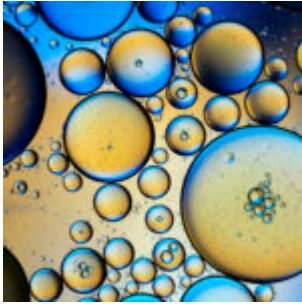


[A Look Ahead in Life Sciences: What We Are Tracking in Q2 2024 and Beyond](#)



As the life sciences, medtech, and diagnostic industries continue to expand and grow increasingly complex, so does the legal, regulatory, and compliance landscape. To help companies and investors navigate the many evolving and emerging laws and regulations across pharmaceuticals, biologics, medical devices, diagnostics, and laboratory testing, our Life Sciences Regulatory & Compliance team has provided an overview of key developments. We update and publish a quarterly tracker detailing these developments. You can read about the Q2 2024 updates [here](#).

[FDA's Laboratory Developed Test \(LDT\) Final Rule Under OIRA Review; Subcommittee on Health to Hold Hearing on Regulation of Diagnostic Tests](#)



On March 1, 2024, the Office of Information and Regulatory Affairs (“OIRA”), Office of Management and Budget (“OMB”), Executive Office of the President **received** the final version of FDA’s rule on regulation of laboratory developed tests (“LDTs”) for administrative review. Having swiftly moved to OIRA review in under 5-months from the publication of the **proposed rule** and under 3-months from the end of its comment period, the rule has undoubtedly been a top priority for the FDA. Further, as of the date of this post, OIRA has **scheduled** four back-to-back meetings with interested stakeholders, all of which are to be held the week of March 18th. All of this signals that the final rule remains on track for potential issuance in April 2024, the target date for final action on the rule as we previously discussed [here](#).

Further, on March 14, 2024, the House Energy and Commerce Committee Chair and Subcommittee on Health Chair announced a subcommittee hearing titled “Evaluating Approaches to Diagnostic

Test Regulation and the Impact of the FDA’s Proposed Rule.” The hearing is scheduled for Thursday, March 21, 2024 at 10:00 AM ET. Additional information on attending or viewing the hearing is available [here](#).

Be sure to bookmark our dedicated [LDT Resource Page](#) to stay informed on the latest news and analyses on LDTs.

[Goodwin’s Annual Rare Disease Symposium](#)



Goodwin’s Life Sciences team will be hosting an upcoming event in our Boston office on March 13, 2024 to spotlight the critical work being done to address the 7,000+ rare diseases that impact more than 300 million people globally.

Join us [in person](#) in our Boston office or attend [virtually](#) for our Annual Rare Disease Symposium on March 13, 2024. Look forward to an afternoon of engaging fireside chats, inspirational presentations, and networking with your peers in the rare disease community. This year’s program will include speakers covering the patient, advocacy, policy, research, and CEO’s perspectives.

[Master\(ing\) Protocols for Randomized Umbrella and Platform Trials](#)



The U.S. Food and Drug Administration (FDA) recently issued a draft guidance, “[Master Protocols for Drug and Biological Product Development](#)”, that echoes and builds on principles that the Agency previously set forth in guidance for [COVID-19 master protocols \(2019\)](#), [master protocols in oncology \(2022\)](#) and [clinical trials for multiple versions of cellular or gene therapy products \(2022\)](#). The draft guidance offers numerous (and at times *very* detailed) recommendations to facilitate the design, efficient analysis of data, and regulatory review of clinical trials conducted under such master protocols.

As a starting point, this draft guidance defines several key terms, including the types of trials that can be conducted under a master protocol:

Master Protocol	a protocol designed with multiple substudies, which may have different objectives and involve coordinated efforts to evaluate one or more medical products in one or more diseases or conditions within the overall study structure.
Umbrella Trial	evaluates multiple medical products concurrently for a single disease or condition
Platform Trial	evaluates multiple medical products for a disease or condition in an ongoing manner, with medical products entering or leaving the platform
Basket Trial	evaluates a medical product for multiple diseases, conditions, or disease subtypes

Master protocols offer sponsors the ability to streamline drug development through shared control groups, study infrastructure and oversight. However, these protocols also involve increased complexities and design challenges that generally require a higher degree of coordination. Here, we highlight some key design and analysis considerations addressed in the draft guidance:

Randomization

Sponsors should consider allocating more subjects to control arms than for each individual drug arm to increase power and reduce the risk of a poorly or highly performing control arm. For a platform trial, a sponsor should create a plan for changes to the randomization ratios that can occur as products enter and exit a platform trial. In umbrella or platform trials comparing different drugs, the sponsor should ensure that the randomization process prevents subjects from being randomized to drugs they are not eligible to receive given each drug's exclusion criteria.

Informed Consent

Sponsors should cover all treatment arms in their informed consent and obtain consent prior to randomization. In a platform trial where drugs are entering and exiting the study, consent forms should be modified accordingly to reflect the drugs currently under evaluation. FDA also recommends the use of a central IRB to review informed consent forms, the protocol, and other relevant documents for monitoring of a trial conducted under a master protocol.

Blinding

Given the potential for different administration methods for various drugs included in umbrella or platform trials, unique blinding challenges may arise and sponsors should discuss their proposed approach to blinding with FDA early in the planning stage.

