“Repurposing drugs for a new indication is a highly efficient use of time and money. Cures Within Reach’s Impact Award enabled Seurat to access a novel delivery system instrumental to our development and to apply for additional follow-on funding. We’re now closer to delivering a new migraine medication to the millions of American migraine sufferers.”

Yuan Zhang, PhD, MS
President & CEO, Seurat Therapeutics
2019 EXECUTIVE SUMMARY

22 active projects at 19 institutions in 17 diseases
6 new clinical repurposing trials started plus 16 in process
6 projects completed in 2019, and 7 completed in 2018

New and Expanding Programs
Focusing on growth and scale, during 2019 we:

- Launched our new disease-focused (Rare Diseases; Oncology; Neurology) and geographic (Chicago; Mid-Atlantic; Developing World) Repurposing Communities to align programming of stakeholders
- Held or supported patient education events in oncology, neurology, autoimmune and more in cities across the US
- Held 2 CureAccelerator Live! pitch events, focused on Rare Diseases (in Philadelphia) and the Developing World (in Boston)
- Launched call for proposals for ReGRoW, an 18-month pilot to build capacity of clinical trial in low and low-middle income countries (LMICs) by providing grants to find treatments for LMICs from within LMICs
- Continued to build self-sustainability with Conditional Payback grants for research institutions and Impact Awards for repurposing startups

Our Newest Success Story
CWR helped to support a clinical study repurposing nabilone to reduce agitation in Alzheimer’s patients.

Dr. Lanctôt of the University of Toronto and Sunnybrook Research Institute has both presented and published results showing positive outcomes, and is now preparing a Phase 2 study.

CWR’S $30,000 contribution helped to leverage > $1.4 million in follow-on funding.

Together We’re Making Real Patient Impact

- 49 Diseases Researched
- 13 Diseases Improved
- 85 Repurposing Projects Funded
- 51 Institutions Funded
- 6 Startups with CWR Investment
- $6 million in Project Funding
- $49 million Follow-On Funding Leveraged
To Our Stakeholders:

On behalf of our Board of Directors and staff, I’m thrilled to share this snapshot of 2019. It was a year filled with growth, transition, new programming – and a consistent focus on impacting patients with unmet medical needs through medical repurposing.

- **We held patient education events on both coasts and in Chicago.**
- **We held 2 CureAccelerator Live! pitch events highlighting rare diseases and diseases of the developing world.**
- **We doubled our grant / award commitments over prior fiscal year.**
- **We launched our ReGRoW Pilot – building capacity for clinical research in the developing world via repurposing.**

We are grateful to ALL of our stakeholders across all our Communities and look forward to an exciting 2020!

Barbara Goodman
President & COO

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. Repurposing is particularly important in diseases where commercial value may be unknown or low, which includes rare diseases, acute diseases and infectious diseases.

Our repurposing research portfolio covers a range of disease areas, from Type 1 diabetes and Alzheimer’s disease to rare blood cancers, rare eye diseases, depression and pediatric malnutrition.

- 45% of ongoing projects are in rare diseases
- 32% pediatric
- 27% oncology
- 27% neurology (mental health and neurological)
- 9% autoimmune / diabetes
- 9% gastrointestinal
- 9% inner ear diseases
- 9% ophthalmic

We are grateful for the support of our sponsors, donors 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 commercial and philanthropic 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
### PROJECTS STARTED IN 2019

**6 new projects at 6 institutions in 5 diseases**

<table>
<thead>
<tr>
<th>Disease Area</th>
<th>Institution</th>
<th>Lead Researcher</th>
<th>Title</th>
<th>Type</th>
</tr>
</thead>
<tbody>
<tr>
<td>GI</td>
<td>OpenBiome, University of Cape Town</td>
<td>Drs. Osman, Budree</td>
<td>The THRIVE Study: Repurposing Fecal Microbiota Transplantation for the Treatment of Pediatric Malnutrition in South Africa*</td>
<td>Other</td>
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<tr>
<td>Inner Ear</td>
<td>Dent Neurologic Institute</td>
<td>Dr. Zhang</td>
<td>Combining an anti-epileptic drug with an anti-anxiety drug to treat the inner ear disorder Ménière’s disease</td>
<td>Drug</td>
</tr>
<tr>
<td>Inner Ear</td>
<td>Hospital of the Ludwig-Maximilians University, Munich</td>
<td>Dr. Strupp</td>
<td>Combining a vertigo drug with a Parkinson’s disease drug to treat the inner ear disorder Ménière’s disease</td>
<td>Drug</td>
</tr>
<tr>
<td>Neuro</td>
<td>Johns Hopkins Medicine</td>
<td>Dr. Kaplin, Wang</td>
<td>Repurposing OTC Cough Medicine as a Rapid Acting Antidepressant</td>
<td>Drug</td>
</tr>
<tr>
<td>Neuro / Rare</td>
<td>University of Texas Health Science Center at Houston</td>
<td>Dr. Furr-Stimming</td>
<td>Treating Irritability in Huntington’s Disease with a Repurposed Neurological Drug</td>
<td>Drug</td>
</tr>
<tr>
<td>Rare</td>
<td>St. Jude Children’s Research Hospital</td>
<td>Dr. Nichols</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!

