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Advancing Rare Disease Measurement With The Rare Disease COA Resource

Naomi Knoble, Division of Clinical Outcome Assessment (DCOA), ODES, OND, CDER, U.S. FDA

Lindsey Murray, Rare Disease COA Consortium

It is estimated that more than 350 million people worldwide, approximately 10% of the global population, half of whom are children, live with a rare disease. Of these rare diseases, fewer than 10% have approved treatments. This significant unmet treatment need has been met with unprecedented response within the global scientific, biopharmaceutical, regulatory, and patient communities, including unprecedented growth in rare disease drug and gene therapy development and approvals. However, the well-known difficulties of conducting clinical trials (e.g., small samples, wide geographic distribution, heterogenous symptoms) and developing products for these rare indications persist, and identifying outcomes remains a hurdle. For efficacy endpoints based on clinical outcome assessments, COAs have not yet been identified, developed, or modified for most rare diseases.

To accelerate rare disease treatments by advancing outcomes measurement, the US Food and Drug Administration (FDA) funded a cooperative agreement to establish the Rare Disease COA Consortium (RD-COAC) in 2019. The RD-COAC officially launched on January 1, 2022, with the mission to enable pre-competitive, multistakeholder collaboration aimed at identifying scientifically sound tools and methodologies for collecting clinically meaningful and patient-centric outcomes data in treatment trials for rare diseases. The RD-COAC has four complementary workstreams to advance COA measurement for rare disease clinical trials: (1) Rare Disease COA Resource; (2) Advancing COA Measurement Topic-Focused Working Groups; (3) Rare Disease Discussion Sessions for pre-competitive collaboration and shared learnings among RD-COAC members; and (4) Dissemination.

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