FasterCures
A CENTER OF THE MILKEN INSTITUTE

November 12, 2014

The Honorable Fred Upton
Chairman
Energy and Commerce Committee
U.S. House of Representatives
2125 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Diana DeGette
Member
Energy and Commerce Committee
U.S. House of Representatives
2368 Rayburn House Office Building
Washington, D.C. 20515

RE: Legislative Proposal for the 21st Century Cures Initiative
Sent via e-mail: cures@house.mail.gov

Dear Chairman Upton and Representative DeGette,

As is evident by our name alone, *FasterCures*' mission is tightly aligned with the stated goal of the Committee's 21st Century Cures Initiative, to accelerate the pace of cures. The listening phase of the Initiative has already done just that by inspiring an intense, intelligent, solutions-oriented dialogue about ways stakeholders across the biomedical system can contribute to faster cures. Of course, sustained and full funding of both NIH and FDA are of paramount importance to the success of the biomedical research system and we urge you to work with two groups we are members of on funding issues - United for Medical Research and the Alliance for a Stronger FDA.

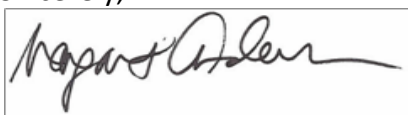
We are honored to have been included in formal sessions with the Committee and in dozens of other stakeholder meetings where ideas have percolated and proposals have emerged. Your visionary leadership and the Committee's roundtable discussions and hearings have produced a great deal of consensus from the community about priority areas of promise and action. You have raised awareness and deepened understanding by bringing a wide range of issues and opportunities into sharper focus for lawmakers and constituents alike.

With the Committee transitioning from listening to legislative mode, we offer a proposal focused on the creation of a public-private partnership, an entity we have referred to as the Partnership to Advance the Science of Patient Input. This proposal reflects insights drawn from *FasterCures'* programs dedicated to venture philanthropy organizations, medical research consortia, patient-centered benefit- risk assessment, and value and coverage. It is informed by interactions with patient-based organizations, industry, academia, government agencies, legislative bodies, investors, healthcare professionals, payers, and the public. It builds on key principles articulated in our written statement of June 25, 2014.

In our analysis of the vast landscape of possibilities, the concept outlined here has enormous potential to advance our shared goal of faster cures by developing science-based methods to elicit, quantify, and utilize patient perspectives to inform and influence decisions throughout the full arc of the discovery, development, and delivery cycle. It addresses many of the needs outlined in the Committee's "Call to Action" that launched the 21st Century Cures Initiative. It echoes many other stakeholders' recommendations and priorities and leverages investments made by the federal government and the private and public sectors.

We would be pleased to discuss this proposal and to provide further supporting detail. For the benefit of every American, we urge the Committee to include this concept in its forthcoming legislation to improve the efficiency and effectiveness of the biomedical system and we look forward to working on this in partnership.

Sincerely,



Margaret Anderson
Executive Director

PROPOSAL TO THE U.S. HOUSE ENERGY AND COMMERCE COMMITTEE
Partnership to Advance the Science of Patient Input

Executive Summary: Patient-centricity is heralded as a major innovating force in research and healthcare. However, at present the knowledge about and methods for capturing, analyzing, and utilizing patient input are decentralized and are undergoing rapid evolutionary change without an understanding of their success or impact. A public-private partnership provides the ideal forum to: assess the current state of understanding of the science of patient input; identify gaps and needs; spearhead development of tools, standards, and methods; and guide application to settings across the full arc of the discovery, development, and delivery cycle to fulfill the promise of a patient-focused biomedical system. For purposes of this proposal, we refer to such a partnership as the Partnership to Advance the Science of Patient Input. It is important to note that robust funding for both the NIH and FDA are critical to the success of this type of work. Stable and full funding for these agencies is of paramount importance.

