"It is abundantly clear that our success in obtaining NIH funding was due to the preliminary data obtained through Cures Within Reach's support. I shall be forever grateful for the support in getting this work started."

Dr. Michael Levine Children's Hospital of Philadelphia

2024 ANNUAL REPORT



Cures Within Reach finds and funds clinical repurposing trials: testing approved therapies to fast-track safe and effective treatments for unsolved diseases.

Our patient-first approach focuses on generating early clinical data to de-risk and catalyze follow-on funding by others for larger, confirmatory trials, building the clinical evidence required for regulatory approval or off-label use.

2024 Achievements

- \$29 million raised for our funded research(ers) in follow-on funding
- 13 new trials started with our funding in infectious diseases, neurology, oncology, rare diseases and more
- 7 trials completed
- 12 trials selected to start in 2025 focused in oncology, pediatrics, veterans, women's health and more
- 48 ongoing clinical trials in
 35 diseases at 36 institutions
 in 11 countries at year-end

Our Newest Success Stories

Blood Cancer: University of Pittsburgh received over \$3.4 million from the FDA plus other private funding to expand a CWR-funded Acute Myeloid Leukemia and Myelodysplastic Syndrome trial from a single site to multi-site with much larger enrollment goals.

Snakebite: Kenya-based KEMRI Wellcome Trust Research Programme's Phase 1 clinical trial, treating snakebite in Kenya with unithiol, completed in 2023. Positive data helped lead to \$5.4 million from Open Philanthropy to support a Phase 2B clinical trial in Brazil and Ghana.

In these two successes alone, \$110,000 from CWR helped de-risk and catalyze over \$9 million in follow-on funding

Together We're Making Real Patient Impact





Diseases Researched



Trials Funded



Institutions Funded



Success Stories \$128 million



Follow-On Raised from Trials To Our Stakeholders:

From all of us at Cures Within Reach, it is my pleasure to share this 2024 summary. We are energized by your continued commitment to our mission, and we are passionate about our rapid impact on patients with unsolved diseases through repurposing: testing already approved therapies for new uses. We look forward to helping even more patients in 2025.

Please help us spread the word to impact more patients and more unsolved diseases by sharing this Annual Report and connecting us to funders and other partners in your network.

2024 Highlights:

- A record \$29 million raised for our funded research(ers) in follow-on funding
- Grew our ongoing portfolio to 48 clinical trials
- Engaged nearly 100 volunteer Grant Review Committee members
- Selected our first clinical trials in Malawi and Mali

In addition to Open Philanthropy expanding our partnership for 2025 – 2026, we are thrilled to begin our partnership with Chan Zuckerberg Initiative to validate AI-driven drug – disease repurposing with an AI-related Request for Proposals coming in 2025. Both partners are also helping continue our expansion of community engagement funding alongside our trials: in 2025 we'll include funding for community engagement in our low and lower-middle income country-based and AI validation trials.

We are grateful for the physician scientists who share their repurposing ideas with us and move this work forward, the patients who participate in these clinical trials and our funding partners, community members and stakeholders. While this Annual Report features a few of our partners, we are incredibly grateful for all who contributed to making 2024 a success.

Together we can improve the quality and length of life for patients faster through repurposing. They are counting on us.

Barbara Goodman President & CEO



OUR MISSION

Cures Within Reach **tests approved therapies for unsolved diseases** by leveraging the speed, safety and cost-effectiveness of repurposing: driving more treatments to more patients more quickly.

We find and fund proof-of-concept clinical trials of drugs, devices and other treatments that, once successful, **de-risk and catalyze follow-on funding** for the larger, confirmatory trials, **building the clinical evidence** required for regulatory approval or to support off-label use by patients everywhere.

OUR IMPACT

Creating Patient Impact: Staying Focused on "Why"

Cures Within Reach and our funding partners are passionate about impacting patients with unsolved diseases by developing "new" treatments using already approved therapies, known as repurposing. As a disease-agnostic, geography-agnostic, risk-mitigating catalyzer, our current portfolio covers a range of disease areas:

- 46% rare diseases
- 40% oncology
- 21% neurology
- 10% infectious diseases
- 10% ear nose throat
- 17% other

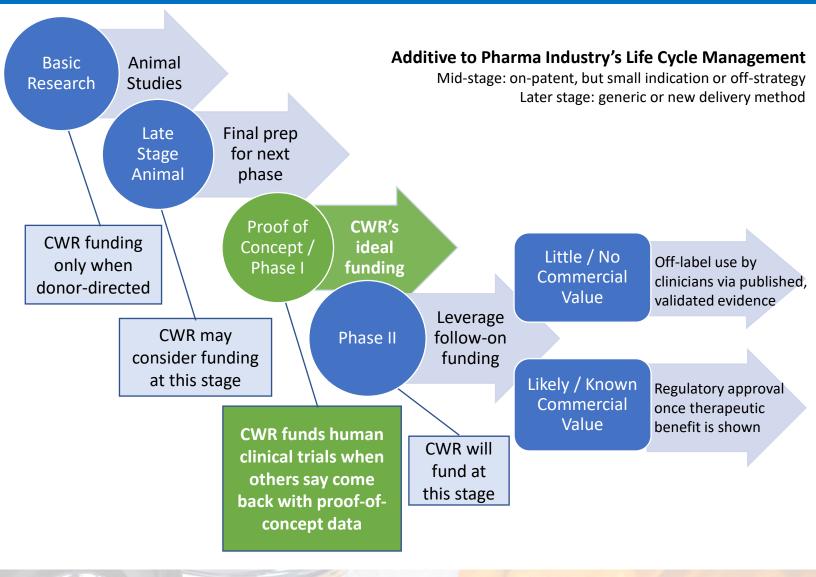
- 74% adult; 20% pediatric; 7% combination
- 83% drug; 17% device/other
- 75% US-based; 25% outside US
- 96% clinical; 4% pre-clinical as of 12.31.2024

