Treatment of Retinoblastoma Patients with 13q Deletion Syndrome in the Intrarterial and Intravitreal Era

David Abramson, M.D.
New York, NY

Lucy Cobbs, MD, Y. Pierre Gobin, MD, Ira Dunkel, MD, Jasmine Francis, MD, FACS

Purpose:
Retinoblastoma patients with 13q deletion syndrome traditionally do poorly with treatment because they have multiple comorbidities which compromise effective treatment. In the past, patient and ocular survival have been poor and complications from systemic chemotherapy common. The purpose of this study is to report on ocular survival, patient survival, second cancers, ocular toxicity (ERG) and hematologic toxicity in deletion patients managed in the intrarterial and intravitreal chemotherapy era at Memorial Sloan Kettering Cancer Center (N.Y.).

Methods:
Retrospective review of 24 eyes in 16 patients with molecularly identified deletion treated at a single tertiary retinoblastoma referral center (Memorial Sloan-Kettering Cancer (N.Y.).

Results:
Twenty-four eyes of 16 patients (8 unilateral and 8 bilateral) were treated at MSK in the intrarterial/intravitreal era with a mean follow-up of 3.5 years. Twenty-five percent of these patients had birth weights below 2500 gm. and all had a myriad of additional systemic findings including growth retardation and intellectual delay. Fifteen of the 16 patients survived (1 patient died of sepsis with metastatic disease at the age 5.2 years when he was 8.5 kg). Five of the 24 eyes were enucleated: 1 prior to being treated at MSK and 4 after treatment. Kaplan Meier ocular survival at 2 years was 76%. No child developed a second malignancy. Seventeen of 28 intrarterial sessions had hematologic Grade 3-4 CTACA toxicity. Of the 5 cycles of systemic chemotherapy given 4 had Grade 3-4 hematologic toxicity. Twenty-nine percent of eyes receiving intravitreal chemotherapy lost more than 25uV of ERG function; no eye receiving intravitreal Topotecan developed reduced ERG function.

Conclusions:
The majority of patients with retinoblastoma and 13q deletion syndrome can now be cured with combined intrarterial and intravitreal chemotherapy based regimes. These patients have more toxicity from systemic chemotherapy than retinoblastoma patients without deletion syndrome so combined intrarterial and intravitreal offers a less toxic regime for these multichallenged children. Despite the lower doses of chemotherapy for the intrarterial/intravitreal group these patients have more hematologic toxicity than non deletion retinoblastoma patients so treatment remains a challenge.