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Cancer Center

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FOR THE MEDIA



Peter Steinherz, Director of the Leukemia and Lymphoma Team, collaborates with other experts to deliver the most-effective available treatments to young patients.

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VIDEO | 02:42

## Rihanna's Story of Treatment on a Clinical Trial with Larotrectinib

Meet Rihanna, who was on a clinical trial with larotrectinib.

[Video Details \(https://www.mskcc.org/videos/rihanna-story-treatment-clinical-trial-larotrectinib\)](https://www.mskcc.org/videos/rihanna-story-treatment-clinical-trial-larotrectinib)

In addition to delivering world-class care, pediatric cancer experts at Memorial Sloan Kettering participate in laboratory and clinical research to develop safer and more-effective therapies, as well as those that will improve the quality of life of children and young adults with cancer.

Our clinical specialists collaborate with laboratory-based scientists to learn how pediatric cancers arise and spread. Using this knowledge, we identify new targets for therapy. Our experts translate findings from the lab to the clinic – and back – to further the development of new pediatric cancer treatments.

We have learned that children and young adults with many types of cancer – particularly [neuroblastoma](#) , some types of [leukemia](#) and [lymphoma](#) , and [sarcomas](#) – benefit from risk-adapted therapy, in which treatment is tailored to the risk of disease progression or recurrence. Risk is often characterized by newly developed tests in the laboratory or by advanced imaging techniques.

Our experience and expertise allow us to provide less-intensive but fully effective treatments resulting in fewer side effects for children whose cancers have a low risk of growing quickly, spreading quickly, or returning after therapy than for children who have high-risk disease.

Our goal at Memorial Sloan Kettering is to cure your child while minimizing the side effects of treatment, so that he or she can resume a life filled with normal childhood joys. To improve our chances of accomplishing this goal, we treat children using some of the following innovative approaches.

## Clinical Trials

Your child may be able to participate in a clinical research study, in which your treatment team evaluates a new, innovative therapy regimen for a particular type of cancer. Our clinical teams have particular expertise conducting phase I and II clinical trials that study treatments in the earliest stages of development.

Due to the dedication of Memorial Sloan Kettering's world-class researchers for more than half a century, information learned through [clinical trials](#) has raised the overall cure rate for pediatric cancers to more than 70 percent.

We are able to offer participation in nationwide clinical trials as a member of the national Children's Oncology Group (COG), the Bone Marrow Transplant-Clinical Trials Network (BMT-CTN), and other multicenter consortia. In addition, Memorial Sloan Kettering serves as the coordinating center for the [Pediatric Oncology Experimental Therapeutics Investigators' Consortium \(POETIC\)](#), which our experts co-founded in 2003. This group works to develop and assess new drug therapies for children, young adults, and adolescents with cancer, especially those who have recurrent disease.

Examples of some of the new and unique treatments being evaluated at Memorial Sloan Kettering include:

## **Antibody-Based Therapies**

Standard treatment for [neuroblastoma](#) has typically involved intensive chemotherapy, often combined with [stem cell transplantation](#), which can lead to severe side effects and a limited chance of a cure. For more than 20 years, Memorial Sloan Kettering investigators have used an antibody – a type of immune system protein – called 3F8 to treat neuroblastoma. 3F8 in combination with surgery and chemotherapy has significantly improved the cure rates for children with high-risk neuroblastoma, while reducing the intensity of chemotherapy required.

Every day, our specialized team of physicians, nurse practitioners, nurses, social workers, and child life specialists routinely treats children with 3F8 in an outpatient setting. We have recently developed an enhanced version of 3F8 that is a near likeness of a human antibody. It promises to provide more specific, powerful treatment for neuroblastoma. Also, because of its makeup, it is a treatment that can be given for longer periods of time than other antibodies.

In addition, our investigators are studying how to use other antibody-based therapies to harness the power of a patient's own immune system to fight neuroblastoma. And in children and young adults with bone [sarcomas](#), we are conducting trials of newly developed antibodies that will direct immune system cells to attack these tumors.

