Rx Innovation

RECOMMENDATIONS FOR THE NEW ADMINISTRATION

FasterCures

A CENTER OF THE MILKEN INSTITUTE

FasterCures is an "action tank" driven by a singular goal: to save lives by **speeding up and improving** the medical research system

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We want to help the new administration advance biomedical innovation.

We used **our unique view** of the challenges across the R&D ecosystem, as well as our ability to tap into a **broad and deep network** of innovators across sectors and diseases, to craft **an itinerary for the administration**

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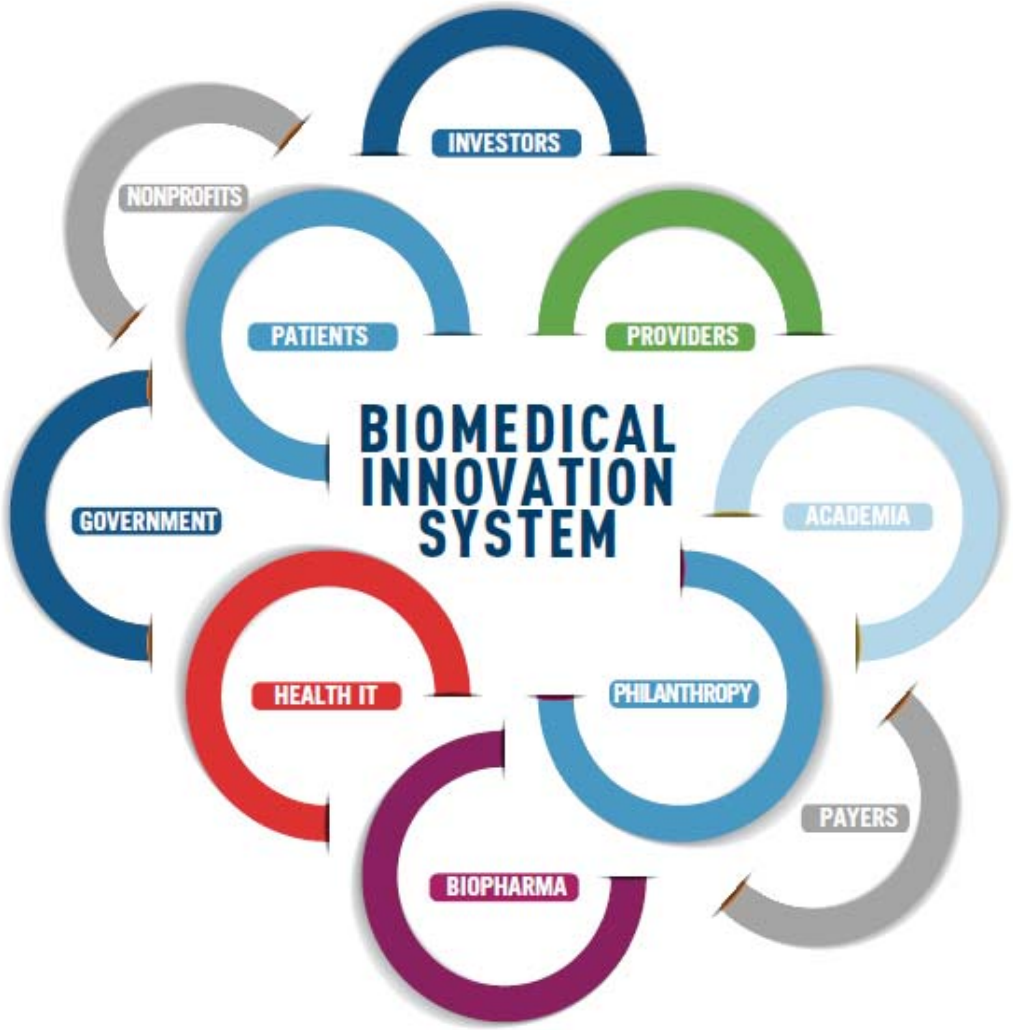
More than 150 people from over 130 organizations



