U.S. FOOD & DRUG ADMINISTRATION

Accelerated Approval Council Activities Report CY 2024

(Required by Section 3210 of FDORA)

Background

The Food and Drug Omnibus Reform Act (FDORA) (Sec. 3210) amended Section 506(c) of the FD&C Act (21 U.S.C. 356(c)). The revisions provided the Food and Drug Administration (FDA) with new authorities, such as the authority to require, as appropriate, that a study or studies to verify and describe the expected clinical benefit be underway prior to approval, or within a specified time period after the approval. The revisions also specified the creation of a coordinating council within the FDA to ensure the consistent and appropriate use of accelerated approval across the FDA. In addition, Section 3210 requires that FDA publish a report on FDA's website on the activities of the Council within a year of the passage of FDORA and annually thereafter. This report is intended to cover activities of the council during the 2024 calendar year.

Activities of the Accelerated Approval Council

As required by FDORA, in 2023 FDA established an accelerated approval council, the Accelerated Approval Coordinating Council (AACC or the Council). The membership of the AACC includes the Directors (or designees) of the Center for Biologics Evaluation and Research (CBER), the Center for Drug Evaluation and Research (CDER) and the Oncology Center of Excellence (OCE). The Chair of the AACC for calendar year 2024 was Peter Marks, M.D., Ph.D.

Other standing members of the AACC include Directors (or their designees) of the Office of New Drugs in CDER, the Office of Orphan Products Development, the Office of Therapeutic Products in CBER, the Office of Medical Policy in CDER, the Office of Neuroscience in CDER, the Office of Vaccines Research and Review in CBER, and the Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine in CDER. In addition to these standing members, ad hoc participants have been invited as needed as determined by the AACC Chair.

The AACC held three meetings during the 2024 calendar year. These meetings included discussion of the different Center priorities for the year related to accelerated approval, updates on recent trends in accelerated approvals, and updates on guidance related to accelerated approval that were in development or going through clearance. Members of the Council achieved alignment on the key policy issues related to implementation of the FDORA Accelerated Approval provisions.

The Council activities included a retrospective on the withdrawal of approval of Pepaxto (melphalan flufenamide), the first product withdrawn using the expedited withdrawal procedures contained in section 3210 of FDORA. This included an overview from the different FDA teams that participated in the withdrawal and their views as to what went well and what changes the Council could consider for future withdrawal proceedings. Following the presentations, the Council discussed potential process improvements. The Council members agreed that the expedited withdrawal procedure was an improvement over the prior withdrawal process, particularly in terms of conserving limited agency resources.

The Council also discussed whether any changes to existing processes should be made for

Council meetings and other actions the Council could take to increase transparency about its activities and considerations around accelerated approval decisions made across the agency. For example, the Council considered what issues should be discussed at the Council and what issues should be discussed in other venues, including the new Rare Disease Innovation Hub.