

# Transplant & Cellular Therapy Center

The region's elite resource for the latest approaches in transplant, CAR T and other cell therapies



Roswell Park is home to **New York State's first cell and gene therapy hub** focused on discovery and production of life-saving cell therapies.

We are a **high-volume center**, providing allogeneic and autologous stem cell transplant to patients of any age.

## What Sets Us Apart

- **CAR T-cell therapies.** We offer Kymriah, Yescarta, Tecartus, Breynzi, Abecma, and Carvykti.
- **Tumor infiltrating lymphocyte (TIL) therapy.** We have ongoing trials of TIL therapy for cervical and non-small cell lung cancer and will offer it for other indications once approved.
- **Gene therapy.** We will soon offer Casgevy™ (exagamglogene autotemcel, exa-cel) for sickle cell disease.
- **Strategies for better transplant outcomes.** We have specific expertise in technologies to reduce complications, including graft-versus-host disease (GVHD), and enhance quality of life.
- **Outpatient therapy.** We offer safe, effective, and convenient outpatient CAR T and autologous stem cell transplantation for eligible patients.
- **Clinical trials of next-generation cell therapies,** including options for solid tumor cancers, and with transplant to prevent disease relapse and GVHD.

# Transplant & Cellular Therapy Center

Roswell Park was one of the first centers in the world to routinely offer blood and bone marrow transplantation to cancer patients. Since 1977, we have performed more than 3,450 transplants and have continually strived to refine the treatment, improve outcomes, reduce complications, and make transplant available to more patients.

We offer the full range of FDA-approved cell, gene, and transplant therapies. Highlights of our program include:

## **EXPERTISE** to improve outcomes and quality of life

We use technologies and practices to substantially reduce risks of transplant-related complications, like clinically relevant or life-threatening acute and chronic GVHD. This allows us to efficiently find young, unrelated stem cell donors to improve outcomes and reduce risks after transplantation.

## A **ONE-STOP** Shop

Our team includes specialists in transplant, hematology, pediatrics, radiation medicine, infectious disease, cardiology and gastroenterology, plus oncology nurses trained in care of immunocompromised patients. We integrate other disciplines and services — dental care, physical and pulmonary therapy, nutrition, psychologists, social workers, child life specialists and more — into the care pathway for all.

## Conditions We Treat

Roswell Park offers transplant and cellular therapies to eligible patients with the following conditions:

- **Leukemia**
- **Lymphoma**
- **Multiple myeloma**
- **Aplastic anemia and other marrow failure syndromes**
- **Amyloidosis**
- **Hemoglobinopathies, including sickle cell disease and thalassemias**
- **Myelodysplastic syndrome**
- **Myeloproliferative neoplasms**
- **Selected solid tumors such as testicular cancer**
- **Graft-versus-Host Disease**

# OUTPATIENT Transplant or Cell Therapy

We understand the emotional and health benefits of outpatient treatment and the value of patients returning home at the end of the day. Eligible patients can receive outpatient CAR T-cell therapy or autologous transplantation, where most aspects of therapy are provided in our ambulatory clinic. We strive to minimize the need for hospital stays to get patients back home and with their loved ones.

## Our beautiful, **MODERN** facility

The state-of-the-art Bill and Jane Greene Transplant & Cellular Therapy Center opened in 2021 on the 6th floor of the hospital. Our outpatient clinic features 12 large rooms to support outpatient services and procedures, increased infusion space, and enhanced privacy in shared and common areas.

## Long-term **SURVIVORSHIP** Clinic

We provide specialized survivorship care that focuses on patients' well-being and includes expert cancer monitoring and screening, managing any new or continuing symptoms or side effects, mental and social support, nutrition and lifestyle guidance, and other assistance — for life.

### This care includes:

- ✓ **Clinical recommendations** and referrals for unique symptoms that can occur after treatment, such as:
  - Skin changes, alopecia
  - Side effects of corticosteroid use
  - Cardiac or pulmonary problems
  - Thyroid changes
  - Cataracts
- ✓ **Bone density surveillance**
- ✓ **Re-immunization tracking**
- ✓ **Routine cancer screening**



# HEMATOPOIETIC Cell Transplantation (HCT)

Our transplant team will partner with you to find the very best option for your patient. We will provide consultation to determine whether transplant is appropriate, and which technique would benefit your patient most.

