Transforming FDA's Approach to Digital Health

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The eminent 19th century physician William Osler once said that "the good physician treats the disease; the great physician treats the patient who has the disease."

But for most of the 20th Century, the medical community generally treated the individual as a collection of symptoms, rather than treating the patient as an individual.

This was aggravated by the evolution of our health care delivery system, which fragmented patient care, and – at times – made patients come to feel like cogs in a wheel.

Digital health holds the potential to make Dr. Osler's vision of patient-centered care come to fruition.

Digital health tools have vast potential improve our ability to accurately diagnose and treat disease. And to enhance the delivery of health care for the individual, making medical care truly patient centric -- empowering the individual.

I know there's often a lot of hype around the promises of digital health. One can ask whether digital health is victim to Amara's law – that famous maxim coined by Stanford University computer scientist Roy Amara. The axiom says, "we tend to overestimate the effect of a technology in the short run and underestimate the effect in the long run."

But when it comes to digital health, from where I sit – and based on what we're seeing at FDA – the long run is now.

And I want to talk to you today about some new steps we're taking to ensure we reap the benefits from this change.

Most notably, we're expanding the opportunities for digital health tools to become a part of drug review, to couple these capabilities to drug delivery to form a drug delivery system.

We're expanding on our novel model for the pre-market review of digital health tools as medical devices, through our new pre-cert program. We're implementing a new approach to the review of artificial intelligence; and we're announcing today a new application of digital health tools to our own work – in this case the pre-market review of drug safety.

And finally, we're unveiling a new digital health incubator at FDA. I want to talk about each of these things today in turn, and how they build on our broader vision.

Powerful applications of digital health technologies are already being commercialized today.

And others will be commercialized in the next few years.

For example, mobile health apps already are empowering consumers to become co-directors of their own health by helping them take more control of things like their diet and lifestyle. And there are a growing number of mobile health applications that have shown promise in areas as diverse as diabetes prevention, asthma, and addiction recovery. ,

According to industry estimates, by the end of this year, 50 percent of the smartphone and tablet users in the U.S. will have downloaded mobile health applications.

In addition to empowering patients, digital tools are giving providers a truly holistic view of patient health and function through new data flows. These tools are helping redesign physician workflow to better coordinate patient care.

The trend towards connectivity and seamless monitoring is reflected in the 51 digital health products authorized by the FDA in 2017. One of those included a sensor embedded in a drug for schizophrenia that can allow patients to share treatment data with their physician through a medical app.

Digital health also offers real opportunities to improve medical outcomes, enhance efficiency, and reduce costs.

Take the example of clinical decision support software for medical imaging: When it comes to diagnosing stroke, every minute counts. Every minute that blood flow to the brain is obstructed can lead to major loss of patient function, and increase the odds that a patient will succumb to the stroke.

In February, the FDA approved a type of clinical decision support software that uses artificial intelligence algorithms to notify a neurovascular specialist faster than would be possible without the software, decreasing time to treatment.

Earlier interventions with effective clot busting drugs or surgery can save brain tissue, prevent deaths, and potentially save hundreds of thousands of dollars in hospital and rehabilitation costs for a single patient.

FDA plays a critical role in supporting this continued innovation as part of our mission to protect and promote public health. First and foremost, we must make sure that our approach to regulating these technologies maintains our scientific gold standard for product safety. We must always put protecting patients at the forefront of what we do.

As part of our mission, we must also take steps to make sure that beneficial new technologies can efficiently advance, and be made availably to patients in a timely way. This is ultimately how patients are going to benefit from science.

Doing so means that we must also recognize that FDA’s usual approach to medical product regulation is not always well suited to emerging technologies like digital health, or the rapid pace of change in this area. If we want American patients to benefit from innovation, FDA itself must be as nimble and innovative as the technologies we’re regulating.

That requires us to take modern and flexible approaches to regulation when it comes to highly novel areas like digital health --- to help encourage more developers to translate advances into clinically actionable tools to benefit patients.

We must give companies and investors the regulatory predictability they need to make the long-term investments, and take the significant risks, that innovation requires.

Investment into the digital health space has soared over the last year. Some of this new capital will help underwrite the development of tools that go on to help patients.

To help advance these opportunities, last summer, I announced the Digital Health Innovation Action Plan.

This new plan outlined our efforts to reimagine the FDA's approach to ensuring all Americans have timely access to high-quality, safe, and effective digital health products.

