February 23, 2015

The Honorable Lamar Alexander
Health, Education, Labor & Pensions Committee
455 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Richard Burr
Health, Education, Labor & Pensions Committee
217 Russell Senate Office Building
Washington, DC 20510

RE: Innovation for Healthier Americans Report

Dear Senators Alexander and Burr,

FasterCures, a center of the Milken Institute, is a non-profit, non-partisan action tank driven by a singular goal – to save lives by speeding up and improving the medical research system. We thank you for the opportunity to submit comments in response to the report issued on January 29, 2015, "Innovation for Healthier Americans." These comments have been informed by our programmatic work as well as outreach to our network of thought-leaders from patient organizations, industry, academia, and healthcare institutions, including our senior fellows and members of our various advisory councils.

FasterCures' mission is tightly aligned with your initiative's stated objective, "to better align public policy to support medical innovation and patient access to new medicines and technologies." Your report depicts the increasing challenge and complexity of advancing discoveries through development, to regulatory approval, and to market. Identifying solutions will rely on participation from the "full spectrum of stakeholders." In this set of comments, we propose three areas where coordinated action could lead to substantive improvements in the system.

1. Transition from FDA's traditional model of engagement with designated patient representatives, to an evidence-based model that can effectively integrate the whole patient experience – including unmet medical needs, risk tolerance, and outcome preferences – into regulatory decision-making.
2. Apply learnings from *FasterCures'* analysis of over 350 consortia through our "Consortia-pedia" program to enhance the many public-private partnerships in which federal agencies, particularly FDA and NIH, are involved to increase their effectiveness and output.
3. Strengthen mechanisms to foster inter-agency collaboration between NIH and FDA in developing regulatory science tools such as biomarkers and clinical outcome assessments with the appropriate regulatory standards from the earliest stage of research. This includes bolstering jointly supported regulatory science training to ensure that our nation's regulatory agency has a workforce with appropriately diverse scientific training to improve the development, review and oversight of new drugs, biologics, and medical devices.

1. Integrating Patient Perspectives in Regulatory Decision-Making

We believe that a more effective and efficient process of developing and deploying medical products begins by understanding the benefit expectations and risk thresholds of the patients for whom these products are intended. The agency's current approach to patient engagement is outlined on pages 14-16 of the January 21,

2015 letter from FDA's Associate Commissioner for Legislation, Thomas A. Kraus, included as Appendix C to the Innovation report.

While the 2012 FDA Safety and Innovation Act created new opportunities for patient perspectives to inform regulatory decisions, we believe more can be done. In December, we submitted a detailed set of recommendations to the FDA in response to Federal Register notice FDA-2014-N-1698 seeking input on this topic. Our recommendations, appended to this letter, have one aim: **to facilitate an intentional evolution from FDA's traditional engagement with individuals who serve as spokespersons for a disease/condition to an evidence-based means of understanding the range of patients' experiences across the lifespan, unmet medical needs, meaningful treatment benefits, risk tolerance, and outcome preferences that is complemented by individual participation of patients and patient advocates in selected roles** (e.g., special government employees (SGEs) serving on advisory committees).

There are presently multiple initiatives to better define patient-centricity and the successful practices that support it. As a regulatory agency subject to stringent statutory requirements that define its interface with the public, FDA's engagement with patients will be subject to some unique restrictions. Even so, a set of guiding principles applied across all the agency's patient engagement activities would serve as both a compass and a scorecard for current and future initiatives. At minimum, we believe that FDA's patient engagement activities should be:

- **Purposeful** – Engagement should be designed and executed with the intent to inform the agency's mission, strategy, and operations, including policy and regulatory decisions. It should be valued by staff at all levels of the agency as integral to their role in protecting and promoting public health.
- **Reciprocal** – Whenever possible, engagement should be predicated on fostering a mutually beneficial information exchange between patients, industry, regulators, and other stakeholders. To elicit useful types of data, expected outcomes and deliverables should be communicated to the stakeholders. This can occur without violating privacy or confidentiality boundaries that safeguard participants.
- **Dynamic** – Much of FDA's interaction with patients and patient communities is currently episodic or cross-sectional, limited to annual forums or single events. Patient needs within a community and across communities change in response to scientific and technologic advances and other circumstances. Engagement activities should seek to build ongoing relationships and maintain updated information. These activities should be designed in such a manner that patient groups of any size are able to organize themselves and effectively engage.
- **Transparent** – In addition to required reporting on patient engagement activities through vehicles such as publicly available meeting agendas and minutes, the outcomes of patient engagement should be visible to the community, particularly when they affect or influence decisions or policy.

