

**TESTIMONY
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**BEFORE THE
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Introduction

Chair Comer, Ranking Member Raskin, and members of the Committee, thank you for the opportunity to testify before you to discuss the Food and Drug Administration's (FDA or the Agency) policies and priorities.

In the United States, the safety of medical, food, and cosmetic products depends on the actions of both FDA and industry. Industry bears the responsibility of creating a supply of medical, food, and cosmetic products that are safe and protect and promote public health. FDA guides and oversees industry to help ensure that Americans can have confidence about the medical, food, and cosmetic products they are using and that they are duly warned about the risks of tobacco products.

FDA's workforce is dedicated to helping Americans face extraordinary challenges and navigate extraordinary opportunities. We are responsible for protecting the public health by ensuring the safety, efficacy, and security of human and veterinary drugs, biological products, and medical devices; and by ensuring the safety of our nation's food supply, cosmetics, and products that emit radiation. FDA also has responsibility for regulating the manufacturing, marketing, and distribution of tobacco products to protect the public health by reducing death and disability caused by use of tobacco products, especially in young people. In addition, the Agency is charged with advancing the public health by helping to expedite innovations that make medical products more effective, safer, and more affordable and by helping the public get the accurate, science-based information they need to use medical products and foods to maintain and improve their health. FDA also plays a significant role in the nation's counterterrorism capability by ensuring the security of the food supply and by fostering development of medical products to respond to deliberate and naturally emerging public health threats.

In the last four years, we have experienced a once-in-a-lifetime global pandemic that resulted in the death of over one million Americans; disrupted our way of life by impacting peoples' mental and physical health and quality of life; led to a decline in trust in public health institutions; and exposed vulnerabilities in global supply chains, especially for medical and food products. We also saw the value and importance of vaccines, therapeutics, and diagnostics for COVID-19, and the role these products played in saving lives. We have extraordinary opportunities as well, with technological advancements such as mRNA vaccines, artificial intelligence (AI), and gene-editing and other gene therapy technology, potentially enabling treatment for thousands of rare diseases that have been unapproachable until now.

We have all witnessed the effect of rapidly advancing technology has had on the 21st Century. FDA is adapting to this evolving world by embracing both the challenges and opportunities we face. We are leveraging flexibilities in our regulatory pathways to enable breakthroughs in medical science that can be translated into medical products that improve health outcomes. We are reshaping our regulatory processes and creating a nimble workforce that adapts to new technologies, medical products, biomedical science, food science, and public health.

Globalization: Lessons Learned from the COVID-19 Pandemic

As we reflect on the devastating losses and lasting impacts the COVID-19 pandemic has had in the United States and worldwide, we are using the lessons learned to be thoughtful about preparing for future public health emergencies and to inform our everyday best practices.

Supply Chain Vulnerabilities

Every industry FDA regulates is at risk from global supply chain issues, and these risks have resulted in real world consequences. During the COVID-19 pandemic, supply chain vulnerabilities, including a lack of resiliency and redundancy, impacted the availability of multiple drug products, medical devices, and infant formula.

Employing its current authorities and resources, the Agency established new and more comprehensive programs to help address these supply chain vulnerabilities, including:

- Monitoring the availability of pharmaceuticals and medical devices by integrating data from a wide variety of mandatory and voluntary reporting sources such as commercially available data sets, product manufacturers, component suppliers, and active ingredient manufacturers;
- Forecasting supply chain vulnerabilities and risks by integrating data sets for analysis using artificial intelligence, including natural language processing and machine learning, thereby enabling preemptive intervention to prevent hundreds of threatened shortages;
- Preventing or mitigating shortages for pharmaceuticals and medical devices with strategies such as shelf-life extensions, expedited review of manufacturing supplements and generic drug applications, exercising temporary enforcement discretion for new sources of medically necessary drugs, and requesting that manufacturers establish risk management plans to mitigate the potential for shortages;
- Initiating a new inspection planning system that will be more efficient, transparent, and adaptable to changing needs, including by returning to a pre-pandemic inspection cadence in which high-impact inspections are prioritized;
- Building a workflow management system for inspections that will provide a single source of data and real-time access to information to support the best regulatory decisions; and
- Developing new approaches to assess facilities using novel advanced techniques, such as remote regulatory assessments and remote interactive evaluations.