We are grateful for the support of our donors, sponsors, funding partners, funded researchers/institutions and patients in making these exciting clinical trials possible.

FUNDING AT THE KEY MOMENT

What we solve: more viable treatment options for patients in need

We provide **seed funds to de-risk and unlock a catalytic effect**, building the **clinical evidence required** for regulatory approval or off-label use by patients everywhere



Known as 'medical repurposing' by researchers, clinicians, patients and nonprofits, it is also considered indication expansion or label extension

DONOR-ADVISED IMPACT PHILANTHROPY

Join us! Spread the word to help bring treatment options to the millions who suffer with unsolved diseases

Philanthropy Focused on Speed-to-Impact

Together with our donor partners, we are de-risking new treatment options for patients, supporting researchers with clinical repurposing ideas to quickly reach patients everywhere.

- Donor partners choose their desired level of engagement in the design, search and selection process our experienced team customizes the approach with you
- Grants starting at \$65,000 and up to \$250,000 can support a small, proof-of-concept human clinical trial or an expansion of an ongoing trial

Donor Goal		Involvement in Selection	2024 Results	
GOLDMAN PHILANTHROPIC In medicine	Build pipeline to impact blood cancers	Foundation's science advisor engaged with our 2-stage selection process	Over \$4 million follow-on raised in 2024; portfolio of 7 ongoing trials plus 1 starting in 2025	
Becky and Lester Knight	Build broad pipeline for Meniere's disease and related disorders	Very involved throughout our 2-stage selection process	Portfolio of 11 ongoing or completed trials	
Open Philanthropy	Tackle high disease burden in LMICs with readily available generics	Involved in up-front RFP design only	5 LMIC-based ongoing trials plus 6 more starting in 2025, and 16 to be selected in 2025 – 2026	

Examples of Donor Partnerships:

Our 20-year successful track record in repurposing is complementary to lengthy *de novo* therapy research and has the potential to significantly reduce the time needed to reach as many patients as possible.

Our Value Proposition:

- An expert team providing bespoke services designed to meet your needs
- Technical expertise in finding, selecting, de-risking and managing our funded clinical trials
- Access to our vast global research network
- An efficient, proven Request for Proposal (RFP) process, including volunteer, external Grant Review Committee members (representing research, industry, clinicians, nonprofits, philanthropy, government and the patient/community voice) that support selection and strengthen trial protocols
- Project and financial progress reports both during and after the clinical trials

Donor-advised funding can support a specific disease, geography, patient population or other theme of a donor's choosing, such as:

- Veterans
- Pediatrics

- Health equity, including women's health and health disparities
- Specific disease areas or geographies

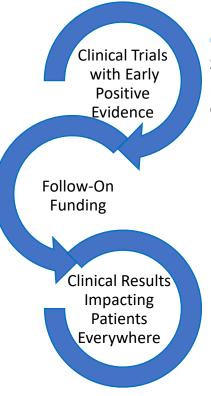
2024 RESULTS

12 New Clinical Trials Selected for Funding

During 2024, we had five funding opportunity Request for Proposals (RFPs): Veterans, Blood Cancers, US Health Equity, LMIC-based researchers and Chicago-based trials. In addition to the 12 new clinical trials selected, these RFPs resulted in:

- 15% average funding rate
- More than 100 Letters of Intent submitted
- Nearly 100 volunteer Grant Review Committee members supported our two-stage process

Measuring Our Success



Our funded research moved forward with follow-on funding, publications of trial results and presentations at conferences, led by researchers as well as by commercial entities.

Clinical Trials with Early Positive Evidence:

Symptoms improved and/or early positive evidence in retinitis pigmentosa, PTSD, a rare pediatric infection, blood cancer and others, including publications/presentations

Follow-On Funding:

A record \$29 million in new funding was reported in 2024 based on our funded research for clinical trial expansion, next phase trials or related clinical trials in several cancers, infectious diseases, PTSD, snakebite and more

Clinical Results Impacting Patients Everywhere:

Increased reimbursement of the PoNS device for multiple sclerosis and stroke, and based on publications, treatments from our trials are being used off-label in rare, infectious and neurodegenerative diseases