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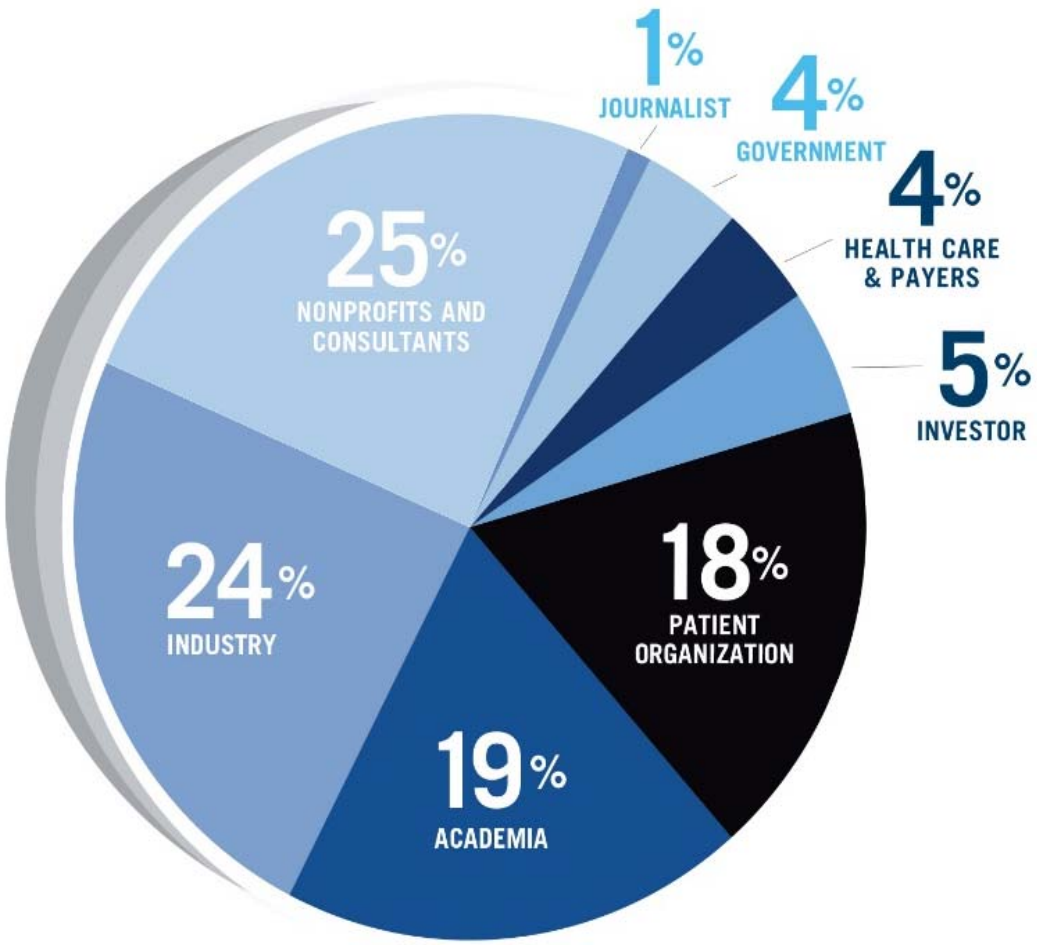

