Board of Governors Meeting
via Teleconference/Webinar

June 21, 2016

12:00-1:30 p.m. ET

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PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Welcome and Introductions

Grayson Norquist, MD, MSPH
Chairperson, Board of Governors

Joe Selby, MD, MPH
Executive Director

Agenda

Time	Agenda Item
12:00	Call to Order, Roll Call, and Welcome
12:00- 12:05	**Consider for Approval:** Minutes of the May 23, 2016 Board Meeting
12:05- 12:35	**Consider for Approval:** • PCORnet Initiative on Health Plan Data Partnership Awards (*Pending Research Transformation Committee Approval)* • Limited PCORI 2016 Funding Announcement: Health Systems Interactions Demonstration Research Projects
12:35- 12:55	**Consider for Approval:** Staff recommendation for Hepatitis C Targeted PFA
12:55- 1:25	Report on Research Synthesis
1:25	Wrap up and Adjournment

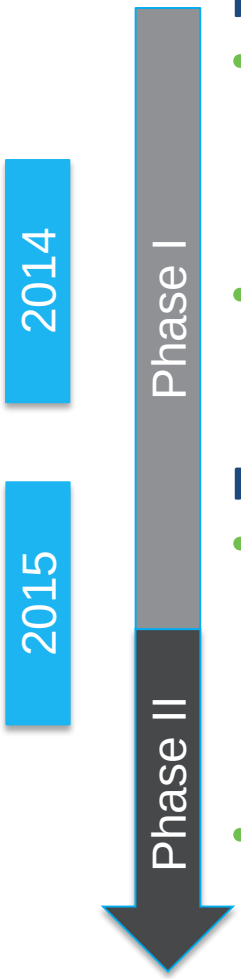
Board Vote

Call for a Motion to:	• **Approve** funding for the May 23, 2016 Meeting Minutes
Call for the Motion to Be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Voice Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

Consider for Approval: PCORnet Initiative on Health Plan/System Data Partnerships (A Stepwise Approach to Collaboration)

Joe Selby, MD, MPH
Executive Director

PCORnet Overview of Complete Data Efforts



Phase I:

- CDRNs
  - Standardized EMR data into the PCORnet CDM
  - Discussed linkage of EMR data and claims data with highly represented Health Plans, Medicare and Medicaid
- PCORI
  - CMS Pilot Project to streamline linkage of CDRN EMR data and Medicare data

Phase II:

- CDRNs
  - Optimizing EMR data for use in research
  - Establishing relationships/agreements with major claims data providers in their region (e.g. REACHnet and BCBS, NYC and Medicaid, ADVANCE and CareOregon)
- PCORI
  - **Engaging Health Plans to supplement the local efforts of the CDRNs from Phase I**
  - Identify additional hurdles to efficient linkage with Medicaid data
  - Continuing CMS Pilot Project work from the end of Phase I

PCORnet Health Plans Initiative: Project Overview

- Critical need for complete longitudinal patient data on entire populations
- During Phase I we recognized the challenges and insufficiencies in having ONLY the CDRNs work to capture health plan data (claims, membership data) independently
- In Phase II, we will connect ALL the data DOTS for a more efficient and feasible centralized approach where PCORI engages directly with major US health plans that cover significant numbers of patients in one or more of CDRNs
- Intent is for up to two awards to align closely with CDRNs around PCORnet demonstration projects, with 3-year duration
  - Year 1: Establish governance and linkages to support the three PCORnet Demonstration Studies: ADAPTABLE and the two Obesity Observational Studies
  - Years 2 & 3: Establish governance and linkages with PCORnet CDRNs and PPRNs to support development of multi-purpose linked datasets for use in future research

Application Evaluation Criteria

1. **Population Overlap**: Demonstrate the potential that the health plan population(s) overlaps with populations cared for by PCORnet CDRNs and PPRNs for each of the three demonstration studies, potential future studies, and for a multipurpose data set

2. **Approach to Governance and Oversight**: Describe an approach for effective linkages to support current and future PCORnet demonstration studies and provide credible evidence of the ability to accomplish effective data linkages in a timely manner

