

Table 1 PLGG with KIAA1549-BRAF fusion: patient characteristics, response, and toxicity with trametinib

| Cases | Age at diagnosis (years); sex | Histologic diagnosis; tumor location | Prior surgery | Prior therapy | Age on treatment onset (years) | Time on treatment (months) | Best overall response | Functional response | Side effects |
|---------|-------------------------------|---|---------------|---|--------------------------------|----------------------------|-----------------------|---|---|
| Case 1 | 1.5; F | PA; medulla oblongata with leptomeningeal dissemination | Biopsy | CPT 11-CDDP; VBL | 5.8 | 20 | PR | Stable | Skin (grade II) Abdominal pain (grade II) Cheilitis (grade I) CPK and AP increased (grade I) |
| Case 2 | 0.5; M | PMA; hypothalamic-chiasmatic | Biopsy, PR | VCR-CBCDA; 5 DR-BEV | 1 | 18 | SD | Stable | Skin (grade I) Cheilitis (grade I) |
| Case 3 | 0.9; F | PA; intramedullary | Biopsy | VCR-CBCDA; CPT 11-CDDP | 12 | 15 | SD | Improvement of brachial hemiparesis, resolution of paresthesia in hands and torticollis | Skin (grade II) Alopecia (grade I) Vasculitis (grade III) CPK and ALT increased (grade I) |
| Case 4 | 1; M | PMA; hypothalamic-chiasmatic | Biopsy | CPT 11-CDDP; VBL; BEV-CDDP; BEV | 7.5 | 5 | SD | Stable | Skin (grade II) Cheilitis (grade I) Mucositis oral (grade I) Abdominal pain (grade I) Fatigue and anorexia (grade II) |
| Case 5 | 4.4; F | PA; hypothalamic-chiasmatic | Biopsy, PR | CPT 11-CDDP; VBL; BEV-CDDP | 12.5 | 8 | PD | Visual deterioration | CPK increased (grade I) Skin (grade II) Alopecia (grade II) Cheilitis (grade III) Mucositis oral (grade II) Fatigue and anorexia (grade II) |
| Case 6 | 5.8; F | PA; intramedullary | Biopsy, PR | CPT 11-CDDP; VBL; BEV-CDDP | 12.3 | 15 | SD | Resolution of paresthesia in hands | Skin (grade I) Alopecia (grade I) Mucositis oral (grade I) CPK and LDH increased (grade I) |
| Case 7 | 0.8; F | PMA; hypothalamic-chiasmatic | Biopsy | VCR-CBCDA; BEV | 2.8 | 6 | PD | Stable | Skin (grade II) Alopecia (grade I) Abdominal pain (grade I) Skin (grade I) |
| Case 8 | 1.8; M | PAs; hypothalamic-chiasmatic | Biopsy | VCR-CBCDA; VBL | 2.9 | 6 | PD | Increased hemiparesis and cranial nerve abnormalities | Skin (grade II) Abdominal pain (grade I) CPK increased (grade II) AST and LDH increased (grade I) |
| Case 9 | 3.9; M | PA; hypothalamic-chiasmatic with leptomeningeal dissemination | PR | CPT 11-CDDP | 9.6 | 14 (continue) | SD | Stable | Skin (grade II) Abdominal pain (grade I) CPK increased (grade II) AST and LDH increased (grade I) |
| Case 10 | 0.4; M | PMA; hypothalamic-chiasmatic | Biopsy, PR | VCR-CBCDA; VBL-BEV | 1.8 | 14 (continue) | SD | Diencephalic syndrome improvement | Skin (grade II) Abdominal pain (grade II) Vomiting (grade I) CPK increased (grade II) Skin (grade I) Cystitis (grade II) Lymph gland infection (grade III) LDH increased (grade I) |
| Case 11 | 1.1; F | GG; intramedullary | Biopsy, PR | CPT 11-CDDP; VBL; VCR-CBCDA; BEV-CPT 11 | 10.8 | 12 (continue) | SD | Stable | |