Fiscal Year 2024 Senate Appropriations Committee
Subcommittee on Agriculture, Rural Development, Food and Drug Administration, and Related Agencies

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Food and Drug Administration Fiscal Year 2024 Appropriations

Chairman Heinrich, 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) 2024 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. We appreciate that the subcommittee provided an increased budget for FDA in FY 2023 to allow the agency to continue carrying out its public health mission. However, the agency's responsibilities increase in complexity each year. For this reason, the agency requires a budget that grows each year. Thus, as the subcommittee begins work on the FY 2024 Agriculture, Rural Development, FDA, & Related Agencies Appropriations bill, **we respectfully ask that you continue to invest in FDA by appropriating $3.914 billion to FDA's budget authority as requested by the Administration.** Such an increase will position FDA to provide access to safe and effective medical products, including advances in technologies that are the foundation for the future 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 help us detect the onset of disease, pre-empt its progression, and improve the quality, accessibility, and affordability of health care.[i]

Budget authority provides FDA with the broadest and most flexible means of meeting all its responsibilities.[ii] Previous increases in budget authority enabled the agency to implement multiple programs facilitating the development of personalized medicine products, which require greater sophistication and expertise to review. Additional increases in FY 2024 budget authority would allow FDA to expand these initiatives and launch new ones with a highly skilled and technically empowered 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, real-world evidence (RWE), and digital health, Congress can help usher in 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 FDA in Personalized Medicine

Thanks in part to a responsive regulatory agency, personalized medicine is progressing steadily. As of 2022, more than 300 personalized treatments are available for patients. That number has continued to grow, with personalized medicines accounting for more than a quarter of all new drugs approved by FDA for each of the past eight years, including 34 percent of the new drugs approved by FDA last year.[iii] These new approvals are helping to transform care for molecularly selected subsets of patients with cancer, where 53 percent of newly approved personalized medicines are indicated, as well as rare, common, and infectious diseases, which account for the rest. In 2022, FDA also approved five gene and cell-based therapies and new diagnostic indications for 12 testing platforms.

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 the safety and efficacy of breakthrough medical products. As personalized approaches to treatment and prevention have grown, new types of drugs, tools, and technologies leveraging complex and sophisticated data, like RWE, have challenged existing regulatory frameworks and processes.

Facilitating the Development of Personalized Medicine Products

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 unmet medical needs, integrating RWE into medical product reviews, and building partnerships to foster digital health and artificial intelligence (AI) technologies. In future years, robust funding from Congress will help FDA continue to 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 FDA has taken action across the agency in recent years to make more rapid decisions and improve communications with medical product developers, the agency's technical infrastructure remains fragmented. In 2021, FDA announced a new Office of Digital Transformation to advance the agency's information technology transformation, which began in 2019. The $10 million included in the Administration's proposed budget for FY 2024 would allow further investments in enterprise data and IT modernization to streamline data practices across centers and help translate FDA's wealth of information into knowledge supporting progress in personalized medicine.

The agency must also bolster its workforce to build in flexibilities across each of its centers and to keep pace with the growing pipeline of cell and gene therapies. Cell and gene therapy holds considerable potential for the treatment of hereditary genetic disorders, including rare, neurodegenerative, and infectious diseases. The number of cell and gene therapy submissions FDA receives is rising sharply. More than 200 new gene therapy Investigational New Drug applications are coming to FDA's CBER annually, up sharply from just over 100 in FY 2017.[iv] CBER provides extensive scientific and regulatory advice to product manufacturers throughout the medical product lifecycle. The center also develops policy and guidance on novel clinical,

scientific, and manufacturing challenges for these products. An increase in FDA's budget authority would allow the agency to continue working with stakeholders to facilitate end-to-end solutions addressing key issues limiting the development and application of gene therapies, like manufacturing challenges, that make these therapies cost-prohibitive and in some cases not commercially viable.

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 the trials needed to validate them.

