OVERVIEW OF LEGISLATIVE PROPOSALS

The FY 2020 Budget Request includes legislative proposals to address drug pricing, medical product shortages, and other priority areas.

DRUG PRICING PROPOSALS

Amend the 180-day Exclusivity Forfeiture Provision Addressing Failure to Obtain Tentative Approval

Currently, a first applicant for a generic drug forfeits 180-day exclusivity if they fail to obtain tentative approval (TA) for their application within a specific timeframe. A first applicant can avoid forfeiture under this provision if the failure to obtain TA is caused by a change in or a review of the requirements for approval imposed after the application filing date. Currently, first applicants with deficient applications benefit from this provision by avoiding forfeiture even though they have deficiencies in their application unrelated to any change in or review of the requirements for approval. The proposal would clarify that the exception to forfeiture will only apply if the change in or review of the requirements for approval was the only cause of the applicant's failure to obtain TA within the specified timeframe. Depending on the factual circumstances, this change can result in increased generic competition and choice for consumers by allowing final approval of competing generic drugs that otherwise would generally have had to wait until the first applicant with the deficient application has been approved and the 180-day exclusivity period has run before being approved by FDA.

Provide FDA Enhanced Authority to Address Abuse of the Petition Process

Section 505(q) of the Federal Food, Drug, and Cosmetic Act (FD&C Act) was intended, in part, to help prevent submission of sham citizen petitions intended to delay the approval of abbreviated new drug applications (ANDAs) and to avoid FDA delaying approval of ANDAs because of such citizen petitions. This proposal would amend section 505(q) to provide FDA with greater flexibility to summarily deny petitions that impede competition and eliminate the mandatory 150-day response timeframe, as it is no longer needed to prevent delay of approval of ANDAs given passage of the Food and Drug Administration Reauthorization Act of 2017, which reauthorized the Generic Drug User Fee Amendments, providing goal dates for FDA to take action on all ANDAs.

Generics Condition on Exclusivity to Spur Access and Competition

Under current law, a first applicant of an ANDA that does not have patent/exclusivity barriers to approval, but is not approvable due to substantive deficiencies that have not been resolved in a timely manner, can block subsequent ANDA approvals under the 180-day exclusivity provisions of the FD&C Act. Similarly, a first applicant whose ANDA is otherwise approvable could intentionally delay seeking final approval, "parking" their 180-day exclusivity and blocking subsequent ANDA approvals. Current law is often insufficient to address these circumstances and, as a result, consumers may be denied access to generic products. This proposal would make the tentative approval of a subsequent generic drug applicant that is blocked solely by a first

applicant's 180-day exclusivity, where the first applicant has not yet received final approval, a trigger to start the first applicant's 180-day exclusivity. The change will enhance competition and facilitate more timely access to generic drugs.

Revision of USP Compendial Compliance Requirements for Biological Products

FD&C Act provisions that relate to U.S. Pharmacopeial (USP) compendial standards, which were originally drafted to apply to drugs approved under section 505 of the FD&C Act, currently apply to biological products licensed under section 351 of the Public Health Service (PHS) Act. Current law could result in delays, related to compliance with USP standards, in the licensure of biosimilar and interchangeable products that meet FDA's robust scientific standards. This proposal would amend section 351(j) of the PHS Act to exclude all biological products licensed under the PHS Act from FD&C Act provisions that relate to USP standards for drugs. FDA seeks this change to facilitate biological product innovation and the timely licensure of biosimilar and interchangeable products.

Codify Our Active Moiety Approach for New Chemical Entity (NCE) Determinations

The Agency's regulation implementing the statutory provisions on 5-year New Chemical Entity (NCE) exclusivity focuses on evaluating a drug's active moiety. Under this regulation, eligibility for NCE exclusivity is available only for a drug containing no active moiety that has been previously approved by FDA. This approach ensures that only the most innovative drugs qualify for NCE exclusivity, while allowing for earlier generic competition for drugs that do not qualify. A recent district court decision (*Amarin Pharmaceuticals Ireland Ltd. v. FDA)* invalidated an FDA NCE exclusivity decision that applied FDA's active moiety regulation based on the court's interpretation of the statute's "plain meaning." This decision has resulted in uncertainty about FDA's ability to continue to apply its regulation. This proposal would codify FDA's long-standing "active moiety" approach, and would provide clarity about Congress' intent that only the most innovative new drugs qualify for NCE exclusivity.

