

## Abstracts

### LTBK-01. UPDATES ON THE PHASE II AND RE-TREATMENT STUDY OF AZD6244 (SELUMETINIB) FOR CHILDREN WITH RECURRENT OR REFRACTORY PEDIATRIC LOW GRADE GLIOMA: A PEDIATRIC BRAIN TUMOR CONSORTIUM (PBTC) STUDY

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The PBTC is conducting a phase II study (NCT01089101) evaluating selumetinib (AZD6244, ARRY-142886), a MEK I/II inhibitor, in children with recurrent/refractory LGG assigned to 6 strata. We present the updated data on Stratum 2 and 5. Also, data on subsequent progression after treatment completion in patients enrolled on Stratum 1 and 3 will be discussed. Finally, we present details on the re-treatment study (PBTC-029C). Both stratum 2 (pilocytic astrocytoma [PA] without common BRAF aberrations) and Stratum 5 (non-pilocytic LGG with BRAF aberrations) met response criteria for expansion (> 2 objective responses in 16 patients), and accrual to a total of 25 patients on each stratum is ongoing. Among 50 patients treated on Stratum 1 (PA with BRAF aberrations) or Stratum 3 (NF-associated LGG), 21 have progressed. Thirteen of 21 have progressed after stopping therapy. The median time to progression for these 13 patients is 119 days (10–928). The re-treatment study has enrolled 25 patients who received a median of 12 re-treatment courses (2–36). The most common attributable toxicities after re-treatment were grade 1 CPK elevation (44%), diarrhea (44%), hypoalbuminemia (40%), elevated AST (36%), rash (36%) and fatigue (32%). The most common grade 3/4 attributable toxicities were grade 3 paronychia (8%), CPK elevation (4%), AST elevation (4%), decreased ejection fraction (4%), neutropenia (4%), elevated triglycerides (4%), peripheral neuropathy (4%) and grade 4 CPK elevation (4%). There is not a significant difference between the toxicities observed during original therapy versus re-treatment. The most current response and patient demographic data will be presented.