NEW CLINICAL TRIALS STARTED IN 2024

13 trials at 11 institutions in 13 diseases in 5 countries

Disease Area	Institution	Title	Туре	Community
Ear Nose Throat	Yale University	Investigating the Effects of Blocking Immune Disease Pathways in Meniere's Disease	Drug, Preclinical	General
Ear Nose Throat	University Hospital of Munich	Treating Vestibular Neuritis Symptoms with a Thyroid Hormone	Drug, Adult	General
Infectious Disease	Université d'Abomey -Calavi	Adding Beta-lactam Antibiotics to Shorten Buruli Ulcer Disease Therapy in Benin	Drug, Adult and Pediatric	Pediatric, Health Equity, LMIC
Inflammatory Disease	University of Illinois Chicago	Treating Chronic Sinus Inflammation with a Probiotic Sinus Wash	Drug, Adult	Chicago, Health Equity
Inherited Disorder	Global Health Uganda Ltd	Adding Zinc to Reduce Sickle Cell Anemia- related Infections in Ugandan Children	Nutraceutical, Pediatric	Pediatric, Health Equity, LMIC
Maternal Health	Busitema University	Using Sodium Bicarbonate to Reduce Maternal / Fetal Death from Acidosis During Obstructed Labor in Uganda	Drug, Adult and Pediatric	Pediatric, Health Equity, LMIC
Neurology	Virginia Commonwealth University	Daily Rotigotine Patch to Enhance Behavioral Therapy for Cocaine Use Disorder Recovery in Veterans	Drug, Adult	Neurology, Veterans, Mid-Atlantic
Neurology	Edward Hines Jr VA Hospital and CARES	Improving Cognitive Function in Veterans with Atypical Parkinsonism with rTMS Brain Stimulation	Device, Adult	Veterans, Neurology, Chicago
Neurology	University of Illinois Chicago	Adding an Antihistamine to Improve Outcomes in Depression	Drug, Adult	Neurology, Health Equity, Chicago
Oncology	University of Colorado, Anschutz Medical Campus	Reducing Side Effects of Breast Cancer Treatments with Oxytocin	Drug, Adult	Oncology, Health Equity
Oncology	University of Illinois Chicago	Glycyrrhizin in Prostate Cancer : A Window-of- Opportunity Clinical Trial	Nutraceutical, Adult	Oncology, Health Equity, Chicago
Oncology	University of Pittsburgh	Reducing Immune-Related Adverse Events by Adding Oral FMT to Solid Tumor Cancer Treatment	Other, Adult	Oncology, Health Equity
Oncology, Rare	Muhimbili University Health and Allied Sciences	Testing a Generic Drug Combination to Treat AML in Tanzania	Drug, Adult	Oncology, Rare, Health Equity, LMIC

ONGOING CLINICAL TRIALS IN 2024 35 trials at 31 institutions in 32 diseases in 8 countries

ongoing trials: page 1 of 2

Disease Area	Institution	Title	Туре	Community
Dermatology	University of Antananarivo	Effect of Methotrexate as a Treatment for Moderate to Severe Atopic Dermatitis in Madagascar	Drug, Adult	Health Equity, LMIC
Ear Nose Throat	House Institute Foundation	Reducing Vertigo and Hearing Loss in Meniere's Disease by Repurposing a Common Allergy Drug	Drug, Adult	General
Ear Nose Throat	University Hospital of Munich	Identifying a Potential Biomarker and Repurposed Migraine Treatment in Meniere's Disease	Drug, Adult	General
Immune Disorder, Rare	St. Jude Children's Research Hospital	Repurposing a Blood Cancer Drug to Treat an Immune Disorder (HLH) in Children	Drug, Pediatric	Rare, Pediatric
Immune Disorder, Rare	Cincinnati Children's Hospital Medical Center	Repurposing a Rare Disease Treatment for the Immune Disorder Eosinophilic Esophagitis	Drug, Adult	Rare, Health Equity
Infectious Disease	George Washington University / Universidad El Bosque	Repurposing a Flu Treatment for Severe Dengue Patients in Colombia	Drug, Pediatric	Pediatric, Mid- Atlantic, LMIC, Health Equity
Infectious Disease	University of Chicago	Can Vitamin D Reduce the Burden of COVID-19 in Chicago?	Nutra- ceutical, Adult	Health Equity, Chicago
Infectious Disease, Rare	International Centre for Diarrhoeal Disease Research, Bangladesh	Assessing Safety and Efficacy of Oral Ivermectin in Post Kala-azar Dermal Leishmaniasis Treatment	Drug, Adult	Rare, Health Equity, LMIC
Inflamma- tory Disease	SIDS Hospital and Research Centre	Combining the Cholesterol-Lowering Drug Simvastatin with N-Acetylcysteine for Chronic Pancreatitis in India	Drug, Adult	Health Equity, LMIC
Inherited Disorder, Rare	University of Chicago	Testing a Low-Dose Anti-inflammatory Tocilizumab for Acute Chest Syndrome in Sickle Cell Disease	Drug, Adult and Pediatric	Rare, Pediatric, Health Equity, Chicago
Neurology	University of California, Los Angeles	Testing a Repurposed Device to Improve the Treatment of Chronic Pelvic Pain in Women	Device, Adult	Neurology, Health Equity
Neurology	Edward Hines Jr VA Hospital and CARES	Using an Approved Depression Treatment to Restore Cognitive Function in Veterans with Mild TBI and PTSD	Device, Adult	Neurology, Veterans, Chicago
Neurology	Advocate Health Care	Testing a Low-Dose of the Hypertension Drug Clonidine for PTSD in Veterans	Drug, Adult	Neurology, Veterans
Neurology	University of Zambia	Testing an Antihypertensive Drug as an Add- On Therapy for Drug-Resistant Epilepsy Patients in Zambia	Drug, Adult	Neurology, Health Equity, LMIC
Neurology	University of North Carolina at Chapel Hill	Estrogen to Prevent Chronic Pain and PTSD Following Sexual Assault	Drug, Adult	Neurology, Health Equity
Neurology, Cardio	University of California San Francisco	Testing the Epilepsy Drug Perampanel to Prevent Seizures After Cardiac Arrest Resuscitation	Drug, Adult	Neurology, Health Equity
Neurology, Infectious Disease	Emory University School of Medicine	Addressing Chronic Pain in HIV Patients with an Approved Addiction Treatment	Drug, Adult	Neurology, Health Equity

