PMC PERSONALIZED MEDICINE COALITION

Talking Points: FY 2019 Appropriations for the NIH and FDA

NIH Funding

- **The Personalized Medicine Coalition requests that the National Institutes of Health (NIH) receive an increase of at least $2.2 billion above the final Fiscal Year (FY) 2018 funding level, totaling at least $38.4 billion,** to maintain the level trajectory of budget increases for the NIH of $2 billion each year; account for biomedical inflation; and ensure that the Innovation Accounts provided through the 21st Century Cures Act supplement the agency's base budget through dedicated funding to specific programs, as intended.

- Investments made in **biomedical research** are critical for finding cures and treatments for diseases that affect millions of Americans. The NIH conducts research that is too expensive and risky for the private sector to undertake alone, and this research has led to major achievements in the understanding of rare diseases and disorders, as well as historically prevalent diseases like Alzheimer's, cancer, and Parkinson's.

- The NIH's **Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) programs** invest millions into health and life science companies creating new therapies. This funding incentivizes industry to conduct high-risk research on potential new drugs and therapies.[1]

- In addition to improving the lives of millions, NIH funding supports over 400,000 non-federal scientists and technical personnel at 2,500 research universities and facilities nationwide. The work of these individuals drives the demand for medical supplies and research equipment. NIH funding ripples far beyond its headquarters in Bethesda, Maryland, to benefit **researchers, manufacturers and suppliers** in every state.

- From 2003 to 2015, the NIH lost more than 20 percent of its purchasing power. This created a hyper-competitive environment in which less than 20 percent of grant applications for highly meritorious research are funded, making it difficult to retain and recruit new- and mid-career biomedical researchers.[2] In order to groom the next generation of researchers, the NIH launched the **Next Generation Researchers Initiative**. Additional funding will help the NIH allocate portions of its base budget to this program, which intends to increase support for early- and mid-career researchers to $1.1 billion over five years.[3]

[1] See https://ncats.nih.gov/smallbusiness/priorities
[2] https://www.nih.gov/about-nih/who-we-are/nih-director/testimony-implementation-21st-century-cures-act-progress-path-forward-medical-innovation
[3] https://www.nih.gov/about-nih/who-we-are/nih-director/fiscal-year-2018-budget-request

- The ***All of Us™*** **Research Program** will collect genetic and health information from one million volunteers for a decades-long research project. To ensure a dataset inclusive of all Americans, in the past year the *All of Us™* Research program awarded its first four community partner awards to organizations well-positioned to engage and enroll communities usually underrepresented in biomedical research, including seniors, Hispanics and Latinos, African Americans, and the LGBTQ community.[4] This program will create an invaluable biomedical data set informing the development of new personalized medicines.

- The **Cancer Moonshot** has given 142 awards, each of which help establish national and international collaborations, to transform the way cancer research is conducted, such as the Partnership for Accelerating Cancer Therapies (PACT), a five-year public-private collaboration between the NIH and 11 leading biopharmaceutical companies to identify, develop and validate standardized biological markers of cancer to advance new immunotherapy treatments.[5] By sharing resources and research, these collaborations will facilitate new discoveries that bring treatment to cancer patients.

- After decades of NIH-funded research in genetics, gene editing is now possible. However, in order to develop new therapies utilizing this technology and hone its methods, more research and tools are needed. In January 2018, the NIH launched the **Somatic Cell Genome Editing program** to research these technologies and educate the scientific community.[6]

- Through the **Accelerating Medicines Partnership**, a public-private partnership between the NIH, the U.S. Food and Drug Administration (FDA), 12 biopharmaceutical and life science companies, and 13 non-profit organizations, the NIH is leading an effort to change the current model for the development of new diagnostics and treatments. Industry and non-profit participants fund 26% of the program, and the collaboration promises to shorten timelines, cut costs, and increase the success rates of new personalized medicines.[7]

FDA Funding

- **The Personalized Medicine Coalition supports the President's proposed increases of $473 million in budget authority for medical product activities at the FDA with a total funding of $3.3 billion (exclusive of $2.5 billion in user fees)** to strengthen the FDA's workforce and advance the availability of innovative drugs and medical devices. The

[4] https://www.nih.gov/about-nih/who-we-are/nih-director/testimony-implementation-21st-century-cures-act-progress-path-forward-medical-innovation
[5] https://www.nih.gov/about-nih/who-we-are/nih-director/testimony-implementation-21st-century-cures-act-progress-path-forward-medical-innovation
[6] https://www.nih.gov/news-events/news-releases/nih-launch-genome-editing-research-program
[7] https://www.nih.gov/research-training/accelerating-medicines-partnership-amp

items proposed include: improvements in drug and device manufacturing, advances in the use of real-world evidence in medical product development, and revisions to the regulatory framework for digital health technology.[8]

- Investments in FDA's staffing needs come at a critical time for personalized medicine. More than 80 percent of FDA's budget is dedicated to personnel needs. Additional funding will ensure the agency can secure and retain the highly trained **workforce** required to carry out its mandate and strategic priorities through 2020.

