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June 4, 2019

The Honorable Chuck Grassley, Chairman
Senate Finance Committee
219 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Ron Wyden, Ranking Member
Senate Finance Committee
219 Dirksen Senate Office Building
Washington, DC 20510

Dear Chairman Grassley and Ranking Member Wyden,

On behalf of the celiac disease patient and caregiver community, the Celiac Disease Foundation (CDF) is writing to express our strong support for the reauthorization of the Patient-Centered Outcomes Research Institute (PCORI) and to request that, as part of the reauthorization, Congress consider the recommendations outlined below that will enable PCORI to continue to fulfill its critically important patient centric mandate.

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For nearly 30 years, CDF has been the nation's leading celiac disease patient advocacy organization, accelerating diagnosis, treatments and a cure for celiac disease through research, education, and advocacy. Celiac disease is a serious autoimmune disorder that afflicts 3 million Americans, though only 20-30% of those living with the disease have been correctly diagnosed. In patients with celiac disease, the small intestine reacts to gluten--a protein found in wheat, barley, and rye--as an invader, unleashing a cascade of immune responses to eliminate it causing severe damage. An estimated 40% of all Americans carry the genetic biomarker for celiac disease; however, the trigger that actually sets off the disease within this defined population is unknown. Celiac disease has more than 200 symptoms, including brain fog, gastrointestinal distress, fatigue, depression, arthritis, and infertility. The comorbidities of celiac disease include a host of other autoimmune diseases, as well as heart disease and cancer. The only approved treatment of celiac disease is strict adherence to a gluten-free diet, but our research shows that 30-50% between of people on the diet continue to report symptoms and/or have intestinal damage. We also know that federal investments in celiac disease research through the NIH have been minimal, especially relative to the massive burden of disease. What is desperately needed by celiac disease patients and caregivers are effective treatments and a cure.

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This is why we at CDF share PCORI's commitment to generating high-quality, evidence-based research that supports patients, family members, caregivers, and health professionals, in making healthcare decisions that reflect patients' needs, preferences, and desired health outcomes. Since its creation in 2010, PCORI has supported more than 600 research-related projects in high impact areas such as cardiovascular disease, prostate cancer, opioid prescribing, type 2 diabetes management, and celiac disease. By requiring that studies engage patients at all stages of the research process, PCORI-supported studies generate data that are more relevant to patients and those who care for them, and are more feasible to implement in clinical practice, including demonstrating the ineffectiveness of the gluten-free diet for celiac disease patients. For the celiac disease community (patients, family members, caregivers, healthcare professionals, payers, government, and other stakeholders), PCORI has been a game-changer. PCORI funding helped CDF create iCureCeliac[®], the nation's only celiac disease patient registry. Research on the iCureCeliac[®] dataset has helped us document that a gluten-free diet does not resolve most patients' symptoms and that treatment alternatives are desperately desired. This finding has helped trigger a push in drug development. We also have a better understanding from the iCureCeliac[®] registry of the massive economic, social and psychological burdens of living with celiac disease. PCORI funding also allowed CDF to recruit and train a national network of Celiac Disease Patient Advocates to engage as active partners in the research process and to speak on behalf of three million Americans with celiac disease to researchers and public policy communities.

CDF strongly encourages Congress to reauthorize PCORI and in doing so, to consider the following recommendations that we believe are essential to furthering PCORI's patient-centric research mission.

Reauthorize PCORI and its current funding mechanism for at least an additional 10 years

Stability in PCORI funding is imperative for the ongoing investment in PCORI-supported research to be most impactful. Biomedical research is a long-term endeavor, and some high quality Comparative Effectiveness Research studies can take 4-5 years to complete. Stable funding will allow for ample time to conclude studies, disseminate the findings, develop implementation tools, and drive implementation where appropriate. Reaffirming the commitment to PCORI for another 10 years will allow the institute to build on its success in changing the culture of research to be more patient-centric, and to enhance its work in partnership with other agencies and stakeholders to support a sustainable infrastructure for disseminating and implementing research outcomes meaningfully into practice.

Continue to exclude cost-effectiveness and extend the exclusion quality-adjusted life years from PCORI-supported studies

CDF believes the reauthorization legislation should continue to specifically prohibit PCORI from conducting cost-effectiveness analyses. CDF supports PCORI's view of cost-effectiveness research, namely that it is not an outcome of direct importance of patients. CDF further requests that Congress exclude quality-adjusted life years (QALY) as a potential outcome evaluated by PCORI-supported studies. QALY is not a patient-centric metric and patients have a long history of opposing its use in health research. By assigning value to perfect health, QALY leads to bias and discrimination against patients with chronic conditions like celiac disease and/or disabilities in seeking access to care that may improve their quality of life.

CDF applauds PCORI's ongoing efforts to support promising interventions that bring together patients and those who care for them to engage in meaningful research that can improve patient outcomes while improving the efficiency of the healthcare system. Unless Congress acts, however, all this work will cease at the end of September 2019. CDF looks forward to engaging with you throughout the reauthorization process.

Thank you considering our recommendations.

Sincerely,

A handwritten signature in blue ink, appearing to read "James J. Watson".

James J. Watson
Chairman of the Board

A handwritten signature in blue ink, appearing to read "Marilyn G. Geller".

Marilyn G. Geller
Chief Executive Officer