



# KYMRIAH™ CAR T-cell therapy

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## What is KYMRIAH™ CAR T-cell therapy?

- KYMRIAH™ CAR T-cell therapy (also known as tisagenlecleucel) is a prescription cancer treatment for patients with B-cell acute lymphoblastic leukemia (ALL) that has relapsed (come back) or is refractory (has not responded to other treatments).
- It was developed by the pharmaceutical company Novartis, and approved for use by the U.S. Food and Drug Administration (FDA) in August 2017.
- This treatment is called *Chimeric Antigen Receptor (CAR) T-cell therapy*. It involves treating patients with cells made from their own immune system's white blood cells. These CAR T-cells help protect the body from disease.

## Who can get KYMRIAH™ CAR T-cell therapy?

- Right now, we use KYMRIAH™ CAR T-cell therapy to treat some children and young adults (up to age 25) with relapsed or refractory B-cell ALL.
- You and your care team will decide if KYMRIAH™ CAR T-cell therapy is right for your child.

## Does my child need to have relapsed or refractory ALL to get KYMRIAH™ CAR T-cell therapy?

Yes. There are other treatments that we use for ALL if your child has not relapsed. We try those treatments first before using CAR T-cell treatment.

## Does KYMRIAH™ CAR T-cell therapy treat other kinds of cancer?

KYMRIAH™ CAR-T-cell therapy does not treat other kinds of cancer at this time.

## Is KYMRIAH™ CAR T-cell therapy a cure for ALL?

The goal of this therapy is to cure ALL; however, your care team will discuss your child's specific case in more detail with you.

## How do I know if KYMRIAH™ CAR T-cell therapy is covered by health insurance?

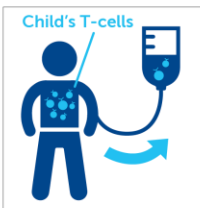
- Because this is so new, it's not yet clear how much insurance providers may cover for this therapy. We encourage you to talk with your insurance company.
- Please talk with your care team. Our staff can help to guide you through the insurance approval process.

## How does KYMRIAH™ CAR T-cell therapy work?

KYMRIAH™ CAR T-cell therapy uses cells made from the patient's immune system (the body's natural defense against illness). It takes many steps to make this treatment and to make sure it is safe for your child. It takes about 3 weeks from collecting the cells to treatment.



1. We will examine your child to see if KYMRIAH™ CAR T-cell therapy is a safe option. A leukemia specialist will look at your child's medical history, general health and see how well their heart, lungs and nervous system work.



2. If this therapy is right for your child, we will collect your child's T-cells through a process called apheresis (a procedure that removes blood cells from the body). We may need to place a central line (catheter) to help collect your child's T-cells. This may mean that your child will have general anesthesia (go to sleep) and have the line placed in an operating room.



3. The collected T-cells are transferred to our cell processing facility, where they are frozen and shipped to the Novartis cell manufacturing lab. There, the T-cells are genetically altered to recognize and kill cancer cells.



4. To keep your child's leukemia under control, your child may get chemotherapy treatment at Dana-Farber/Boston Children's Cancer and Blood Disorders Center or at your primary hospital while the T-cells are being processed in the lab.



5. When your child's engineered T-cells are returned from the lab, they are kept frozen until your doctor says your child is ready for treatment. When your child is ready, your child will be admitted to the Bone Marrow Transplant Unit at Dana-Farber/Boston Children's Cancer and Blood Disorders Center. Your child will have 6 days of preparation chemotherapy before the engineered T-cells are given intravenously (through an IV line), like a blood transfusion.