ONGOING CLINICAL TRIALS IN 2024 35 trials at 31 institutions in 32 diseases in 8 countries

ongoing trials: page 2 of 2

Disease Area	Institution	Title	Туре	Community
Oncology	University of Illinois Chicago	Using a Generic Drug Combination in Early- Stage Hormone Receptor Positive Breast Cancer Patients	Drug, Adult	Oncology, Health Equity, Chicago
Oncology, Rare	University of Pittsburgh	Investigating a Rare Disease Drug to Treat AML and MDS Patients with Post-Stem Cell Transplant Relapse	Drug, Adult	Oncology, Rare
Oncology, Rare	The University of Texas MD Anderson Cancer Center	Adding Metal Detoxification Drugs to Improve Childhood AML and Other Blood Cancer Outcomes	Drug, Pediatric	Oncology, Rare, Pediatric
Oncology, Rare	Emory University School of Medicine	Combining Approved Drugs for Alcoholism and Cancer to Treat Diffuse Large B-cell Lymphoma	Drug, Adult	Oncology, Rare
Oncology, Rare	Brown University / Rhode Island Hospital	Repurposing the Prostate Cancer Drug Degarelix to Treat Bladder Cancer	Drug, Adult	Oncology, Rare, Health Equity
Oncology, Rare	City of Hope	Repurposing Venetoclax and Glofitamab to Improve Patient Response in Mantle Cell Lymphoma	Drug, Adult	Oncology, Rare, Health Equity
Oncology, Rare	Colorado State University	Evaluating an Approved Drug Combination Plus Immunotherapy in Canine Metastatic Osteosarcoma	Drug, Preclinical	Oncology, Rare
Oncology, Rare	University of Michigan	Reducing CAR T-cell Therapy Complications in Relapsed Lymphoma Patients by Adding Natalizumab	Drug, Adult	Oncology, Rare
Oncology, Rare	Brigham Young University	Treating Anemia in Myelofibrosis with Nelfinavir	Drug, Adult	Oncology, Rare
Oncology, Rare	Children's Hospital of Philadelphia	Preventing Relapse After Bone Marrow Transplant in Pediatric Acute Lymphoblastic Leukemia with a Personalized Treatment	Drug, Pediatric	Oncology, Rare, Pediatric, Mid-Atlantic
Oncology, Rare	University of California, Irvine	Treating a Group of Rare Blood Cancers, Myelo- proliferative Neoplasms, with N-acetylcysteine	Drug, Adult	Oncology, Rare
Oncology, Rare	Loyola University Medical Center	Repurposing the Blood Pressure Drug Minoxidil for the Treatment of Recurrent Chemo-resistant Ovarian and Other Gynecological Cancers	Drug, Adult	Oncology, Rare, Chicago
Oncology, Rare	The University of Texas MD Anderson Cancer Center	Enhancing Treatment Response in Recurrent / Metastatic Osteosarcoma with Hydroxychloroquine	Drug, Pediatric	Oncology, Rare, Pediatric, Health Equity
Oncology, Rare	University College Hospital Ibadan	Adding the Antibiotic Clofazimine to Chemotherapy for Triple Negative Breast Cancer in Nigeria	Drug, Adult	Oncology, Rare, Health Equity, LMIC
Oncology, Rare	University of Illinois Chicago	Repurposing a Diagnostic Device to Address Health Disparities in Esophageal Cancer Screening and Outcomes	Device- Diagnostic, Adult	Oncology, Rare, Health Equity, Chicago
Rare	Children's National Research Institute	Using Steroids to Improve Outcomes in Rhabdomyolysis in Pediatric Patients	Drug, Pediatric	Rare, Pediatric, Health Equity, Mid-Atlantic
Rare, Liver Disease	Vietnam National Children's Hospital / Big Leap Research	Improving Outcomes for the Rare Liver Disease Biliary Atresia With a Cancer Drug in Vietnam	Drug, Pediatric	Rare, LMIC, Pediatric, Health Equity
Rare, Vascular	Stanford University	Treating Vascular Malformations Using the Cancer Drug Trametinib	Drug, Pediatric	Rare, Pediatric

