

Inside Philanthropy

Hiding in Plain Sight: A Funder Hunting for Lifesaving Alternate Uses for Existing Treatments

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July 20, 2021-The process of developing new drugs, medical devices and other treatments can be, and usually is, painfully slow (fast-tracked COVID-19 vaccines notwithstanding). It might take years or decades from the initial discovery of a drug candidate to testing and regulatory approval for real-world health needs. But there's another very large supply of potential medicines and other therapies hiding in plain sight: the thousands of already-approved drugs and treatments that may be extremely effective for uses other than their initial indication.

Cures Within Reach is a Chicago-based funder that's leading repurposing-oriented research. It's 100% focused on the validation of already approved

therapies for new health needs—about 80% of the projects it funds are pharmaceuticals, with the rest of support going to medical devices, diagnostics, nutraceuticals and supplements.

Depending on the year, some 75% to 85% of Cures Within Reach’s funding comes from generous individual donors, families, as well as corporate foundations. Many of the individual donors are driven by personal experience with a disease and want to generate treatments fast. CWR receives some funding from corporate supporters too, most of them within the health and pharmaceutical industry. In recent years, the Takeda Pharmaceutical Company has been CWR’s largest corporate supporter by far. Other ongoing support has come from Horizon Therapeutics.

For patients with hard-to-treat and thus far incurable conditions, including rare diseases that have not attracted substantial research, the study of already-approved therapies has a couple of crucial benefits. Most importantly, of course, it may uncover a lifesaving or quality-of-life-improving therapy. Secondly, it may well provide such relief years faster than a de novo (new) drug on the lengthy and expensive track from basic science to clinical studies to regulatory approval.

“Most universities and philanthropic organizations look at the long-term, funding bench research all the way through translation. And there’s nothing wrong with that—it’s an important part of the ecosystem,” said Barbara Goodman, president and CEO of Cures Within Reach. But the focus on new science and breakthrough treatments isn’t the only way to get people the help they need. “For patients with unmet medical needs and unsolved diseases, repurposing is an incredibly innovative way to fast-track impact and improve patients’ lives.”

According to one estimate, the development of a new drug is likely to take 13 to 15 years at a cost of \$2 billion. But an already-approved drug can be tested and reach the market in as little as five years, at total costs of around \$350 million.

Repurposing research wasn't always CWR's game plan. Originally, the foundation was more typical in its funding approach, supporting general science and the development of new drugs and therapies. But over the years, they recognized the great potential of repurposing and shifted all their support to this type of research. "We saw an unmet need in the philanthropic community to fund research to focus on things that already have been shown to be safe and effective, and to fast track them straight into patients in a new proof-of-concept or pilot study."

CWR's approach is, in fact, working. Among the successful projects the foundation has supported in recent years are:

- Repurposing of the generic organ transplant drug sirolimus can greatly help children with autoimmune lymphoproliferative syndrome (ALPS), an ultra-rare blood disorder. Previously, kids with ALPS were in and out of hospitals and emergency rooms regularly, but a couple of sirolimus pills a day, while not a cure, enables them to live healthier, better, longer lives.
- Repurposing of a device called the portable neurostimulator (PoNS), originally used to treat balance disorders, to help people with multiple sclerosis regain and improve physical function, including walking and arm movement. Additional research showed that the device, which stimulates the tongue with electrical signals, can also help people with stroke, Parkinson's, and traumatic brain injury.
- Repurposing of the drug thalidomide, a treatment for leprosy, for treating multiple myeloma, a blood cancer. Given early, the drug can prevent people with asymptomatic multiple myeloma from progressing to symptomatic disease.
- Repurposing of the generic tuberculosis vaccine BCG to help patients with type 1 diabetes. CWR-supported researchers showed that just two doses of BCG can maintain stable and long-term correction of blood sugars for people with type 1 diabetes. Clinical trials of BCG, including in children, are ongoing.

CWR has funded over 100 projects, 33 of which are ongoing today, says Goodman. Like many philanthropic funders working in health and medicine, CWR aims to make small but catalytic investments in the form of seed funding for pilot and other clinical studies that researchers need to obtain larger grants, perhaps from the NIH or other public sources, to support the work needed to get the therapies to patients. Goodman said CWR's approximately \$7 million in repurposing research grants have enabled its researchers to raise another \$67 million in follow-on funding from the NIH and other sources.

Such repurposing research seems to be an area tailor-made for wider philanthropic support. Philanthropy can be less concerned with the financial returns on its investments, and thus less exclusively focused on the discovery of new and potentially money-making drugs and other treatments. As a result, philanthropy is in a position to give researchers and universities the support they need to find solutions for rare common diseases, or in less profitable marketplaces that may not provide many funding opportunities. In the process, they might make a huge difference in the lives of people who have few other avenues for hope.