3. **Data Linkages**: Show the ability to accomplish the data linkage activities within the specified time frame that would augment current CDRN and PPRN data

4. **Engaging in and Sustaining a Collaborative Research Network**: Demonstrate an understanding of active collaboration and partnership with health systems and plans, patients and clinicians

Application Review: Process Overview

- PCORI received 3 applications from major health plans with coverage overlap with one or more CDRNs
- All were discussed during the application review
- Interviews were held with 2 of the health plans to further evaluate the application evaluation criteria and allow the applicants to respond to all concerns identified during Merit Review
- We are proposing to fund the 2 highest scoring applications

Slate Summary

*2 Recommended Projects**

Health Plan	Total Budget
1	$5.20M
2	$3.70M

**All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract.*

Application Highlights

Application Criterion	Highlights
Population Overlap	• Solid preliminary data, demonstrating the overlap with PCORnet CDRNs and project feasibility
Approach to Governance and Oversight	• Established and ongoing collaboration with the CDRN organization leaders • Project teams are extremely active in Sentinel initiative • Strong relationship between the Health Plan and project team
Data Linkages	• Demonstrated a history of diverse linkage capabilities; including extensive experience with Sentinel data and linkage of this data
Engaging in and Sustaining a Collaborative Research Network	• Commitment to collaborations with a variety of stakeholders engaged in PCORnet (*patients, providers, health systems)* • Commitment to share lessons learned via the PCORnet Commons

Slate Overview:
Health Plan Infrastructure Awards

2
New Projects

PFA	Direct Costs Allotted	Proposed Direct Budget
PCORnet Initiative on Health Plan/System Data Partnerships (A Stepwise Approach to Collaboration)	$8,000,000	$7,659,294

Total proposed budget = $8,836,132 (direct + indirect costs)

**All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract.*

Board Vote

Call for a Motion to:	• **Approve** funding for the Health Plan Infrastructure Awards
Call for the Motion to Be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Roll Call Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

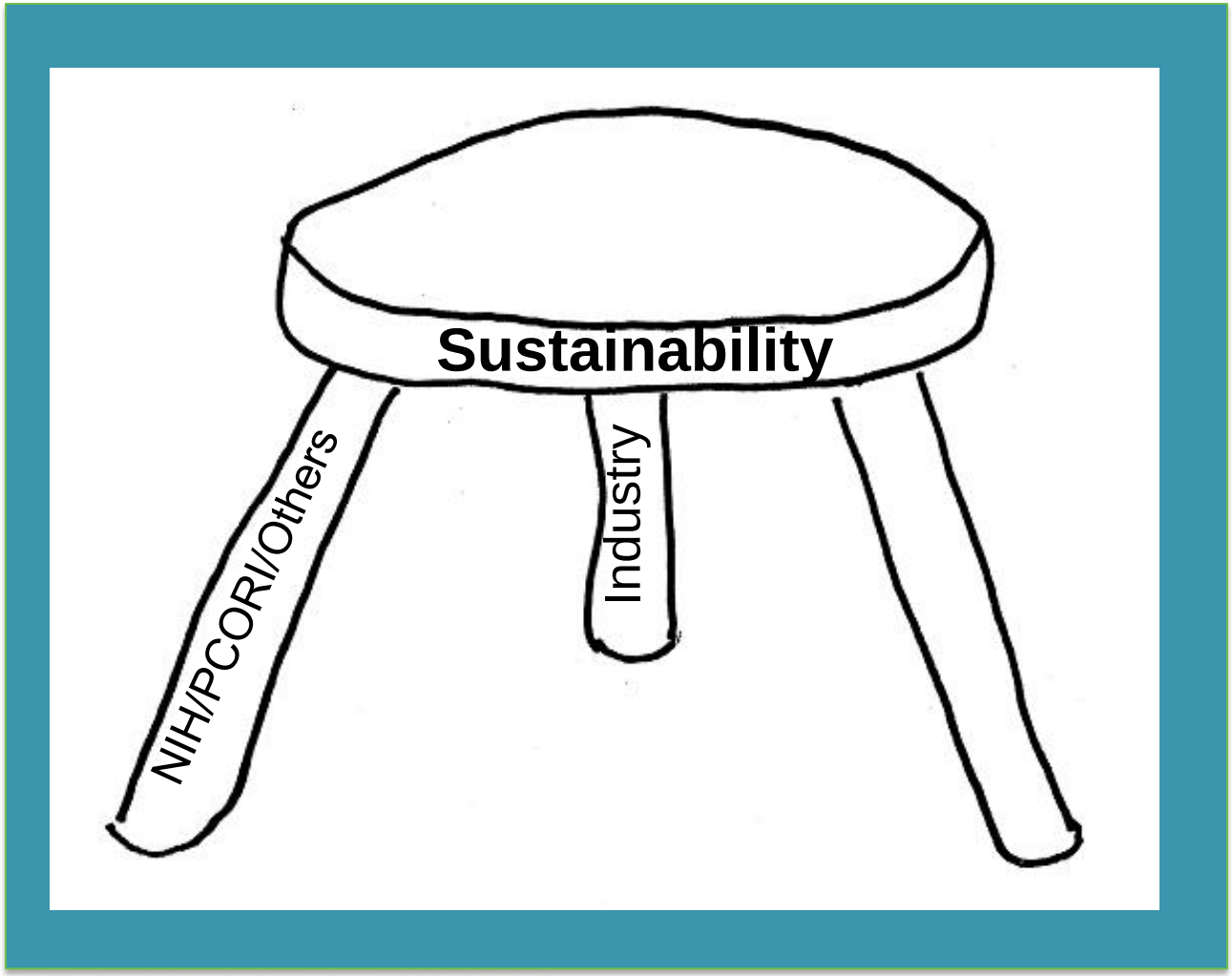
Consider for Approval: Health Systems Interactions Demonstration Research Projects