SECTORS REPRESENTED IN THE INTERVIEWS

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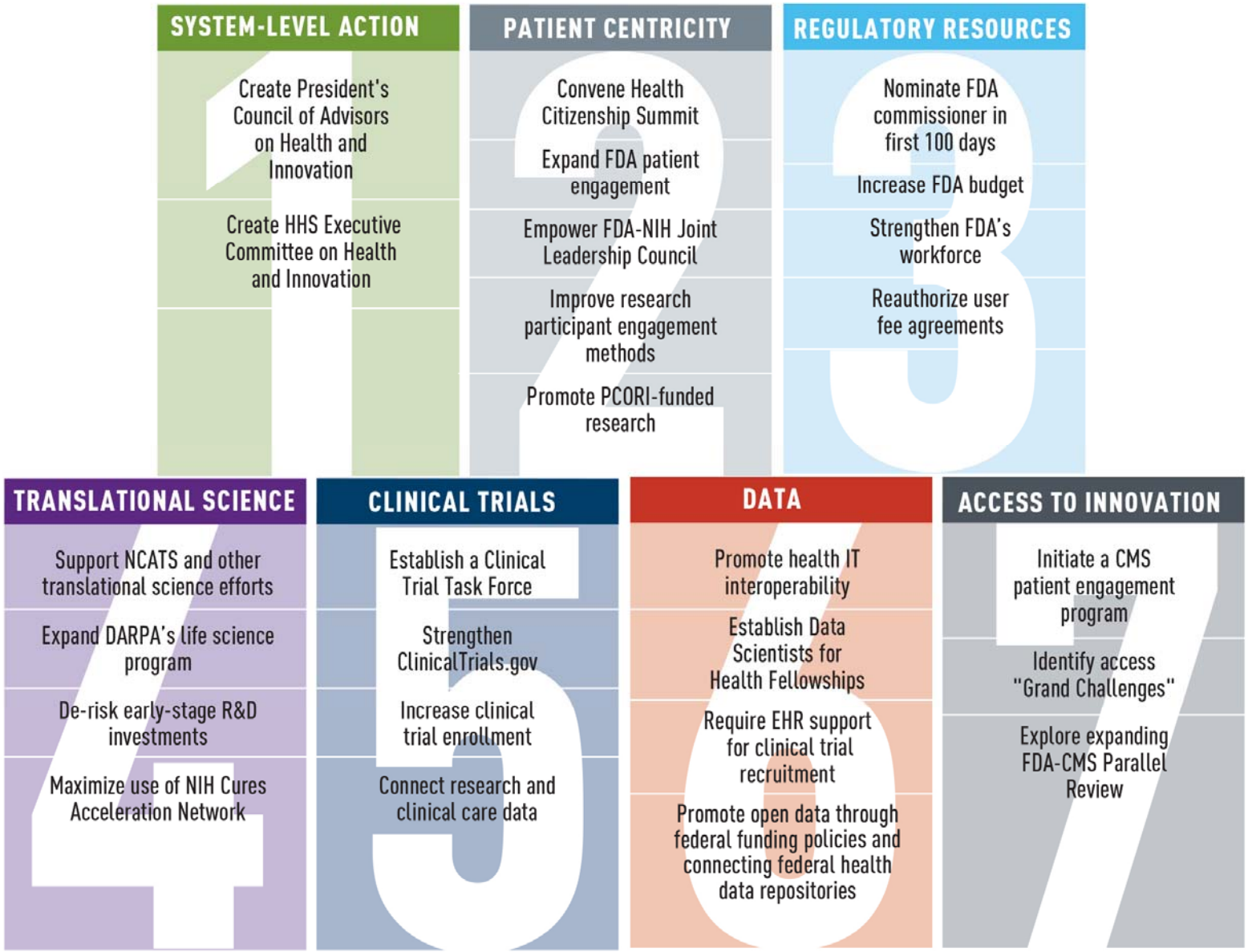
RX FOR INNOVATION RECOMMENDATIONS



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1. System-Level Action

Address common challenges of biomedical innovation and health care through a cross-sector council to advise the president

There is currently no vehicle in the Executive Branch for cross-agency and cross-sector systems-level thinking to facilitate dialogue, articulate priorities and build consensus around solutions to the biggest challenges in health care and biomedical research.

1. System-Level Action recommendations

1.1 Create a President's Council of Advisors on Health and Innovation within the first 100 days.

1.2 Create an Executive Committee on Health and Innovation within the Department of Health and Human Services (HHS).

2. Patient Centricity

Invest in developing and advancing the science of patient input and bolster engagement activities to ensure all stakeholders can benefit from and realize the opportunities of patient centricity.

There is growing recognition that effectively integrating patient perspective data into medical product development and regulatory decision-making will enable researchers and companies to develop and, ultimately, deliver treatments better suited to patients' needs, leading to better overall outcomes.

2. Patient Centricity recommendations

2.1 Convene a health citizenship summit within the first year.

2.2 Support and expand existing efforts at the Food and Drug Administration (FDA) to identify how patient input can be collected, evaluated and incorporated in product development and regulatory decision-making.

2.3 Empower the existing FDA-National Institutes of Health (NIH) Joint Leadership Council to support research to advance regulatory science, including the science of patient input.

2.4 Incorporate throughout all NIH-supported research the core principle from the Precision Medicine Initiative's *All of Us* ® program, that research studies enroll "participants," not "subjects."

2.5 Promote Patient-Centered Outcomes Research Institute-funded research so that all stakeholders (FDA, industry, patients) can learn from the results and from the patient-centered methodology.

