

# RTW Charitable Foundation 2021 Annual Report +

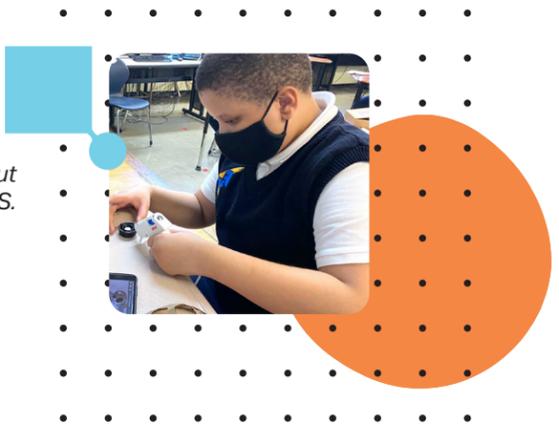


“ One thing we like to say at RTW is **‘innovation is a team sport’** and the same goes for **impact.**”

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Student learning about genetics with BioEYES.





Dear Friends,

**2021 was exactly the kind of year the RTW Charitable Foundation was built for.** The pandemic's impact here in New York was disproportionately felt among the underserved communities we're dedicated to serving. The rollout of the COVID vaccine created an urgent and highly visible need for both education and financial support. And the heroic response of the medical community opened the world's eyes to the power of scientific innovation to reduce human suffering.

**Feeling that urgency, we redirected our entire humanitarian grant cycle to focus exclusively on pandemic recovery.** We identified 12 partners in our community to support both financially – with a total of \$1 million in grants – and through the time and efforts of our people. We focused our support on organizations that serve communities of color, women, and immigrants.

For families and individuals impacted by rare diseases, 2021 was especially hard. The sense of isolation, fear, and uncertainty that is a constant feature of their lives was only magnified by the pandemic. **Our research support focuses on bringing hope to these families through the possibility of a cure.** Last year we provided \$1.4 million in grants to rare disease research teams. Our own Research Institute identified its first program focused on developing a therapy for Christianson syndrome, an ultra-rare genetic disorder. This is what we had in mind when establishing the foundation: a potentially treatable condition with a population too small to attract the attention of a traditional investor or innovator seeking a financial return. The only way to make a difference for these families is through an organization like ours. Following the science for the sole purpose of human impact.

Our already strong team grew even stronger this year by establishing new operational capabilities and bringing on additional passionate, science-focused team members. We have confidence in this organization and the foundational team we have developed. One thing we like to say at RTW is "innovation is a team sport" and the same goes for impact. **Together with our partners and community, we will continue to create health impact for our underserved neighbors and families facing rare disease.**

Sincerely and gratefully yours,

Roderick Wong, MD  
Chairman

Deborah Slipetz, PhD  
Executive Director

## Who We Are +

**RTW Charitable Foundation (RTWCF) was born at the intersection of scientific progress and humanitarian efforts. There are over 10,000 rare diseases, yet less than 5% of them have effective treatments.**

RTWCF scientists collaborate with research teams, families, and advocacy organizations from around the world to work toward treatments and improve patient access to affordable care. We are committed to supporting people living with rare diseases and underserved communities in New York City. We build partnerships with community-based organizations to respond to the urgent public health needs of our neighbors.

## MISSION

To power rare disease research, medical innovation, and humanitarian collaborations to improve the health of underserved communities.



*RTWCF and NICE at the co-created Vaccine Access Block Party in Queens.*

## Rare Disease Research +

**In 2021, RTWCF granted \$1.4M to advance the work of 10 research teams across six countries.** These teams are working on disease areas ranging from the identification of biomarkers for Bohring–Opitz Syndrome to the development of sensory prosthesis for people with no proprioception. RTWCF and its collaborators are passionate about supporting people with rare diseases to live healthier, longer lives.

