“Why is Celgene supporting Cures Within Reach? I think I can speak for the company and I can answer the question in one sentence: the patients are waiting.”

Dr. Jerry Zeldis, Celgene Corporation, Global Health Repurposing Awardee

“The research that I’ve done so far tells me this is the only organization on this planet that’s pursuing, repurposing research consistently and in an organized fashion.”

Joel Dysart, Retired Management Consultant, Cures Within Reach supporter

“Repurposing is about bringing drugs to market at a much lower cost and getting them into the hands of people that really need it when they need it. That to me is such an important thing to embrace and really get behind. Because it could really move the perception that people have of the industry as a whole, to a much different spot than where it currently is.”

Dimitri Drone, Partner, Global Pharmaceutical and Life Sciences Leader, PwC
OUR YEAR IN REVIEW

Autism, lymphoma, Alzheimer’s, Parkinson’s, autoimmune disorders, lung cancer, breast cancer, pediatric rare diseases...chances are that one or more of these conditions has impacted your life and the lives of those you love. When disease strikes you or your loved one, the odds are there is no cure, and finding an effective treatment may feel like it is taking forever. Cures Within Reach and our global supporters are working together to create a growing, self-sustaining system for the repurposing of existing drugs, devices and nutriceuticals to create effective treatments for unsolved diseases.

How?

BY CONNECTING RESEARCH AND FUNDING,

BY FACILITATING CROWD-SOURCING AND COMMUNITY, AND

BY IDENTIFYING NEW ECONOMIC ENGINES AND FINANCIAL MODELS TO INCENTIVIZE REPURPOSING,

AT CURES WITHIN REACH, WE WORK TO CATALYZE REPURPOSING RESEARCH TO QUICKLY AND AFFORDABLY IMPROVE PATIENT LIVES.

In the 2015-16 fiscal year, Cures Within Reach continued building our global community of stakeholders passionate about repurposing on CureAccelerator™ (cureaccelerator.org), with several funded repurposing research Requests for Proposals, and an inaugural “100 Projects in 100 Days” challenge, which was a big success. We received positive news on research projects previously initiated with our funding, which have now yielded results that are saving patient lives. We entered into a rare disease research funding partnership with the Canadian Institutes of Health Research, that will extend through to 2018. Our initial research institution partnerships were renewed, and we added many new partners. Our big-picture, global initiatives looking at new ways to finance generic drug repurposing research have made significant progress. And our passion about repurposing is spreading through international speaking engagements and Chicago-based, one-of-a-kind repurposing events.

Thank you for being a part of our growth, and for helping us create answers to the urgent question many patients are asking: When will there be an affordable treatment for my disease?
Our research project funding is directed at proof of concept research, primarily small clinical trials, that can result in immediate improvement in clinical care. In the 2015-16 fiscal year, we ended with 16 repurposing projects in process, spanning many disease areas and patient populations. A quick breakdown:

**PROJECT TYPE**

- Preclinical
- Clinical
- Both

**PATIENT GROUP**

- Pediatric
- Adult
- Both

## IMPROVING STEM CELL TRANSPLANTS FOR BLOOD CANCERS

We celebrate the outcomes published this year by Dr. Luis Porrata, who made an amazing discovery that has resulted in a significantly improved therapy for patients with Non-Hodgkin’s Lymphoma. We were a lead funder in this project, which repurposed existing stem cell transplant devices and techniques in a new way to create a more effective therapy for patients. This new and improved protocol has been adopted into the standard of care at the Mayo Clinic and has been disseminated to other physicians through 2016 publication in the *Biology of Blood and Marrow Transplantation*.

Dr. Porrata’s clinical trial sought to improve the existing autologous stem cell transplant (ASCT) protocol used to treat non-Hodgkin’s lymphoma patients. The existing method involved removing healthy blood cells from a patient, treating the patient with chemotherapy to kill the cancer, and then re-injecting the patient with their removed cells to regenerate a healthy blood system. By simply increasing both the total amount of cells and the relative amount of a specific cell type (natural killer cells) that are harvested by the current devices and later re-injected into the patients, the success rate of the procedure is increased. For almost no cost, and no additional risk, the rate of durable remissions seen in patients can increase from 50% to 70%! These protocol improvements can save lives and reduce healthcare costs for patients who suffer from a variety of blood cancers. Almost 600,000 ASCTs are performed around the world each year. This success could save up to 180,000 lives per year!

NUTRICEUTICALS FOR AUTISM

Results from Dr. Sarah Keim’s work at Nationwide Children’s Hospital in Ohio, show promising data that Omega-3, -6, and -9 supplementation in children 18-42 months old can reduce symptom presentation and support language development in patients with autism, and with ADHD. Publication is expected before the end of 2016.

