DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration
Silver Spring, MD 20993

STATEMENT

OF

SCOTT GOTTLIEB, M.D.

COMMISSIONER OF FOOD AND DRUGS
FOOD AND DRUG ADMINISTRATION

DEPARTMENT OF HEALTH AND HUMAN SERVICES

BEFORE THE

SUBCOMMITTEE ON AGRICULTURE, RURAL DEVELOPMENT, FOOD AND DRUG ADMINISTRATION, AND RELATED AGENCIES

UNITED STATES SENATE

April 24, 2018

Good afternoon Chairman Hoeven, Ranking Member Merkley, and Members of the Subcommittee. Thank you for the opportunity to appear before you today to discuss the President's Fiscal Year (FY) 2019 Budget request for FDA.

First, I would like to thank the Committee for your continued support of the Agency. FDA has received strong bipartisan support throughout the appropriations process in recent years and FY 2018 was no different. I believe this support reflects our shared commitment to the vital role FDA has protecting and promoting the public health. The funding this Subcommittee provides is essential to the Agency fulfilling its mission. The professional staff of FDA is grateful for the support of their work and the funding increases the Subcommittee provided FDA in FY 2018.

Last year was a record year for medical products at FDA in several ways: novel drug approvals, generic drug approvals, and novel medical device approvals. The record number of approvals in 2017 reflects the remarkable opportunities we have at this critical inflection point in science and technology. Advances in medicine and science are providing opportunities that can fundamentally change health in America and the outlook for many patients with life threatening and chronic diseases. At the same time that we have many new opportunities offered by advanced medical technologies; we cannot lose sight of our commitment to the public health basics – reducing smoking rates, preventing kids from initiating use of tobacco products, supporting healthy food choices that can lead to better nutrition and reduced risk of disease, and increasing vaccination rates. FDA is committed to all of these and other public health goals. With your continued support, we have more opportunity to deliver on the promises of science than at any other time in the past.

The funding provided in the President's FY 2019 Budget (Budget) will allow the Agency to sustain its current work—protecting the safety of the food and medical products consumers use every day—and build on these efforts by requesting additional resources to make significant progress on several important fronts; including, fostering innovation and competition to bring better and more

affordable products to market, combatting the opioid epidemic, and implementing the 21st Century Cures Act (Cures).

Overall, the Budget requests $5.8 billion in total resources for FDA—which is an increase of $663 million or 13 percent above the FY 2018 Annualized Continuing Resolution. At this total level, the Budget includes an increase of $473 million in budget authority and an increase of $190 million in user fees. The Budget requests considerable new resources for FDA and makes significant new investments in advancing critical areas of science, domestic technology, and public health.

As the regulatory Agency responsible for ensuring the safety and effectiveness of more than $2.4 trillion worth of products used by consumers, I remain steadfast that these funds are critical investments in our public health agency. They will allow us to more efficiently advance safe and effective new opportunities to Americans.

I. FY 2019 Initiatives

The Budget includes an increase of approximately $400 million in additional resources to advance initiatives to promote public health and spur growth in the domestic economy. These new initiatives provide a renewed focus to some of the Agency's most intensely followed issues—facilitating regulatory pathways to increase patients' access to safe and effective drugs, biologics, and medical devices, and access to higher quality compounded drugs for the patients who need them; better informing patients and providers about pre-and post-market safety of medical products; and, increasing competition among generic drugs in order to lower the cost of generic drugs.

I believe that these new efforts represent discrete areas where targeted additional resources can help the Agency make a meaningful difference for American consumers. A few examples of the new initiatives are described below.

