



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

August 2022

Over the course of 2022, Children's National Hospital has continued to forge ahead on pediatric cancer research. Dr. Anthony Sandler and the neuroblastoma research team have remained focused on developing personalized therapeutic tumor vaccines and treatments, which aims to induce immunogenicity, and render neuroblastoma tumors visible to the immune system. Having published this initial work in the *Journal for Immunotherapy of Cancer* in Spring 2021, the team worked over the last year to advance the research. These new findings continued to show that by blocking the ApoE checkpoint, we can increase the vaccine's efficacy in our murine tumor models. Additionally, the team is preparing a manuscript of their work exploring the CD24Fc fusion protein as a pathway to suppress immune-related adverse effects in patients for publication. Validating and disseminating our findings amongst our peers is an essential component of the research process and eventual clinical trial design.

The team has now further validated and translated these findings from the laboratory to the bedside. Notably this has include gathering samples from patients and testing the patient's own immune cells to be activated against the tumor cells using the techniques discovered in the laboratory. So far, they are seeing very promising results with inhibiting the MYC oncogene with small molecule inhibitors and activating the patient's t-cells against their own tumor cells in the laboratory. As a means to early translation, the team is considering activated t-cell infusions as an intermediary treatment option while continuing to follow the whole cell tumor vaccine strategy.

Should their translational investigations continue to show promise, the team will pursue a clinical trial protocol that would incorporate all three aforementioned techniques. The aim would be to secure IRB and FDA approval and begin trial recruitment in the coming years.

The support of the Catherine Elizabeth Blair Memorial Foundation has allowed us to generate new knowledge and potential neuroblastoma treatment options to save children's lives. Children's National is deeply grateful to partner together to advance our shared mission to find a cure and bring hope the families.