Good morning. My name is Kim McCleary and I'm Managing Director of FasterCures. Thank you for the opportunity to participate on this Science, Academia and Innovation panel this morning. For those who may not be familiar with the organization that I represent, *FasterCures* is a nonprofit, nonpartisan DC-based center of the Milken Institute. We work across diseases in partnership with all stakeholders – government, patient organizations, industry, academics and health care professionals – to identify and knock down barriers that add time and expense to the process of getting promising therapies from the lab to patients.

It is a real honor to be part of this meeting the process of launching a period of intensified dialogue about medical product review and regulation in the context of user fee agreements. Many of us see this opportunity for dialogue and discussion as perhaps another venue in which to continue working on some of the themes that have been at the forefront of the U.S. House of Representatives Energy and Commerce Committee's 21st Century Cures Initiative, a bipartisan effort that culminated on Friday in a somewhat historic House vote to approve substantial legislation that promises to accelerate progress for patients. In fact, from the Initiative's launch more than a year ago, a key theme at the many hearings, roundtables and town halls was the need to expand opportunities for patient perspectives to shape and influence medical product development and regulation. The bill includes some important provisions to expand patient-focused medical product development. We view the Senate's deliberations as well as the user fee agreement process as key opportunities to explore how we might do even more to more actively integrate patient perspectives into decision-making about the discovery, development, regulation and delivery of medical solutions.

FasterCures has recently launched a program to advance the science of patient input that bridges work that initially focused on patient-centered benefit-risk assessment, value and coverage with our affinity network of 80 high-performing venture philanthropy patient organizations. As this new era of patient-centricity evolves from being an ideal to reality, we recognize the need to support it with evidence-based methods that can inform decision-making at every step of the process of developing a new medical product and deploying it safely to patients, from carefully characterizing unmet medical need, to product concept, design and engineering, to clinical testing, to regulatory approval, to post-market surveillance. Our program is working collaboratively with others to establish ways of successfully engaging patients, eliciting their perspectives and preferences, and utilizing data to inform decisions.

One of the underlying principles of our organization is collaboration and we have been fortunate to work with Dr. Jeffrey Shuren and his team at CDRH as part of the Medical Device Innovation Consortium's Patient Centered Benefit-Risk project. As you've heard Bill Murray describe, the MDIC-led public-private partnership has recently produced crucial new tools to advance the science of patient input, including a catalog of methods and a framework for incorporating patient perspectives into the development of medical devices. CDRH worked actively in the development of those products and also conducted a parallel process to issue draft guidance for how it would consider patient preference data in the review of PMAs, HDE applications, and de novo requests as well as labeling.

This recent guidance builds on a 2012 guidance regarding principal factors FDA considers when making benefit-risk determinations during the premarket review process. And it further builds on work led by Drs. Telba Irony and Martin Ho to develop a tool to estimate the minimum weight loss acceptable by a patient to receive a device with a given risk profile and the maximum mortality risk tolerable in exchange for a given weight loss. This tool and the discrete choice methodology-driven study behind it, was helpful to regulators in approving a new weight loss device, the Maestro Rechargeable System, in early 2015, providing a new therapeutic option to some patients with obesity. It also demonstrated a means by which regulatory decision-making could be more patient-centered.

FasterCures strongly commends CDRH's leadership to stimulate and foster this area of science and the dialogue it has fostered not only with sponsors of medical device trials but also the impact it is having on biopharmaceutical companies. In fact, this work has recently been cited by the Biotechnology Innovation Organization in a white paper issued at its international convention held last month on a lifecycle approach to benefit-risk assessment as a mechanism to both solicit patient perspectives on areas of unmet medical need and assess patient preferences, and to align with FDA on key benefit-risk considerations.

We recognize the CDRH/CBER guidance as an invitation to industry and to patient organizations to respond to the interest of regulators to consider patient preferences as they review premarket applications. We are working to help build understanding, expand capacity, and enable execution to foster generation of high-quality, representative, rigorous data of regulatory value, that also adds value to patients through better products and more robust communication with health care professionals

who will help them make decisions about the diagnostics and devices they have access to.

In fact, the utility of the patient preference tools that CDRH has supported and issued was made quite real to me last week as I was reading the transcript of a meeting of FDA's Oncology Drugs Advisory Committee in which the advisors were asked to discuss – not simply vote yes or no on – the key questions of risk vs. benefit of an experimental therapy to treat squamous non-small cell lung cancer, a deadly condition that has not seen a new treatment in more than 15 years. The dialogue by advisors at the ODAC meeting wrestled with the sponsor's data that showed a statistically significant improvement in overall survival of 1.6 months and a .2 month improvement in median progression-free survival. The ODAC chair, a professor of oncology at Johns Hopkins University, highlighted that patients with this form of lung cancer have been left behind other advances and that the modest survival benefit is real and an incremental step toward progress. Reading each of the advisor's comments, I was left feeling that this situation represented a really good illustration of a preference sensitive decision and that data about the affected community's range of minimum expectations for benefit and maximum threshold for harm might have helped inform the dialogue and, ultimately, the regulators' decision on whether to approve this therapy.

As we think about the next several years, leading up to agreement on MDUFA-IV and over its life, 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, FDA's patient engagement activities should be purposeful, reciprocal, dynamic and transparent. Allow me to unpack each of those four concepts a bit more:

1. **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.
2. **Reciprocal** – Whenever possible, engagement should be predicated on fostering a mutually beneficial information exchange between patients, industry, regulators, and other stakeholders. This can occur without violating privacy or confidentiality boundaries that safeguard participants.

3. **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.
4. **Transparent** – In addition to 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.

In addition to continuing the excellent work conducted under the Patient Preference Initiative, we encourage CDRH to take two further steps to enhance its interaction with patients:

- Establish the Patient Engagement Panel of Medical Devices Advisory Committee to shape development of guidance or other policy on incorporating patient views into the total product lifecycle of medical devices.
- Seek authority to provide patient representatives serving on the CDRH Advisory Committee with full voting status so that this perspective is afforded equal weight to other forms of expertise on the committee.

In closing, it's important to note that to realize the changes suggested here, FDA must have sufficient resources – both financial and human – to effectively integrate patients in regulatory decision-making and evaluate patient-centered information. This means ensuring that reviewers have the training and tools required to properly assess and analyze data about patient perspectives. We recognize this will also require a shift in culture and we are grateful for the leadership Dr. Shuren has provided as well as Dr. Robert Califf's strong interest in this area. As we've seen already, public-private partnerships like MDIC can be a useful mechanism to enhance this regulatory science, and we must adequately fund these initiatives to ensure that these initiatives advance.

I'd like to thank FDA again for the opportunity to participate in this meeting and to present a patient-centered perspective of MDUFA and its programs. *FasterCures* looks forward to working with FDA and other stakeholders to enhance the science of patient input and to secure the resources and tools FDA needs to effectively incorporate patient perspectives into regulatory decision-making.