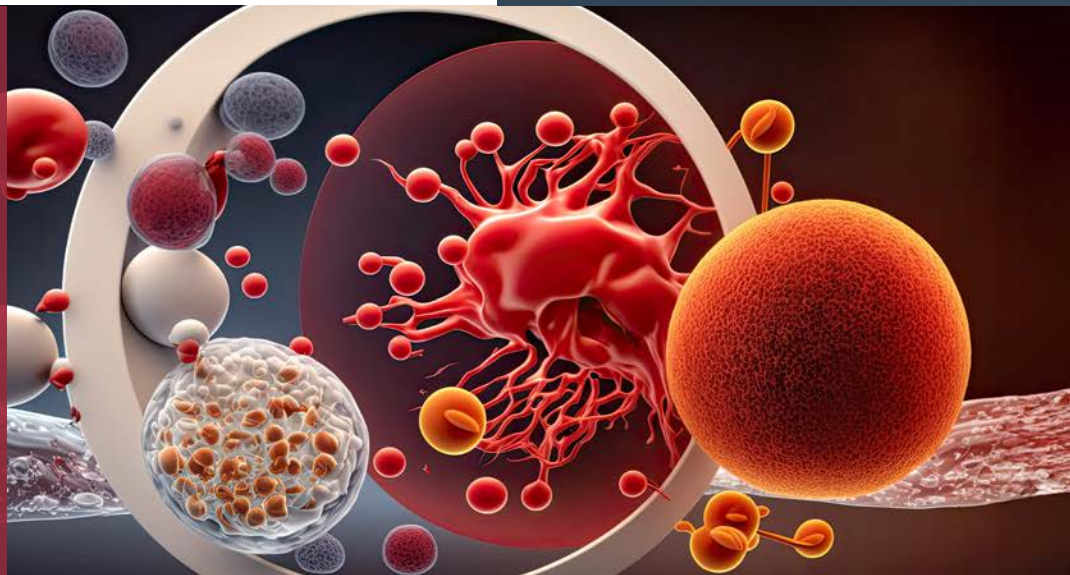
We offer customized HCT for patients of any age, including:

- **Autologous**
- **Allogeneic, including expertise in HLA-mismatched transplant**
- **Haplo-identical**
- **Gene therapy**
- **Reduced intensity**
- **Myeloablative**

## Improved outcomes for stem cell transplant

We use the latest strategies and technologies for

- **Optimizing survival and cure rates**
- **Decreasing complications and relapse**
- **Monitoring immune response**
- **Reducing duration of immune suppression**
- **Shortening inpatient stays**
- **Improving quality of life and patient experience**



## These strategies include:

- **Prioritizing age <40 for donor selection**, even if it means going to an unrelated matched or mismatched donor.
- **GVHD prophylaxis** with post-transplant cyclophosphamide-based regimens. Our goal is to initiate taper of systemic immune suppression by day +60.
- **Early discharge** post-transplant



“These new methods make grade III-IV acute GVHD and severe chronic GVHD rare, allow long term immune tolerance off systemic prophylaxis, reduce toxicity and improve outcomes post-transplant.”

