Assessment of Prevention, Diagnosis, and Treatment Options

Advisory Panel Webinar

April 28, 2015
1:00-3:00pm ET

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Welcome and Introductions

Welcome

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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.

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Advisory Panel Chair

Alvin I. Mushlin, MD, ScM

Chair, Panel on the Assessment of Options
Chairman, Department of Public Health, Weill Cornell Medical College; Public Health Physician-in-Chief, New York Presbyterian Hospital/Weill Cornell Medical Center

Agenda

Welcome and Introductions

Objectives and Procedures

Discussion of Topics

Closing

Submitting Questions:

Submit questions via the chat function in Meeting Bridge.

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Objectives and Procedures

Objectives

- Review 4 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 4 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

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Discussion of Topics

Long-Term Outcomes for Drug Treatment vs Non-Drug Treatment in Prediabetes

- Despite the absence of symptoms, individuals with prediabetes have poorer quality of life and a shorter life span than the population without impaired glucose. There is a large burden of prediabetes in the U.S. population, with 37% of the adult population having prediabetes. Therefore, high priority should be given to research to determine the best strategies to prevent the progression of prediabetes to diabetes.

- **Potential Research Areas:**
  - Studies that directly compare all of the new medication treatments for diabetes to lifestyle interventions for this population of patients with prediabetes. A network meta-analysis could also be performed to identify the studied treatment options and identify the most reasonable for inclusion in a trial.
  - New research could evaluate drugs other than metformin. Metformin was compared with lifestyle intervention in the Diabetes Prevention in 2002.
  - Despite no difference in the prevalence of prediabetes across ethnicities, there are differences in type 2 diabetes by ethnicity. Examining why these differences in the progression to diabetes exist could be explored in future research.

Long-Term Outcomes for Early Treatment vs Treatment Initiated After Type II Diabetes Diagnosis

- Nearly 50% of the adult U.S. population has prediabetes or diabetes, with the proportion of the population with diabetes expected to increase by 2050. Many people with prediabetes will progress to type 2 diabetes (40-60%). Delaying diagnosis of type2 diabetes can delay the onset of the numerous complications of type 2 diabetes as well as the disease's substantial effects on the health care system. The large burden of disease justifies CER with high priority.

- **Potential Research Areas:**
  - Studies comparing when to start medication treatment after failed lifestyle changes for prediabetes. Definitive research on this topic could drastically change the use of metformin therapy for diabetes prevention.
  - Studies comparing new weight-loss and type 2 diabetes drugs versus or adjuncts to metformin. Understanding the safety of these medications and risk-benefit profile for prediabetes versus type 2 diabetes is needed.
  - Wearable technology may be able to increase compliance with lifestyle modifications or allow researchers to compare the intensity levels of different lifestyle modifications required to have an effect on long-term outcomes. These technologies may be especially useful to increase self-monitoring in individuals with prediabetes who do not want to take medications.
  - Given the broad implications of prediabetes and diabetes and the fact that they are almost purely driven by lifestyle, research to promote population-level lifestyle change (i.e., built environment, policy, behavior) are particularly important.

High-Intensity Statin vs Low-Intensity Statin in Prevention of Cardiovascular Disease (CVD)

- Statins are among the most prescribed drugs in the U.S. and the world. While the effectiveness of statins to reduce the risk of CVD is well-established, statin therapy may be associated with adverse effects such as muscle problems and an increased risk of developing type 2 diabetes mellitus. Low- and moderate-intensity statin therapies are available in generic forms and are associated with fewer adverse events than high-intensity statin therapy. Because many more people became eligible for statins under the 2013 ACC/AHA guidelines, it is especially compelling to compare the benefits and risks of high- versus low-intensity statin therapy for primary prevention

- **Potential Research Areas:**
  - Studying differences in risk/benefit profiles of statin therapy based on the recent modifications to the ACC/AHA risk estimator tool are needed.

  - Anti-PCSK9 is a new agent under investigation for lowering LDL-c.33 Investigating the role of Anti-PCSK9 combined with different intensities of statin therapy is needed.

  - Benefits for "lower intensity is better" for primary prevention are extrapolated from secondary prevention and from the meta-analyses showing incremental reduction in atherosclerotic CVD risk of 11 per 1000 persons over 5 years for every 1 mmol/L reduction in LDL-c. However, there are little data for those in the very low risk group (10-year predicted atherosclerotic CVD risk below 5%).

Antiretroviral drugs (3TC/FTC + boosted PI vs 2NRTI + boosted PI) in the treatment of HIV Infection

- As people are generally infected with HIV at relatively young ages, HIV is now considered a chronic illness in the U.S.. Although many treatment options are available, there are many side effects and long-term consequences of HIV treatment. Non-adherence is a major concern because of the potential to develop resistance to HIV medications. Thus, CER on alternative approaches to improving medication adherence and compare the effectiveness of different regimens on resistance could have a great impact in reducing the societal burden.

- **Potential Research Areas:**
  - Future research could address patient preferences regarding the choice of different first-line therapies, taking into consideration side-effect profiles.
  - Studying patient-important outcomes to improve adherence and decrease resistance is needed.
  - As the population with HIV ages, side effects and the impact of treatment on diseases of aging (e.g., heart disease) is of increasing importance, leading to need for continued development of regimens that are better tolerated with fewer side effects

Closing

Next Meeting

- Next webinar will be held on Friday, May 1, 2015 from 12:00-2:00pm ET. Panelist will discuss and vote on 5 new clinical effectiveness research topics

- 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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