We are also working to further pharmaceutical manufacturing innovation and quality to facilitate domestic advanced manufacturing, which is a collective term for new medical product manufacturing technologies that can improve drug quality, address shortages of medicines, and speed time-to-market. Adoption of advanced manufacturing technologies could also result in fewer interruptions in production. For example, since 2014, the Center for Drug Evaluation and Research's (CDER) Emerging Technology Program has held over 160 meetings with sponsors to discuss, identify, and resolve potential technical and regulatory issues regarding the development and implementation of novel technology. Additionally, CDER's work on advanced manufacturing has facilitated the approval of 21 applications that use advanced manufacturing for a variety of purposes, including continuous manufacturing of finished dosage forms, a dialysis solution, an active pharmaceutical ingredient, a biological product; 3-D printing technology; advanced process analytical technologies for monitoring and control of a drug

substance bioprocess; and a novel glass container closure system for a parenteral drug product. The Center for Biologics Evaluation and Research's (CBER) Advanced Technology Team offers pre-submission regulatory support by meeting with prospective innovators and developers of advanced manufacturing technologies to provide informal consultation during early-stage development. CBER also funds intramural and extramural research to produce tools to support the development and adoption of innovative and advanced manufacturing for complex biologics, such as vaccines, exosomes, and cellular products.

Similarly, we are working to improve manufacturers' quality management systems to support a more reliable drug supply chain. CDER is developing a quality management maturity (QMM) program that will encourage drug manufacturers to implement quality management practices that go beyond current good manufacturing practice (CGMP) requirements in order to minimize risks to product availability and better assure reliable market supply. Drug manufacturers can achieve higher levels of QMM when they successfully integrate business and manufacturing operations with quality practices and technological advances to optimize product quality, enhance supply chain resiliency, and drive proactive continuous improvement. Adopting advanced quality management practices supports a more reliable drug supply chain by reducing the occurrence of quality-related failures and improving the ability of establishments to maintain performance during expected and unexpected supply chain disruptions.

FDA remains concerned about the consolidation within the infant formula industry, which relies on inflexible supply chains with insufficient redundancies. There are only three major domestic producers of these formulas—only two of which produce specialized medical formulas. This means that any potential disruption—whether it be a recall or a natural disaster—could stress formula supply. We are adopting a regulatory framework to encourage more resiliency and redundancy in the infant formula supply, as required in Food and Drug Omnibus Reform Act of 2022 (FDORA), but many of the factors that influence global supply chains and business decisions are outside of FDA's control. As part of this work, on March 28, 2023, FDA released the Immediate National Strategy on Infant Formula to further increase the resiliency of the U.S. infant formula market.¹

FDA is working within its authorities to help mitigate supply chain issues, but FDA's authorities were not designed with a complex 21st Century supply chain in mind, including the consumer's expectations for the availability of essential products. Efforts are underway across government to address these challenges, encouraging redundancy and resiliency measures throughout the supply chain, as well as requiring a broad governmental and industry effort with shared responsibility. However, the Agency has identified numerous gaps in its authorities to protect consumers and patients and the supply chain generally. Providing greater transparency into supply chains is needed to improve resiliency and prevent and mitigate shortages of medical products and the food supply. These priorities will enhance national security and improve public health preparedness.

The FY 2025 President's Budget includes several critical legislative proposals intended to promote FDA's efforts to bolster supply chains and address current vulnerabilities. For drug

¹ <https://www.fda.gov/news-events/press-announcements/fda-outlines-immediate-national-strategy-further-increase-resiliency-us-infant-formula-market>

products, this includes enhancing FDA’s insight into the drug supply chain to more effectively identify risk of a shortage, closing a loophole that does not require manufacturers to notify FDA if a likely shortage is caused by a spike in demand, understanding supplier reliance, evaluating facilities that plan to produce and import products that do not require an application, and requiring manufacturing facilities to submit Site Master Files containing information about their quality management policies, controls, and activities. Another proposal would more closely align reporting for drug and medical device manufacturers by ensuring medical device manufacturers notify FDA of a shortage beyond those connected to a public health emergency. Similarly, to prevent critical food shortages, we are requesting the authority to clarify what is required of manufacturers of critical foods when there is a likely and meaningful disruption in the U.S. supply.