A NEW TREATMENT FOR CHRONIC LIMB ISCHEMIA

One project selected for funding in 2016 repurposes an approved drug and an approved device in a unique combination therapy to literally save the limbs of those suffering from chronic limb ischemia (CLI). This is a condition in which circulation to the limbs, usually legs, is severely impaired and often leads to amputation. CLI affects over 1,000,000 people in the United States, and is projected to increase significantly in the coming decades. Cures Within Reach is funding an 8 patient clinical trial at the University of Illinois at Chicago to test the combination of a device that helps to pump blood through the affected limb, and a drug that increases blood vessel re-growth.
PARTNERING TO SUPPORT RARE DISEASE RESEARCH

Cures Within Reach has historically funded one disease-specific project at a time, based on funds in hand supplied by funding partners with a particular disease interest. We are now working with funders who give us the freedom to create funding portfolios and programs in which Cures Within Reach and our advisors select the most promising projects, regardless of disease focus, to create greater patient and financial impact. As an initial step in this direction, we formed a partnership with the Canadian Institutes of Health Research, and will be selecting 10 projects during 2016-18 to co-fund. All projects were will be sourced through our CureAccelerator™ platform, must be drug repurposing clinical trials for rare disease, and must involve collaboration between US and Canadian institutions. Projects using generic drugs will be given preference. With 7,000 rare diseases in the world, and very little funding to drive toward new treatments, Cures Within Reach is bringing repurposing to these patients in need. Operational support for this program is being provided by the Mindset Foundation. Initial funding announcements will be released late in 2016.

---

CURRENT CURES WITHIN REACH PROJECTS

Cures Within Reach and our funding partners have a portfolio of current repurposing research projects, most of which are proof of efficacy clinical trials that should produce data sufficient to support physician off-label use, which is the fastest way to get “new” treatments to patients with unsolved diseases.

<table>
<thead>
<tr>
<th>Dr. Krista Lanctôt</th>
<th>University of Toronto</th>
<th>A new opportunity to treat behavioral problems in Alzheimer’s patients</th>
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</thead>
<tbody>
<tr>
<td>Dr. Jeff Borgia</td>
<td>Rush University Medical Center</td>
<td>Repurposing a blood test to improve outcomes for high-risk lung cancer patients who are diagnosed through low-dose CT screening</td>
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<tr>
<td>Name(s)</td>
<td>Institution</td>
<td>Project Description</td>
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<tr>
<td>Dr. James Young, Dr. Max Fitzgerald</td>
<td>Rush University Medical Center</td>
<td>Repurposing a device to treat abnormalities of gait and balance in MS, Parkinson’s, stroke and TBI</td>
</tr>
<tr>
<td>Dr. George Havelka, Dr. Darwin Eton</td>
<td>University of Illinois at Chicago</td>
<td>Saving limbs: Helping nature do its magic in patients with Chronic Limb Ischemia</td>
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<tr>
<td>Dr. Marc-Eric Halatsch</td>
<td>Ulm University</td>
<td>Combining nine repurposed drugs with a current chemotherapy treatment in recurrent brain cancer</td>
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<tr>
<td>Dr. Zachary Cox</td>
<td>Vanderbilt University Medical Center</td>
<td>Comparison of diuretic combination therapies in heart failure patients</td>
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<tr>
<td>Dr. Azra Raza, Dr. Naomi Galili</td>
<td>Columbia University Medical Center</td>
<td>Genetic sequencing to identify treatment targets in the rare blood disease MDS-RARS</td>
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<td>Dr. Denise Faustman</td>
<td>Massachusetts General Hospital</td>
<td>Repurposing a vaccine for Type I Diabetes</td>
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<td>Dr. Pamela Itkin-Ansari</td>
<td>Sanford Burnham Medical Research Institute</td>
<td>Repurposing an FDA approved encapsulation device to create an artificial pancreas for Type 1 Diabetes</td>
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<td>Dr. Ron Crystal</td>
<td>Weill Cornell Medical College</td>
<td>Repurposing gene therapy for Batten Disease</td>
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<tr>
<td>Dr. Sarah Leary</td>
<td>Seattle Children’s Hospital</td>
<td>Repurposing drugs for recurrent pediatric brain tumors</td>
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<tr>
<td>Dr. Sarah Keim</td>
<td>Nationwide Children’s Hospital</td>
<td>Treating the developmental consequences of prematurity with a repurposed nutriceutical</td>
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<tr>
<td>Dr. Chani Traube</td>
<td>Weill Cornell Medical College</td>
<td>Repurposing an approved drug to treat pediatric delirium</td>
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<td>Dr. Logan Wink</td>
<td>Cincinnati Children’s Hospital Medical Center</td>
<td>Repurposing a generic drug in autism spectrum disorder</td>
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<tr>
<td>Dr. Gregory Riggins</td>
<td>Johns Hopkins Medicine</td>
<td>A Phase I Trial for recurrent pediatric brain cancers using a repurposed generic drug</td>
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<tr>
<td>Dr. Stacy Kahn</td>
<td>University of Chicago</td>
<td>Fecal microbiota transplantation patient registry for children with C-Difficile</td>
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</table>
In June 2015 we launched CureAccelerator, the world’s first online, interactive platform dedicated to Repurposing Research. As an online, open access tool, CureAccelerator connects funders, researchers, clinicians, industry, government and patient groups to drive Repurposing Research. This tool provides a way for Cures Within Reach to expand its own research sourcing and funder connections, while also building a repurposing research community that can coordinate efforts to improve patient lives.

