Fiscal Year 2019 Agriculture, Rural Development, Food and Drug Administration, & Related Agencies Appropriations Testimony

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Chairman Hoeven, Ranking Member Merkley 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) 2019 appropriations. PMC is a nonprofit education and advocacy organization comprised of more than 200 institutions across the health care spectrum. PMC recognizes the crucial role the FDA plays in promoting medical product innovation. As the subcommittee begins work on the FY 2019 Agriculture, Rural Development, FDA, & Related Agencies Appropriations bill, we ask that you increase the FDA's budget authority for medical product activities by $473 million as proposed in the President's FY 2019 budget request.

PMC supports FDA Commissioner Scott Gottlieb's plans for utilizing additional FY 2019 funding[1] to expedite the development of new therapies for patients with unmet medical needs; to advance the use of real-world evidence; to foster the growth of digital health technologies; and to enhance research on rare diseases. We believe that investing in these initiatives and strengthening the FDA's workforce will facilitate patients' access to personalized medicine products.

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 records, circumstances, and values, personalized medicine allows doctors and patients to develop targeted treatment and prevention plans.[2] 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.[3]

I.) The Role of the FDA in Personalized Medicine

Personalized medicine is a rapidly growing field. A 2015 study found that companies nearly doubled their R & D investment in personalized medicines over five years and expect to increase their investment by an additional third over the next five years.[4] According to the same study, biopharmaceutical researchers also predict a 69 percent increase in the number of personalized medicines in development over the next five years.

[1] https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm596554.htm
[2] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[3] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[4] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/pmc-phrma-personalized-medicine-investment-21.pdf

The number of personalized medicines approved by the FDA per year has increased from 5 percent of new drugs in 2005[5] to 33 percent in 2017.[6] For the past four years, personalized medicines have accounted for more than a quarter of new drugs approved by the FDA each year.[7]

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, technologies, and therapies using genetic information have challenged existing regulatory frameworks. In 2017, for example, FDA set six regulatory precedents including: the record approval of 16 new personalized medicine drugs; the approval of the first three gene therapies, two of which were cancer immunotherapies; the first approval of a tissue-agnostic cancer therapy; the first authorization for marketing of health-related genetic tests directly to consumers; the first approval of a personalized medicine biosimilar; and the first joint approval and coverage decision between the FDA and the Centers for Medicare & Medicaid Services (CMS) for a next-generation sequencing test.[8] Robust funding is needed to help the FDA keep pace with rapid biomedical innovation and facilitate access to personalized medicine.

II.) Facilitating the Development of New Personalized Medicine Products

By passing the *21st Century Cures Act (The Cures Act)*, Congress acknowledged the need for an additional focus on and funding for FDA. The *Cures Act* authorized FDA to help further modernize drug, biological, and device product development and review, and to create greater efficiencies and predictability in product development and review. The *Cures Act* also improved the FDA's ability to hire and retain scientific, technical, and professional experts in specialized areas. As preventive, diagnostic, therapeutic, and analytical methods become more complex, these experts will help FDA approve novel products based on high-quality evidence. The following areas, which received attention from Congress during consideration of the *Cures Act*, present opportunities across the FDA to bolster personalized medicine.

Expediting Product Development

Congress called on the FDA to establish one or more inter-center institutes to help develop and implement processes for coordination of activities in major disease areas between the drug, biologics, and device centers. FDA established the Oncology Center of Excellence (OCE) in January of 2017 to create a unified policy approach and clinical review for all drugs, biologics, and devices used in medical oncology. OCE leverages the combined talents and skills of all FDA regulatory scientists and reviewers who work in medical oncology product review and serves as a single point of contact for external stakeholders for FDA's work in cancer. OCE helps expedite the development of oncology and hematology medical products and supports an integrated approach to the clinical evaluation of drugs, biologics, and devices for the treatment of cancer. In its first year, OCE was essential to FDA's approval of two cell-based gene therapies and three in

[5] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[6] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PM_at_FDA_2017_Progress_Report.pdf
[7] http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report
[8] http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report

vitro diagnostic tests. If properly resourced, the Center of Excellence model is one that could be transformative in other disease areas with unmet need.