FDA also seeks authority to modernize regulation of critical foods to help ensure the safety of foods consumed by one of the most vulnerable populations—infants and young children. We propose doing this by, among other things, establishing binding contamination limits in foods and requiring critical foods manufacturers to report product positive test results for pathogens to FDA regardless of whether the product has left the facility. The Agency is also requesting new authorities to improve our oversight and enforcement activities, including to require provision of requested records or other information in advance of (or in lieu of) inspections of food, tobacco, and cosmetic facilities; requiring certain remote regulatory assessments; and requiring the destruction of FDA-regulated product offered for import that has been refused entry and presents a significant health concern. FDA stands ready to work with Congress on these and other legislative proposals.

Preparing for Public Health Emergencies

FDA has played a key role in pandemic and public health emergency response. Preparation for future public health emergencies depends on diverse strategies—often decades in the making—as well as the establishment and refinement of authorities and flexibilities that allow the Agency to identify and mitigate risks while promoting innovation.

FDA used every tool in its toolbox during the COVID-19 public health emergency. FDA helped make COVID-19 vaccines available as quickly as possible while upholding our rigorous scientific and regulatory standards by streamlining reviews. During the once in a lifetime pandemic, FDA scientists and employees worked around the clock, cooperatively, intensively, and efficiently alongside researchers and manufacturers to minimize the time between the clinical development process, manufacturing scale-up, and the regulatory review process. COVID-19 vaccines are one of the most significant and important public health interventions in our history, and the available data continue to demonstrate that vaccines produce a substantial reduction in the most serious outcomes of COVID-19, including severe illness, hospitalization, and death.

In addition, FDA granted emergency use authorization to over 500 COVID-19 tests including point-of-care (POC) tests, rapid at-home tests; multi-analyte tests that can detect both COVID-19 and flu; tests using various sample types, including saliva tests; and the first test using breath samples. FDA was also the first regulatory agency to encourage development of at-home and

over-the-counter (OTC) tests. We were the first to provide emergency use authorization for an at-home test, and we ultimately authorized 41 OTC tests for emergency use, 11 of which were authorized in four weeks or less, and five of which were authorized within a week.

We also approved and authorized several treatment options for COVID-19 with a major effect on the risk of severe illness, hospitalization, and death. The Agency will continue to support efforts on future COVID-19 vaccine and treatment needs, including pivoting as the virus adapts and continuing to help advance medical products to protect the most vulnerable populations. We are also leveraging lessons learned from the COVID-19 response in our everyday reviews, including review of rare disease therapies where ongoing and informal communication with the Agency can be especially beneficial.

Additionally, when tackling a public health crisis, accurate, science-based communication is of paramount importance. Improving trust in science-based organizations requires a collaborative effort. FDA is working to build more effective relationships with the public, front line clinicians (doctors, nurses, pharmacists, etc.), biomedical scientists, and educators; we plan to improve their ability to provide more accurate and reliable information to inform choices about health and healthcare.

As we reflect on the devastating losses and lasting impacts the COVID-19 pandemic has had worldwide, we will use the lessons learned and knowledge gained to be thoughtful about preparing for future public health emergencies and to inform our future response efforts both in times of crisis and in everyday best practices. This includes continuing to leverage existing relationships with entities outside of FDA in emergency response situations. The Agency's capacity to drive future emergency responses depends on positive relationships and communication channels from continued collaborations with regulatory, academic, state, tribal, local, territorial, and industry partners even in the absence of a crisis.

Improving Life Expectancy

The trends in life expectancy in the United States are concerning, which show us below most of our high-income peer countries. Many highly educated and wealthy Americans have a life expectancy near that of other high-income countries, while various combinations of race, ethnicity, sex, lifestyle, education, and rural or underserved urban location result in a large part of the U.S. population with high mortality rates and poor health throughout their lifespan—from high infant mortality rates to excess deaths from chronic diseases.

