Progress in 2025: Discovery to Delivery

When Children's National Chief of Surgery, Anthony Sandler, MD, and his research team set out to train a child's own immune cells to fight neuroblastoma, they weren't just refining a lab technique. They were daring to reimagine the future of cancer therapy.

That vision is now closer to becoming a reality.

The team made strong progress in 2025. A landmark report published in Frontiers in Immunology in June 2025 unveiled the team's groundbreaking method for training a patient's own blood cells to attack tumors. The preclinical study showed that in both lab and animal models, these trained immune cells, known as TAT-T cells, eradicated or suppressed tumors without harmful side effects. It also revealed why this approach works so well. By blocking a key cancer-promoting gene called MTC and adjusting a pathway known as STING, the team made tumor cells more visible to the immune system. This insight could transform not only treatment for neuroblastoma, but also potentially many other solid tumors.

The publication of Dr. Sandler's study has sparked excitement at Children's National, and the momentum continues to build. Now solidly in Phase 2 of this journey, the lab spent the past year deepening the science behind this breakthrough and preparing for the clinical trial.

"This publication is an important milestone. It confirms that what we saw in early experiments holds in rigorous testing, and it gives us the foundation to move this work from the bedside to the lab bench and back again to the bedside. No one else is exploring this specific approach to cell therapy." – Dr. Anthony Sandler, Senior Vice President and Surgeon-in-Chief | Joseph E. Robert Jr., Center for Surgical Care

What's Next: Continued Progress Towards a Clinical Trial

Over the last year, the team began to zero in on the exact immune cells that recognize and attack cancer. They isolated a key group of cells, called CD8 T cells, to see how well they destroy tumors compared with the body's broader immune

response. The team also tested them in a melanoma and astrocytoma mouse models to pinpoint which T-cell receptors, or "sensors," are best at finding and killing tumor cells. This helps confirm that our approach works against a wider range of solid tumor types.

Each experiment brings us closer to what Dr. Sandler calls the secret recipe: the precise conditions that make the trained T cells so effective. *"Every step gives us more confidence that this can work in patients,"* he says. Two more papers detailing findings are in progress.

The Good Manufacturing Practice (GMP) facility team, led by Patrick Hanley, PhD, completed three more technology transfer runs to replicate lab methods at clinical scale. They also did six pilot runs to confirm they can manufacture the cells safely and consistently. *"We continue to see the same strong results in our manufacturing runs that we see in the lab,"* Dr. Hanley says. *"That consistency tells us we're ready to move toward the clinic."*

Next, the team will fine-tune every detail to get this therapy ready for the next big milestone: clinical testing.

They aim to pinpoint the T-cell receptors and immune cells that are most effective at killing cancer, while also running the final tests in the GMP facility to make sure those cells stay strong and effective when produced for patients. At the same time, Drs. Sandler and Hanley are finalizing the Investigational New Drug (IND) application and drafting the Phase I clinical trial protocol for FDA submission. Most research takes three to four years to reach this stage, but Children's National is doing it in just two, thanks to strong preclinical results.

Once approved, the team hopes to begin enrolling patients soon after. The clinical trial will include up to 10 children with high-risk solid tumors, including neuroblastoma, and feature both cell manufacturing and immune monitoring. It will span two years, followed by three years of patient monitoring. Two highly regarded pediatric oncologists, Holly Meany, MD and Amy Hont, MD, will lead the trial, bringing deep expertise in pediatric immunotherapy. The team also aims to raise awareness of the therapy's promise and secure an additional $1 Million in funding needed to launch and complete the trial without delay.

"It's heartbreaking to see children who have run out of options," Dr. Sandler says. *"But we're so close to a new treatment showing enormous promise. With continued support, we can take the next step and finally bring this approach to the bedside."*