A Closer Look at the White House's Precision Medicine Initiative

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On January 20, 2015, as part of his State of the Union address, President Obama announced the upcoming launch of the White House's Precision Medicine Initiative (Initiative).[1] The Initiative seeks to allocate $215 million in 2016 toward a series of efforts aimed at collecting and using genomic, lifestyle and other clinical data in furtherance of precision medicine research.[2] The response to the Initiative has been rapid and significant: in the short time since its announcement, a two-day workshop on the Initiative has been held, with working papers and presentations from multiple stakeholders, and an advisory committee has been formed to help develop and implement a significant portion of the Initiative. Viewed alongside other noteworthy developments from the past six months, the Initiative exemplifies current trends and challenges related to health technologies, data privacy and security, and biomedical research, and provides insight into the future of data-driven medicine.

Precision Medicine Goals

Precision medicine is not new. Sometimes called personalized or tailored medicine, precision medicine is an approach to diagnosis and treatment that departs from the standard "one-size-fits-all" approach to care.[3] Although some in the field argue that the difference between the terms "precision medicine" and "personalized medicine" is negligible, the term precision medicine has been actively developed and popularized within the last 10 years as a way to more accurately emphasize molecular-level information aiding patient diagnosis and treatment decisions.[4] Precision medicine takes as its starting point the basic idea that an individual's genetic makeup, environment, lifestyle and other patient-specific information may be predictive not only to the individual's future health, but also to the individual's responsiveness to treatment alternatives.[5] For example, a precision medicine approach to treating a patient might involve genetic testing to help a treating provider determine which of several available therapeutic agents for a given condition will yield the best patient response. Precision medicine therefore prioritizes therapeutic and cost-control objectives by providing the best care quickly and without costly missteps.

Aspects of the Initiative

The Initiative aims to support precision medicine and its goals in two principal resource-building ways. First, the Initiative intends to provide funding to various agencies for the evaluation and reform of existing processes and

[1] Fact Sheet, The White House, Office of the Press Sec'y, President Obama's Precision Medicine Initiative (Jan. 30, 2015).

[2] Office of Mgmt. & Budget, Exec. Office of the President, Budget of the United States Government, Fiscal Year 2016, 19 (2015).

[3] Francis Collins & Harold Varmus, Perspective, *A New Initiative on Precision Medicine*, New Eng. J. Med. (Jan. 30, 2015).

[4] Alla Katsnelson, *Momentum Grows to Make 'Personalized' Medicine More 'Precise,'* 19 Nature Med. 249, 249 (Mar. 2013).

[5] Lindsay Holst, *The Precision Medicine Initiative: Data-Driven Treatments as Unique as Your Own Body*, White House Blog (Jan. 30, 2015, 9:19 AM).

regulations to better address the large-scale data collection and analysis that is foundational to precision medicine.[6] Second, the Initiative seeks to provide research funding to expand efforts in oncology genomics and to develop a research cohort study of at least one million Americans who would participate by sharing genomic and clinical data, biospecimens and biofluids, and other information.[7] This collected information would be shared with both researchers and participants in a variety of ways that might include electronic health record (EHR) and mobile health device utilization. Together, these efforts would result in a more coherent and harmonized regulatory framework to facilitate precision medicine, while building the data and recruitment resources to leverage that improved framework.

The Initiative also intends to focus on the following goals:

- Ensuring a commitment to rigorous privacy protections by identifying and addressing precision medicine data privacy and security-related issues
- Supporting clinical trials, in partnership with pharmaceutical companies, to test specific drug therapies selected using precision medicine techniques
- Developing a new approach to the U.S. Food and Drug Administration's (FDA's) approval for next generation sequencing technologies
- Supporting new interoperability standards for cross-system data exchanges as part of the national research cohort[8]

Funding

President Obama's 2016 proposed budget plans to distribute the allocated funds to three different agencies: $130 million to the National Institutes of Health (NIH) to aid in the development of the national research cohort; $70 million to the National Cancer Institute (part of NIH) to aid the research and application of the genomic drivers of cancer; $10 million to the FDA to support the development and regulation of databases and next generation sequencing technologies; and $5 million to the Office of the National Coordinator for Health Information Technology (ONC), which helps to coordinate federal data privacy policy, to develop interoperability standards addressing the privacy and security of sharing research data.[9]

These allocations require congressional approval, and there have been early signs of bipartisan support for the Initiative.[10] This funding is part of an overall research funding package proposal (including a nearly $1 billion increase in funding to the NIH) that reflects the Obama administration's intention to invest in biomedical research innovations.[11]

Developing the Initiative: Workshops, White Papers and Advisory Panels

Many of the Initiative's specific research aims and measures are understandably still under development, so it remains unclear how much data the Initiative will gather and the methods it will use to do so. On February 11 and 12, 2015, the NIH held a workshop on the creation of the precision medicine cohort, exploring the opportunities and operational challenges of this undertaking with experts and leaders from the public and private sectors.[12]

Prior to the workshop, the NIH also assembled four working groups. Each group developed a white paper to aid the discussion at the workshop. The focus of these white papers is cohort identification and recruitment; participant engagement, data privacy and return of results; data collection and mobile technologies; and EHR opportunities and challenges for

[6] Fact Sheet, The White House, *supra* note 1.

