Building Value in Clinical Repurposing Opportunities

CuresWithinReach

Findacure
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Mission

Improve patient quality and length of life by leveraging the speed, safety and cost-effectiveness of medical repurposing research ***driving more treatments to more patients more quickly***

Value Driving Catalyst, bringing stakeholders together to undertake repurposing research opportunities

Value Driving Facilitator, building, managing and growing CureAccelerator as a central repurposing platform and creating other opportunities to bring together repurposing research stakeholders

Together We're Making Real Patient Impact

49 Diseases Researched	**13** Diseases Improved	**85** Repurposing Projects Funded	**51** Institutions Funded
6 Startups with CWR Investment	**7** Companies Commercializing CWR Projects	**$6 million** in Project Funding	**$49 million** Follow-On Funding Leveraged from CWR's Funds

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Definitions

- **Repurposing**: finding a new disease indication for a drug, device or nutraceutical already approved for human use
- **Repositioning / Rescue**: finding an indication for a human safe pipeline compound
- **Commercial**: able to generate a profit for the organization by bringing it to market through regulatory approval
- **Philanthropic**: able to be used by physicians and patients but unable to generate a profit in the market for an organization

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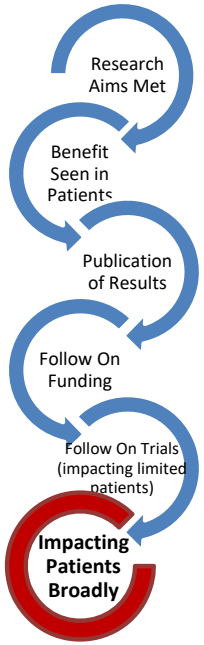
Repurposing a Transplant Drug Saves Kids with ALPS, a Rare but Deadly Blood Disorder

Measuring CWR's Success



The Children's Hospital of Philadelphia®
Hope lives here.

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 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 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 10 articles based on this research

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Types of CWR Project Funding

	Organization	Commercial Value	Payback	Size	Reviewers
Research Grants	Nonprofit academic or research institution	Rarely	None	US$50,000 - $250,000	Grant Review Committee and SAB
Research Grants with conditional payback	Nonprofit academic or research institution	Possibly	Possibly fixed payback	US$50,000 - $250,000	Grant Review Committee and SAB
Impact Awards	For profit small businesses	Likely	Possible – linked to equity	US$25,000 - $50,000	Business Development / Commercialization Committee

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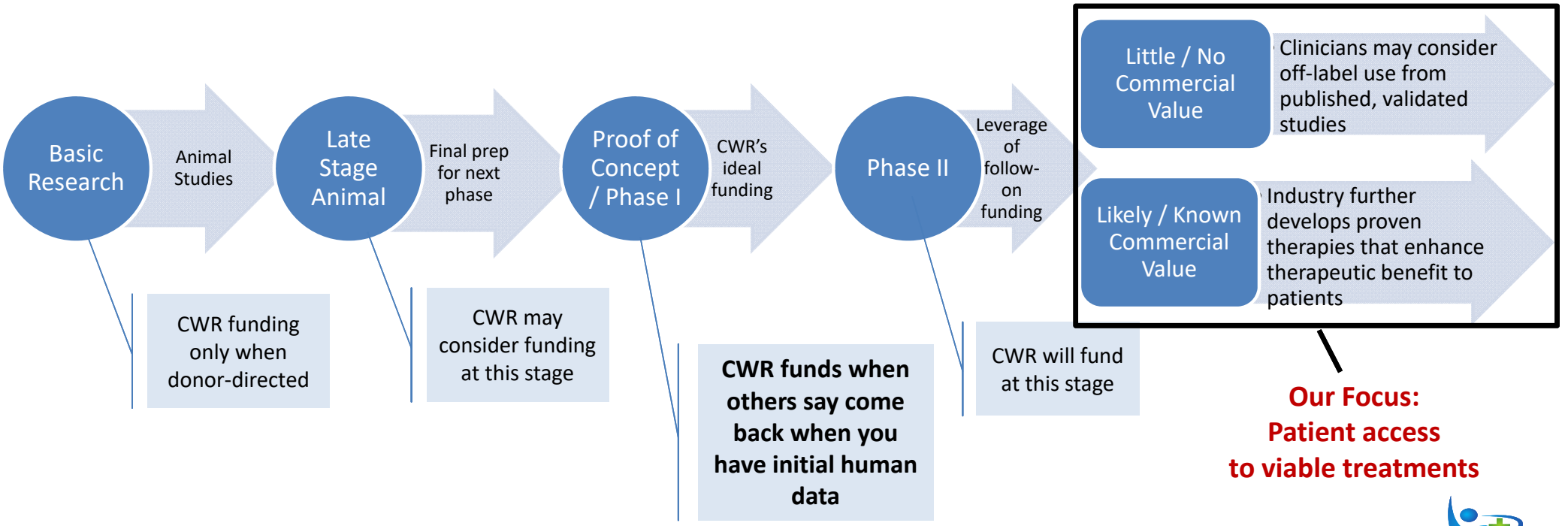
We Provide Clinical Trial Funding at Critical Stages of Therapy Development



