Fiscal Year 2020 Senate Appropriations Committee Outside Witness Testimony

Submitted by
Cynthia A. Bens, Senior Vice President, Public Policy, Personalized Medicine Coalition
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Prepared for the
Subcommittee on Agriculture, Rural Development, Food and Drug Administration, and Related Agencies

Regarding
Food and Drug Administration Fiscal Year 2020 Appropriations

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) 2020 appropriations. PMC is a nonprofit education and advocacy organization comprised of more than 230 institutions across the health care spectrum. PMC recognizes the crucial role the FDA plays in promoting medical product innovation and protecting public health. As the subcommittee begins work on the FY 2020 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 $316 million in additional programming as requested in the President's budget.**

PMC supports the FDA's plans for utilizing additional FY 2020 funding[1] to promote the development of innovative medical products and implement the 21st Century Cures Act, among other activities. PMC also thanks the Committee for the increase appropriated to FDA in FY 2019. 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.[2] Additional increases in FY 2020 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.[3] Personalized health care has the capacity to detect the onset of disease at its earliest

[1] https://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/BudgetReports/UCM633738.pdf
[2] https://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/BudgetReports/UCM603315.pdf
[3] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf

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.[4]

I.) The Role of the FDA in Personalized Medicine

Personalized medicine is a rapidly evolving field. The number of personalized medicines approved by the FDA annually has increased from 5 percent of new drugs in 2005[5] to a record 42 percent in 2018.[6] For each of the past four years, personalized medicines have accounted for more than a quarter of new drugs approved by the FDA.[7] Of the 25 new personalized medicines approved in 2018, 24 benefited from a form of expedited FDA review pathway, such as priority review, fast-track, or breakthrough therapy designations.[8]

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 2018, for example, FDA set a number of regulatory milestones including: the record approval of 25 new personalized medicine drugs; the second approval of a tissue-agnostic cancer therapy based on a biomarker instead of tumor type; the first approval of a therapy from a new class of personalized drugs called small interfering ribonucleic acid (siRNA) treatments; the first marketing authorization for pharmacogenetic and cancer risk-related genetic tests sold directly to consumers; and the recognition of the first public database to support regulatory oversight of genetic variant/disease claims: Clinical Genome Resource (*ClinGen*) database.[9] As these precedents demonstrate, the scope of the FDA's regulatory processes are changing.

II.) Facilitating the Development of Personalized Medicine Products

The FDA is taking a number of steps to modernize its regulatory processes, such as increasing the use of expedited approval pathways, integrating RWE into medical product reviews, building a framework for digital health regulation, and improving clinical trials. 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

One way that the FDA is expediting product development is through the FDA's Breakthrough Devices Program for unmet medical needs. This program was established in 2016 by the *21st Century Cures Act (Cures Act),* and it has improved patient access to novel technologies.[10] In 2018, FDA received twice as many breakthrough designation requests than 2017.[11] FDA

[4] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[5] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[6] http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report
[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
[9] http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report
[10] https://www.fda.gov/NewsEvents/Testimony/ucm614607.htm
[11] https://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/BudgetReports/UCM633738.pdf

estimates that its Breakthrough Devices program will increase as much as 20 percent per year over the next 10 years, requiring additional resources to support its growth.[12]

FDA continues to take actions across the agency that enable the agency to make more rapid decisions and improve communication to facilitate the success of expedited pathways. Re-organization at the agency in 2019 streamlined communication between the FDA center directors, other senior leadership, and the FDA Commissioner. CDRH also integrated premarket and postmarket programs to transition to a total product lifecycle approach to device evaluation and monitoring.[13] These are all positive steps, but, to accommodate the expected increase in novel medical products, additional funding is needed for FDA to develop additional systems, including a knowledge management system for drugs and devices, that can provide rapid access to and in-depth analysis of information submitted to FDA pre- and post-market. Investments in these systems made now will make the review of product applications and significantly more efficient.

Modernizing Clinical Trials

More rare diseases and cancers are being defined by biological markers, making smaller groups of patients who are more likely to respond to targeted treatments candidates for participation in trials. Trials that rely on identification of patients by biological markers, such as enriched trials and trials with master protocols, 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 can not afford to run trials needed to validate them.[14] In addition to issuing guidances on these approaches in 2018, FDA's Natural History Grants Program funded six new natural history studies for rare diseases, or studies looking at patient experiences and the progression of symptoms over time.[15] Additional resources would help the FDA support 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.[16]

Advancing the Use of Real-World Evidence (RWE)

Traditional post-market studies require years to design and complete and cost millions of dollars.[17] 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. FDA's Sentinel System and National Evaluation System for health Technology (NEST) programs, using largely claims data and registry data, respectively, are helping FDA monitor the safety of approved medical products. This year FDA announced a five-year strategy to expand the Sentinel Program to access real world data and utilize advances in data science.[18] Additional funding would enable FDA to build on these programs and launch in 2020 a New Medical Data Enterprise to conduct near-real-time evidence evaluation of the electronic health records for at

[12] https://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/ScienceBoardtotheFoodandDrugAdministration/UCM556618.pdf
[13] https://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDRH/ucm633229
[14] https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm633500
[15] https://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/BudgetReports/UCM633738.pdf
[16] https://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/BudgetReports/UCM633738.pdf
[17] https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm627760.htm
[18] https://www.fda.gov/NewsEvents/Newsroom/FDAVoices/ucm629326.htm

least 10 million individuals. This program would cover data gaps in Sentinel and NEST for FDA-regulated products not currently easily assessed using existing systems and promises to better inform product review and ultimately patient care.[19]

Fostering Digital Health Technologies

Data-capturing technological devices, or digital health technologies, can play a key role in the collection of RWE. In 2018, FDA moved forward with its plan to update its regulatory approach to digital health by advancing its pilot pre-certification program, or one-time premarket review, for lower-risk digital health technologies and by launching the *FDA Premarket Digital Safety Program* and *Digital Health Incubator*. Additional funding would enable the agency to build greater capacity to evaluate and recognize third party certifiers and create a cybersecurity unit.[20] These efforts to streamline and design flexible regulatory pathways around specific technologies will facilitate patient access to the latest technologies. These initiatives may also 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. 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.

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 $75 million in FY 2020 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.[21] 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. Annual allocations to the Innovation Account will begin to taper off after FY 2020; however, the science behind product development will continue to increase in complexity. Increases in the FDA's budget authority appropriations are also 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. Additional budget authority appropriations for the FDA in FY 2020 will help the agency chart an efficient path for advancing innovative medical product development. The subcommittee's support for a $316 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 2020, and we will gladly provide additional information on the programs described in our testimony upon request.

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[19] https://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/BudgetReports/UCM633738.pdf

[20] https://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/BudgetReports/UCM633738.pdf

[21] https://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/ScienceBoardtotheFoodandDrugAdministration/UCM556618.pdf