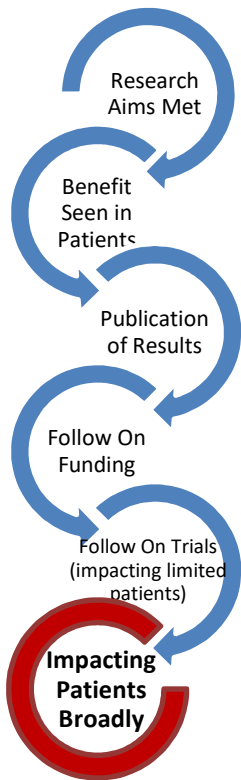


Repurposing a Transplant Drug Saves Kids with ALPS, a Rare but Deadly Blood Disorder



Measuring CWR's Success



In 2004, Cures Within Reach funded **Dr. David Teachey** at **Children's Hospital of Philadelphia** to repurpose **sirolimus**, a generic transplant drug, for a pediatric, ultra-rare blood disorder, **Autoimmune Lymphoproliferative Syndrome (ALPS)**. In less than 36 months, Dr. Teachey demonstrated that the drug helped mice with this disease, and then he showed the same with kids who had this disease. 85% of the kids who were treated were in remission after just 90 days on the drug, and these were kids that had failed all other therapies and were slowly dying. Many of these kids have been in remission many years later, taking just two pills each day. Their healthcare costs have gone way down, and they and their families have an almost normal life.

Based on this success in ALPS, in 2011 CWR funded Teachey's follow-on research to repurpose the same drug and in five additional diseases (Evans disease, systemic lupus erythematosus, autoimmune hemolytic anemia, idiopathic thrombocytopenic purpura and common variable immunodeficiency) and the same thing happened: 63% kids went into remission and have been living almost normal lives!



KEY FACTS

- CWR funded \$73,000 for two projects, in 2004 and 2011, with total project budgets of \$78,000

IMPACT

- This treatment is being used for ALPS patients and around the world with great success, as well as in 5 other pediatric autoimmune diseases
- Teachey raised more than \$1.2 million in following on funding from the NIH and others
- Teachey published more than 12 articles based on this research