A. Advancing Modern Drug and Biological Product Manufacturing Technologies, Through the Development of Efficient Regulatory Pathways

Advanced manufacturing technologies, such as continuous manufacturing, can improve the agility, flexibility, cost and robustness of drug and biologic manufacturing processes. These technologies have great potential to accelerate new, more targeted therapies, enhance product quality and bolster stability in the U.S. drug supply to meet patient needs. For example, continuous manufacturing has the potential to help address and eliminate drug shortages and reduce recalls related to problems with product or facility quality. Further, with continuous manufacturing platforms for biologics, vaccine manufacturing, for example, could be ramped up on short notice, and vaccines themselves adapted over a shorter time period to address infectious diseases, such as the flu. This would help address the challenge we face annually with the flu season. As a result of the long manufacturing cycle required when using traditional egg based manufacturing technologies, manufacturers must select the flu strain that will be included in the upcoming flu season's vaccines six months prior to the flu season. By reducing the amount of time it takes to ramp up manufacturing, we can start manufacturing closer to the start of flu season to minimize or eliminate the matching issues we face, to varying degrees, each year and increase the efficacy of the flu vaccine. Advances in manufacturing could provide a more certain, and nearer-term opportunity to address challenges with flu vaccine supply and effectiveness while we continue to work on longer term, newer vaccine technology such as a universal vaccine.

Despite the promise of these manufacturing improvements, industry remains reluctant to invest in these platforms. Additional regulatory principles and tools to help manufacturers implement and assess these platforms could accelerate their adoption. It would give manufacturers greater certainty that products developed through continuous manufacturing can be efficiently reviewed and gain market entry.

In these ways, FDA can encourage industry to utilize these new technologies by developing a science-based framework that includes the regulatory tools and guidance for how products developed in these systems will be evaluated. As an additional benefit, these small-footprint, high-technology manufacturing platforms are likely to be domiciled in the U.S. As a result, their adoption could return more product manufacturing to domestic sites, which could help public health, enhance our national security, and foster job creation. The Budget requests $58 million in FY 2019 for advancing this initiative.

B. Establishing the Outsourcing Facility Sector as a Robust and Reliable Source of Compounded Products

Since Congress passed the Drug Quality and Security Act in 2013 (DQSA), patient access to better quality compounded drugs has been a chief concern for the Agency, physicians, and patients alike. Outsourcing facilities have the ability to produce and distribute larger amounts of compounded products to meet the needs of individual patients for whom such drugs are appropriate, including by making office stock that is used by hospitals and clinics. Outsourcing facilities are an important component to our health system and the Agency is committed to clarifying and appropriately tailoring policies so they can continue to provide drugs, based on the clinical need for a compounded medicine, so patients have access to the products they need.

The Budget proposes the creation of a "Center of Excellence on Compounding for Outsourcing Facilities" and expanded FDA engagement with outsourcing facilities and states to help the pharmacy outsourcing industry grow to meet its intended function and adhere to good manufacturing practices (GMPs) to protect patient health. We see an opportunity to make investments in regulatory policy and personnel that could help more compounding pharmacies become outsourcing facilities. This, in turn, would allow these pharmacies to grow and serve more patient needs, including through the provision of office stock compounded under GMP standards, promoting access to better quality compounded drugs for patients who have medical needs that

otherwise cannot be met by an FDA approved drug. These new outsourcing facilities would represent a largely domestic industry and a new area of growth in the healthcare sector. The Budget requests $25 million in FY 2019 for this initiative.

C. Bring MedTech Manufacturing Home: Advance Medical Device Manufacturing and Quality

The Budget proposes to establish a voluntary program for device manufacturers to receive an independent assessment of manufacturing and product quality criteria intended to demonstrate sustained organizational excellence. This would make the process for introducing innovations in how medical devices are manufactured more efficient and predictable. In turn, this program would encourage device manufacturers to make investments to re-tool their manufacturing processes in ways that can facilitate manufacturing innovation, encourage investment in new production methods and materials, and lead to better medical products.