Enhancing Research on Rare Diseases

Identifying the biological markers of rare diseases presents great opportunity for new treatments and cures. FDA's Orphan Products Grants Program recently provided $17 million in funding to support 15 new clinical trials on products for rare diseases, and for the first time the program funded natural history studies, or studies looking at patient experiences and the progression of symptoms over time.[9] Two of these studies examine biological markers and could provide key information for product development about how rare diseases progress.[10] Additional resources would help the FDA develop clinical trial networks to create an understanding of the natural history and clinical outcomes of rare diseases, which FDA would leverage when promising medical products are identified for patients.[11]

Fostering Digital Health Technologies

Data-capturing technological devices, or digital health technologies, can play a key role in the collection of real-world evidence. Currently, FDA is piloting pre-certification, or one-time premarket review, for lower-risk digital health technologies, similar to the FDA's new approach to oversight of direct-to-consumer genetic health risk tests.[12] FDA would use the additional funding to create a Center of Excellence on Digital Health that would build new capacity to evaluate and recognize third-party certifiers as well as support a cybersecurity unit.[13] These efforts to streamline and design regulatory pathways around specific technologies will facilitate patient access to the latest technologies. The foundation laid at the FDA for digital health 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.

Advancing the Use of Real-World Evidence

The use of medical data collected outside of a clinical trial, or real-world evidence, presents significant opportunities to improve patient access to personalized medicine. Because real-world evidence has the potential to provide information on populations that are not always captured in clinical trials conducted in traditional settings, the *Cures Act* required FDA to evaluate the potential use of real-world evidence to support the approval of new indications for already approved drugs or to help support or satisfy post-approval study requirements. In addition to the real-world evidence work already underway to fulfill the *Cures Act* provisions for drugs, the National Evaluation System for health Technology Program, which is directed by CDRH in collaboration with medical device stakeholders, promises to drive down the time and cost of real-world data collection and analysis for medical devices.[14] Despite enthusiasm for real-world

[9] https://blogs.fda.gov/fdavoice/index.php/2018/02/taking-new-steps-to-meet-the-challenges-of-rare-diseases-fda-marks-the-11th-rare-disease-day

[10] https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm579375.htm

[11] https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm596554.htm

[12] https://www.fda.gov/downloads/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDRH/CDRHVisionandMission/UCM592693.pdf

[13] https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm596554.htm

[14] https://www.fda.gov/downloads/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDRH/CDRHVisionandMission/UCM592693.pdf

evidence and efforts to establish a framework and methodologies for applying real-world evidence in the regulatory context, additional funding is needed to support systems that enable the FDA to use real-world evidence for purposes beyond adverse event monitoring.

The *Cures Act* included more than 50 sections requiring FDA involvement.[15] For FY 2019, the *Cures Act* authorized $70 million for the FDA through the Innovation Account. Appropriating these funds is important, but we contend that this amount of funding alone is insufficient to fully support the agency. Activities that fall outside the scope of the *Cures Act* include CDRH's plans to develop final and draft guidances on next-generation sequencing and the co-development of diagnostic tests with therapeutic products.[16] These efforts to reduce uncertainties surrounding regulatory oversight of diagnostic tests will help streamline the path to market for personalized medicine products which are invariably informed by a diagnostic test.

III.) Conclusion

PMC appreciates the opportunity to highlight the FDA's importance to the success of personalized medicine. Additional budget authority appropriations for the FDA in FY 2019 will help the agency maintain a highly skilled workforce that charts an efficient path for advancing innovative medical product development. The subcommittee's support for a $473 million increase in budget authority appropriations will bring us closer to a future where every patient benefits from an individualized approach to health care. PMC looks forward to working with you as you contemplate the appropriate levels of funding for the FDA in FY 2019 and we will gladly provide additional information on the programs described in our testimony upon request.

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[15] https://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/ScienceBoardtotheFoodandDrugAdministration/UCM556618.pdf
[16] https://www.fda.gov/medicaldevices/deviceregulationandguidance/guidancedocuments/ucm580172.htm