Fiscal Year 2021 Agriculture, Rural Development, Food and Drug Administration, & Related Agencies Appropriations Testimony

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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) 2021 appropriations. PMC is a nonprofit education and advocacy organization comprised of more than 230 member institutions across the health care spectrum. As the subcommittee begins work on the FY 2021 Agriculture, Rural Development, FDA, & Related Agencies Appropriations bill, **we respectfully ask that you increase the FDA's budget authority appropriations for medical product activities by at least $70 million above the FY 2020 enacted appropriations level.**

We sincerely appreciate the $141 million in no-year monies provided for FDA's response to COVID-19 across two of the recent supplemental appropriations bills. PMC recognizes the crucial role the FDA plays in promoting medical product innovation and protecting public health, which is exemplified in its leadership in expediting the development and authorization of medical countermeasures to COVID-19. We know that FDA will use these funds effectively and appropriately. Our FY 2021 "ask" does not include the FY 2020 supplemental in the agency's base. However, we still urge the Committee to give this ask serious consideration.

PMC supports the FDA's plans for utilizing additional FY 2021 funding to promote the development of innovative medical products and other activities.[i] PMC also thanks the Committee for the increase appropriated to FDA in FY 2020. This increase enabled the FDA to advance programs in the areas of real-world evidence (RWE), digital health, rare disease research, and expedited therapeutic development for patients with unmet medical needs. Additional increases in FY 2021 will allow the FDA to expand these initiatives and launch new ones, which all require a highly skilled and technical workforce. We believe sustained investment in the FDA is necessary so that the agency can keep pace with rapid biomedical innovation and ultimately facilitate patients' access to the latest discoveries and technologies in personalized medicine.

Personalized medicine, also called precision or individualized medicine, is an evolving field in which physicians use diagnostic tests to identify specific biological markers, often genetic, that help determine which medical treatments and procedures will work best for each patient. By combining this information with an individual's medical history, circumstances, and values, personalized medicine allows doctors and patients to develop targeted treatment and prevention plans. Personalized health care has the capacity to detect the onset of disease at its earliest stages, pre-empt the progression of disease, and, at the same time, increase the efficiency of the health care system by improving quality, accessibility, and affordability.[ii]

I.) The Role of the FDA in Personalized Medicine

Thanks in part to an intentionally supportive regulatory environment in the U.S., personalized medicine is a rapidly evolving field. For each of the past five years, personalized medicines have accounted for a quarter or more of the new drugs approved by FDA, with a record of 42 percent in 2018.[iii] In 2005, personalized medicines accounted for only 5 percent of new drug approvals.[iv]

The most recent approvals address the root causes of rare diseases in many patients for whom there were no options before, including those diagnosed with spinal muscular atrophy, Duchenne muscular dystrophy, acute hepatic porphyria, cystic fibrosis, and sickle cell disease. As of 2020, more than 180 personalized medicines are on the market and available for patients,[v,vi] and the field shows no signs of slowing down: In 2018, 55 percent of all oncology trials involved the use of biomarkers, compared to 15 percent in 2000,[vii] and as of January 2020, FDA has over 900 active Investigational New Drug (IND) applications for gene therapies.[viii]

The FDA is the gateway for personalized medicines 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) all have individual responsibilities for evaluating medical products for their safety and efficacy. As a more personalized approach to treatment has grown, new types of drugs, tools, and technologies using genetic information have challenged existing regulatory frameworks and processes.

II.) Facilitating the Development of Personalized Medicine Products

The FDA is taking a number of steps to modernize its regulatory processes, such as streamlining its organizational and technical infrastructure to shorten review times, improving clinical trials in oncology and rare disease, integrating RWE into medical product reviews, and building frameworks to regulate digital health and artificial intelligence (AI) technologies. To keep pace with rapid scientific innovation, robust funding is needed to help the FDA build upon this work and bring personalized medicines 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. Re-organizations at the agency in 2019 streamlined communication between the FDA center directors, other senior leadership, and the commissioner. CDRH also integrated 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.[ix] Additional funding would enable the agency to continue its *Technology Modernization Action Plan* to develop an integrated, modernized knowledge management system that can provide rapid access to and in-depth analysis of information submitted to FDA pre- and post-market.[x] Such a system promises to make medical product reviews, post-market surveillance, and cybersecurity efforts more efficient and informative.

