Fiscal Year 2023 Senate Appropriations Committee Outside Witness Testimony
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Subcommittee on Agriculture, Rural Development, Food and Drug Administration, and Related Agencies
Food and Drug Administration Fiscal Year 2023 Appropriations

Chairwoman Baldwin, Ranking Member Hoeven and distinguished members of the subcommittee, the Personalized Medicine Coalition (PMC) appreciates the opportunity to submit testimony on the U.S. Food and Drug Administration (FDA)'s fiscal year (FY) 2023 appropriations. PMC is a nonprofit education and advocacy organization comprised of more than 220 member institutions across the health care spectrum who are working together to advance personalized medicine in ways that benefit patients and health systems. While the COVID-19 pandemic has significantly impacted FDA's work since 2020, the pandemic did not slow the extraordinary pace of scientific innovation in personalized medicine. We appreciate the funding increases provided by the Subcommittee in the FY 2022 omnibus for FDA, which allowed the agency to continue carrying out its public health mission and support the delivery of the targeted health care interventions that are the foundation of personalized medicine. As we look toward a post-pandemic future and the subcommittee begins work on the FY 2023 Agriculture, Rural Development, FDA, & Related Agencies Appropriations bill, **we respectfully ask that you continue to invest in the FDA by appropriating $3.653 billion to FDA's budget authority as requested by the Administration.**

Personalized medicine, also called precision or individualized medicine, is an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient or use medical interventions to alter molecular mechanisms that impact health. By combining data from diagnostic tests with an individual's medical history, circumstances, and values, health care providers can develop targeted treatment and prevention plans with their patients. Personalized medicine promises to detect the onset of disease, pre-empt its progression, and improve the quality, accessibility, and affordability of health care.[i]

FDA's growing responsibilities require a budget that grows. While user fee funding and one-time supplemental funds have been robust, these must be spent within specified parameters. Budget authority provides FDA with the broadest and most flexible means of meeting all its responsibilities.[ii] Previous increases in budget authority have enabled the FDA to advance multiple programs facilitating the development of personalized medicine products, which have become more complex and require greater sophistication and expertise to review. Additional increases in FY 2023 budget authority will allow the FDA to expand these initiatives and launch new ones, which all require a highly skilled and technical workforce equipped with modernized analytical tools that support science-based decision-making. By increasing federal investment in FDA activities fostering the development of innovative medical products, clinical trial design, real-world evidence (RWE), and digital health, Congress can help advance a new era of personalized medicine at a pivotal moment, promising a brighter future for health systems and patients with unmet medical needs.

The Role of the FDA in Personalized Medicine

Thanks in part to a responsive regulatory agency, personalized medicine has seen steady progress. As of 2020, more than 286 personalized treatments are available for patients.[iii] That number has continued to grow, with personalized medicines accounting for more than a quarter of all new drugs approved by FDA each of the past seven years, including 35 percent of the new drugs approved by FDA in 2021.[iv] With more than half of new personalized treatments being approved for indications outside of oncology, these new approvals are also helping transform care for molecularly selected subsets of patients with cancer as well as rare, common, and infectious diseases. In 2021, FDA also approved two new chimeric antigen receptor (CAR) T-cell-based immunotherapies, approved new diagnostic indications for nine testing platforms, and recognized the validity of a public tumor-mutation database that will help physicians target treatments to only those patients who will benefit.

The FDA is the gateway for many personalized medicine breakthroughs entering the market. FDA's Center for Devices and Radiological Health (CDRH), Center for Drug Evaluation and Research (CDER), and Center for Biologics Evaluation and Research (CBER) each have responsibilities for evaluating medical products for their safety and efficacy. As personalized approaches to treatment and prevention have grown, new types of drugs, tools, and technologies leveraging complex and sophisticated data, like genomic information and RWE, have challenged existing regulatory frameworks and processes.

Facilitating the Development of Personalized Medicine Products

The FDA is taking a number of steps to modernize its regulatory processes, such as streamlining its technical and data infrastructure to shorten review times, improving clinical trials to address disparities and unmet medical needs, integrating RWE into medical product reviews, and building partnerships to foster digital health and artificial intelligence (AI) technologies. Robust funding from Congress will help the FDA build upon this work and bring personalized medicine products to patients as efficiently as possible.

Expediting Product Development

As a byproduct of scientific breakthroughs, the amount and variety of data that FDA generates, needs and uses is rapidly increasing. Even though the FDA has taken action across the agency in recent years to make more rapid decisions and improve communications with medical product developers, the pandemic has highlighted the agency's fragmented technical infrastructure. For example, there are more than 30 data systems utilized in premarket review for devices, and these legacy data systems are outdated, complex, fragmented, and time-consuming to use.[v] In 2021, FDA announced a new Office of Digital Transformation to advance the agency's information technology transformation, which began in 2019. Additional funding will enable FDA to build upon ongoing efforts to streamline data practices across centers and help translate FDA's wealth of information into knowledge supporting progress in personalized medicine.

Furthermore, the agency must bolster its workforce to build in flexibilities across each of its centers and to keep pace with the growing pipeline of gene therapies. FDA staff have taken extraordinary steps to respond to the coronavirus public health emergency. This stretched capacity, however, is not without consequences. In April 2021, CDRH announced that, due to limited resources and delays in review timelines, it would be declining in vitro diagnostic pre-

submission requests that do not fall into certain priority categories.[vi] While Congress has appropriated no-years monies to the agency to address some of the pandemic's resource demands, FDA is only able to hire permanent full-time employees through increased budget authority from Congress. Even though CDRH is now aiming to return to normal review of pre-submissions over the course of 2022,[vii] continued increases in FDA's budget authority would allow the agency to address restraints on workload capacity and build in future flexibilities as new medical product submissions increase in number and become more complex. For example, the scientific review of gene therapies requires the evaluation of highly complex information and, thus, reviewers with highly specific expertise. As of January 2020, FDA had over 900 active Investigational New Drug applications for gene therapies,[viii] and by 2025 the agency anticipates it will be approving 10 to 20 cell and gene therapy products per year.[ix] In addition to supporting FDA's regulatory science initiatives advancing cell and gene therapy, increased funding will ensure the agency can grow its workforce to prepare for a growing pre- and post-market workload of new treatments for patients that promise to address the root causes of disease.