Our Current Research

23 projects at 20 institutions in 17 diseases: 50% are in Rare Diseases

Disease Area	Institution	Lead Researcher	Name	Type	Other
Neuro / Rare	Georgetown University	Dr. Anderson	Using a Cancer Drug in Huntington's Disease	Drug	Adult
Neuro / Rare	University of Texas Health Science Center at Houston	Dr. Furr-Stimming	Treating Irritability in Huntington's Disease with a Repurposed Neurological Drug	Drug	Adult
Oncology / Rare	Ulm University	Dr. Halatsch	Combining 9 Repurposed Drugs with a Current Chemotherapy Treatment in Adult Brain Cancer	Drug	Adult
Oncology / Rare	University of Michigan	Dr. Swiecicki	Repurposing Old Drugs as New Therapies for Metastatic Thyroid Cancer	Drug	Adult
Oncology / Rare	Massachusetts General Hospital / Harvard Medical School, VUmc Cancer Center Amsterdam	Drs. Tannous, Arrillaga-Romany, Kouwenhoven	A Novel Combination of Generic Chemotherapy Drugs to Treat Brain Cancer	Drug	Adult
Oncology / Rare	University of Michigan	Dr. Pettit	Using a Skin Cancer Drug to Improve Current Treatment in a Rare Blood Cancer, Myelofibrosis	Drug	Adult
Ophthalmic / Rare	University of Michigan	Dr. Zacks	Testing a Malarial Drug in a Rare Ophthalmic Condition, Retinitis Pigmentosa	Drug	Adult
Rare	CHOP & Hospital for Sick Children	Drs. Levine, Sochett	Repurposing an Antibiotic to Treat a Defect in Vitamin D Metabolism	Drug	Pediatric
Rare	Hospital for Sick Children, NIH	Drs. Dowling, Bonnemann	TAM4MTM: Tamoxifen Therapy for Myotubular Myopathy	Drug	Pediatric
Rare	St. Jude Children's Research Hospital	Dr. Nichols	Repurposing a Blood Cancer Drug to Treat an Immune Disorder (HLH) in Children	Drug	Pediatric

All funded by impact philanthropic donors, nonprofits, corporate partners, investors and others interested in impacting the lives of patients living with disease

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Funding Opportunity RFPs Open Now

- ReGRoW Pilot: impacting low and low-middle income countries *(proposals due March 1)*
- **Rare blood cancers / rare sight impairment disease retinitis pigmentosa** *(proposals due March 6)*
- Meniere's disease: both clinical and pre-clinical *(proposals due March 20)*
- Canine diseases / comparative biology *(proposals due March 20)*

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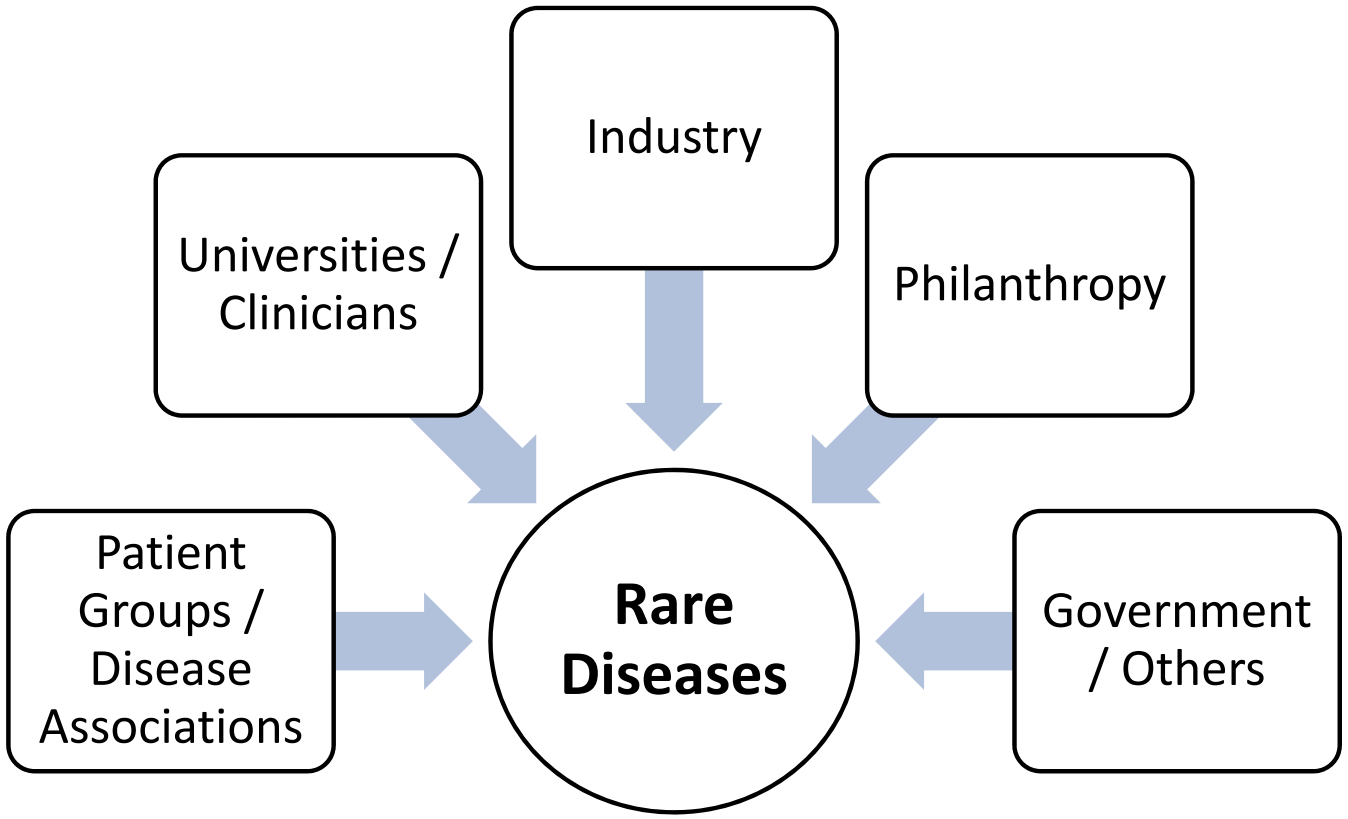
Our Rare Disease Community Serves Many Stakeholders