As we experience an unprecedented reversal of previous progress in chronic diseases, mental health, and drug overdoses, FDA is reviewing what we can do to help the industries we regulate more successfully promote health and longevity. The dramatic improvement in cancer and rare disease therapies and the COVID-19 response shows that if we are strategic in pursuit of the science, we can move disease outcomes in a positive direction, particularly if we combine improved screening, diagnosis, and therapy with access and affordability.

Poor nutrition, tobacco use, and drug overdoses are also key health factors directly in our purview, and FDA can make important differences in these areas. Fully recognizing the importance of social determinants of health and health system priorities, we are working to

understand all that we can do within our sphere of direct influence to reverse these negative trends.

Food Safety and Nutrition

FDA continues to make great progress on our food safety and nutrition efforts, which can help to combat the epidemic of diet-related disease.

The United States has one of the safest food supplies in the world as judged by an independent review by *The Economist*. Tremendous progress has been made, working with the broad ecosystem of states, territories, local governments, tribes, and the industry to make the American food supply as safe as it has ever been. Since the passage of the FDA Food Safety Modernization Act in 2011, we have worked diligently to implement the landmark law and modernize our food safety regulatory oversight and capabilities. We have also developed and used advanced technology, such as whole genome sequencing,² to reduce foodborne illness cases, enhancing not only our response to outbreaks but also our surveillance of foodborne pathogens.

As part of our role in safeguarding the food supply, FDA is continuing to take action. For example, as part of the Agency's work to continue enhancing the safety of infant formula, FDA sent a letter to industry with recommendations for industry-wide improvements to identify and address *Cronobacter*. We are also working to learn more about *Cronobacter* to provide guidance to industry to help control for it in the manufacturing environment. Additionally, we are evaluating the use of chemicals as food ingredients and substances that come into contact with food and monitor the food supply for contaminants. The Agency takes action when we find that a food is unsafe. For example, in 2023, FDA took action within a few days after it was made aware of extremely high levels of lead contamination in certain applesauce products. FDA informed the public and worked with state officials and the manufacturer, collecting additional information to investigate the source of lead contamination, and taking steps to remove all unsafe products from the market as well as implementing increased screening of imported products. This example also highlights the important role that state, local, and territorial governments play in helping to ensure a safe food supply. However, we currently face limitations on sharing certain regulated commodity information in real time with states. We are requesting the authority for focused disclosure of non-public information to state, local, and U.S. territorial government agencies with counterpart functions related to FDA-regulated products, which would allow FDA to take swift action to protect the supply chain integrity and the public.

In addition to monitoring the food supply in general, FDA has prioritized reducing childhood exposure to contaminants in food. Environmental contaminants can be present in foods because they are in the soil, water, or air where foods are grown, raised, or processed. FDA's Closer to Zero initiative sets forth the Agency's approach to reducing exposure to lead, arsenic, cadmium, and mercury in foods commonly eaten by babies and young children to the lowest possible levels. We have prioritized babies and young children given their vulnerability to the harmful effects of these contaminants. It is a multi-phase, science-based, iterative approach to encourage industry to adopt agriculture and processing practices and achieve our goal of getting levels of these environmental contaminants in foods closer to zero over time.

² <https://www.fda.gov/food/whole-genome-sequencing-wgs-program/genometrakr-fast-facts>

Nutrition is also a priority at FDA. Americans are facing an ever-growing epidemic of diet-related chronic diseases such as cardiovascular disease, diabetes, and arthritis, with many of these problems associated with combinations of poor nutrition and obesity. We are committed to finding new ways to help consumers build healthy eating patterns, including through improved information about healthier nutritional choices, given our unique position and authorities. Front and center is FDA's development of a front-of-package labeling system to quickly communicate and provide additional context to certain nutrition information. This has the potential to be as iconic as the Nutrition Facts label. Front-of-package labeling could help consumers, especially those who may be less familiar with nutritional information, identify foods that can help them build a healthy eating pattern.

