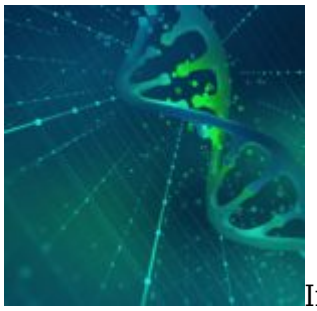
Lawsuit Filed Challenging FDA Final Rule Regulating Laboratory Developed Tests

On May 29, 2024, a lawsuit was filed in the U.S. District Court for the Eastern District of Texas, challenging the U.S. Food and Drug Administration's **final rule** concerning the regulatory status of laboratory developed tests ("LDTs") under the Federal Food, Drug and Cosmetic Act ("FDCA"). As detailed in our prior analysis (**here**), the final rule amended the FDA's existing regulations to make explicit the agency's interpretation that LDTs are "devices" under the FDCA, and established a five-stage plan to phaseout the agency's current general policy of "enforcement discretion" with respect to LDTs.

With the final rule's July 5 effective date looming, two entities—a trade association and a laboratory—filed suit in federal court to overturn the final rule. In this Insight, we briefly summarize the legal theories advanced in the lawsuit and likely next steps.

Read the full alert **here**.

Designating a Platform Technology: FDA's Long-Awaited Draft Guidance



In newly released **Draft Guidance** from the U.S. Food and Drug Administration (FDA) entitled, *Platform Technology Designation Program for Drug Development*, the FDA addresses its new designation program for platform technologies, which is intended to bring efficiencies to drug development, manufacturing, and review processes for applications that incorporate designated platform technologies.

Read the full alert **here**.

FDA Finalizes Rule and Sets Course to Phase In Oversight of Laboratory Developed Tests

 On May 6, 2024, following more than a decade of discourse with interested stakeholders on potential approaches to regulation of laboratory developed tests (LDTs), the U.S. Food and Drug Administration (FDA) published its **final rule** setting forth its framework for oversight of LDTs. The final rule and accompanying policy to phase out the agency's general policy of "enforcement discretion" for LDTs comes roughly six months after FDA published its **proposed rule** that outlined the agency's proposed approach to increasing oversight over LDTs. As detailed in our prior analyses of the proposed rule (see **here** and **here**), FDA proposed to implement a **phaseout policy** that would, across five stages and within four years, apply to clinical laboratories offering tests as LDTs the same regulatory requirements applicable to in vitro diagnostics (IVDs).

The proposed rule received more than **6,500 comments**, and while FDA did not change its amendments to the regulation or meaningfully modify the phaseout timeline, FDA has significantly modified its phaseout policy to extend full or partial enforcement discretion to additional categories of LDTs, creating a framework whereby the agency intends to take a more targeted enforcement approach, particularly in the near-term, to addressing LDTs.

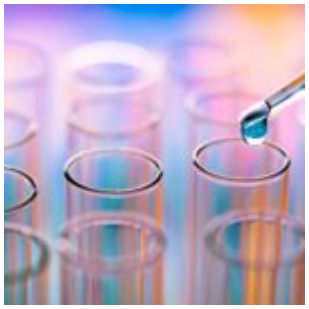
You can read our more in our **Insight**, where **Steven Tjoe**, **Matt Wetzel**, and **Sukrti Thonse** highlight the key features of the final rule and five-stage phaseout policy. Be sure to bookmark our dedicated **LDT Resource Page** to stay informed on the latest news and analyses on LDTs.

FDA Issues Final Rule on Regulation of Laboratory Developed Tests

 On April 29, 2024, the U.S Food and Drug Administration (FDA) announced its **final rule** on Laboratory Developed Tests (LDTs). This final ruling amends the FDA's regulations to make explicit that *in vitro* diagnostic products (IVDs), including those manufactured by laboratories, are devices under the Federal Food, Drug, and Cosmetic Act (FD&C Act). Alongside the amendment, FDA issued its policy to phase in regulatory requirements for certain LDTs over the course of four years.