CLINICAL TRIALS SELECTED IN 2024

12 trials expected to start in 2025

Disease Area	Institution	Title	Туре	Community
Gastro- intestinal	KEMRI – Kenya Medical Research Institute	Repurposing Lactoferrin and Lysozyme to Address Environmental Enteric Dysfunction in Kenya	Nutraceutical, Pediatric	Pediatric Health Equity, LMIC
Immune Disorder	University of Chicago	Non-invasive Monitoring to Detect Food Allergic Reactions	Device, Adult and Pediatric	Pediatric, Health Equity, Chicago
Infectious Disease	International Centre for Diarrhoeal Disease Research, Bangladesh	Assessing a Combination Therapy for Helminthiasis in Bangladeshi Children	Drug, Pediatric	Pediatric, Health Equity, LMIC
Infectious Disease	University of Science, Technology and Engineering of Bamako	Comparing Two Drugs to Treat P. Malariae in Mali	Drug, Adult and Pediatric	Pediatric, Health Equity, LMIC
Maternal Health	Baylor Foundation Malawi	Preventing Prematurity with Xylitol in Malawi	Nutraceutical, Adult and Pediatric	Pediatric, Health Equity, LMIC
Metabolic Disorder	University of Nigeria Teaching Hospital	The Effect of Metformin on Weight Loss in Non-Diabetic, Obese Nigerians	Drug, Adult	Health Equity, LMIC
Neurology	University of California, Davis	Using Fresh Frozen Plasma to Improve TBI Outcomes	Biologic, Adult	Neurology, Veterans
Neurology	Providence VA Medical Center and Ocean State Research Institute	Treating PTSD with Stellate Ganglion Block via TMS Neuromodulation	Device, Adult	Neurology, Veterans
Neurology	University of Illinois Chicago	Replenishing Serotonin to Relieve Cognitive Symptoms of Long COVID	Nutraceutical, Adult	Neurology, Chicago
Oncology	University of Lagos	Treating High-grade Cervical Intraepithelial Neoplasia with a Skin Cancer Drug in Nigeria	Drug, Adult	Oncology, Health Equity, LMIC
Oncology, Dermatology	The University of Texas Health Science Center at San Antonio	Treating Radiation-Related Dermatitis with Topical Esomeprazole	Drug, Adult	Oncology, Health Equity
Oncology, Rare	Washington University in St. Louis	Improving Access to CAR T with Radiation in Relapsed / Refractory Multiple Myeloma	Device, Adult	Oncology, Rare

"By working with Cures Within Reach to fund LMIC-led medical research, we can create a **more equitable global health ecosystem**. When we empower researchers and clinicians in their own communities, we not only **accelerate medical breakthroughs** but also build a research infrastructure to serve historically underserved populations."



CLINICAL TRIALS COMPLETED IN 2024

7 trials at 6 institutions in 6 diseases in 2 countries

Disease Area	Institution	Title	Туре	Community
Autoimmune	Massachusetts General Hospital	Repurposing a Vaccine for Type I Diabetes	Vaccine, Adult	General
Ear Nose Throat	Dent Neurologic Institute	Combining an Anti-epileptic Drug with an Anti-anxiety Drug to Treat the Inner Ear Disorder Meniere's Disease	Drug, Adult	General
Ear Nose Throat	ibs.GRANADA	Searching for Pharmacogenetic Targets in Meniere's Disease Patients	Diagnostic, Preclinical	General
Infectious Disease, Ear Nose Throat, Rare	University of Utah	Repurposing the Antiviral Drug Valganciclovir to Treat Cytomegalovirus-Induced Hearing Loss	Drug, Pediatric	Rare, Pediatric
Neurology	Rush University Medical Center	Enhancing PTSD Treatment For Veterans Via Prebiotic Supplementation	Nutraceutical, Adult	Neurology, Veterans, Chicago
Oncology, Rare	University of Michigan	Repurposing Old Drugs As New Therapies for Metastatic Thyroid Cancer	Drug, Adult	Oncology, Rare
Ophthalmology, Rare, Inherited Disorder	University of Michigan	Testing a Generic Malarial Drug in a Rare Ophthalmic Condition, Retinitis Pigmentosa	Drug, Adult	Rare

GRANT REVIEW COMMITTEE

One of Our Secrets? Grant Review Committee Members Drive Our Success

In 2024, these 96 volunteers, representing research, clinicians, industry, nonprofits, philanthropy, government and the patient/community voice to provide both scientific and patient / community perspectives, **helped us select 12 new clinical trials**. We are so grateful for the time, effort and insights that these Grant Review Committee members provide.

These volunteers:

- Reviewed over 100 LOIs and over 25 full proposals to help us select 12 clinical trials for our funding
- For our LMIC initiative alone, represented 18 countries on 4 continents
- Strengthened our selected clinical trials with their expertise and suggestions

Learn more about and join our Grant Review Committee: visit <u>https://bit.ly/cwrreviewers</u>.

