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Considering Functional Outcomes as Efficacy Endpoints in Pediatric Low-Grade Glioma Clinical Trials: An FDA Educational Symposium

Jason Fangusaro [1], Robert A Avery [2], Michael J Fisher [3], Roger J Packer [4], Karin S Walsh [5], Antoinette Schouten-van Meeteren [6], Dominik Karres [7], Diana Bradford [8], Vishal Bhatnagar [8], Harpreet Singh [9], Paul G Kluetz [10], Martha Donoghue [8], Elizabeth S Duke [8]

Affiliations

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Abstract

In October 2022, the Food and Drug Administration (FDA) Oncology Center of Excellence (OCE) hosted an educational symposium entitled, "Considering Functional Outcomes as Efficacy Endpoints in Pediatric Low-Grade Glioma (pLGG) Clinical Trials." The symposium brought together patient advocates, regulators from the FDA and the European Medicines Agency (EMA), and an international group of academic thought leaders in the field of pediatric neuro-oncology to discuss the potential role of functional outcomes, including visual acuity, motor function, and neurocognitive performance, as endpoints in clinical trials enrolling patients with pLGG. The panel discussed challenges and opportunities regarding the selection, implementation, and evaluation of clinical outcome assessments in these functional domains and outlined key considerations for their inclusion in future clinical trial design and role in new drug development.