## 2021 Research Grants +

- + **Murdoch Children’s Research Institute** (Nicole Van Bergen, PhD): Study of disease-causing mutations in TRAPPC4
- + **University of Utah** (Rajeshwary Ghosh, PhD): New therapeutic strategy for Desmin-related myopathy
- + **University of Pennsylvania** (David Fajgenbaum, MD, MBA, MSc): Understanding Idiopathic Multicentric Castelman Syndrome
- + **University of Utah** (Stephen Pulst, MD): Gene therapy for Christianson syndrome

- + **University of California Los Angeles** (Bianca Russell, MD): Natural-history study of Bohring–Opitz Syndrome
- + **Brown University** (Eric Morrow, MD, PhD): Translational research and natural history of Christianson syndrome
- + **NYU Grossman School of Medicine** (Gilad Evrony, MD, PhD): NYU Pediatric Undiagnosed Diseases Program
- + **Global Genomic Medicine Collaborative** (Alan Shuldiner, MD & Toni Pollin, MS, PhD, CGC): Diagnosis of rare diseases in underserved populations
- + **St. John Eye Hospital** (David Verity, MD, MA & Human Rishmawi, MD): Procurement of equipment to screen for retinopathy of prematurity
- + **Stanford University** (Allison Okamura, PhD) & **National Institutes of Health** (Carsten Bönnemann, MD): Development of sensory prosthesis for people with PIEZO2 mutations



Ophthalmologists in Gaza screen infants for Retinopathy of Prematurity.

## Making a Difference to the Diagnostic Odyssey +

For families facing rare disease, it can take 4 to 5 years to reach a diagnosis, and in some cases, it takes more than a decade. Timely and correct diagnosis gives patients and care teams a basis for accurately managing or even treating the condition. Two projects that RTWCF funded in 2021 focus on improving access to diagnosis, bringing underserved communities cutting-edge testing and clear results.



Gilad Evrony, MD, PhD

**The NYU Pediatrics Undiagnosed Diseases Program** applies novel genomics technologies to the care of children with rare diseases. Many of the patients they serve are from low-income or immigrant families, which do not typically have access to basic genetic testing. Funding from RTWCF supported the program, led by Dr. Gilad Evrony, to offer families advanced genomic testing methods, including long-read sequencing, optical genome mapping, and RNA-sequencing.

“Eleven years, I was waiting for answers. Finally, I got somebody who knows what’s going on.”

— Quote from family participating in the NYU Pediatrics Undiagnosed Disease Program



Alan Shuldiner, MD

To date, 208 individuals have been enrolled in the program. In the first six months of the grant, five people were diagnosed with extremely rare pulmonary and neurological diseases. In the same time period, the program received 40 referrals from across New York State and enrolled 35 new participants that met their criteria.



Toni Pollin, MS, PhD, CGC

**The Global Diagnosis Network: Diversity Rare Undiagnosed Diseases Program**, led by Alan Shuldiner, MD at the Global Genomic Medicine Collaborative and Toni Pollin, MS, PhD, CGC at the University of Maryland School of Medicine, is a pilot program aimed at providing families in low- and middle-income countries with access to diagnostic testing for rare diseases. Starting in Mexico, Malaysia, Sri Lanka, South Africa, Chile, and Nepal, RTWCF support is making it possible to provide clinics with the staff and equipment needed for exome sequencing, diagnostic testing, and genetic counseling.



## Joining the Bespoke Gene Therapy Consortium +

In 2021, RTWCF joined the **Bespoke Gene Therapy Consortium (BGTC)**, a collaborative network led by the Foundation for the National Institutes of Health (NIH). By bringing together government agencies, pharmaceutical companies, scientists, and non-profit organizations, the BGTC aims to streamline the development of gene therapy for people with ultra-rare genetic diseases.



## Research Institute +

In collaboration with expert research teams, RTWCF conducts rare-disease research through its Research Institute. **Working towards a world free of ultra-rare diseases, RTWCF's scientists contribute decades of drug development expertise and experimental support to identify promising treatments.**

RTWCF has established its own laboratory to support the development of gene therapies for our inaugural project focused on Christianson syndrome and other ultra-rare diseases. Our capabilities include vector optimization, small scale production of viruses, and the development of in-vitro screening and functional assays.