"All the submissions that I reviewed were especially promising and worthy of advancement. Very exciting to see the potential." *A Grant Review Committee member* for LMICs representing an international NGO

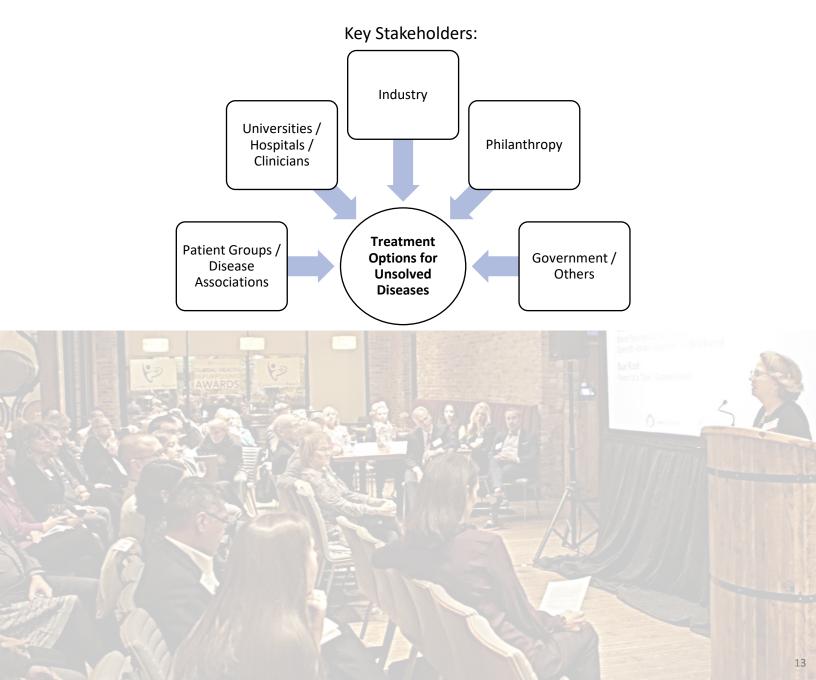
ADDING VALUE THROUGH OUR COMMUNITIES

In 2024, we continued building our nine communities that align with our stakeholders' strategic interests:

- Disease-specific: Rare Diseases, Oncology and Neurology (including neurodegenerative, mental health, pain)
- Populations: Pediatrics, Veterans/Military and Health Equity (impacting women's health, health disparities and underrepresented researchers)
- Geographic: Chicago, Mid-Atlantic and Low and Lower-Middle Income Countries

Most of our trials overlap communities. For example, our ongoing clinical trial in pediatric osteosarcoma impacts our Pediatrics, Oncology, Rare Diseases and Health Equity communities.

These virtual communities engage key stakeholders with an aligned goal: bring viable treatment options to patients with unsolved diseases wherever they live.



VETERANS / MILITARY

Our growing Veterans Community includes **10 clinical trials**: 4 ongoing, 4 completed (in PTSD, depression, TBI and nerve damage) plus 2 more starting in 2025. We began funding 2 clinical trials in 2024:

- Improving Cognitive Function in Veterans with **Atypical Parkinsonism** at Edward Hines Jr. VA Hospital in Illinois
- Combining a Drug with Therapy for Veterans with **Cocaine Addiction** at Virginia Commonwealth University

We also selected **2 more clinical trials** that will start in 2025:

- Using Fresh Frozen Plasma to Improve **TBI** Outcomes at University of California, Davis
- Treating PTSD with Stellate Ganglion Block via TMS Neuromodulation at Providence VA Medical Center and Ocean State Research Institute



Of our 48 ongoing trials at year-end, 4 are in Veterans / Military

Philanthropic Partners:



Arthur Thomas Alm Trust





Anonymous

PEDIATRICS

Our growing Pediatrics Community now has **25 clinical trials**: 13 ongoing, 7 completed trials plus 5 more selected in 2024.

The 5 new pediatric trials expected to start in 2025 include:

- Preventing Prematurity with Xylitol in Malawi at Baylor Foundation Malawi
- Assessing a Combination Therapy for **Helminthiasis** in Bangladeshi Children at International Center for Diarrheal Diseases Research, Bangladesh
- Repurposing Lactoferrin and Lysozyme to Address **Environmental Enteric Dysfunction** in Kenya at KEMRI Kenya Medical Research Institute
- Non-invasive Monitoring to Detect Food Allergic Reactions at University of Chicago
- Comparing Two Drugs to Treat **P. Malariae** in Mali at University of Science, Technology and Engineering of Bamako

Inaugural Philanthropic Partner:



Of our 48 ongoing trials at year-end, 13 are in Pediatrics or both Adult and Pediatrics

US HEALTH EQUITY: IMPACTING HEALTH DISPARITIES OR UNDERREPRESENTED PATIENTS OR RESEARCHERS

US-Based Health Equity Trials

In 2024, we continued building our growing portfolio in US-based health equity. **Three new trials** were selected in 2024 and will start in 2025:

- Non-invasive Monitoring to Detect
 Food Allergic Reactions at the University of Chicago
- Replenishing Serotonin to Relieve Cognitive Symptoms of Long COVID at the University of Illinois Chicago
- Treating Radiation-Related
 Dermatitis with Topical
 Esomeprazole at The University of
 Texas Health Science Center at San
 Antonio



Of our 48 ongoing clinical trials at year-end, 19 are US-based health equity

WOMEN'S HEALTH: GROWING IMPACT IN THE US AND LMICS

The historically low funding of women's health by the NIH, industry and overall has led to both significant gaps in knowledge and limited treatment options for conditions that disproportionately affect women. This is exacerbated by exclusion of women from clinical trials, often due to a pregnancy risk, and has led to a lack of data on how treatments affect women differently than men.