The focus of CureAccelerator is “proof of concept” clinical trial research, repurposing drugs, devices and nutriceuticals already approved for human use. On CureAccelerator, funders have direct access to find and support repurposing research ideas, researchers can post ideas and connect with peers and funding, and clinicians can contribute their experience of off-label use in need of validation. The Cures Within Reach staff is curating the space by driving engagement, verifying expert users, and conducting scientific review. As a repurposing research sourcing and community building tool, CureAccelerator will support all of our initiatives moving forward.

THREE-YEAR GOAL: A VITAL NON-PROFIT AND FOR-PROFIT REPURPOSING COLLABORATION HUB AND MARKET

How did we do in year one?

- 860 Active users
- 43 Academic Institution Partners
- 120 Projects posted
- 11 Requests for Proposal

OUR FIRST CUREACCELERATOR ENGAGEMENT CAMPAIGN

From November 2015 to February 2016, we ran a communication campaign to galvanize researchers and clinicians to post new repurposing projects and ideas to CureAccelerator, proving to the world that developing repurposed treatments is a critical piece of the healthcare puzzle.
Our results? Over 100 projects posted, initiated from 52 research institutions in 7 countries. The projects proposed repurposed treatments for a wide variety of diseases, including 35 in oncology, 18 addressing neurology and neurodegenerative concerns and 19 in pediatric or rare diseases.

We loved the “100 in 100” campaign, and plan to focus the next cycle on engagement of clinicians with real-life knowledge of repurposing possibilities, based on off-label prescription experience. This will be launched in conjunction with a social partnership with Doximity, a vibrant, online physician’s network.

NEW RESEARCH INSTITUTION PARTNERS

Many hours were spent to vitalize our research partner network in 2015-16, with great results. New and renewed partner agreements signed in this fiscal year include:

- Albert Einstein College of Medicine
- Houston Methodist Research Institute
- Indiana Center for Biomedical Innovation
- Indiana Clinical & Translational Sciences Institute
- Medical College of Wisconsin
- Oregon Health & Science University
- Rosalind Franklin University
- Sanford Research
- Stanford University
- The Hospital for Sick Children
- University of Michigan
- University of Minnesota
- University of New Mexico
- University of Pennsylvania
- University of Sheffield
- University of Vermont

OUR FIRST LIVE PITCH EVENT

In March, 2016, our Young Professional’s Board created and executed what we believe is the world’s first live pitch event dedicated to repurposing research. CureAccelerator Live! hosted six researchers presenting their vetted repurposing research projects in a live format, and awarded funding to the project with the most audience votes. The projects covered a range of diseases and conditions, with the winning project focusing on chronic limb ischemia. This was an exciting night that will definitely be repeated.
The ROI of Repurposing Research is profound. Improved quality and length of patient lives in the near-term are immediate benefits. At the same time, there is often a dramatic reduction in healthcare costs as patients replace their high cost medical care that is not working, with lower-cost, effective repurposed drugs, device and nutriceuticals, possibly in generic form. Harnessing emerging social finance mechanisms, we are working to create the “missing financial incentive” for Repurposing Research. One mechanism, a Social Impact Bond, works by capturing a portion of the healthcare cost savings that Repurposing Research generates and using those funds to repay the investors who supported the proof of concept clinical trials.

In 2015 we partnered with the MaRS Centre and the Mindset Foundation in Canada to conduct a feasibility study, at no cost to Cures Within Reach. We also began to engage with the UK charity Findacure, the social finance company Numbers4Good, the consultancy Costello Medical, and the National Health Service in the England, to begin development of a pilot “Generic Drugs for Rare Diseases” Social Impact Bond in England, again at no charge to Cures Within Reach. We are also working with the Canadian Government to begin discussions about a similar Canadian Social Impact Bond, and we applied to the United Nations to be part of their Solutions 2016.