I committed to an approach that would help leverage digital health as a tool to empower consumers and to break down the fragmented silos that constrain healthcare delivery.

To expand progress in this area, I laid out several key goals.

Among them, was increasing the number and expertise of digital health staff at the FDA, launching the digital health software precertification pilot program within CDRH, and issuing new guidance to modernize our policies and outline our efforts to promote innovation in digital health.

Digital Health Innovation Action Plan

I'm pleased to announce that we've made substantial progress on these goals. In addition to updating you on what we've accomplished, I also want to announce several new initiatives that we're unveiling for the first time today.

These new efforts will continue to advance our framework for how we help promote innovation in this field.

Today we're issuing a new policy that streamlines the path for digital health products that contain several functions; some of which are subject to FDA's regulatory oversight as medical devices, while other functions, in isolation, are not.

This new guidance is another part of our action plan. It explains FDA's regulatory approach and policy for these multiple function digital devices and clarifies where the FDA will and will not be reviewing certain software functions included in such devices, provided they don't increase the risk or adverse effect of the function under FDA review.

For example, take a hospital monitor that detects and transmits vital patient signal information like heart rhythms into a patient's electronic health record. Here, FDA would only review the heart monitor function unless the transmission function adversely impacts the safety or effectiveness of the monitor capability. In that case, the developer only needs to show that they've addressed any potential for an adverse impact between the two different functions – the one FDA oversees, and the one it does not.

In issuing this guidance, FDA is taking additional steps to clarify what technologies won't fall under avoidable regulation. Our goal is to allow developers to efficiently incorporate into their products the latest advances in technology, while focusing FDA's review on the safety and effectiveness of the higher-risk medical device functions that diagnose or treat patients. We believe this approach will encourage more innovation in this important field.

Additionally, we've made significant progress in the last year on our device center's precertification pilot program.

Since announcing the nine pilot participants in September, the team has been working with the participants and other stakeholders to start assembling the essential building blocks of this unique and innovative program.

I'm pleased to announce another important update on the program. We're making available the first draft of our Working Model, which provides our vision on various aspects of the program and our steps for expanding it. The model is posted to our website. We're also sharing a new roadmap that outlines how we plan to develop the program.

The goal of this program is to develop a tailored and pragmatic framework that trusts the excellence of organizations, but also continually verifies the safety, effectiveness, and performance of software as a medical device. It leverages the transparency of a sponsor's culture of quality and its evidence of organizational excellence.

This is the first, high-level draft of what will be several iterations of our working model for this new program.

The critical piece we need moving forward is input from developers, patients, providers, and members of the public.

So throughout the working model, you'll see that we've drawn up "challenge questions" about each component of the program that we want answers and input on. We've designed the Pre-Cert program to be an iterative, collaborative experience. Your feedback is key to its success.

We're committed to launching "Pre Cert 1.0", a first version of the program by the end of 2018. Once we get this framework firmly in place, it'll be further refined in 2019.

In advancing our approach to digital health, we started by focusing on efforts by our medical device center, given that Center's long experience regulating software incorporated into medical devices. However, digital health impacts almost every facet of health care. So today I want to announce some new steps we're taking to broaden the opportunities offered by digital health across our other medical product centers.

Launch of Program to Apply Digital Health to Drugs

Today, I'm announcing that we'll expand the opportunities to use digital health tools as part of drug development and, in the process, enable new innovation to improve patient care.

As we begin for the first time to address the role of digital health in drug development, we'll work to ensure our regulatory approach reflects the novel nature of these products and encourages and supports their innovation. We must recognize the potential of digital health as a new tool to improve the safety and effectiveness of drug delivery.

We know that to enable these opportunities, we need clear policies for how the review and validation of digital health tools can be baked into drug development programs.

Mobile devices and software linked to specific drugs can help patients stay on therapy for treatments where medication compliance is traditionally a challenge. For instance, it can help patients, and their physicians, confirm that patients have taken their medication and easily incorporate information into electronic health records.

To take another example: Software can help cancer patients monitor side effects of their treatment by using smartphone cameras and facial recognition software to objectively classify pain symptoms and track cognitive performance.

It can also allow sponsors to comply with post-market surveillance requirements. In some cases, these tools might allow for safety and efficacy claims supported by data collected through software or sensors embedded in smart devices – like increased activity, improved mood, or greater social interactions for patients treated for severe depression.

