House Subcommittee on Agriculture, Rural Development, Food and Drug Administration, and Related Agencies Fiscal Year 2024 Appropriations for FDA

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Chairman Harris, Ranking Member Bishop 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. 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]

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. By increasing federal investment in FDA activities fostering the development of innovative medical products Congress can help usher in a new era of personalized medicine.

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.[ii] 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'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 personalized medicine breakthroughs. 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, integrating RWE into reviews, and fostering digital health and artificial intelligence (AI)

technologies. Robust funding from Congress will help 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 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. The $10 million included in the Administration's budget for FY 2024 would allow further investments in enterprise data and IT modernization to streamline data practices across FDA centers and help translate FDA's wealth of information into knowledge supporting progress in personalized medicine.

The agency must also bolster its workforce across each of its centers to keep pace with the growing pipeline of new therapies. Cell and gene therapy holds considerable potential for the treatment of hereditary genetic disorders and more than 200 new gene therapy Investigational New Drug applications are coming to FDA's CBER annually.[iii] CBER provides extensive scientific and regulatory advice and develops policy 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 address 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 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.[iv] By providing $30 million for the FDA's Orphan Products Grant Program to support the development of products to treat orphan or rare diseases, this subcommittee can respond to the documented economic burden rare diseases present to families and the country.[v] Further, Congress passed the *Act for ALS Act* which authorized $2.5 million in funding for FDA.[vi] The subcommittee should provide the agency with the full authorized funding level so FDA can facilitate access to therapies for ALS while continuing to advance new medical products for other rare diseases.

The Administration's FY 2024 budget proposal also calls for $50 million for FDA to advance the President's Cancer Moonshot goals. If appropriated, these resources will assist in the expansion of FDA's efforts to facilitate the approvals of innovative new cancer treatments. These funds would enhance agency-wide efforts to improve evidence generation for underrepresented subgroups in oncology clinical trials and support pragmatic, decentralized trials and the development of sources of evidence that incorporate patient-generated data and RWE.[vii]

Advancing the Use of Real-World Evidence (RWE)

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 and the agency continues work to expand the use of fit-for-purpose RWD to generate RWE in regulatory

decision-making regarding medical product effectiveness. 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)

The Digital Health Center of Excellence (DHCoE) is focused on helping FDA staff and external stakeholders advance the development and review of digital health and AI/machine learning (ML) technologies. 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.[viii] Additional appropriated resources would enable FDA to continue clarifying regulatory pathways for digital health stakeholders and strengthen the agency's partnerships on AI/ML, wearable technology, software, and patient-generated health data. These activities will help FDA achieve their goals of getting high quality health technologies to patients in areas that are becoming increasingly important for personalized medicine.

Conclusion

PMC appreciates the opportunity to highlight FDA's importance to the success of personalized medicine. A budget authority appropriation for FDA in FY 2024 of $3.914 billion will help the agency continue 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://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/report.pdf
[iii] https://www.fda.gov/media/166182/download
[iv] https://www.fda.gov/industry/medical-products-rare-diseases-and-conditions/orphan-products-grants-program
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[vii] https://www.whitehouse.gov/ostp/news-updates/2023/03/09/fact-sheet-cancer-fy24/
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