Working with partners around the globe to consider and act upon ways to expand this model to meet major global needs will be a major focus of 2017.
SHIFTING TO A GLOBAL BRAND

After several successful years hosting the BioScience Awards Midwest, we re-branded our annual gala awards event to reflect the global outreach and impact of our mission and the growth of the global repurposing community. Highlights of the 2016 event include the meaningful sharing of patient stories, robust support from Celgene in honor of their Chief Medical Officer, Dr. Jerry Zeldis, and Micah Materre from Chicago’s WGN News as our guest emcee. The event significantly increased volunteer leadership and financial support for Cures Within Reach and our repurposing research mission!

SAVE THE DATE: JUNE 27, 2017
GLOBAL HEALTH REPURPOSING AWARDS, CELEBRATING THE ROLE OF REPURPOSING IN RARE DISEASE
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<tr>
<th>Month</th>
<th>Event</th>
<th>Details</th>
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<tbody>
<tr>
<td>September</td>
<td>Disruptive Innovations US, Boston, MA “A New Global Web Platform Driving Repurposed Cures to Patients with the Unsolved Diseases”</td>
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<td>Cambridge Rare Disease Summit 2015, Cambridge, UK &quot;Alternative Financing in Rare Disease Research&quot;</td>
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<td></td>
<td>13th Annual Discovery on Target Conference, Boston, MA Strategies for Rare Diseases: Update on Scientific Breakthroughs and Novel Approaches &quot;The Past, Present and Future of Rare Disease Drug Repurposing&quot;</td>
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<td>October</td>
<td>Research in Your Backyard, Chicago, IL We Work for Health (In conjunction with iBIO, Cures Within Reach and the Illinois Medical District Commission) Luncheon</td>
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<td>Minnesota Clinical Research Alliance St. Paul, MN &quot;CureAccelerator™, the world's first interactive, online platform for repurposing research&quot;</td>
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<td>November</td>
<td>Cures Within Reach Annual Meeting</td>
<td>Launched “100 projects in 100 days” campaign on CureAccelerator</td>
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<td>February</td>
<td>DPHARM EUROPE, London, &quot;A Powerful New Economic Engine to Drive Cures to Patients&quot; with the British NHS, Findacure, Costello Medical, Numbers for Good</td>
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<td>CureSearch Catapult, Menlo Park, CA, &quot;New Models for Utilizing Existing Assets for Pediatric Oncology&quot;</td>
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<td>March</td>
<td>Inaugural CureAccelerator Live! event in Chicago, produced by the CWR Young Professionals Board, the first live pitch event highlighting repurposing research, funding the selected project, for chronic limb ischemia at UIC.</td>
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<td>3rd International Congress on Research of Rare and Orphan Diseases, Barcelona, Spain, &quot;Back to the Future-How Drug Repositioning Has and Will Create Treatments for Unsolved Diseases&quot;</td>
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<td>April</td>
<td>MedCity INVEST, Chicago, IL &quot;Beg, Borrow and Steal: Repurposing Non-Healthcare Financial Instruments to Drive New Treatments to Patients&quot;</td>
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<td>Health Experience Refactored Conference, HxRefactored, Boston, MA &quot;To Heal &amp; Cure: Clinical Trial Experiences&quot;</td>
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</tbody>
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May
Indication Expansion and Drug Repurposing Summit, Washington, DC "Partnerships and Innovations in Orphan Drug Repurposing"

June
Global Health Repurposing Awards (formerly BAM) gathers 250 attendees to celebrate repurposing and patient impact

5th Annual Drug Repositioning, Repurposing and Rescue Conference in Chicago, “Overcoming Philanthropic and Commercial Roadblocks to DRRR”

Longest Day of Golf raises over $30K for chronic limb ischemia repurposing trial

CWR invited to participate in Joe Biden’s Cancer Moonshot Summit, Washington D.C.

July
CWR begins discussion with IBM Watson Health to determine if the Watson Discovery Engine can help repurposing research

FINANCIAL SUMMARY

Like many organizations, our revenue impacts our ability to pursue our mission and vision. Thanks to the support of donors listed in this report, the volunteers who have helped with projects and at events, the generous sponsor and foundation support we have received, and our committed board members and staff, we are able to celebrate our “mission progress,” fueled by our steadily increasing fundraising outcomes.

![TOTAL CONTRIBUTIONS](chart1.png)

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