These are just a few use cases. Given the falling costs and increasing quality of ubiquitous smart devices, developers can find new ways to use cloud based services and tools to support patient health and support more effective treatments, tailored to a patient's needs and preferences.

To help expand the potential for digital health to address these possibilities, we'll be advancing a policy framework in this area through new guidance. And we'll be seeking public input on the right approach to incorporating software that's designed to be used with prescription drugs.

We want to assist innovation in this space. So we're asking how FDA can promote innovation in the development of digital health functions, as well as how these novel products can be integrated into advanced therapeutic options for patients. We'll also be requesting input on how to support the development of digital health tools that are included as part of approved drugs, and how to properly regulate in this space when we know that software undergoes rapid cycles of innovation, and is frequently updated and improved.

FDA will be opening a public docket to seek input from both innovators on the cutting edge of developing these technologies, as well from as from the providers and patients that hope to benefit from these advancements.

Building off the approach of our device center, we'll clarify that not all FDA requirements apply every time a digital health tool is employed in relation to a prescription drug.

There's a wide variety of ways in which apps and software can enhance health care. And our mission at the FDA is to apply our regulations under a balanced, risk-based framework—one that protects patients and allows digitization in the prescription drug setting to flourish.

Ultimately, our goal in establishing this framework will be to develop an efficient pathway for the review and approval of digital health tools as part of drug review, so that these tools reach their full potential to help us treat illness and disease, and encourage synergies between software and therapeutics that meet FDA's gold standard for safety and effectiveness.

Artificial Intelligence

One of the most promising digital health tools is Artificial Intelligence, particularly efforts that use machine learning.

AI holds enormous promise for the future of medicine, and we're actively developing a new regulatory framework to promote innovation in this space and support the use of AI-based technologies. So, as we apply our Pre-Cert program—where we focus on a firm's underlying quality— we'll account for one of the greatest benefits of machine learning – that it can continue to learn and improve as it is used.

Employing the Pre-Cert approach to AI may allow a firm to make certain minor changes to its devices without having to make submissions each time. And, we'll make sure that other aspects of our regulatory framework, such as new software validation tools, are sufficiently flexible to keep pace with the unique attributes of this rapidly advancing field.

We know that to support the widespread adoption of AI tools, we need patients and providers to understand the connection between decision-making in traditional health care settings and the use of these advanced technologies.

We know that our approach to AI regulation must establish appropriate guardrails for patients. And even as we cross-new frontiers in innovation, we must make sure that these novel technologies can deliver benefits to patients by meeting our standards for safety and effectiveness.

The technology won't be scaled or reimbursed without that level of confidence that it protects and promotes patients.

We expect to see an increasing number of AI-based submissions in the coming years, starting with medical imaging devices, and we're working with experts in the field.

This includes AI experts with prior experience in sectors like finance that are already widely using AI platforms for fraud detection. As we develop our own policies, we want to understand how these technologies can be validated, and how patients and providers can be confident that they're reliable, unbiased, and will help improve health outcomes.

Our approach to AI will also focus on the ways in which real world data flows. This includes structured and unstructured data from pathology slides, electronic medical records, wearable devices, and insurance claims data. We want to better understand, and unlock ways, that this data can be used to inform development and validation of AI devices.

In time, AI might even be taught to explain itself to clinicians. This field has already advanced much more quickly than we first expected, as have its practical applications to patients.

We expect that AI tools can become even more predictive as additional real world data is fed into these algorithms.

This technology also has the potential to significantly reduce costs from complications of chronic disease.

For instance, diabetic retinopathy is the most common cause of vision loss among the more than 30 million Americans living with diabetes and the leading cause of vision impairment and blindness among working-age adults.

In early April, the FDA approved the first medical device that combined a special camera and artificial intelligence to detect greater than a mild level diabetic retinopathy in adults who have diabetes in a primary care setting.

Early detection is a critical part of preventing serious vision problems. And yet eye care specialists don't screen about half of diabetic patients on an annual basis.

If the AI detects mild retinopathy, primary care physicians can refer patients to an eye care specialist.

Eventually, AI tools could be integrated directly into smartphones or wearable devices for a variety of early detection applications, reducing the need for expensive specialists visits, while increasing the likelihood that we're catching potentially serious problems early.

These are no longer far-fetched ideas.

We know that to keep pace with innovation in these fast-moving fields, FDA itself must do more to leverage digital health tools and analytics internally to help the agency develop new regulatory tools and advance its own work.

