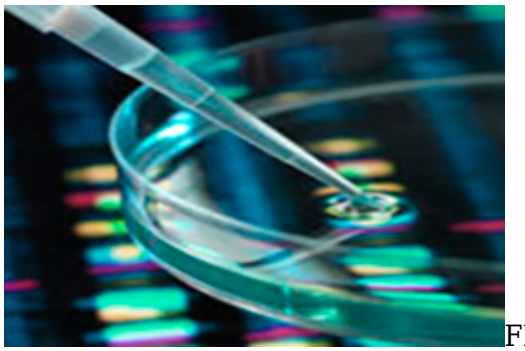
Move Fast: FDA is Accepting Submissions for the Pilot Program Class for FDA Commissioner's National Priority Voucher Program



FDA is now accepting submissions to the Commissioner's National Priority Voucher (CNPV) pilot program, and with only five vouchers to be awarded as part of the initial year of the program, the competition is anticipated to be fierce. It has been a little over a month since the FDA **announced** the CNPV pilot program, and the FDA has now provided additional information to help interested companies through the process and criteria for applying for these vouchers.

On June 17, 2025, the FDA announced that through the CNPV program, selected sponsors will receive non-transferable vouchers that can be redeemed for expedited review of their drug or biologic product candidates. The FDA touts the CNPV program as a "novel" priority program that "shortens [the agency's] review time from approximately 10-12 months to 1-2 months following a sponsor's final drug application submission." The vouchers awarded through the program can be applied to drug or biologic product candidates in any area of medicine and will focus on companies that are aligned with the following national priorities:

1. Addressing a health crisis in the US,
2. Delivering more innovative cures for the American people,
3. Increasing affordability,
4. Addressing unmet public health needs, and
5. Increasing domestic drug manufacturing as a national security issue.

In an update posted July 22, 2025, the FDA provided **examples** of each of the national priorities that could make a company or its drug candidate eligible for a CNPV voucher. Of notable interest to the rare disease community, FDA's example for addressing a large unmet medical need specifically includes condition(s) that available therapies do not adequately diagnose or treat, "including drugs to treat or prevent rare diseases."

Here are four things to know about the CNPV program, based on the information the FDA has provided thus far:

- **Participation Process**: Interested and eligible companies should submit a statement of interest to FDA through the **CNPV Program Submission** page. Interested companies can submit a maximum of one statement of interest each, although the FDA has indicated that vouchers can be granted for review of a specific drug or as an undesignated voucher, allowing a company to use the voucher for review of an application for a drug "at the company's discretion subject to consistency with the program's objectives." The FDA will select

companies based on the submitted statement of interest for "possible acceptance" into the pilot program. These statements are short—just 350 words or fewer—and should discuss one national priority the drug development program addresses and any specific issue(s) for which the company may be seeking enhanced communications with FDA to facilitate program development. If the program addresses more than one national priority, companies should identify the primary national priority in their statement of interest.

- **Submission and Review Process**: The CNPV program submissions will be evaluated by a senior, multi-disciplinary committee of experts, led by FDA's Office of Chief Medical and Scientific Officer, and the committee will pre-review the submitted statements of interest and convene for a 1-day "tumor board style" meeting. The Commissioner's **YouTube announcement** for the program explains that such meetings allows experts "to consider hard questions in light of all the latest clinical evidence," and the CNPV committee plans to utilize a similar approach. Companies must be prepared to respond promptly to any FDA inquiries about their submission. FDA is accepting statements of interest on a rolling basis, and although there is not a specific deadline for submissions, we recommend that interested companies act with urgency in order to get considered for the initial pilot program class.
- **CNPV Voucher Benefits**: As **highlighted** by FDA, a CNPV voucher entitles the company holding it to enhanced communications and rolling review to allow for a shortened review time. The FDA plans to provide a limited number of vouchers to companies aligned with US national priorities. A non-transferable voucher issued by the FDA could either be directed at a specific product or awarded to a company as an "undesignated voucher" that the company could use for a new drug at its discretion and consistent with the CNPV program's objectives. The FDA has published a frequently asked questions document, "**FAQs: Commissioner's National Priority Voucher Program**," and notes that this page will be updated regularly as questions arise.
- **Alignment with President Trump's Executive Order**: Among the national priorities that the CNPV program seeks to advance is the goal to increase affordability of drugs and biologics, and that goal is a direct focus of President Trump's May 12, 2025, **Executive Order** on drug pricing, signaling the Administration's goal of "equalizing" prices among the United States and other developed countries throughout the world. Among other directives, the Executive Order directs FDA to contemplate approaches that may involve pricing (for example, examining whether case-by-case importation of products would be appropriate if manufacturers do not lower their prices or whether there may be some sort of action with respect to the product's approval). *See **Goodwin Alert** on the Most Favored Nation Drug Pricing Executive Order*. Companies are paying attention. In just the last couple weeks, two large drug makers have announced direct-to-consumer programs to offer a low-cost option to patients.

