to guide the process of topic selection. This form is organized by these selection criteria.

SEE HOW YOUR TOPIC WILL BE EVALUATED

We would like to understand important aspects of the health care service you are interested in, including to whom it applies, what benefits or harms are of greatest interest, and with what other health care services or tests you think it should be compared. Your answers to these questions will help us phrase your suggestion as one or more research questions that could be answered through AHRQ EHC comparative effectiveness or effectiveness products.

LEARN MORE ABOUT EHC RESEARCH PROJECTS

To nominate a topic for research in this program, please fill in the form below as completely as possible and click on “submit” at the end. If you prefer to submit a paper copy, you may print the form and mail it. If you have any supporting documents you would like to include with your nomination, you can include them (if mailing), send them as additional attachments (if e-mailing), or you can upload them while submitting the online form. All topic nominations, including those submitted on paper, will appear in the Read Suggested Topics for Research section of the Web site. Click here to view an example of a completed form.

Thank you for participating in the program!
### TOPIC NOMINATION

1. **Your Nomination** – Briefly describe a specific question, or set of related questions, about a health care test or treatment that this program should consider.

In patients with cystic fibrosis (CF), what is the effectiveness of recombinant human growth hormone (rhGH) in improving intermediate health outcomes, such as pulmonary function and nutritional status?

**Examples:**
- For patients with low bone density, what is the comparative effectiveness of exercise versus drug therapy to treat or prevent osteoporosis?
- For adult patients with a major depressive disorder (MDD), what are the comparative risks and benefits of older versus newer antidepressants?
- In patients with cystic fibrosis, what is the effectiveness of recombinant human growth hormone (rhGH) in improving intermediate health outcomes, such as pulmonary function and nutritional status?

2. **Does your question include a comparison of different health care approaches?**
   (If no, your topic will still be considered.)
   - □ No
   - ☒ Yes  If yes, explain the specific technologies, devices, drugs, or interventions you would like to see compared:
   
   Usual care for patients with CF plus rhGH compared to usual care without rhGH

**Examples:**
- Calcium versus biphosphonates for the prevention of vertebral fractures
- Core needle biopsy versus open surgical biopsy for diagnosing breast lesions
- Antireflux medication versus diet and exercise for the control of acid reflux symptoms

3. **What patients or group(s) of patients does your question apply to?**  *(Please include specific details such as age range, gender, coexisting diagnoses, and indications for therapy.)*

Patients with cystic fibrosis.

Are there subgroups of patients that your question might apply to? *(For example, an ethnic group, stage or severity of a disease.)*
Sub-groups include: age (pre-pubertal, adolescent, adult); baseline clinical status (height, weight, pulmonary function, exercise tolerance, nutritional status); and/or the nature, extent, and effectiveness of prior treatment(s).

4. Describe the health-related **benefits** you are interested in. *(For example, improvements in patient symptoms or problems from treatment or diagnosis.)*

- Improvements in primary outcomes, such as growth, frequency of antibiotic treatments, frequency of hospitalization, quality of life, morbidity, and mortality.
- Improvements in intermediate outcomes, such as pulmonary function, exercise tolerance, nutritional status, and bone mineralization.

5. Describe any health-related **risks, side effects, or harms** that you are concerned about.

- Serious harms from the use of rhGH, including glucose intolerance, diabetes, hypoglycemia, lymphoid overgrowth, intracranial pressure, coarsening of facial features, and long-term increased risk of colonic, lymphatic, or other malignancies.

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**APPROPRIATENESS FOR EHC PROGRAM**

6. Does your question include a health care drug, intervention, device, or technology available (or likely to be available) in the U.S.?