This more modern and nimble framework would make it more efficient for device developers to innovate manufacturing processes in ways that can allow devices to better meet the needs of patients and the expectations of providers – such as through intelligent, automated processes that monitor and record manufacturing quality metrics, incorporating features and technological characteristics that can contribute to better options and higher quality that achieves their clinical purpose. Implementing this framework would help increase manufacturing innovation, accelerate availability of high-quality devices to patients and foster a competitive marketplace around device quality similar to other industries, such as automotive and aerospace, that could advance device innovations, reduce manufacturing costs and improve the quality and safety of medical devices. As medical devices become more complex – and given the frequent modifications made to devices – spurring advanced manufacturing and creating a competitive marketplace for device quality is critical for both driving technological innovations and assuring patient safety. The Budget requests $12 million in FY 2019 for this initiative.

D. Create a New Medical Data Enterprise

Advances in technology have the potential to improve the availability and utility of real world evidence (RWE) and real world data[1] (RWD). This data provides researchers the opportunity to answer questions about treatment effects and outcomes more efficiently, saving time and money, while yielding answers more relevant to broader populations of patients than might be possible in a specialized research environment. This data can also help streamline clinical development and inform the safe and effective use of medical products.

The Budget proposes to advance the use of this real-world experience to better inform patient care and provide more efficient, robust and potentially lower-cost ways to develop clinical data that can inform product review and promote innovation. The Budget requests funding to establish a new capability, including the development of data and analytical tools, to conduct near-real-time evidence evaluation down to the level of individual electronic health records for at least 10 million individuals in a broad range of U.S. healthcare settings.

Expanding the FDA's capacity to use RWE to evaluate medical products would generate processes that could improve the efficiency of the regulatory process, better inform patients and providers about pre-and post-market safety, reduce some of the burdens that drive up the time and cost required to bring beneficial innovations to the market, and address barriers that can make certain important safety and effectiveness information around the real-world use of products hard to collect and evaluate. Harnessing the power and potential of RWE is especially critical for finding treatments

[1] Examples of RWD include data derived from electronic health records (EHRs), claims and billing data, data from product and disease registries, patient-generated data including in-home use settings, and data gathered from other sources such as mobile devices that can provide information about health status.

and cures for rare disease. Establishing a strong understanding of each disease and completing clinical trials is especially challenging; however, RWE holds enormous promise for improving understanding of disease and thus, development of treatments and cures. Funding in this area will allow FDA to develop regulatory standards for use of RWE to support medical product applications. The Budget requests $100 million in FY 2019 for this initiative.

E. Facilitate Growth and Spur Transformation of the Digital Health Technology Industry by Shifting Regulation to an Efficient and Novel Framework for Reliable Post-Market Oversight

Digital health products are at the forefront of helping us diagnose and treat our society's increasingly complex medical needs. Health care providers use the latest technological tools to help them screen, detect and treat diseases ranging from cancers to neurological conditions like Alzheimer's. Patients utilize mobile medical applications to manage conditions like diabetes and treat substance use disorder. However, the current regulatory framework is not well-suited for these modern technologies. Every single day, these technologies are helping patients and doctors make health care decisions.

As part of our efforts to support timely access to safe and effective digital health products, the FDA is working collaboratively with industry, patients and providers to establish a new paradigm for digital health technologies under which a company could market lower-risk products without FDA premarket review and market higher-risk products following a streamlined FDA premarket review if the company receives a prior third-party certification for engaging in high-quality software design and testing (validation) and ongoing maintenance. This regulatory model would be fully proven and expanded from its current pilot status to a broader program. For low-risk products, rather than evaluate each individual digital health product before the product comes to market, the FDA would instead focus its resources on validating the quality of a firm's software design and the firm's methods for certifying the quality and reliability of its underlying software performance. The agency

would further reduce the time and cost of market entry of digital health technologies while assuring appropriate patient safeguards by relying on postmarket collection of real-world data to support new and evolving product functions. The Budget also proposes the creation of and resources for a Center of Excellence on Digital Health to establish this regulatory paradigm, build new capacity to evaluate and recognize third-party certifiers, and support an internal cybersecurity unit and an external public-private partnership of experts to complement the advances in software-based devices. Implementing these regulatory innovations and information technology improvements are essential for advancing technologies to improve the health and quality of life of patients while assuring critical safeguards. The Budget requests $70 million in FY 2019 for this initiative.

