

2023 Annual Report



Our Mission:

We power community initiatives and scientific research to improve the health of underserved populations.

Table of Contents

04	—	Message from the Chairman
06	—	Overall Impact
08	—	Community Engagement
10	—	Grant Partners
14	—	BioQuest Mentoring Program
24	—	Days of Action & Community Partners
26	—	Ultrarare Disease Research
30	—	Research Grants
32	—	Advisory Services
34	—	Research Partners
38	—	Team & Board of Directors
39	—	Financial Report

Message from the Chairman

Dear Friends,

2023 was a year of growth and consolidation for the RTW Foundation. While remaining true to our mission to improve the health of underserved populations, we have sharpened our focus on two areas: Community Engagement and Ultrarare Disease Research. We believe that these are the areas in which we can make the biggest impact through our expertise and financial resources.

The Foundation remains committed to supporting the health needs of New Yorkers, and as we emerged from the COVID pandemic, we turned our attention to long-term health-equity challenges of underserved communities across New York City. Our Community Grants reflect this strategy, and we are honored to support our partners to fulfill their mission.

In addition to this grant support, we continued to work alongside our local partners during our Days of Action, co-creating health fairs, distributing groceries, and raising awareness about a variety of social services available to New Yorkers. Days of Action continue to be a key element of our approach: going beyond financial support to contribute with our time and energy to the growth of our communities.

One Community Engagement program that I'm particularly excited about is BioQuest — a new STEM mentoring program that we are piloting through the 2023–2024 academic year. Through BioQuest, mentors from the Foundation, RTW Investments, and RTW-incubated company Yarrow Biotechnologies share their passion for science and research

with middle- and high-school students in the Bronx, empowering future innovators. The program, a partnership with Areté Education, Hunts Point Alliance for Children, BioBus, and Weill Cornell Medicine, involves lab visits, career education, innovation competitions, and hands-on science experiments. BioQuest is off to a great start and will be a staple of our Community Engagement programming for years to come.

The Foundation's commitment to ultrarare-disease research also remains unwavering. In 2023, we focused our strategy on two fronts: funding drug-discovery projects for ultrarare conditions and providing mentoring and advisory services to disease foundations interested in the discovery of treatments and cures.

Our global Request for Research Proposals was a great success, attracting nearly 100 projects from 14 countries. We are delighted to support eight research groups on their mission to deliver therapies and new hope to patients and their families.

Speaking of families, they are often the spark that ignites the quest for therapies for their loved ones. Family-run disease foundations are an increasingly important part of the drug-discovery ecosystem, and we want to recognize and foster their contributions. To that end, we have put our drug-discovery expertise to the service of disease foundations, providing them with free, unbiased advice on their journey towards finding cures. Our initial experience has been incredibly positive, and we plan to expand this program to increase our impact.



I encourage you to read the entire report and learn about all these initiatives. Your support and partnership are what make this possible. I'm very proud to see how the RTW Foundation team has embraced our community and promoted the advancement of science. I look forward to seeing what, together, we will achieve in 2024.

Sincerely and gratefully yours,

Roderick Wong, MD
Chairman

Overall Impact

These figures are inclusive of our overall efforts 2021-2023.

Access to Care

 **80,000+**

New Yorkers with improved access to care.

 **61,000+**

with improved health literacy.

 **2,348**

received mental health support, including **908** front-line healthcare workers.

 **11,500**

vaccinated against COVID-19.

Quality of Care

 **205**

Provided training and salary support to **205** healthcare workers to provide linguistically and culturally relevant integrative health & mental health care in New York and beyond, improving the quality of care accessible to underserved communities.

 **3**

community health publications highlighting research conducted by our grant partners at Weill Cornell and Mount Sinai.

 **\$1.16M**

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

 **12**

Provided drug-discovery advice to **12** disease foundations working to develop therapies for their patient populations.

 **8**

Our efforts in the Bespoke Gene Therapy Consortium helped lead to the selection of a final **8** clinical programs and **8** collaborative research grants launched in 2023.

