

December 4, 2012

Joe V. Selby, M.D., M.P.H. Executive Director, Patient-Centered Outcomes Research Institute (PCORI) 1828 L Street, NW, Suite 900 Washington, DC 20036

Dear Dr. Selby:

Thank you for extending us the opportunity to participate in today's workshop "What Should PCORI Study? A Call for Topics from Patients and Stakeholders." NORD looks forward to working with you to reach out to rare disease patient advocacy organizations who may still be interested in working with the Institute but who otherwise may have been unable to participate today.

On the subject of PCORI's engagement with the rare disease community more broadly, it is our view that the expert advisory panels for rare diseases should serve on an ad hoc basis to provide PCORI with expertise specific to a rare disease and that the panels ought to facilitate engagement and communication with the patient community, to include, but not limited to, patients, caregivers, providers, patient organizations, researchers, foundations, as well as experts from other Federal partners including the NIH and FDA.

Given the lack of clarity as to PCORI's interpretation of the statutory language contained in the ACA with regard to expert advisory panels for rare disease, NORD remains concerned that PCORI may be interpreting these provisions to suggest that one advisory panel is sufficient to aid the Institute in the design of all research studies in rare diseases and additionally to determine the relative value and feasibility of conducting such studies.

It is our view that the intent of Congress in this regard was to provide PCORI with the flexibility needed to assure that those making decisions about patient-centered outcomes research in a specific rare disease were also experts in the needs of patients with that rare disease. The NIH's Rare Disease Clinical Research Network (RDCRN) is an excellent model in this regard as patients and patient advocates are involved in the RDCRN at every level.

Sincerely,

Peter L. Saltonstall
President & CEO