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# Treatment response as surrogate to predict risk for disease progression in pediatric medulloblastoma with persistent MRI lesions after first-line treatment

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## Abstract

**Background:** This study aims at clarifying the impact of persistent residual lesions following first-line treatment for pediatric medulloblastoma.

**Methods:** Data on 84 pediatric patients with medulloblastoma and persistent residual lesions on centrally reviewed MRI at the end of first-line therapy were analyzed.

**Results:** Twenty patients (23.8%) had residual lesions in the tumor bed (R+/M0), 51 (60.7%) had distant lesions (R0/M+) and 13 (15.5%) had both (R+/M+). Overall response to first-line therapy was minor or partial ( $\geq 25\%$  reduction, MR/PR) for 64 (76.2%) and stable disease (SD) for 20 patients (23.8%). Five-year post-primary-treatment progression-free (pptPFS) and overall survival (pptOS) were superior after MR/PR (pptPFS:  $62.5 \pm 7.0\%$ [MR/PR] vs.  $35.9 \pm 12.8\%$ [SD],  $p=0.03$ ; pptOS:  $79.7 \pm 5.9\%$ [MR/PR] vs.  $55.5 \pm 13.9\%$ [SD],  $p=0.04$ ). Further, R+/M+ was associated with a higher risk for progression (5-year pptPFS:  $22.9 \pm 17.9\%$ [R+,M+] vs.  $72.4 \pm 12.0\%$ [R+,M0];  $p=0.03$ ). Watch-and-wait was pursued in 58 patients, while  $n=26$  received additional treatments (chemotherapy only,  $n=19$ ; surgery only,  $n=2$ ; combined,  $n=3$ ; valproic acid,  $n=2$ ), and their outcomes were not superior to watch-and-wait (5-year pptPFS:  $58.5 \pm 7.7\%$  vs.  $51.6 \pm 10.7\%$   $p=0.71$ ; 5-year pptOS:  $76.3 \pm 6.9\%$  vs.  $69.8 \pm 9.7\%$ ,  $p=0.74$ ). For the whole cohort, five-year pptPFS by molecular subgroup (58 cases) were WNT: 100%, SHH:  $50.0 \pm 35.4\%$ , Group-4,  $52.5 \pm 10.5$ , Group-3  $54.2 \pm 13.8\%$ ; ( $p=0.08$ ).

**Conclusion:** Overall response and extent of lesions can function as surrogate parameters to predict outcomes in pediatric MB patients with persistent lesions after first-line therapy. Especially in case of solitary persistent medulloblastoma MRI lesion, additional therapy was not beneficial. Therefore, treatment response, extent/kind of residual lesions and further diagnostic information needs consideration for indication of additional treatments for persisting lesions.

**Keywords:** MRI; children; medulloblastoma; persistent residual disease.

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