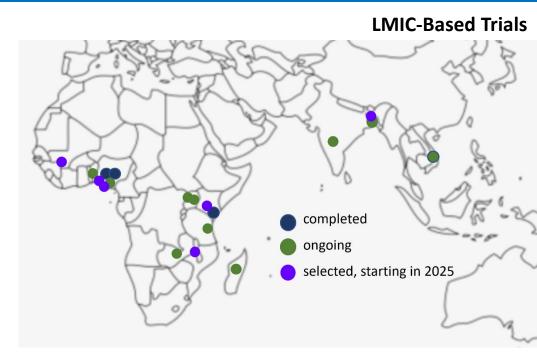
Our fast-growing Women's Health portfolio, within our Health Equity efforts broadly, includes clinical trials in breast cancer (3 different trials), ovarian cancer, pre-cervical cancer, maternal health (2) and chronic pain (2). Of our 48 ongoing trials at year-end, 7 are in Women's Health

LOW / LOWER-MIDDLE INCOME COUNTRIES: IMPACTING LMIC-BASED PATIENTS WITH CLINICAL TRIALS LED BY LMIC-BASED RESEARCHERS

In 2024, our funding supported 4 new LMIC-based trials in maternal health, infectious disease, inherited disorders and oncology, plus we selected 6 more clinical trials to start in 2025. Our 2024 LMIC RFP included:

- A record 50 Letters of Intent submitted from 20 countries
- 40 Grant Review Committee members from 18 different countries

With Open Philanthropy's renewed partnership, we will select 16 more LMIC-based trials in 2025 and 2026, which, for the first time, will include funding of community engagement alongside these trials.



Of our 48 ongoing trials at year-end, 10 are LMIC-based

The 6 new clinical trials selected to start in 2025:

- **Preventing Prematurity** with Xylitol in Malawi at Baylor Foundation Malawi
- Assessing a Combination Therapy for Helminthiasis in Bangladeshi Children at International Center for Diarrheal Diseases Research, Bangladesh
- Repurposing Lactoferrin and Lysozyme to Address
 Environmental Enteric Dysfunction in Kenya at
 KEMRI Kenya Medical Research Institute
- Treating High-grade Cervical Intraepithelial Neoplasia with a Skin Cancer Drug in Nigeria at University of Lagos
- The Effect of Metformin on Weight Loss in Non-Diabetic, Obese Nigerians at University of Nigeria Teaching Hospital
- Comparing Two Drugs to Treat P. Malariae in Mali at University of Science, Technology and Engineering of Bamako

Lead Partner:



A New Success Story: Snakebite

CWR's second LMIC-based clinical trial funded, Kenya-based KEMRI Wellcome Trust Research Programme's Phase 1 clinical trial treating snakebite in Kenya with unithiol, completed in 2023. Positive data led to **\$5.4**

million from Open Philanthropy to support a Phase 2B clinical trial in Brazil and Ghana, investigating unithiol and another drug as potential treatments for snakebites. In these countries, snakebites are potentially fatal and require urgent treatment. If successful, both medicines would represent inexpensive treatments that could be quickly deployed in rural settings.

This \$50,000 pivotal trial provided data to support \$5.4 million in follow-on funding.

RARE DISEASES: A PIONEER IN HOPE FOR RARE PATIENTS

While we are disease-agnostic, since our inception nearly 20 years ago, Rare Diseases has always been and remains a clear focus of our efforts: roughly 50% of our portfolio impacts the thousands living with a rare disease without treatment options. Our fast-track approach of derisking repurposed therapeutic options for rare diseases accelerates their availability by building the clinical data required for regulatory approval or, more frequently for rare diseases, through off-label use.

Of our 48 ongoing trials at year-end, 22 are in Rare Diseases

A New Success Story: Acute Myeloid Leukemia

In 2024, the University of Pittsburgh received over \$3.4 million from the FDA plus other private funding to expand a CWR-funded Acute Myeloid Leukemia and Myelodysplastic Syndrome trial from one site into a larger trial with much higher enrollment goals. "Cures Within Reach is the first external grant mechanism believing in our study concept and proposal," said Dr. Ito. "Thanks to CWR's support, we can now expand the trial to a multicenter, well-designed trial."

The \$75,000 pivotal trial provided data to support more than \$4 million in follow-on funding.

AI VALIDATION AND OTHER DEVELOPMENTS

In 2024, we announced a new AI initiative planned for 2025. Funded by Chan Zuckerberg Initiative, we will fund trials to clinically validate AI-generated drug–disease matches. Results of these trials, all with preclinical data generated by AI, will build the clinical evidence required for regulatory approval or off-label use of repurposed therapies.