## Finding a Cure for Christianson Syndrome +

**Christianson syndrome (CS)** is a rare disease characterized by intellectual disability, small head size, seizures and the loss of full control of bodily movements. This X-linked genetic disorder primarily affects the brain development in boys with a symptom onset at age 2 to 4.



Eric Morrow, MD, PhD

**Eric Morrow, MD, PhD and Stephan Pulst, MD**, in collaboration with RTWCF, have made promising strides toward developing a gene therapy for Christianson syndrome. Dr. Morrow has a decade of experience evaluating patients with CS and researching the cellular and molecular biology of the disease. Dr. Morrow and his team at Brown University are conducting a natural history study, identifying the natural progression of the disease in both male and female carriers. RTWCF is supporting Dr. Morrow's efforts to create the necessary infrastructure for a multi-site natural history study of CS, including identification of clinical outcome measures and biomarkers of disease that will be critical for conducting clinical trials for any potential CS therapeutics.



Stephan Pulst, MD

Dr. Pulst and his team at the University of Utah are testing a gene therapy for CS in *Shaker* rats. The Pulst lab, including postdoctoral fellow Collin Anderson, has demonstrated that *Shaker* rats share a mutation in the same gene that causes Christianson syndrome patients and display characteristic hallmarks of the disease. Their study is the first to provide a proof-of-concept that gene therapy could attenuate the disease in a rodent model. RTWCF is working with both the Morrow and Pulst labs to optimize this therapeutic strategy and generate sufficient preclinical data to support a future clinical trial in CS patients.

*Christianson syndrome is estimated to affect about 1,500 families in the United States..*



“ A natural history study for Christianson syndrome...has the potential to vastly improve the lives of those living with the syndrome and their families and caregivers. ”

— Dr. Eric Morrow, MD, PhD

# COVID-19 Relief +

## \$1 million was granted to 12 COVID relief and research initiatives in NYC.

RTWCF launched its first grant cycle to support the urgent needs of New Yorkers in the face of the COVID-19 pandemic. From fighting food insecurity to providing small businesses with vital economic support to stay open, RTWCF partnered with 10 community-based organizations and two research hospitals to serve those hardest hit by the pandemic, namely low-income families, people with unstable housing, immigrant families, and communities of color.

“ When the COVID-19 pandemic began ravaging New York, it was instantly clear that this was where RTW Charitable Foundation could best be of service to the city we call home. We selected our grantees because they have an impressive track record, deep ties to their communities, and are nimble in responding to the needs of those they serve. We are honored to be able to partner with them and support their work, on which many New Yorkers depend for their lives and livelihoods. ”

— Dr. Roderick Wong, MD, Chairman of RTW Charitable Foundation

## Building Education Access +

**Areté Education** designs interactive afterschool and summer programs to teach students in the South Bronx about leadership skills, wellness, diverse career paths, and arts & culture.

During the pandemic, children living in temporary housing had limited access to education when schools moved to remote learning. With RTWCF’s support, Arété Education created the Arété Hope Network Program to provide direct assistance to families struggling during the pandemic. Eighteen students and families with unstable housing received stipends, groceries, hotspots, laptops, and mentoring to improve children’s attendance rates and academic performance.



Students from Arété Education share their stories at an RTWCF Day of Action.

Students’ engagement and attendance improved dramatically: all students in warning groups labeled “chronically absent” or “severely chronically absent” moved out of those warning groups during the intervention. Ninety-three percent of participants had attendance rates of 80% or higher including 50% with perfect attendance through the course of the program.

## Improving Vaccine Access +

In August 2021, some NYC neighborhoods had less than 35% of residents fully vaccinated, when the city average was roughly 55%. The New York City Department of Health and Mental Hygiene found that distrust in government and drug companies created hesitancy in vaccine uptake.



### New Immigrant Community Empowerment (NICE)

supports immigrant workers and their families by advocating for workplace safety and rights, providing skills training, and connecting families with resources. During the pandemic, NICE expanded their services to

include food access, vaccine support, and financial assistance. RTWCF partnered with NICE to distribute 10,000 meal and grocery packages, conduct comprehensive community outreach around COVID-19 vaccination safety, and throw a Vaccine Access Block Party. In 2021, NICE helped over 5,000 people access COVID vaccines by translating appointment registration documents, educating people on the vaccine, and accompanying people to appointments.

