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Abstract title	**TEMOZOLOMIDE AS A SINGLE AGENT OR IN COMBINATION FOR PATIENTS WITH HIGH RISK REFRACTORY OR RELAPSED NEUROBLASTOMA: EXCELLENT TOLERANCE AND SUSTAINED DISEASE RESPONSES**

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Abstract text **Background/Objectives:**

Phase II trial data proves temozolomide (TMZ) is effective and tolerable for neuroblastoma treatment, but there are no large cohort published data, little 'real-world' data, and variation in modality of use.

Design/Methods:

Descriptive, retrospective study in 3 French centres for children with refractory/relapsed high-risk neuroblastoma (HR-NBL) diagnosed 1/1/04-31/12/17, follow-up to 31/10/18, receiving single-agent or combined TMZ, assessing efficacy and tolerability of TMZ.

Results:

Refractory/relapsed disease was diagnosed at a median age of 4y9m (range 8m-18y11m) for the 147 children included.

Sixty-one had induction-refractory disease; 30 responded sufficiently to TMZ-based chemotherapy to proceed to high dose chemotherapy (HDC). At last follow-up, 21/30 were alive (18 CR/PR, 3PD), compared with 8/31 (5 CR/PR, 3 PD) in non-HDC cohort. Overall best response rate (CR/PR) was 48%. Median PFS was 1y1m (95%CI 6m-1y7m), OS was 2y9m (95%CI 1y10m-5y10m). Eighteen patients never progressed/relapsed over a median follow-up of 2y4m (range 7m-11y2m).

Eighty-six patients with relapsed disease received a median number of 3 (1-33) cycles, 36 received TMZ alone. Overall best response rate was 44%, 31% had SD. Median PFS was 4m (95%CI 3m-5m), median OS 11m (95%CI 8m-1y1m).

Nineteen of the 147 patients received 12 or more consecutive cycles of TMZ-based chemotherapy, 13 are alive (9 CR/PR, 4 PD) with a median follow-up of 4y11m (range 1y7m-8y9m). Eight of these patients have stopped chemotherapy and are relapse-free with a median follow-up of 1y10m (range 4m-7y2m).

TMZ was well tolerated; toxicity delayed 8.8% of cycles, no-one required treatment withdrawal for toxicity. Performance scales were good (≥80%) after 6 months treatment. No patients developed secondary malignancies, over a median follow-up of 12m (range 0m-10y10m).

Conclusions:

TMZ-based regimens can gain, and maintain, disease control in HR-NBL, enabling consolidation for refractory disease. In relapse, prolonged use is well tolerated, with good performance score. Long-term survival was observed in some patients after treatment withdrawal.