Fiscal Year 2025 Food and Drug Administration Appropriations

House Appropriations Subcommittee on Agriculture, Rural Development, Food and Drug Administration, and Related Agencies

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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) 2025 appropriations. PMC is a nonprofit education and advocacy organization comprised of more than 200 institutions from across the health care spectrum who support a growing field that depends upon the FDA. As you begin work on FY 2025 appropriations, **we respectfully ask that you provide $3.896 billion to the FDA for budget authority with at least $775 million for human drug programs, $280 million for biologics and cell and gene therapy review programs, and $478 million for devices and radiological health.**

Personalized 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. This approach promises to detect the onset of disease, pre-empt its progression, and improve the quality, accessibility, and affordability of health care.[i]

The Role of the FDA in Personalized Medicine

As of 2023, more than 300 personalized treatments and over 140,000 diagnostic products have been made available to patients.[ii] These numbers continue to grow, with personalized

medicines accounting for more than a quarter of all new drugs approved by the FDA since 2015.[iii]

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. New types of drugs, tools, and technologies leveraging complex and sophisticated data have challenged existing regulatory frameworks and processes.

Facilitating the Development of Personalized Medicine Products

Previous increases in budget authority have enabled the FDA to implement multiple programs facilitating the development of personalized medicine products, which require greater sophistication and expertise to review. The requested FY 2025 budget authority will allow expansion of these initiatives.

Expediting Medical Product Development

As a byproduct of remarkable scientific breakthroughs, the number of cell and gene therapy submissions to the FDA is rising sharply — and this increase is expected to continue. FDA's CBER has been supporting the fast pace of products advancing in clinical development by providing extensive scientific and regulatory advice to product manufacturers throughout the medical product lifecycle, including feedback on manufacturing, preclinical, and clinical topic areas. CBER also develops guidance explaining FDA's interpretation of and policy on a regulatory issue. An increase in FDA's budget authority would allow the agency to continue working with stakeholders to facilitate for key issues limiting the development and application of

cell and gene therapies, thus addressing challenges that make these therapies cost-prohibitive and in some cases not commercially viable.

Treatments are now within reach for many rare diseases, but regulatory activities that support the development of products to treat orphan or rare diseases must be maintained. For example, critical pilot programs for rare diseases such as the Support for clinical Trials Advancing Rare disease Therapeutics (START) program allow select manufacturers of novel drug and biological products to engage with the FDA and facilitate the progression of development programs. FDA's Rare Neurodegenerative Disease Grant Program, established by *Act for ALS*, funded five new awards last year to support efficient natural history or biomarker studies for inheritable pediatric and adult neurodegenerative diseases and increased support for an ongoing large study to include genetic testing and other work needed to increase that study's likelihood of informing future clinical trials. By adequately funding CDER and CBER, Congress will demonstrate an understanding of the documented economic burden rare diseases present to families and the country — and a commitment to reducing it.

The FDA's Oncology Center of Excellence (OCE) continues to advance the Administration's Cancer Moonshot. OCE launched projects which seek to facilitate more diverse trial populations, more rapid enrollment, reduced attrition, and improved dose optimization of cancer treatments to maximize the efficacy and tolerability of cancer therapeutics. If appropriated, FY 2025 resources will bolster FDA's efforts to meet the Cancer Moonshot's goals and speed up progress against deadly and rare cancers.

Advancing the Use of Real-World Evidence (RWE), Digital Health Technologies and Artificial Intelligence (AI)

The proliferation and widespread adoption of electronic health records and digital health solutions have made real-world evidence (RWE) an attractive source for clinical and translational research. FDA continues to work on expanding the use of RWE in regulatory decision making regarding medical product effectiveness. FDA has also published a series of foundational guidances regarding regulatory considerations for use of RWE and established a new Advancing RWE Program for certain drug sponsors to communicate with FDA staff about the use of RWE in medical product development before study initiation.

Innovations in digital health technologies (DHTs) and artificial intelligence (AI) approaches provide new opportunities to derive important insights from the vast amount of data generated by patients and help to individualize care. In FY 2023, FDA published a final guidance on the use of DHTs in clinical investigations and a Framework for the use of DHTs in Drug and Biological Product Development. In May 2023, FDA published a discussion paper, *Using Artificial Intelligence & Machine Learning in the Development of Drug & Biological Products*, to foster a dialogue on the use of AI in medical product development. FDA can continue clarifying pathways for internal and external stakeholders and strengthen agency partnerships focused on AI/ML and DHTs with additional appropriated resources.

Conclusion

A budget authority appropriation for the FDA in FY 2025 of $3.896 billion will help the agency foster the development of innovative medical interventions. By investing in the FDA now Congress can usher in a new era of personalized medicine at a pivotal moment for our health system and patients living with unmet medical needs.

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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/10.1002/ajmg.c.31881
[iii] https://www.personalizedmedicinecoalition.org/wp-content/uploads/2024/02/report-3.pdf