Patient Advocacy

Our Commitment to Patients

CRISPR Therapeutics is developing gene-based medicines with the potential to transform the lives of people with serious diseases. This mission defines our company, unites our teams, and inspires our work.

We are ready to **break through, together.**

At CRISPR Therapeutics, we approach patient advocacy by:

Learning

We listen to patient communities to gain a more complete understanding of serious diseases and their impact on patients' lives.

Informing

We inform patient communities about our research when possible and appropriate, while following guidelines and regulations that govern our industry.

Achieving Together

We foster relationships with patient communities and organizations based on transparency, compassion, and respect.

Download a copy of CRISPR Therapeutics' guiding principles for patient advocacy here.

CRISPR Therapeutics' Patient Advocacy Charter

Patient Organizations

Patient organizations bring together communities, educate patients, and help advocate for research, funding, and treatments. CRISPR Therapeutics is proud to work with patient organizations and find ways to partner with them to accelerate progress toward our shared goals.

Many patient organizations are credible and reliable sources of information and patient support, including the groups listed below. CRISPR Therapeutics provides these links as a resource but does not endorse specific patient organizations or their communications.

Hemoglobinopathies

- Cooley's Anemia Foundation
- Sick Cells
- Sickle Cell Consortium
- Sickle Cell Disease Association of America, Inc.
- Sickle Cell Disease Coalition

Kidney Cancers

- International Kidney Cancer Coalition
- KidneyCAN

Lymphoma

- Leukemia & Lymphoma Society
- Lymphoma Research Foundation

Oncology

- American Cancer Society

To learn more about our current patient advocacy initiatives or to discuss ways to collaborate with us, **please contact us at patientadvocacy@crisprtx.com.**

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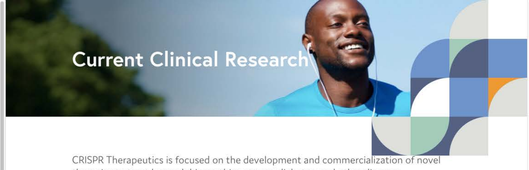


Current Clinical Research

CRISPR Therapeutics is focused on the development and commercialization of novel therapies to treat hemoglobinopathies, cancer, diabetes, and other diseases.

Learn about our current clinical research and trials in hemoglobinopathies and oncology below.

Sickle Cell Disease and β-thalassemia

Sickle cell disease and β-thalassemia are inherited blood diseases that result from mutations in the gene that makes hemoglobin. Hemoglobin is a molecule that is responsible for carrying oxygen within red blood cells as they travel in the bloodstream.[1,2]

People who have **sickle cell disease** have red blood cells that take on a crescent or sickle shape under certain circumstances. The most common symptoms patients with sickle cell disease have are anemia, frequent infections, and pain.[2]

People who have **β-thalassemia** have less hemoglobin. This can result in anemia, or fewer red blood cells than normal, for which patients may require frequent red blood cell transfusions that can have long-term negative consequences on the heart, liver, and other organs.[1]

Some symptoms of anemia include:[1,3]

- Dizziness
- A fast heartbeat
- Shortness of breath
- Pale skin

Both diseases require lifetime treatment. They can result in painful symptoms, the need for regular transfusions, and frequent hospitalizations. Complications from both diseases can cause someone to die earlier than would be expected.[1,2]

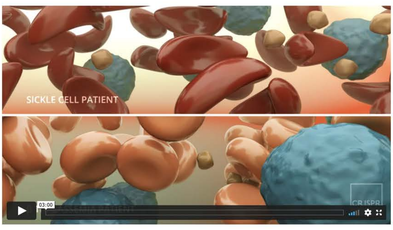
Get the Facts on Gene Editing for Hemoglobinopathies

Download the infographic

CRISPR in Hemoglobinopathies

CRISPR Therapeutics is researching a gene-editing approach designed to edit blood cells to increase hemoglobin.

CRISPR Therapeutics' gene-editing approach is still being investigated in clinical trials, and its safety and efficacy have not been established. It is not approved for use in patients in the United States or in any other countries.



CRISPR/Cas9 is a gene editing technology that works by making precise, directed changes to DNA.

Learn more in our video

Oncology

CRISPR Therapeutics' cancer programs are investigating blood cancers such as **B- and T-cell lymphomas** and **multiple myeloma** as well as **renal cell carcinoma**, a type of solid tumor.

B cells and T cells are types of white blood cells called lymphocytes. They are part of the immune system, which helps your body fight infections and other disease.

