

## Charter of the Advisory Panel on Rare Disease

November 2013

Research on rare diseases is challenged by small patient populations and other unique needs and considerations. According to the National Institutes of Health and for the purposes of this panel, the term 'rare disease' means any disease or condition that affects less than 200,000 persons in the United States.

### **Purpose**

The Advisory Panel on Rare Disease ("RD Panel") will advise and provide recommendations to PCORI's Board of Governors, Methodology Committee, and staff on the conduct of patient-centered comparative clinical effectiveness research in rare diseases and on coordination and engagement with the rare disease research community, including other funders of research on rare diseases and existing infrastructure dedicated to research on rare diseases. The RD Panel will not serve in an official decision-making capacity, but its recommendations and advice will be taken into consideration by the Institute's Board of Governors, Methodology Committee, and staff.

The RD Panel will:

- Provide input to PCORI on research needs of the rare diseases community and on specific issues and concerns in conducting research on rare diseases;
- Identify infrastructure (data sources, tools, etc) that currently exist and can be a resource for conducting research;
- Serve on or assist in identifying experts to serve on *ad hoc* panels to assist in evaluating, designing and conducting PCORI-funded research specific to a rare disease; and
- Provide ongoing feedback and advice on evaluating and disseminating PCORI's research portfolio on rare diseases.
- Consider study findings and advise on targets and strategies for PCORI dissemination efforts;
- Identify opportunities for collaboration with existing international, federal, public and private entities doing similar work in the rare disease space; and
- Advise other PCORI committees and panels to ensure the unique considerations of rare disease are addressed.

A PCORI Scientific Program Director will be designated by PCORI to support and oversee RD Panel activities.

### **Authority**

PCORI's Advisory Panels are governed by the provisions of Public Law 111-148, which sets forth standards for the formation and use of Advisory Panels.

PCORI's authorizing legislation allows the Institute to appoint permanent or *ad hoc* expert Advisory Panels as determined appropriate to assist in identifying research priorities and establishing the research project agenda. PCORI's authorizing legislation also provides that in the case of a research study for rare disease, PCORI will appoint an expert Advisory Panel for purposes of the study.

### **Composition and Structure of RD Panel**

PCORI aims to involve patients, caregivers, clinicians, other stakeholders, and the organizations representing these stakeholders in a partnership of shared accountability for PCORI's research priorities and research agenda.

Membership on the RD Panel will allow for meaningful interactions amongst individuals with different strengths, backgrounds, and areas of expertise.

The RD Panel will consist of 12 to 15 members appointed by PCORI Board of Governors. No fewer than 33 percent of RD Panel members will be selected from persons who are rare disease patients, caregivers, or representatives of rare disease advocacy organizations. Consistent with the legislative mandate, the remainder will include representation by practicing and research clinicians, experts in scientific and health services research, health services delivery, and evidence-based medicine, insurers, the life sciences industry, and, as appropriate experts in integrative health and primary prevention strategies. Members may also include other stakeholders, such as representatives of employers, and policy makers.

PCORI will ensure the AP includes individuals who, by virtue of their special expertise are qualified to provide advice on rare disease issues, including:

- The severity of rare diseases;
- The unmet medical need associated with rare diseases;
- The willingness and ability of individuals with a rare disease to participate in clinical trials;
- An assessment of the benefits and risks of therapies to treat rare diseases;
- The general design of clinical trials and observational studies for rare disease populations and subpopulations; and
- The demographics and the clinical description of patient populations.

The RD Panel may also invite experts to meetings of the panel to address specific issues being considered by the panel, where there is not sufficient expertise on the panel.

A chairperson (and a co-chairperson if desired) will be appointed by the Institute's Board of Governors to facilitate the RD Panel's activities in conjunction with the Institute's designated Scientific Program Director. The chairperson may assemble subcommittees composed of the RD Panel's members to examine special issues and to facilitate activities related to the scope of work described in this charter.