If a company is selected as one of the five pilot participants in the initial year of the CNPV program, the FDA states that the "voucher process must be commenced within two years" after receipt of the CNPV, although we note that the current information provided by the Agency does not expressly state whether an NDA or BLA must be submitted within two years. Since the voucher can be applied to a product "at any stage of development," we anticipate that this two-year timeframe may be an area where FDA will provide more clarity as it selects sponsors for the program.

We encourage interested stakeholders to reach out to a member of the Goodwin **Life Sciences Regulatory and Compliance** team for further questions or assistance with submitting a statement of interest for the CNPV program.

FDA's Push for "Radical Transparency": Key Takeaways from the Agency's Publication of Complete Response Letters

On July 10, 2025, the U.S. Food and Drug Administration (FDA) **announced** publication of over 200 complete response letters (CRLs) issued in response to applications submitted to FDA for approval of drugs or biologics between 2020 and 2024. The FDA has described this move as a step toward the Agency's "broader initiatives to modernize and increase transparency."

CRLs are formal communications sent to applicants when the FDA has completed its review of an application but determined that it cannot approve the application in its current form. Until now, the Agency has only made CRLs available as part of larger approval package files on the Drugs@FDA online database (i.e., after product approval). While the CRLs released this week continue to be limited to approved products—and have been redacted to remove trade secrets and confidential commercial information—the FDA has, for the first time, provided these documents in a central database on **openFDA**. A few key highlights:

- While many of these CRLs have already been disclosed as part of the "Other Action Letters" section of publicly posted drug approval packages, some have not.
- There are multiple CRLs for supplemental New Drug Applications (sNDAs) that had not yet been disclosed, reflecting the fact that approval packages for sNDAs are not consistently posted in the same manner as original NDA approvals.
- Some of these CRLs were issued for products approved before 2020, suggesting that the CRL database scope may exceed the time frame identified in the FDA's announcement.
- At least one CRL has been posted for a product approved as recently as June 2025. For this product, no other portions of the approval package (beyond the label and approval letter) have yet been posted on Drugs@FDA.

Notably, the FDA's announcement references a 2015 analysis conducted by FDA researchers, which found that sponsor disclosures of CRLs did not consistently provide full detail regarding the Agency's specific concerns. The FDA's highlighting of this finding, coupled with the Agency's statement that it plans to publish additional CRLs from its archives, warrants attention from sponsors, especially public company sponsors.

Sponsor disclosures regarding CRLs are always closely scrutinized, and the FDA's move to (1)

centralize and regularly release CRLs, and (2) publish additional CRLs (e.g., those for sNDAs, or very recently approved products) is likely to invite further scrutiny—by investors, analysts, competitors, and patient communities. Sponsors should prepare disclosures around receipt of a CRL with the expectation that the CRL itself will become public upon approval of an application. Even where a product is ultimately approved, third parties may make comparisons between a sponsor's characterization of a CRL and the later-posted CRL itself.

According to the FDA, publication of CRLs is just one step in the Agency's broader transparency push. Our team will continue to monitor the frequency and scope of additional releases, as well as any opportunities for interested stakeholders to provide comments or feedback to FDA on its plans.