3. Regulatory Resources

Provide FDA with the tools and resources required to sustain its critical mission and continue to advance innovative regulatory policy.

FDA-regulated products account for about 20 cents of every dollar spent by American consumers, yet the agency is continually strapped for financial resources and human capital.

3. Regulatory Resources recommendations

3.1 Nominate an FDA commissioner within the first 100 days.

3.2 The importance and priority of FDA should be reflected in the budget.

3.3 Take action to help address FDA's persistent challenges to building and maintaining its workforce of highly trained scientists, clinicians, statisticians and engineers.

3.4 Support the timely reauthorization of the Prescription Drug User Fee Act and Medical Device User Fee Amendments.

4. Translational Science

Build bridges across the "Valley of Death" to move basic science discoveries closer to products that will help patients.

The lack of funding, technical expertise and incentives – as well as the high risk of failure – for the important translational science needed to turn a promising basic research insight into a transformational therapeutic has been a major impediment to "faster cures."

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4. Translational Science recommendations

4.1 Maintain strong support for the National Center for Advancing Translational Sciences and other NIH and U.S. government initiatives aimed at building tools and expertise to bridge the translation gap.

4.2 Make life sciences a greater focus for the Defense Advanced Research Projects Agency.

4.3 Investigate the creation of an innovation investment fund, bond or other mechanism to de-risk early-stage investments in biomedical innovation serving high-need areas.

4.4 Maximize use of NIH's Cures Acceleration Network.

5. Clinical Trials

Create a revolution in clinical trials through a focused effort to leverage new technologies and accelerate existing efforts across the public and private sectors.

Clinical trials are the longest and most expensive phase of medical product development. Identifying appropriate patients and recruiting them into a clinical trial are key bottlenecks for biomedical innovation.

5. Clinical Trials recommendations

5.1 Establish a White House "Clinical Trial Task Force."

5.2 Strengthen ClinicalTrials.gov by enforcing current law requiring data submission and by investing in the platform to improve the user experience for all the system's stakeholders.

5.3 Support and strengthen efforts to increase enrollment in clinical trials, reducing development costs and timelines.

5.4 Accelerate joint FDA-NIH-Office of National Coordinator (ONC) efforts to overcome the disconnect between data generated during research and during clinical care.

6. Data

Enable health data to flow freely and empower patients to control their own data.

We won't be able to fully benefit from "big data" in health until these data are integrated into a seamless system, instead of a multitude of silos.

6. Data recommendations

6.1 Enable interoperability by strengthening enforcement tools and requiring that open, non-proprietary application program interfaces be built into health information technology (IT) systems.

6.2 Establish a "Data Scientists for Health" fellowship program to provide opportunities for top scientists to collaborate with government staff on biomedical research, delivery and reimbursement challenges.

6.3 ONC should explore new health IT certification requirements that require that electronic health records have the ability to support and accelerate recruitment of participants into clinical trials.

7. Access to Innovation

Ensure that patients can access innovative therapies and cures in a sustainable way.

There is growing concern that some medical products may successfully move through clinical development and achieve regulatory approval, only to be unavailable to patients because of prohibitively high prices, unfavorable coverage decisions and high out-of-pocket costs.

7. Access to Innovation recommendations

7.1 Initiate a program at the Centers for Medicare & Medicaid Services (CMS) by the end of 2017 where its staff engage directly with patient communities.

7.2 Establish a working group at HHS to identify access "Grand Challenges."

7.3 Explore expanding FDA-CMS Parallel Review.

What is #HealthCitizenship?

- Increased and active engagement from individual citizens – healthy and not – in their own health and the biomedical innovation system
- #HealthCitizenship requires 2-way engagement between individual citizens *and* health/research institutes to maximize benefits to research, development and access

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How can we mobilize and inspire #HealthCitizenship?

- Embrace patient centricity in research, development and regulatory approval
- Design clinical trials to address the questions of most importance to patients, and employ innovative trial designs and technology to lower the logistical hurdles/burdens of participation
- Share data across institutional lines and with study participants
- Determine value of medical products and interventions in ways that meaningfully incorporate patient perspectives

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The Path Forward

Biomedical innovation is vital to America's health and economic well-being. *FasterCures* looks forward to working with the administration, Congress and stakeholders across the system to realize these proposals and improve the biomedical innovation system for the benefit of all citizens.

www.fastercures.org/innovation

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