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FDA Presses Forward on PDUFA VII CGT Commitments with Release of Draft Guidance on Clinical Trial Design, Methods for Capturing Postapproval Data

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FDA recently released two draft guidance documents for industry related to cell and gene therapy (CGT) products as the agency seeks to advance the efficient development and review of safe and effective CGT products and fulfill related commitments set forth in the 2022 reauthorization of the Prescription Drug User Fee Act (PDUFA VII). This latest guidance may be of particular interest to CGT developers and various patient groups.

The first draft guidance provides recommendations to sponsors planning clinical trials for CGT products intended for use in a disease or condition that affects a small patient population. It expands on FDA's existing guidance related to rare disease drug development by providing additional recommendations for the planning, design, conduct and analysis of CGT trials to facilitate FDA's assessment of product effectiveness. In the draft, FDA details a non-exhaustive list of designs sponsors may consider, including single-arm trials utilizing participants as their own control, disease progression modeling and externally controlled studies. FDA also suggests several issues for sponsors to consider concerning participant selection, such as evaluating the appropriateness of requiring participants to have exhausted available therapies for clinical trial entry. FDA also recommends that product sponsors discuss options for innovative clinical trial designs with the agency as early as possible. In announcing this guidance, FDA requested comments be submitted by November 24, 2025.

The second draft guidance discusses the methods for collecting postapproval study data for CGT products. Because CGT products are often approved based on small clinical trial populations, the guidance lays out how postapproval monitoring can increase understanding

of long-term safety and efficacy, help guide safe clinical use, and inform subsequent regulatory decision making. The guidance outlines several methods and approaches for capturing postapproval marketing data, including (1) using real-world data and real-world evidence (e.g., following FDA's existing real-world evidence program); (2) using electronic health records, medical claims and vital statistics data to, for example, assess rates of clinical outcomes in CGT-treated patients; (3) using registries, which can track long-term durability of response after exposure to CGTs or surveil for malignancies after receiving CGT products; and (4) decentralized data collection outside of the traditional clinical sites to facilitate more accessible and less burdensome data collection. The draft guidance acknowledges that limited preapproval data can be balanced with additional postapproval study data for CGT products, while also considering data quality, patient privacy and the unique needs of pediatric patients. In announcing this guidance, FDA requested comments be submitted by December 24, 2025.

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