Modernizing Clinical Trials

More rare diseases and cancers are being defined by biological markers, creating smaller groups of patients who are more likely to respond to targeted treatments and are candidates for participation in trials. Trials that rely on identification of patients by biological markers, such as enriched trials, trials with master protocols, and in silico trials using computer modeling, present opportunities to streamline clinical research, especially in cases where a scarcity of patients makes a randomized control infeasible and where important personalized medicines may be delayed or discarded because FDA cannot afford to run trials needed to validate them. In addition, ensuring that personalized medicines are impactful to all patients requires the inclusive and equitable representation of patients with diverse characteristics and health needs in clinical research. In 2021, FDA launched the Enhance Equity Initiative to improve the diversity of participants in clinical trials, and FDA continues to consider opportunities for allowing for remote participation in research through decentralized trials. FDA is also supporting clinical trials networks in rare diseases and is working to bring clarity to the emerging area of individualized drugs developed for a single, "n-of-one" patient diagnosed with a very rare genetic disease and when traditional clinical trials are not an option. These initiatives promise to foster a more agile clinical research enterprise for personalized medicine that helps address unmet medical needs and disparities in clinical research.

Advancing the Use of Real-World Evidence (RWE)

Traditional post-market studies require years to design and complete and cost millions of dollars. The use of medical data collected outside of a clinical trial, or RWE, played a vital role in answering key questions about COVID-19 and related therapeutics, diagnostics, and vaccines.[x] FDA's response to the pandemic has demonstrated how RWE can be used to understand the utility of new treatments and diagnostics, as well as improve patient access to personalized medicine. In addition to developing guidance on RWE, FDA is working to expand its Sentinel System and NEST (National Evaluation System for health Technology) programs to monitor the safety and effectiveness of approved medical products. Increased budget authority for FDA would create a more reliable source of funding for and strengthen programs like Sentinel and NEST, which provide national resources for filling data gaps in existing surveillance systems,

improving regulatory decision-making, and improving the quality of RWE available to health care providers and patients to make more informed treatment decisions.

Fostering Digital Health Technologies and Artificial Intelligence (AI)

Data-capturing technological devices, or digital health technologies, and AI can play a key role in the collection and analysis of RWE. In 2020, CDRH launched the Digital Health Center of Excellence to build partnerships and develop best practices advancing the development and FDA review of these cutting-edge technologies. Over the past year, FDA continued to advance its pilot precertification program for medical device software and initiated seven regulatory science research projects.[xi] By informing FDA's approach to regulatory oversight of these emerging technologies, additional investment in these initiatives may encourage the use of personalized medicine by helping match new personalized medicine products with the patients who are most likely to benefit or by helping to identify potentially serious therapeutic side effects sooner. Digital health technologies can also play a key role in enabling remote participation in trials. This foundation laid at the FDA for digital health and AI will become increasingly important for personalized medicine as patients assume a larger role in managing their own health care and are more informed by their ability to access data about their unique biology.

***Implementing the* 21st Century Cures Act (Cures Act)**

By passing the *Cures Act*, Congress acknowledged the need for an additional focus on and funding for FDA. These resources have facilitated some of the programs mentioned above, but this funding alone is insufficient to fully sustain the agency's work in these critical areas. The *Cures Act* authorizes $50 million in FY 2023 for the FDA through the Innovation Account,[xii] but as annual allocations have tapered off, the science behind product development continues to increase in complexity. Increases in the FDA's budget authority appropriations are necessary for the agency to continue the important programs launched by the *Cures Act* and to build on them in ways that help the FDA evaluate novel personalized medicines and technologies. To help ensure activities mandated under new initiatives are fully funded, we support the proposed no-year money requested by the Administration for pandemic response ($1.6 billion) and the Cancer Moonshot ($20 million).

Conclusion

PMC appreciates the opportunity to highlight the FDA's importance to the continued success of personalized medicine. A budget authority appropriation for the FDA in FY 2023 of $3.653 billion will help the agency chart an efficient path for advancing innovative medical product development and bring us closer to a future in which every patient benefits from a personalized approach to health care.

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[i] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_The_Personalized_Medicine_Report_Opportunity_Challenges_and_the_Future.pdf
[ii] https://acrobat.adobe.com/link/track?uri=urn:aaid:scds:US:fbda0765-44c4-47d2-aa11-71a4721a4d3f#pageNum=2
[iii] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_The_Personalized_Medicine_Report_Opportunity_Challenges_and_the_Future.pdf
[iv] https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/Personalized_Medicine_at_FDA_The_Scope_Significance_of_Progress_in_2021.pdf
[v] https://www.fda.gov/news-events/fda-voices/how-cdrhs-digital-transformation-initiative-will-strengthen-premarket-review-program
[vi] https://www.fda.gov/news-events/fda-voices/year-pandemic-how-fdas-center-devices-and-radiological-health-prioritizing-its-workload-and-looking
[vii] https://www.fda.gov/news-events/fda-voices/looking-ahead-2022-fdas-center-devices-and-radiological-health-manages-sustained-increase-workload
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