The terms of members will be staggered so not all members rotate off the panel simultaneously. Initially, half the members will be appointed for a one-year term and half appointed to a two-year term. Thereafter, terms for all members will be for two years. Members may not serve for more than two terms. Panel participation will not be extended beyond the life of the RD Panel's complete work and charter. Any member may resign at any time by giving written notice to the chairperson of the RD Panel. Vacancies will be filled at the discretion of PCORI's Executive Director.

Management and support services will be provided by PCORI staff.

#### **Ad-Hoc Expert Advisory Panels**

In the case of a research study for each rare disease, the RD Panel shall assist PCORI in identifying experts to serve on a condition-specific *ad hoc* advisory panel to assist in evaluating, designing and conducting PCORI-funded research and determining the relative value and feasibility of conducting the research study. The Chair of the RD Panel will appoint members for any *ad hoc* rare disease advisory panels, which may be drawn from members of the RD Panel as well as other individuals with appropriate expertise in the rare disease to be studied. *Ad hoc* rare disease advisory panels will work collaboratively with the RD Panel as requested. Management and support services will be provided by the PCORI program officer responsible for management of the research study.

#### **Panelist Applications and Selection**

PCORI will initiate an open call for applications via the PCORI Web site and other modes of communication when it is seeking members for an Advisory Panel. Prospective panelists are invited to submit an application online to be considered for a position on the RD Panel.

PCORI strives for inclusiveness and diversity in age, ability, gender, ethnicity, race, sexual orientation and gender identity, education, socioeconomic status, and geography in the selection of panelists.

The application review and panelist selection process for the RD Panel will be based on experience, background, ability to contribute to the scope of work described in this charter, and a prospective panelist's commitment to advancing the mission and goals of the Institute.

The Institute's Board of Governors will have final approval of the RD Panel's membership roster.

### **Meetings**

Meetings shall be conducted in an open forum and records of the proceedings kept in accordance with PCORI's policies and procedures. All meetings will have an agenda, which will be issued to panelists and made available to the general public at least three business days prior to the meeting.

Meetings of the full panel will be called by the chairperson with the agreement and consent of the designated Scientific Program Director, who shall develop and approve the agenda and be present at all meetings. Notice of all meetings shall be given to the public at least three business days before the meeting is set to occur.

A majority of the members of an advisory panel shall constitute a quorum, and a roll call must be taken at the beginning of each meeting. In accordance with the RD Panel's advisory role, all votes and recommendations are nonbinding to the Institute.

### **Compensation, Travel, and Expenses**

Members who are not full-time Federal employees are eligible for compensation, consistent with PCORI policies and procedures. The amount of compensation shall be set by PCORI's Executive Director, based on the nature and amount of services to be provided, consistent with PCORI policies.

Travel and other expenses incurred during the conduct of PCORI business will be paid for by the Institute only if the expenses are reasonable and they comply with PCORI's policies and procedures.

All payments will be made to individual panel members and not to employers, organizations, or third parties. Individuals serving on an advisory panel may decline compensation or reimbursement of expenses at their discretion.

### **Conflict of Interest**

All RD Panel members shall abide by the Institute's Conflict of Interest Policy. Members will be asked to disclose any potential conflicts upon joining the RD Panel. The Institute's Executive Director or designee shall be responsible for identifying conflicts and determining what actions would be necessary to ensure that a panelist does not participate in matters in which such a conflict would or could exist.

In general, appointment to the RD Panel will not lead to ineligibility for funding because: all meetings will be public; members will not have access to confidential, nonpublic information; and panelists will provide input, but will not be responsible for final decisions.

### **Termination Date**

This charter will remain in effect for two years beginning on the day of the first RD Panel meeting. It is subject to review, reauthorization, amendment, or termination by the Board of Governors or its designee.

### History:

*Approved by the PCORI Board of Governors 11/18/2013*