High-dose chemotherapy and craniospinal irradiation-sparing approach for WNT medulloblastoma of early childhood

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Abstract

Background

Adjuvant chemotherapy and craniospinal irradiation led to excellent survival in older children (≥3 years) with WNT medulloblastoma, allowing for ongoing careful treatment de-escalation. However, treatment with standard dose chemotherapy alone or in combination with involved field radiotherapy to the tumor bed only had high rates of distant treatment failure. In younger children, WNT medulloblastomas are extremely rare and the survival following high-dose chemotherapy (HDC) and radiation-sparing strategies has not been reported.

Patients and methods

Through international collaboration, we assembled a cohort of young children with WNT medulloblastoma treated with HDC and craniospinal irradiation avoidance to describe their survival and neurocognitive and ototoxicity profile.

Results

Five patients, diagnosed at median age of 7.0 years (range 2.7–7.5) underwent HDC and autologous stem cell transplantation. None of them received adjuvant radiotherapy. All patients were alive beyond four years from diagnosis. Evaluated patients had neurocognitive abilities reported within

low average to average range.

Conclusion

In this cohort, the survival of young children with WNT MB treated with HDC alone was excellent. The rare possibility of a molecular diagnosis of WNT MB in early childhood should not be viewed as an exclusion criterion to enroll on infant brain tumor strategies relying on HDC consolidation.

Clinical trial number

Not applicable.