- Thanks in part to user-fee funding, the annual number of novel devices approved by FDA quadrupled from 2009 to 2017.[9] The number of personalized medicines approved by FDA each year has also increased from 5% in 2005 to 26% in 2016.[10] Additional funding will allow FDA to **improve therapies available to patients and lower medical costs** by facilitating the development of new therapies for unmet medical needs, advancing the use of real-world evidence, and fostering the growth of digital health technologies.

- The use of real-world evidence presents significant opportunities to improve patient access to personalized medicine. The **National Evaluation System for health Technology (NEST)**, a program directed by the FDA Center for Devices and Radiological Health (CDRH) in collaboration with medical device stakeholders, promises to drive down the time and cost of real-world data collection and analysis.[11] However, additional funding is needed to support new systems at FDA enabling the agency to use real-world evidence in deciding on expanded use indications, new clearances, and new approvals.

- Using real-world evidence sometimes requires data from digital health technologies. 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.

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[8] See https://strengthenfda.org/2018/03/03/making-the-case-the-alliances-ask-for-fy-19/

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

[10] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf

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

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

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

- In accordance with priorities established in the 21st Century Cures Act, FDA is working to strengthen its relationships with the patient community and incorporate their perspectives into product development. For example, as part of its 2020 strategic priorities, CDRH intends to establish at least 10 **collaborative communities**, forums of public and private sector members including patients, to solve regulatory issues for medical devices.[14]

- In the coming year, CDRH plans to develop final and draft guidances on several issues related to personalized medicine, including next-generation sequencing and the co-development of diagnostics with therapeutic products.[15] These efforts to reduce uncertainties surrounding regulatory oversight will **streamline the path to market** for personalized medicine products.

The Basics of Personalized Medicine

- **Definition of 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 records, circumstances, and values, personalized medicine allows doctors and patients to develop targeted treatment and prevention plans.[16]

- **Benefits of personalized medicine:** 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.[17]

- **The challenge:** Brining personalized medicine to every patient will require the health care system to transform away from one-size-fits-all, trial-and-error medicine toward a new, targeted approach that utilizes patients' molecular information to inform health care decisions.[18]

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

[15] https://www.fda.gov/medicaldevices/deviceregulationandguidance/guidancedocuments/ucm580172.htm
[16] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[17] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf
[18] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The-Personalized-Medicine-Report1.pdf

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The Growth of Personalized Medicine in Statistics

- In 2003, the NIH announced the completion of the Human Genome Project. Now, for the past four years, personalized medicines have accounted for more than one of every four new drugs approved by the FDA.[19]

- In 2017, FDA set six regulatory precedents, including the approval of the first three gene therapies, two of which were cancer immunotherapies, the first approval of a tissue agnostic indication for cancer therapy, first authorization for marketing of health-related genetic tests directly to consumers, first approval of a personalized medicine biosimilar, and the first FDA/CMS joint approval and coverage decision for a next-generation sequencing test.[20]

- Biopharmaceutical companies nearly doubled their R&D investment in personalized medicines over five years, and expect to increase their investment by an additional 1/3 over the next five years.[21]

- Biopharmaceutical researchers predict a 69% increase in the number of personalized medicines in development over the next five years.[22]

- A survey of leading manufacturers of personalized medicine companies identified scientific discovery as the biggest challenge facing personalized medicine, followed closely by regulatory and reimbursement barriers.[23]

- The number of personalized medicines approved by the FDA per year has increased from 5% of new molecular entities in 2005[24] to 33% in 2017.[25]

[19] http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report
[20] http://www.personalizedmedicinecoalition.org/Resources/Personalized_Medicine_at_FDA_An_Annual_Research_Report
[21] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/pmc-phrma-personalized-medicine-investment-21.pdf
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[25] http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PM_at_FDA_2017_Progress_Report.pdf