



FDA Seeks Feedback on Rare Disease Drug Development

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On October 14, 2026, the Food and Drug Administration (FDA) announced the launch of a Rare Disease Framework Initiative to advance a regulatory framework “purpose-built” for the development and review of rare disease therapies. Developing therapies for very small patient populations can present challenges in drug development and the vast majority of rare diseases have no FDA-approved treatments. This initiative is intended to help respond to these challenges.

The Framework Initiative is led by FDA’s Rare Disease Innovation Hub, in partnership with the Office of the Commissioner, Center for Biologics Evaluation and Research, and Center for Drug Evaluation and Research, and is intended to result in a rare disease drug development and review process that: (1) provides greater clarity, consistency and predictability regarding rare disease regulatory decision-making; (2) embeds the patient voice in the development and review process for rare disease therapies; (3) further leverages external expertise to support a tailored approach in regulatory review; (4) builds shared resources, training and institutional knowledge on rare disease drug development and review; (5) applies risk-based approaches; and (6) champions the use of cutting-edge science, novel methods and clinical trial designs, and innovative statistical approaches in the development of rare disease therapies.

In conjunction with announcing the Framework Initiative, FDA issued a Request for Information to seek feedback on persistent challenges for the development of rare disease therapies and how a tailored approach to regulatory review might mitigate them. The agency is seeking suggestions and comments in four broad areas of focus: (1) Fit-for-purpose review of rare disease therapies based on attainable evidence, particular disease states, and population; (2) consistency and predictability of FDA’s rare disease drug development and

review regulatory requirements; (3) FDA engagements with external parties—including patients, disease experts and researchers—to inform review decision-making for rare disease therapies; and (4) considerations for chemistry, manufacturing and controls specific to rare disease therapies. The agency further announced a “Fit-for-Purpose Rare Disease Framework Initiative” public meeting being convened in collaboration with the Reagan-Udall Foundation on October 28, 2026. Comments may be submitted at the public meeting, and to the public docket from October 28 until December 11, 2026.

Rare disease drug development is a consistent area of bipartisan focus on Capitol Hill, and this latest initiative and renewed focus on how FDA could advance a more fit-for-purpose regulatory paradigm may inform policymakers’ work on rare disease policy more broadly as Congress considers the next Prescription Drug User Fee Act reauthorization next year.

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