Relatedly, FDA is also working on an improved framework to regulate dietary supplements. This enormous industry continues to grow, and dietary supplements are now part of everyday life for many families. We will continue to work with Congress to gain authority to require dietary supplement manufacturers to list their products, including their ingredients, with FDA. In the long run, with these authorities, we would have a better understanding of how the landscape is evolving with new ingredients coming onto the market, so that FDA will be better able to quickly identify dangerous or illegal products and protect consumers.

Combating the Overdose Crisis

Preventing substance use disorders and reducing overdose deaths remain areas of major focus for FDA. We continue to take actions aligned with the HHS Overdose Prevention Strategy and the President's Unity Agenda commitment to beating the opioid epidemic, and the four priorities outlined in FDA's Overdose Prevention Framework, including primary prevention, harm reduction, evidence-based treatment, and protecting the public from unapproved, diverted, or counterfeit drugs. With overdose deaths at an all-time high, FDA is doing all we can to help ensure life-saving opioid overdose reversal agents, like naloxone, are more accessible. We also required industry to update the prescribing information for all opioids to provide additional guidance for safe use. In alignment with recommendations from an independent evaluation of our opioid-related activities, we are focusing on evidence generation and clinical trial designs to better understand when long-term use of opioids is beneficial and when they should be discontinued in a safe manner, as well as considering additional authorities needed to further help ensure the safe use of these products.

Cannabis and Hemp-Derived Products

In January 2023, FDA concluded that the existing regulatory pathways for foods and dietary supplements are not appropriate for cannabidiol (CBD). However, we recognize that consumers want access to these products. That is why FDA announced that the Agency is prepared to work with Congress on a new regulatory pathway that would provide access, safeguards, and oversight over products containing CBD in ways that existing pathways cannot. Under the Federal Food, Drug, & Cosmetic Act, any substance, including CBD, must meet specific safety standards to be lawfully marketed as a dietary supplement or food additive.³ The use of CBD raises safety concerns, especially with long-term use. Studies have shown evidence of liver toxicity, interactions with certain medications and possible harm to the male reproductive system. CBD

³ <https://www.fda.gov/news-events/press-announcements/fda-concludes-existing-regulatory-frameworks-foods-and-supplements-are-not-appropriate-cannabidiol>

exposure is particularly concerning for children and during pregnancy. Accordingly, FDA has concluded that a new regulatory regime is needed, which could encourage better information to inform consumers about their choices. In the meantime, FDA continues to assess the evolving information base and build awareness for Americans.

Regulating Tobacco Products

FDA's Center for Tobacco Products (CTP) reached several important milestones in 2023, including the release of a new five-year strategic plan in December. The plan was the outcome of a year-long process that sought input from CTP staff and external stakeholders, including a public listening session. Guided by the new strategic plan, CTP aims to reduce the negative health effects caused by tobacco product use by providing information to discourage people from starting to use tobacco products, encourage people who use tobacco products to quit, and reduce the harm caused by tobacco product use.

Over the past year, there has been significant progress in key tobacco product use behaviors in the U.S. population. For example, cigarette smoking—which is responsible for the overwhelming burden of tobacco-related disease and death in the United States—has declined steadily among adults and youth for decades and currently remains at unprecedented lows. Moreover, over the past year, there was a significant decrease in overall tobacco product use among high school students, which was primarily driven by a decline in e-cigarette use. There are now less than half the number of U.S. youth using e-cigarettes compared with the peak in 2019. However, despite these important wins for public health, our work is not finished. Almost 30 million U.S. adults continue to smoke cigarettes and in 2023, more than two million U.S. youth use e-cigarettes. We will continue to advance public health through our application review process, regulation development, and public education efforts.

Regarding compliance and enforcement actions, in 2023, the Agency issued warning letters to more than 120 manufacturers and distributors and more than 400 retailers; filed over 40 civil money penalty complaints against manufacturers and over 65 against retailers; and collaborated with our federal partners at the U.S. Customs and Border Protection to seize approximately 1.4 million units of unauthorized e-cigarette products with an estimated retail value of more than \$18 million; among other actions. We remain committed to advancing our important, life-saving vision to reduce the health burden of tobacco product use in the United States, which remains the leading cause of preventable disease and death nationally.

