Philanthropy’s Role in Catalyzing New Uses for Approved Treatments

Barbara Goodman* and Clare Thibodeaux, PhD

Cures Within Reach, Chicago, Illinois, USA

*Correspondence to: Barbara Goodman, Cures Within Reach, Chicago, 134 N LaSalle, #1130 Illinois, USA.
E-mail: barbara@cureswithinreach.org

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ABSTRACT

Foundations, nonprofits, non-governmental organizations, and other philanthropic organizations are frequently involved in supporting drug development. Cures Within Reach, a US-based nonprofit leader of testing already approved therapies for new indications in unsolved diseases, has been providing seed funds for first-in-human clinical trials for over 15 years. Philanthropy plays a critical role in risk mitigation of investigator-initiated trials, funding proof-of-concept, pivotal clinical trials as well as later-stage trials. Efforts such as these are complementary to the pharmaceutical industry’s portfolio life cycle management and complementary to governments’ funding of translational medicine initiatives. Results from smaller, earlier trials provide the data needed for industry, government agencies, and others to fund the larger confirmatory trials. Together, these data build evidence for publication, allow clinicians to make their own decisions with their patients (off-label use), or support regulatory approval by the regulatory agencies. In addition, these efforts are not a question of commercial opportunity via intellectual property versus charity, as these are not mutually exclusive—and often are not yet clearly known at earlier clinical trial stages.

KEYWORDS

clinical trials; drug repurposing; repurposing; nonprofit; philanthropy; patient impact; off-label.

SUMMARY

There are many philanthropies that fund clinical repurposing trials, the testing of already approved therapies for new indications. This includes disease-specific nonprofits and non-governmental organizations (NGOs), such as the Alzheimer’s Drug Discovery Foundation, Anticancer Fund, Leukemia & Lymphona Society, and others. This also includes disease-agnostic nonprofits and NGOs, such as Cures Within Reach (CWR), BIO Ventures in Global Health, Drugs for Neglected Diseases initiative and others. Finally, there are government and government-connected groups, such as the US National Institutes of Health’s NCATS, RePo4EU, REMEDI4ALL, SIMPATHIC consortium, and others. Each of these plays an important role in finding and funding these clinical trials, with the goal of bringing new treatment options to patients with unmet medical needs using approved treatments.

In order for any drug repurposing treatment to reach patients everywhere (not just a few patients participating in a clinical trial) and for these treatments to become a first-line treatment option or standard of care accepted by clinicians everywhere, positive data from randomized controlled trials (RCTs) are critical. Philanthropy often plays a key role in funding these RCTs by

a) mitigating risk;

b) supporting these before the end result is known; and

c) often focusing on underfunded areas of need.

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First, philanthropy’s role in mitigating risk includes supporting proof-of-concept / pivotal funding and investigator-initiated trials; when artificial intelligence / machine learning (AI/ML) may provide hypotheses or reduce pre-clinical needs; and when real world evidence (RWE) and/or natural history registries may provide data and case reports:

- A successfully completed phase IIA trial at the Obafemi Awolowo University in Nigeria: Treating Tuberculosis with the Lipid Lowering Drug Atorvastatin in Nigeria. This is CWR’s newest success story. Tuberculosis (TB) remains a global health threat, causing over a million deaths annually. Current treatments require months-long antibiotic regimens, contributing to emerging drug resistance. In early 2023, Dr. Olanisun Olufemi Adewole at the Obafemi Awolowo University in Nigeria published results of his pivotal phase IIA clinical trial, adding atorvastatin to the standard of care for TB patients, showing that this safe, generic cholesterol-lowering drug may accelerate the clearance of the TB bacteria in patients when added to standard treatments. With this success, Dr. Adewole raised over $300,000 in follow-on funding to support a larger confirmatory study. The $50,000 pivotal trial funded by CWR provided data to support more than $300,000 in follow-on funding.

- An ongoing clinical trial now at the Children’s National Research Institute: Using Steroids to Improve Rhabdomyolysis Outcomes in Pediatric Patients. Due to case reports and RWE, the institution considers this treatment to be a first-line treatment option for their patients. This RCT will provide the clinical evidence to go along with the RWD for this generic drug to potentially be used off-label.

Second, philanthropy’s role in supporting these clinical trials before the end result is known includes on-patent and/or off-patent issues, and whether there is a regulatory approval opportunity with label change or if off-label use is the goal. Philanthropy-funded clinical repurposing trial examples are as follows:

- A successfully completed clinical trial at the Children’s Hospital of Philadelphia: Repurposing a Transplant Drug Saves Kids with autoimmune lymphoproliferative syndrome (ALPS), a Rare but Deadly Blood Disorder. This pediatric trial led to sirolimus, a generic drug, becoming a first-line treatment option for ALPS patients everywhere, used off-label in the United States, Europe, and elsewhere.

- An ongoing clinical trial now at St. Jude Children’s Research Hospital: Repurposing a Blood Cancer Drug to Treat an Immune Disorder (HLH) in Children. This pediatric trial is testing a currently on-patent drug.

Finally, philanthropy’s role in focusing on underfunded areas of need is critical in underfunded areas of pediatrics, in rare diseases; within low and lower-middle income countries (LMICs); and to impact health disparities. Philanthropy-funded clinical repurposing trial examples are as follows:

- A successfully completed clinical trial at the KEMRI Wellcome Trust: Testing the Safety of a Metal Poisoning Drug to Treat Snakebite in Kenya. This LMIC-based trial is also an example of philanthropy’s role of support before the end result is known.

- An ongoing clinical trial now at The University of Texas MD Anderson Cancer Center: Adding Metal Detoxification Drugs to Improve Childhood Acute Myeloid Leukemia Outcomes. This pediatric trial is not only in an underfunded area of clinical research, but CWR is supporting the addition of this pediatric arm to an already ongoing adult clinical trial.

Note: There continues to be varying definitions used across stakeholders of “repurposing.” For CWR, drug repurposing is finding a new disease indication for a drug already approved for human use by the US Food and Drug Administration or the European Medicines Agency or any regulatory agency in one disease, irrespective of whether the same or different dose or delivery method is used. This differs from what CWR considers “repositioning” or “rescue” which is finding a new disease indication for a human-safe, still-in-the-pipeline compound that has never reached a regulatory agency.

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REFERENCES