



Rx **for** Innovation

RECOMMENDATIONS FOR THE NEW ADMINISTRATION



FasterCures
A CENTER OF THE MILKEN INSTITUTE



Preface

Dear friends and colleagues,

Every four years, the United States votes to elect the president of the United States. With that election comes the responsibility of overseeing the administration's vision for the future.

The *FasterCures* Rx for Innovation project aimed to get a wide-ranging perspective on what the next president should prioritize in the biomedical research space. We are grateful to the more than 150 people who took the time to speak with us and offer their perspectives on the tangible things that should be done and what the future could look like.

I was struck by the intense optimism people had, not only for what innovation lies ahead but also for the opportunity that this transfer of power offers. That was not an indictment of what has gone before, but instead a recognition that a new administration brings with it new perspectives and the chance to take a critical look at what needs attention.

I'd like to issue a call to action that we keep biomedical research and the innovation that is possible on the top of the next president's to-do list. We cannot afford to hit pause on the momentum that exists in science. There is too much at stake both in terms of the world-class scientific system that exists in the United States and, even more importantly, the promise of what that science could offer to patients who are waiting for advances.

There was a strong thread throughout our interviews about the role that data from all of us can and should play in future discoveries. Just like everyone should exercise his or her civic duty to vote in elections, we heard the call for a new movement: Health Citizenship (look for this #HealthCitizenship icon throughout the report to identify these areas). The idea would be to inspire citizens (healthy and not) to engage more fully in their own health and in the biomedical innovation system – through clinical trials, the science of patient input and more.



Citizenship implies a reciprocal relationship between individuals and the larger whole. We must have a system that makes it both easy and necessary to engage citizens and to meaningfully incorporate the perspectives of citizens/patients into the system. The next president should take a leading role in this movement by bringing together all of the agencies that work in science and health to move it forward.

So let us not waste a moment. Interviewees expressed great optimism about the power of what science is bringing forth. It is both evident and palpable in many areas – for example, cancer (and, specifically, the power of immunotherapy), precision medicine models and data gathering, and understanding the microbiome.

All of us stand ready to work to advance science to better our understanding of disease and health and to promote #HealthCitizenship for the betterment of all of us, because we will all be patients one day. We heard a resounding call to weave all of the sectors together into a true system. The Greek philosopher Aristotle told us that the whole is greater than the sum of its parts. If that opportunity for synergy can be furthered for the citizens being told daily that they have a disease or illness with limited treatment options, I think we'll all raise our hands to help the next president advance that goal.

Sincerely,

A handwritten signature in black ink, appearing to read 'Margaret Anderson', with a long horizontal flourish extending to the right.

Margaret Anderson
Executive Director
FasterCures

November 11, 2016

A note to readers:

The Rx for Innovation project was supported by *FasterCures'* general operating budget; funding did not come from any single source. All of the interviews, analysis, research and writing was done by *FasterCures* staff; no outside consultants were employed. *FasterCures* had final editorial control over all of the project's outputs.

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Introduction

Biomedical innovation is vital to America's health and economic well-being. There is critical need for it. President-elect Donald Trump is set to take office on Jan. 20, 2017, and, based on our work, we know thought leaders in biomedical innovation have many ideas for how the new administration can strengthen and support innovation. At *FasterCures*, we utilized our vast network of stakeholders across every sector in the innovation ecosystem, we've listened to their insights and ideas, performed our own research and analysis and then converted it into this set of recommendations and ideas.

FasterCures was founded to shine a light on the biomedical innovation system as a whole – government, academia, industry, patients and beyond and understand the challenges and the opportunities (Figure 1). *FasterCures* has a keen focus on understanding and addressing the obstacles that are slowing progress for everyone, regardless of disease or sector. We interviewed more than 150 leaders across all sectors to listen to their ideas and help us answer one basic question: What are the opportunities for the next president to propel biomedical innovation forward? Here are *FasterCures'* suggestions for how those ideas can be translated into action.

A New Frontier of Discovery

The life sciences will be as transformative to the U.S. economy and society in the early 21st century as the Internet was in the late 20th century. Research and development for disease treatments is still very long, risky and expensive (Figure 2), but the convergence of scientific disciplines is bringing forth an array of powerful scientific advances. We are programming the body's own immune system to attack cancer. We've created molecular scissors to precisely edit DNA. We are mining vast data sets to better understand the brain, the microbiome and much, much more.

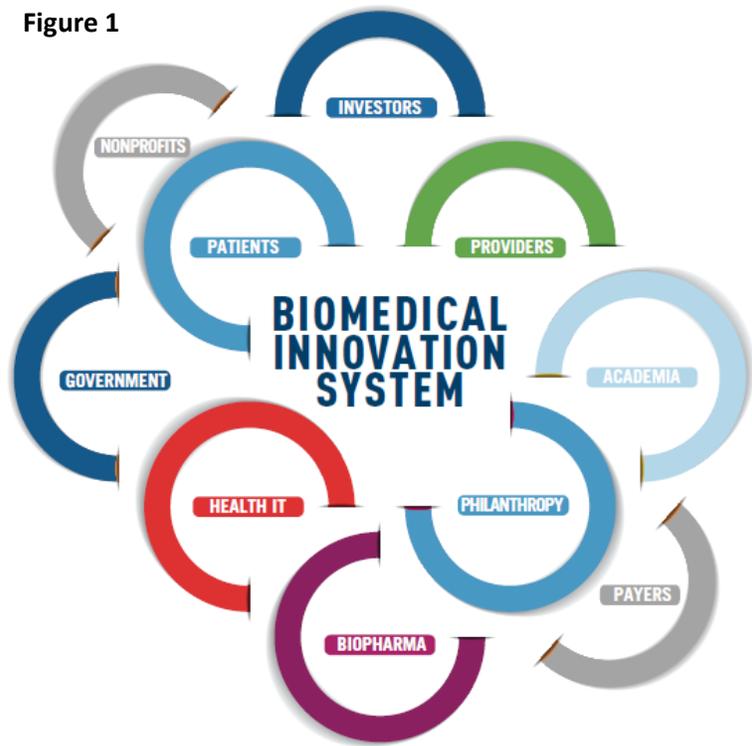
These benefits, and U.S. leadership in the life sciences, did not happen by accident and are not guaranteed to continue indefinitely. Decades of federal commitment to funding biomedical research and promoting innovation through policy are responsible for the benefits we see today.