6. Your child will stay in the hospital on average 30 days so we can watch for any side effects.

## What are the risks/side effects of KYMRIAH™ CAR T-cell therapy?

- One of the most common side effects is **cytokine-release syndrome (CRS)**. T-cells make cytokines as part of their work to fight diseases. These are chemicals that help fuel the body's immune response. CRS happens when the body makes a lot of cytokines too fast and puts them into the bloodstream. This can lead to high fevers and sudden drops in blood pressure.
- **B-cell aplasia** is a possible side effect. B-cells make **antibodies** that kill disease cells. B-cell aplasia happens when a large amount of B-cells are killed by CAR T-cell treatment. Many patients get **immunoglobulin therapy** (extra antibodies) to help prevent disease when a patient doesn't have B cells.
- **Cerebral edema** (swelling in the brain) is a rare side effect that can sometimes be life-threatening. If it occurs, it usually is in patients with more advanced leukemia.

## How will my child feel after KYMRIAH™ CAR T-cell therapy?

The treatment can cause short-term problems with memory and coordination. It can also cause sleepiness, confusion, weakness, dizziness and seizures.

## What does it mean to be a certified treatment center? Can any hospital give KYMRIAH™ CAR T-cell therapy?

Only hospitals that meet quality and technical standards set by Novartis can offer this treatment. Dana-Farber/Boston Children's Cancer and Blood Disorders Center has been approved by Novartis as a certified treatment center for KYMRIAH™ CAR T-cell therapy.

## Can I get KYMRIAH™ CAR T-cell therapy outpatient?

We feel that KYMRIAH™ CAR T-cell therapy should be an inpatient treatment. Due to the possible risks, we think it is safer to treat patients here in the hospital so that we can watch them closely for how they are reacting.

## Who will be part of my child's KYMRIAH™ CAR T-cell therapy treatment team?

- You will have a specialized care team to guide you through the process. Members of the team may include:
  - Bone marrow transplant specialist
  - Boston Children's Blood Lab (Apheresis team)
  - Cell manufacturing core facility (CMCF) group
  - Gene therapy specialist
  - Leukemia specialist
  - Nurse coordinators
  - Psychosocial support team
  - Staff nurses and clinical assistants

## How long will we need to be in the Boston area?

- You will need to stay within 2 hours of Dana-Farber/Boston Children's Cancer and Blood Disorders Center for at least 4 weeks from the time of the CAR T-cell infusion.
- If your child was sent from another hospital, we will transfer your child's care to your home hospital.

## What resources are available to support my family?

- Dana-Farber Cancer Institute's Pediatric Resource Program provides resource assistance to eligible families whose children are currently receiving treatment at Dana-Farber/Boston Children's Cancer and Blood Disorders Center.
  - To speak with a resource specialist, please ask any member of your care team for a referral or call one of our resource specialists directly. Please visit the Pediatric Resource Program website for contact information: [dana-farber.org/for-patients-and-families/care-and-treatment/support-services-and-amenities/pediatric-resource-program/](http://dana-farber.org/for-patients-and-families/care-and-treatment/support-services-and-amenities/pediatric-resource-program/).
- Boston Children's Hospital's Hale Family Center for Families is available to answer questions, provide resources and help you during your time at the hospital. For more information:
  - Call 617-355-6279
  - Email [center.families@childrens.harvard.edu](mailto:center.families@childrens.harvard.edu)
  - Visit [childrenshospital.org/patient-resources/family-resources/the-center-for-families](http://childrenshospital.org/patient-resources/family-resources/the-center-for-families)

## Contact us

**For more information about KYMRIAH™ CAR T-cell therapy**, please contact Dana Farber/Boston Children's Cancer and Blood Disorders Center, Gene Therapy Program:

- **Phone:** (617) 632-5064
- **Website:** [danafarberbostonchildrens.org/innovative-approaches/gene-therapy.aspx](http://danafarberbostonchildrens.org/innovative-approaches/gene-therapy.aspx)
- **Email:** [gene.therapy@childrens.harvard.edu](mailto:gene.therapy@childrens.harvard.edu)

**For patient care assistance**, please call the following phone numbers:

- Jimmy Fund Clinic (617) 632-3270  
Business Hours 8 a.m. to 5 p.m., Monday through Friday
- Page Operator (617) 632-3352  
Off hours: weekdays after 5 p.m., holidays, and weekends  
Page pediatric hematology/oncology fellow-on call
- Life Threatening Emergencies dial 911