[7] *Precision Medicine Initiative: What are the longer-term goals?*, NIH (last reviewed Feb. 9, 2015).

[8] *Precision Medicine Initiative: What are the near-term goals?*, NIH (last reviewed Feb. 9, 2015); Fact Sheet, The White House, *supra* note 1.

[9] Fact Sheet, The White House, *supra* note 1.

[10] Press Release, U.S. Senate Chairman's Press, Alexander to Meet with White House on Precision Medicine Initiative (Jan. 30, 2015), (indicating Senator Alexander's enthusiasm in working with the President on the Initiative); Press Release, Tom Wilbur & Lynn Turner, Congressman Fred Upton Press Releases, ICYMI: It's in the genes, (Feb. 2, 2015), (indicating Congressman Upton's view of precision medicine as a "natural fit" in the 21st Century Cares initiative discussion).

[11] Jeannie Baumann, *New NIH Advisory Panel will Develop Million-Person Cohort for Obama Plan*, 9 Bloomberg BNA Life Sci. L. & Indus. Rep. 225 (Feb. 13, 2015) (available by subscription).

[12] *Precision Medicine Initiative: NIH Workshop on Building a Precision Medicine Research Cohort*, NIH (last reviewed Feb. 13, 2015).

research.[13] The papers mirror the topics explored at the workshop and provide helpful insight into the details that must be resolved before the Initiative can be fully operational.

For example, the white paper from the working group focusing on cohort building emphasized the need to utilize subjects and data from existing longitudinal studies in the cohort, envisioning a large consortium of cohorts with a central infrastructure to minimize the time and cost constraints of such an endeavor.[14] The working group on participant engagement similarly addressed the cost, participant attrition, data-sharing and governance challenges of the Initiative.[15] The white paper from the working group focusing on EHR opportunities and challenges explored a variety of pathways by which participants' clinical data could become available to researchers, including existing research cohorts, organizational partnerships and individual releases. This paper also focused on enhancing patients' access to their own clinical and administrative data, and emphasized that privacy pathways/consent management systems would be imperative to the Initiative's implementation.[16] Finally, the white paper from the data collection and mobile technologies working group emphasized that the widespread use of mobile technology would be key to capturing and tracking relevant health data for the Initiative.[17]

During the Initiative workshop, NIH leaders announced the creation of a new working group of the NIH Advisory Committee. This group will develop the specifics of the million-person research cohort envisioned by the Initiative and will make recommendations regarding its implementation to the director of the NIH. Kathy Hudson, NIH deputy director for science, outreach and policy, and Richard Lifton, Yale University's Genetics Department chairman, were appointed to co-chair the group. The NIH plans to name the rest of the panel in the coming weeks and have the group provide an initial report to the Advisory Committee by September 2015.[18]

While its specifics remain under development, the Initiative is another source of public support and funding for potential future breakthroughs using precision medicine and other novel approaches; it aims to create a framework where researchers, and eventually clinicians, can utilize as many individuals as possible to collect as much data as possible to improve the quality and efficacy of medicine.

Departing from the Status Quo

The Initiative's announcement is part of a trend that has emerged during President Obama's tenure that shows the current administration confronting and supporting the modernization of U.S. medicine in the genomic age. Not only has the president issued significant reports on big data and genomic privacy, the administration has also begun examining and reforming major rules affecting progressive research, such as the Health Insurance Portability and Accountability Act (HIPAA) and the Federal Policy for the Protection of Human Subjects (Common Rule). Although the funding allotted by the Initiative is small compared to the overall budgets for the NIH (around $30.3 billion[19]), FDA (around $4.4 billion[20]) and ONC (around $60.4 million[21]), the Initiative's real significance may be the national policy recognition of the need to reconfigure treatment approaches. The Initiative recognizes the public and private sector's interest in precision medicine and views precision medicine as a powerful tool, rather than a passing

[13] *Precision Medicine Initiative: Workshop Planning Team White Papers*, NIH (last reviewed Feb. 13, 2015).

[14] A preliminary NIH inventory report has already identified 50 large-scale research cohorts, comprising approximately 12.3 million individuals enrolled in 65 studies, according to the document. Rebecca Baker et al., *Building a Consortium of Cohorts – Cohort Identification and Participant Recruitment* 1-3 (NIH Precision Medicine Initiative Cohort Working Group, Draft Report, 2015).

