DRUG DEVELOPMENT TOOL
LETTER OF INTENT DETERMINATION
DDT COA #000128

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Dear Drs McCarrier and Serrano,

We have completed our review of the Letter of Intent (LOI) for Drug Development Tool (DDT) COA #000128 received on January 27, 2020 by the CDER Clinical Outcome Assessments (COA) Qualification Program, submitted under section 507 of the Federal Food, Drug, and Cosmetic Act.

The LOI is for the Symptom Assessment for Bronchiectasis (SABRE) a patient reported outcome (PRO) proposed for the assessment of disease-related symptoms in adult patients, 21 years and older, with non-cystic fibrosis bronchiectasis (NCFBE) with or without non-tuberculous mycobacterial (NTM) infection.

FDA has completed its review and has agreed to accept your LOI into the CDER COA Qualification Program.

FDA's response to the questions included in the LOI can be found below.

1. Question 1: Does the Agency agree with our planned approach to use qualitative insight from the concept elicitation and cognitive interviews, conducted in groups of NCFBE patients with and without co-occurring NTM, to determine if there is a need for different PRO item content between the two patient groups?

   FDA Response: We agree that obtaining patient input in both groups of NCFBE patients (with and without co-occurring NTM) is warranted. Furthermore, we recommend you obtain qualitative evidence from patients with all levels of NCFBE severity as well as identify the types of NTM patients you will be including in your research. Note that the patients participating in your qualitative research should be similar to those that will be included in NCFBE clinical trials.

We recommend providing more details regarding how you plan to obtain qualitative evidence; therefore, we request that you send us any protocols related to your proposed qualitative research for our review and comment. We also recommend that you schedule an interim meeting with us to discuss your proposed plans for obtaining qualitative evidence.

2. Question 2: Procedurally, what process will be required within the qualification program to revise the stated COU should our qualitative and/or psychometric research findings suggest a need for supplemental instrument content (e.g., an add-on module to the core SABRE instrument) to address any identified differences in the set of symptom concepts deemed relevant and important by NCFBE patients with and without co-occurring NTM infection?

   FDA Response: We acknowledge that you are currently proposing to develop one instrument for use in both subpopulations while also acknowledging that it is unclear whether patients with NCFBE with NTM necessitate a different measure from those without NTM. At this time, it is premature to fully comment on the process for any revisions that may be made to the currently proposed context of use. At the appropriate time, we will review the qualitative evidence you provide and provide a recommendation(s) on how to best move forward.

In preparing to submit a Qualification Plan (QP), please ensure that the QP submission addresses the scientific issues and the recommendations outlined below.

1. In Section 1.1 of your QP, provide explanation regarding the gaps for existing, bronchiectasis-related COAs, such as the Quality of Life Questionnaire-Bronchiectasis NTM Module (QOL-B NTM).

2. Note that the COA DDT Qualification program qualifies instruments for use as a primary and/or secondary endpoint only, therefore we request that you revise your endpoint model to exclude the exploratory endpoint definition, as well as, the endpoint for quantifying effectiveness of treatment in open label trials and/or observational research.

Though the next milestone submission you would be working towards is a Qualification Plan (QP), you may submit your **qualitative protocol and results** for FDA review and comment prior to submitting your QP.

The following weblink contains the contents to include in your submission to reach the next milestone (Qualification Plan): www.fda.gov/media/123245/download. Please contact the CDER COA Qualification Program at COADDTQualification@fda.hhs.gov should you have any questions (refer to DDT COA #000128).

Sincerely,

Elektra J. Papadopoulos -S

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