STAR (Sickle Cell Transplant Advocacy & Research Alliance) is a non-profit 501 C3 organization founded in 2014 by a group of pediatric hematology and hematopoietic stem cell transplant physicians to transform the lives of children suffering from Sickle Cell Disease (SCD) by curing sickle cell through bone marrow transplantation (BMT) and gene therapy. STAR is supported by 39 leading Children’s Hospitals across North America.

Mission: To help cure Sickle Cell Disease.

Vision: To greatly increase awareness, accessibility & success of Bone Marrow Transplant (BMT) and Gene Therapy as cures for Sickle Cell Disease.

Why is STAR needed?
• Established SCD organizations provide support services for families caring for children with this devastating disease.
• No large organization exists with a focus only on research to cure sickle cell disease.
• This research is best advanced through clinical studies involving many different institutions.
• STAR provides critical funding and an organized collaborative network for this research.

What is Sickle Cell Disease?
• Sickle cell disease is an inherited blood disorder that causes red blood cells to become sickle shaped and rigid, which obstructs blood flow.
• It affects people of African, Hispanic, Middle Eastern, Asian, Indian and Mediterranean descent
• SCD causes lifelong pain and can result in stroke, organ damage and early death.
• People with sickle cell disease have described the pain as “like a hammer, beating and beating,” and “like being hit by a truck and getting run over.”
• African Tribal names for Sickle Cell Disease include: Chwechweechwe (relentless perpetual chewing), Adep (beaten up) and Hem Kom (body biting)
• In 1973, the average life span for someone with SCD was 14 years.
• In 2018, the average life expectancy for someone with Sickle Cell Anemia in the US was approximately 48 years, while it is only 8 years in much of Africa.

How many Americans suffer from SCD and have the Sickle Cell Trait (SCT)?
• SCD affects approximately 100,000 Americans and 3 million have the Sickle Cell Trait.
• SCD occurs among about 1 out of every 365 African-American births, while about 1 in 12 African-American babies is born with the SCT (8%).
• SCT is not a disease, but having it means that a person has inherited the sickle cell gene from one of his or her parents and is a trait “carrier” who can pass it on to their children. People with SCT usually do not have any of the symptoms of SCD and live a normal life.
How does a bone marrow transplant (BMT) work?
- A bone marrow transplant is a medical procedure, not a surgery.
- It involves replacing the patient’s bone marrow that makes the abnormal red blood cells with healthy bone marrow cells.
- It involves giving the patient medicines to eliminate their abnormal bone marrow and to suppress their immune system, so that they can accept the healthy donor cells.
- Donor cells are introduced into the blood, like a blood transfusion.
- Patient typically takes immune suppressive medicine for up to 12 months post-transplant.

Why don’t all children with sickle cell disease get a bone marrow transplant?
- Bone marrow transplants have serious associated risks including death.
- Bone marrow transplants work best with a healthy “matched” sibling donor.
- Through research led by STAR investigators, we can refine the transplant process to decrease risks and safely perform transplants using alternative donors so one day all children with sickle cell disease can look forward to a long, healthy life.
- Few transplants were performed before 2000, though there has been a significant increase in BMT use over the past 20 years as safety and success of the procedure has improved.

What is Gene Therapy (GT)?
- Definition: "The replacement or correction of abnormal genes in order to treat genetic disorders"
- Actively researched for SCD, but treatment is only available through clinical trials
- Modifies patients bone marrow cells so that they produce normal red blood cells
- The GT Patient receives their own genetically modified cells to replace their damaged cells

What Sickle Cell Disease research initiatives is STAR currently conducting or completed?
1. Retrospective Registry of Transplants to Cure Sickle Cell Disease (Data Collected 2019, Analysis ongoing)
2. Finding Better Ways to Prevent Graft Vs Host Disease: Abatacept (Completed 2019)
3. Finding Better Ways to Prevent Graft Vs Host Disease: Abatacept: ASCENT (Expected Completion 2022 )
4. Developing a More Effective Approach to Platelet Transfusion (Completed 2019, Results published 2020)
5. Performing BMT Early to Maximize Benefits (Expected Completion 2022)
6. Developing a Safer Way to prevent BMT Rejection: SUN (Expected Completion 2021)
7. Project Sickle Cure: Creating a Resource for Future Discovery (Opened Q1 2021)

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- On the web at: www.curesicklenow.org
- On Facebook at https://www.facebook.com/CureSickleNow/
- To Get Involved: Jessica Salomon, Executive Director, STAR, curesickle@gmail.com
- To learn about STAR Research initiatives:
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Contact STAR to learn more, get involved or donate at: www.curesicklenow.org