### PROJECTS COMPLETED IN 2019

**6 completed projects at 6 institutions in 6 diseases**

<table>
<thead>
<tr>
<th>Disease Area</th>
<th>Institution</th>
<th>Lead Researcher</th>
<th>Name</th>
<th>Type</th>
</tr>
</thead>
<tbody>
<tr>
<td>Neuro</td>
<td>Cincinnati Children's Hospital Medical Center</td>
<td>Dr. Wink</td>
<td>Repurposing a Generic Drug in Autism Spectrum Disorder</td>
<td>Drug</td>
</tr>
<tr>
<td>Neuro</td>
<td>University of Toronto</td>
<td>Dr. Lanctôt</td>
<td>A New Opportunity to Treat Behavioral Problems in Alzheimer’s Patients</td>
<td>Drug</td>
</tr>
<tr>
<td>Oncology / Rare</td>
<td>Johns Hopkins Medicine</td>
<td>Dr. Riggins</td>
<td>A Phase I Trial for Recurrent Pediatric Brain Cancers Using a Repurposed Generic Drug</td>
<td>Drug</td>
</tr>
<tr>
<td>Ophthalmic</td>
<td>U Penn</td>
<td>Dr. Kim</td>
<td>Repurposing a Nutraceutical to Treat a Type of Age-Related Macular Degeneration</td>
<td>Nutraceutical</td>
</tr>
<tr>
<td>Rare</td>
<td>Children’s National and University of Illinois at Chicago</td>
<td>Drs. Nadler, Holterman</td>
<td>Using a Cancer Drug to Treat a Rare Pediatric Liver Disease</td>
<td>Drug</td>
</tr>
<tr>
<td>Vascular</td>
<td>University of Illinois at Chicago</td>
<td>Drs. Havelka, Eton</td>
<td>Saving Limbs from Chronic Ischemia: Helping Nature Do Its Magic</td>
<td>Combo</td>
</tr>
</tbody>
</table>
## CONTINUING PROJECTS IN 2019

