



Charter of the Advisory Panel on Rare Disease

Purpose

The Advisory Panel on Rare Disease (RDAP) 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. It will provide recommendations on how to improve the quality of rare disease applications received by PCORI, on appropriate methods for designing studies and analyzing data from comparative clinical effectiveness research in rare diseases, and on approaches for recruiting and engaging patients. It will also provide recommendations 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 RDAP 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.

PCORI's Chief Science Officer or his or her designee will support and oversee RDAP 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 by PCORI.

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. Based on explicit directives in the law, PCORI has appointed a permanent expert Advisory Panel for Clinical Trials and this Advisory Panel on Rare Disease. Furthermore, PCORI appoints other permanent and ad hoc expert Advisory Panels when there is a demonstrated need.

Function and Scope of Work

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.

The RDAP 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.

Composition and Structure

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 RDAP will allow for meaningful interactions amongst individuals with different strengths, backgrounds, and areas of expertise.

The RDAP will consist of 10 to 15 members appointed by PCORI Board of Governors. No fewer than 33 percent of RDAP 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 RDAP 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 RDAP 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 Chair (and a Co-chair, if desired) will be appointed by the Institute's Board of Governors to facilitate the RDAP's activities. The Chair may assemble ad hoc rare disease advisory panels composed of the RDAP's members to examine special issues and to facilitate activities related to the scope of work described in this charter.

Members will serve three-year terms. Terms shall be staggered with a goal of having a balanced number of members appointed each year and a diverse representation of member expertise. To implement staggered terms, initially some members will be appointed for a one-year term, some for a two-year term, and some for a three-year term. Members will not serve more than one full three-year term. Panel participation will not be extended beyond the life of the RDAP's complete work and charter. Any member may resign at any time by giving written notice to the Chair of the RDAP. Vacancies will be filled at the discretion of PCORI's Executive Director. Any panel member who is appointed to fill a vacant position shall serve for the remainder of the term of the vacated position, if any, to retain the staggered-term structure for the RDAP.

Management and support services will be provided by PCORI staff.

RDAP Ad-Hoc Expert Advisory Panels

In the case of a research study for each rare disease, the RDAP 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 RDAP will appoint members for any ad hoc rare disease advisory panels, which may be drawn from members of the RDAP 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 RDAP as requested. Ad hoc advisory panels will have a defined scope of work, and members will be appointed for the duration of the scope of work. 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 RDAP.

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 RDAP 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 RDAP's membership roster.

Meetings

Meetings of the RDAP 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. However, if the RDAP is addressing confidential information, the Chair of the panel may convene a meeting that is closed and members may be asked to sign a nondisclosure agreement.

RDAP ad hoc advisory panel meetings will typically be closed to the general public as they will mostly address confidential information.

Meetings of the full panel will be called by the Chair with the agreement and consent of the Chief Science Officer (CSO) or the CSO's designee, 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 RDAP'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 RDAP members shall abide by the Institute's Conflict of Interest Policy. Members will be asked to disclose any potential conflicts upon joining the RDAP. Each panelist shall work with the Institute's Executive Director or designee to identify conflicts to consider appropriate actions so that a panelist does not participate in matters in which a conflict exists.

In general, appointment to the RDAP will not lead to ineligibility for funding because: 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 until terminated by the Board of Governors. It is subject to review, amendment, and termination by the Board of Governors. This charter will be reviewed on an annual basis.

History:

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

Amended and approved by the PCORI Board of Governors 4/21/2015

Amended and approved by the PCORI Board of Governors 8/16/2016