F. Modernize Generic Drug Development and Review

Increasing patients' access to more affordable prescription drugs is a top issue for Americans and one that I am personally dedicated to addressing during my tenure as Commissioner. Last May, in one of my first actions as Commissioner, I announced FDA's Drug Competition Action Plan. The plan has three overarching principals: eliminate gaming by branded companies that can delay generic drug entry; resolving scientific and regulatory obstacles that can make it difficult to earn approval of generic versions of certain complex drugs; and improving the efficiency and predictability of FDA's generic review process to reduce the time it takes to get a new generic drug approved and lessen the number of review cycles undergone by generic applications before approval. As part of this process, we also prioritized review for certain generic applications where there was a lack of competition, a situation under which consumers could face high prices for some very old medicines.

The Budget builds on this plan by proposing to establish a new review platform to modernize generic drug review and support efforts to update generics labels. This investment will foster greater use of lower-cost generics as a way to improve patient access and create competition in order to lower drug costs. For example, too many generic labels remain out-of-date because there is no longer a sponsor involved that is able to update the labels with new information about safe and effective

new uses for these medicines. If we incorporate into generic drug labels all of the current information about their safe and effective use, it could promote more prescribing of generic drugs, reducing overall healthcare costs. The Budget requests $38 million in FY 2019 for this initiative.

II. Opioids

One of my highest priorities as FDA Commissioner is combatting the ongoing crisis of opioid addiction. As part of this commitment, we are reexamining the Agency's authorities and policies in this area and working to ensure that FDA has the proper tools and resources to address this epidemic as it continues to evolve.

Thanks to the support of this Subcommittee, FDA received a total of $94 million in the FY 2018 Consolidated Omnibus Appropriations Act (P.L. 115-141) signed into law last month to address the interdiction of illegal drugs, including narcotics, through our work in the international mail facilities. Recognizing the Agency's responsibility in helping to curb the flow of these counterfeit and illegal drugs from entering our communities, and the critical public health obligation that the FDA was entrusted with through these additional resources, I am pleased to report the Agency will start to invest this new funding in purchasing equipment and assessing additional technologies we might deploy before the end of this fiscal year.

But FDA's efforts related to addressing the opioid crisis are broad, and there are many new programs we would like to pursue in this mission. The Budget requests an additional $10 million to provide technical assistance related to clinical study design related to medication-assisted treatments, and to also accelerate the development of non-opioid treatments, devices to treat chronic pain, and generic versions of opioid drug products with abuse deterrent formulations. One way FDA will use the resources is funding studies to identify additional tools and methodologies that can be used to evaluate whether differences in formulations impact abuse deterrence. These are small steps, but collectively, over time I am confident they can work together, along with the efforts of so many other of our federal, state, and local partners to make a difference.

III. Cures

Implementation of Cures has been another top priority of the Agency over the past 16 months. Cures includes provisions that have the potential to impart far-reaching effects on scientific advancements in medical product development. The new law complements many efforts underway at FDA. All of these efforts are aimed at transforming the way we support product development and maintaining FDA's gold standard for safety and effectiveness. Toward these efforts, last June, the Agency published our required work plan for implementing Cures, which includes details of how the Agency will utilize the $500 million in authorized new funds over nine years. For FY 2019, the Budget requests a total of $70 million to support our implementation work.

I want to thank you for your support on this vital work so far. I look forward to working with you on supporting the Agency's implementation efforts in the future.

VI. Conclusion

The last year was historic for the Agency. We are diligently working on a number of fronts and the vital work we do provides Americans with better ways to improve their health and welfare, and empowers consumers to make informed choices about the products they use and the foods they feed to their families. The Budget will help FDA maintain and complement our current efforts, as well as provide a renewed focus and investment in some of the Agency's and the nation's top public health priorities. I look forward to answering your questions today and to working with all of you going forward.