For many rare diseases, treatments are now within reach. Incentives and regulatory pathways, however, must be maintained for all rare diseases to make development financially viable. FDA's Orphan Products Grant Program supports the development of products to treat orphan or rare diseases including programs to support clinical trials, natural history studies, and new authority to fund grants addressing regulatory science challenges.[v] Advances in the understanding of rare diseases and novel technology platforms have transformed many disease pipelines. By providing $30 million for the Orphan Products Grant Program, this subcommittee can respond to the documented economic burden rare diseases present to families and the country.[vi]

To help expand the development of new scientific approaches and tools available for advancing effective medical products that can prevent, diagnose, mitigate, and treat rare neurodegenerative diseases, Congress passed the *Act for ALS Act*. This bill authorized $2.5 million in funding for staffing to support implementation.[vii] To allow FDA to facilitate access to therapies for neurodegenerative diseases such as amyotrophic lateral sclerosis (ALS) while continuing to focus on innovation and scientific advancement of new medical products to address other critical and rare diseases, the subcommittee should provide the agency with the full authorized funding level.

The Administration's FY 2024 budget proposal also calls for $50 million for FDA to advance the President's Cancer Moonshot goals. These funds would enhance agency-wide efforts to improve evidence generation for underrepresented subgroups in oncology clinical trials. They would also support pragmatic, decentralized trials and the development of sources of evidence that incorporate patient-generated data and RWE.[viii] If appropriated, these resources will assist in the expansion of FDA's efforts to facilitate the approvals of innovative new cancer treatments by international regulatory authorities at the time of FDA approval, thus fostering collaboration on cancer treatments with other countries with standards comparable to those prevailing in the U.S.

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 data collected outside of a clinical trial plays a vital role in answering key questions

about therapeutics, diagnostics, and other healthcare interventions. The proliferation and widespread adoption of electronic health records, as well as other emerging digital health solutions, have made real-world data (RWD) an attractive source of data for clinical and translational research. FDA has a long history of using RWD and RWE to monitor and evaluate the post-market safety of approved medical products. Advances in the availability and analysis of RWD have increased the potential for generating robust RWE to support FDA regulatory decisions.[ix] FDA continues to work to expand the use of fit-for-purpose RWD to generate RWE in regulatory decision-making regarding medical product effectiveness. RWE has even supported some approvals of applications meeting evidentiary standards. FDA has also published a series of foundational draft guidances regarding the use of RWD, including assessing whether RWD, such as registries, are fit for use. These foundational guidances will be followed by further guidance on clinical trial and other study designs using RWD. For this type of information to be useful in achieving the goal of generating and using RWE and RWD for personalized medicine, the data must be of high quality. Increased budget authority for FDA would create a more reliable source of funding for FDA's activities that strengthen programs focused on improving the quality of RWE and RWD.

Fostering Digital Health Technologies and Artificial Intelligence (AI)

CDRH launched the Digital Health Center of Excellence (DHCoE) to help both FDA staff and external stakeholders advance the development and FDA review of digital health technologies.[x] The DHCoE is focused in multiple areas including AI and machine learning (ML). AI/ML technologies have the potential to transform health care by deriving new and important insights from the vast amount of data generated during the delivery of health care every day.[xi] Digital health and AI are becoming 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. Additional appropriated resources would enable FDA to continue developing training programs to clarify regulatory pathways for internal FDA and external digital health stakeholders through the DHCoE. They would also strengthen the agency's partnerships focused on AI/ML and wearable technology, software, and patient-generated health data. Activities in these areas will help patients achieve their goals of getting high quality digital health technologies to patients.

Conclusion

PMC appreciates the opportunity to highlight FDA's importance to the continued success of personalized medicine. A budget authority appropriation for FDA in FY 2024 of $3.914 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] https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/report.pdf
[iv] https://www.fda.gov/media/166182/download
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[viii] https://www.whitehouse.gov/ostp/news-updates/2023/03/09/fact-sheet-cancer-fy24/
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