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2 potential biomarkers for ASD clear first step in FDA drug development tool qualification process

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Aripiprazole and risperidone have been approved by the Food and Drug Administration (FDA) to treat irritability associated with autism spectrum disorder (ASD) in children and adolescents. However, no drugs have been approved to treat the core symptoms of ASD (i.e., persistent deficits in social communication and social interaction, and restricted, repetitive patterns of behavior, interests or activities).

One challenge in developing drugs is that the heterogeneity of core symptoms among clinical trial participants meeting diagnostic criteria for ASD may obscure the benefits of novel treatments that are effective only in a subgroup of patients. Using biomarkers to define subgroups that may be more responsive to novel drugs in development may be one way to meet this challenge.

As part of the implementation of the 21st Century Cures Act, the FDA updated the process for qualifying biomarkers for use in drug development. A biomarker is a defined characteristic that is measured as an indicator of normal biological processes, pathogenic processes or responses to an exposure or intervention, including therapeutic interventions. Biomarkers can be molecular, histologic, radiographic or physiologic characteristics. FDA qualification of a biomarker means that within a specific context of use, the biomarker can be relied upon to have a specific interpretation and application in drug development and regulatory review. The qualification process includes three submission stages: a letter of intent, a qualification plan and a full qualification package.

In 2019 and 2020, the FDA accepted letters of intent for two proposed biomarkers for ASD: N170 to Upright Faces and Oculomotor Index of Gaze to Human Faces. The proposed N170 biomarker is described as the latency and peak amplitude of a brain waveform, measured by EEG, after viewing human faces. The Oculomotor Index biomarker is described as the proportion of time spent gazing at human faces, measured by an eye tracker, as compared with other locations on the screen. Both letters of intent were submitted by the Foundation for the National Institutes of Health Autism Biomarkers Consortium for Clinical Trials (FNIH ABC-CT). After developing acceptable biomarker qualification plans, the FNIH ABC-CT will conduct qualification studies and submit full qualification packages for the FDA to determine whether the data support qualification.

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