

SJRET6: Protocol for the Study and Treatment of Participants with Intraocular Retinoblastoma

Retinoblastoma treatment at St. Jude Children's Research Hospital focuses on: SURVIVAL • OCULAR SALVAGE • VISION PRESERVATION

250 to 300 children in the U.S. annually are found to have retinoblastoma
40% bilateral retinoblastoma

60% unilateral retinoblastoma



Clinical Presentation

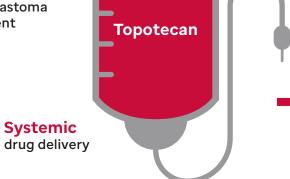
- · Leukocoria · Red or painful eye with glaucoma
- Strabismus
 Orbital cellulitis

- Unilateral mydriasis
- · Heterochromia iridis

A primary hurdle for chemotherapeutic treatment of retinoblastoma is drug penetration through the blood-ocular barrier. SJRET6 addresses the challenge by using a more effective agent and a novel drug-delivery approach to evaluate whether topotecan and carboplatin in upfront therapy improves ocular survival in participants with advanced bilateral retinoblastoma.

Novel

chemotherapeutic agent for retinoblastoma treatment



Carboplatin Standard chemotherapeutic agent for retinoblastoma treatment Subconjunctival drug delivery

St. Jude preclinical discoveries

- Substantial penetration of blood-ocular barrier
- Optimal activity against retinoblastoma when combined with carboplatin

Clinical significance

- Effective and feasible as upfront therapy for advanced bilateral disease in previous St. Jude clinical trial
- Minimizes long-term risk of secondary malignancies

St. Jude preclinical discovery

• Effective penetration of the vitreous with local delivery

Clinical significance

- Improves drug concentration to the tumor
- Increases retinal and vitreous penetration
- Limits systemic toxicity

