Fiscal Year 2022 House 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 2022 Appropriations

Chairman Bishop, Ranking Member Fortenberry 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) 2022 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. The global COVID-19 pandemic did not slow the extraordinary pace of scientific innovation in personalized medicine. The widely variable effects of COVID-19 have only highlighted the need for personalized medicine to move further and faster and for Congress to continue its investment in FDA. As the subcommittee begins work on the FY 2022 Agriculture, Rural Development, FDA, & Related Agencies Appropriations bill, **we respectfully ask that you increase the FDA's appropriation by no less than $200 million above the FY 2021 budget authority level** so that the agency can continue to carry out its public health mission and support the delivery of the targeted health care interventions that are the foundation of personalized medicine.

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]

Previous increases in funding have enabled the FDA to advance multiple programs facilitating the development of personalized medicine products. Additional increases in FY 2022 will allow the FDA to expand these initiatives and launch new ones, which all require a highly skilled and technical workforce. By increasing federal investment in FDA activities fostering the development of innovative medical products, clinical trial design, real-world evidence, 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

The rapid progress we have seen over the past year, from mRNA vaccine development, diagnostic testing, and variant sequencing, to beginning to understand how human genomic variation influences infectivity, disease severity, vaccine efficacy, and treatment response, show that science is leading the health system away from one-size-fits-all, trial-and-error medicine and toward an era of personalized medicine.[ii, iii] This progress was in part made possible by years of diligent funding from Congress to advance the necessary regulatory science and infrastructure.

Thanks in part to a responsive regulatory agency, personalized medicine has seen steady progress in recent years. As of 2020, more than 286 personalized treatments are available for patients.[iv] Personalized medicines accounted for 39 percent of the new drugs FDA approved last year, topping one-third of new drug approvals for the third time in the last four years.[v] This is a sharp increase since a decade ago, when personalized medicines accounted for less than 10 percent of newly approved therapies. These new approvals help transform care for molecularly selected subsets of patients with cancer, rare diseases, and common/infectious diseases. FDA also approved the first blood-based biomarker tests for cancer that will help guide targeted treatment strategies for patients who are unable to undergo invasive operations to obtain tissue biopsies. The emergence of blood-based biomarker testing may also usher in a new era in which cancers are detected at earlier stages, when they may be easier and less expensive to treat.

The FDA is the gateway for 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 using genetic information 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

The FDA continues to take actions across the agency that enable it to make more rapid decisions and improve communications with medical product developers. In recent years, the agency has worked toward integrating premarket and post-market programs to transition to a total product lifecycle approach to device evaluation and monitoring. These are positive steps, but the agency's technical infrastructure remains fragmented. For example, with over 30 data systems in the Devices Program, reviewers need to access up to 10 different systems during the review process.[vi] Additional funding would enable the agency to continue its *Data Modernization Action Plan* beginning with near term "driver projects" to promote consistent, repeatable data practices across centers that build foundational capabilities.

The agency must also bolster its workforce to keep pace with the growing pipeline of gene therapies, as well as build in workforce flexibilities across each of its centers. As of January 2020, FDA had over 900 active Investigational New Drug applications for gene therapies.[vii] The scientific review of gene therapies requires the evaluation of highly complex information and, thus, reviewers with highly specific expertise, and by 2025, the agency anticipates it will be approving 10 to 20 cell and gene therapy products per year.[viii] Additional funding would help FDA grow its workforce to prepare for an increasing pre- and post-market cell and gene therapy workload. FDA staff have taken extraordinary steps over the past year to respond to the

coronavirus public health emergency, with CDER staff absorbing the workload of around 250 full-time employees and CDRH seeing a 38 percent increase in pre-market submissions. This stretched capacity, however, is not without consequences. In April 2021, CDRH announced that, due to limited resources and delays in review timelines, it will be declining in vitro diagnostic pre-submission requests that do not fall into certain priority categories.[ix] 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. Increasing FDA's base funding level will allow the agency to begin addressing restraints on workload capacity and build in future flexibilities.

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 2020, FDA finalized guidance outlining approaches to enhancing the diversity of participants in clinical trials, and the COVID-19 pandemic has prompted FDA to consider additional opportunities allowing for remote participation in research through decentralized trials. FDA is also supporting clinical trials networks in rare diseases and 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 where 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, has played a vital role in answering key questions about COVID-19 and related therapeutics, diagnostics and vaccines as standards of care evolved rapidly.[x] These experiences over the past year have 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 March 2021, CDRH published an analysis of 90 examples of different types of regulatory submissions supported by RWE, and later this year CDER plans to publish draft guidance on how RWE can contribute to the assessment of safety and effectiveness in regulatory submissions. FDA is also 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 product review, 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 advancing the development and FDA review of cutting-edge digital health technologies. Over the past year, FDA also released an action plan for innovation in medical device software using AI and machine learning, held a public meeting to discuss the use of real-world data generated from patients through digital health technologies, and published learnings from its pilot precertification program for medical device software. FDA recently granted Breakthrough Designation to an AI platform that would aid clinicians in identifying patients at increased risk of developing atrial fibrillation (AFib) or atrial flutter. AFib is highly prevalent in the older adult population, and AFib-related strokes are disabling and costly. This AI platform utilizes ECGs to predict, among people without a previous history of AFib, who would develop it within the next 12 months. 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 their genomic data.

***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 the FDA. These resources have facilitated many 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 2022 for the FDA through the Innovation Account,[xi] but as annual allocations start to taper 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 build on them in ways that help the FDA evaluate novel personalized medicines and technologies.

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 2022 that is no less than $200 million above the FY 2021 base appropriations level 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://doi.org/10.1016/j.cell.2021.01.015
[iii] https://doi.org/10.1038/s41586-020-2817-4
[iv] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_The_Personalized_Medicine_Report_Opportunity_Challenges_and_the_Future.pdf
[v] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PM_at_FDA_The_Scope_Significance_of_Progress_in_2020.pdf
[vi] https://www.fda.gov/media/135078/download
[vii] https://www.fda.gov/news-events/press-announcements/fda-continues-strong-support-innovation-development-gene-therapy-products
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[ix] https://www.fda.gov/news-events/fda-voices/year-pandemic-how-fdas-center-devices-and-radiological-health-prioritizing-its-workload-and-looking
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[xi] https://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/ScienceBoardtotheFoodandDrugAdministration/UCM556618.pdf