A Shifting Paradigm

There are more than 10,000 known prevalent and rare human diseases and fewer than 10 percent of these have an approved primary therapy. This enormous gap represents serious unmet medical need with millions of patients' lives hanging in the balance. In many of the roundtable discussions and hearings convened under the 21st Century Cures Initiative, individuals representing diverse stakeholder groups spoke persuasively about the promise of a more patient-focused system of biomedical research and care to narrow this gap. Yet until fairly recently, patients and patient groups were considered special interests rather than partners. This is changing, with more patients becoming pro-active participants in the system and more patient-based organizations becoming research engines themselves. Patient-based non-profits are making strategic research investments informed by a detailed understanding of the therapeutic development pipeline. They are building registries, biorepositories, and clinical trials networks. They convene experts to develop care guidelines and accreditation standards and they provide data to payers to improve access to care through informed coverage and reimbursement policies. They are the catalysts for a 21st century of cures.

Research institutions are gaining respect for the content expertise housed in patient communities and they are increasingly interested in engaging patients in the prioritization of basic and translational research to ensure that their needs are understood, their viewpoints are reflected, and their networks are engaged. Two examples of federal funders leading this trend are the Department of Defense Congressionally Directed Medical Research Program's inclusion of consumers in the scientific review of research applications and the NIH's National Center for Advancing Translational Science's formation of a Patient Engagement Subcommittee of its Advisory Council.

The long, costly, and complex process of developing and approving new medical products has traditionally occurred without much direct interaction with patients, aside from the vital role they play as subjects in clinical studies. Some innovative pharmaceutical and biotechnology companies have recognized that bringing patient perspectives closer to all aspects of the research and development enterprise has the potential to focus resources on therapies that patients truly value, potentially saving time and expense. We have seem those efforts grow in recent years.

Congress also recognized the opportunity to benefit from patients' perspectives in regulatory decision-making with passage of the 2012 Food and Drug Administration Safety and Innovation Act (FDASIA) and related user fee agreements that created new programs to expand patient input and consider patient perspectives in the structured assessment of benefits and risks. Increased attention from regulators in patient views will almost certainly spur even more interest from industry sponsors.

In the past, regulatory approval of new medical products defined success. But a new benchmark is achieving a "reimbursable label." This requires sponsors to satisfy payers' expectations for evidence that a medical product improves the way a patient feels or functions when making coverage determinations, a different threshold for some products than regulators require. Demonstrating clinically meaningful benefit can be linked to patient-reported outcomes, but evidentiary standards, even for public payers including the Centers for Medicare and Medicaid Services, are not easy to gauge.

Finally, the Patient-Centered Outcomes Research Institute (PCORI) was created as a provision of the 2010 Patient Protection and Affordable Care Act to improve the quality and relevance of evidence available to help patients, caregivers, clinicians, employers, insurers, and policy makers make informed health decisions. PCORI has involved patients and other consumers in all facets of its planning, implementation, and evaluation and it has made patient engagement a requirement for all research it supports.

These trends have created a new currency for patient data and have intensified the need to sharpen the science of how patient input is collected and made actionable. Stakeholders across the research and care enterprise are working – mostly independently – to define and scale patient engagement, develop instruments to measure patient-reported outcomes, quantify preferences, and incorporate patient perspectives and insights into decision-making processes and work flows. However, there is little documented evidence of successful practices to emulate or failed experiments to avoid that could inform programs, guide resource allocations, or shape policy. Concerns about privacy, conflicts of interest, and other ethical, legal, and regulatory barriers – actual or perceived – add further uncertainty.

The Promise of Partnership

Public-private partnerships (PPP) are neutral forums where entities that represent the interests of society, such as government agencies and non-profit organizations, collaborate with the commercial sector to advance a mutual interest or address a shared challenge. PPPs can be small and temporary or formal and institutional. Successful PPPs are built on a commitment to outputs that benefit the whole, rather than a single group. The structure provides a means to integrate resources and to identify, manage, and isolate conflicts of interest to preserve integrity. The opportunity they provide to pool resources, leverage assets, and access specialized expertise and information in a safe harbor makes PPPs a particularly appealing structure to tackle complex challenges that affect multiple stakeholders. *FasterCures* has spearheaded efforts to characterize these consortia and the metrics for their success through our Consortia programmatic efforts.