Most Favored Nation Drug Pricing Executive Order Resurrects Prior President Trump Policy

On May 12, 2025, President Trump signed the most recent Executive Order on drug pricing, **Delivering Most-Favored-Nation Prescription Drug Pricing to American Patients**. This latest Executive Order simultaneously pushes key stakeholders (i.e. foreign governments and drug manufacturers) to modify their current practices while threatening potential most-favored nation (MFN)-based price caps and other scrutiny. The Executive Order Fact Sheet is available **here**.

Read the full alert **here**.

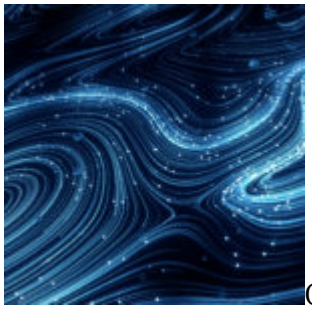
Charting a Conditional Approval Pathway for Rare Disease Drugs – A Top Priority for a Revamped FDA?

On April 18, U.S. Food and Drug Administration (FDA) Commissioner Marty Makary **announced plans** to roll-out a new approval pathway for rare disease drugs. Commissioner Makary's comments build on sentiments expressed across both the patient community and industry that rare disease drug development needs greater regulatory flexibility in order to speed access to treatments for patients with no or limited options. This is an initiative that has also been **trumpeted by Janet Woodcock**, former Principal Deputy Commissioner and Acting Commissioner of the FDA, in her work since retiring from the FDA. Prior legislative proposals (including the "Promising Pathway Act" **proposed** in 2024) have attempted to create a time-limited conditional approval pathway in the rare disease space, and Commissioner Makary's remarks may signal a renewed push for action.

In last week's interview, Commissioner Makary emphasized the following potential eligibility factors in how he is thinking about a new "conditional" approval pathway: rare conditions affecting only a small number of people, where a randomized clinical trial has not been conducted and is not feasible, but where a "plausible mechanism" physiologically exists. Commissioner Makary also noted that post-approval monitoring of adverse events and other data may be an important tool to support more flexible regulatory decision making about drug approvals.

Whether *and when* the FDA or Congress will take further steps in outlining a conditional approval pathway, and what form that outline may take (e.g., Agency guidance, expansion of the current accelerated approval authorities, or new legislation), remains unclear at this time. This is an area rare disease researchers and developers should monitor for developments, including any opportunities to provide comments to the FDA on its potential plans.

How to (Finally) Get Your SIUU Out: FDA Issues Final Guidance on Communicating Off-Label Scientific Information


On January 7, 2025, FDA announced the availability of a final guidance document titled "Communications From Firms to Health Care Providers Regarding Scientific Information on Unapproved Uses of Approved/Cleared Medical Products." The **final guidance**

supersedes the agency's revised draft guidance of the same title issued in October 2023 (see our analysis of the draft guidance **here**) and includes several key updates, including further describing scientific standards for appropriate source publications, providing additional examples of the separate dissemination of information on approved and unapproved uses in different scenarios, and expanding the section on firm-generated presentations with further context on what is permitted and what would be viewed as inappropriate when an SIUU communication includes a source publication and firm-generated content.

Several of these updates appear to be responsive to comments from industry stakeholders on the draft guidance. For example, the draft guidance stated that source publications for SIUU communications should describe "scientifically sound" studies and analyses that provide "clinically relevant" information. Multiple commenters requested that the "clinically relevant" and "scientifically sound" concepts be either removed or more clearly defined. The final guidance no longer contains the "clinically relevant" terminology, but provides some further recommendations on what constitutes a "scientifically sound" study or analysis, noting for example that certain early-phase studies *could* meet this standard.

Similar to the draft guidance, the final guidance document is written in a question and answer format and addresses: (1) what firms should consider when determining whether a source publication is appropriate to be the basis for an SIUU communication; (2) what information should be included as part of an SIUU communication; (3) how SIUU communications should be presented (e.g., the format and accompanying disclosures); and (4) recommendations for specific types of materials (including reprints and clinical reference resources). The final guidance includes a new question and answer focusing specifically on recommendations for firm-generated presentations.