The FDA will host a webinar to provide an overview of the final rule on May 14, 2024. A link to register can be found **here**. The final rule is expected to have profound effects on many LDT developers. Goodwin's **Life Sciences Regulatory & Compliance Team** are ready to work with clients to navigate the challenges that the final rule may pose. Our breakdown and analysis of the rule will be upcoming on **Goodwin's LDT Resource page**.

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.

FDA Targets April 2024 for Laboratory Developed Test (LDT) Final Rule

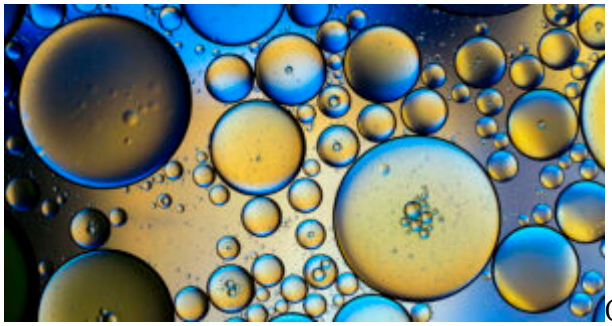
On December 6, 2023, the Office of Information and Regulatory Affairs ("OIRA") released the **Fall 2023 Unified Agenda of Regulatory and Deregulatory Actions** (the "Agenda"), a semiannual compilation of information regarding regulations under development by federal agencies. In its **preamble**, the 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**.

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**.

Recent FDA Initiatives to Support Development of Individualized Cell and Gene Therapies and Rare Disease Therapies

 Last month, FDA issued a **Request for Information** (RFI) in the Federal Register seeking information and comments from interested stakeholders regarding "critical scientific challenges and opportunities to advance the development of individualized cellular and gene therapies (CGTs)." Individualized CGTs are therapies "developed for a single patient (or a very small number of patients) based on designing or engineering a product that specifically targets the mechanism underlying a patient's (or small number of patients') illness."

FDA's request focuses on three core areas:

1. Manufacturing: Manufacturing and product quality challenges and opportunities for individualized CGTs in light of, for example, small batch sizes, tailoring of batches to individual patients, and need for rapid testing and release.

On this topic, FDA asks:

i. *Given the challenges to develop consistent manufacturing strategies for CGTs designed for a*

very small number of patients or an individual patient, how can manufacturers leverage their prior experience manufacturing one CGT to support subsequent development and approval of another related, but distinct CGT (potential areas for leveraging may include manufacturing process validation, control strategy, assay validation, and drug product stability studies)?

ii. *When the batch size of a CGT is very small, what are some challenges and solutions regarding the volume of product (or number of vials) needed for batch release testing, stability testing, retention of reserve samples, and comparability studies?*

iii. *What are some challenges and solutions for individualized CGTs that need to be tested and released rapidly, either because the product has a very short shelf life or because the patient's clinical status may be rapidly declining and treatment is urgently needed?*

iv. *For many individualized CGT products, each batch is tailored to an individual patient (e.g., autologous CAR–T cells, tumor neoantigen vaccines, certain genome editing products). For such products, what are some challenges and solutions for assuring that each batch has adequate potency to achieve the intended therapeutic effect?*

v. *What are some challenges and solutions for individualized genome editing products that aim to treat monogenic diseases for which the target gene has different mutations in different patients?*

2. Nonclinical development: The use of nonclinical data to support individualized CGTs, considering the lack of relevant animal models in many instances, the uniqueness or limited applicability of individualized CGTs, and the potential of using prior knowledge from other CGTs—for example, where gene therapy vector products use the same vector backbone.

On this topic, FDA asks:

i. *What nonclinical studies could be leveraged in support of a related product using similar technologies? What nonclinical studies are important to conduct with each final clinical product?*

ii. *What nonclinical development approaches could be considered when there are no relevant animal models or animal models are unable to replicate each individual disease/condition?*

iii. *For patient-specific products where evaluating each individual product is infeasible or impractical, what is the role for nonclinical studies conducted with representative product(s)?*

iv. *What are the opportunities and challenges with using computational approaches to support nonclinical development?*

3. Clinical Development: Clinical development of individualized CGTs, considering for example the infeasibility (for ethical or other reasons) of conducting randomized controlled studies, novel endpoints, and limitations in statistical analyses.