Joe Selby, MD, MPH
Executive Director

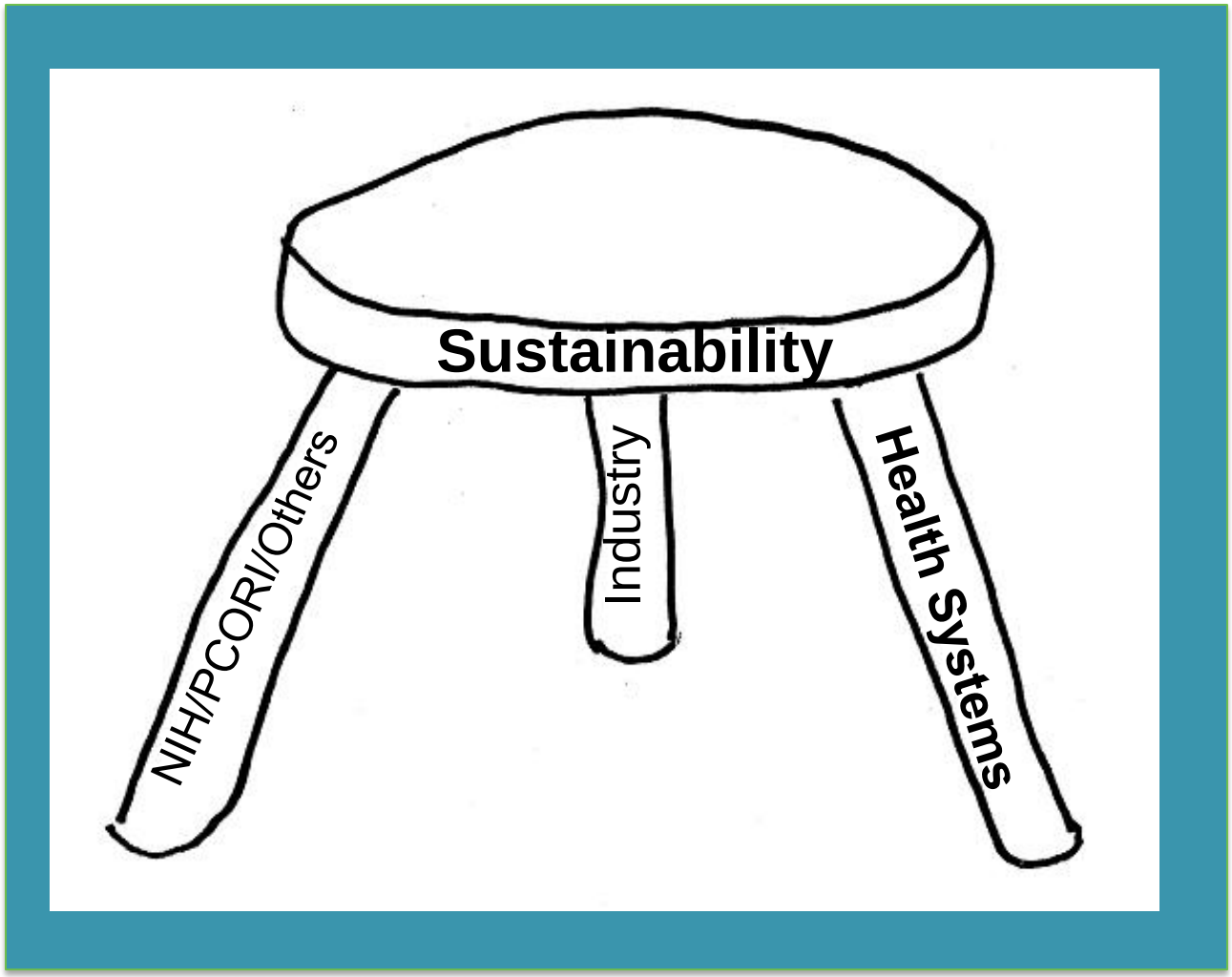
Purpose

- Opportunity for Clinical Data Research Networks (CDRNs) to test their capacity to conduct research with health system leaders and broaden the scope of research that adds value to multiple health systems
- Collaborative meetings were held to identify and prioritize research activities of high interest to health systems and clinicians
- Priority topics for these projects were determined at a cross-network PCORnet/National Academy of Medicine Meeting in January 2016
- PCORI sought to fund up to five, one-year research studies

PCORnet Sustainability Plan – 3 Primary Sources of Support



PCORnet Sustainability Plan – 3 Primary Sources of Support



Project Goals

- Leverage **PCORnet data resources**
- **Involve iterative review and discussion** between PCORnet researchers, clinicians, and health systems leaders
- Work may be **comparative, descriptive, or evaluate** utility of new data sources for addressing questions of health system leaders
- Focus is on needs of learning healthcare systems to support **improved decision-making** and learning by health systems, care providers, patients and families
- **Evaluate PCORnet's capacity** to support collaborative research
- Broaden the availability of **PCORnet's shared tools and resources** (the PCORnet Commons)

Application Review Criteria

- Potential for study to fill gaps in current evidence at the system level and generate actionable evidence
- Potential for the study findings to be rapidly adopted into health care delivery
- Scientific merit (research design, analysis, and outcomes)
- Patient-centeredness
- Patient and stakeholder engagement

Application Review: Process Overview

- PCORI received 7 applications
- All 7 were discussed during the in-person merit review
- We are proposing to fund the 4 top-scoring applications out of the 7
- This slate was considered and recommended for funding by the Selection Committee on June 15, 2016

Slate Summary

*4 Recommended Projects**

Project Title	Participating CDRNs and Health Systems	Total Budget
Identifying and Predicting Patients with Preventable High Utilization	**3 CDRNs, 20 health systems**	$1.20M
Automating Quality and Safety Benchmarking for Children: Meeting the Needs of Health Systems and Patients	**2 CDRNs, 17 health systems**	$0.70M
The Impact of Patient Complexity on Healthcare Performance	**2 CDRNs, 105 health systems**	$1.00M
Variation in Case Management Programs and their Effectiveness in Managing High-Risk Patients for Medicare ACOs	**2 CDRNs, 22 health systems, 3 accountable care organizations (ACOs)**	$1.40M

