“Cures Within Reach is fulfilling an absolutely critical need. It has shown great vision, being at the forefront of trying to match promising repurposing projects with the resources that they need, whether it be financial or advice. And Cures Within Reach has always put patients first.”

Kevin Grimes, MD
Co-Director, SPARK at Stanford, Stanford University
2020 EXECUTIVE SUMMARY

We find and fund clinical trials using approved therapies to fast-track safe and effective treatments into new indications. The COVID-19 pandemic highlighted these advantages. Our patient-first, disease-agnostic approach focuses on early data to catalyze follow-on funding by others.

30 ongoing projects at 26 institutions in 19 diseases including 13 new trials started, 4 completed, plus 17 more in process

Pandemic Spotlight Brings New Initiatives

- Funded our first Health Disparities project to impact Black and Latinx populations in COVID-19, and another COVID-19 trial using fluvoxamine
- Selected 4 projects as part of ReGRoW, led by minority and underserved researchers
- Launched Clinical Repurposing for Veterans to impact veterans’ issues, and began planning a pediatrics initiative
- Continued our Repurposing Community efforts, including 4 new projects in infectious diseases, 3 in rare diseases, 2 in oncology, 1 in neurology, 3 in Chicago and 2 in low/low-middle income countries
- Held 2 virtual patient education events on working with regulatory agencies and on pediatrics, and held 2 virtual CureAccelerator Live! pitch events, focused on Rare Diseases and on Chicago

Our Newest Success Story

CWR helped support a clinical study repurposing 2 melanoma biologics plus radiation for Stage 4 non-small cell lung cancer patients. Early results were presented at ASCO and ASTRO in 2020, and the University of Chicago is preparing an expansion phase, funded by a major pharma company, starting in early 2021.

CWR’s $15,000 contribution helped to leverage major industry support of the follow-on clinical study.

Together We’re Making Real Patient Impact

<table>
<thead>
<tr>
<th>Diseases Researched</th>
<th>56</th>
</tr>
</thead>
<tbody>
<tr>
<td>Projects Funded</td>
<td>97</td>
</tr>
<tr>
<td>Institutions Funded</td>
<td>61</td>
</tr>
<tr>
<td>Diseases Improved</td>
<td>13</td>
</tr>
<tr>
<td>Follow-On Raised by Researchers</td>
<td>$67 million</td>
</tr>
</tbody>
</table>
To Our Stakeholders:

On behalf of our Board of Directors, staff and volunteers, I’m excited to share our progress from 2020, even during the ongoing pandemic. The COVID-19 pandemic shined a spotlight on what we’ve known for over a decade: the fastest way to therapeutic impact is using approved therapies in randomized controlled trials.

Our 2020 highlights:

- We nearly doubled our ongoing projects in 2020, from 16 to 30.
- We launched our Clinical Repurposing for Veterans Initiative.
- We funded our first project impacting health disparities and our first 4 in infectious diseases.
- We held 4 virtual patient education and CureAccelerator Live! pitch events.

We are grateful to ALL of our stakeholders, both new and renewing, across all our Repurposing Communities – and look forward to an exciting 2021!

Barbara Goodman
President & CEO

Creating Patient Impact – Remembering Our “Why”

Cures Within Reach and our stakeholder partners are passionate about creating positive patient impact by developing “new” treatments through repurposing. As 2020 made clear, patients with unmet medical needs are in always in crisis-mode, and patients and families want and deserve solutions whether there is commercial value or philanthropic opportunity.

Our current repurposing research portfolio covers a range of disease areas, from infectious diseases, oncology and mental health to rare blood cancers, rare eye diseases and even a possible snakebite treatment.

- 37% rare diseases
- 20% oncology
- 20% neurology (mental health and neurological)
- 20% inner ear diseases
- 13% infectious diseases
- 10% ophthalmic

- 78% adult; 22% pediatric
- 77% drug; 23% device/other
- 83% US-based; 17% outside US
- 93% clinical; 7% pre-clinical

We are grateful for the support of our donors, sponsors and funding partners in making these exciting research studies a possibility.
Cures Within Reach improves 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. By supporting the unrealized clinical potential and missed therapeutic opportunities in existing medicine and science, we fund the clinical testing of approved drugs, devices and nutraceuticals for unsolved disease indications that can serve both philanthropic and commercial needs.