The final guidance also provides an expanded list of examples of communication techniques that FDA regards as "encouraging" an unapproved use of a medical product. In addition to celebrity endorsements, premium offers, and gifts (which were noted in the draft guidance), the final guidance identifies emotional appeals unrelated to scientific content, promotional tag lines, and jingles, along with "calls to value" that "pre-judge the benefit(s) of the medical product for individual patients" (e.g., "Click here to start improving your patients' lives today"), as techniques that would take a firm-generated presentation *outside* the scope of the guidance's enforcement policy.

FDA has submitted the guidance to the Office of Management and Budget for review and clearance of certain information collection provisions contained in the guidance. As such, the final guidance is not for current implementation, but we expect to see a Federal Register notice about the final guidance's applicability once this administrative step is complete.

Please contact any of the authors or your Goodwin attorney if you have any questions about this final guidance.

How to Get Your SIUU Out: FDA Provides Long-Awaited Update for Industry on Communicating Off-Label Information

On October 23, 2023, FDA announced the availability of a revised draft guidance titled "Communications From Firms to Health Care Providers Regarding Scientific Information on Unapproved Uses of Approved/Cleared Medical Products." The draft guidance supersedes the agency's 2014 draft guidance, "Distributing Scientific and Medical Publications on Unapproved New Uses," and it provides more direction for industry on how information regarding unapproved uses of approved/cleared medical products can appropriately be shared with healthcare providers (HCPs).

The draft guidance coins a new acronym, SIUU, for scientific information on unapproved uses of an approved/cleared medical product, and provides recommendations for how to communicate SIUU in a "truthful, non-misleading, factual, and unbiased" manner. FDA explains that HCPs can prescribe medical products for unapproved uses when they determine that an unapproved use is medically appropriate for a given patient, but it is critical that company communications about unapproved uses include all of the information necessary for HCPs to evaluate the strengths, weaknesses, validity, and utility of the information about the unapproved use to make these determinations.

The revised draft guidance is organized in a question and answer format and addresses: (1) what firms should consider when determining whether a source publication is appropriate to be the basis for an SIUU communication; (2) what information should be included as part of an SIUU communication; (3) how SIUU communications should be presented (e.g., the format and accompanying disclosures); and (4) recommendations for specific types of materials (including reprints, clinical reference resources, and firm-generated presentations of scientific information from an accompanying reprint).

For industry stakeholders looking to understand what is new and/or different about these recommendations relative to the 2014 draft guidance, we note that the agency continues to recommend providing disclosures about how the information in these communications compares with the FDA-approved labeling, and that such communications be non-promotional in nature. However, the revised draft guidance provides more insight into what studies or analyses are "scientifically sound" and provide "clinically relevant information," such that they could be the basis for SIUU communications. Scientifically sound studies or analyses should "meet generally accepted design and other methodological standards for the particular type of study or analysis performed, taking into account established scientific principles and existing scientific knowledge." Clinically relevant information is information that is pertinent to HCPs when making clinical practice decisions for an individual patient. FDA notes that while randomized, double-blind, controlled trials are the most likely to provide scientifically sound and clinically relevant information, other types of well-designed and well-conducted trials, or even analyses of real-world data, could also generate this type of information. In contrast, studies that lack detail to permit scientific evaluation, communications that "distort" studies, and data from early stages of development that are not borne out in later studies are examples of information that may not be appropriate as the basis of SIUU communications.

Another clear theme in the revised draft guidance is the need to separate SIUU communications from promotional communications. FDA explains that the use of "persuasive marketing techniques"

(such as celebrity endorsers, premium offers, and gifts) suggests a firm may be trying to convince an HCP to prescribe or use a product for an unapproved use, not merely presenting scientific content to help an HCP make an informed clinical practice decision, and thus would fall outside the scope of the enforcement policy outlined in the revised draft guidance. FDA also recommends several ways to separate SIUU communications from promotional communications, including using "dedicated vehicles, channels, and venues" for SIUU communications that are separate from those used for promotional communications—such as distinct web pages that do not directly link to each other, sharing the types of information via separate email messages, and dividing booth space to separate the presentation of these types of information at medical and scientific meetings. In addition, FDA advises that if a media platform has features (such as character limits) that do not allow a company to provide the disclosures recommended for an SIUU communication, then that platform should not be used to disseminate SIUU, but could be used to direct HCPs to an SIUU communication (e.g., via a link to a website).