## **Radiolabeled Antibody Therapies**

Brain tumors and other cancers that travel to the brain from the rest of the body are very difficult to cure. We have found that antibodies can kill some kinds of cancer cells in the brain and spinal cord, also known as the central nervous system (CNS).

Memorial Sloan Kettering is the only Center in the United States that is studying treatment with a

radiolabeled antibody that delivers radiation specifically to [CNS tumors](#) . This antibody, called 8H9, can be tagged with high doses of radiation and delivered to the spinal fluid to help kill cancer cells.

We have seen the most-promising results of this therapy in children with neuroblastoma that has relapsed in the CNS. Many of these children have experienced long-term remissions. Together with our colleagues in nuclear medicine, radiation safety, medical physics, and neurosurgery, we are working to further improve the way this radiation-antibody therapy can help achieve a lasting cure.

We are also using antibodies developed at Memorial Sloan Kettering for the treatment of another aggressive cancer that can affect children and young adults: [desmoplastic small round cell tumor \(DSRCT\)](#) . Our patients [diagnosed with DSRCT](#) begin [treatment](#) with an intensive multi-agent chemotherapy protocol combined with the drug bevacizumab. If possible, they then have surgery to remove the tumor, followed by additional chemotherapy. One of our current clinical trials is testing a radiolabeled antibody that is directly injected into abdomen where these tumors are hiding.

## Cell-Based Treatments

Our bodies require a healthy immune system to respond to and control infections. A specialized type of white blood cell, called a T lymphocyte, helps fight infections. When patients receive a [blood stem cell or bone marrow transplant](#) , they initially have a weak immune system with few T lymphocytes, and may have trouble controlling the rise of certain viruses such as cytomegalovirus (CMV) and Epstein Barr virus (EBV). This can lead to serious complications, including the development of another type of cancer.

Memorial Sloan Kettering investigators were the first to show that EBV could be better controlled in children who received small numbers of T cells from a bone marrow donor. We also demonstrated that we could give children these donor T cells more safely by first expanding them in the laboratory and “teaching” T cells to recognize and destroy only virus-infected abnormal cells. This strategy is called adoptive immunotherapy. Our physicians are conducting clinical trials to determine how to best ensure a successful transplant while limiting side effects related to T cells.

For many people with EBV- or CMV-related complications after an organ or bone marrow transplant, T cells from the donor are not available. In these cases, we are studying the use of virus-fighting T cells from someone other than the donor, which could be a safe and effective way to treat infections in a wide range of patients.

We are also using adoptive immunotherapy as a strategy for targeting cancer cells that produce certain proteins abnormally. Two of the proteins are abnormally produced by many types of cancer cells, but not in most healthy cells: WT1, a protein named for [Wilms' tumor](#) , and NY-ESO-1, a protein discovered at Memorial Sloan Kettering. In clinical trials, we are growing T lymphocytes directed against WT1 in the

laboratory to treat patients in whom standard therapy failed to control their disease.

## Vaccines and Immunity-Modulating Therapies

Memorial Sloan Kettering researchers are also investigating other therapies that target the immune system. We are conducting clinical trials of ipilimumab, an antibody against a molecule found on the surface of some lymphocytes. Masking this molecule with the antibody can free the immune system to attack tumor cells more intensely.

For patients with [osteosarcoma](#) , we are conducting trials of muramyl tripeptide. This compound stimulates white blood cells known as macrophages to attack tumor cells. In a randomized clinical trial, our researchers found that muramyl tripeptide increased the survival of patients with osteosarcoma when added to a chemotherapy regimen.

A powerful vaccine directed at tumor cells has shown encouraging results in patients with high-risk metastatic [neuroblastoma](#) . In the past, it was nearly impossible to keep patients with relapsed neuroblastoma in a second remission. With the introduction of these vaccines, most patients are alive and well.

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