- Yes [x]
- No
- Unsure

7. Which priority area(s) and population(s) does this topic apply to? *(check all that apply)*

**EHC Priority Conditions** *(updated in 2008)*

- Arthritis and nontraumatic joint disorders
- Cancer
- Cardiovascular disease, including stroke and hypertension
- Dementia, including Alzheimer’s

**AHRQ Priority Populations**

- Low income groups
- Minority groups
- Women
- Children [x]
- Elderly
- Individuals with [x]
special health care needs, including individuals with disabilities or who need chronic care or end-of-life health care

Federal Health Care Program

- Medicaid
- Medicare
- State Children’s Health Insurance Program (SCHIP)
- Other

8. Describe why this topic is important.

The severity of CF varies from patient to patient. Both the respiratory and digestive systems can be seriously impacted, and the life expectancy for people with CF has been much lower than the national average life expectancy. However, with improvements in treatments for patients, life expectancy has the potential to increase greatly. It is unclear at this time whether rhGH causes more harm than good for patients with cystic fibrosis. Despite the evidence for rhGH improving height and weight in patients with cystic fibrosis, it is not conclusive that this treatment effectively and safely increases lung function. No cure for cystic fibrosis exists at this time; therefore, it is imperative that safe, supportive treatments that relieve symptoms and complications be established.

9. What specifically motivated you to ask this question? (For example, you are developing a clinical guideline, working with a policy with large uncertainty about the appropriate approach, costly intervention, new research you have read, items in the media you may have seen, a clinical practice dilemma you know of, etc.)

Despite the lack of evidence for its use, we are experiencing an increase in reimbursement needs for rhGH in cystic fibrosis patients of all age groups and disease severity.

10. Does your question represent uncertainty for clinicians and/or policy-makers? (For example, variations in clinical care, controversy in what constitutes appropriate...
Proper dosages and patient selection criteria for rhGH have not been determined. In addition, the long-term effects of this treatment have not been studied.

**POTENTIAL IMPACT**

11. How will an answer to your research question be used or help inform decisions for you or your group?

RhGH in addition to usual care for cystic fibrosis patients represents a costly intervention and a large amount of clinical uncertainty exists with this treatment. At best, this treatment has been shown to improve height and weight, but there have been minimal improvements noted in pulmonary function. Additionally, optimal doses and duration of rhGH have not been established. A review on this topic may shed light on which patients are the best candidates for treatment and clarify the balance of potential benefits and risks of treatment. This clarification is needed to inform policy decisions.

Describe the timeframe in which an answer to your question is needed.

This is an urgent need as the demand for reimbursement for rhGH treatment is increasing.

12. Describe any health disparities, inequities, or impact on vulnerable populations your question applies to.

Cystic fibrosis is the second most common life-shortening, child-onset genetic disease in the US. It is more common among Caucasians. The life expectancy for people with cystic fibrosis has been much lower than the national average life expectancy.

**NOMINATOR INFORMATION**

13. In order to help us to understand the context of your health care question, it would be helpful to know more about you. The answers you give will not influence the progress of your suggestion.

   a. Choose a description that best describes your role or perspective: (you may select more than one)
b. Are you making a suggestion as an individual or on behalf of an organization?
   □ Individual
   X Organization

c. If organization, please state the name of the organization: (optional)

d. Please tell us how you heard about the Effective Health Care program

   I heard about the Program from a colleague.

e. Your name and contact information: (optional)

   Your personal identification will not be displayed in the public reading room, nor will it influence your nomination for Effective Health Care Research. It will only be used to contact you for additional information about your nomination if necessary. It is not mandatory that you provide your contact information, but it is often helpful for us to contact the nominator when we need clarification about a research question.

   Your Name:
   Your Title:
   Your Organization:
   Your e-mail address:
   Confirm your e-mail address:

   May we contact you if we have questions about your nomination?
   □ Yes
   □ No
Thank you for suggesting a topic. If you have answered all of the necessary questions, checked your answers, and are happy with your form, please e-mail the completed form to Effectivehealthcare@ahrq.hhs.gov, or you may print out the completed form and mail it to:

AHRQ Effective Health Care Program  
c/o Scientific Resource Center at Oregon EPC  
Center for Health Research  
3800 N Interstate Avenue  
Portland, OR 97227-1110  
Attn: Michelle Eder

If you have comments about the form or suggestions for improvement, please e-mail the AHRQ Effective Health Care Program at Effectivehealthcare@ahrq.hhs.gov.

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