Advancing rare disease measurement through the Rare Disease Clinical Outcome Assessment Consortium

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Abstract

There is a significant unmet need to develop and evaluate new treatments for people living with one of approximately 8000 rare diseases. Well-known difficulties in conducting clinical trials (e.g., small samples, wide geographic distribution, heterogeneous symptoms) and developing products for these rare indications persist. Identifying outcomes in rare disease clinical trials remains a hurdle that contributes to the challenges for drug and gene therapy development due to uncertainty about what aspects of a condition to measure for safety and efficacy and often with no regulatory approval precedent. 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, multi-stakeholder 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. This review provides an overview of the RD-COAC's activities to date, as well as future directions and opportunities to collaborate.

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