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# A Phase 2 PBTC Study of Selumetinib for Recurrent/Progressive Pediatric Low-Grade Glioma: Strata 2, 5, and 6 with Long-term Outcomes on Strata 1, 3, and 4

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## Abstract

**Background:** PBTC-029B was a phase 2 trial evaluating efficacy of selumetinib in children with recurrent/progressive low-grade glioma. We report results of strata 2, 5, and 6 with updated survivals for strata 1, 3, and 4.

**Methods:** Stratum 2 included recurrent/progressive pilocytic astrocytoma (PA) not associated with neurofibromatosis type-1 (NF1) that screened negative for the BRAF-KIAA1549 fusion and BRAFV600E mutation. Stratum 5 enrolled non-PA that screened positive for one of the BRAF aberrations. Stratum 6 enrolled children who consented to tissue screening, but there was an assay failure. For long-term survivals, stratum 1 included non-NF1 PA positive for one of the BRAF aberrations; stratum 3 included NF1-associated pLGG; and stratum 4 included non-NF1 optic pathway/hypothalamic tumors.

**Results:** Stratum 2: among 14 evaluable patients, there was 1 partial response (PR), 7 stable disease (SD) and 6 progressive disease (PD); overall response rate (ORR) was 7.1%. Two-year progression-free survival (PFS)/overall survival (OS) were 57.1%/100%, respectively. Stratum 5: among 23 evaluable patients, there was 1 complete response (CR), 4 PR, 12 SD, and 6 PD; ORR was 21.7%. Two-year PFS/OS were 74.8%/100%, respectively. Stratum 6: among 26 evaluable patients, there were 7 PR, 14 SD, and 5 PD; ORR was 26.9%. Two-year PFS/OS were 72.0%/100%, respectively. The median follow-up for patients on strata 1, 3, and 4 without events are 60.4, 60.4, and 58.1 months, and 5-year PFS/OS were 30.8%/88.9%, 54.2%/100%, and 51.0%/100%, respectively.

**Conclusions:** Selumetinib provided stability and responses across many pLGG subgroups, and some patients achieved prolonged disease control without additional therapy.

**Keywords:** Recurrent; and MEK inhibitor; pediatric low-grade glioma (pLGG); selumetinib.

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