

**Supplementary table 2. Central nervous system findings before and after the end of the clinical trial\***

Patient	Age and gender	Gene <i>VHL</i> <sup>12</sup>		Pre-existent HB (MRI)		Changes at the end (MRI)	
		Locus	Mutation	Brain	Spinal Cord	Brain	Spinal Cord
1	36 M	Intron 2	Splice g.8665, A > G; c.DNA 463,+2A > C	2RCH-1LCH-1V	No	1LCH - growth in nodule, cyst and oedema	No
2	33 F	Intron 2	Splice g.8665, A > G; c.DNA 463,+2A > C	1MO	No	Growth in nodule	No
3	50 F	Exon 3	c.501 A > T	2LCH	No	No	No
4	20 M	Exon 3	c.501 A > T	1LCH- 1MO	T7-T10-T12-L1	MO- growth in nodule and cyst	No
5	15 M	Ex1-2-3	del complete <i>VHL</i>	1MO	1C4, 2C5, 2C6, 1D5, 1L1	No	Growth in nodule and oedema of 2 cervical HB
6	38 F	Exon 1	del exon 1	No	No	<i>Withdrawn</i>	<i>Withdrawn</i>
7	22 M	Exon 3	c.486, C > G	No	1T2-1T3	No	No

The number before the acronym represents the number of tumours. RCH - Right cerebellar hemisphere; LCH - Left cerebellar hemisphere; MO - medulla oblongata; V - Vermis; HB - Hemangioblastoma; CT - Clinical trial.

*\*All patients were scheduled for an MRI study before the start of the clinical trial (performed between 1-3 months before) and a new one in the last month of the CT.*