**All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract.*

Identifying and Predicting Patients with Preventable High Utilization

- **Project Focus**:
  - Naming and prioritizing groups of preventable high utilizers (a priori) with patient and health system stakeholders
  - Identifying and categorizing patients into these groups with the use of linked data sets and computable phenotypes
  - Developing and comparing the effectiveness of different methodological approaches and data sources to predict a patient's annual risk of falling into one of these groups
  - Disseminating results to healthcare delivery system stakeholders throughout PCORnet via PCORnet Commons
- **Sources of Data:** Administrative claims, EMR
- **Leveraging the PCORnet Common Data Model (CDM):** Uses the full range of CDM data tables and will develop new computable phenotypes for high utilizers
- **Measure of Success/Potential Impact**: Early detection of high utilizers to facilitate more targeted and coordinated interventions
- 3 CDRNs and 20 health systems involved
- **Total Project Cost: $** 1,198,793

Automating Quality and Safety Benchmarking for Children: Meeting the Needs of Health Systems and Patients

- **Project Focus**:
  - Testing the validity of three measures of safety and quality across several health systems using the PCORnet CDM (gold standard chart review)
  - Evaluating the value of these measures to healthcare delivery systems leaders in three high priority measure areas:
    - transcranial Doppler (TCD) screening for children with sickle cell disease
    - appropriate antibiotics for ear infections
    - cholesterol and glucose testing for children on antipsychotic medications
- **Sources of Data:** EMR and patient electronic charts
- **Leveraging the PCORnet CDM:** These three measures test and extend the ability of CDM to detect unsafe care using data available via the CDM (e.g., diagnosis, medications, radiographic and laboratory data)
- **Measure of Success/Potential Impact**: Allows the healthcare delivery system leaders to compare performance on the three measures while also testing the accuracy of data elements and quality measures in the PCORnet CDM
- 2 CDRNs and 17 health systems involved
- **Total Project Cost: $** 670,898

The Impact of Patient Complexity on Healthcare Performance

- **Project Focus**:
  - Engaging stakeholders (patients, clinicians, delivery systems leaders) to define "complexity" in terms of comorbid diagnosis and available social determinants of health (SDH) data and to study the relationship of complexity to quality of care measures
  - Assessing whether clinic-level summaries of patients' comorbidity and SDH data correlate with the variability in quality of care measures
  - Identifying implications of data/findings and potential dissemination strategies
- **Sources of Data:** EMR, Medicare, SDH (poverty level, insurance status, race/ethnicity, homelessness, migrant worker status, language), geocoded, comorbidity risk data
- **Leveraging the PCORnet CDM:** The 2 CDRNs will utilize patient and community level social determinant data from their enhanced CDMs for linkage to clinical and claims data to provide a comprehensive assessment of complexity
- **Measure of Success/Potential Impact**: Develop a method to combine patients' clinical and social complexities in accounting for health systems' quality scores that will benefit all health systems in PCORnet
- 2 CDRNs and 105 health systems involved
- **Total Project Cost:** $ 985,686.00

Variation in Case Management Programs and their Effectiveness in Managing High-Risk Patients for Medicare ACOs

- **Project Focus**: Characterizing and examining the effectiveness of case management compared to usual care (no programs) in preventing hospital events for patients at 3 Medicare ACOs
- **Sources of Data:** EMR, Medicare
- **Leveraging the PCORnet CDM:** Linkage of Medicare claims data to CDM data at individual ACOs. Test capacity for sharing of de-identified CDM data for combined analysis
- **Comparator(s)**: case management programs, no case management programs
- **Outcome(s) of Interest**: Composite outcome of hospital events (inpatient admissions, observation stays, and emergency department visits) examining the effectiveness of case management programs compared to usual care
- **Study Design**: observational, retrospective cohort
  - **Sample Size:** 5,250 ACO patients
- 2 CDRNs, 22 health systems, and 3 ACOs involved
- **Total Project Cost:** $1,364,818