Publications:

Acker KP, Deborah A. Levine, Mathew Varghese, Katherine A. Nash, Arindam RoyChoudhury, Erika L. Abramson, Zachary M. Grinspan, Will Simmons, Alan Wu, and Jin-Young Han. 2022. "Indications for Hospitalization in Children with SARS-CoV-2 Infection during the Omicron Wave in New York City" *Children*. 9(7): 1043. <https://doi.org/10.3390/children9071043>

Narayanan N, Samantha Langer, Karen P. Acker, Steven D. Rosenblatt, Will Simmons, Alan Wu, Jin-Young Han, Erika L. Abramson, Zachary M. Grinspan, Deborah A. Levine. 2023. "COVID-19 is Observed in Older Children During the Omicron Wave in New York City". *The Journal of Emergency Medicine*. 64(2): 195-199. <https://doi.org/10.1016/j.jemermed.2022.09.036>

Tabacof L, Putrino D, et al. 2022. "Post-acute COVID-19 Syndrome Negatively Impacts Physical Function, Cognitive Function, Health-Related Quality of Life, and Participation". *American Journal of Physical Medicine & Rehabilitation*. 101(1): 48-52.

Community Engagement

BioQuest student extracting DNA from strawberries with mentors at our Johnson & Johnson lab





As we emerged from the pandemic, we shifted our focus from COVID response and recovery toward addressing long-term health equity challenges affecting underserved communities in New York.

Health equity is the perspective that all people deserve full, healthy lives and that we can and should minimize disparities in health and access to quality care. At RTW Foundation, we partner with local organizations to address disparities in care through evidence-based initiatives at both community and systemic levels.

underserved communities in a variety of ways. The community-based services we support include free dental, blood pressure, and glucose screenings, street-based treatment for substance use disorder, and linguistically and culturally relevant mental health resources. Our partners also provide appointment incentives, mobility & transportation support, and referrals to low- or no-cost care options..

Our grant partners across New York City are improving access to care for

Left: A BioQuest student and her family enjoying the Back-to-School Fair in Hunts Point
Right: RaisingHealth providing free health screenings at community health fair



Community Grant Partners

Community member receiving free glucose test at a Mixteca health fair

Meet the Partners

Providing Street-based Care to Patients with Substance Use Disorder

NYC had a record high rate of overdose deaths in 2023. With our support, Harlem United's Street-based Care Team added a nurse practitioner and expanded services to include wound care, vaccines, Buprenorphine treatment, HIV testing, and referrals to additional services.

Over five months the team far exceeded their goals, completing 1,409 outreach contacts, helping 50 clients begin Buprenorphine treatment (with 60% engaging in regular maintenance), and distributing 1,478 health & naloxone kits.



Above: Harlem United's street-based team providing care to community member

Empowering Latin-American Immigrants Through Community Health Services

Mixteca is also exceeding their goals through broad-reaching monthly community health events.

In six months, they've reached 1,886 people through their events, providing free HIV, dental, and diabetes screenings, flu vaccinations, and resources on topics like health insurance and preventative care.



“ This health fair has been a lifeline for us. As immigrants, navigating the healthcare system can be daunting, and having these resources available in our community makes a world of difference...Their efforts truly empower us to take charge of our well-being, and we are immensely grateful. ”

– Community member & asylum seeker



Above: The RTW Holistic Integrative Health Nursing Training Program graduation
Bottom Left: Mixteca team member providing free PPE at a community health event

Introducing New Integrative Care Training to Nurses at NYU Langone

With our support, NYU Langone developed and piloted the RTW Holistic Integrative Health Nursing Training Program with 10 fellows from various high-need and high-risk specialty areas across NYU Langone Health including Labor & Delivery, Oncology Radiation, Emergency Department, Operation Room, the Post-Anesthesia Care Unit, Psychiatry, and Transplant.

Fellows were trained in non-pharmacological holistic integrative health approaches, self-reflection, self-care, and mentorship to address the challenges of prolonged stress, burnout, and compassion fatigue among medical professionals and their patients. Fellows received over 200 hours of intensive mentorship and each fellow launched their own applied programs in their departments after completing the program.

Their projects have had outsized impact, reaching 618 fellow nurses, 18 nursing leaders, and 70 patients across four NYU Langone Health campuses.

“ I am so proud of myself and all I accomplished during the fellowship! My heart is full because several of my oncology patients have used the breathing techniques I taught them while at home with their families and it helped them all with their stress. ”

– Rosanna Schifilliti, RN, BSN

2023 Community Grant Partners

Take a look at the initiatives our 2023 grant partners are implementing to improve health access in their communities across New York City.