“ We say this often — RTWCF was one of the first to support our recovery efforts and helped pave the way to our impact and reimagined programs. We’re excited to reach new heights together. ” — Vanessa Marquez, VP of Development and Marketing at NICE



In 2021, RTWCF held 10 volunteer opportunities for RTW employees from painting outdoor dining structures with **Think!Chinatown** and **Send Chinatown Love** to delivering meals to homebound seniors with **New York Cares**. RTWCF has built a culture of engagement with and support for our local community!



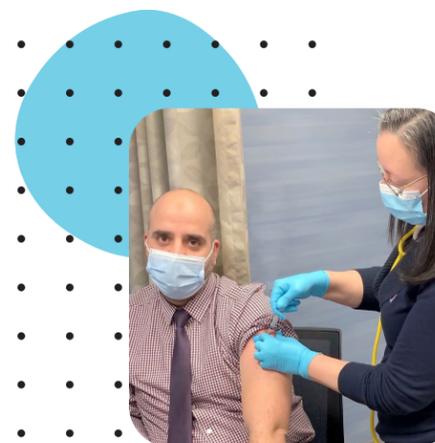
## Early Funders of COVID Research +

**Early in the pandemic, RTWCF partnered with Weill Cornell Medicine to respond to the COVID crisis in the heart of the U.S. epicenter, New York City.** Dr. Zach Grinspan and Dr. Erika Abramson acted quickly to launch a large-scale COVID-19 study aimed at understanding the epidemiology of COVID-19 in pediatric patients. They used the INSIGHT Clinical Research Network, a database that includes five major New York City academic medical centers, and created a weekly working group of statisticians and doctors from different subspecialties to utilize the data. The team launched eight studies around pediatric COVID prevalence and symptoms. Their work has been published in multiple peer-reviewed journals and presented at national conferences and meetings.



*Drs. Green and Levine with their colleagues presenting at the 2022 Pediatric Academic Societies conference.*

**Dr. Deborah Levine, Dr. Stephen Oh, and Dr. Cori Green at Weill Cornell Medicine** conducted a cross-sectional time-series analysis of pediatric mental health emergencies across the first three waves of the COVID-19 pandemic. Their research demonstrated increased proportions of pediatric mental health emergency department visits, especially during Wave 2, with the magnitude of the increase being dependent on specific mental health diagnoses and sociodemographic factors. Increases in mental health emergencies were associated with COVID-19 case prevalence, but not with the societal restrictions placed as a result of the pandemic. Their findings have been presented at two national meetings and will be published in peer-reviewed journals. Understanding the mental health needs of children throughout the pandemic informs more tailored care and policy initiatives for future infectious disease outbreaks.



*Harlem United and NICE supported vaccine access in the community.*

Post-Acute COVID-19 Syndrome (PACS), better known as Long COVID, has affected millions of people recovering from an acute infection. In February 2021, RTWCF partnered with **Icahn School of Medicine at Mount Sinai** to support their research on the pathophysiology of PACS. Dr. David Putrino and his team have published their findings identifying objective blood biomarkers associated with the condition and making recommendations for effective intervention protocols in the care of patients with PACS. The team continues to research the body's physiological response to COVID and Long COVID and care for PACS patients through their Precision Recovery Program.

## 2021 Humanitarian Grant Partners +

VACCINE ACCESS	EDUCATION	ECONOMIC SUPPORT	HOUSING	COVID RESEARCH



## Patient Support Program +

2021 saw the approval of a new treatment for Spinal Muscular Atrophy (SMA). This therapy slows the degeneration of muscle function in people affected by this rare disease.