The urgent need to bring greater organization and rigor to patient engagement and patient input is one such challenge. A PPP would provide the ideal forum to: assess the current state of

understanding of the science of patient input; identify gaps and needs; spearhead development of tools, standards, and methodologies; and guide application to settings across the full arc of the discovery, development, and delivery cycle to fulfill the promise of a patient-focused biomedical system. For purposes of this proposal, we refer to such a partnership as the Partnership to Advance the Science of Patient Input.

This cross-sector Partnership would provide a neutral collaborative environment to align interests, integrate multiple disciplines and types of expertise, and harness knowledge and data from diverse sources that currently reside in various government departments and agencies, academic and professional organizations, the private sector, and not-for-profit entities. High-level leadership and active participation from the government sector (including the National Institutes of Health, Food and Drug Administration, and Centers for Medicare and Medicaid Services), the private sector (including pharmaceutical companies, biotechnology companies, device companies, diagnostic companies, and private payers), and the public sector (including patients, health care consumers, voluntary health organizations, and academic researchers) will be vital to its success. Partners would contribute human, intellectual, and financial resources and would participate in governing the PPP. A third party manager provides overall program management support, facilitates timely communication between participants, partners, and external stakeholders, and helps to resolve conflicts and questions. The manager also stewards the products of the PPP, monitoring adoption and bringing new opportunities to the attention of the governing body.

The Accelerated Medicines Partnership of the NIH, FDA, 10 biopharmaceutical companies and numerous non-profit organizations, managed by the Foundation for the NIH serves as an appropriate governance model. *FasterCures* can provide additional guidance on a governance structure at the Committee's request; such guidance would be based on its programmatic work in the Consortia-pedia report that documents current practices among more than 400 biomedical research consortia. Defining a formal governance structure will be essential to establish expectations and trust needed to keep participants engaged, as well as ensure a high level of accountability.

Building on Prior Investments

The science of patient input has grown organically in response to a broad variety of needs and specialized interests. Several substantive federal investments in programs designed to capture patient input provide a strong basis for a focused Partnership effort. To highlight a few:

Patient-Reported Outcomes Measurement Information System (PROMIS): Funded by the National Institutes of Health, PROMIS aims to provide clinicians and researchers access to efficient, precise, valid, and responsive adult– and child–reported measures of health and well–being. PROMIS tools measure what patients are able to do and how they feel by asking questions. PROMIS' measures can be used as primary or secondary endpoints in clinical studies of the effectiveness of treatment.

Study Endpoints and Labeling Development (SEALD): Supported by the FDA in the Center for Drug Evaluation and Research, SEALD advances innovation and excellence in clinical trial measurement of treatment benefit. This includes the development and implementation of standards for clinical outcome assessments used as effectiveness endpoints and review policies to provide medical product labeling that is accurate, consistent, and useful.

Patient Preference Initiative: FDA's Center for Devices and Radiologic Health (CDRH) established this initiative to provide the information, guidance and framework necessary to incorporate patient preferences on the benefit-risk tradeoffs of medical

devices into the full spectrum of CDRH regulatory processes and to inform medical device innovation by the larger medical device community.

Registries for Evaluating Patient Outcomes: A User's Guide: This reference published by the Agency for Healthcare Research and Quality (AHRQ), now in its third edition, provides information on the design, operation, and analysis of patient registries. In 2010, the User's Guide was updated with a focus on collecting information to assess patient outcomes.

Some of the other existing resources funded by government, public and private entities are listed in Table 1 at the end of this proposal. It is not intended to be a complete listing, but merely an illustration of the types of existing U.S.-based efforts that support further expansion of this field and its potential to transform the biomedical ecosystem through stronger coordination of efforts and investments. A comprehensive landscape assessment performed as an early step in the Partnership would serve to inventory past investments and existing tools in order to identify and prioritize gaps and needs.