East Harlem Health Outreach Partnership (EHHOP)

EHHOP is a free clinic in East Harlem run by Mount Sinai medical students. With this grant they will increase access to primary care and specialist visits by providing transportation support, financial incentives, and medical equipment to patients with mobility limitations.

Hamilton-Madison House

Hamilton-Madison House is providing a yearlong fellowship for a postdoctoral psychology graduate, to improve the pipeline of mental health professionals serving the pan-Asian-American community and to expand access to behavioral health services for an additional 30-40 patients at their clinic.

Harlem United

Harlem United is providing low-threshold medical services for people with opioid dependency through their street-based medicine team. With this grant they have expanded their team and the services they provide, including Buprenorphine treatment.

Mixteca

With this grant, Mixteca is providing monthly community health events for the Latin-American community in Brooklyn. Events include free health screenings, resources in multiple languages, and health education workshops.

RaisingHealth Partners

Through the Community Health Clinic Pop-Up Program, RaisingHealth is hosting free monthly health clinics and workshops for low-income immigrant families in Sunset Park, Brooklyn. Clinical partners and multilingual community health workers provide resources, screenings, and referrals to community members.

Razom

The Razom Ukrainian Response Initiative is hosting monthly health events to provide Ukrainian refugees in New York with first aid items and emergency health funds before community members are eligible for insurance coverage.

Weill Cornell Medicine

Weill Cornell is analyzing patient data across the pediatric and epilepsy units to develop and implement quality measures to improve the gaps in care across race and insurance status, contributing to systematic improvements and more equitable care.

Syrian American Medical Society (SAMS) Foundation

SAMS Foundation received our 2023 Emergency Response Grant to respond to survivors of the severe earthquakes in Turkey and Syria in February 2023.

2023 STEM Education Partners

BioBus

BioBus provides diverse opportunities for students grades K-12+ in New York City to discover, explore, and pursue science. Through their mobile lab and explore programs, we've supported BioBus to reach an additional 815 students so far. BioBus has also been an integral collaborator in the co-creation of our STEM mentoring program, BioQuest.

BioEYES

BioEYES has been providing K-12 science education programs in Philadelphia since 2002. With our support and in partnership with The Center for Excellence in Youth Education at Icahn School of Medicine, BioEYES has expanded to NYC and reached over 1,400 students in East Harlem with their genetics labs for grades 3-8.

Rutgers New Jersey Medical School

Rutgers New Jersey Medical School provides a scholarship annually through the International Health Education and Learning (iHEAL) program. Scholars have traveled to Uganda and Ghana to conduct research and provide community health support in diverse low-resource contexts.

BioQuest students with mentor Ovid Amadi at RTW's first career day



In 2023, we launched BioQuest, a STEM mentoring program, to leverage our collective expertise and inspire students to pursue career paths in biotechnology, medicine, and science.

Over the past year, over 75 middle school and high school students engaged in hands-on experiments and activities alongside 21 mentors from RTW Investments, RTW Foundation, and Yarrow Biotechnologies.

This program was co-created with Areté Education, Hunts Point Alliance for Children, BioBus, and Weill Cornell Medicine. We are grateful for the insights, time, and work

that these collaborators have contributed to make this program come to life! Given the overwhelmingly positive feedback from students, mentors, and partners, we look forward to scaling BioQuest in year two, reaching additional students and further preparing students to be successful in STEM fields. Read on to learn more about the program and please reach out if you are interested in being involved with BioQuest!

BioQuest Mentoring Program



Student exploring hands on science activities with BioBus at the BioQuest Kick-off Event

BioQuest Collaborators



Areté Education

Areté Education provides academic, social, and emotional support to students while working hand in hand with their schools. Arété extends the traditional school day with learning experiences that are fully integrated into school communities.



Weill Cornell Medicine

For over 100 years, Weill Cornell Medicine has been a community of world-class physicians, researchers and educators committed to excellence in patient care, scientific discovery and the education of future physicians in New York City and around the world.



Hunts Point Alliance for Children

Hunts Point Alliance for Children (HPAC) aims to expand the hopes and potential of Hunts Point children—nurturing young scholars along their path to college or career by unifying a diverse collective of community organizations to connect every family with the resources they deserve.



BioBus

BioBus helps NYC students K-12+ to discover, explore, and pursue science. They focus on students excluded from the scientific community due to factors such as race, gender, economic status, and physical access.



