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

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Chairman Aderholt, 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) 2019 appropriations. PMC is a nonprofit education and advocacy organization comprised of more than 200 institutions across the health care spectrum. **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[i] to expedite the development of new therapies for patients with unmet medical needs; to enhance research on rare diseases; to foster the growth of digital health technologies; and to advance the use of real-world evidence. 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 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.[ii] Personalized health care promises to detect the onset of and pre-empt the progression of disease as well as improve the quality, accessibility, and affordability of health care.[iii]

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.[iv] 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. In addition, the number of personalized medicines approved by FDA per year has increased from 5 percent of new drugs in 2005[v] to 33 percent in 2017,[vi] and personalized medicines have accounted for more than a quarter of new drugs approved by FDA each year for the past four years.[vii]

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' 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, among others: the record approval of 16 new personalized medicine drugs; the approval of the first three gene therapies, two of which were cancer immunotherapies; and the first joint approval and coverage decision between the FDA and Centers for Medicare & Medicaid Services (CMS) for a next-generation sequencing test.[viii] Robust funding will help 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 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 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. In its first year, OCE was essential to FDA's approval of two cell-based gene therapies and three in vitro diagnostic tests. If properly resourced, the COE model 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.[ix] Two of these studies examine biological markers and could provide key information for product development about

how rare diseases progress.[x] Additional resources would help FDA develop clinical trial networks to better understand the natural history and clinical outcomes of rare diseases, which FDA would leverage when promising medical products are identified for patients.[xi]

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.[xii] 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.[xiii] As patients assume a larger role in managing their own health care and are more informed by their genomic data captured by these devices, FDA's efforts to streamline and design regulatory pathways around specific technologies will facilitate patient access to the latest technologies, and consequently, to personalized medicine.

Advancing the Use of Real-World Evidence: The use of medical data collected outside of a clinical trial, or real-world evidence (RWE), presents significant opportunities to improve patient access to personalized medicine. Because RWE has the potential to provide information on populations that are not always captured in traditional clinical trials, the *Cures Act* required FDA to evaluate the potential use of RWE 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 RWE 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.[xiv] Despite enthusiasm for RWE and efforts to

establish a framework and methodologies for using RWE in regulatory oversight, additional funding is needed to enable FDA to use RWE beyond adverse event monitoring.

The *Cures Act* included more than 50 sections requiring FDA involvement.[xv] For FY 2019, the *Cures Act* authorized $70 million for FDA through the Innovation Account. Appropriating these funds is important, but this amount of funding alone is insufficient to fully support FDA. 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.[xvi] These efforts to reduce uncertainties surrounding regulatory oversight of diagnostic tests will 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. 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.

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[i] https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm596554.htm
[ii] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[iii] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[iv] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/pmc-phrma-personalized-medicine-investment-21.pdf
[v] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[vi] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PM_at_FDA_2017_Progress_Report.pdf
[vii] http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report
[viii] http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report
[ix] 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
[x] https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm579375.htm
[xi] https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm596554.htm
[xii] https://www.fda.gov/downloads/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDRH/CDRHVisionandMission/UCM592693.pdf
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