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FDA Continues Focus on Rare Disease Drug Development, Announces Evidence Principles

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On September 3, 2025, the Food and Drug Administration (FDA) announced Rare Disease Evidence Principles (RDEP) with processes aimed at providing greater predictability and facilitating the development and review of drugs intended to treat rare diseases with very small patient populations and significant unmet medical needs driven by a known genetic defect. In conjunction with the agency's announcement of the new processes jointly proposed by the Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Research (CBER), the agency also unveiled additional information regarding eligibility and the potential for post-marketing requirements for those sponsors who participate in it.

The agency aims to provide greater regulatory certainty through RDEP, including providing sponsors with greater clarity on the kind of evidence that can demonstrate substantial evidence of effectiveness given the inherent challenges in developing medicines for such small patient populations. Drug development is not one-size-fits-all and rare disease drug development presents particularly unique challenges not typically encountered in the development of drugs intended to treat larger disease patient populations. It can be particularly challenging to produce evidence to meet the statutory requirements for demonstrating efficacy through traditional trial designs and the required number of clinical studies for rare diseases given the smaller patient population. Under the RDEP process, effectiveness may be established based on one adequate and well-controlled study with robust confirmatory evidence, which may include strong mechanistic or biomarker evidence,

evidence from relevant non-clinical models, clinical pharmacodynamic data and/or case reports, expanded access data, or natural history studies.

Drug developers may want to consider how RDEP may fit within their rare disease drug development programs. RDEP may also be of interest to rare disease patients and Congress given the ongoing interest in these policy areas and ahead of the next Prescription Drug User Fee Act (PDUFA) reauthorization which is often a time in which lawmakers focus on FDA's rare disease focused work.

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Food and Drug Administration (FDA)