December 17, 2018
Leveraging the Power of I-O to Treat Primary Brain Tumors

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

PURPOSE: To enable learning, analysis, discussion, and networking to advance the successful development of immuno-oncology (I-O) treatments for brain tumor patients.

Key topics included:

- A landscape analysis on the state of immunotherapy R&D to date for primary brain tumors.
- An evaluation of the opportunities to bring I-O therapies to brain tumor patients.
- The development of concrete scientific proposals to help answer the following questions that currently hinder I-O development in neuro-oncology:
  - How the blood-brain barrier (BBB) impacts the potential for I-O in brain tumors.
  - How to assess potential immunosuppressive effects of standard of care on I-O therapies for brain tumor patients.
  - How to assess if an I-O therapy is working in the brain.
  - How to differentiate pseudo-progression from actual progression.
  - Defining endpoints to evaluate these therapies.

- Evaluating industry priorities and understanding the regulatory environment.

Major conclusions included:

- The neuro-oncology field needs to address multiple challenges in turning immunologically "cold" brain tumors "hot."
- It is important to develop better models for preclinical research if I-O approaches, prioritize among the many multiple agents available, and make dose and timing determinations.
- The field needs to evaluate the effects of standard of care on I-O therapy response.
- The field needs a better understanding and method to analyze tumor microenvironmental changes during I-O treatment.
There are multiple promising areas for further research, including:
  o Neoadjuvant and combination approaches
  o Tumor-host and microenvironment studies
  o Single-cell sequencing
  o Personalized vaccines
  o Metabolic I-O
  o I-O delivery methods