PROPOSALS TO ADDRESS MEDICAL PRODUCT SHORTAGES

Lengthen Expiration Dates to Mitigate Critical Drug Shortages

Shortages of drugs that are life-supporting, life-sustaining, or intended for use in the prevention or treatment of a debilitating disease or condition, can be exacerbated when drugs must be discarded because they exceed a labeled shelf-life due to unnecessarily short expiration dates. This proposal would expand FDA's authority to require, when likely to help prevent or mitigate a shortage, that an applicant evaluate, submit studies to FDA, and label a product with the longest possible expiration date that FDA agrees is scientifically justified.

Improving Critical Infrastructure by Requiring Risk Management Plans

This proposal would expand FDA's authority to require application holders of certain drugs to conduct periodic risk assessments to identify the vulnerabilities in their manufacturing supply chain (inclusive of contract manufacturing facilities) and develop plans to mitigate the risks associated with the identified vulnerabilities. Currently, many applicants lack plans to assess and

address vulnerabilities in their manufacturing supply chain putting them at risk for drug supply disruptions following disasters (e.g., hurricanes) or in other circumstances.

Improving Critical Infrastructure Through Improved Data Sharing: Requiring More Accurate Supply Chain Information

This proposal would clarify FDA's authority to require information that would improve FDA's ability to assess critical infrastructure as well as manufacturing quality and capacity. For example, FDA is seeking to require detailed drug listings for finished drug product or in-process material, regardless of whether they were directly or indirectly imported into the U.S.

Device Shortages

No law requires medical device manufacturers to notify FDA when they become aware of a circumstance that could lead to a device shortage. Such circumstances may include, for example: discontinuation of a device; interruption of the manufacture of the device, e.g., due to scarcity of a raw material or unavailability of a component part; or loss of or damage to a manufacturing facility. This proposal would ensure FDA has timely and accurate information about likely or confirmed national shortages of essential devices to enable FDA to take steps to promote the continued availability of devices of public health importance. Specifically, FDA is seeking authority to: require firms to notify FDA of an anticipated significant interruption in the supply of an essential device; require all manufacturers of devices determined to be essential to periodically provide FDA with information about the manufacturing capacity of the essential device(s) they manufacture; and authorize the temporary importation of devices whose risks presented when patients and healthcare providers lack access to critically important medical devices outweigh compliance with U.S. regulatory standards.

OTHER FDA PROPOSALS

Updating the Labeling of Generic Drugs After the New Drug Application or New Animal Drug Application Reference Listed Drug is Withdrawn

FDA is aware that generic drug labeling sometimes becomes outdated after approval of the reference listed drug (RLD) is withdrawn at the request of its sponsor. This proposal would give FDA explicit authority to update the labeling of generic drugs with withdrawn new drug application (NDA) or new animal drug application (NADA) RLDs when the generic labeling becomes outdated, including to update the uses to reflect the current state of the science. This would ensure that labeling of generic drugs continues to provide healthcare professionals and consumers with the most up-to-date information about the use of the drugs even after the NDA or NADA RLD is no longer on the market.

Exemptions from "Wholesale Distribution" for Dispenser-to-Dispenser Transactions and for Entities That Distribute Drugs under Federally-Administered Programs

This proposal would allow certain dispenser-to-dispenser sales of drug products to be exempted

from the definition of wholesale distribution and authorize FDA to exempt entities that distribute drugs under Federally-administered programs from the wholesale distributor licensing requirements of the Drug Supply Chain Security Act (DSCSA). The proposed changes to the statutory definition of "wholesale distribution" would decrease regulatory burdens under the DSCSA for certain entities and help ensure patients who rely on Federally-administered programs have access to needed drug products.

