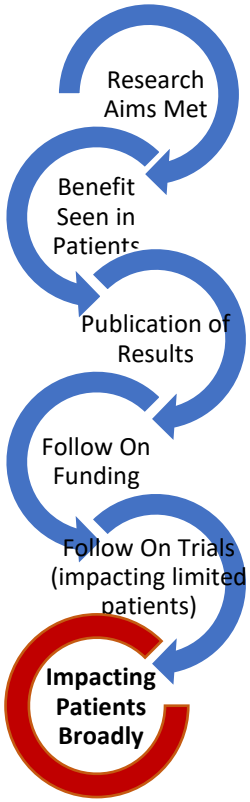
Repurposing a Transplant Drug Saves Kids with ALPS, a Rare but Deadly Blood Disorder

Measuring CWR's Success



Children's Hospital of Philadelphia
RESEARCH INSTITUTE

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 Dr. Teachey's follow-on research to repurpose the same drug in five additional diseases (Evans syndrome, systemic lupus erythematosus, autoimmune hemolytic anemia, idiopathic thrombocytopenic purpura and common variable immuno-deficiency) 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 as standard of care for ALPS patients around the world with great success, as well as in 5 other pediatric autoimmune diseases
- Teachey raised more than **$1.2 million in follow-on funding** from the NIH and others
- Dr. Teachey published more than 10 articles based on this research

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