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Measuring Change in Rare Disease Outcomes: Implications From Patient-Focused Drug Development Guidance

Value in Health

Interpreting within-subject meaningful change in rare disease outcomes requires innovative qualitative and quantitative mixed methodological approaches due to heterogeneity among patients and limited same size. The implication for clinical research and regulatory science focuses on the ability to communicate findings at the individual and group level. This themed section will focus on innovative methodologies and research designs focused on evaluation of meaningful change in rare disease, specifically considering the Patient Focused Drug Development (PFDD) guidance and the 2009 FDA Guidance for Industry Patient Reported Outcomes.

There are many challenges to assessing meaningful change in rare indications due to limited sample size, heterogeneity of symptoms, and the varying respondents (eg, clinician, observer, patient) reporting on behalf of the patient. In rare disease, quantitative techniques such as anchor-based methods may not be sufficient due to the reduced sample size, which may necessitate the use of other approaches to understand meaningful changes to patients, such as the use of qualitative methods. This themed section aims to present challenges in interpreting clinical outcome assessment (COA) endpoints in rare disease and provide a forum to share research designs and analytic methods for assessing meaningful change in rare disease considering the new PFDD COA guidances.

Specifically, authors of papers submitted to this Themed Section should present multiple research designs (ie, qualitative, quantitative, and mixed methods analytic techniques) with accompanying data visualization approaches for deriving, analyzing, and communicating meaningful change in rare disease. The degree to which each method and research design can be implemented to support the derivation of within-subject meaningful change thresholds and inclusion of related endpoints in clinical research and regulatory science should be presented with special attention to the evidentiary guidelines set forth in the PFDD COA guidances.

This themed issue encourages research, evaluation, and synthesis of methodologies implemented to measure and present meaningful change with a focus on alignment with regulatory Patient Focused Drug Development guidance.

Focus areas:

- Introduction to Clinical Outcome Assessment Strategy in Rare Disease: Sample Size and Heterogeneity
- Research Designs for Deriving Meaningful Change in Rare Disease
- Methodological Approaches for Deriving Within-Patient Meaningful Change: Leveraging Qualitative and Mixed Methods
- Patient-Focused Drug Development Guidances: Evidentiary Considerations for Rare Disease

Please direct any content-related questions to **Stacie Hudgens** (stacie.hudgens@clinoutsolutions.com). Submissions received before **April 30, 2025** will have the best chance of being published in *Value in Health* in 2025. All invited papers will undergo the journal's peer-review process before the Editors make final decisions about papers to be included in this themed section of *Value in Health*. Authors should submit manuscripts through the journal's online submission system at https://mc.manuscriptcentral.com/valueinhealth and be sure to indicate in their cover letter that the paper is to be considered as part of the Measuring Change in Rare Disease Outcomes theme.

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505 Lawrence Square Blvd South, Lawrenceville, NJ 08648

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