Value Drivers:

- More "shots on goal" for unmet medical needs within strategically aligned interests
- Investing when commercial value is still unknown
- Exposure to and engagement with patient-centered, patient-focused groups
- Double bottom line: patient impact; success may be reinvested



CWR Provides:

- Partnering with a global repurposing leader
- Vetted due diligence process and management of funded projects during and after
- Neutrality and conflict-free selection process
- Leverage! Seed funding often leads to follow-on funding
- Community of like-minded stakeholders

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Donor-Advised Impact Philanthropy

Impact philanthropy with faster patient impact, customized to a funder's goals

- Donors have **flexibility and control** over involvement during the search, selection and approval processes
- CWR provides access to our entire **50+ research partner network**; a due diligence process using **external grant reviewers** representing research, industry, clinicians and patients to support project selection; expertise in finding, selecting, derisking and managing repurposing specific clinical research projects; both project and financial progress reports during and after the project
- CWR funding of clinical repurposing projects often **catalyzes follow-on funding** from NIH, foundations and investors

Donor-advised funding can support a specific disease or geographic region

- Research grants **starting at US$50,000 can support a small, proof-of-concept human clinical trial**

Recent Examples

- An anonymous family funded an RFP to select two clinical trials in Huntington's disease
- A private foundation supports Impact Awards for Chicago repurposing startups
- Funds pooled from two pharma companies to support an RFP in Rare Diseases, selecting the winner at a CureAccelerator Live! event

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Building Philanthropic Value

- **Rare Diseases**
  - Castleman's disease: David Fajgenbaum, MD discovers his own treatment via sirolimus
  - Glioblastoma at Massachusetts General / Harvard and VUmc Cancer Center in Amsterdam
- **Neglected Diseases**
  - DND*i* has worked since 2003 with a worldwide network of partners to develop 7 new treatments from existing molecules and recombining drugs to bring better treatments to patients for malaria, Chagas disease, leishmaniasis, and pediatric HIV
- **Other Diseases**
  - Alzheimer's disease at University of Toronto

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Building Commercial Value with IP

- **Expansion or new method of use**
  - Ciclopirox prodrug for bladder cancer from CicloMed, a spinout of the University of Kansas Cancer Center
  - Nilotinib for Parkinson's, Huntington's and other neurodegenerative diseases at Georgetown University
  - Ruxolitinib for Hemophagocytic lymphohistiocytosis (HLH) at St Jude Children's Research Hospital
- **Dosing or formulation changes**
  - Viagra
  - Duexis at Horizon Therapeutics
- **Change in delivery mechanism**
  - Treatment for migraines using IGF-1 at Seurat Therapeutics

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Building Commercial Value

- **Combining approved therapies**
  - VXYEOS at Jazz Pharmaceuticals
  - Duexis

- **Regulatory exclusivity**
  - Pediatric use: nilotinib by Novartis for blood cancers
  - Orphan designation: mifepristone for Cushing's syndrome by Corcept Therapeutics

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Q&A

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