



Hyundai Hope on Wheels Hyundai Scholar Research

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Rhabdomyosarcoma (RMS) is the most common soft tissue sarcoma in children and adolescents, accounting for 5-8% of childhood cancers. The two major subtypes of rhabdomyosarcoma (RMS) are the alveolar (ARMS) and embryonal (ERMS) forms. Alveolar rhabdomyosarcoma accounts for approximately 20% of all cases. The alveolar subtype has been associated with more aggressive disease, increased rates of metastasis and an unfavorable outcome. Two specific gene abnormalities have been identified in alveolar rhabdomyosarcoma. These abnormalities involve genetic rearrangements of PAX3 or PAX7 and Forkhead (FKHR) genes which are normally involved in muscle development and cell division, respectively. These rearrangements produce abnormal, cancer causing fusion proteins that seem to be important for tumor formation, although the mechanism is not fully understood. Tumors involving the PAX3-FKHR genetic defect result in higher risk disease.

RNA interference, RNAi, has emerged as a leading candidate strategy to suppress disease genes. Small inhibitory molecules (microRNAs) can be designed to specifically target any gene based on its DNA sequence. My research will focus on the development of an RNAi viral vector to suppress PAX3-FKHR in cells and in mouse models of ARMS. The primary goal of this research is to reduce PAX3-FKHR protein and decrease or eliminate ARMS tumor growth through PAX3-FKHR targeted inhibition. RNAi therapy will provide further insight in the development of ARMS and provide a potential therapeutic tool in adjunct to current therapies for this more aggressive form of rhabdomyosarcoma.