



An Update from Children's National Hospital to The Catherine Elizabeth Blair Memorial Foundation

August 2023

Children's National Hospital is at the forefront of research for children with neuroblastoma. Through the leadership of Anthony Sandler, M.D. and steadfast philanthropic support from partners including The Catherine Elizabeth Blair Memorial Foundation, our team is on the cusp of immunotherapy clinical trials for the treatment of neuroblastoma.

Pre-clinical studies on neuroblastoma immunotherapies have advanced our understanding of the tumor micro-environment and mechanism of immunogenicity. His team has further evaluated patient immunity induced by training patient T-cells to eradicate their tumors, which has laid the groundwork for translational research. In Spring and Summer 2023, the laboratory team dedicated themselves to validating these techniques through repeated experiments with patient tumors and blood to fully understand the potency of these trained T-cells. Additionally, they recapitulated these studies back in the murine models and have successfully proven that the personalized T-cells eliminate the neuroblastoma tumor.

The team is currently focused on evaluating autoimmunity in the murine model as a way to protect children's vital organs from adverse immune reactions to the immunotherapy. This approach is essential to not only improve the cure rates for neuroblastoma, but also to foster better life-long outcomes for survivors. Dr. Sandler aims to publish their findings in early 2024, a key step to secure FDA approval to progress with a clinical trial.

Over the coming year, Dr. Sandler and the team will aim to catalyze the final stage of discovery and achieve the following objectives: illustrate that T-cell therapy is safe and effective in animal models of neuroblastoma, build capacity for production of T-cells for individual therapy within our Good Manufacturing Practices Facility at Children's National and advance to in-human clinical trials. We are committed to pushing the boundaries of medicine because we envision a future where survival rates and outcomes for neuroblastoma improve drastically.