A REPORT PREPARED FOR KIDZ1STFUND

Making a difference for kids with Fanconi anemia

Through your catalyzing leadership since 2011, Kidz1stFund has accelerated unprecedented progress in FA research at the University of Minnesota. Your generosity—and the support of the thousands of people you have inspired to join you—makes such a difference to the work we are doing to discover new ways of helping children like Ethan. We are also applying those discoveries to begin making a difference for millions of other people who need a bone marrow transplant or who have diseases like leukemia, and cancers of the head and neck.

Inspiring hope: A legacy of changing lives

Fifteen years ago, long-term survival after bone marrow transplant for children with FA was only about 30 percent. Today at University of Minnesota Masonic Children's Hospital, it is **better than 90 percent,** thanks to decades of research and teamwork at one of the leading Comprehensive Fanconi Anemia Centers in the nation.

The generosity of Kidz1stFund has fueled advances in blood and marrow transplant (BMT) for treating FA, building on our 50-year history as a pioneer in BMT.

Despite the advances, it is still difficult to get all FA patients through the challenges of BMT, as they routinely face life-threatening infections and other complications. While transplant remains the standard of care and best hope for most patients with FA today, we are driven to make the treatment easier for them—and to make sure that *more* patients not only survive but *thrive* after transplant.

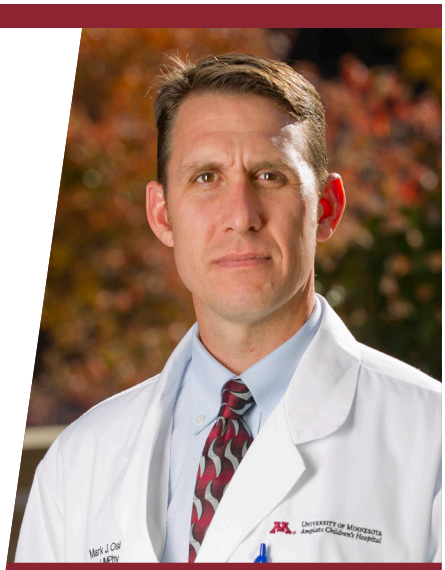
At the same time, we are pursuing new avenues of research to ultimately move beyond BMT to even safer, more effective forms of treatment for FA—as well as identifying new ways to prevent and treat cancers that are particularly common in FA patients and that also affect millions of others.

With the support of Kidz1stFund, we are making great strides on both of these important journeys.

With the help of Kidz1stFund, we have accomplished so much

Thanks to generous Kidz1stFund gifts totaling a remarkable $8.5 million to date, we are:

- Developing exciting strategies for **improving outcomes after transplantation.**
- **Conducting gene therapy research** aimed at correcting the defective FA gene in patients who have the disease and eliminating the need for conventional BMT—and the risks that it carries.



"Gene therapy and stem cell expansion, stem cell engineering, and differentiation technologies are next generation therapies that are progressing at a pace that we hope will be applicable to the current generation of patients. Kidz1stFund has been a phenomenal partner in aiding us as we translate these therapies into application for human use."

– Mark Osborn, Ph.D.
Assistant Professor, Division of Pediatric Blood and Marrow Transplantation

- Manipulating the patient's immune system and the donor's cells to **minimize the risk of infection** while at the same time allowing newly transplanted cells to grow.
- Using our new **CliniMACS® Prodigy system,** a leap forward in automated cell processing, to determine whether it is possible and safe to treat or prevent life-threatening viral infections in FA patients who are undergoing transplantation.
- Developing a program to study all aspects of FA patients' health to **reduce their risk for malignancies** and to optimize their physical, mental, and psychosocial well-being.
- Examining the causes of head and neck cancer in FA patients, ultimately **improving prevention and treatment options.**
- Applying our FA discoveries to **improve bone marrow transplants for other life-threatening diseases** such as myeloma and ovarian cancer.

Top FA donor: Kidz1stFund!

The support of your family—and the community that you have inspired—has propelled Kidz1stFund to become a leading donor to FA research in the country, and the impact of your giving cannot be overstated.

We are honored and inspired by your commitment to fighting FA.

"Time is never on our side for children with FA. ***With the help of Kidz1stFund, we are getting research done faster.*** *We are making a difference in changing the practice of medicine—not only for people with FA, but for those with other life-threatening conditions that aren't so rare."*

— Margaret L. MacMillan, M.D.
Professor of Pediatrics; Director, University of Minnesota Fanconi Anemia Comprehensive Care Clinic, Pediatric BMT Program, University of Minnesota Masonic Children's Hospital

Candi Fisher & Jimbo Fisher consult with Dr. Margaret MacMillan