



# REPURPOSING A TRANSPLANT DRUG SAVES KIDS WITH ALPS, A RARE BUT DEADLY BLOOD DISORDER



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 immunodeficiency) with similar results: 67% of kids with multilineage cytopenias went into remission.

## Key Facts & Impact

Cures Within Reach funded two projects: a preclinical trial in 2004 and clinical trial in 2011 with a total budget of \$78,000.

Dr. Teachey raised more than **\$1.2 million in follow-on funding from the NIH and others**, and published more than 10 articles allowing for broad off-label use of sirolimus.

Sirolimus, a generic drug, is now **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.

## Measuring CWR's Success

