**Fiscal Year 2025 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 2025 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) 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 the subcommittee begins work on the FY 2025 Agriculture, Rural Development, FDA, & Related Agencies appropriations bill, **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.** This level of funding is necessary to position the FDA to provide access to safe and effective medical products, including advances in technologies that are the foundation for the future 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]

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 implement multiple programs facilitating the development of personalized medicine products, which require greater sophistication and expertise to review. An increase in FY 2025 budget authority will allow the FDA to expand these initiatives and launch new ones with 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, real-world evidence (RWE), digital health and artificial intelligence/machine learning (AI/ML), 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 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.[iii] These numbers continue to grow, with personalized medicines

accounting for more than a quarter of all new drugs approved by the FDA since 2015.[iv] More than half of new personalized treatments are now approved for indications outside of oncology. Two commercially available products have new expanded indications to treat very young children with cystic fibrosis (CF) — a vulnerable population with great unmet needs. Another product is now approved for treatment of unresectable or metastatic melanoma in pediatric patients 12 years and older. These new approvals and expanded uses of already approved drugs are helping transform care for molecularly selected subsets of patients with cancer as well as rare, common, and infectious diseases. Continued progress cannot be taken for granted. The FDA must be provided with the necessary resources to remain a responsive regulatory agency.

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 have challenged existing regulatory frameworks and processes.

**Facilitating the Development of Personalized Medicine Products**

The FDA is taking steps to modernize its regulatory processes by, for example, improving clinical trials to address unmet medical needs, integrating RWE into medical product reviews, and fostering the advancement of digital health and 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 Medical Product Development***

As a byproduct of scientific breakthroughs, the number of cell and gene therapy submissions to the FDA is rising sharply — and this increase is expected to continue. Cell and gene therapies have potential to treat serious diseases and conditions, including cancer, genetic diseases, and infectious diseases, with many such products being developed to address unmet medical needs. 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 policy and guidance on novel clinical, scientific, and manufacturing challenges for cell and gene therapy products. The guidance documents explain FDA's interpretation of and policy on a regulatory issue. The documents are primarily for industry but also serve the needs of other stakeholders and internal staff. The number of new gene therapy applications has more than doubled in recent years, with an increasing number yielding marketing applications. An increase in FDA's budget authority would allow the agency to bolster its workforce and continue working with stakeholders to facilitate end-to-end solutions 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 incentives and regulatory pathways must be maintained for all rare diseases to make development financially viable. The FDA

supports the development of products to treat orphan or rare diseases including programs to support clinical trials, natural history studies, and the new authority to fund grants addressing regulatory science challenges. For example, FDA's Accelerating Rare disease Cures (ARC) Program seeks to accelerate and increase the development of safe and effective treatment options that will address the unmet needs of patients with rare diseases by bringing together CDER's collective expertise and activities to provide strategic overview and coordination of CDER's rare disease activities. CDER's ARC program has additionally supported the launch of critical pilot programs for rare disease drug development such as the Support for clinical Trials Advancing Rare disease Therapeutics (START) program. START will allow select manufacturers of novel drug and biological products to benefit from early, more rapid, enhanced, ad hoc communications with the FDA and facilitate the progression of a development program to pivotal clinical study stage or the pre-BLA or pre-NDA meeting stage.

To help expand the development of new scientific approaches and tools available for advancing effective medical products that may prevent, diagnose, mitigate, and treat rare neurodegenerative diseases, Congress passed the *Act for ALS Act*. This bill established the FDA Rare Neurodegenerative Disease Grant Program, which funded five new awards to support efficient natural history or biomarker studies for various rare neurodegenerative diseases in children and adults. One of these awards supports a natural history study for the familial form of amyotrophic lateral sclerosis (ALS) and other very rare motor neuron diseases, which may drive the development of "disease agnostic" digital biomarkers. Another award provides for biomarker studies in each of two inheritable rare neurodegenerative diseases — myotonic dystrophy type 1 and Niemann-Pick type C — both of which cause early death. Last year, FDA also increased support for an ongoing large ALS natural history study funded in FY 2022 to include genetic testing and work needed to increase the 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 Project Pragmatica, which seeks to introduce efficiencies and enhance patient centricity by integrating aspects of clinical trials with real-world routine clinical practice, which can reduce the burden of trial participation. The goal is to facilitate more diverse trial populations, more rapid enrollment, and reduced attrition. The OCE-led Project Optimus, a multi-disciplinary effort to improve dose optimization of cancer drugs and biologics to maximize the efficacy as well as the safety and tolerability of cancer therapeutics, published a draft guidance for industry, *Optimizing the Dosage of Human Prescription Drugs and Biological Products for the Treatment of Oncology Diseases*. If appropriated, FY 2025 resources will bolster FDA's efforts to meet the Cancer Moonshot's goals to speed up progress against deadly and rare adult and pediatric cancers through exploring efforts to improve cancer diagnosis; target the right treatments to the right people; learn from all patients; diagnose cancer sooner; support patients, survivors, and caregivers; and address inequities.

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

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 (EHRs), as well as other emerging digital health solutions, have made real-world data (RWD) an attractive source 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 but FDA requires a more reliable source of funding. FDA continues to work to expand the use of fit-for-purpose RWD to generate RWE in regulatory decision making regarding medical product effectiveness. In 2023, FDA approved a new dosing regimen for a drug to treat pediatric patients with certain types of seizures based on a non-interventional study utilizing data from EHRs. FDA has also published a series of foundational draft guidances regarding the use of RWD and final guidances on regulatory considerations for use of RWE, as well as considerations for use of registries and data standards for submissions containing RWD. The agency established a new Advancing RWE Program to provide another pathway for certain drug sponsors to meet with FDA staff before protocol development or study initiation to discuss the use of RWE in medical product development.

Innovation in digital health technologies (DHTs), including electronic sensors, computing platforms and information technology, provide new opportunities to obtain clinical trial data directly from patients. In FY 2023, FDA published the final guidance on use of DHTs in clinical investigations. The agency's Framework for the use of DHTs in Drug and Biological Product Development details activities for the FDA's DHT Program. AI/ML approaches also 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. Digital health and AI are 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. In May 2023, CDER, CBER, and CDRH published a discussion paper, *Using Artificial Intelligence & Machine Learning in the Development of Drug & Biological Products*, that aims to foster dialogue on the use of AI and ML in drug and biological product development, and the development of medical devices to use with these treatments. Additional appropriated resources will enable FDA to continue clarifying pathways for internal and external stakeholders and strengthen agency partnerships focused on AI/ML, wearable technology, software and patient-generated health data.

## 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 2025 of $3.896 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://doi.org/10.1002/ajmg.c.31881

[iv] https://www.personalizedmedicinecoalition.org/wp-content/uploads/2024/02/report-3.pdf