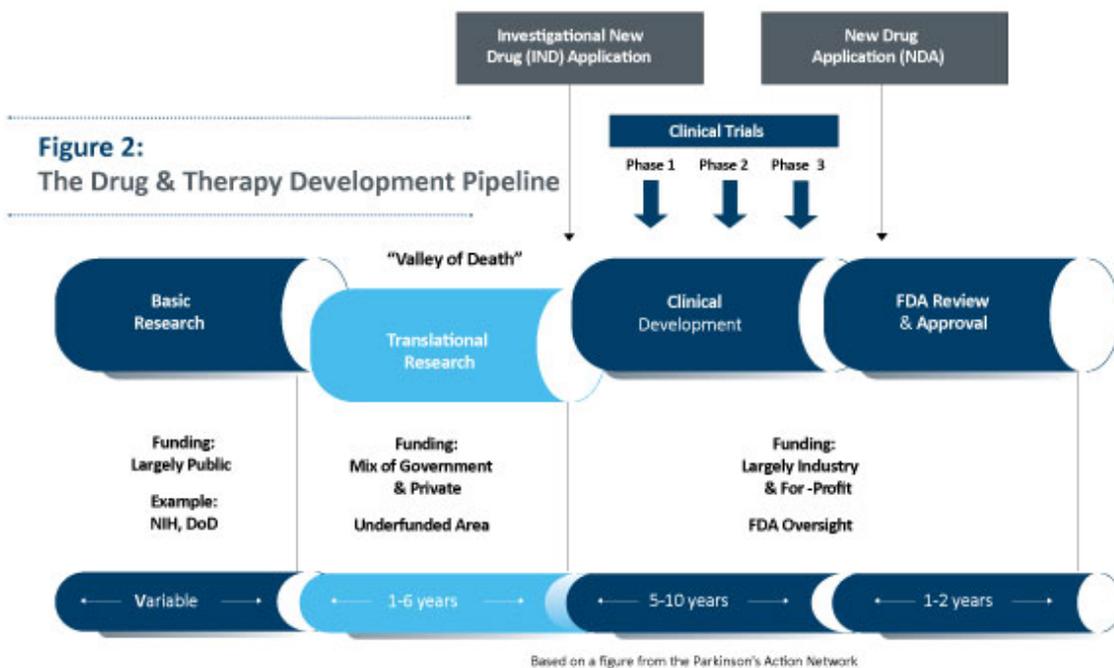
Impacts of Innovation

- Research-related gains in average life expectancy for the period from 1970 to 2000 have an economic value estimated at **\$95 trillion**, about **\$3.2 trillion per year**.¹
- Cancer death rates have been dropping by more than 1 percent annually for the past 15 years.² Each 1 percent reduction in cancer deaths has a present value of nearly **\$500 billion** to current and future generations of Americans. A full cure would be worth approximately **\$50 trillion** — more than three times today's GDP.³
- The NIH alone through its \$32 billion budget supports more than **350,000 U.S. jobs** and **\$60 billion of economic activity** annually.⁴

We know the international landscape is changing – countries are strengthening their life science infrastructure, the U.S. life science workforce is under stress⁵, the biopharmaceutical industry is facing increased scrutiny and the U.S. health-care system is undergoing an evolution (perhaps even a revolution). In the face of these developments, strong leadership and constructive policies will be required from the next president to foster and advance biomedical innovation that benefits all citizens.

Figure 1

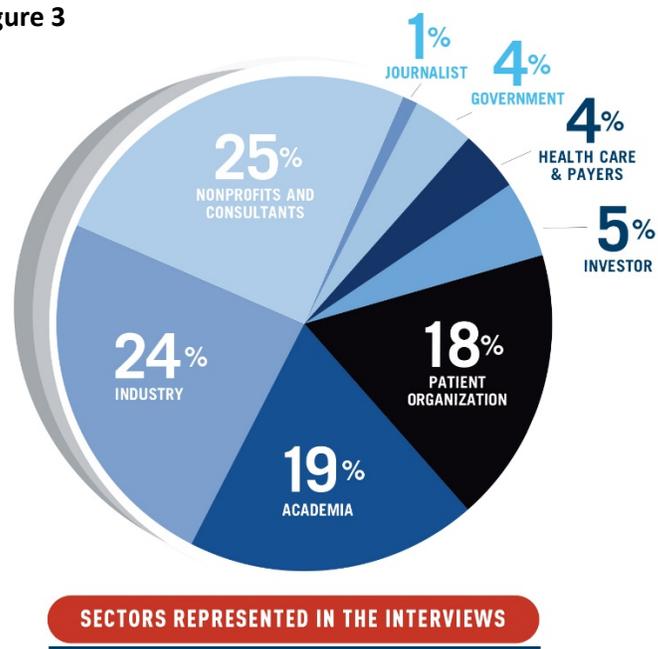




Rx for Innovation: What Did *FasterCures* Do?

FasterCures has a unique network spanning all sectors of the biomedical innovation ecosystem, including representatives from academia, the life sciences industry, government, disease foundations and patient organizations, philanthropic organizations, investors, health-care providers and payers. From July through October 2016, we reached out to this network and interviewed more than 150 thought leaders from all sectors (Figure 3). We also established a portal on our Web site to take in suggestions from anyone who is motivated to help improve the enterprise.⁶ Additionally, in August 2016 we convened a group of 20 policy leaders to present the initial themes we had identified and obtain feedback.

Figure 3



The interviewees included scientists and clinicians (running the gamut from a post-doctoral student, to an oncologist using cutting-edge immunotherapies, to a Nobel laureate), current and former public servants (including three former U.S. Food and Drug Administration commissioners), biotech and pharma executives,

patient advocates, venture philanthropists and more. The full list can be found in Appendix 1. All the interviews were done on a not-for-attribution basis, and an interviewee’s participation in the project does not constitute or imply endorsement of *FasterCures*’ analysis or this report’s recommendations.

We used a brief discussion guide to focus the interviews on key issues related to how the next president can help accelerate the development of new treatments and interventions and also strengthen the overall biomedical innovation system (Appendix 2).

What Did *FasterCures* Learn?

While *FasterCures* did not seek consensus from interviewees, there was substantial agreement that all sectors want to be part of a holistic system. We heard many calls for cross-sector collaboration to promote system-level thinking aimed at achieving the common goal of delivering better interventions, therapies and potentially cures to patients. The 26 recommendations in this report emerged from seven areas:

1. System-level action
2. Patient centricity
3. Regulatory resources
4. Translational science
5. Clinical trials
6. Data
7. Access to innovation

Unifying all of our specific recommendations, there is an opportunity for the next president to catalyze a “Health Citizenship” movement to mobilize citizens – both healthy and not – to engage with the innovation system in new and important ways.

The biomedical innovation system is fueled by people – patients, clinicians, caregivers, researchers, regulators, product developers, investors and many more. In today’s landscape of tablets, smartphones, wearable devices and social media, there are opportunities for clinicians and researchers to engage the citizenry like never before to design treatments and studies that take advantage of 24/7 data collection and connectivity. We observed broad agreement that the more Americans that are contributing to all facets of research and development, the faster the system will be.



As President Trump takes office, it is clearly not an option to ignore the power of biomedical research to the United States and to the world’s future. The public and private facets of the biomedical innovation ecosystem are too intertwined – and the health of the population too central to the health of the country – for the next president to not do everything possible to capitalize on the opportunities that emerged from *FasterCures*’ deep dive into the system.

U.S. Government: Key Players in the Biomedical Innovation System	
National Institutes of Health (NIH)	<ul style="list-style-type: none"> • With a budget of \$32 billion in fiscal year (FY) 2016,⁷ NIH is the world's largest funder of biomedical research. It supports scientists in all 50 states and the District of Columbia. However, flat budgets from FY2003 to FY2015 resulted in NIH losing 22 percent of its purchasing power.⁸ • In addition to funding shortfalls, the research workforce that NIH supports is facing a considerable demographic challenge. The average age when a scientist receives his or her first major grant from NIH (called an R01) has increased from approximately 37 in 1980 to 42 in 2014.⁹ NIH is funding an aging workforce; 10 percent of R01 grants now go to researchers aged 66 and older, while only 2 percent go to those under age 35.¹⁰ For researchers who have gotten their first R01 in recent years, approximately 40 percent never receive a second one.¹¹ • Consider that David Baltimore, one of this project's interviewees, received the Nobel Prize in 1975 at age 37.¹² In today's world, he may not have gotten his first NIH R01 grant for another five years.
Food and Drug Administration (FDA)	<ul style="list-style-type: none"> • FDA regulates 20 cents of every dollar spent by U.S. consumers annually,¹³ but its total FY2016 budget of \$4.8 billion¹⁴ was less than that year's \$5.1 billion operating budget for Montgomery County, Md.,¹⁵ where FDA is headquartered. • FDA reviews large, complex data packages for each new drug, biologic, vaccine and medical device that seeks marketing approval in the U.S. In 2015, the agency approved 45 new drugs¹⁶ and six new recombinant therapies,¹⁷ for a 66-year high.¹⁸ • Yet, the agency is continually being asked to do more with less. The agency's appropriated budget has seen only single-digit percentage increases each year from FY2010 through FY2016.¹⁹ By FY2016, industry-provided user fees had increased to 43 percent of the agency's total budget.²⁰ Combined, these trends result in an agency that has less and less flexibility to allocate its limited resources in the ways necessary to accomplish its vital mission. • At same time, the agency is struggling to maintain its workforce given the complex federal hiring process and fierce competition from industry for highly trained medical product reviewers.²¹ FDA Commissioner Robert Califf has identified strengthening the agency's workforce as his top priority.²²
Centers for Medicare and Medicaid Services (CMS)	<ul style="list-style-type: none"> • As the largest payer of medical products and services in the United States,²³ CMS reimbursement policies have a significant ability to impact the entire biomedical innovation ecosystem. • The agency is currently undergoing a seismic shift to value-based payments for medical services and products. Current goals are for 90