**16 projects in process at 13 institutions in 14 diseases**

<table>
<thead>
<tr>
<th>Disease Area</th>
<th>Institution</th>
<th>Lead Researcher</th>
<th>Title</th>
<th>Type</th>
</tr>
</thead>
<tbody>
<tr>
<td>Autoimmune</td>
<td>Massachusetts General Hospital</td>
<td>Dr. Faustman</td>
<td>Repurposing a Vaccine for Type I Diabetes</td>
<td>Drug</td>
</tr>
<tr>
<td>Autoimmune / Ophthalmology</td>
<td>Northwestern University</td>
<td>Dr. Basti</td>
<td>Treating Cataracts in Diabetic Patients Using a Surgical Device</td>
<td>Device</td>
</tr>
<tr>
<td>GI</td>
<td>Boston Children's Hospital</td>
<td>Dr. Kahn</td>
<td>Fecal Microbiota Transplantation Patient Registry for Pediatric C-diff</td>
<td>Other</td>
</tr>
<tr>
<td>Neuro</td>
<td>Medical College of Wisconsin</td>
<td>Dr. Alvarez</td>
<td>Repurposing an Antipsychotic Drug as Treatment for Pediatric Delirium</td>
<td>Drug</td>
</tr>
<tr>
<td>Neuro</td>
<td>University of Pennsylvania Medicine</td>
<td>Dr. Oathes</td>
<td>Using Imaging Techniques to Guide Targeted Brain Stimulation in the Treatment of Depression and Post-traumatic Stress Disorder</td>
<td>Device</td>
</tr>
<tr>
<td>Neuro</td>
<td>The Pennsylvania State Univ., Milton Hershey Medical Center</td>
<td>Dr. Elfar</td>
<td>Repurposing a Multiple Sclerosis Drug in Severe Limb Trauma*</td>
<td>Drug Into Diagnostic</td>
</tr>
<tr>
<td>Neuro / Rare</td>
<td>Georgetown University</td>
<td>Dr. Anderson</td>
<td>Using a Cancer Drug in Huntington’s Disease</td>
<td>Drug</td>
</tr>
<tr>
<td>Oncology</td>
<td>The Hospital for Sick Children</td>
<td>Dr. Suwwan</td>
<td>Low-level Laser Therapy to Address Treatment Side Effects in Pediatric Cancer Patients</td>
<td>Device</td>
</tr>
<tr>
<td>Oncology</td>
<td>University of Chicago</td>
<td>Drs. Chmura, Bestvina</td>
<td>Innovations in Combination Therapies for Non-Small Cell Lung Cancer</td>
<td>Drug</td>
</tr>
<tr>
<td>Oncology / Rare</td>
<td>Massachusetts General Hospital / Harvard, VUmc Cancer Center Amsterdam</td>
<td>Drs. Tannous, Arrillaga-Romany &amp; Kouwenhoven</td>
<td>A Novel Combination of Generic Chemotherapy Drugs to Treat Brain Cancer</td>
<td>Drug</td>
</tr>
<tr>
<td>Oncology / Rare</td>
<td>Ulm University</td>
<td>Dr. Halatsch</td>
<td>Combining Nine Repurposed Drugs With a Current Chemotherapy Treatment in Adult Brain Cancer</td>
<td>Drug</td>
</tr>
<tr>
<td>Oncology / Rare</td>
<td>University of Michigan</td>
<td>Dr. Swiecicki</td>
<td>Repurposing Old Drugs as New Therapies for Metastatic Thyroid Cancer*</td>
<td>Drug</td>
</tr>
<tr>
<td>Oncology / Rare</td>
<td>University of Michigan</td>
<td>Dr. Pettit</td>
<td>Using a Skin Cancer Drug to Improve Current Treatment in a Rare Blood Cancer</td>
<td>Drug</td>
</tr>
<tr>
<td>Ophthalmology / Rare</td>
<td>University of Michigan</td>
<td>Dr. Zacks</td>
<td>Testing a Generic Malaria Drug in a Rare Ophthalmic Condition</td>
<td>Drug</td>
</tr>
<tr>
<td>Rare</td>
<td>Children’s Hospital of Philadelphia, Hospital for Sick Children</td>
<td>Drs. Levine and Sochett</td>
<td>Repurposing an Antibiotic to Treat a Defect in Vitamin D Metabolism</td>
<td>Drug</td>
</tr>
<tr>
<td>Rare</td>
<td>Hospital for Sick Children, National Institutes of Health</td>
<td>Drs. Dowling and Bönnemann</td>
<td>TAM4MTM: Tamoxifen Therapy for Myotubular Myopathy</td>
<td>Drug</td>
</tr>
</tbody>
</table>

* winner of CureAccelerator Live!
OUR 2019 SUCCESS STORIES

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.

Many of our funded research(ers) published, presented and raised follow-on funding during 2019. Highlights:

Research Aims:
Identified dose of repurposed drug for Phase 2 study in a rare pediatric liver disease (Nadler/Holterman)

Patient Benefit:
Repurposed drug reduced agitation in Alzheimer’s patients and reduced caregiver burden (Lanctôt)

Publications and Presentations:
Published results impacting pancreatic cancer, heart failure, Alzheimer’s, pediatric c. diff infections and the cognitive effects of prematurity

Follow-On Funding:
More than $1.7 million was raised in 2019 for next phase or related projects

Follow-On Trials:
Active planning for a follow-on trial in Alzheimer’s (Lanctôt) and the addition of a second enrollment site in myelofibrosis (Pettit)

Impacting Patients More Broadly:
The first-ever guidance in the use of fecal microbiota transplantation (FMT) in pediatric patients (Kahn)

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

Our Focus: Patient Access to Viable Treatments

- **Basic Research**
  - Animal Studies
  - CWR funding only when donor-directed

- **Late Stage Animal**
  - Final prep for next phase
  - CWR may consider funding at this stage

- **Proof of Concept / Phase I**
  - CWR's ideal funding
  - CWR funds when others say come back when you have initial human data

- **Phase II**
  - Leverage of follow-on funding
  - CWR will fund at this stage

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

- **Likely / Known Commercial Value**
  - Industry further develops proven therapies that enhance therapeutic benefit to patients
Philanthropy with faster patient impact, customized to a funder’s goals

- Donors have flexibility and control over how much or how little involvement they have during the search, selection and approval processes

Donor-advised funding can support a specific disease or geographic region

- Impact a specific disease (i.e. Huntington’s disease) or a category of diseases (any neurodegenerative disease)
- Fund a specific institution (Johns Hopkins University), region (any Chicagoland institution) or more broadly (within North America)
- Research grants starting at $50,000 can support a small, proof-of-concept human clinical trial

CWR funding often catalyzes follow-on funding from the NIH, foundations and investors.

