



# RARE BRAIN TUMORS

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*XXV World Congress of Neurology (WCN 2021)  
Teaching Course Neuro-Oncology  
(WFN/Society of Neuro-Oncology – Joint Session: Standards of Care in Neuro-Oncology)  
October 3-7, 2021*

## **CONFLICT OF INTEREST**

I have received grants and honoraria for Lectures and Advisory Boards from MSD, Astra Zeneca, Merck Serono, Celldex Therapeutics, Novartis, Puma Technology, Abbvie and Mundipharma.

# LEARNING OBJECTIVES

- To update on the new WHO classification of Brain Tumors of 2021.
- To define the frequency of druggable molecular alterations within different tumor histologies.
- To clarify the rate and duration of response to the different targeted agents.
- To discuss the heterogeneity of response to targeted agents and the mechanisms of resistance.

## KEY MESSAGE : STATE OF ART

- Targeting BRAF mutation in pleomorphic xanthoastrocytomas and gangliogliomas is successful in up to 40% of patients.
- Targeting mTOR pathway in SEGAS of tuberous sclerosis yields a tumor growth and epilepsy control in up to 70% of patients.
- Targeting VEGF in bilateral schwannomas of neurofibromatosis type 2 allows a striking hearing improvement.
- Ongoing trials are exploring the efficacy of IDH 1/2 mutation and NTRK fusion inhibitors.

## **KEY MESSAGE: NEXT STEPS**

- To define the optimal timing for treatment (early versus late) and duration of treatment in the different tumor types
- To optimize treatment of secondary resistance with the combination of agents targeting different molecular pathways
- To explore new tools for monitoring response (advanced neuroimaging, radiomics, liquid biopsy) and new trial design.

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