Our Goals

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

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

We drive market impact and health savings
- To patients and patient groups / disease associations
- From academia / researchers
- With the healthcare industry and payers
- With support from the government, philanthropy and others
## NEW PROJECTS IN 2020

### 13 projects at 13 institutions in 9 diseases

<table>
<thead>
<tr>
<th>Disease Area</th>
<th>Institution</th>
<th>Title</th>
<th>Type</th>
</tr>
</thead>
<tbody>
<tr>
<td>Infectious Disease</td>
<td>Obafemi Awolowo University, Nigeria</td>
<td>Treating Tuberculosis with the Lipid Lowering Drug Atorvastatin in Nigeria</td>
<td>Drug</td>
</tr>
<tr>
<td>Infectious Disease</td>
<td>George Washington University, Universidad El Bosque</td>
<td>Repurposing a Flu Treatment for Severe Dengue Patients in Colombia</td>
<td>Drug</td>
</tr>
<tr>
<td>Infectious Disease</td>
<td>University of Chicago</td>
<td>Can Vitamin D Reduce the Burden of COVID-19 in Chicago?</td>
<td>Nutraceutical</td>
</tr>
<tr>
<td>Infectious Disease</td>
<td>Northwestern University</td>
<td>Testing an antidepressant (fluvoxamine) to slow progression of COVID-19</td>
<td>Drug</td>
</tr>
<tr>
<td>Inner Ear</td>
<td>Curtin University</td>
<td>Testing a generic diuretic to improve drug delivery to the inner ear in Ménière’s disease</td>
<td>Drug</td>
</tr>
<tr>
<td>Inner Ear</td>
<td>House Ear Institution</td>
<td>Reducing vertigo and hearing loss in Ménière’s disease by repurposing a common allergy drug</td>
<td>Drug</td>
</tr>
<tr>
<td>Inner Ear</td>
<td>The Feinstein Institutes for Medical Research</td>
<td>Investigating the effects of repurposed diuretic, steroid and immunosuppressive drugs in Ménière’s disease</td>
<td>Drug</td>
</tr>
<tr>
<td>Inner Ear</td>
<td>Medical University of South Carolina</td>
<td>Assessing the efficacy of an antidepressant for improving vertigo attacks in Ménière’s disease</td>
<td>Drug</td>
</tr>
<tr>
<td>Neuro</td>
<td>Shirley Ryan AbilityLab</td>
<td>Repurposing a Blood Cancer Treatment to Treat Muscle Contractures in Cerebral Palsy Patients</td>
<td>Drug</td>
</tr>
<tr>
<td>Oncology / Rare</td>
<td>Children’s Hospital of Philadelphia</td>
<td>Preventing Relapse After Bone Marrow Transplant in Pediatric Acute Lymphoblastic Leukemia with a Personalized Treatment</td>
<td>Drug</td>
</tr>
<tr>
<td>Oncology / Rare</td>
<td>University of California, Irvine</td>
<td>Treating a Group of Rare Blood Cancers, Myeloproliferative Neoplasms, with a Nutraceutical</td>
<td>Nutraceutical</td>
</tr>
<tr>
<td>Ophthalmology / Rare</td>
<td>University of California, Davis</td>
<td>Repurposing Bone Marrow Stem Cell Therapy for Vision Loss Caused by Retinitis Pigmentosa</td>
<td>Other</td>
</tr>
<tr>
<td>Other</td>
<td>KEMRI-Wellcome Trust Research Programme, Kenya</td>
<td>Testing the Safety of a Metal Poisoning Drug to Treat Snakebite</td>
<td>Drug</td>
</tr>
</tbody>
</table>

## PROJECTS COMPLETED IN 2020

### 4 projects at 4 institutions in 4 diseases

<table>
<thead>
<tr>
<th>Disease Area</th>
<th>Institution</th>
<th>Name</th>
<th>Type</th>
</tr>
</thead>
<tbody>
<tr>
<td>Oncology</td>
<td>University of Chicago</td>
<td>Innovations in Combination Therapies for Non-Small Cell Lung Cancer</td>
<td>Drug</td>
</tr>
<tr>
<td>Oncology / Rare</td>
<td>Ulm University</td>
<td>Combining Nine Repurposed Drugs With a Current Chemotherapy Treatment in Adult Brain Cancer</td>
<td>Drug</td>
</tr>
<tr>
<td>Rare</td>
<td>Children’s Hospital of Philadelphia, Hospital for Sick Children</td>
<td>Repurposing an Antibiotic to Treat a Defect in Vitamin D Metabolism</td>
<td>Drug</td>
</tr>
<tr>
<td>GI</td>
<td>OpenBiome, University of Cape Town</td>
<td>The THRIVE Study: Repurposing Fecal Microbiota Transplantation for the Treatment of Pediatric Malnutrition in South Africa</td>
<td>Other</td>
</tr>
</tbody>
</table>