Increase the Statutory Maximum and Add an Inflation Factor for FDA's Export Certificate Fee

Export certificates are required by some countries for a company to export a product from the U.S. into the requesting country. Multiple FDA centers provide export certificates in exchange for export certificate fees. Current law, originally enacted in 1996, limits the maximum export certification fee to $175, which is less than the current cost per certification to run this program. This proposal would increase the statutory maximum for the export certification fee to $600 per certification and include a provision to adjust this cap for inflation.

Advisory Committees/Public Discussions

Data and information relating to an issue that is appropriate for public consideration may be provided to FDA through varied medical product submission pathways like annual reports, periodic safety update reports, general correspondence, and in withdrawn submissions. These pathways can be outside the scope of regulations authorizing disclosure of summary safety and effectiveness information pursuant to a Commissioner's Finding, and FDA therefore typically cannot disclose these data and information at an FDA advisory committee or other appropriate public meeting without the sponsor's permission. This limitation hinders FDA's ability to have full and complete public discussions about important scientific and regulatory issues and this proposal would provide clear authority for FDA to publicly disclose a summary of any safety and/or effectiveness data and information pursuant to a determination that it is appropriate for public consideration of a specific issue; for example, for consideration at an open session of an FDA advisory committee; an FDA public hearing; or a public congressional hearing.

Post-Approval Quality Updates

FDA has commonly requested that applicants agree (or commit) to provide certain information or studies in post-approval supplements or reports to address residual quality risks that are identified pre-approval but are not found to be significant enough to delay approval. Unlike post-marketing requirement studies, reports on quality-related post-approval agreements are not legally enforceable requirements. FDA, therefore, has limited ability to take enforcement action if an applicant does not submit the agreed-upon information, short of proposing to withdraw approval of the application. This proposal would grant FDA authority to require NDA, biologics license application (BLA), or abbreviated new drug application (ANDA) applicants to submit a post-approval quality update to provide information or implement changes needed to ensure ongoing quality and, therefore, safety and efficacy of the product once approved and marketed.

Medical Device Cybersecurity

Currently, there is no statutory requirement (pre- or post-market) that expressly compels medical device manufacturers to address cybersecurity. This proposal would advance medical device safety by ensuring FDA and the public have information about the cybersecurity of devices. Specifically, FDA seeks to require: that devices have the capability to be updated and patched in a timely manner; that premarket submissions to FDA include evidence demonstrating the capability from a design and architecture perspective for device updating and patching; a phased-in approach to a Cybersecurity Bill of Materials (CBOM), a list that includes but is not limited to commercial, open source, and off-the-shelf software and hardware components that are or could become susceptible to vulnerabilities; and that device firms publicly disclose when they learn of a cybersecurity vulnerability so users know when a device they use may be vulnerable and to provide direction to customers to reduce their risk. The proposal also seeks to improve proactive responses to cybersecurity vulnerabilities.

Performance Criteria for Premarket Notification Determinations

Under this proposal, FDA would establish a voluntary alternative to the premarket notification (510(k)) pathway that would allow manufacturers of certain well-understood device types to rely on objective safety and performance criteria to demonstrate substantial equivalence, enabling FDA to help improve safety and performance and ensure new products can more easily reflect beneficial new advances. Current law requires sponsors of 510(k)s for medical devices to demonstrate substantial equivalence by comparing the intended use and technological characteristics of their device to a predicate device. The proposal would permit the marketing of certain Class II and Class I medical devices requiring premarket notification if such devices demonstrate conformance with pre-specified safety and performance criteria established by FDA based on the performance of modern predicates as well as FDA-recognized performance standards, or other FDA-recognized national and international standards, if applicable and appropriate, and as explained in Level 2 guidance. This voluntary alternative would provide more direct evidence of the safety and performance of a device and better information for patients and providers to make well-informed health care decisions while fostering a competitive marketplace for safer, more effective devices.