> "Cures Within Reach is providing a framework for validating and iterating AI models towards endpoints and treatments that are truly transformational... Cures Within Reach's long-standing leadership in bringing repurposed treatments to patients with unmet medical needs will be a powerful lens through which to evaluate the promise of AI-driven predictions, particularly for rare diseases and other under-incentivized disease areas."

> > Chan Zuckerberg Initiative 😚

In addition to this exciting AI Validation effort, we continue to explore other developments impacting patient treatment options that will have a positive impact on our mission in 2025, including:

- The addition of real world evidence and natural history as control arms, building upon data robustness of randomized control trials
- European initiatives to facilitate label extensions for generic drugs to enhance patient adoption
- Cell and gene therapies and related technologies as more become approved treatments and eligible for our funding

COMMUNITY ENGAGEMENT

Since 2020, all of our clinical trials impacting health disparities require the engagement of community**based organizations** alongside the trials. In 2024, we expanded this by offering optional community engagement funding to other US-based trials. This community engagement component, funded in partnership with Burroughs Wellcome Fund and others, supports social services organizations, community health, other healthcare nonprofits and faith-based groups, and strengthens our clinical trials through better inclusion of underrepresented communities and patients throughout the entire clinical process – before the trial begins enrollment and during the trial, as well as supporting post-trial dissemination of the results.



We hold community engagement training sessions for our funded researchers and clinical teams, with an engagement consultant available as they plan and implement their community engagement activities. At year-end, we had 12 ongoing trials with community engagement.

In 2024, philanthropic partners Open Philanthropy and Chan Zuckerberg Initiative joined Burroughs Wellcome Fund in providing funding for this effort.

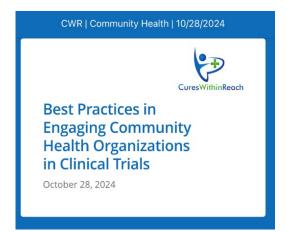
Engaging Community Health Organizations in Clinical Trials

In October 2024, we held a convening event to support clinical trial researchers and clinical trial teams, focused on engagement of community health organizations in clinical trials.

Moderated by Tammy Collins, PhD of Burroughs Wellcome Fund, speakers represented community health, clinical researchers and industry:

- Dyan Bryson, Total Diversity Clinical Trial Management and Inspired Health Strategies
- Abiola Ibraheem, MD, University of Illinois Chicago
- Anne Marie McKenzie-Brown, MD, Emory University
- Beth Tumiel, LCSW, Erie Family Health Centers

This session focused on the importance of not underestimating the value of community health organizations to support screening and enrollment opportunities for active clinical trials with sites nearby. Speakers discussed best practices when engaging local hospitals, community health centers, federally qualified health centers and other community health organizations trusted by patients, yet often unaware of clinical trials.









Live Panel Please submit your questions in the right hand engagement panel

GOLF FUNDRAISER

More than 50 golfers participated in our annual golf fundraiser held at **Rich Harvest Farms**,

raising more than \$110,000 towards our mission. A portion of the proceeds helped fund new clinical trials impacting pediatrics and veterans.





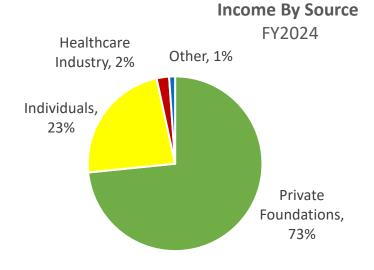




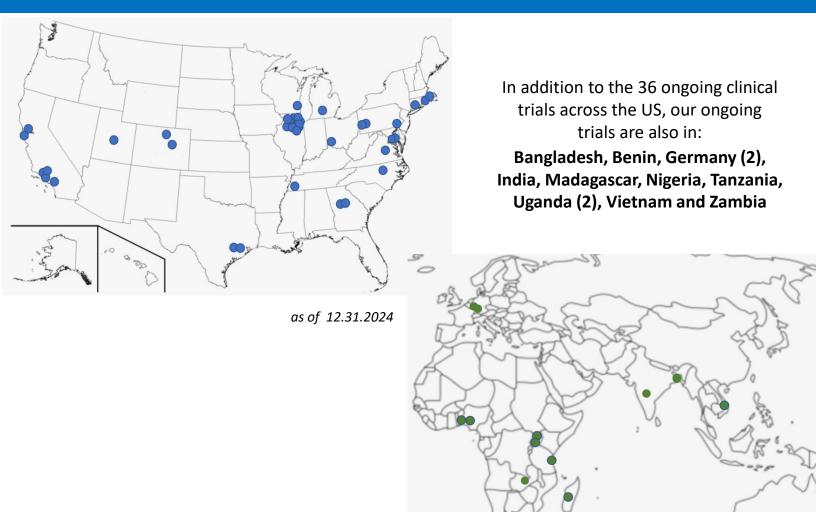




Clinical Trial Funding Awarded



BY GEOGRAPHY



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