* winner of CureAccelerator Live!  
^ pre-clinical
“Drug development in pediatrics is slow and inefficient. Pediatric trials face many hurdles that adult trials do not... It is because of these unique challenges that **drug repurposing is such an important and promising strategy for pediatrics.**”

Mary B. Leonard, MD, MSCE
Physician in Chief, Lucile Packard Children’s Hospital Stanford
and Department of Pediatrics Chair, Stanford School of Medicine

## ONGOING PROJECTS IN 2020

17 projects at 14 institutions in 14 diseases

<table>
<thead>
<tr>
<th>Disease Area</th>
<th>Institution</th>
<th>Title</th>
<th>Type</th>
</tr>
</thead>
<tbody>
<tr>
<td>Autoimmune</td>
<td>Massachusetts General Hospital</td>
<td>Repurposing a Vaccine for <strong>Type I Diabetes</strong></td>
<td>Drug</td>
</tr>
<tr>
<td>Autoimmune / Ophthalmology</td>
<td>Northwestern University</td>
<td>Treating <strong>Cataracts in Diabetic Patients</strong> Using a Surgical Device</td>
<td>Device</td>
</tr>
<tr>
<td>GI</td>
<td>Boston Children's Hospital</td>
<td>Fecal Microbiota Transplantation Patient Registry for <strong>Pediatric C-diff</strong></td>
<td>Other</td>
</tr>
<tr>
<td>Inner Ear</td>
<td>Dent Neurologic Institute</td>
<td>Combining an anti-epileptic drug with an anti-anxiety drug to treat the inner ear disorder <strong>Ménière's disease</strong></td>
<td>Drug</td>
</tr>
<tr>
<td>Inner Ear</td>
<td>Hospital of the Ludwig-Maximilians University, Munich</td>
<td>Combining a vertigo drug with a Parkinson’s disease drug to treat the inner ear disorder, <strong>Ménière's disease</strong></td>
<td>Drug</td>
</tr>
<tr>
<td>Neuro</td>
<td>Johns Hopkins Medicine</td>
<td>Repurposing OTC Cough Medicine as a Rapid Acting <strong>Antidepressant</strong></td>
<td>Drug</td>
</tr>
<tr>
<td>Neuro</td>
<td>University of Pennsylvania Medicine</td>
<td>Using Imaging Techniques to Guide Targeted Brain Stimulation in the Treatment of <strong>Depression</strong> and <strong>Post-traumatic Stress Disorder</strong></td>
<td>Device</td>
</tr>
<tr>
<td>Neuro</td>
<td>The Pennsylvania State University, Milton S. Hershey Medical Center</td>
<td>Repurposing a Multiple Sclerosis Drug in <strong>Severe Limb Trauma</strong> *</td>
<td>Drug Into Diagnostic</td>
</tr>
<tr>
<td>Neuro / Rare</td>
<td>Georgetown University</td>
<td>Using a Cancer Drug in <strong>Huntington’s Disease</strong></td>
<td>Drug</td>
</tr>
<tr>
<td>Neuro / Rare</td>
<td>University of Texas Health Science Center at Houston</td>
<td>Treating Irritability in <strong>Huntington’s Disease</strong> with a Repurposed Neurological Drug</td>
<td>Drug</td>
</tr>
<tr>
<td>Oncology</td>
<td>The Hospital for Sick Children</td>
<td>Low-level Laser Therapy to Address Treatment Side Effects in <strong>Pediatric Cancer Patients</strong></td>
<td>Device</td>
</tr>
<tr>
<td>Oncology / Rare</td>
<td>Massachusetts General Hospital / Harvard, VUmc Cancer Center Amsterdam</td>
<td>A Novel Combination of Generic Chemotherapy Drugs to Treat <strong>Brain Cancer</strong></td>
<td>Drug</td>
</tr>
<tr>
<td>Oncology / Rare</td>
<td>University of Michigan</td>
<td>Repurposing Old Drugs as New Therapies for <strong>Metastatic Thyroid Cancer</strong></td>
<td>Drug</td>
</tr>
<tr>
<td>Oncology / Rare</td>
<td>University of Michigan</td>
<td>Using a Skin Cancer Drug to Improve Current Treatment in a <strong>Rare Blood Cancer</strong></td>
<td>Drug</td>
</tr>
<tr>
<td>Ophthalmology / Rare</td>
<td>University of Michigan</td>
<td>Testing a Generic Malaria Drug in a <strong>Rare Ophthalmic Condition</strong></td>
<td>Drug</td>
</tr>
<tr>
<td>Rare</td>
<td>Hospital for Sick Children, National Institutes of Health</td>
<td>TAM4MTM: Tamoxifen Therapy for <strong>Myotubular Myopathy</strong></td>
<td>Drug</td>
</tr>
<tr>
<td>Rare</td>
<td>St. Jude Children's Research Hospital</td>
<td>Repurposing a Blood Cancer Drug to Treat an Immune Disorder (HLH) in Children *</td>
<td>Drug</td>
</tr>
</tbody>
</table>