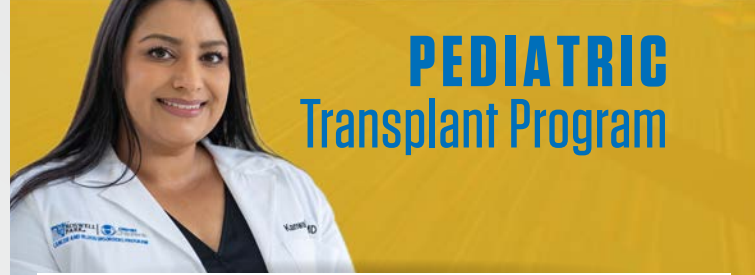
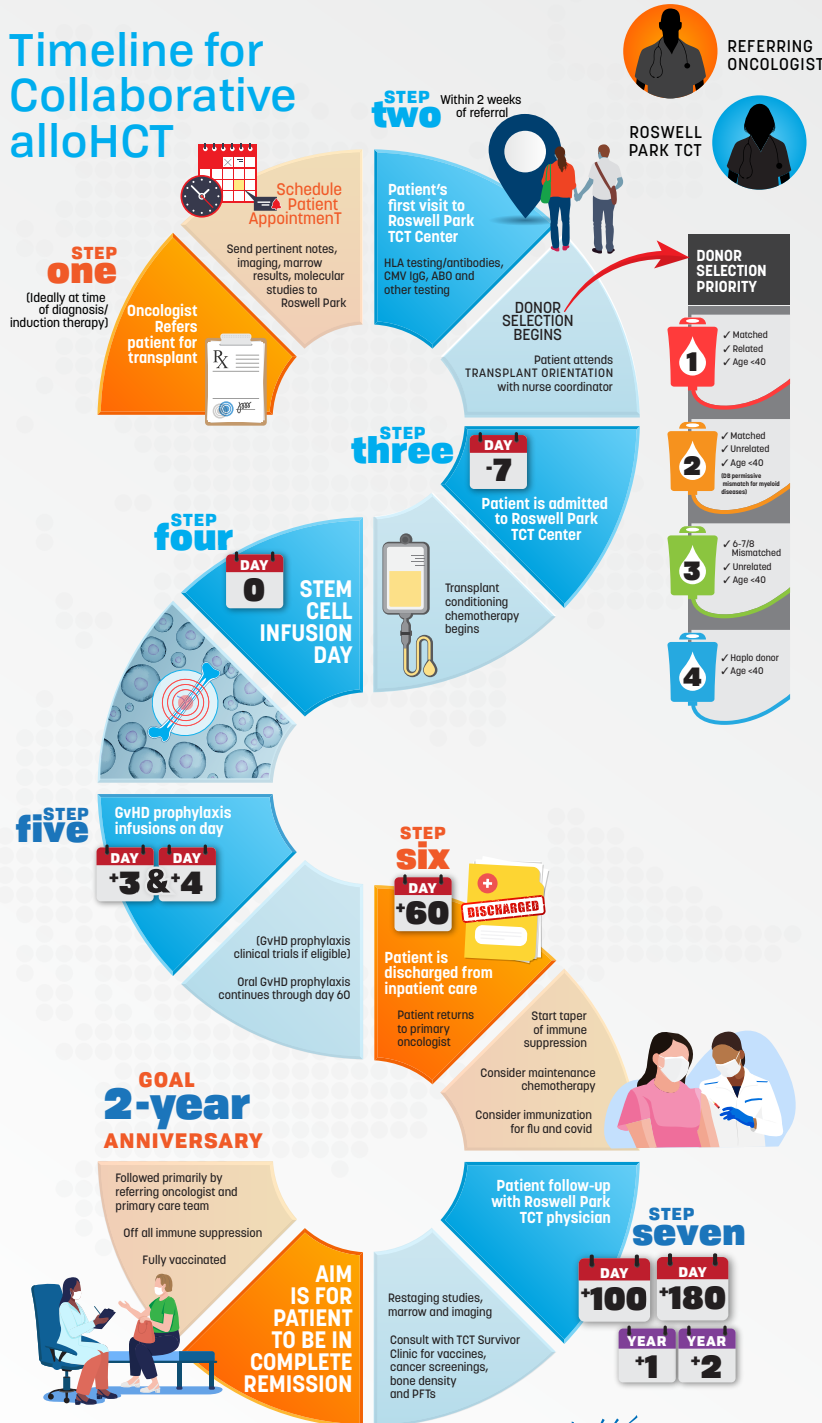
— Brian Betts, MD

We partner with referring physicians to reduce inpatient duration and return patient to care of primary oncologist sooner.

# COLLABORATIVE Care

While many aspects of transplant and cell therapy care will be managed at Roswell Park during the first 1-3 months, we aim to have patients back with their referring oncologists after 30 days post autologous transplantation or CAR T, or after 60-100 days following allogeneic transplantation. We will continue to see patients for comprehensive response assessments at 3 months, 6 months, 1 year, and 2 years post-transplant.

