An Analysis of Clinical Outcome Assessment Trends for Neurological and Psychiatric Drug Approvals

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SUBMISSION DETAILS

What is the Methodological Question Being Addressed? Are there any trends of clinical outcome assessment (COA) types amongst new molecular entities (NME) labeling approvals for neurological and psychiatric drugs?

Introduction The COA Compendium is part of FDA's efforts to foster patient-focused drug development. The Compendium is a living document intended to facilitate communication and to provide clarity and transparency to drug developers and researchers by collating and summarizing clinical outcome assessment (COA) information for many different diseases and conditions into a single resource. The COA Compendium collates COAs from approved drug labels dating back to 2003. There is an opportunity to review potential trends of COAs from New Molecular Entities (NMEs) that can span more than a decade. NMEs can be approved under a New Drug Application (NDA) via type 1 (New Molecular Entity) or type 1,4 (New combination with at least one of the active moieties as an NME) as well as Biologics License Applications (BLA). The COA Compendium also describes and references COAs qualified through CDER's Drug Development Tool (DDT) Qualification Program.

Methods The Drugs@FDA database was used to collate all BLAs, Type 1s and Type 1/4s approved from January 2003 to December 2018. Each approval was categorized by FDA therapeutic area review division based on indications and/or clinical trials within labeling. There were a few approvals that were excluded from the analysis due to the following reasons: same generic name, same NDA and/or BLA number, earlier initial approval date, and biosimilars. Efficacy supplements (ES) were also excluded, as the focus is only on NME labeling.

The COA Compendium was utilized to quantify the COA types (e.g. a patient-reported outcome, observer-reported outcome, clinician-reported outcome, or performance outcome assessment). Each COA tool type was tallied from each disease state, COA Context of Use and drug. Within each drug approval, multiple COAs of the same type were counted once for purposes of the analysis.

Results There were over 1500 NDA and BLA approvals during the analysis period, which included 494 NME approvals. Of the 494 NME approvals, 56 were from either neurological or psychiatric therapeutic areas. There were a few entries within the COA Compendium that had multiple indications approved for one drug, which is reflected in the data presented. Some of the entries within the COA Compendium were considered either as ES or did not fall under the search parameters and thus were excluded from analysis. The total number of NME approvals have been steadily increasing from 2015 to 2018. All four major COA types have been utilized within both neurological and psychiatric drug approvals largely without any noticeable trends. However, PRO

measure use has significantly increased in 2018 due to new approvals in both migraines and seizure medications.

Conclusion In neurology and psychiatry, NME approvals have been steadily rising, with PRO measure use increasing significantly in 2018. Ultimately, the COA Compendium is a useful tool for assessing how COAs have been used in clinical trials to support labeling claims and can enable evaluation of trends in COA use.

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