Attachment to

Guidance on Qualification Process for Drug Development Tools

Evaluating Respiratory Symptoms in Chronic Obstructive Pulmonary Disease, a Patient-Reported Outcome Instrument for the Measurement of Severity of Respiratory Symptoms in Stable Chronic Obstructive Pulmonary Disease: Qualification for Exploratory Use

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 90 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to http://www.regulations.gov. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document, contact Dr. Elektra Papadopoulos at 301-796-0900.

This draft guidance, when finalized, will represent the current thinking of the Food and Drug Administration (FDA or Agency) on this topic. It does not establish any rights for any person and is not binding on FDA or the public. You can use an alternative approach if it satisfies the requirements of the applicable statutes and regulations. To discuss an alternative approach, contact the FDA staff responsible for this guidance as listed on the title page.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)

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Clinical/Medical

Evaluating Respiratory Symptoms in Chronic Obstructive Pulmonary Disease, a Patient-Reported Outcome Instrument for the Measurement of Severity of Respiratory Symptoms in Stable Chronic Obstructive Pulmonary Disease: Qualification for Exploratory Use
Guidance for Industry[1]

DDT Type: Clinical outcome assessment (COA)

DDT Tracking Number: [DDTCOA-0000017]

Referenced COA: Evaluating Respiratory Symptoms in Chronic Obstructive Pulmonary Disease (E-RS: COPD)

Type of COA: Patient-reported outcome (PRO) instrument

The Center for Drug Evaluation and Research (CDER) has determined that the E-RS: COPD is qualified for exploratory use as a PRO instrument to measure respiratory symptoms in patients with stable COPD in the context of use described below.

The contact information for public access to the E-RS: COPD and its user manual also appear below.

Section I: Concept of Interest

The E-RS: COPD total score measures respiratory symptoms of stable COPD.

Section II: Context of Use

The E-RS: COPD total score is qualified for exploratory use as a PRO instrument to measure respiratory symptoms of stable COPD in clinical studies. Additional development work is needed to further assess measurement properties, including the ability to detect clinically meaningful change with treatment or to assess the effect of treatment on reducing respiratory symptoms from baseline levels.

We encourage additional research and analyses to evaluate the E-RS: COPD's longitudinal measurement properties including the amount of change in an individual patient that can be considered meaningful for use in the interpretation of effectiveness. We expect that as further experience with the instrument is gained, the qualification statement will be expanded to aid in interpretation of clinically meaningful change.

[1] This guidance has been prepared by the Office of New Drugs in the Center for Drug Evaluation and Research at the Food and Drug Administration.

A. Study population

Adult outpatients with stable COPD

B. Clinical trial design

Superiority trial

C. Endpoint positioning

The E-RS: COPD total score is currently qualified as an exploratory endpoint in clinical studies.

D. Labeling or promotional claim(s) based on the COA

The E-RS: COPD total score ultimately is intended to support labeling claims related to change in overall respiratory symptoms of stable COPD.

Section III: COA Interpretation (If Available)

We recommend that the proposed responder definitions be evaluated further. When designing clinical trials, sponsors should discuss with the appropriate CDER review division how E-RS: COPD may be used.

Section IV: Contact Information for Access to the Qualified COA

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Instructions for Use in a Regulatory Submission: Please reference DDT # [DDTCOA-0000017] and this guidance in your application.