Emerging Technologies

Staying ahead of the rapid advancements made across regulated industries is one of the most challenging and exciting aspects of FDA's work. Biomedical discovery science, computing, and engineering take decades to mature to develop safe and effective products. FDA is most effective when our regulatory strategies provide pathways for innovative products to be developed in a manner that most effectively balances innovation, access, and safety. We are at such a point with gene editing for rare diseases and in the application of artificial intelligence across the spectrum of products FDA regulates. Through cooperation with private industry, Congress, and the public, we believe that FDA can help harness these groundbreaking advancements to benefit the American people.

Artificial Intelligence (AI) and Machine Learning (ML)

FDA recognizes that the increased use of AI, including ML, across all our regulated industries presents new and unique challenges and opportunities. For example, FDA has seen a significant increase in the number of drug and biologic application submissions using AI/ML components over the past few years. We have been preparing for this major technology wave and, to respond to this evolving landscape, FDA has accelerated its efforts to create an agile regulatory ecosystem that can facilitate innovation while safeguarding public health.

As part of this effort, CDER, in collaboration with the CBER and the Center for Devices and Radiological Health (CDRH), issued an initial discussion paper to communicate with a range of stakeholders and to explore relevant considerations for the use of AI/ML in the development of drugs, biological products, and medical devices intended to be used with drugs.⁴ The Agency will continue to solicit feedback as it advances regulatory science in this area.

CDRH has reviewed and authorized a growing number of devices with AI/ML through various authorization pathways across many different fields of medicine—and expects this trend to continue. Supporting responsible development of AI/ML medical devices and assuring timely access for U.S. patients has been a top priority for years; the Center continues work with innovators and relevant institutions to have clear, predictable, and timely potential paths to market to facilitate patient access to innovative, safe, and effective devices. FDA has approved, authorized, or cleared over 700 AI/ML devices, including:

- A cardiac ultrasound software that uses AI to guide the user;
- An AI-based device that assists clinicians in detecting lesions (such as polyps or suspected tumors) in the colon in real time during a colonoscopy;
- A diagnostic aid for autism spectrum disorder; and
- An AI-based device to detect greater than a mild level of the eye disease diabetic retinopathy in adults who have diabetes.

Overall, FDA’s efforts are focused on ensuring that the United States is a leader in AI/ML medical devices, including by ensuring that the Agency’s approaches keep the United States ahead of its global competitors and so AI is developed and deployed responsibly in healthcare. FDA believes there is no “one-size fits all” approach for AI. FDA’s Digital Health Center of Excellence within CDRH leads policy and evaluation of medical devices with AI and other digital health technologies at FDA and has worked to be a resource for developers of cutting-edge technology by publishing an AI Action Plan, providing guidance, issuing guiding principles on good machine learning practices, and engaging on efforts towards international harmonization, among other efforts.

AI will undoubtedly play a critical role in medical product development, and FDA is making every effort to remain nimble and support innovation. Furthermore, the use of AI to optimize supply chains and reach consumers is already having a significant impact on food and tobacco, and we will be prepared to deal with the changing landscape beyond the medical product arena. As we consider and adopt a risk-based regulatory framework we will need Congress’ support. We look forward to working together to promote innovation while protecting patient safety.

⁴ <https://www.fda.gov/media/167973/download?attachment>

Real World Data and Evidence

FDA has a long history of using real-world data (RWD) and real-world evidence (RWE) to support marketing authorization of medical devices and monitor and evaluate the post-market safety of drugs and devices. FDA is working with sponsors, patient advocates, and other stakeholders, such as data aggregators, to realize the full potential of RWD and RWE in clinical trial design to facilitate development of novel medical products. We are committed to realizing the full potential of fit-for-purpose RWD to generate RWE that will advance the development of therapeutic products and strengthen regulatory oversight of medical products across their lifecycle.

New Approvals and Pathways

Accelerating the availability of safe and effective drugs and therapeutics that treat serious diseases is in everyone's interest, especially when the drugs are the first available treatment or have advantages over existing treatments. The Agency continues to evaluate a wide variety of new drugs, biologics, and therapies, including for new settings, new uses, and new patient populations, and whether such products meet the requirements for marketing authorization. As a science-led organization, FDA uses the best scientific and technological information available to make decisions through a deliberative process.

