

The EXACT-PRO Initiative: Development of a Standardized Outcome Measure for Evaluating Exacerbations of Chronic Obstructive Pulmonary Disease

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Background: There is no standard, validated method for evaluating the effect of treatment on exacerbations in COPD. **Objective:** This multi-sponsor initiative brought together clinical, research, methodology, and regulatory experts to develop a new patient-report outcomes (PRO) instrument to measure the frequency, severity, and duration of exacerbations in clinical trials of COPD. **Methods:** Phase I included a literature summary, patient focus groups and 1:1 interviews with COPD patients, and two expert panels to inform instrument content and structure. Themes and concepts identified in patient qualitative data informed instrument development. Experts critiqued the methods and results, identified issues to explore during cognitive debriefing, and assisted in the design of a prospective study to evaluate the properties of the tool. Phase II involved a two-group observational study of patients with a clinician-confirmed exacerbation who completed the daily diary via PDA on Days 1-28 of their exacerbation and Days 60-67. Another group considered clinically stable completed the diary over seven days. **Results:** Phase I: 71 US patients with an exacerbation within six months participated in focus groups and interviews, yielding 23 items for testing. Phase II: Data from 222 acute and 188 stable US patients were used to reduce the tool to 14 items and test reliability and validity (reported elsewhere). **Conclusions:** This multi-sponsor, collaborative effort involving clinical, research, regulatory, and patient experts led to the development and validation of the **EX**acerbations of **Chronic Pulmonary Disease Tool (EXACT)**, a new measure for evaluating outcomes of treatment in patients with COPD.

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