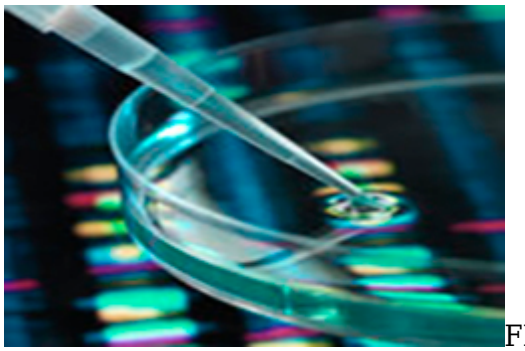
Move Fast: FDA is Accepting Submissions for the Pilot Program Class for FDA Commissioner's National Priority Voucher Program



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.

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.

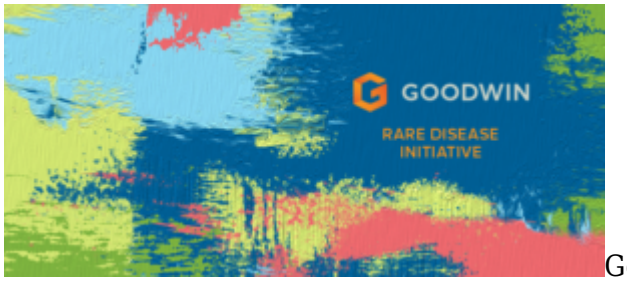
Whether *and when* the FDA or Congress will take further steps in outlining a conditional approval pathway, and what form that outline may take (e.g., Agency guidance, expansion of the current accelerated approval authorities, or new legislation), remains unclear at this time. This is an area rare disease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

Goodwin's 2025 Rare Disease Symposium: Momentum Builds for Addressing Critical Diagnosis and Treatment Gaps

Attendees at this year's **symposium** were optimistic about the potential for progress, citing momentum from new FDA initiatives, growing legislative support, and increased global innovation in research and development. These efforts, alongside increased patient advocacy and a presidential administration focused on speeding patient access, could lead to significant advances in rare disease treatments and cures in 2025.

Read the full insight **here**.

Goodwin Invites You to Join Us For Our Rare Disease Symposium 2025



Goodwin's **Life Sciences** team is excited to host its Annual Rare Disease Symposium in Boston on February 5, 2025. Participants are invited to join for an afternoon of engaging fireside chats, inspirational presentations, and networking with peers in the rare disease community.

Please see the agenda below and register to attend **in-person** or via our **virtual webinar** to join us.

Agenda

12:00 PM – 1:00 PM EDT | Welcome & Networking Lunch

1:00 PM – 4:30 PM EDT | Rare Disease Symposium Program

- **The Patient View**
  - David Downing, GRIN1 Dad
  - Jaime McHugh, Rare Disease Mom and NORD Running for Rare Champion
- **The Research View**
  - Dr. Shira Rockowitz, PhD, Data Science Director, Boston Children's Hospital, Children's Rare Disease Collaborative Co-Leader
  - Dr. Piotr Sliz, PhD, Vice President, Chief Research Information Officer & Associate Professor, Boston Children's Hospital, Children's Rare Disease Collaborative Co-Leader
- **The FDA View**

    - Amy Rick, Director of Strategic Coalitions for FDA's Rare Disease Innovation Hub
- **The Policy View**
    - Karin Hoelzer, Senior Director, Patient Advocacy, BIO
    - Jack Kalavritinos, Founder, JK Strategies and the Washington Health Innovation Council, and Former Director, HHS Office of Intergovernmental & External Affairs
    - Judy Stecker, SVP, Burson, and Former HHS Deputy Chief of Staff for Strategy & Operations – Rare Disease Parent & Founder, Wheeler's Warriors
- **The View from the National Organization for Rare Disorders**
    - Pamela Gavin, Chief Executive Officer, NORD
- **The View from the Rare As One Network**
    - Heidi Bjornson-Pennell, Senior Program Manager, Science in Society, and Lead, Rare As One Network
- **The Biotech CEO View**
    - Paula Ragan, PhD, CEO, X4 Pharmaceuticals

4:30 PM – 5:30 PM EDT | Networking Reception

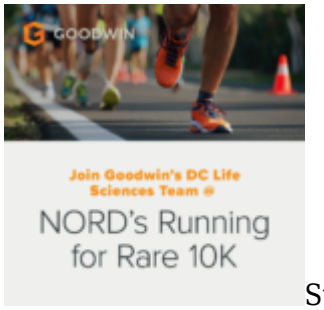
We look forward to kicking off **Rare Disease Month** with you!

New Momentum for a Time-Limited Conditional Approval Pathway for Rare Disease Drugs

 On October 4, 2024, a US House version of the revised Promising Pathway Act (PPA) 2.0 was introduced, sponsored by Rep. Bruce Westerman (R-AR). The bill (**H.R.9938**) mirrors a US Senate version that was introduced in May 2024 (**S.4426**) that would authorize the US Food and Drug Administration (FDA) to grant time-limited conditional approval to drugs for rapidly progressive, terminal diseases with substantial unmet need for treatments that are eligible for the Orphan Drug Act and result in a substantially shortened lifespan, substantial reduction in quality of life, or other substantial adverse health effects.

Read the full insight **here**.

Running for Rare for NORD



Support Goodwin's DC Life Sciences Team as they raise money for the National Organization for Rare Disorders (NORD) as part of its "Running for Rare" team at the annual 10K race held during the upcoming DC Marine Corps Marathon on October 27th.

Assist NORD's mission to drive public policy, accelerate research and improve care for people living with rare diseases by donating **here**.

Learn more about Goodwin's Rare Disease Initiative **here**.

NORD Annual Breakthrough Summit

In October, NORD will also hold its annual Breakthrough Summit in Washington, DC on October 20-22, 2024. This event draws over 1,000 attendees including patients/caregivers, patient advocacy organizations, and healthcare, biotech, and medical technology companies. Registration is available **here**. This year, **Matt Wetzel** will take part in a panel discussion on the growing role of medical devices in the rare disease community.

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.

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.

Leveraging Investigator-Initiated Trials in Rare Disease Drug Development

Investigators interested in rare disease treatment development have the opportunity to approach drug and biologic developers to obtain investigational drug supply for trials in which the investigators, typically at academic institutions, act as sponsor-investigators. Similarly, companies open to extending their product development pipelines can look to investigator-initiated trials as a

mechanism to better understand the overall safety profile for their product candidates while exploring the potential therapeutic utility of their product candidates in diseases where unmet medical needs remain. So often, those needs exist in rare diseases where populations are small and investment returns are difficult to project. Drug developers deciding whether to supply investigational products to sponsor-investigators looking to explore therapeutic potential in areas of their research interests should evaluate what level of involvement to exercise over the investigator-initiated trial. We highlight some of these considerations below.

Ultimately, drug developers hold the decision-making power over whether to allow investigator-initiated research for their product candidates. Thereafter, the contracting process around the setup of an investigator-initiated trial and clinical supply agreement provides drug developers the opportunity to negotiate their level of involvement in the research of their candidates. In negotiating the setup of investigator-initiated research supply, drug developers often balance their support of research into what are often unmet needs with limited company resources, limited supply that may be available and any potential risks that may flow from trial learnings in the proposed disease space. As an upside, investigator-initiated trials afford developers the opportunity to extend their research reach and product development pipelines, so any interest by investigators to conduct research with industry candidates warrants consideration.