Researchers Receive Grant to Study Retinoblastoma in Children

J. William Harbour, M.D., and Daniel Pelaez, M.D., researchers at Sylvester Comprehensive Cancer Center and Bascom Palmer Eye Institute at the University of Miami Miller School of Medicine, have been awarded a $250,000 grant by Alex’s Lemonade Stand Foundation for their 2018 Innovation Grant application titled “Molecular Landscape for Targeted Therapy in Retinoblastoma.”

A photographic image showing a retinoblastoma.

Retinoblastoma is the most common type of eye cancer in infants and children under the age of six. While in this country the vast majority of these young patients survive, retinoblastoma is an important causes of pediatric cancer death in less developed countries across the world.
The results of this two-year project could break new ground in the development of novel treatments for retinoblastoma, with the goal of providing better outcomes and fewer side effects.

“We anticipate that this project could lead the way to the first targeted therapeutic strategy in retinoblastoma that will allow us to treat the disease while preserving vision and saving the eye in these children,” said Dr. Harbour, who is associate director for basic research at Sylvester and vice chairman for translational research at Bascom Palmer.

Retinoblastoma has been linked to a mutation in RB1, a tumor suppressor gene. One in four affected children inherit this mutation from a parent, and most often in these cases, both eyes are affected. Even though treatment for the disease has evolved over the years, most retinoblastoma survivors will suffer from blindness or require surgical removal of the affected eye.

“Our recent discoveries, and completion of this project, could change that,” said Dr. Harbour. “By linking the most common mutations found in retinoblastoma tumors to a previously undiscovered functional pathway, we open the possibility of a rational therapeutic approach that capitalizes on a vulnerability that many or all retinoblastomas may share.”