



A Phase 2 Open Label Trial of Atorvastatin to Prevent Platinum-induced Hearing Loss in Newly Diagnosed Children with High-Risk Neuroblastoma

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Platinum-based chemotherapies (cisplatin and carboplatin) are core components in the treatment of many pediatric cancers, including high-risk neuroblastoma (HRNBL). One of the most significant side effects of these chemotherapies is irreversible hearing loss. Unfortunately, rates of platinum-induced hearing loss are very high in the high-risk neuroblastoma population, with over half of patients experiencing significant hearing loss. This communication-impacting hearing loss is especially detrimental during critical developmental periods such as early childhood, when many patients with high-risk neuroblastoma are diagnosed.

Statins are a class of medications approved for the treatment of high cholesterol, but these drugs can also protect the fragile hair cells in the ears from platinum-induced ototoxicity. Statins are generally quite safe and cost-effective, making them an appealing class of medications to use. ACCL2521 is a phase 2 study through the Children's Oncology Group that will test the efficacy of atorvastatin in preventing platinum-induced hearing loss and examine biomarkers that might influence or predict patient response.

Impact: Hearing loss has profound impacts on both short- and long-term quality of life, negatively affecting neurocognitive function, academic and job achievement, social well-being, and mental health. Parents of survivors also experience significant distress associated with their children's hearing loss. Thus, hearing loss from platinum therapy creates a significant and lifelong burden for survivors of childhood cancer and their families. This clinical trial will test a promising otoprotection intervention, aiming to improve the lives of young children with cancer.