July 13, 2018
Opportunities for Repurposing Drugs for Brain Tumors

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

PURPOSE: Diving into another key takeaway that was identified during the inaugural NBTS Research Roundtable: evaluating opportunities to repurpose existing drugs and compounds for the treatment of brain tumors.

Key topics included:

- A consensus definition of what the community means by “drug repurposing” and reviewing relevant examples.
- Identifying best approaches for developing the rationale to prioritize agents/pathways for repurposing efforts in neuro-oncology.
- Evaluating challenges within the current brain tumor therapy development ecosystem and identifying opportunities to address them in order to facilitate repurposing efforts.
- Identifying concrete action steps for stakeholders individually/collectively that can advance opportunities for drug repurposing to bring therapies to brain tumor patients.

Major conclusions included:

- A consensus framework defining the term “drug repurposing for brain tumors,” consisting of:
  - Off-label use of existing FDA-approved agents for non-cancer indications
  - New use of investigational or approved agents for other cancers
  - New testing of investigational agents that were stopped for further development because of lack of efficacy and/or brain penetration/toxicity or other reasons.
- There are multiple steps the community can take to identify and prioritize repurposing targets for brain tumors, including strengthening pre-clinical evidence for potential repurposed agents and leveraging biomarkers and genetic targets to re-analyze failed drugs based in specific patient subpopulations.
• There are opportunities to tap into existing legal and reimbursement frameworks to support repurposing activities.
• The neuro-oncology field should pursue specific target areas of interest and reach more patients.