Contributions to Infrastructure Development

Project	Leveraging the PCORnet Common Data Model
Identifying and Predicting Patients with Preventable High Utilization	• Develop new specifications for claims and social determinant data
Automating Quality and Safety Benchmarking for Children	• Implement the three measures that test ability of CDM to detect unsafe care using available data (e.g., diagnosis, medications, radiographic and laboratory data) and evaluate reliability of these measures against manual chart review.
Impact of Patient Complexity on Healthcare Performance	• Utilize patient and community level social determinant data from their enhanced CDMs for linkage to clinical and claims data to provide a comprehensive assessment of complexity.
Variation in Case Management Programs and Their Effectiveness in Managing High-risk Patients	• Linkage of Medicare claims data to CDM data at individual ACOs. • Test capacity for sharing of de-identified CDM data for combined analysis.

Slate Overview:
Health Systems PCORnet Research Demonstration Projects

4
New Projects

PFA	Total Costs Allotted	Proposed Total Budget*
Health Systems PCORnet Research Demonstration Projects	$4,000,000	$4,220,195

*Total budget = direct + indirect costs

**All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract.*

Board Vote

Call for a Motion to:	• **Approve** funding for the Health Systems PCORnet Research Demonstration Projects
Call for the Motion to Be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Roll Call Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

Staff Recommendations on a Possible Funding Announcement for Studies of Immediate vs. Delayed Treatment for Hepatitis C

Based on Summary of Recent PCORI Stakeholder Workshop

Joe Selby, MD, MPH
Executive Director

Chronology of PCORI Activity Related to Hepatitis C

- October 2014: Multi-stakeholder Advisory Board identifies four key research questions related to screening, diagnosis, treatment of Hepatitis C (HCV) in the era of direct-acting anti-virals (DAAs)
- December 2014: PCORI Board of Governors approves a Hepatitis C Targeted Funding Announcement (PFA) covering the 4 questions, up to $50M in total
- February 2015: Hepatitis C PFA released
- September 2015: Board awards 2 projects for total of $29 million
- March 2016: The Board endorses the re-release of the Hepatitis C PFA to address 2 revised research questions, including a proposal for a revised randomized trial
- April 2016: Communications from several patient organizations raise concerns about the newly proposed randomized trial
- May 2016: Re-convening multi-stakeholder panel originally convened in 2014

Staff's Recommendations

Based on our estimation of the low practical value of the proposed study for guiding practice, on substantial concerns about the feasibility of such a study, on challenges related to interpreting the results, and on ethical concerns voiced by many, the staff recommends that PCORI NOT proceed with posting a funding announcement on this topic.

The Original Four Questions
(Approved by PCORI Board of Governors, December 2014)

1. **Screening and Diagnosis**

   Which screening methods and testing strategies in which settings lead to the best detection rates?

√ 2. **Care Delivery**

   Alternative ways to deliver care to high risk populations

√ 3. **Head-to-Head Treatment Comparisons of New Therapies**

   Are there differences in terms of long-term virologic response, adverse effects between different new regimens of oral antivirals for treatment of hepatitis C infection in various patient populations?

4. **Studies of the Effect of Delayed Treatment in Newly Diagnosed Hepatitis C**

   What are the comparative benefits and harms of starting treatment immediately after a diagnosis of HCV infection versus active surveillance (starting treatment only when the patient shows progression of liver disease or other manifestations of Hepatitis C infection)?

√ *Study funded, September 2015*

New Research Question #4 (proposed in March 2016)

Focus on Patient-Reported Outcomes in Delayed vs Immediate Treatment of Hepatitis C

- "Among patients with early stage hepatitis C infection (defined as fibrosis stage 0-2) who do not have immediate access to direct-acting antiviral (DAA) treatment, what are the short-term benefits and harms of DAA treatment on patient-reported outcomes such as quality of life, fatigue, depression, malaise, etc. at treatment end and at 1 year?"
- **Key Features**:
  - Double-blind, placebo-controlled randomized trial of immediate vs. delayed treatment
  - Study populations are those whose coverage currently restricts access to DAA treatment in low-risk patients with early stage HCV liver disease
  - 1 year follow-up looking at patient-reported outcomes including quality of life, functional status, symptoms of fatigue, mental cloudiness, depression
  - **Treatment for all control arm patients immediately at end of 1 year**