Progressive Approval for Devices

This proposal would permit FDA expedited access to devices that would otherwise be reviewed under the premarket approval or de novo classifications pathways if they are intended to treat or diagnose a life-threatening or irreversibly debilitating disease or condition and address an unmet medical need using a two-step approval. These devices would be eligible for provisional approval based on a demonstration of safety and performance plus additional risk mitigations and could remain on the market after an established time period only after a demonstration of reasonable assurance of safety and effectiveness. Companies would be required to gather postmarket data from established data sources to assure timely evidence generation. Permitting an initial, provisional approval of a device based on a standard of safety and performance would encourage manufacturers to seek introduction of their devices in the U.S. earlier, thereby allowing patients with few to no options for treatment earlier access to important medical

technology. Moreover, if a company did not demonstrate reasonable assurance of safety and effectiveness within a reasonable amount of time after initial approval is granted, the initial approval would automatically sunset and the device could no longer be legally marketed. This proposal would help improve patient access to technologies for some of the most challenging health circumstances, and would provide accountability to ensure that devices fully demonstrate safety and effectiveness to remain on the market.

Special Controls Via Order

Under this proposal, FDA would modernize the process to impose, add, revise, or eliminate special controls for class II devices by using an administrative order rather than regulation. This would ensure FDA is equipped to provide a nimbler process to mitigate risk and address safety signals in the postmarket setting, and allow timely patient access to innovative technologies. Currently, FDA can require companies to implement mitigations (e.g., labeling, user training, device features) through the imposition of additional special controls. However, because the establishment of special controls requires rulemaking, which can entail extensive resources and time, it can be challenging for FDA to mitigate risk and address safety issues quickly, and it can also delay marketing of useful devices that could benefit patients with appropriate risk mitigation measures. This proposal would increase transparency about FDA expectations and requirements for ensuring a device's safety and effectiveness and allow FDA to act more quickly in the interest of patients.

Enable FDA to Phase Out Publication of Animal Drug Approval Information in the Code of Federal Regulations

Under current law, when a new animal drug application is approved or conditionally approved, the Secretary must publish a Federal Register (FR) document that provides notice of the approval and creates a regulation for inclusion in the Code of Federal Regulations. This proposal would enable FDA to phase out publication of new animal drug approvals in the FR and make this information publicly available online only.

Enhance Availability of Generic Animal Drugs

This proposal would allow FDA to clarify labeling requirements for generic animal drugs by explicitly including an exception from the requirement that a generic animal drug's labeling be the same as the labeling of a reference-listed new animal drug (RLNAD) where the RLNAD is approved in more than one species. The exception would allow a generic animal drug manufacturer to seek approval for fewer species than on a RLNAD's labeling, particularly in situations where obtaining bioequivalence information for all species is impractical or scientifically challenging.

Enable Certain Products to be Excluded from Definition of "New Animal Drug" to Allow Their Regulation as Pesticide

This proposal would revise the definition of "new animal drug" to provide the ability to exclude certain products or categories of products that FDA and EPA agree are more appropriately regulated as pesticides under the Federal Insecticide, Fungicide, and Rodenticide Act. Revising the definition would increase transparency and decrease regulatory uncertainty, which currently contributes to inefficiencies and increased costs for sponsors.

Strengthen FDA's Implementation and Enforcement of DSHEA

In the 25 years since the Dietary Supplement Health and Education Act of 1994 (DSHEA) was enacted, the dietary supplement market in the U.S. has grown from approximately 4,000 products to somewhere between 50,000 and 80,000 products. Under current law, FDA is not clearly authorized to require listing of individual dietary supplement products on the market, and the Agency has no convenient mechanism for compiling basic information about those products. This proposal would require all products marketed as "dietary supplements" to be listed with FDA and give FDA authority to act against non-compliant products and the manufacturers and/or distributors of such products. This would allow FDA to know when new products are introduced, quickly identify and act against dangerous or otherwise illegal products, and improve transparency and promote risk-based regulation.

Amend FDA Authorities to Strengthen FDA's Training Programs

Due to gaps in current law, FDA has been unable to establish a comprehensive, in-house training program that meets its needs. FDA has relied heavily instead on participation in a contract program, known as the Oak Ridge Science Institute for Science and Education (ORISE) program, under an interagency agreement with the Department of Energy, to meet the agency's training needs. This proposal would enable FDA to establish its own comprehensive training. Because trainees will be subject by law to the same legal and ethical requirements as FDA employees, trainees will be subject to the same non-disclosure prohibitions as FDA employees and will be provided access to confidential information only on an as-needed basis.