Assessment of Prevention, Diagnosis, and Treatment Options

Advisory Panel Webinar

May 1, 2015
12:00-2:00pm ET

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PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Welcome and Introductions

Welcome

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Clinical Effectiveness Research
PCORI

Housekeeping

- Today's webinar is open to the public and is being recorded.
  - Members of the public are invited to listen to this webinar.
  - Topic briefs and other materials are available on the PCORI site.
  - Comments may be submitted via chat or email to advisorypanels@pcori.org. No public comment period is scheduled today.

- If you experience any technical difficulties, please alert us via chat or email support@meetingbridge.com.

- For those on the call, please remember to speak loudly and clearly into your phone. Please mute the lines unless you are speaking.

- Where possible, we encourage you to avoid technical language in your discussion of these topics.

Advisory Panel Chair

Margaret F. Clayton, RN, PhD

Co-chair, Panel on the Assessment of Options

Associate Professor, College of Nursing and

Co-Director of the PhD Program, University of Utah

Agenda

Welcome and Introductions

Objectives and Procedures

Discussion of Topics

Closing

Submitting Questions:

Submit questions via the chat function in Meeting Bridge.

Objectives and Procedures

Objectives

- Review 5 new clinical effectiveness research priority topics and prioritize these topics for further consideration as research priority areas

- Possible pathways for funding:
  - Add to Pragmatic Studies funding announcement
  - Add to Single Topic Targeted funding announcement

Procedures for reviewing topics and voting

At today's meeting

- Review 5 research topics (20 minutes per topic)
  - Background (2-3 minute)
  - Panel Discussion (18 minutes)
    - *What is the important clinical question?*
    - *What are the gaps in current research?*
    - *Could research close these gaps?*
    - *How does the topic meet the 5 PCORI criteria?*

Following today's meeting

- Participants in today's meeting will be emailed a link to Survey Gizmo ranking for completion by May 8, 2015
- Results will be shared via email to panelists within 3 days and posted online

Discussion of Topics

Stem Cell Transplantation vs Immunosuppressive Therapy for Severe Aplastic Anemia in Children and Young Adults

- Acquired aplastic anemia in children and young adults is rare. Only 2 of every 1 million children aged 15 and younger are diagnosed each year. About 500 children in the United States are diagnosed annually. Given patients' young age, caregiver burden and lost work productivity are significant. Young patients often have a lifetime of frequent and burdensome complications and treatments such as transfusions. Research findings may be applicable to other bone marrow failure syndromes, such as those that are inherited or in the elderly.

- **Potential Research Areas:**
  - High-quality comparative effectiveness research involving multiple centers to create sufficiently large studies will be important as more unmatched donor transplantations are conducted and interventions and outcomes for unmatched donor transplantation continue to improve.
  - There is a need for research in minorities and mixed races and the impact of the availability/unavailability of donors for these patients within their families or bone marrow donors.
  - Examining patient-reported outcomes in addition to mortality is needed.

Early Therapy vs Observation for Monoclonal Gammopathy in the Prevention of Multiple Myeloma

- Although multiple myeloma (MM) is rare (only 1% of all cancers), monoclonal gammopathy of undetermined significance (MGUS) is common affecting 3-4% of the population over the age of 50. Even if progression from MGUS to MM is rare, at 1% a year, MGUS affects a significant proportion of the population; therefore, there is a need to identify patients at higher risk and evaluate alternative approaches to prevent progression. Treatments for MM are expensive and use large amounts of health care services. Even with treatment (including transplantation), the MM often causes significant quality of life issues due to fractures and other complications.

- **Potential Research Areas:**
  - Patient-reported outcomes have not previously been well-integrated into the evaluation of treatment alternatives, including concerns about progression and side-effects of treatment.
  - If ongoing research identifies biomarkers that identify patients at high risk of progression to myeloma, reevaluation of CER on monitoring or potential treatment might be indicated.

