

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



Measuring CWR's Success

Clinical Trials With Early Positive Evidence

Follow-On Funding for Follow-On Clinical Trials

Clinical Results Impacting Patients Broadly

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 he showed the same with pediatric ALPS patients in a clinical trial: 85% who were treated were in remission after just 90 days on the drug. Many of these patients had failed other therapies and were slowly dying, but now many are still in remission years later with just two pills each day. Their healthcare costs have declined, and they can lead an almost normal life. Based on this success, CWR funded Dr. Teachey's clinical trial testing sirolimus in four more diseases (Evans syndrome, systemic lupus erythematosus, autoimmune hemolytic anemia, and common variable immuno-deficiency) with similar results: 67% of kids with multilineage cytopenias went into remission.



KEY FACTS AND IMPACT

- CWR funded two projects: a preclinical trial in 2004 and clinical trial in 2011 with a total budget of \$78,000.
- Sirolimus, a generic drug, is **used off-label** and considered a **first-line treatment option and standard of care for ALPS patients** around the world, as well as in 4 other pediatric autoimmune diseases
- Dr. Teachey raised more than **\$1.2 million in follow-on funding** from the NIH and others
- Dr. Teachey published more than 10 articles allowing for broad off-label use of sirolimus