[15] Dixie Baker et al., *Participant Engagement, Data Privacy, and Novel Ways of Returning Information to Participants* 3, (NIH Precision Medicine Initiative Cohort Working Group, Draft Report, 2015).

[16] Rex Chisholm et al., *Opportunities and Challenges Related to the Use of Electronic Health Records Data for Research* 1-3, (NIH Precision Medicine Initiative Cohort Working Group, Draft Report, 2015).

[17] Rick Cnossen et al., *Data Collection and Mobile Technologies* 1, 4 (NIH Precision Medicine Initiative Cohort Working Group, Draft Report, 2015).

[18] Baumann, *supra* note 11.

[19] *NIH Budget*, NIH (last reviewed Jan. 29, 2015).

[20] FDA, Dept. of Health & Human Servs., Fiscal Year 2016 Justification of Estimates for Appropriations Committees 3 (2015).

[21] ONC, Dept. of Health & Human Servs., Fiscal Year 2016 Justification of Estimates for Appropriations Committee 8 (2015).

trend.[22] The Initiative also recognizes the need for multi-stakeholder buy-in and involves the technology, medical, research and patient populations.[23] For example, the Initiative emphasizes patient engagement as integral to success by incorporating patients and subjects into the planning and research process and building the patient engagement concept into most of its endeavors. Even the Initiative's budget, spread among several different agencies, may be viewed as a high-level acknowledgment that precision medicine, and the Initiative itself, requires a coordinated, interdisciplinary effort to accomplish its goals.

The Initiative in Context

The Initiative tracks the current landscape of policy statements and proposed regulations aimed at modernizing medical data stewardship around three principles—data privacy, data security, and data utility. It is the latest attempt to balance the potential utility of large-scale data collection and data sharing against the privacy and security concerns that come with that data, and should be examined in light of other recent proposals. Some of these other proposals include the following:

- August 27, 2014: The NIH issued its Genomic Data Sharing Policy, which promotes the sharing of large-scale genomic data generated from NIH-funded studies and includes new subject data protections.[24]
- November 19, 2014: The NIH proposed a draft policy to ensure all NIH-funded trials are registered for and submit summary results to clinicaltrials.gov.[25]
- January 23, 2015: The FDA permitted the marketing of the first system of mobile medical applications for continuous glucose monitoring.[26]
- January 27, 2015: The House of Representatives' Energy and Commerce Committee released its highly anticipated discussion draft of the 21st Century Cures Initiative legislation. This draft targeted comprehensive research and medical innovation reform, while incorporating patient perspectives and modernizing regulatory frameworks.[27]

These regulatory efforts and the Initiative share several common themes. First, they indicate that the current research infrastructure—for example, the regulations, research modalities, data capture and sharing pathways, and funding—is anachronistic and inadequate to harness genetic and genomic data on the scale contemplated by the Initiative.[28] They also acknowledge the emerging privacy and security concerns stemming from large-scale research and data-sharing endeavors, and, in varying degrees, contemplate updated privacy and security protections to address these issues. Ultimately, these data stewardship efforts point to shortcomings that cannot be addressed in a piecemeal fashion and thus require comprehensive change. They are also valuable acknowledgements that scientific and medical advancements require modernized infrastructure and better resources to ensure optimal research power, appropriate and relevant security protocols, and practical and reliable data privacy practices in the future.

In context, the Initiative is also important because it shows a growing interest in data sharing, aggregation and utilization on a very large scale. This may be evidence that the social and legal perspectives on clinical/genomic privacy are evolving. This trend may also suggest a general acceptance of increased data sharing as a necessary driver of innovation. Given this momentum, it is reasonable to expect continued efforts to modernize the U.S. research system, and to ultimately expect a significant shift away from the current regulatory framework.

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[22] *The Promise of Precision Medicine*, White House, (Jan. 30, 2015), (featuring Jo Handelsman, White House Associate Director for Science, who notes "Precision medicine is now. We're already using it, and we know it works.").

[23] *Precision Medicine Initiative: Who will participate?*, NIH (last reviewed Feb. 9, 2015).

[24] NIH, Genomic Data Sharing Policy, Notice No. NOT-OD-14-124 (Aug. 27, 2014).

[25] NIH, NIH Request for Public Comments on the Draft NIH Policy on Dissemination of NIH-Funded Clinical Trial Information, Notice No. NOT-OD-15-019.

[26] FDA News Release, FDA, FDA Permits Marketing of First System of Mobile Medical Apps for Continuous Glucose Monitoring (Jan. 23, 2015).

[27] 21st Century Cures Act, H.R. Comm. Energy & Commerce, 114th Cong. (2015) (Discussion Draft Document).

[28] Baumann, *supra* note 11.

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