PCORI Webinar – "Can CER Help Answer Questions about Hepatitis C?" – May 18th, 2016

- PCORI reconvenes stakeholders from its first meeting of October 2014 to solicit input on the proposed new question

- Comments solicited:
  - Likely usefulness and relevance of this study when completed – to patients, clinicians, policymakers
  - The scientific merit of the study
  - Ethical aspects of the study
  - Other Comments

Participants and Order of Commenters – May 18th Webinar

1) Patients and patient representatives
2) PCORI Board members
3) Clinicians
4) Employers (invited)
5) Hospitals
6) Industry (manufacturers)
7) Payers
8) Patients and patient representatives

Summary of Comments from Webinar

- Some patients initially voiced acceptance of the study, but many of these favored an even shorter study so that patients could get access to treatment earlier
- Other participants disagreed, feeling that a “too-short” study could risk missing important differences or identifying only very short-term benefits that were gone by 1 year
- Several ethical concerns raised:
  - Funding a trial means “implicit acceptance” of delaying treatment for an agent that is curative
  - Either a trial or an observational study was seen by some as “taking advantage of patients without access.”
  - “This would not be allowed today by IRBs in many European countries because nearly 100% have access to treatment”

Summary of Comments from Webinar (cont'd)

- Skepticism that a trial showing benefits of immediate treatment on short-term outcomes would change payers' minds
- Knowledge that you no longer have HCV is itself beneficial, relieves stress, and this would be entirely missed in a placebo-controlled study
- A profound sense among patients and clinicians that treatment is associated with a number of obvious clinical benefits – reduction of fatigue, depression, mental cloudiness, pain
- Payer representatives at the meeting were not highly vocal, but generally were modestly supportive of the study

Summary of Comments from Webinar (cont'd)

- It was pointed out in some states, when insurance, even Medicaid, denies coverage, patients are appealing and most appeals are settled in patients' favor. Some said it would be important that the study not interfere with this right to appeal
- Others pointed out that coverage is expanding gradually, making recruitment and retention in the study more challenging
- By end of the two-hour conversation, the patients' level of opposition to the trial seemed to have risen

Staff Summary

- **Utility/Importance**
  - This question will be of decreasing practical importance as we move toward treatment for all; prices are decreasing because of competition and additional new agents; appeals on denial of treatment are being supported; lawsuits are appearing against delayed treatment
  - Conclusions of this study would not change practice: if study shows no differences in short-term outcomes, it would still be considered important to treat by most; if it shows differences, it would not by itself change payers' minds
- **Scientific Validity**
  - Strong disagreement about optimal study length: 1 yr vs. less
  - Inability to study impact of knowledge of cure on symptoms

Staff Summary Cont'd

- **Ethical Considerations**
  - Most who spoke at webinar had some ethical concerns
  - The concern about "implied acceptance" of withholding treatment in soliciting such a study is acknowledged
  - The concept that such a study would be "taking advantage" of a disadvantaged population is less clear but was mentioned frequently

- **Challenges to Feasibility**
  - Recruitment – in face of changing coverage
  - Identifying a source to pay for the study drug

Staff's Recommendations

Based on our estimation of the low practical value of the proposed study for guiding practice, substantial concerns about the feasibility of such a study, challenges related to interpreting the results, and ethical concerns voiced by many, the staff recommends that PCORI NOT proceed with posting a funding announcement on this topic.

Board Vote

Call for a Motion to:	• **Approve** staff recommendation to NOT proceed with posting a funding announcement for a study on the short-term outcomes of delayed vs. immediate treatment of Hepatitis C
Call for the Motion to Be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Roll Call Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

Wrap Up and Adjournment

Grayson Norquist, MD, MSPH
Chairperson, Board of Directors