We recognize that such evolution will take time and will require dedicated resource investment and ongoing collaborative efforts by the agency, sponsors of medical product development, and the patient community. 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.

In a second appendix, please find attached a proposal for such a partnership we refer to as the Partnership to Advance the Science of Patient Input. There are substantial existing federal investments that could be brought together to build the science of patient input, some of the same ones you have outlined in your report and that we have identified in the appendix.

2. Increasing the Impact of Ongoing Public-Private Partnerships

Section VIII of the Innovation report highlights the collaborative initiatives undertaken by NIH and FDA to address scientific deficits in the innovation ecosystem. Public-private partnerships among academia, government, patients, industry and others are highlighted in this section. The report questions how these consortia can be better leveraged and held accountable for their outcomes.

To begin to address these important issues, we draw attention to a body of work created by *FasterCures* through its Consortia-pedia program launched in 2012. *FasterCures* initiated the Consortia-pedia project to better understand the rapid growth of the research-by-consortium trend and the breadth and scope of approaches that consortia have adopted to bring together non-traditional partners with a shared R&D goal.

Our executive summary analyzes **21 consortia,** including several of those highlighted in your Innovation report, that represent the diversity of models used to bring together cross-sector partners to accelerate biomedical research. It highlights the key findings of the Consortia-pedia project and offers a framework to better understand the consortium approach. It presents a series of questions to be contemplated by those seeking to create a new collaborative effort or expand existing ones, and identifies seven partnership components that cut across efforts and define the nature of each consortium:

1. Mission and governance
2. Financing
3. Human Capital
4. Intellectual Property
5. Data-sharing
6. Patient participation
7. Measurement of value and impact

A further report, "Consortium Sandbox: Building and Sharing Resources," published in June 2014 in *Science Translational Medicine* analyzes 369 consortia to better understand the intended scientific output and the players behind these collaborations. We are now collaborating with FDA, the Institute of Medicine, and University of California at San Diego to further expand this project with a wiki-style database of existing consortia and a set of metrics that can be used to evaluate the effectiveness, efficiency, and output of consortia. We would be pleased to brief the Committee on these efforts as we believe they will be complementary to your work.

3. **Strengthen Inter-Agency Collaboration and Training for Regulatory Science Tools**

The Innovation report highlights the imperative to "ensure that the scientific advancement and new regulatory tools resulting from our investments in research through the NIH are fully leveraged by the FDA when reviewing medical products" and asks whether "scientists with NIH funding (where appropriate) [should] be

encouraged to frame their findings in language that meets FDA standards?" It describes multiple efforts by NIH and FDA to identify biomarkers, clinical outcome assessments, and other tools that will help make drug development more efficient.

As highlighted in Section V of the report and in the FDA's January 2015 letter, the Joint Leadership Council formed in 2010 by FDA and NIH "to help ensure that regulatory considerations form an integral component of biomedical research planning and that the latest science is integrated in the regulatory review process" recognized the need for greater collaboration. Its focus to-date on a limited number of well-defined projects may provide sufficient experience to now expand its scope. We strongly encourage the Committee to explore the possibility of strengthening the Joint Leadership Council with expanded authorities that would accelerate its progress toward the shared goal of improved public health by leveraging the strengths of each agency.

We believe there is also opportunity to integrate training programs as part of the Council's coordinating function. The Innovation report brings into sharp relief the current disparity between the surplus of young scientists dependent on support from NIH to launch and sustain early careers in academic and clinical research (documented in pages 6-8) and the persistent challenges FDA faces recruiting and retaining qualified personnel to fill scientific and regulatory positions (detailed by FDA in Appendix C). Addressing this imbalance will likely require experimentation with a variety of programs directed from a holistic perspective of the nation's scientific and regulatory needs and opportunities for the present and for generations to come. The Joint Leadership Council is at least one of the venues where this very important exploration should occur and new pilot programs should emanate.

We applaud your efforts to date on this important initiative. We welcome the opportunity to discuss these comments and to provide additional input as the Committee continues its path toward legislative action on a bill that will generate broad support and, when enacted, will speed medical progress and improve public health.

Sincerely,

Margaret Anderson
Executive Director