* winner of CureAccelerator Live!
Drug repurposing trials “streamline the pathway to finding urgently needed COVID-19 treatments by repurposing either licensed or late-stage-development medicines and testing them in a way that identifies the most promising agents for larger clinical studies in the most expedient way possible.”

Francis Collins, MD, PhD, Director
National Institutes of Health (nihrecord.nih.gov)

OUR 2020 RESULTS

While the COVID-19 pandemic certainly slowed our ongoing projects, our funded researchers were still able to publish, present and raise follow-on funding.

Measuring CWR’s Success

Research Aims Met

Benefit Seen in Patients

Publication of Results

Follow On Funding

Follow On Trials (impacting limited patients)

Impacting Patients Broadly

We track our funded research projects during the project timeline, and we follow the researcher and the repurposed therapy for years afterwards, providing measures of success over time.

Research Aims:
Successful safety assessment of a combination of 9 generic drugs in adult brain cancer

Patient Benefit:
Positive results in some patients seen after 12 months in adult brain cancer

Publications and Presentations:
Presented results impacting lung cancer at ASCO; publications in pancreatic cancer and in other diseases

Follow-On Funding:
More than $15 million was reported during 2020 for next phase or related projects

Follow-On Trials:
Active planning for a follow-on trial in lung cancer, rare pediatrics and others

Impacting Patients More Broadly:
Training tool developed for optimizing treatment with a repurposed device in pancreatic cancer

To read more details, visit: cureswithinreach.org
CWR ENABLES CLINICAL TRIAL FUNDING AT CRITICAL STAGES OF THERAPY DEVELOPMENT

Additive to Life Cycle Management:
Mid-stage: on-patent, but small indication or off-strategy
Later stage: generic or new delivery method

Little / No Commercial Value
Clinicians may consider off-label use from published, validated studies

Likely / Known Commercial Value
Industry further develops approved therapies that enhance therapeutic benefit to patients

Our Focus: patient access to viable treatments, while building a pipeline of opportunities

We aren’t funding to the finish line, but providing enough seed funds to achieve catalytic effect
CWR funding often catalyzes follow-on funding from the NIH, foundations, industry and investors.

CWR provides:
- Access to our 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, de-risking and managing our funded projects
- Both project and financial progress reports during and after the project

Donor-advised funding can support a specific disease, geography, patient population or other theme
- Impacting Health Disparities
- Impacting Veterans’ Issues
- Led by Minority or Underserved Researchers
- In Pediatrics
- In a specific disease area or geography

Repurposing Communities
In 2020, we continued building our Repurposing Community model to align with our stakeholders’ strategic interests:
- Disease-specific: Oncology; Rare Diseases; and Neurology
- Geographic: Chicago; Mid-Atlantic; and the Developing World
- Starting soon for patient groups: Veterans, Pediatrics, Health Disparities

These virtual communities engage key stakeholders at both the community level as well as with our overall organization. Stakeholders include:
- Patient groups and disease associations
- Universities and research institutions
- Healthcare industry and service providers (who join annually)
- Philanthropy (who join annually)
- Government / others
ADDING VALUE THROUGH REPURPOSING COMMUNITIES

