



December 13, 2019

Process and Evidence Requirements for Developing a New Brain Tumor Endpoint

Roundtable participants include clinicians, researchers, representatives of biopharmaceutical companies, patients, patient advocates, and regulators.

PURPOSE: To evaluate concepts for the eventual development of a new imaging endpoint for brain tumors that builds on incremental levels of evidence from qualitative and visual assessments using various quantitative approaches.

Key topics included:

- A review of the issues related to the use of imaging to assess tumor response to therapy.
- An evaluation of regulatory considerations related to radiographic endpoints, outcomes measures, and clinical benefit assessments.
- Addressing imaging interpretation issues, including imaging acquisition, standardization of read paradigms, and accounting for variations across geographies and equipment.
- Accounting for disease distinctions in response assessment.
- Considering the potential of a novel framework for considering “rate of change” as a measurement of clinically meaningful therapeutic effect.

Major conclusions included:

- All patients should have pre-treatment, baseline scans.
- There is a need to develop quantifiable measures for evaluating response through imaging.
- Stable disease can be clinically meaningful and there is a need to clarify what disease stability means, including how best to assess “rate of change.”
- Imaging, data collection, and assessment approaches should be standardized, and the field can move toward use of volumetric segmentation.
- There is willingness among key stakeholders to incorporate some of these mechanisms into imaging charters of current trials.

- The meeting led to the establishment of a small working group charged with developing recommendations for a new clinical imaging endpoint for brain tumors, with the goal of better capturing and documenting clinical benefit of drugs seeking labeling approval in neuro-oncological indications. A multi-sector working group will develop recommendations for moving toward an improved, validated imaging endpoint.