Eddie Ndopu

**Eddie Ndopu** is a United Nations Sustainable Development Goals Advocate from South Africa living with SMA. RTWCF teamed up with Eddie to support his care and treatment in the United States. After establishing access to a care team and this life-extending treatment, RTWCF supported Eddie in securing long-term funding for this therapy. Read Eddie's powerful reflection from his first day of treatment:

“ Today was literally several years in the making. I officially became one of only a handful of young adults on the planet to get [treatment] to dramatically slow down the muscle weakness associated with Spinal Muscular Atrophy—the rare, degenerative condition y’all know I’ve been living with since the age of two. As soon as my first dose was administered...I burst into tears. And so did the other people in the room who made this day possible. Thank you, thank you, thank you to **@rtwcharitablefoundation** for the greatest set of gifts I could possibly receive: longevity, improved quality of life and greater agency. Today is the beginning of the rest of my life. More to come! ”

—Eddie Ndopu

Eddie continues to make a big impact on the world stage as an advisor to the World Economic Forum, Verizon, and The Value 500. A thought leader and activist, Eddie continues to use his voice to advocate for equity and human rights. RTWCF is proud to have contributed to Eddie's ability to live a full, empowered life.

< Rod Wong, Stephanie Sirota, Gaurav Shah, and Eddie Ndopu at RTW.

In 2021, RTWCF assembled a highly skilled team to accomplish our strategic goals. Led by pharmacologist and former executive director of imagine@Merck Deborah Slipetz, PhD, the RTWCF team includes expert scientists and non-profit leaders motivated to implement our shared mission.

## Team +



**Deborah Slipetz, PhD**  
Executive Director



**Nate Pelsma, MBA**  
Chief Operating Officer



**Joe Katakowski, PhD\***  
Director, RTW Research Institute



**Juan Carlos López, PhD**  
Manager Director,  
Research Grants



**Sarah Garwood, MA**  
Humanitarian Grants  
Manager



**Lauren Batres,**  
Executive Assistant &  
Project Manager

## Board of Directors +



**Roderick Wong, MD**  
Chairman



**Stephanie A. Sirota, MA**  
President



**Marti Speranza Wong, MBA**  
President, Community  
Engagement

\* Joined in 2022



*RTWCF volunteers painting outdoor dining structures with local business owners in Chinatown.*

## Collaborate With Us +

Reach out! We would love to hear about what you're working on in rare-disease research, advocacy, or community programming.

If you are interested in collaborating on rare disease research, contact [grants@rtwcf.org](mailto:grants@rtwcf.org). To start a conversation about humanitarian or community health programs, contact Sarah Garwood at [sg@rtwcf.org](mailto:sg@rtwcf.org).

## Join the Conversation +

**LinkedIn:** RTW Charitable Foundation

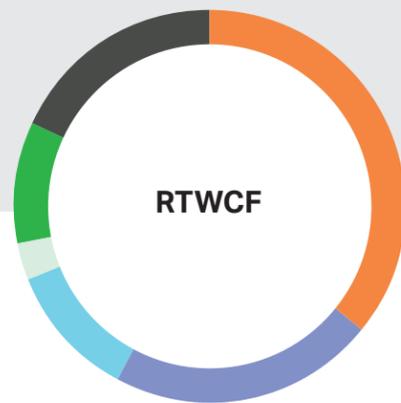
**Instagram:** @rtwcharitablefoundation

**Twitter:** @CharitableRTW

## Financial Report +

### RTW Charitable Foundation 2021 Consolidated Operating Expenses As of December 31, 2021

Research Grants	\$1,485,000.00
Humanitarian Grants	\$930,000.00
Patient Support Grants	\$476,327.00
Policy Grants	\$110,000.00
<b>TOTAL GRANTS</b>	<b>\$3,001,327.00</b>
Program-related Operating Expenses	\$412,730.50
Other Operating Expenses	\$749,614.50
<b>TOTAL EXPENSES</b>	<b>\$4,163,672.00</b>



- HUMANITARIAN GRANTS **22%**
- RESEARCH GRANTS **36%**
- PATIENT SUPPORT GRANTS **11%**
- POLICY GRANTS **3%**
- PROGRAM-RELATED OPERATING EXPENSES **10%**
- OTHER OPERATING EXPENSES **18%**

\* Humanitarian grants total includes \$800,000 in grants paid from the RTW Charitable Foundation Donor Advised Fund and are not included in the FY21 RTW Charitable Foundation audited financial statements.



RTW volunteers at a Habitat for Humanity NYC demolition day.