Tools and Processes That Span the Full Arc of Discovery, Development, and Delivery

Building on existing resources, the Partnership would form teams to develop and validate tools such as standards, methods, and instruments to elicit, collect, store, and utilize patient input. Well-defined pilot projects and demonstration models in targeted populations or focused clinical areas are likely to precede more generalizable approaches. Specific projects might be organized according to objectives such as expanding patient participation (as might be useful for a patient registry, clinical trial, prevention program, or surveillance network), measuring meaningful benefit (to determine efficacy, guide product labeling, establish value, or improve adherence), or assessing unmet needs (for making research resource allocations, identifying therapy targets, understanding risk tolerance, or assessing product satisfaction). Of paramount importance will be approaching the development process with the intention of deriving cross-cutting benefits that meet needs across the full arc of the biomedical ecosystem, rather than the interests of any single stakeholder group or participating institution.

Envisioned as an extension of the 21st Century Cures Initiative through authorizing legislation that the U.S. House Energy and Commerce introduces, the Partnership is proposed to have near-term and lasting applications for federally supported activities that improve public health. Specific functions that could be enhanced include the allocation of federal research funds, regulation of medical products, and coverage for healthcare products and services that are more strongly aligned with patient needs, priorities, and expectations. Outputs of the Partnership have potential to inform executive branch programs, policies and rulemaking, yet do not supplant authorities previously granted to federal departments or agencies. The Partnership may also serve as a valuable resource for informing future legislative priorities.

TABLE 1: Existing Resources for Building the Science of Patient Input

Government	Academic & Non-Profit Organizations	Private Sector
Agency for Healthcare Research and Quality: Consumer Assessment of Healthcare Providers and Systems, National Quality Measures Clearinghouse, Registry of Patient Registries and User's Guide to Registries for Evaluating Patient Outcome Measures	Brookings Institution: Enhancing the Use and Development of Patient-Reported Outcome Measures in Drug Development Centre for Innovation in Regulatory Science (CIRS): Unified Methodologies for Benefit-Risk Assessment (UMBRA)	Mayo Clinic: Shared Decision-Making National Resource Center Optum: SF Health Surveys PatientCrossroads: Connect Patients Like Me: Open Research Exchange

CMS initiative Partnership for Patients - http://partnershipforpatients.cms.gov/ Department of Defense Congressionally Directed Medical Research Program: Consumer Involvement program Food & Drug Administration (FDA)-Center for Devices and Radiologic Health: Patient Preference Initiative FDA-Center for Drug Evaluation and Research: Patient Focused Drug Development Initiative and Study Endpoints and Labeling Development FDA-Office of the Commissioner: Patient Representative Program and Patient Network National Cancer Institute: Outcomes Research Branch National Institutes of Health: Patient Reported Outcome Measurement Information System (PROMIS)	Clinical Trials Transformation Initiative: Best Practices for Engagement with Patient Groups in Clinical Trials Critical Path Institute: Patient-Reported Outcomes Consortium and Electronic PRO Consortium Genetic Alliance: Platform Engaging Everyone Responsibly Center for Medical Technology and Policy: Green Park Collaborative International Society for Pharmacoeconomics and Outcomes Research (ISPOR): Outcomes Guidelines Research Index Medical Device Innovation Consortium: Patient-Centered Benefit-Risk Assessments National Health Council: Information Collection Tool for Patient Organizations and Implementation Manual National Organization of Rare Disorders: Registry Platform National Quality Forum: PROs in Performance Measurement Parent Project Muscular Dystrophy: Draft FDA Guidance project Patient-Centered Outcomes Research Institute: National Patient-Centered Clinical Research Network, engagement methodology and patient-centered research methodology	Sanofi: Partners in Patient Health 23andMe: Participatory research