For example, in 2023 CBER approved gene therapies for the treatment of rare diseases such as sickle cell disease and Duchenne muscular dystrophy. CBER also approved vaccines to prevent respiratory syncytial virus (RSV) disease (including the first maternal vaccination to protect infants from RSV disease). In 2023, CDER also approved therapies for neurological conditions, such as Alzheimer's disease and migraines. Moreover, we took approval actions for drugs targeting type 2 diabetes in children, different types of anemia, and chronic weight management, among other heart, blood, kidney, and endocrine disorders. In oncology, approvals included treatments for colorectal, prostate, lung, and low-grade gliomas (tumors that start in the brain). In the area of women's health, CDER approved treatments for postpartum depression, hot flashes due to menopause, and over-the-counter oral contraception.

The Agency is also committed to helping patients with rare diseases, recognizing that they often have no or few treatment options. In 2023, over half of new molecular entities approved were indicated for orphan-designated diseases or conditions. Examples include a therapy for acid sphingomyelinase deficiency (Niemann-Pick disease type A, B, A/B, an inherited disease that affects fat metabolism); generalized pustular psoriasis (a life-threatening skin disease); and obstructive hypertrophic cardiomyopathy (a disease in which the heart muscle thickens).

Through our Humanitarian Device Exemption pathway, we approved devices for acute kidney injury due to sepsis in children as small as 22 pounds, and a non-invasive and radiation-free therapy for osteoid osteomas, a benign but painful bone tumor that occurs predominantly in children and young adults. In addition, there have been approvals to prevent, diagnose or treat rare diseases including drugs for Friedreich's ataxia (an inherited, degenerative disease that damages the nervous system); paroxysmal nocturnal hemoglobinuria (a disease that causes red blood cells to break apart); and activated phosphoinositide 3-kinase delta (a genetic disorder that impairs the immune system).

FDA balances regulatory flexibility, necessary to address challenges in rare disease drug development, with the need to adhere to statutory and regulatory requirements for establishing safety and effectiveness to support an approval. Regulatory flexibilities include, for example, Accelerated Approval based on a surrogate or intermediate clinical endpoint for serious conditions with an unmet medical need, reliance on one adequate and well-controlled trial, use of natural history study data as a source of external control data, novel trial designs, and novel statistical methodologies.

In addition, under FDA’s Breakthrough Devices Program, the Agency may consider health disparities and unmet needs in rare disease populations when evaluating requests for breakthrough device designation. That program has recently supported the marketing authorization of digital health technologies assisting in the management of rare conditions such as seizure disorders and obstruction to cerebrospinal fluid flow. FDA knows much of the groundbreaking technology in science will benefit rare disease populations in particular, and we are already thinking through creative solutions and changes to our regulatory processes that may be needed as these innovations come to the market.

FDA also continues its commitment to fostering innovation in the medical device industry and helping to bring important new technologies to patients, as we continue to see high numbers of novel medical device authorizations. In 2023, CDRH authorized the highest number of novel devices on record (excluding EUAs) in CDRH’s more than 40-year history.⁵ One example includes our clearance of the first over-the-counter fentanyl test—a testament to the Agency’s commitment to further advance bringing health care into the home setting.

Animal Health and the One Health Approach

The world is in a period of rapid change in a way that calls for the evolution of animal and veterinary products. Climate change, human encroachment on previously wild environments, international conflict, and global travel and trade all have the potential to increase disease transmission, environmental stressors, and pathologic challenges. FDA and the industries we regulate have access to an array of modern technologies to combat these threats, including new technologies that can deliver a more resilient and robust food production system, to help ensure food-producing animals and their environments are healthier, hardier, and deliver consumers more choices in the foods they eat. These technologies can be used to monitor, prevent, control, and treat increasing animal health challenges, such as zoonotic and animal infectious disease threats—leading to healthier animals, communities, and ecosystems.