Companies may already be following many of the recommendations in the revised draft guidance, but the updates and clarifications throughout reflect FDA's continued emphasis on ways to appropriately share accurate, scientifically sound data with HCPs to inform clinical practice decisions. In line with the agency's 2018 guidances on **communicating information that is consistent with product labeling** and **communicating with payors, formulary committees and similar entities**, this draft guidance acknowledges the evolving realities of medical product communications and provides guardrails for companies to assess whether and how to communicate product information that is not included in its FDA-required labeling, while at the same time reminding the industry that there are "multiple important government interests" served by statutory requirements for premarket review and the prohibition on introducing a misbranded product into interstate commerce.

Comments on the draft guidance are due December 24, 2023, and can be submitted to the docket available **here**. Please contact any of the authors or your Goodwin attorney if you have any questions about this revised draft guidance.

PhRMA Issues Updates to Longstanding Code, Addresses OIG's Speaker Program Guidance

PhRMA, the pharmaceutical manufacturer trade association, **announced on Fri. August 6** that it has revised its **longstanding Code on Interactions with**

Health Care Professionals. The revisions, which relate to the Code's treatment of speaker programs, track concerns in a **Special Fraud Alert** released late last year by the US Department of Health and Human Services Office of Inspector General. This alert criticized the drug and medical device industry practice of engaging healthcare providers to deliver educational content to potential customers or users of products through so-called "speaker programs." The OIG found in its report that speakers were selected based on past or anticipated business; that attendees of these programs were offered remuneration in the form of lavish meals and alcohol; that programs were often held in high-end locations, often without an agenda, and often without any educational content delivered at all. The OIG also noted its findings that attendees of speaker programs regularly attend the same program more than once, calling into question their educational value. The alert expressly notes OIG's "skepticism" about such programs.

PhRMA appears to be the first of the major medical products trade associations to update its code of ethics based on the OIG's November 2020 alert. The PhRMA Code revisions from August 6 appear to address the criticisms raised by OIG. PhRMA expands its section 7 discussion of Speaker Programs, emphasizing the importance of speaker programs as a real and legitimate avenue of educating customers and product users about the benefits, risks, and science of particular products. Among the revisions:

- The PhRMA Code reiterates that incidental meals of modest value may still be offered to attendees but that they should be subordinate in focus to the educational presentation. The revisions also make it clear that companies should not pay for or provide alcohol at a speaker program, one of the OIG's chief complaints in the November 2020 alert.
- The revisions make clear that the purpose of any speaker program must be to present substantive educational information designed to help address a bona fide educational need among attendees, and that only those with a bona fide educational need should be invited. The revisions also highlight that repeat attendance at a program on the same or substantially same topic is generally not appropriate unless there is a bona fide educational need for the additional information.
- PhRMA emphasizes that the venue should be conducive to informational communication – no extravagant venues, luxury resorts, high-end restaurants, or entertainment/sporting venues.
- Further, the PhRMA Code also spotlights the fact that speakers should be engaged following the guidelines for engaging consultants as described in the PhRMA Code – including selection based on expertise and professional qualifications rather than past or anticipated business.

Revisions to the new PhRMA Code become effective January 1, 2022. This gives companies just a few months to evaluate their compliance policies and to update messaging to their employees regarding the appropriate set-up and operation of speaker programs, if any revisions to current practices are required.

If you have questions about this update, please contact Matt Wetzel (**mwetzel@goodwinlaw.com**, (202) 346-4208).

FDA Answers New Questions on Foreign Trial Sites Operating Under INDs

 On May 19, 2021, the U.S. Food and Drug Administration (FDA) released an **updated guidance** in draft form on how to complete the Statement of Investigator form (Form FDA 1572). The guidance addresses frequently asked questions from sponsors, clinical investigators, and institutional review boards (IRBs), and it provides new information on waivers of the Form FDA 1572 signature requirement, which is particularly relevant for sponsors of clinical trials that include sites located outside the U.S.