## Timeline for Collaborative alloHCT



## PEDIATRIC Transplant Program

Pediatric patients are cared for through a joint venture between Roswell Park and John R. Oishei Children's Hospital of Buffalo.



This unique partnership allows our youngest patients to receive cutting-edge oncology care, transplant, and cellular therapies along with the pediatric care and subspecialties of a freestanding children's hospital, while remaining close to home.

The joint program provides transplant and cellular therapies to pediatric patients of all ages for the following diagnoses:

- **Aplastic anemia, Fanconi anemia and other marrow failure syndromes**
- **Hemophagocytic lymphohistiocytosis**
- **Leukemia**
- **Lymphoma**
- **Malignant brain tumors**
- **Myelodysplastic and myeloproliferative disorders**
- **Neuroblastoma**
- **Sickle cell disease**
- **Thalassemia**
- **Immunodeficiency disorders**



# Chimeric Antigen Receptor (CAR) T-CELL THERAPY

CAR T-cell therapies, made from a patient's own T cell lymphocytes, offer new hope and potentially curative options for patients with relapsed or refractory disease, including:

- **Large B cell lymphoma**
- **Mantle cell lymphoma**
- **Follicular lymphoma**
- **Acute lymphoblastic leukemia**
- **Multiple myeloma**

Roswell Park is an authorized treatment center for all FDA-approved CAR T-cell therapies:

- ✔ **Abecma™**
- ✔ **Breyanzi™**
- ✔ **Carvykti™**
- ✔ **Kymriah™**
- ✔ **Tecartus™**
- ✔ **Yescarta™**

With CAR T-cell therapies, the patient's T cells are collected, genetically modified to recognize and attack cancer cells, multiplied into the millions, and infused back into the patient.

## Tumor-Infiltrating Lymphocyte (TIL) THERAPY

A cutting-edge cell therapy, TIL treatment offers a promising strategy for the treatment of advanced solid tumor cancers. Using each patient's own T cells — collected from within their own tumor tissue — TIL therapy multiplies these cells and returns them to the patients. These cells need no engineering. They already know how to find and infiltrate the tumor.

Roswell Park currently offers TIL therapy through clinical trials for eligible patients with:


- **cervical carcinoma**
- **non-small cell lung cancer**

The first FDA approval of a TIL therapy to be used in patients with metastatic melanoma is expected in the coming months and Roswell Park will offer this as well.

### TIMELINE for TIL therapy


Receiving TIL therapy is a multi-step process that involves:

- **Surgery to remove the tumor or metastatic lesions.** T cells are retrieved and multiplied into millions.
- **Non-myeloablative chemotherapy** given over 7 days.
- **TIL infusion.** Receiving TIL therapy is a one-time intravenous (IV) infusion.
- **High dose interleukin 2 (IL-2) therapy.** Up to 6 doses over 3 days to stimulate T cell and other immune cell growth.



Diagnosed with aggressive and treatment resistant infantile ALL, Chasity underwent CAR T-cell therapy followed by stem cell transplant at age 7 months.

**Today she is thriving.**



# GENE-EDITED Cellular Therapy

## A breakthrough treatment for sickle cell disease

Casgevy™ (Exagamglogene autotemcel or exa-cel), alters the genes of patients' own hematopoietic stem cells to produce higher levels of fetal hemoglobin in red blood cells. This elevation has been shown to reduce painful and debilitating sickle cell crises and alleviate the need for transfusions in patients with beta thalassemia.

Patients' own hematopoietic stem cells and progenitor cells are collected from peripheral blood, sent to the lab for gene editing, and the edited cells (exa-cel) are returned to the patient as an autologous stem cell transplant.



Roswell Park expects to begin offering this treatment for eligible patients with sickle cell disease soon.