	<p>percent of Medicare fee-for-service payments to be tied to value or quality by 2018.²⁴ This movement toward value-based reimbursement is echoing throughout the entire U.S. system. The growing changes to how drugs and other medical products are paid for will likely require changes in the business models of many components of the biomedical innovation system.</p>
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U.S. Congress: Innovation-related Initiatives	
21 st Century Cures	<ul style="list-style-type: none"> • 21st Century Cures is a bi-partisan initiative in Congress to institute important reforms across the biomedical innovation ecosystem and provide additional resources to federal agencies.²⁵ H.R. 6²⁶ was passed by the House of Representatives in July 2015, and a package of related bills has been debated in the Senate.²⁷ As of this writing in early November 2016, the final legislation is still pending. • While the final legislative package is not set, provisions that are under consideration are designed to enhance patient centricity in regulatory decision-making, data sharing and electronic health record (EHR) interoperability, innovations in clinical trials, and hiring and retention at FDA, among many others.
FDA user fee reauthorization	<ul style="list-style-type: none"> • Most applications submitted to FDA for approval of medical products are charged a so-called “user fee.”²⁸ Currently there are user fee agreements covering the review of drugs, devices, generic drugs and biosimilars. Each agreement is negotiated by FDA and industry every five years but must be enacted into law by Congress through authorizing legislation. The legislative package frequently includes other FDA-related provisions in addition to the user fee agreements. • The current set of user fee agreements expire on Sept. 30, 2017. Negotiations for each user fee agreement have been completed, and commitment letters outlining FDA’s proposed performance goals and procedures for the Prescription Drug User Fee Act (PDUFA), the Medical Device User Fee Agreement (MDUFA), the Generic Drug User Fee Agreement (GDUFA) and the Biosimilar User Fee Act (BsUFA) have been made available for public comment. Each agreement, including any revisions incorporated as part of the public comment period, must be transmitted to Congress by Jan. 15, 2017.²⁹ This means that the next administration will need to work with Congress to pass them into law.

STAGES OF SCIENTIFIC RESEARCH

Basic research aims to advance knowledge, without a specifically envisaged or immediately practical application. Basic research projects aim to improve the scientific theories that explain or inform what may cause, drive and impact a disease.

Translational research is often referred to as bench-to-bedside research, and serves as the bridge between basic and clinical research. Translational research projects apply an iterative and multidirectional process to a) transform basic research discoveries into new drugs, devices and interventions and b) utilize findings from the clinic to inform new research in order to refine or expand an innovation.

Clinical research addresses disease prevention, treatment, diagnosis and relief from disease-related symptoms in human subjects. Clinical research projects focus on the safety and effectiveness of medications, devices, diagnostics and treatment regimens intended for human use.

Source: Milken Institute Center for Strategic Philanthropy

1. System-level Action

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

In our interviews it was abundantly clear that the challenges we face in advancing medical progress are increasingly complex and require ever more collaboration among sectors, including government, academic research institutions, industry, philanthropists, disease foundations and patient groups. It is also clear that the medical innovation ecosystem and the health-care delivery ecosystem – including providers and payers – are increasingly interdependent. Concerns about rising costs are escalating. Improving patient outcomes is becoming even more central to everyone’s interests.

Interviewees expressed a desire for more prioritization and strategic planning about the best use of health resources – not just government resources like NIH, FDA and CMS, but also resources across sectors.

We heard concerns about duplication of effort, lack of attention to best practices and inability to scale or replicate successful programs within and outside of government. We heard a call for more attention to health disparities by income and ethnicity, which impact not only the health of individuals but also our ability to advance innovation.

There is currently no vehicle in the Executive Branch, including the Department of Health and Human Services, 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. While the President’s Council of Advisors on Science and Technology advises the president on issues requiring technical understanding, we see a need for a similar body reflecting the perspectives of all health and biomedical innovation stakeholders.

RECOMMENDATIONS:

- 1.1 Create a President’s Council of Advisors on Health and Innovation within the first 100 days.** The council would include leaders from the entire biomedical research and health-care ecosystem: research institutions, product developers, patients, providers and payers. This council would not set policy but would be a vehicle to promote cross-sector communication and collaboration and to provide insight and advice to the administration on issues that impact the health-care and “cures” enterprises.



"If you were designing government [health programs] from scratch, would it look as it does today?" – Patient Advocate



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

The committee would be convened by the HHS secretary and would inform and be informed by the President's Council of Advisors on Health and Innovation described above.

Membership would consist of senior leadership (and dedicated staff) from the agencies under the department's purview, as well as representatives of other relevant government departments and agencies, such as the Department of Defense (DoD), the Department of Veterans Affairs (VA) and the National Science Foundation. Since the Affordable Care Act became law, HHS has been focused on health-care reform and implementation. However, the department is sitting astride most of the important government resources (*e.g.*, NIH, FDA, CMS) devoted to fostering innovation for future health-care needs. This committee would ensure coordination, collaboration and communication among the agencies regarding important national health and research priorities and the government address them.



"You want change? You have to look at who are the drivers, bring all of them to the table and look at all the intended and unintended consequences." – Health Policy Expert



"Government must be open to partnering on a thought leadership basis, not villainizing private sector." – Industry Executive

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.

Patient centricity has been called the “blockbuster drug of the 21st century.”³⁰ There is growing recognition that integrating patient perspective data into medical product development will enable researchers to develop treatments better suited to patients’ needs, leading to better overall outcomes. At the same time, patients themselves are mobilizing in new and innovative ways to identify and create opportunities to engage with researchers, industry and regulators to ensure that their voices are part of the process. To that end, a new “science of patient input” is emerging, calling on methods from the fields of health economics, outcomes research, epidemiology, social sciences and marketing to accomplish these goals.^{31, 32}



"We need to support the system in a way that brings the patient voices to the fore."
– Academic Researcher

The importance of this issue was reflected in many interviews, as stakeholders representing diverse sectors of the biomedical ecosystem used words like “essential” and “transformational” when describing the potential impact of patient centricity on medical product development. At the same time, there was widespread acknowledgement that the science of patient input will stall without systemic support –“it won’t just happen, it has to be supported.” It is therefore critical to take collective steps to expand the capacity of academia, industry, patient organizations and health-care institutions to advance and apply the science of patient input in a meaningful and effective way.

During the course of our stakeholder interviews, the concept of a new Office of Patient Engagement in HHS was suggested. However, there were voices both for and against this idea. Although such an office could serve to empower and elevate patient engagement, many cited concerns, and we at *FasterCures* agree, that creating such an office risks delegating patient engagement to an isolated role that is disconnected from the everyday work of the agencies. It could make it far too easy to see patient engagement as a separate work stream when the end goal is to integrate patient engagement efforts into all levels of the biomedical development and health-care delivery ecosystem. The following recommendations are intended to further this integration while avoiding the creation of artificial boundaries.



"How could patient engagement be harnessed as a force for innovation?" – Health Policy Expert

RECOMMENDATIONS

2.1 Convene a health citizenship summit within the first year.

Many stakeholders acknowledged that to continue to advance patient engagement there must be a more coordinated and purposeful approach. To that end, the White House should host a “Health Citizenship Summit” by the end of 2017, which would bring together thought leaders from NIH, FDA, the Centers for Disease Control and Prevention (CDC), CMS, Health Resources and Services Administration, Agency for Healthcare Research and Quality, HHS headquarters and other agencies such as DoD and VA. External stakeholders representing each sector of the biomedical ecosystem, including patients, payers, providers, academia and industry would also be invited to participate. The summit would serve to:

- Create an opportunity for each agency to demonstrate how it is currently integrating patient engagement into its mission area and using it to inform decision-making, and for industry, academia and patient organizations to describe their efforts and learn how best to connect with their federal partners.
- Enable the public and private sectors to collaboratively brainstorm opportunities to mature the science of patient input and make joint commitments to continue engagement efforts at all levels, with the shared goal of a biomedical ecosystem that better aligns with patients’ needs and produces better health outcomes. This approach to obtain private sector commitments has been used successfully by the Cancer Moonshot and the Precision Medicine Initiative (PMI).
- Facilitate opportunities to translate learnings from ongoing work to integrate the science of patient input into medical product development and regulatory decision-making, and into efforts to assess the value of medical treatments.



"We need to get the right therapy, to the right patient, at the right time." – Academic Researcher

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

Numerous entities are eager to increase patient engagement in medical product development, yet regulatory uncertainty may be delaying or stalling progress. The FDA’s Center for Drug Evaluation and Research (CDER) and Center for Devices and Radiological Health (CDRH) have launched initiatives aimed at advancing the science of patient input that must be further



supported and fostered to realize maximum potential. Efforts should also be made to encourage and enable FDA to ensure consistency across all of its centers with respect to patient centricity. Different approaches by centers for drugs, devices and biologics will only serve to further complicate a challenging landscape for the patients who wish to engage in this aspect of medical product development.

- Existing initiatives in CDER include the Patient-Focused Drug Development program launched under PDUFA V and proposals to expand upon these efforts in PDUFA VI. Specifically, the PDUFA VI commitment letter³³ outlines a series of guidance documents that are critical to advancing the capabilities of all stakeholders working in this field. Accordingly, as described elsewhere in this report, supporting timely re-authorization of PDUFA VI will help FDA, industry, patients and others understand and execute methods to collect and provide patient input. Specifically, the planned guidance documents will:
 - provide guidance outlining approaches to collect comprehensive and representative patient and caregiver data,
 - identify processes and approaches to determine what impacts are most important to patients,
 - provide guidance on approaches to identify and develop measures of disease impact to facilitate meaningful patient input in clinical trials and
 - update guidance on patient-reported outcome measures and address methods to better incorporate clinical outcome assessments into endpoints.
- CDRH has issued final guidance on the use of patient preference information when seeking regulatory approvals of medical devices.³⁴ CDRH has also cited promoting “a culture of meaningful patient engagement,” as one of its top strategic priorities for 2016-2017. The proposed MDUFA IV commitment letter also includes a series of provisions dedicated to “advance patient input and involvement in the regulatory process.”³⁵ To ensure that these initiatives succeed, CDRH must have necessary resources and support to further strengthen and build upon these important efforts. Timely reauthorization of MDUFA IV is therefore critical.



"We have everything we need to complete a revolution in R&D, and the revolution will be driven by patients." – Biomedical Research Expert

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

There has been considerable focus on how best to promote the regulatory science critical to FDA’s mission;³⁶ however, in interview after interview, stakeholders expressed concern that the agency still lacks the necessary tools, funding and mandate. The Joint Leadership Council³⁷ needs to be reinvigorated, and FDA and NIH resources need to be put towards specific important regulatory science topics. For example, a new “Science of Patient Input” program should be established at NIH that would support the scientific underpinnings of patient input. This approach would leverage NIH’s existing funding, as well as its scientific and grant-making infrastructure. However, funding decisions would

be jointly approved by NIH and FDA to ensure alignment with the missions of both agencies.

2.4 Incorporate throughout all NIH-supported research the core principle from PMI’s *All of Us* program, that research studies enroll “participants,” not “subjects.”

Through the *All of Us* study, NIH will engage at least 1 million volunteers to participate in a collaborative, longitudinal research effort to improve our understanding of disease and develop more precise medical treatments.³⁸ White House and NIH leaders have consistently and intentionally used the term “participants” rather than “subjects” when describing the people who would be part of the study. This decision acknowledged the critical importance of building a partnership of trust and respect with study participants, especially those from populations typically underrepresented in biomedical research.³⁹ As part of this effort, NIH created the Participant Technologies Center to design engagement policies and technologies to effectively recruit and engage participants throughout the life of the project.⁴⁰ This philosophy, and the supporting policies and technologies, should be replicated throughout the biomedical research enterprise.



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

The Patient-Centered Outcomes Research Institute (PCORI) was authorized by the Affordable Care Act of 2010 as a nonprofit, nongovernmental organization with a focus on supporting research that includes authentic involvement of patients and other stakeholders.⁴¹ Since its inception, PCORI has supported a substantial amount of patient-centered research, including the creation of PCORNet, a collaboration of clinical research centers and patient organizations “to transform the culture of research from one directed by researchers to one driven by the needs of patients and other healthcare stakeholders.⁴²” Many of the stakeholders we spoke with acknowledged the importance of PCORNet to highlighting the value of patient centricity and promoting the science of patient input.



“It’s not just having a patient at the table but understanding their value and why they are there.” – Patient Advocate

3. Regulatory Resources

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

The FDA is responsible for “protecting the public health by ensuring the safety, efficacy, and security of human and veterinary drugs, biological products, and medical devices; and by ensuring the safety of our nation's food supply, cosmetics, and products that emit radiation...[and for] helping to speed innovations” in these products.⁴³ When considering its entire mission, not just medical products, FDA-regulated products account for about 20 cents of every dollar spent by American consumers (seven of those cents are for medical products).⁴⁴ However, the agency is continually strapped for financial resources and human capital. Indeed, FDA’s total FY2016 budget of \$4.8 billion⁴⁵ was less than that year’s \$5.1 billion operating budget for Montgomery County, Md.,⁴⁶ where FDA is headquartered.⁴⁷



“There’s still a resource issue [at FDA] – it’s difficult for them to get ahead, not enough [funding] in their budget.” – Health Policy Expert

To ensure that drugs are safe and effective, FDA reviews large and complex data packages for each new drug, biologic, vaccine and medical device that seeks marketing approval in the United States, and also engages in extensive post-marketing surveillance efforts. In 2015, the agency approved 45 new drugs⁴⁸ and six new recombinant therapies,⁴⁹ combining for a 66-year high. **During our interviews, stakeholders from across the system expressed that the agency and its leadership are on the right path as they work to improve the efficiency of medical product reviews, engage with patients, increase overall transparency and ultimately approve safe and effective products.**

Interviews surfaced a variety of views on the location of FDA within the federal infrastructure and specifically whether FDA should remain within HHS or become an independent regulatory agency. While some were supportive of this idea, others expressed reservations. Any such change should therefore not proceed without input from the broader community to determine the impact of separating FDA from HHS at a time when the biomedical innovation system is seeking collaboration, including among FDA and other HHS agencies such as NIH and CMS. The recommendations we put forward here are focused on more targeted steps the next president can take to support the FDA and ensure it is well-resourced to carry out its important public health mission.

RECOMMENDATIONS:

3.1 Nominate an FDA commissioner within the first 100 days.

The FDA is faced with challenging decisions that affect the public health of all Americans on a daily basis. Strong, uninterrupted leadership from a confirmed commissioner is essential to accomplishing FDA’s mission and to strengthening and expanding upon an array of initiatives, such as advancing patient engagement and ensuring a qualified and

robust workforce. The current commissioner, Robert Califf, has precisely the leadership skills and technical expertise that the agency needs, and *FasterCures* would support his continuation in this mission-critical role.⁵⁰



“FDA is good at developing faster reviews, but accelerating clinical research is hard.” – Investor

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

We recommend *at least* a 7 percent annual increase for FDA’s appropriated budget. The agency is continually being asked to do more with less. The agency’s appropriated budget (*i.e.*, not including user fees) has seen only single-digit percentage increases each year from FY2010 through FY2016.⁵¹ Indeed, the FY2017 budget request sought only a 0.5 percent increase over FY2016. By FY2016, industry-provided user fees had increased to 43 percent of the agency’s total budget.⁵² Combined, these trends result in an agency that has less and less flexibility to allocate its limited resources in the ways necessary to accomplish its vital mission of regulating drugs, food and medical devices to protect the public health.

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.

With 711 vacancies out of 5,525 positions at FDA’s Center for Drug Evaluation and Research alone,⁵³ there is a critical need to improve FDA’s ability to hire and retain qualified professionals. The agency recruits staff from a very small pool of highly skilled experts, competing with industry to attract top talent.⁵⁴ Full utilization of existing hiring and pay authorities, coupled with the introduction of new ones tailored to FDA such as H.R. 6⁵⁵ and S. 2700⁵⁶ being considered by the 114th Congress, will help the agency better compete with the biopharmaceutical industry to attract hire, and retain well-qualified technical staff. Additionally, *FasterCures* recommends that the next administration revise Office of Management and Budget memorandum M-12-12 to allow for FDA staff to travel to and attend scientific conferences and other external technical training. Such training is vital to ensure FDA’s fluency in cutting-edge science and is also an important tool for retaining top staff.

3.4 Support the timely reauthorization of PDUFA and MDUFA.

Throughout our interviews, we heard from many stakeholders about the importance of ensuring a timely reauthorization of the prescription drug (PDUFA⁵⁷) and medical device (MDUFA⁵⁸) user fee agreements that will expire on Sept. 30, 2017. The authorizing legislation enacting the user fee agreements must be signed into law by mid-summer to ensure the FDA can continue to function without disruption. PDUFA was first enacted in 1992, and MDUFA was enacted in 2002. Both have been reauthorized every five years. However, this is the first time that all the medical user fee agreements (including BsUFA and GDUFA) will be negotiated under one administration and then passed into law during a different administration. Both the PDUFA and MDUFA commitment letters reflect careful, year-long negotiations between industry and FDA, as well as input from

the stakeholder community. In addition to including many important resources to support medical product review, the proposed commitment letters for each of these user fee agreements contain resources specifically dedicated to enhancing the integration of patient perspectives into regulatory decision-making and strengthening the agency's workforce. PDUFA, in particular, also outlines specific FDA hiring and retention goals that will be supported through user fees. We recommend that the next president support the timely reauthorization of the user fee agreements.



"We want an empowered, science-based agency." – Industry Executive

4. Translational Science

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

Basic biomedical research provides the fuel for new disease therapies and cures, and *FasterCures* has always been a strong supporter of basic science funding. However, it is only part of the puzzle when it comes to delivering new medical products to patients. The need for more and better translational research is also a key component of our work. Eighty to ninety percent of research projects fail before they ever get tested in humans.⁵⁹ And fewer than 12 percent of compounds that make it into clinical trials prove their safety and efficacy and are approved by the FDA.⁶⁰ The lack of funding, technical expertise and incentives – as well as the high risk of failure – for the important steps of translational science needed to turn a promising basic research insight into a therapeutic that can change the course of a disease has been a major impediment to “faster cures.” It is a “Valley of Death” that can slow or even stop the progress of good ideas (Figure 2).



It's not trivial to take ideas from laboratory into the clinic. – Health Policy Expert

We have produced reports defining the challenges⁶¹ we must overcome in the translational research process and highlighting models of collaboration⁶² that can assist the early stages of product development. We advocated for the creation of NIH’s National Center for Advancing Translational Sciences (NCATS), the mission of which is “to transform the translational science process ... to develop innovations to reduce, remove or bypass costly and time-consuming bottlenecks in the translational research pipeline.”

Interviewees agreed that NIH’s budget must have robust annual increases. Flat budgets from FY2003 to FY2015 resulted in NIH losing 22 percent of its purchasing power.⁶³ In FY2016, the agency received a \$2.1 billion (6.6 percent) increase,⁶⁴ and this trend must continue. However, there is debate among members of the biomedical innovation system about the right balance of federal investment in translational versus basic science. Approximately 52 percent of NIH’s research budget is directed at basic science, and the ratio of basic to applied has stayed constant in recent years.⁶⁵ **In our interviews, we heard a diversity of views. Some felt that NIH should focus on supporting basic science in academia, and that academic scientists aren’t well positioned to do translational science and product development. We also heard from many interviewees, however, that government does have an important role to play in helping to ensure that the research taxpayers fund has the greatest chance of benefiting patients, by working to “de-risk” the early stages of development and moving promising ideas to a “proof of concept” that will motivate the biopharmaceutical industry and private capital to invest. It is *FasterCures’* view that we must do both.**

There are two general ways in which the federal government can support translational science. First, it can develop methods, tools, infrastructure and resources that will benefit whole field of research, shrink the time and cost of the preclinical and early-clinical stages of clinical development, and provide platforms on which individual products can be built. Second, government can create financing mechanisms that will help incentivize translational development of high-need treatments where the commercial incentives are weak.

RECOMMENDATIONS:

4.1 Maintain strong support for NCATS and other NIH and U.S. government initiatives aimed at building tools and expertise to bridge the translation gap.

While an appropriate balance with supporting continued basic scientific inquiry must be maintained, the administration should recognize the important role government has in making sure promising science does not sit on the shelf.

- NIH should adopt the recommendations of the National Academies' 2013 review of the Clinical and Translational Science Awards (CTSA⁶⁶) program⁶⁷, particularly those related to strengthening NCATS' leadership of the program, building on the strengths of individual CTSAAs and formalizing and standardizing evaluation processes.
- NIH programs such as the Centers for Accelerated Innovations and Research Evaluation and Commercialization Hubs⁶⁸ are providing translational expertise and resources to promising NIH-funded investigators. Outside of NIH, programs such as the National Science Foundation's I-Corps⁶⁹ are taking similar approaches. These programs should be scaled and replicated across all the NIH institutes and centers.

4.2 Make life sciences a greater focus for DARPA.

The Defense Advanced Research Projects Agency (DARPA) has played a unique role in technology development since its creation after Sputnik's launch. Arguably, the major contributor to its success is its culture of risk tolerance, mission focus and active program management. To harness that culture for biomedical innovation, the next president could consider expanding funding for DARPA's Biological Technologies Office⁷⁰ and link that funding to partnerships with NIH such as those already occurring around the Brain Research through Advancing Innovative Neurotechnologies (BRAIN) initiative.⁷¹



"If we're going to bend the cost curve of developing new drugs, we need to understand the process of translation as a predictive science, not an experimental exercise. We have to decrease the cost of production. But we don't understand the 'physics' of translation." – Biomedical Research Expert

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.

Within its first year, the administration should task the Office of Science and Technology Policy with bringing together federal science and health agencies, the Department of the Treasury and other government experts to work with relevant private-sector partners to propose a novel financing mechanism, such as a co-investment fund or a bond, that would allow the government to incentivize translation and commercialization of potentially transformative ideas that need de-risking. *FasterCures* has made a detailed proposal for one such tool,⁷² and other examples include the Megafund,⁷³ the UK-based Dementia Discovery Fund,⁷⁴ the Israel Life Sciences Fund and the Alzheimer's research bonds being proposed by New York⁷⁵ and other states. Developing a more robust understanding of the unique nature of risk in biomedical research portfolios and how to mitigate it will be a critical first step to ensure that whatever tools are proposed will be appropriate to the task.

4.4 Maximize use of NIH's DARPA-like Cures Acceleration Network.

In 2010, Congress created the Cures Acceleration Network (CAN),⁷⁶ now housed within NCATS, which is authorized to invest up to \$500 million annually to advance the development of "high-need cures" and reduce significant barriers between research discovery and clinical trials. So far there has never been more than \$25 million appropriated for CAN.⁷⁷ CAN was also given flexibility in its funding mechanism, known as "other transactions authority" (OTA)⁷⁸ to provide the flexibility needed to support cutting-edge research in the private sector. CAN awards are intended to more closely resemble the directed and actively managed projects at the widely praised DARPA. However, only 20 percent of CAN's funding can be deployed this way.⁷⁹ The administration should seek to increase CAN's budget and raise the cap on use of OTA to encourage more innovation in high-need areas.

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 infrastructure that underlies the development, evaluation and regulatory approval of all medical products. They are also the longest and most expensive phase of medical product development^{80, 81}. Without clinical trials, we would never know if a medical product is safe and effective. **In the course of our interviews, we found widespread dissatisfaction with the status quo and a true sense that now was the time to make changes in the process.**

With the emergence of innovative tools like real-world evidence⁸², telemedicine, adaptive trial designs⁸³, mobile health apps and more, the next president has the opportunity to promote a revolution in technologies and methodologies that can lower costs and shorten timelines of biomedical innovation. But government can't do this on its own. Fortunately, these important challenges are already being tackled in various ways at places such as the private-sector collaboration TransCelerate⁸⁴ and the public-private Clinical Trials Transformation Initiative.⁸⁵

Enabling the FDA to maintain strong standards for safety and efficacy, while accelerating clinical development, will require intense collaboration among all stakeholders and innovation in the design of a new generation of clinical trials. However, like bridges, water systems and other traditional infrastructure, trials themselves are frequently seen as a means to an end and therefore do not receive the focused attention and investment needed to keep them strong and make needed improvements.

RECOMMENDATIONS:

5.1 Establish a White House “Clinical Trial Task Force”

The Clinical Trial Task Force (CTTF) would focus federal activity on advancing the tools, technologies and methodologies that underpin this essential piece of infrastructure and provide a platform for government, academia and industry to facilitate collaboration across sectors.

- CTTF could be chaired by the director of the Office of Science and Technology Policy and include all relevant cabinet agencies (e.g., HHS, DOD and VA). The FDA-NIH Joint Leadership Council⁸⁶ could serve as the executive secretariat, highlighting the connection between NIH's clinical research and FDA's regulatory decision-making.



“With clinical trials research we need a better infrastructure, more trained people... I would start by convening a blue ribbon panel... You really want the best.” – Health Policy Expert



- The task force would initially convene a Blue Ribbon Panel to establish a prioritized list of actions including scientific projects, policy updates, funding opportunities (from both public and private sources) and the role of health citizenship in accelerating this crucial component of health infrastructure. Blue Ribbon Panel members should come from government, industry, academia and the patient community.
- CTF would use the panel’s prioritized list to drive activities across the federal government and in partnership with academia and the private sector.



“There needs to be a more reliable way to assess efficacy and safety, using observational and real-world evidence.” – Industry Leader

5.2 Strengthen ClinicalTrials.gov by enforcing current law⁸⁷ requiring data submission and by investing in the platform to improve the user experience for all the system’s stakeholders.

There has been widespread lack of compliance with, and enforcement of, existing law requiring submission of data to ClinicalTrials.gov by both companies and universities.^{88, 89} The government recently issued updated regulations reaffirming and clarifying reporting requirements and exemptions, as well as penalties for non-compliance. *FasterCures* supports and encourages a more focused commitment of resources to enforce these requirements, as well as strengthen the penalties if necessary.^{90, 91} With this new influx of data, ClinicalTrials.gov will need to address long-standing critiques to ensure it is user-friendly for the entire ecosystem, including academia, industry, patient organizations and individual citizens.



5.3 Support and strengthen efforts to increase enrollment in clinical trials.

Perhaps the most persistent obstacle to successful completion of clinical trials is recruitment. Studies have found that as few as 3 percent of adult cancer patients participate in clinical trials.⁹² While most trials ultimately enroll enough participants, it can take twice as long as estimated, and as many as 48 percent of the individual sites in a trial under-enroll study volunteers.⁹³ These recruitment challenges not only make it difficult to launch and maintain trials but the resulting delay also adds significant expense to medical product development.

- The White House, working with HHS, should develop an outreach and social media campaign – perhaps using #HealthCitizenship as a tool – to educate and galvanize the public to be active participants in the biomedical research and development process.
- NIH and other government sponsors of research should embrace, model and, where appropriate, incentivize the recommendations outlined in the Clinical Trials Transformation Initiative’s report on “Efficient and Effective Clinical Trial



Recruitment Planning.⁹⁴ These recommendations guide clinical trial developers to incorporate input and reflect the needs of all stakeholders involved in the study, not just those of the researchers, which in turn will lead to improved recruitment, retention and results.

- Apply learnings from ongoing efforts in NIH’s Precision Medicine Initiative *All of Us* research cohort to identify and develop methods to more effectively engage participants, especially those from underserved or hard-to-reach communities in clinical research.

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

These two “parallel universes” of data are defined by different software platforms, regulations and methodologies for collection and analysis.^{95, 96} Uniform data standards and clinical data elements will be an important first step to linking these two “universes.” This is a key roadblock, and the initiative must be provided with sufficient staff and funding necessary to achieve success. Once this barrier is addressed, clinical trial innovations like real-world evidence will be able to realize their potential to accelerate the development of new therapies and cures.

6. Data

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

Massive volumes of data are generated as part of biomedical research and everyday clinical care. While data have always driven biomedical innovation, the development of the internet and big data tools provide new opportunities to collaborate, analyze and accelerate progress. However, we won't be able to fully benefit from these data until they are integrated into a seamless system, instead of a multitude of silos. **A desire for meaningful interoperability among all health information technology (IT) systems and also patient empowerment regarding their own data were key themes that emerged from our stakeholder interviews.**

FasterCures has long been focused on how health IT can benefit both clinical care and biomedical research. In 2005 and 2011, we published reports with recommendations for integrating research needs into the health IT system to enhance clinical care and development of more effective interventions, therapies and cures.^{97, 98} The Office of the National Coordinator for Health IT (ONC) has presented its vision for enabling connectivity between the clinical care and research data systems to be incorporated into health IT by 2024.⁹⁹ The system needs to start building this infrastructure now. There is no time to lose!

The federal government made major investments in the expansion of health IT and data infrastructure projects over the last decade, including \$35 billion in electronic health record system incentive payments¹⁰⁰ alone. The HITECH Act¹⁰¹ has prompted hospital adoption of basic EHRs to rise from fewer than 10 percent in 2008 to more than 80 percent in 2015.¹⁰² Data infrastructure has been a major focal point of several marquee federal research initiatives including the Cancer Moonshot, the BRAIN Initiative and the Precision Medicine Initiative's *All of Us* research cohort. We must not let these investments go to waste but continue to build upon and improve upon this important work.

If research systems are to harness data within and across the health-care enterprise, there will need to be agreement on the standards and policies necessary for interoperable data exchange. When achieved, interoperability will allow data to move seamlessly between, and be understood by, IT systems and mobile devices at different hospitals, research centers, doctors' offices and in patients' homes. Standards that incorporate the needs of both clinical care and biomedical research would streamline data interchange between electronic source data in clinical EHRs and research-oriented IT systems (e.g., registries, distributed research networks, public health databases). This, in turn, would



"It's not the government's job to own or hoard data; they need to release it back out into the system." – Biomedical Research Expert



"We need to know how the FDA is going to use all the data that are coming from patients – what's meaningful and valuable to them?" – Patient Advocate

improve collaboration across the system and streamline regulatory submissions for new medical products.

As the worlds of biomedical research and clinical care come together, the patient will be at the center. There was tremendous interviewee excitement for the ways in which digital technologies and initiatives like PMI will allow patients to engage with their health data in new ways. There is ongoing discourse on providing patients with more control over their health data, but that doesn't mean that patients will lock away their data. In one study, more than 70 percent of patients were willing to share their personal health information so researchers can better understand diseases and develop new ways to prevent, treat and cure them.¹⁰³



“Our data are not integrated! We can't build on the success or knowledge of the work that came before us.” – Patient Advocate

RECOMMENDATIONS:

6.1 Enable interoperability by strengthening enforcement tools and requiring that open, non-proprietary APIs be built into health IT systems.

Now that EHR adoption is widespread, the government must ensure that meaningful interoperability follows. ONC has obtained “interoperability pledges” from major health systems and health IT vendors.¹⁰⁴ ONC's Sync for Science¹⁰⁵ and Blue Button¹⁰⁶ efforts also promote interoperability and movement of data. However, government needs tools to effectively enforce these promises. If 21st Century Cures does not become law before Inauguration Day, the next president should support legislation similar to the 21st Century Cures provisions that would prevent health data blocking and empower HHS to investigate and seek civil monetary penalties from offenders (H.R. 6¹⁰⁷ and S. 2511¹⁰⁸ in the 114th Congress). In addition to new enforcement tools, requiring open and non-proprietary application programming interfaces (APIs) for ONC certification of health IT products¹⁰⁹ could be a technical solution that would allow health IT users – clinicians, researchers, entrepreneurs and patients – to innovate and easily bring disconnected data together to meaningfully improve health care and biomedical research.