On this topic, FDA asks:

i. *What are challenges and strategies/opportunities with interpreting efficacy data from individual patients (including expanded access) and small groups of patients? What*

opportunities are there in leveraging prior and/or collective experiences?

ii. *What strategies can be utilized to accumulate and interpret safety data in personalized/individualized CGTs?*

iii. *For genetic disorders with clear genotype-phenotype associations for disease manifestations or severity, what opportunities are there for tailoring treatments and study design to specific genotypes/phenotypes?*

FDA also requested input on several additional significant questions:

i. *What additional major scientific challenges to advance the development of individualized CGTs should be considered?*

ii. *What existing best practices or scientific approaches should be leveraged to address any of these challenges? Are there specific opportunities for collaborations to advance the development of individualized CGTs?*

iii. *Are there specific areas where flexibility in regulatory approaches would improve the feasibility of developing and commercializing individualized CGTs?*

Comments are due on November 20, 2023.

At the end of last month, FDA also **announced** a pilot program "to help further accelerate development of rare disease therapies." The program, titled Support for clinical Trials Advancing Rare disease Therapeutics ("START"), will include selected sponsors with an active IND for products meeting certain eligibility requirements. Products regulated by CBER are eligible for the program only if they are a gene or cell therapy treatment for a rare disease or condition that is "likely to lead to significant disability or death within the first decade of life." Products regulated by CDER are eligible only if they are "intended to treat rare neurodegenerative conditions, including those of rare genetic metabolic type." Participants selected for the pilot program will "be able to obtain frequent advice and regular ad-hoc communication with FDA staff to address product-specific development issues, including, but not limited to, clinical study design, choice of control group and fine-tuning the choice of patient population."

FDA will accept applications to the START program beginning January 2, 2024 and until March 1, 2024.

Mark Your Calendars: This Halloween, Don't Miss FDA's LDT Webinar

The U.S. Food and Drug Administration (FDA) has announced an upcoming **webinar** on its **proposed rule** on the regulation of laboratory developed tests (LDTs).

The webinar is scheduled for **October 31, 2023 from 1:00 – 2:00 PM ET** and will include an overview of the proposed rule, a description of the proposed phaseout of FDA's general enforcement discretion approach to LDTs, and a question and answer session. Stakeholders must submit questions by **October 23, 2023** to be considered for the discussion.

For our detailed analysis of the 83-page proposed rule, please see our two-part Insight series: **Part I: Underpinnings of FDA's Proposed Rule** and **Part II: FDA's Proposed Phaseout Policy – Key Considerations & Open Questions**.

If you have questions on the proposed rule or its potential impact, contact the authors or a member of the **Goodwin Life Sciences Regulatory & Compliance** team.

FDA's Proposed Rule for Oversight of Laboratory Developed Tests: Part II: FDA's Proposed Phaseout Policy – Key Considerations & Open Questions

After an over decade-long discourse amongst interested stakeholders, on October 3, 2023, FDA unveiled its **proposed rule and policy** to increase oversight over LDTs.

If finalized as proposed, FDA would implement a new "phaseout policy" that would, across five stages and within four years, apply the same regulatory requirements applicable to in vitro diagnostics (IVDs) on the majority of clinical laboratories offering tests as LDTs. Once implemented, tests offered as LDTs that do not meet the applicable regulatory requirements, including premarket review and the quality system regulation, may be expected to come off the market.

In our **first post** in this Insight series, we recapped the underpinnings of the proposed rule and policy, including the significant discussions contained in the proposed rule on (1) the rationale for the agency's proposed phaseout policy and (2) FDA's legal authority for issuing the rule.

In this Insight, we provide our full analysis of FDA's proposed five-stage phaseout policy and the open questions that remain. Read the full Insight **here**.