Second-Line Drug Therapies After Failed Metformin Use in Type 2 Diabetes

- Approximately 60% of patients with type 2 diabetes are started on metformin. Forty-five percent of patients who initiate metformin will require intensification of anti-hyperglycemic therapy within a year of first use. Intensification includes increased dose of metformin or the need of two or more drugs to achieve adequate glycemic control. The effects on quality of life, productivity, functional capacity, mortality and use of health care services for individuals who require second-line therapy are not well described. Presumably individuals who require second-line therapy have worse profiles for each of these outcomes than patients who do not require second-line therapy.
- **Potential Research Areas:**
  - Understanding the efficacy, safety, patient preferences and ideal placement of the approved medications in the type 2 diabetes treatment algorithm is needed and makes research on this topic very compelling
  - New research should focus on the safety of second-line treatments so that patients can make informed decisions about which treatment is best for them, given the comparable short-term efficacy of the available treatment combinations. Evidence on long-term effectiveness and safety is still needed.
  - Studying the patient-identified benefits, like decreased fear of walking or ability to participate in the preparation of family meals, in addition to measuring health care provider important benefits like hemoglobin A1c control, may help patients make better treatment decisions based on what is most important to them.
  - Information combining patient preferences with effectiveness and safety to personalize treatments among the many second-line treatment options is needed.

Optimal Timing for Reduced-Intensity Conditioning Allogeneic Hematopoietic Stem Cell Transplantation for Older Patients

- Myelodysplastic syndromes (MDS) are a group of hematologic stem disorders where the bone marrow fails to produce blood cells, resulting in pancytopenia, and characterized by inefficient hematopoiesis and increased apoptosis. MDS is not common; there are about 13,000 persons diagnosed each year in the United States, or 4.8 persons per 100,000 in the population. MDS is more common in men and the risk of developing MDS increases with age; there are very few cases of patients younger than 40 years old, and almost all patients are older than 60 years. MDS, although rare, causes significant and prolonged deficits in quality of life, has a high risk of mortality and is associated with high use of health care services.

- **Potential Research Areas:**
  - Given the paucity of existing high-quality evidence, particularly research including patient-reported outcomes, and the new development of U.S consortiums and a registry, new CER is likely to help guide clinical decision-making and recommendations for the NCCN guideline.
  - Given uncertainty on patients with intermediate risk for progression to leukemia, better evidence for the appropriate timing of transplantation is needed.
  - Research on regimens with a more tolerable side effect profile for maintenance therapy after transplantation is also needed.

Narrow-Spectrum Antibiotics vs Broad-Spectrum Antibiotics for Pneumonia in Adults

- One study estimated that 915,900 episodes of community-acquired pneumonia (CAP) occur in adults greater than or equal to 65 years of age each year in the U.S. Patients diagnosed with CAP have a significant short-term decrease in quality of life due to symptoms, and typically miss at least one week of work or school even when not admitted. If admitted to a hospital, loss of productivity can go up to 2 or 3 weeks. In 2013, CAP was the 9th leading cause of death in the US, causing around 53,000 deaths (the mortality rate is 16.9 per 100,000). Despite recommendations to use broad-spectrum antibiotics for CAP, mortality from CAP has not decreased significantly over years.

- **Potential Research Areas:**
  - In patients with CAP, new research could help to improve patient-centered outcomes by providing information about the comparative effectiveness on 1) narrow versus broad-spectrum antibiotic for empiric therapy and/or definitive therapy; 2)shorter versus longer antibiotic therapy; and 3) approaches to de-escalate antibiotic therapy on patient-centered outcomes.
  - There is a need for further research on establishing CAP diagnosis rapidly in clinical practice with respect to whether CAP is present, whether hospital admission is required, the type of pathogen (i.e., bacteria or virus, colonization or infection), and the causative bacteria, with a focus on patient-centered outcome.

Closing

Next Meeting

- Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options in-person meeting is scheduled for July 9-10, 2015. The meeting will occur in Washington, DC.

Thank you for your participation.

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