Form 1572 is an agreement signed by an investigator to provide certain information to the sponsor and comply with FDA regulations on conducting a clinical investigation of an investigational drug or biologic, and under 21 CFR Part 312, an investigator must sign this agreement before participating in a trial. FDA's **previous guidance** on the Form 1572 requirements and process, issued in 2010, touches briefly on the responsibilities of investigators conducting foreign studies under an investigational new drug application (IND) in the U.S., but it does not go into detail on how sponsors should proceed when an ex-U.S. investigator cannot or will not sign the 1572 (e.g., because the commitments for investigators on the Form 1572 extend beyond or conflict with what local law requires).

Under the updated guidance, FDA provides detailed steps for sponsors to request a waiver of the Form 1572 signature requirement for foreign investigators. A Form 1572 waiver allows a trial at a foreign site to take place under an IND even when the investigator cannot or will not sign the Form 1572, as noted above. When requesting a waiver, the sponsor should propose an alternative course of action to adequately satisfy the purpose of a signed Form 1572, and the sponsor must request and receive a 1572 waiver for an investigator before the study is initiated at the investigator's site. Importantly, the guidance provides examples of sponsor and investigator commitment statements that would satisfy FDA's guidelines for granting a waiver, and FDA recommends using these templates to enable FDA's efficient review of a waiver request.

Overall, the guidance provides greater clarity on when a Form 1572 waiver would be needed and how a sponsor can obtain one. Sponsors planning to conduct a clinical study at a foreign site under an IND should review the updated guidance and, if a waiver is needed, factor in time for submission and FDA review of a waiver request before initiating the trial at a foreign site. Additionally, sponsors should ensure that clinical trial agreements with foreign sites contemplate Form 1572 completion and signatures and/or waivers when necessary.

Are Pre-Approval and Pre-Licensure Inspections Limiting Approvals During

COVID-19?

 In this post, we discuss FDA's conduct of inspections of manufacturing facilities for new drugs and biologics during the COVID-19 pandemic. These inspections, known as pre-approval and pre-licensure inspections (PAIs/PLIs, respectively), are performed to give FDA assurance that a manufacturing site named in a new drug or biologics license application is capable of manufacturing the product according to current good manufacturing practices (cGMPs) and producing the product at commercial scale.

In **July**, FDA resumed limited domestic on-site inspections after temporarily postponing all domestic and foreign routine surveillance facility inspections in March. Since **June**, FDA had conducted only mission-critical domestic inspections. Currently, domestic on-site inspections are pre-announced and are prioritized on a newly developed rating scale that uses real-time data on the number of COVID-19 cases in a local area to qualitatively determine when and where it is safest to conduct inspections. Foreign PAIs/PLIs continue to be temporarily postponed unless deemed mission-critical. FDA may deem an inspection mission-critical based on a variety of factors including, but not limited to, whether the product has received breakthrough therapy or regenerative medicine advanced therapy designation.

In response to COVID-19, FDA has used, on a limited basis, various tools to conduct alternative inspections. These tools include the use of FDA's authority under Section 704(a)(4) of the FD&C Act, which enables the Agency to request records directly from facilities "in advance of or in lieu of" drug inspections. In addition, FDA has indicated that it may also look to records of recent inspections and information shared by foreign regulatory partners through mutual recognition agreements. And while the concept of virtual inspections has been floated, it remains to be seen if video-based or other virtual inspection strategies can be used to fulfill PAI/PLI requirements and how long such proposals may take to implement.

Worryingly, FDA explains in its **August 2020 guidance** that should the Agency determine that a PAI/PLI is necessary, and such an inspection cannot be completed during the review cycle due to restrictions on travel or other COVID-19-related risks, FDA generally intends to issue a Complete Response letter or may defer action. The guidance, along with a number of concerns raised quietly by sponsors regarding delayed inspections leading or potentially leading to Complete Response letters, paints a potentially ominous picture for drug and biologic approvals and the advancement of the public health over the coming months. Sponsors submitting marketing applications in the near-term would be wise to proactively prepare for discussion of alternative inspection approaches during the review of their applications.