CWR provides:

- Access to our entire 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 repurposing-specific clinical research projects
- Both project and financial progress reports during and after the project

REPURPOSING COMMUNITIES

In 2019, we launched our Repurposing Community model creating Repurposing Communities that align with our stakeholders’ strategic interests:

- Disease-specific: Oncology; Rare Diseases; and Neurology
- Geographic: Chicago; Mid-Atlantic; and the Developing World

Plus the Central Community covering the entire repurposing ecosystem

These virtual communities engage key stakeholders at both the community level as well as with our central organization. Stakeholders include:

- Patient groups and disease associations
- Universities and research institutions
- Healthcare industry and service providers
- Philanthropy
- Government / others
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 launched in 2019, we represented the Repurposing Community voice as we organized, spoke at or participated in a variety of patient education events in >5 cities in each of our disease areas and geographic areas of interest, including:

- Blood cancers in the Bay Area
- Rare diseases in Philadelphia
- Rare oncology in DC
- Alzheimer’s disease in NYC
- Diabetes in Chicago
- Repurposing off-patent drugs in DC

The Future of Patient Care in Acute Myeloid Leukemia and Other Blood Cancers

September 10, 2019

Hosted at

Jazz Pharmaceuticals

PATIENT EDUCATION

During 2019, we held 2 CureAccelerator Live! philanthropic pitch events: for the Developing World in Boston in May, and on Rare Diseases in the Mid-Atlantic region in June. This event:

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

GLOBAL HEALTH
REPURPOSING
AWARDS

2019 Janet Davison Rowley Patient Impact Research Award
Denise Faustman, MD, PhD
of Massachusetts General Hospital and Harvard Medical School
to honor her groundbreaking work repurposing a generic tuberculosis vaccine in Type 1 diabetes, including her ongoing Phase II study, and her published long-term results of the Phase I study

2019 Golan Christie Taglia Patient Impact Philanthropy Award
Alzheimer’s Drug Discovery Foundation
to honor ADDF’s leadership in cutting edge therapy discovery by advancing repurposing research. Cures Within Reach partnered with ADDF on a recently completed repurposing research project in Alzheimer's disease.

accepting on behalf of ADDF: Howard Fillet, CSO

2019 Patient Impact Industry Award
Jazz Pharmaceuticals
accepting on behalf of Jazz: Bruce Cozadd, Chairman & CEO
to honor Jazz’s focus on improving patient care via VYXEOS, utilizing proven therapies to enhance the therapeutic benefit to acute myeloid leukemia patients
This pilot program builds capacity for clinical trial research in the low and low-middle income countries (LMICs) by providing repurposing research grants to clinicians and researchers based in LMICs to find treatments for patients within LMICs. The ReGRoW funding opportunity RFP launched in late 2019 to fund at least three projects starting in 2020.

### 2018
- Finalized pilot planning and RFP
- Stakeholder outreach

### Jan-Jun 2019
- Continued stakeholder outreach
- Launched ReGRoW Pilot; exhibit at CUGH conference
- Held CureAccelerator Live! for the Developing World in May

### Jul-Dec 2019
- Signed winning project of CureAccelerator Live!
- Finalized marketing and outreach plan for ReGRoW RFP
- Launched ReGRoW RFP to LMICs
- Continued stakeholder outreach; recruit reviewers

### Jan-Dec 2020
- Review and prioritize proposals; select those qualifying for full grant applications
- Hold CureAccelerator Live! for the Developing World
- Review and select from full grant applications
- Sign and begin selected projects
### BY THE NUMBERS

**CY2019 Income By Source**
- Private Foundations, 75%
- Healthcare Industry, 10%
- Non-Healthcare Industry, 1%
- Individuals, 14%