The agency must also bolster its workforce to keep pace with the growing pipeline of gene therapies. By 2020, the FDA anticipated that it will be receiving more than 200 gene therapy INDs per year. By 2025, the agency anticipates it will be approving 10 to 20 cell and gene therapy products per year.[xi] The scientific review of gene therapies requires the evaluation of highly complex information and, thus, reviewers with highly specific expertise. CBER's eventual goal is to add about 50 clinical reviewers to the group charged with overseeing the clinical investigation, development, and review of these products. Additional funding would help FDA grow its workforce accordingly.

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 medicines may be delayed or discarded because FDA cannot afford to run trials needed to validate them. To move towards a more agile clinical research enterprise, in 2019, the FDA released guidance documents on enrichment strategies for clinical trials as well as broadening patient eligibility in oncology clinical trials to be more representative of real-world oncology care.[xii]

In 2019, the FDA's Orphan Products Clinical Trials Grant program also funded twelve new research grants supporting product development to meet the needs of patients impacted by a variety of rare diseases, mainly those affecting children and cancers.[xiii] During its 35-year history, the program has supported research leading to approvals of more than 60 treatments for rare diseases, including Duchenne Muscular Dystrophy and sickle cell disease.[xiv] Due to advances in personalized medicine, the complexity of the science associated with orphan drugs is increasing and, as a result, so are the costs of trials. Additional resources would ensure the FDA continues supporting clinical trial networks that create an understanding of the clinical outcomes of rare diseases and the progression of symptoms over time, which the agency would leverage when promising medical products are identified for patients.

Advancing the Use of Real-World Evidence (RWE)

Traditional post-market studies require years to design and complete and cost millions of dollars.[xv] The use of medical data collected outside of a clinical trial, or RWE, presents significant opportunities to decrease these costs and improve patient access to personalized medicine. FDA's Sentinel System and NEST (National Evaluation System for health Technology) programs are helping FDA monitor the safety of approved medical products. In FY 2019, Congress provided the initial funding for NEST,[xvi] which would link and synthesize data from different sources across the medical device landscape, including clinical registries, electronic health records and medical billing claims to generate RWE. FDA since announced its participation in the NEST Coordinating Center Collaborative Community, a forum of private- and public-sector members committed to developing methodologies behind the system.[xvii] Additional funding to expand these RWE programs would enable FDA to fill data gaps in existing surveillance systems, improve product review, and improve the quality of RWE available to health care providers and patients to make better 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 of RWE. In 2019, FDA issued guidance documents clarifying how the FDA sees its role in advancing safe and effective digital health technologies through a risk-based approach.[xviii] FDA also announced steps toward a new regulatory framework for medical devices using advanced AI algorithms.[xix] 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. Additional funding would increase FDA's ability to create a program, similar to its digital health

program, focused specifically on advancing and promoting the development of consumer-friendly AI technologies.[xx] The 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. The *Cures Act* authorized $70 million in FY 2021 for the FDA through the Innovation Account to help further modernize drug, biological, and device product development and review; to create greater efficiencies and predictability in product development and review; and to hire and retain scientific, technical, and professional experts in specialized areas.[xxi] The *Cures Act* 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. As annual allocations to the Innovation Account start to taper off, the science behind product development continues to increase in complexity. Increases in the FDA's budget authority appropriations, in addition to funding authorized to the Innovation Account, 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 effectively evaluate novel personalized medicines and technologies.

III.) 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 2021 that is at least $70 million above the FY 2020 level for medical product activities, in addition to the $70 million scheduled for the *Cures Act* Innovation Account, will help the agency chart an efficient path for advancing innovative medical product development and bring us closer to a future where every patient benefits from an individualized approach to health care.

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i https://www.fda.gov/media/135078/download
ii http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
iii http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report
iv http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report
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