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.

Data scientists are perhaps the most highly sought after technical experts today – across all sectors of the economy. To maximize the appeal to top data scientists who have many options outside of health and especially outside of government service, the fellowship should be run out of the White House. We recommend that it be a dedicated sub-program of the U.S. Digital Service. Fellows would spend one or two years tackling data challenges pertinent to medical research and health across the federal government. Participating agencies would include FDA, NIH, CMS, CDC, VA and DoD.



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

Identifying appropriate patients and recruiting them into a clinical trial is a key bottleneck for biomedical innovation. Studies have found that as few as 3 percent of adult cancer patients participate in clinical trials.¹¹⁰ Today, EHRs used for routine clinical care typically do not have functionality that supports matching patients to appropriate clinical trials that are being run by academia and industry. Adding this functionality to the requirements for ONC certification of health IT products, and therefore a requirement for Meaningful Use EHR incentive payments,¹¹¹ will ensure it is a priority for IT product developers. Current progress has been made on an ad hoc basis as individual medical centers seek to incorporate clinical trial matching into their existing EHRs.¹¹² Best practices for federal requirements can be derived from these trailblazers and from engagement with the patient community.



*“We need to fight for open data.” –
Biomedical
Research Expert*

6.4 Ensure that federally supported biomedical research data are not hoarded and that the data repositories being developed as part of studies will enable data sharing and interoperability.

Expanding research efforts, integration of EHRs and patient-generated data all need a robust infrastructure to curate and share these data. There is the danger that these multiplying data repositories will become disconnected “silos of excellence.” Innovation will be accelerated by creating an interconnected system. NIH could take the lead by requiring that the data repositories being built for federal initiatives such as the Cancer Moonshot and PMI’s *All of Us* study use common data standards and architectures that enable connectivity, perhaps by leveraging existing repositories like the Database of Genotypes and Phenotypes.¹¹³ Additionally, NIH could explore how best to revise its grant-making policies to incentivize (or perhaps require) grant recipients to make their data outputs publicly available to the entire biomedical ecosystem, including patients. For example, the degree to which a researcher commits to open data principles in their NIH grant application could be made part of the application’s score and/or an application that commits to such principles could receive a more forgiving pay-line.

7. Access to Innovation

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

Access to treatment threatens to become a new “Valley of Death” for medical innovations. There has been considerable public and private investment in translational science to overcome the “Valley of Death” that results when promising basic research discoveries aren’t able to transition into clinical development.¹¹⁴ Based on *FasterCures*’ previous work¹¹⁵ and on the interviews done for this project, 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. As the debate over drug pricing intensified in 2015 and 2016, we witnessed a disturbing trend of different sectors retreating into their corners rather than coming together to tackle this complex issue. At *FasterCures*, we support policies that foster innovation for the ultimate benefit of patients. That said, the most innovative treatment on the market provides absolutely no benefit to the patient who can’t afford to pay for it. We did not set out in these interviews to find a solution to drug pricing issues. However, what did emerge from our conversations is a need for all sectors to have open and honest dialogue about how to price and pay for cures. *FasterCures* is committed to working with all members of the system to develop a solution.

FasterCures believes the transition of the health-care system (including CMS) to value- and quality-based methodologies for reimbursing medical products and services will help realign incentives to get “the right therapy, to the right patient, at the right time,” as one of our interviewees said. However, there is still uncertainty as to how best to pay for therapies and cures in a value-based way. Resolving this uncertainty will help ensure that both individuals and the system as a whole pay for what works. Moreover, a value-based reimbursement system will serve to incentivize development of products that truly add value.

Private-sector payers and biopharmaceutical companies have begun to experiment with solutions that improve access to necessary information and remove barriers to value-based payment.¹¹⁶ CMS has also indicated that it would like to start piloting value-based purchasing of certain drugs.¹¹⁷ A number of organizations have begun working on value frameworks to inform drug pricing and other aspects of the innovation system (e.g., the American Society for Clinical Oncology,¹¹⁸ the Institute for Clinical and Economic Review,¹¹⁹ the National Comprehensive Cancer Network¹²⁰ and the Memorial Sloan Kettering Cancer Center¹²¹). *FasterCures* is collaborating with Avalere Health to develop a value framework that is informed by and incorporates the patient perspective.¹²²



“Access is the next ‘Valley of Death’... You’ve got the drug approved but no one is going to pay for it.” – Health Policy Expert



“How do we make incentives that reward true innovation and significant advancement in treatment?” – Payer

A concerted effort involving all stakeholders (including patients) will be required to build frameworks and other tools that effectively inform new reimbursement policies so that innovation is nurtured, analyzed and incorporated as a key goal. Intense listening and engagement will be required on all sides so that patients can continue to access innovative therapies and cures in a manner that is sustainable for both individual patients and for the system as a whole. The following recommendations are focused on actions that the government can take, understanding that private payers will also have a critical role to play. Ultimately, *FasterCures*, the leaders we interviewed and the entire system want patients to have access to the medical products they need, in manner that is affordable and sustainable.

RECOMMENDATIONS:

7.1 Initiate a program at CMS by the end of 2017 where its staff engage directly with patient communities.

As CMS transitions to value-based purchasing of drugs and other medical products, CMS should understand patient perspectives on value with respect to their disease state, treatment options and the reimbursement landscape.

- CMS could be guided by FDA’s embrace of patient-engagement as a core component of its regulatory decision-making process. The Patient-Focused Drug Development program has convened Voice of the Patient meetings where FDA staff have engaged directly with the patient communities from more than 20 diseases.¹²³ Meetings like these are just the start of a meaningful patient-engagement process, but would help ensure that CMS is fully informed and transparent as it develops its value-based coverage and reimbursement policies.



7.2 Establish a working group at the Department of Health and Human Services to identify access “Grand Challenges.”

These challenges would, when solved, improve value and coverage decision-making with respect to new medical products, enhancing access through improved patient-centricity, transparency and efficiency.

- Challenges could be research topics, tools, frameworks or other advances that could be applied to value determinations in either public- or private-sector payers.
- Challenges could be solicited from the entire system: government and private health-care payers, medical providers, patient organizations, health policy experts, academic researchers and the biopharmaceutical industry.
- The HHS working group would then prioritize the challenges and make recommendations to the HHS secretary as to how resources could be best deployed to solve them.

7.3 Explore expanding FDA-CMS Parallel Review

Numerous stakeholders expressed concerns about the length of time it can take after FDA approval for payers, such as CMS, to evaluate whether and how to cover a product. This can significantly delay patient access to new treatments.

- FDA and CMS have recently concluded a successful pilot program (which some interviewees viewed as an excellent model) in which they reviewed new medical device applications in parallel, enabling CMS' coverage decision to come more quickly after FDA's regulatory decision. (The program is now permanent.¹²⁴) The administration should explore expanding this approach to drugs. Some interviewees did express concern that the approach may not be transferable from devices given technical differences in drug coverage determination processes. Any expansion must be accompanied by increased resources for both agencies so they can effectively execute the program.



“Who wouldn’t want every patient in America to be getting cutting-edge care [and] access to [clinical] trials? It helps everybody.” – Academic Researcher

Appendix 1: Interviewees

FasterCures thanks all the thought leaders who took the time to speak with us for this project. All interviews were conducted on a not-for-attribution basis. Agreement to be interviewed and acknowledgement here does not constitute endorsement of the report contents. Titles and affiliations were accurate at the time of the interviews, August through October 2016.