FDA’s Center for Veterinary Medicine (CVM) takes a One Health approach, recognizing that human, animal, and environmental health are connected, and that innovative transdisciplinary approaches and technologies hold great potential to address challenges across these sectors. CVM is facilitating animal and veterinary product development across the board, developing strategies for approval pathways that enable the relevant industries to develop more safe, novel products and products for unmet human and animal needs—such as monoclonal antibody therapies, gene therapies, intentional genomic alterations (IGAs) in animals, cell-based animal

⁵ <https://www.fda.gov/media/175479/download?attachment>

foods, and animal cell and tissue-based products. We are continuously improving efficiencies in our thorough, risk and science-based review processes.

Agency's Largest Reorganization in History

Lastly, the Agency has made significant progress on plans to create a unified Human Foods Program (HFP) and a new model for the Office of Regulatory Affairs (ORA). These proposed changes will strengthen the Agency's oversight and protection of the human food supply and enhance our oversight of the broad expanse of regulated products. After more than a year of proposal development and engaging with internal and external constituencies following the findings and recommendations of the Reagan-Udall Foundation (RUF) evaluation, FDA transmitted a reorganization package to Congress on March 8, 2024.

FDA's reorganization proposal is Agency-wide, closely tracks the Agency's implementation of the RUF recommendations, and enhances the Agency's infant formula response processes following the Abbott infant formula recall in February 2022. For example, FDA's vision is to:

- Unify human foods functions currently split across the Agency, including the Center for Food Safety and Applied Nutrition (CFSAN), the Office of Food Policy and Response (OFPR), and certain ORA functions into the new HFP under the direction of the Deputy Commissioner for Human Foods.
- Create a Nutrition Center of Excellence that prioritizes the Agency's efforts to reduce diet-related chronic disease. We have proposed establishing an Office of Critical Foods (OCF), as directed by the 2023 Consolidated Appropriations Act, within this Center.
- Reevaluate the Agency's infant formula staffing needs and provided substantive input for the development of OCF. FDA has now begun to announce, interview, and staff many positions related to the OCF as well as other positions, which will help to support infant formula oversight and regulation.
- Establish an Office of Integrated Food Safety System Partnerships, which will enable greater collaboration with our state and local regulatory partners and support state-level inspectional activities to help meet the vision of an Integrated Food Safety System, as envisioned in the FDA Food Safety Modernization Act of 2011, more effectively.
- Improve the process for handling whistleblower and consumer complaints at HFP and the Centers, which will refine the processing of complaints, ultimately improving our ability to detect and address issues sooner.
- Focus ORA's mission and rename ORA as the Office of Inspections and Investigations (OII), solidifying its role as the frontline of FDA's field-based inspection, investigation, and import operations.
- Establish a specialized critical foods investigator cadre, which will solely focus on the inspection and oversight of the infant formula (and other critical foods) industry. Hiring for the cadre is currently underway, and specialized training programs are continuously being developed, piloted, and deployed as a part of that effort.
- Establish an Office of the Chief Medical Officer (OCMO) in the Office of the Commissioner to strengthen central coordination of cross-agency medical issues, including special populations such as people with rare diseases and children. This includes a new Office of

Public Health Preparedness and Response to support medical countermeasure policy, emergency preparedness work, and medical product shortage coordination across FDA.

- Merge the Office of Counterterrorism and Emerging Threats (OCET) and the Office of Regulatory Science and Innovation (ORSI) to form a new office. This new merged office in the Office of the Chief Scientist (OCS), proposed as the Office of Regulatory and Emerging Science, will strengthen support of regulatory science and preparedness research efforts.
- Create an Office of Enterprise Transformation in the Office of the Commissioner to work across FDA to drive high-priority cross-cutting business process improvement efforts in order to use Agency resources more efficiently and strategically.

FDA remains committed to keeping Congress and the public up to date as the proposal is finalized.

Conclusion

The essential work of the Agency continues in thousands of ways that Americans and the world count on every day. This work is accomplished thanks to the dedication and perseverance of FDA staff. We look forward to continuing to work with Congress on the Agency's mission, including by strengthening FDA's authorities in the areas of supply chain resiliency, hemp-derived products, infant formula, and more. Thank you again for the opportunity to testify.