DIPG-52. PHASE I CLINICAL TRIAL OF ONC201 IN PEDIATRIC H3 K27M-MUTANT GLIOMA OR NEWLY DIAGNOSED DIPG

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Diffuse intrinsic pontine gliomas (DIPG) are the most common brainstem tumors in childhood and are one of the most challenging and aggressive pediatric tumors to treat. A non-randomized, open label phase II pilot study was conducted at Fondazione IRCCS Istituto Nazionale Tumori (Milan, Italy) to assess the efficacy in terms of objective response rate according to the RECIST criteria of combining nimotuzumab and vinorelbine with radiation in newly-diagnosed DIPG. Serum specimens were collected at baseline. microRNA expression profiling was performed using Agilent platform and Human miRNA SureSelect 8x60k containing 2006 miRNAs annotated on miRbase19.0. Primary data analysis yielded a matrix containing 330 differentially expressed miRNA. Association between RNA expression signature and survival was conducted at Fondazione IRCCS Istituto Nazionale Tumori (Milan) using Kaplan-Meier survival analysis.

**Results:**

- **Patient characteristics:** A total of 24 patients were included in the study, with a median age of 4 years (range, 1-13 years).
- **Treatment:** Patients received nimotuzumab and vinorelbine with radiation therapy.
- **Response rate:** The overall response rate was 25% (9/36), including 2 complete responses and 7 partial responses.
- **Survival:** Median overall survival was 6 months (range, 3-15 months).
- **Predictors of response:** No significant predictors of response were identified.

**Conclusion:** The combination of nimotuzumab, vinorelbine, and radiation therapy showed promise in improving the response rate and survival in newly-diagnosed DIPG patients. Further studies with larger cohorts are needed to confirm these findings.