Amy Abernathy
Chief Medical Officer and Senior Vice President of Oncology
Flatiron Health

Jeff Allen
President and CEO
Friends of Cancer Research

Clay Alspach
Principal
Leavitt Partners

Aparna Higgins
Senior Vice President, Private Market Innovations and Center for Policy and Research
America's Health Insurance Plans

Christopher Austin
Director, National Center for Advancing Translational Sciences
National Institutes of Health

Peter Bach
Director of Center for Health Policy and Outcomes
Memorial Sloan Kettering Cancer Center

Verne Backus
President
Occupational Consultations

David Baltimore
President Emeritus and Robert Andrews Millikan Professor of Biology
California Institute of Technology

Anna Barker
Professor and Director, Transformative Healthcare Networks; Co-Director, Complex Adaptive Systems Network
Arizona State University

Anthony Barrueta
Senior Vice President of Government Relations
Kaiser Foundation Health Plan, Inc.

Ronald Bartek
President and Co-Founder
Friedreich's Ataxia Research Alliance

David Beier
Managing Director
Bay City Capital

Cynthia Bens
Vice President, Public Policy
Alliance for Aging Research

Gordon Bernard
Associate Vice-Chancellor for Research and Melinda Owen Bass Professor of Medicine
Vanderbilt University

Aron Betru
Managing Director, Center for Financial Markets
Milken Institute

Ann Bonham

Marc Boutin
CEO
National Health Council

John Bridges
Associate Professor
Johns Hopkins Bloomberg School of Public Health

Joel Brill
Chief Medical Officer
Predictive Health

Tricia Brooks
Senior Director
Novo Nordisk A/S

Nancy Brown
CEO
American Heart Association

Jennifer Bryant
Senior Vice President, Policy and Research
PhRMA

Craig Burns
Vice President, The Center for Policy and Research
America's Health Insurance Plans

Atul Butte
Professor of Pediatrics
University of California, San Francisco

Eric Cantor
Vice Chairman and Managing Director
Moelis & Company

Tanisha Carino
Vice President, U.S. Public Policy
GlaxoSmithKline

Maria Carrillo
Chief Science Officer
Alzheimer's Association

R. Alta Charo
Warren P. Knowles Professor of Law and Bioethics
University of Wisconsin Law School

Bill Chin
Chief Medical Officer and Executive Vice President, Science and Regulatory Affairs
PhRMA

Aneesh Chopra
President
NavHealth

Ron Cohen
President and CEO
Acorda Therapeutics

Tony Coles
Chairman and CEO
Yumanity

Robert Cook-Deegan
Professor, School for the Future of Innovation in Society
Arizona State University

Noah Craft
Co-Founder and CEO
Science 37

Jason Cross
Chief Innovation Officer
Foundation for Commercializing Innovation

Gregory Daniel
Deputy Director
Duke-Margolis Center for Health Policy

Deanna Darlington
Director, Patient Advocacy and Allied Development
Amgen

Alex De Winter
Director, Healthcare Ventures
General Electric

Kara Dennis
Managing Director, Mobile Health
Medidata Solutions

Mary Dwight
Senior Vice President for Policy and Patient Assistance Programs
Cystic Fibrosis Foundation

Kafui Dzirasa
Assistant Professor of Psychiatry and Behavioral Sciences
Duke Institute for Brain Sciences

Cartier Escham
Executive Vice President for Emerging Companies
Biotechnology Innovation Organization

David Fajgenbaum
Executive Director
Castleman Disease Collaborative Network

Shamiram Feinglass
Vice President, Government and Regulatory Affairs
Danaher

Tim Fenton
Vice President, Global Government Relations
Thermo Fisher Scientific

Erika Ferguson

Andrea Ferris
President
LUNGeivity

Liz Fowler
Vice President, Global Health Policy
Johnson & Johnson

Leonard Freedman
President
Global Biological Standards Institute

Pat Furlong
Founding President and CEO
Parent Project Muscular Dystrophy

Robert Gellman
Privacy and Information Policy Consultant

Dalvir Gill
CEO
TransCelerate BioPharma Inc.

Adam Gluck
Vice President, Government Affairs & Patient Advocacy
Biogen

Lynn Goldman
Michael and Lori Milken Dean
The Milken Institute School of Public Health at George Washington University

Steven Grossman
Deputy Executive Director
Alliance for a Stronger FDA

Jennifer Hall
Chief, Institute for Precision Cardiovascular Medicine
American Heart Association

Margaret Hamburg
Foreign Secretary
National Academy of Medicine

Kathi Hanna
Science and Health Policy Consultant

Christopher Hansen
President
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Cancer Action Network

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Kay Holcombe
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Celgene

Thomas Insel
Neuroscientist
Verily Life Sciences

Elizabeth Iorns
Founder and CEO
Science Exchange

Annalisa Jenkins
CEO
Dimension Therapeutics

Stephen Johnson
Chief Intellectual Property & Policy Officer
One Mind Initiative

Lyric Jorgenson
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Michael Kaplan
Executive Director
Melanoma Research Alliance

Taha Kass-Hout
Kass-Hout Consulting

Adam Keeney
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Regis Kelly
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Isaac Kohane
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Crystal Kuntz
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America's Health Insurance Plans

Mark Laabs
Founder and Chairman
Rare Cancer Research Foundation

Debra Lappin
Principal
FaegreDB Consulting

Jonathan Leff
Partner
Deerfield Management

Bennett Levitan
Senior Director, Benefit-risk Assessment
Johnson & Johnson

Mark Lim

Geoffrey Ling
Professor
Uniformed Services University of the Health Sciences

Andrew Lo
Professor of Finance, and the Director of the Laboratory for Financial Engineering
MIT Sloan School of Management

Katherine Maynard
Principal
PWR

Mark McClellan
Director
Duke-Margolis Center for Health Policy

Anna McCollister-Slipp
Chief Advocate for Participatory Research
Scripps Translational Science Institute

Nancy McGee
Executive Vice President
Avalere Health

Michelle McMurry-Heath
Vice President, Regulatory Affairs for Medical Devices and Diagnostics
Johnson & Johnson

Robert Meyer
Director
Virginia Center for Translational and Regulatory Sciences, University of Virginia

Howard Moon
Executive Director, Global Government Affairs
Amgen

Theresa Mullin
Director, Office of Strategic Programs, Center for Drug Evaluation and Research
U.S. Food and Drug Administration

Bernard Munos
Founder
InnoThink

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Appendix 2: Discussion Guide



Discussion Guide

- Introduction
 - We want to make sure our next President doesn't waste a minute of time in getting to faster cures.
 - We are tapping into our network of stakeholders across the biomedical research ecosystem – from development to delivery – to inform the itinerary for the next Administration to get to this future state.
 - We will engage directly with the next Administration and will also disseminate our findings broadly.
 - We will not quote you by name, but plan to include your name in an Acknowledgement section of the final report. Please let us know if you prefer to remain off that list.

We intend for our conversation to be split between:

1. Identifying key opportunities to accelerate and strengthen the innovation ecosystem.
2. Digging into how the next Administration could realize those opportunities.

1. On which of these issues – or others not included here – do you think the next Administration can make significant progress?

Accelerating Treatments & Interventions

- ___ Patient Engagement – realizing the benefits of patient centricity in biomedical product development
- ___ Unlocking Discoveries – improving the system for biomedical discovery, enabling the next big scientific opportunities
- ___ Data, Data, Data – translating the promise into real world benefits for R&D, regulatory science, access to new treatments, and more
- ___ Collaboration 2.0 – enhancing team science, cross-sector collaboration, data sharing, and more
- ___ Crossing the Valley of Death – funding, de-risking, and advancing promising medical products

Strengthening the System

- ___ Federal Resources and Capabilities – identifying the right structures and tools, strengthening the workforce, funding the mission
- ___ Human Capital – harnessing and promoting a strong life science workforce in academia, government, and industry
- ___ Value – defining it, measuring it, delivering it, paying for it
- ___ The Economy – maximizing short and long term benefits of life science investments
- ___ Other?

2. For each of the top issues you've cited:
 - A) What changes need to happen to help solve this problem?
 - B) To achieve this change, what actions could the following stakeholders take?
 - 1) The Administration (*i.e.*, White House, Departments, and Agencies)
 - 2) The Congress
 - 3) Stakeholders outside government

- Concluding thoughts:
 - Are there any other issues we should be thinking about?
 - Who else should we talk with?

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