## CLINICAL TRIALS

We have numerous clinical trials evaluating novel approaches for patients undergoing transplant and cell therapies, such as:

- **Novel options for conditioning regimens, GVHD prophylaxis, and post-transplant maintenance therapy**
- **New approaches to prevent bone mineral density loss or infection.**
- **CAR T-cell therapies for other cancer types, including:**
  - acute myeloid leukemia (AML)
  - solid tumors such as breast, lung, prostate and gastrointestinal cancers
- **TIL therapy for solid tumor cancers**

Scan the QR Code to see all our clinical trials



## IN THE PIPELINE

Roswell Park has committed the investment, resources and focus to accelerate the development of next-generation CAR T-cell therapies, and several clinical trials are expected to open in the coming year.

Some highlights of this work include:

- **A new CAR T therapy to prevent relapse and GVHD** in patients with leukemia.
- **Combining CAR T-cell therapy with other immune cells** and components of the immune system to improve the treatment's effectiveness.
- **Identifying new antigens** to re-engineer the T cells to target other cancer types.
- **Breaking down the barriers presented by solid tumors** to make this treatment effective for solid tumor cancers, which represent 80% of all cancers.

## GMP ENGINEERING & CELL MANUFACTURING Facility

Cell therapy requires the ability to engineer and manufacture therapeutic cells in a sophisticated, dedicated facility built for that purpose. Few centers across the country were involved in cell-based cancer treatments at any level when we opened our Therapeutic Cell Production Facility with one GMP cell production "clean room" in 2010.

**Today, our facility includes six cell production clean rooms — and 14 more are under construction now!**

Our in-house cell production eliminates the need to rely on external manufacturing partners and helps us to open more clinical trials faster. Patients who need these treatments don't have time to wait.

ROSWELL PARK HOUSES  
NEW YORK STATE'S  
**FIRST**

## CELL and GENE THERAPY HUB

to develop life-saving technologies and innovative engineered cell treatments.



Yeong "Christopher" Choi, PhD, MBA, led the first iteration of cell therapy production at Roswell Park with a focus on dendritic cell vaccines and engineered T cell receptors (TCRs). Today, he is expanding the facility to manufacture and test new and emerging cellular therapies.

[RoswellPark.org/GMP](http://RoswellPark.org/GMP)

# Meet the TEAM

We've put together a world class team of physicians and scientists — who were central to recent breakthroughs in cellular therapy — to expand our clinical capacity, further development of next-generation cellular therapies, and speed our ability to bring game-changing emerging treatments to more patients.

**Our transplant physicians are members of:**



**Renier Brentjens, MD, PhD**  
Deputy Director  
Chair, Department of Medicine  
Professor of Oncology



**Marco Davila, MD, PhD**  
Senior Vice President and Associate  
Director for Translational Research



**Brian Betts, MD**  
Professor of Oncology  
Vice Chair of Strategic Initiatives



**Shernan Holtan, MD**  
Professor of Oncology  
Chief, Blood and Marrow Transplantation



**Ehsan Malek, MD**  
Director of Multiple Myeloma  
Translational Research



**Anuradha Krishnamurthy, MBBS**  
Associate Director of Early Phase Clinical Trials  
for Solid Tumor Cell Therapies



**Maureen Ross, MD, PhD**  
Professor of Oncology



**Philip McCarthy, MD**  
Professor of Oncology  
Director Emeritus of the Transplant  
& Cellular Therapy Center



**Grant Schofield, MD**  
Professor of Oncology

**WHEN to refer a transplant patient?  
AT TIME OF DIAGNOSIS.**

The ideal time to refer a patient is at time of diagnosis or induction therapy. Even if your patient is in remission, immediate referral is advised to begin preliminary donor search and stem cell collection.

**REFER A PATIENT**  
Call 716-845-RPMD (716-845-7763)



## PEDIATRIC TRANSPLANT TEAM



**Kanwaldeep Mallhi, MD**  
Director, Pediatric Transplantation  
and Cellular Therapy



**Meghan Higman, MD, PhD**  
Assistant Professor of Oncology



**Matthew Barth, MD**  
Assistant Professor of Oncology



**Nataliya Buxbaum, MD**  
Assistant Professor of Oncology

ROSWELL PARK'S PROGRAM HOLDS THESE DISTINCTIONS:

