RTW Charitable Foundation
2022 Annual Report

rtw Charitable Foundation

Who We Are:

Founded in 2018 as the philanthropic arm of RTW Investments, LP, RTW Charitable Foundation powers rare disease research, medical innovation, and humanitarian collaborations to improve the health of underserved communities. We provide capital, expertise, and research support to help scientists push projects forward. We partner with local organizations to improve health equity across communities in New York City.

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Brain Science Club with grant partner Hunts Point Alliance for Children's Executive Director, Natasha Atkins

Message from the Chairman

Dear Friends,

Last year was a big one for the RTW Charitable Foundation. Given the long-term impacts of the COVID-19 pandemic, we remained committed to supporting New Yorkers' health needs through vaccine education and mental health support programming. Beyond that, we focused on growing and expanding our core areas of ultrarare disease research and humanitarian support in our local New York City communities. The Russian invasion of Ukraine in February sparked the development of our first Emergency Response Grant program, allowing us to partner with the amazing organization, Razom for Ukraine, to provide health aid and resources to Ukrainian first responders. This grant program is now a fixture of our humanitarian arm and will continue to be deployed to support urgent needs beyond New York City.

We have always believed that the patient voice should be involved from the earliest stages of rare disease research, education, and policy programs. These patients and their caregivers in many ways know the diseases better than their doctors and have firsthand experience with the challenges of care and everyday life. That's why last year we established our first Rare Disease Advisory Board made up of patient advocates across a range of rare diseases. Moving forward, this group will meet regularly with our team to weigh in on a range of issues and provide their perspectives on our programming as it's being developed.

That same patient-first approach was on display at our first-ever research symposium focused on Christianson Syndrome. Our Research Institute team brought together research and advocacy partners from the Christianson Syndrome Association, the University of Utah, and Brown University for a full day of presentations, storytelling, and collaboration.

In addition to financially supporting our grant partners, we invested our time and energy into the community too. RTW volunteers stood shoulder to shoulder with ACT Care Foundation, distributing groceries to over 250 families, with Razom, packing tactical medical kits for first responders, and with our friends at Jefferies to co-host a successful fundraising event supporting Welcome to Chinatown. This is a key element of our humanitarian approach: going beyond financial support to truly engage alongside our partners in our communities.

There is far more I could say about the great work this team has done this year, but my space is limited. Please read on about the many projects our small but mighty team is driving forward. I'm so proud of their work and the mission we continue to advance with each year.

Sincerely and gratefully yours,

Roderick Wong, MD
Chairman

Overall Impact

Impact at a Glance

Ultrarare Disease Research

$1.5M+

invested in drug-discovery projects to find cures for neglected ultrarare diseases.

Research Institute

4

Designed **4** new vectors & initiated cell-based assays to evaluate AAV vector performance.

STEM Education

530+

students grades 3-8 participated in STEM education programs & 12 teachers were trained.

Bespoke Gene Therapy

8

Contributed to the advancement of innovative therapy development programs for **8** ultrarare diseases as a member of the Bespoke Gene Therapy Consortium.

COVID Response Impact

Grants

$1.5M

in support of local pandemic response initiatives across New York City.

Vaccines

10,922

people vaccinated against COVID-19, supported by nurses, translators, and community health workers.

Mental Health

112

Supported mental health training and care for **112** frontline healthcare providers.

COVID Research

Funded COVID research at Weill Cornell and Mount Sinai.

Food Security

112,100

meals provided to New Yorkers.

Housing

240

safe, improved affordable housing provided for low-income families.

Small Business Support

30

small businesses supported in Manhattan's Chinatown.

Remote Learning

70+

underserved families provided with tutoring and education support, as they navigated remote learning during the pandemic.

These figures are inclusive of our overall pandemic response efforts 2020-2022.

Joe Katakowski, PhD, Director of the RTWCF Research Institute, at JLABS

Research Institute — Christianson Syndrome Research

RTWCF established its own laboratory within Johnson & Johnson's biotech incubator, JLABS @ NYC, to support the development of gene therapies for our inaugural project focused on Christianson Syndrome (CS) and other ultrarare diseases.

Our capabilities include vector optimization, small-scale production of viruses, and the development of in vitro screening and functional assays. The Research Institute laboratory enables RTWCF to play a more active role in the development of therapeutics, offering research support to our collaborators. To date, our team has designed 4 new vectors and initiated cell-based assays to evaluate AAV vector performance.

Christianson Syndrome (CS) is a rare X-linked disease that affects brain development primarily in boys. Symptoms, including epilepsy, ataxia, and developmental delays, appear between 2 and 4 years of age, progressively worsening during the first decade of life.

Dr. Stefan Pulst and his team at the University of Utah have tested a gene therapy for CS in *shaker* rats. Pulst and postdoctoral fellow Collin Anderson have shown that *shaker* rats share a mutation in the same gene that is affected in CS patients and display characteristic hallmarks of the disease. Funded in part by RTWCF, their study was the first to provide proof of concept that a gene-replacement therapy could attenuate the disease in a rodent model. The study was published in the journal Human Molecular Genetics.

At the Research Institute, RTWCF continues to support efforts to develop and test more translationally relevant versions of a gene therapy, working toward a treatment for CS patients.

Translating Science into Therapies at the RTWCF Symposium on CS

Success in research, particularly in rare diseases, requires data sharing, communication, and collaboration. That's why in May 2022, RTWCF hosted a symposium devoted to Christianson Syndrome research.

Drs. Eric Morrow and **Judy Liu** from Brown University and **Drs. Stefan Pulst** and **Collin Anderson** from the University of Utah shared their latest discoveries and research plans with family advocates and expert scientists. Our Research Institute Director **Dr. Joe Katakowsk**i presented preclinical plans for the development of a gene-replacement for Christianson Syndrome. **Debbie Nash**, Founder and President of the Christianson Syndrome Association (CSA), shared her perspective and experience as a mother, caregiver, and advocate.

Debbie Nash during the CS Symposium

Drs. Morrow and Liu during an interview with RTW's Josh Baldwin

Patients, families and caregivers at the CSA Family Conference at Brown University

Sharing Progress and Hope at the CSA Family Conference

Continuing the theme of collaboration and partnership in research, we participated in the 2022 CSA conference sponsored by the Christianson Syndrome Assocation and Brown University in October.

The annual conference is an opportunity for patients, family members, physicians, and researchers to connect and share information. Family members shared their experience in identification of symptoms, eventual diagnosis, and their experiences and learnings from caring for children with CS. Physicians discussed their findings from clinical observations and researchers discussed recent developments in our understanding of the cellular biology of the disease and potential therapies or treatment solutions. RTWCF staff and grant recipients presented at the conference. **Dr. Eric Morrow** presented, an "Update on CS Research: Pathways to new Treatments", **Dr. Collin Anderson** presented a "Gene Replacement Therapy in the Shaker Rat Model of Christianson Syndrome" and **Dr. Joe Katakowski** presented on "Developing a gene therapy for Christianson syndrome".

An exciting theme at this year's meeting was an emphasis on new potential therapeutic approaches for CS, whereas previous meetings focused more on describing the disease's mechanisms and monitoring patients' symptoms. Parents and patients were treated to a tour of Dr. Eric Morrow's laboratory at Brown University and a visit to the Roger Williams Park (RWP) Zoo.

> "This was the first time that I have been able to speak to many family members directly affected by a disease, while also working on a potential therapeutic for that disease. I had not anticipated that I would be so affected and inspired when hearing their stories. Interacting with the families and patients was profoundly motivational and I am so grateful to have had the opportunity. I could not wait to get back to my laboratory and get to work."
>
> **– Joe Katakowski, PhD, Director, Research Institute**

Ultrarare Disease Research

Section of the mouse brain stained to reveal the localization of neurons (magenta), cell nuclei (blue) and the CTIP2 protein (green). Picture courtesy of grant recipient Joseph Dougherty (Washington University in St. Louis)

> “Ultrarare diseases represent a huge unmet medical need. We are proud to support efforts to find cures.”
>
> **- Juan Carlos López, PhD**
> **Manager Director, Research Grants**

In 2022, RTWCF granted ~$1.5M to 10 research teams in the United States and Australia. These teams are working to develop therapeutics for a wide range of ultrarare diseases that affect no more than a few hundred people worldwide. We are passionate about supporting research on such rare, neglected diseases, bringing hope to people living with these conditions.

2022 Research Grants

- **Gene therapy for IPEX syndrome.** Rosa Bacchetta (Stanford University)
- **Antisense oligonucleotide therapy for MYT1L syndrome.** Joseph Dougherty (Washington University in St. Louis)
- **RNA-based therapeutics for limb-girdle muscular dystrophy D1.** Andrew Findlay (Washington University in St. Louis)
- **Development of platform rAAV9 gene therapy products for the treatment of mucopolysaccharidosis IIID.** Haiyan Fu (University of North Carolina)
- **Translational studies of Christianson Syndrome.** Stefan Pulst and Collin Anderson (University of Utah)
- **SIGMAR1 agonists for Timothy Syndrome.** Masayuki Yazawa (Columbia University)
- **Therapeutic interventions for Bain Syndrome.** Jennifer Bain (Columbia University)
- **Development of small molecules to treat glutaric aciduria type 1.** Sander Houten (Mount Sinai School of Medicine)
- **Natural history and preclinical research into Christianson Syndrome.** Eric Morrow (Brown University)
- **Creation of a humanized model of Malan Syndrome and development of antisense oligonucleotides against this disease.** Craig McIntosh (Murdoch University) and Michael Piper (University of Queensland)

Academic Partnership to Advance Ultrarare Disease Treatments

This year we partnered with Columbia University to create the RTWCF Accelerator for Ultrarare Diseases, a 13-week event focused on educating faculty on the path to take their innovations from the lab to the clinic.

The RTWCF Accelerator was open to scholars from Mount Sinai School of Medicine and Albert Einstein College of Medicine, enabling us to extend our outreach to multiple academic institutions in New York City.

At the end of the event, the teams pitched their projects to a panel of drug-discovery experts, including RTWCF staff. We awarded grants to three projects that came out of the accelerator (see page 13). RTWCF is proud to support this work and hope that the results of this research will ultimately result in new therapeutic options for patients living with ultrarare diseases.

Bespoke Gene Therapy Consortium

In 2022, we participated in the Bespoke Gene Therapy Consortium (BGTC), providing funds and expertise to speed the development of gene therapies for ultrarare diseases.

Experts from our team contributed to the BGTC Steering Committee, making decisions on grant funding and new membership applications. On the Preclinical Subcommittee, RTWCF played a role in evaluating preclinical disease program submissions for BGTC-sponsored clinical trials and in drafting guidance for preclinical development to be presented to the Food and Drug Administration (FDA). On the AAV Biology Subcommittee, we contributed to shaping two grant cycles focused on advancing our understanding of viral vectors to improve therapeutic delivery.

A Therapy for Malan Syndrome

Malan Syndrome is an overgrowth disorder that affects the brain, bones, and other organs. RTWCF is supporting two Australian scientists working to develop a treatment for this condition.

People with Malan Syndrome are missing one copy of the NFIX gene, and the remaining copy cannot make enough protein to prevent the disease. Would it be possible to activate the remaining copy of NFIX to compensate for the missing copy? With support from RTWCF, two Australian scientists — **Craig McIntosh** (Murdoch University) and **Michael Piper** (University of Queensland) — have set out to answer this question. Specifically, they are developing antisense oligonucleotides (ASOs) to target regulatory regions of NFIX, looking for ASOs that increase protein expression from the remaining copy. The team has already identified promising ASOs. They will now evaluate their therapeutic potential in a new mouse model of Malan Syndrome, also created with a grant from the RTWCF.

RTWCF's Chief Operating Officer Nate Pelsma and Dr. Craig McIntosh during the Malan Syndrome Family Conference at the University of North Carolina

Rare Disease Advisory Board

In 2022, we assembled an advisory board of leaders with lived experience in rare disease. The group provides feedback on RTWCF education initiatives and is executing projects relevant to the unique needs of the rare disease communities with whom they work.

The RTWCF team with the Rare Disease Advisory Board

Dani Farber

Dani is an Associate Portfolio Manager with Schonfeld and Vice-President of the Kleine Levin Syndrome (KLS) Foundation. With RTWCF support, he is improving the KLS Patient Registry to increase patient participation, improve data and our understanding around the natural history and symptoms of KLS, and connect more patients and families with a community of support.

Cristina Cassanova Might

Cristina is the Founder and CEO of Welcomed Co, a lifestyle brand that curates and creates beautiful, humanizing products, content and services for accessibility and inclusion – enabling people of all abilities and ages to live their best lives. She serves on the Board of Directors for the EveryLife Foundation for Rare Diseases and the Alabama Assistive Technology Act Council.

Jenny Hsieh

Jenny is the Chief Strategy Officer at Actinium Pharmaceuticals and the Co-Founder and President of the Danon Foundation. The Danon Foundation is creating a guidebook with key information and resources on how to manage day-to-day life with Danon Disease, tailored to different audiences, including healthcare providers, patients, and caregivers.

Danielle Kinsey

Danielle is a Surgery Resident at the University of Missouri-Kansas City, University Health Truman Medical Center. Combining her knowledge as a trained medical provider and her passion for poetry, Danielle is developing a children's book for families living with rare diseases.

Humanitarian Program

RTW volunteers at a food distribution event with ACT Care Foundation

As the immediate threats of the COVID pandemic waned, long-term social and economic challenges continued to affect underserved communities across New York City. We listened to our grant partners' priority needs and focused on local health-oriented programs to provide support around COVID vaccine education and access, mental health, and food security.

2022 Humanitarian Grants

COVID Vaccine Education & Access
- ACT Care Foundation
- Harlem United
- New Immigrant Community Empowerment (NICE)

STEM Education
- BioEYES

Mental Health & Wellness
- Association to Benefit Children (ABC)
- African International Collaborative Center (AICC)
- Hunts Point Alliance for Children
- NYU Langone
- Violence Intervention Program

Emergency Response
- Razom

> "We work with some incredible, compassionate local leaders who pour their time and energy into positively impacting peoples' lives, easing barriers to accessing quality healthcare, and building community. It's rewarding to support, learn from, and collaborate with this dedicated network of changemakers."
>
> **– Sarah Garwood, MA**
> **Assistant Director, Humanitarian Program**

RTW volunteers during an ACT Care Foundation food drive

Supporting Local Food Security and Community Health Workers

Throughout the pandemic, ACT Care Foundation leapt into action distributing fresh food and PPE to low-income families across Flatbush, Brooklyn.

As a member of the NYC COVID-19 Vaccine Equity Partner Engagement network, they provided resources, booked vaccine appointments for community members, and connected families with other social services. In the first six months of our partnership, ACT was able to increase their COVID education events from one to four times per week, providing COVID vaccines information to 26,000+ people. Through their community health workers and outreach, they booked vaccine and booster appointments for 2,167 people. They also had an impressive impact in meeting families' food needs, increasing their food distribution events from two to four times per month and providing 17,100 people with fresh produce and healthy food to nourish their families.

RTW staff with BioEYES Program Coordinator Auset Taylor and teacher Alex Gordon

> "The program was a terrific chance for students to use authentic lab techniques and scientific reasoning."
>
> **– Alex Gordon, BioEYES teacher**

Inspiring Young Scientists

The University of Pennsylvania's BioEYES program has been providing lab experiences and science education programming to students at no cost for the past 20 years.

In partnership with RTWCF, BioEYES is now benefiting underserved students in New York City for the first time. BioEYES and The Center for Excellence in Youth Education at Icahn School of Medicine have recruited and trained 11 teachers and reached 510 students in Harlem and Upper Manhattan public schools over the past year. Students from grades 3 to 8 are guided through age-appropriate genetics labs, observing zebrafish with microscopes in their classrooms. In the upcoming year, they aim to reach a total of 1,860 students, train more teachers, and introduce on-site summer sessions and research opportunities for high school students and teachers. 2022 marked our second year partnering with BioEYES and Mount Sinai Icahn School of Medicine to support STEM education for students in East Harlem.

Responding to Urgent Health Needs in Ukraine

In 2022, we acted quickly to meet the urgent health needs of Ukrainians.

Razom, meaning “together” in Ukrainian, is a nonprofit organization based in New York City led by Ukrainians and Ukrainian-Americans dedicated to supporting a more prosperous and democratic Ukraine. RTWCF partnered with Razom in March 2022 to support their Emergency Response Program, delivering critical first aid and medical supplies to first responders and hospitals in Ukraine.

Our contribution provided 14,000 tactical medical backpacks and 38,000 bandages, tourniquets, and burn and wound dressings. RTW staff also volunteered at Razom’s warehouse in July, packing tactical medical kits to be sent to Ukraine. As the war continues into its second year, we are proud to support Razom and their expansive work to meet the needs of Ukrainians.

> “RTW Charitable Foundation’s partnership has enabled us to provide medical and humanitarian aid and support vulnerable citizens during this ongoing crisis. As a network of Ukrainians and Ukrainian-Americans with strong ties to Ukraine, this work is personal to us. Having partners who understand that and who support our work helps us provide life-saving medical supplies to those who are defending our country and victims of the unprovoked Russian invasion.”
>
> **– Dora Chomiak, President, Razom Board of Directors**

RTWCF volunteers with the Razom team

Raising Funds with Welcome to Chinatown

During the pandemic, Asian American and Pacific Islander New Yorkers were hit hard by the economic downturn and rise in anti-Asian discrimination. In May 2022, we collaborated with Welcome to Chinatown and Jefferies to celebrate AAPI history month and support small business owners in Manhattan’s Chinatown.

With our partners, we raised over $350,000 to provide grants to business owners and fund innovative initiatives through Welcome to Chinatown’s Longevity Fund.

So far, 10+ businesses have been supported, including businesses affected by the Mulberry Street fire. Funds were used to revitalize the 88 East Broadway Mall, an effort to bring life & business back into a historically vibrant commerce center for Chinatown. Our support enabled Welcome to Chinatown to hire an AAPI design and research studio to develop the schematic design for the new Small Business Innovation Hub Welcome to Chinatown is establishing. They are also launching the Longevity Fund Acceleration & Incubation Program for new businesses in Chinatown as a response to the findings of their Chinatown Impact Study.

RTWCF, Jefferies, and Welcome to Chinatown at the fundraiser

RTW volunteers and community partners at Day of Action Health Fair

"At RTWCF we're focused on improving the health of underserved communities whether that's through partnerships with local organizations taking on health equity challenges in their communities or researching therapeutics for patients and families facing ultrarare diseases. We're honored to collaborate with strong non-profit leaders and scientists across New York City and beyond."

– Marti Speranza Wong, President, Community Engagement

Team

Nate Pelsma, MBA
Chief Operating Officer

Juan Carlos López, PhD
Manager Director, Research Grants

Joe Katakowski, PhD
Director, Research Institute

Sarah Garwood, MA
Assistant Director, Humanitarian Program

*We're grateful to **Deborah Slipetz** and **Lauren Batres** for their contributions to the Foundation in 2022.

Board of Directors

Roderick Wong, MD
Chairman

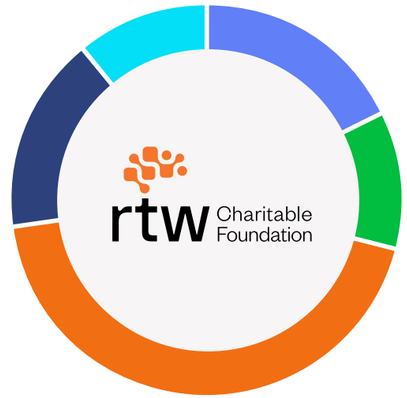
Stephanie A. Sirota, MA
President

Marti Speranza Wong, MBA
President, Community Engagement

Financial Report

2022 Functional Expenses
As of December 31, 2022

Research Institute	829,148
Research Grants Program	2,160,192
Humanitarian Program	901,232
Policy Institute	495,749
Management & General	538,614
Total	**$4,924,936**



- Research Institute – **17%**
- Research Grants – **44%**
- Humanitarian Program – **18%**
- Policy Institute – **10%**
- Management & General – **11%**

* Unaudited financial statements for 2022

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