Safety Data

Safety data from a master protocol can be considered part of overall safety database but data from other sources may be needed to support approval. The type of master protocol and the drugs being evaluated will impact the approach to safety data collection. FDA also recommends that a data monitoring committee (DMC) or other independent, external entity review accumulating safety and efficacy data to minimize inadvertent dissemination of information that could pose risks to trial

integrity.

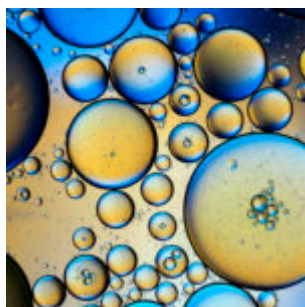
Regulatory Review Considerations

Each master protocol should be submitted as a new IND, and FDA recommends that the sponsor request a pre-IND meeting to discuss the protocol and other IND submission details. Given the potentially rapid pace of changes in a master protocol, the draft guidance recommends specific procedures for protocol amendments, including cover letters for each protocol amendment that update on the status of each drug and notifying the RPM at least 48 hours before submitting any protocol amendment that could substantively affect the master protocol. The IND should also include a well-designed communication plan to facilitate timely and effective communication between multiple stakeholders, including rapid communication of serious safety information and protocol amendments to investigators and FDA.

* * * *

Comments on this draft guidance are due February 22, 2024. Please contact the authors or your Goodwin attorney with any questions or if you would like to submit a comment.

[A Look Ahead in Life Sciences: What We Are Tracking in Q1 2024 and Beyond](#)



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[FDA Targets April 2024 for Laboratory](#)

[Developed Test \(LDT\) Final Rule](#)

On December 6, 2023, the Office of Information and Regulatory Affairs (“OIRA”) released the [Fall 2023 Unified Agenda of Regulatory and Deregulatory Actions](#) (the “Agenda”), a semiannual compilation of information regarding regulations under development by federal agencies. In its [preamble](#), the Department of Health and Human Services (“HHS”) notes that the regulatory actions forecasted for the Agenda reflect the priorities of HHS Secretary Xavier Becerra and the Biden-Harris Administration, HHS, and the U.S. Food and Drug Administration (“FDA”).

As we analyzed in detail in recent articles (see [here](#), [here](#) and [here](#)), the [proposed rule](#) for laboratory developed tests (“LDTs”) was released in October 2023. Citing factors including “extensive background of public comment on this topic” and “the public health benefits of proceeding expeditiously,” FDA [declined](#) to extend the 60-day comment period, which closed on December 4, 2023. FDA received over [6,000 comments](#) from individual citizens, laboratories, academic medical centers, and other industry stakeholders. As part of the Agenda, FDA has [updated](#) the target date for final action on the LDT proposed rule to **April 2024**.

FDA is under no obligation to publish the LDT rule according to the schedules reflected in the Unified Agenda. If the rule and related LDT policy are finalized as proposed by April 2024, **high-risk LDTs** may be called-in for premarket review as early as **October 1, 2027**. Subsequently, **low-to-moderate risk LDTs** may be called-in for premarket review as early as **April 1, 2028**.

To stay informed on the latest news and analysis affecting LDTs, be sure to bookmark our dedicated [LDT Resource Page](#).

[Significant 340B Drug Pricing Program Litigation May Impact 340B Scope](#)



Two recent federal court cases signal new significant developments with respect to the 340B Drug Pricing Program. Specifically: (1) new federal district court litigation challenging a recent HRSA Notice involving 340B Program “child site” registration and eligibility; and (2) a court decision in other litigation that implicates the scope of the 340B “eligible patient” definition. Details regarding these developments are in the client alert.

Read the client alert [here](#).

[2023 State Drug Transparency Law Development Update](#)

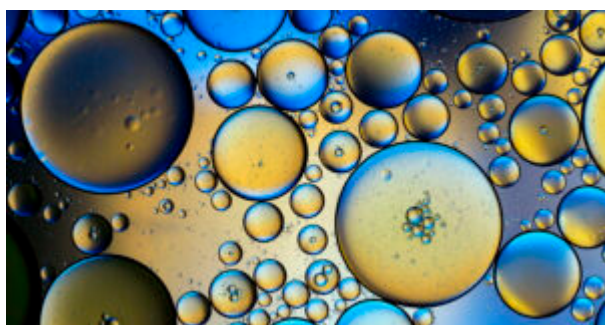


In October 2021, we [reported](#) on an uptick in the passage of state drug price transparency legislation. As an update to that report, as of October 2023, approximately 22 states have now passed drug price transparency laws creating new requirements for drug manufacturers.

Each state has its own unique set of requirements, but reporting is often completed via an online portal administered by the state's implementing agency. Generally, these laws require manufacturers to report pricing and other information related to the cost, development, and sale of drugs to the state or state-affiliated entities. Some states will use this data to produce public reports about the cost of prescription drugs with the goal of creating pricing transparency for drug manufacturers as well as to educate the state legislature and public about the drug pricing process.

Read the full alert [here](#).

[Newly Launched: Goodwin's Laboratory Developed Tests Resource Page](#)



Our Life Sciences Regulatory & Compliance team has launched a new resource page, keeping you up-to-date on the latest regulatory developments affecting laboratory developed tests (LDTs). Our dedicated LDT page provides foundational materials, legislative and regulatory history, and updates and analyses regarding initiatives to increase oversight over LDTs, including FDA's LDT Proposed Rule (October 2020). Our Life Sciences Regulatory & Compliance team will continue to keep this page updated with the latest happenings.

Read the full announcement [here](#).

[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.