BioBus Chief Scientific Officer, Latasha Wright, guiding BioQuest students through a microscope activity on their mobile lab



RTW Team with collaborators from Arété Education and Hunts Point Alliance for Children

Explore the Inaugural Year of BioQuest

Sep

Program Launch

Students and their families gathered together with BioQuest mentors and collaborators for a Kick-Off Event in September. Everyone enjoyed breakfast, participated in a scavenger hunt activity, and learned more about the new STEM mentoring program. After the launch, students and mentors explored hands-on microscope activities with BioBus at the Back to School Fair in Hunts Point.



Nov

Lab Day at Johnson & Johnson

BioQuest teamed up with Johnson & Johnson Innovation – JLABS to create a unique experience for middle school and high school students from the South Bronx on November 2nd. For the first time ever, JLABS @ NYC hosted students to explore their eye-catching incubator space for startup companies in the life sciences and healthcare industry. BioQuest mentors led hands-on lab activities including extracting DNA from strawberries, and team pipette racing. Students got to explore the space, engage with their mentors, and meet the Head of JLABS US Northeast. Many of these students had never been to an incubator space before and this event was an inspiring experience for them to consider a potential future career in the life science industry.



Mar

Innovation Day at Weill Cornell

BioQuest students designed solutions to community health challenges in teams with their mentors. Students pitched their innovative ideas and "invested" in the most impactful proposals.



“

The most exciting part of BioQuest was getting out of my comfort zone and trying something new! ”

– Ashley, 9th grade



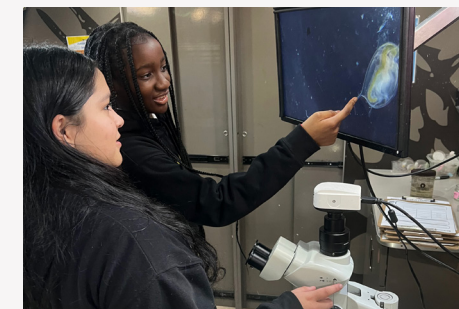
RTW Career Day

RTW Investments hosted its first-ever career day as a part of the inaugural year of BioQuest. RTW Investments is a life science investment and innovation firm dedicated to solving the most challenging, unmet patient needs. RTW originates companies, fuels growth, and nurtures the most promising science and medicine. Students learned about career paths in biotech and science, participated in activities with their mentors, met our CEO, and explored the investment firm offices.

“

I liked how I learned about things I haven't before like the DNA extraction. I want to learn more about the staff. All of them are cool. ”

– Gilbert, 6th grade



Experiments with BioBus

Students conducted experiments using the scientific method alongside their mentors and the BioBus team. Participants explored single and multicellular organisms using microscopes and other hands-on tools aboard the BioBus. The excitement and discovery were palpable!



Liberty Science Center + Graduation Celebration

Students, mentors, and collaborators enjoyed a day at Liberty Science Center. The day concluded with a graduation celebration for students who participated in our inaugural year of the BioQuest program!

Oct

Feb

Apr

BioQuest students and mentors at Johnson & Johnson's innovation space, JLABS



“

I felt intrigued during the whole thing. I learned how science can be mixed with investing and the impact of combining them. I want to learn more on what else investing can have a mix with. ”

– Manuel, 11th grade

BioQuest students and mentors on RTW Career Day



“

I loved how we were able to do hands on activities. I wish we could have stayed longer! I learned what a pipette is and how to use it. I also learned how to extract DNA. I wish I could come back every week! ”

– Emily, 7th grade

RTW volunteers at the summer Day of Action Health Fair in Sunset Park, Brooklyn



Right: RTW volunteers, partners, and community members at Health Fairs in Brooklyn