These include tools such as digital biomarkers for early disease diagnosis, or using data from EHRs to enable pragmatic clinical trials at the point of care.

These kinds of tools can help us make drug and device development more predictable, efficient and more reflective of patients' real-world experience. As we do, it'll become easier to streamline our workflows and foster collaboration.

Launch of a New Premarket Digital Safety Program

We're announcing one of those new steps today, with the launch of a new Premarket Digital Safety Program.

This program enables a unified data standard for meeting electronic reporting requirements under the expedited safety-reporting regulations of an Investigational New Drug Application. This new program will initially involve the FDA's Oncology Center of Excellence, and the agency's drugs and biologics centers. Let me give you a brief overview of how this new premarket safety program works:

Sponsors of clinical trials conducted under an investigational new drug application must report serious and unexpected suspected adverse reactions to the FDA and participating investigators in 15 or 7 days, depending on the type and severity of the event. One of the FDA's primary roles at the IND stage of development is ensuring patient safety.

Following a detailed examination of the submission and a review process for premarket safety reports, we found inefficiencies resulting from a disjointed, analog workflow.

Sponsors are submitting premarket safety reports to the FDA on PDF files or on paper via fax or hand-delivered mail.

Medical officers and clinical reviewers must review and track each report individually, reading thousands of narratives to understand the safety profile of investigational new drugs and biologics. A recent internal survey of the FDA's office of hematology and oncology products revealed that reviewers spend an average of 16 percent of their time managing these paper-based premarket safety reports.

Mapping our workflow made it clear that a big portion of that time is spent on administrative and file management tasks, rather than signal detection and medical review.

Reading PDF narratives is not an ideal way to go about detecting drug safety signals. It makes the existing analog process a burden on the FDA, sponsors, investigators, and Institutional Review Boards. More important, it makes it more difficult for reviewers to focus their time on the primary mission – monitoring the safety of patients.

Sifting the signal from the noise through PDF reports is not only difficult; it precludes the use of analytical methods for improved early detection of safety signals through post-market surveillance programs. Or better understanding therapeutic class effects to inform appropriate risk mitigation strategies early in clinical development programs.

This fragmented analog workflow is also a burden on the sponsor for meeting reporting requirements.

This process was badly in need of change.

So the FDA recently ran a successful pilot developing and demonstrating the feasibility of digital submission process where premarket safety reports are transmitted to the FDA as data sets that can be easily visualized and analyzed.

We believe that new digital framework can significantly improve the efficiency and accuracy of the premarket safety submission and review process, saving hundreds of review hours every month when the program is fully implemented.

Launch of FDA's New Digital Health Incubator

Finally, to support the integration of data analytics into regulatory decision making, we're taking another new step with the creation of an internal data science incubator called the Information Exchange and Data Transformation; or INFORMED.

The initial focus of this new tech incubator will be, among other things, the conduct of regulatory science research in areas related to health technology and advanced analytics related to cancer. Our goal will be to help modernize our framework for advancing promising digital health tools.

Among the ongoing INFORMED projects are collaborations with Project Data Sphere, a nonprofit open-access cancer data repository, aimed at developing algorithms for classification of tumor dynamics using medical imaging data.

We're also working on a joint fellowship program with the National Cancer Institute to design and develop digital biomarkers as drug development tools. These types of foundational research will guide the industry forward.

We've also launched a fellowship program with Harvard on AI and Machine Learning focused on designing, developing and implementing machine learning and artificial intelligence algorithms for regulatory science applications.

It'll look at developing new clinical endpoints and signal detection methods for evaluation of the safety and effectiveness of therapies. These efforts also will help us develop new approaches for understanding variations in individual patient experience using diverse data sets from clinical trials, EHRs, and biometric monitoring devices.

It will also look at the development of principles and definitions for validity and strength of AI/ML-derived evidence in the context of product approval and regulations. These types of foundational advances will guide the innovation forward. As many of you know, the biggest barrier to innovation is the costs and risks of new product development. It can take decades to translate promising ideas into products to extend and improve human lives.

New digital tools can help us reduce those costs and risks by helping us match products with the patients who are most likely to benefit, or help us identify potentially serious side effects sooner. They can break down artificial barriers between research and real-world practice, improving both. These are enabling. They'll spark other innovations in care.

And so, these technologies are also prompting FDA to rethink our own mandate, and how we enable safe, effective innovation in this novel area. We share your urgency to advance this innovation in care. And we're committed to doing our part to support its benefits to patients.

Thank you.

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