**CY2019 Income By Donation Size**
- <$500, 80%
- $500 - $5000, 12%
- $5000 - $50,000, 6%
- >$50,000, 2%

### FUNDING PROVIDED

<table>
<thead>
<tr>
<th>Organization</th>
<th>Commercial Value</th>
<th>Payback</th>
<th>Size</th>
<th>Reviewers</th>
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<tbody>
<tr>
<td>Research Grants</td>
<td>Nonprofit academic or research institution</td>
<td>None</td>
<td>None</td>
<td>$50,000 - $250,000</td>
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<td>Research Grants with conditional payback</td>
<td>Nonprofit academic or research institution</td>
<td>Maybe / Unknown</td>
<td>Possibly fixed payback</td>
<td>$50,000 - $250,000</td>
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<tr>
<td>Impact Awards</td>
<td>For profit small businesses</td>
<td>Likely</td>
<td>Possible – linked to equity</td>
<td>$25,000 - $50,000</td>
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### New Grant / Award Commitments

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<thead>
<tr>
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<th>Amount</th>
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<tr>
<td>FY2017</td>
<td>$200,000</td>
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<tr>
<td>FY2018</td>
<td>$300,000</td>
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<tr>
<td>FY2019</td>
<td>$400,000</td>
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### Revenue Growth

<table>
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<tr>
<th>Year</th>
<th>Revenue Growth</th>
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<tbody>
<tr>
<td>FY2015</td>
<td>&lt;$500 80%</td>
</tr>
<tr>
<td>FY2016</td>
<td>$500 - $5000, 12%</td>
</tr>
<tr>
<td>FY2017</td>
<td>$5,000 - $50,000, 6%</td>
</tr>
<tr>
<td>FY2018</td>
<td>$50,000 - $250,000</td>
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<tr>
<td>FY2019</td>
<td>$25,000 - $50,000</td>
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### CY2019 Income By Source

<table>
<thead>
<tr>
<th>Source</th>
<th>%</th>
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<tbody>
<tr>
<td>Private Foundations</td>
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<tr>
<td>Healthcare Industry</td>
<td>10%</td>
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<tr>
<td>Non-Healthcare Industry</td>
<td>1%</td>
</tr>
<tr>
<td>Individuals</td>
<td>14%</td>
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### FY2017 FY2018 FY2019

<table>
<thead>
<tr>
<th>Year</th>
<th>Amount</th>
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<tbody>
<tr>
<td>FY2017</td>
<td>$500,000</td>
</tr>
<tr>
<td>FY2018</td>
<td>$700,000</td>
</tr>
<tr>
<td>FY2019</td>
<td>$900,000</td>
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</table>

### FY2017 FY2018 FY2019

<table>
<thead>
<tr>
<th>Year</th>
<th>Revenue Growth</th>
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<tr>
<td>FY2017</td>
<td>$5000 - $50,000, 6%</td>
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<tr>
<td>FY2018</td>
<td>$5,000 - $50,000, 6%</td>
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<td>FY2019</td>
<td>$50,000 - $250,000</td>
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### FY2017 FY2018 FY2019

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<tr>
<td>FY2018</td>
<td>$50,000 - $250,000</td>
</tr>
<tr>
<td>FY2019</td>
<td>$25,000 - $50,000</td>
</tr>
</tbody>
</table>
OUR LEADERSHIP

Cures Within Reach Leadership
Barbara Goodman, President & COO
Clare Thibodeaux, PhD, Director of Scientific Affairs

Cures Within Reach Board of Directors
Margaret Christie, JD, Chair, Golan Christie Taglia LLP
Lucy Mancini-Newell, Secretary, Kiran Consortium LLC
Matthew Rich, Treasurer, PwC
Kristina Allikmets, MD, PhD, Takeda Pharmaceuticals

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Tim Cunniff, PharmD, Paragon Pharmaceuticals

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Ed Kahn, Rediscovery Life Sciences
Robert Metz, previously with Horizon Therapeutics
Norbert Riedel, PhD, Aptinyx

Steve Braun, Northwestern Mutual
Elizabeth Downey, Former Chair, Retired
Nick Manusos, Fujifilm Pharmaceuticals
Robert Metz, previously with Horizon Therapeutics
Nancy Sullivan, Illinois VENTURES llc

Douglas Feinstein, PhD, University of Illinois at Chicago
Andrés Klein, PhD, Universidad del Desarrollo
Shuli Kulak, MD, BioMarin Pharmaceuticals
Mitchell Seymour, PhD, University of Michigan
Larry Sklar, PhD, University of New Mexico

Michael S. Rosen, Rosen BioScience Strategies
Adriann Sax, Orsenix
Eric Waehner, Recordati Rare Diseases
2019 COMMUNITY MEMBERS AND PARTNERS

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The Searle Funds
at The Chicago Community Trust