If genes that control the growth of lymphocytes no longer work properly, these cells can multiply out of control or live longer than they normally would. This condition is called lymphoma.[4]

Symptoms of B-cell and T-cell lymphoma can vary in severity, and include swollen lymph nodes that may be painful, fever, night sweats, weight loss, chills, tiredness, and itching.[5]

Multiple myeloma is a type of cancer that develops in the bone marrow, the spongy material found in the center of most bones. Bone marrow is one of the places the body makes blood cells, including plasma cells. Plasma cells make antibodies, which the body uses to attack and kill germs.[6]

Multiple myeloma causes plasma cells in the marrow to multiply out of control. As a result, the marrow produces fewer:

- Red blood cells (anemia), which can lead to fatigue and weakness
- White blood cells (leukopenia), which weakens the immune system and increases the risk of infection
- Platelets (thrombocytopenia), which can lead to abnormal bleeding and bruising[6]

Multiple myeloma can cause tumors in the bones that can lead to pain, fractures and loss of bone mass in the spine, skull, and ribs.[6]

Renal cell carcinoma, or RCC, is a type of kidney cancer. In renal cell carcinoma, cells in the kidney multiply and create tumors. In its advanced form, RCC cells have spread to other parts of the body.[7] Symptoms of RCC include blood in the urine, pain or pressure in the side or back, swelling in the ankles and legs, high blood pressure, anemia, tiredness, weight loss, and fever.[7]

What is Car T Cell Therapy?

Download the infographic

CRISPR Therapeutics' gene-editing approach to cancer uses CAR T cells. These are T cells that have been modified to include a chimeric antigen receptor (CAR) on their surface. With this receptor, CAR T cells are able to find and kill cancer cells.

CRISPR Therapeutics' gene-editing approach is still being investigated in clinical trials, and its safety and efficacy have not been established. It is not approved for use in patients in the United States or in any other countries.

What are Clinical Trials?

A clinical trial is a study used to learn more about a treatment for a disease or condition. People with the condition being studied are enrolled as volunteers. The purpose of a trial is to determine the safety of a treatment and how well it works.

Clinical trials follow strict guidelines laid out by federal regulatory agencies, such as the U.S. Food and Drug Administration.[8]
For more information, visit the U.S. Food and Drug Administration's website.

Current Clinical Trials

The therapies that CRISPR Therapeutics is investigating are not approved by the FDA or any other countries' Health Authority. This means that their safety and efficacy are still being studied in clinical trials. You can find more information about our current trials at clinicaltrials.gov or use the links below:

- Sickle Cell Disease and β-thalassemia
- B-cell malignancies
- T- or B-cell malignancies
- Renal cell carcinoma
- Multiple myeloma

You can also talk to your health care provider about clinical trials and if enrolling in a trial may be right for you.

References

1. Beta thalassemia. Genetics Home Reference, U.S. National Library of Medicine. https://ghr.nlm.nih.gov/condition/beta-thalassemia. August 17, 2020. Accessed: September 18, 2020.

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3. Mayo Clinic. Anemia: Symptoms & causes. Mayo Clinic Website. Published August 16, 2019. Accessed December 15, 2020. https://www.mayoclinic.org/diseases-conditions/anemia/symptoms-causes/syc-20351360

4. What is the difference between B-cell lymphoma and T-cell lymphoma? Dana Farber Cancer Institute. https://blog.dana-farber.org/insight/2019/06/what-is-the-difference-between-b-cell-lymphoma-and-t-cell-lymphoma/. June 18, 2019. Accessed: September 18, 2020.

5. What is lymphoma? Lymphoma Research Foundation. https://lymphoma.org/aboutlymphoma/. Accessed: September 18,2020.

6. Multiple myeloma. Genetics Home Reference, U.S. National Library of Medicine. https://ghr.nlm.nih.gov/condition/multiple-myeloma. August 17, 2020. Accessed: September 18, 2020.

7. Kidney cancer - introduction. https://www.cancer.net/cancer-types/kidney-cancer/introduction. November 25, 2019. Accessed: November 11, 2020.

8. ClinicalTrials.gov. Learn About Clinical Studies. ClinicalTrials.gov. Updated March 2019. Accessed December 04, 2020. https://clinicaltrials.gov/ct2/about-studies/learn

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Expanded Access to Investigational Medicines

CRISPR Therapeutics is focused on the development and commercialization of novel therapies to treat hemoglobinopathies, cancer, diabetes, and other diseases.

At this time, CRISPR Therapeutics does not provide access to investigational products outside of clinical trials. We encourage patients to participate in clinical trials of our investigational therapies whenever possible, because clinical trials are designed, conducted, and monitored to ensure that the safety and efficacy are appropriately evaluated before they are submitted to regulatory agencies for review with the intent to make them more broadly available to patients.

You and your health care provider may learn more about our clinical trials by going to the pipeline section of our website, or visiting www.clinicaltrials.gov and searching for CRISPR Therapeutics.

If you are a health care provider who is interested in learning more about one of our investigational therapies, or a physician with questions about participation in one of our clinical trials, please submit a request to medicalaffairs@crisprtx.com. The company will acknowledge questions as soon as possible, usually within 5 business days of receipt.

If applicable, this website will be updated with hyperlinks to the relevant expanded access information on www.clinicaltrials.gov upon activation. CRISPR Therapeutics reserves the right to revise this expanded access policy at any time.

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