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 or triple bottom line: patient impact; economic development from follow-on funding; success reinvested in future projects

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
As part of our Repurposing Communities programming, we represented the voice of repurposing as we organized, spoke at or participated in a variety of patient education events in a variety of areas of interest, including:

- COVID-19 and infectious diseases
- Pediatrics
- 505(b)(2) Regulatory Process
- Rare diseases
- Impact philanthropy

**GLOBAL HEALTH REPURPOSING AWARDS**

Patient Education Events to Honor our 2020 Awardees:

- **SPARK** AT STANFORD
- **DNDi** Drugs for Neglected Diseases initiative

**How Patients Impact the 505(b)(2) Regulatory Process** on Oct 19
**Repurposing for Pediatric Patients: Best Practices and Lessons Learned** on Nov 2

During 2020, we held 2 virtual CureAccelerator Live! philanthropic pitch events: for Rare Diseases in June and for Chicago in September. These events:

- Bring clinical research to a public setting
- Showcase 3 to 5 projects selected from an RFP via an external review committee
- Select a winning clinical repurposing project after Q&A from an expert panel
HONORING LEADERSHIP

GLOBAL HEALTH REPURPOSING AWARDS

2020 Janet Davison Rowley Patient Impact Research Award

at Stanford School of Medicine, Stanford University
to honor its support of the translational development of repurposing opportunities for both commercial and philanthropic treatments for patients with unmet medical needs, in a range of diseases via drugs, devices and other technologies

accepting on behalf of SPARK: Kevin Grimes, Co-Director

2020 Golan Christie Taglia Patient Impact Philanthropy Award

2020 Patient Impact Industry Award

accepting on behalf of DNDi: Nathalie Strub-Wourgaft, Director of Neglected Tropical Diseases

accepting on behalf of Camargo: Ken Phelps, President & Founder

to honor its efforts to develop 8 new treatments from existing molecules and recombining drugs to bring better treatments to patients with malaria, Chagas disease, sleeping sickness, leishmaniasis and pediatric HIV

to honor to honor its dedication to supporting the regulatory approval and development of approved therapies that enhance patient outcomes through the 505(b)(2) pathway and similar pathways worldwide
ReGRoW builds capacity for clinical trial research in the low and low-middle income countries (LMICs) by providing repurposing research grants to underserved clinicians and researchers based in LMICs to find treatments for patients within LMICs.

During 2020, we received over 30 proposal submissions from 12 LMICs on 4 continents. After a 2-stage review process, supported by our Science Advisory Board, over 20 volunteer, external members of our Grant Review Committee and input from our ReGRoW funding partners, 4 clinical repurposing projects were selected for funding in 2020:

- Treating Snakebite in Kenya
- Infectious Disease in Nigeria
- Infectious Disease in Kenya
- Rare Pediatrics in Vietnam

“...By doing drug repurposing, it is very clear that you’re saving not only money, but time... Repurposing will and can accelerate access to treatments whilst minimizing the risks and costs.”

Dr. Nathalie Strub-Wourgraft, Director of Neglected Tropical Diseases Drugs for Neglected Diseases initiative

GOLF FUNDRAISER

Although postponed from the Spring to the Fall, our annual golf fundraiser, held at Rich Harvest Farms, raised more than $60,000 towards our mission. A portion of the proceeds helped to fund four infectious disease projects: two in COVID-19, one in dengue and one in tuberculosis.
BY THE NUMBERS

Private Foundations, 73%
Individuals, 6%
Healthcare Industry, 19%
Non-Healthcare Industry and Other, 2%

FY2020 Income By Source
- Private Foundations, 73%
- Healthcare Industry, 19%
- Individuals, 6%
- Non-Healthcare Industry and Other, 2%

FY2020 Income By Source
- FY2017
- FY2018
- FY2019
- FY2020

New Grant / Award Commitments
- FY2017
- FY2018
- FY2019
- FY2020

Revenue Growth
- FY2017
- FY2018
- FY2019
- FY2020

BY GEOGRAPHY

In addition to 25 projects across the US, our ongoing projects are also in:
- Australia
- Canada
- Germany
- Kenya
- Nigeria
Cures Within Reach Leadership
Barbara Goodman, President & CEO
Clare Thibodeaux, PhD, Director of Scientific Affairs

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