Days of Action

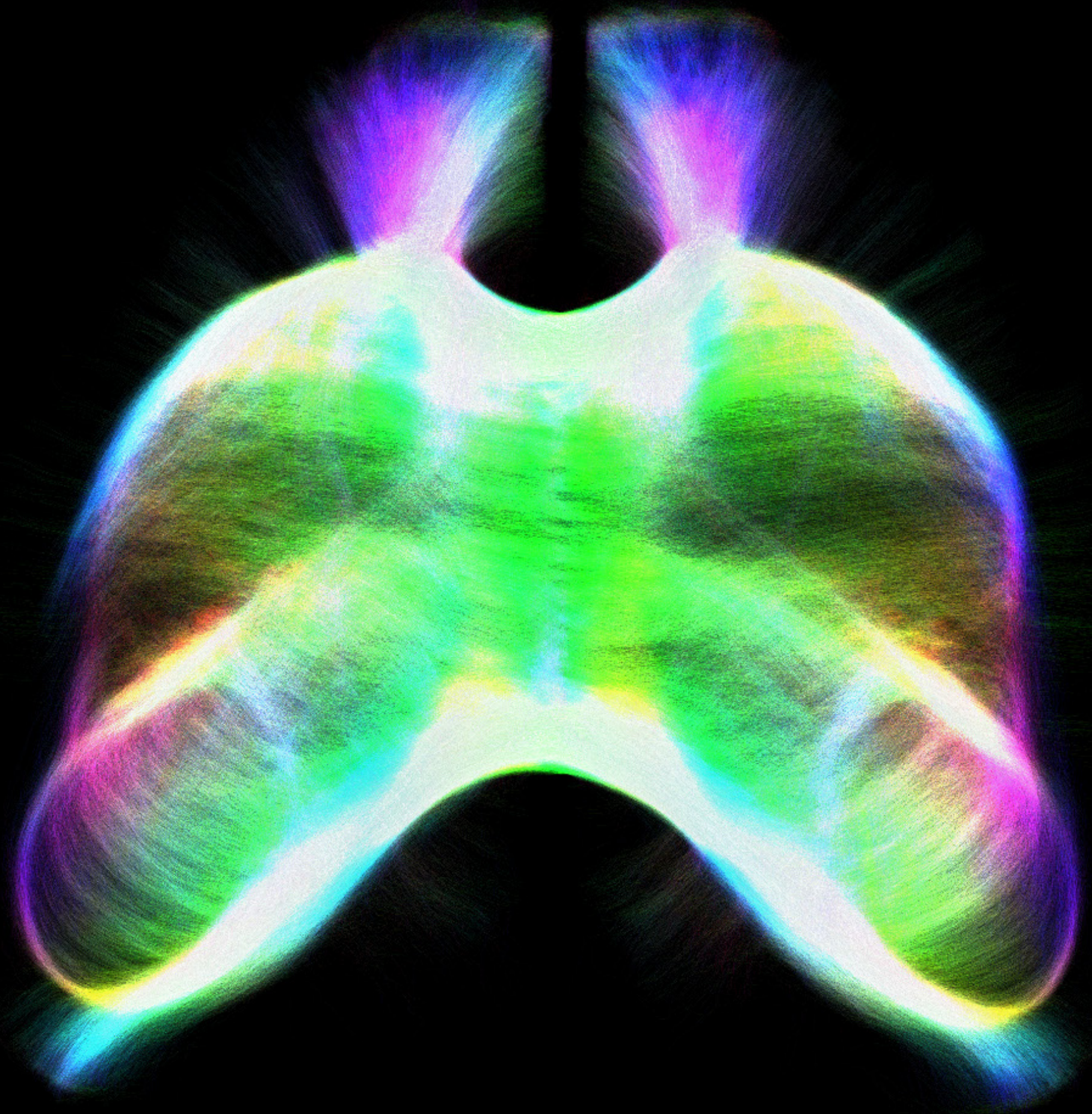
In the spirit of partnership and collaboration, we co-created Community Health Fairs in East Flatbush and Sunset Park to promote access to low- and no-cost health resources, provide free services, and distribute fresh groceries to families in the community.

With ACT Care Foundation and Assemblywoman Monique Chandler-Waterman's team, we raised awareness about free mental health resources, provided a high-energy fitness demo, and gave out PPE and groceries for 250 people.

We collaborated with RaisingHealth Partners, Send Chinatown Love, and Mixteca to host a pop-up health fair in Sunset Park. Families from the neighborhood and new immigrant families enjoyed music, kids' activities, and fresh groceries to take home for the week ahead. Free hypertension screenings were available alongside resources on a variety of free and low-cost health and social services. Community health workers gave presentations on hypertension and NYC Care resources in English, Spanish, and Chinese.



Ultrarare Disease Research



Diffusion tensor MRI of an adult mouse brain.
Courtesy of Justin Jones and Michael Piper,
University of Queensland



Navigating the drug-discovery pathway is challenging, and these challenges multiply in the context of ultrarare diseases. As the mission of RTW Foundation is to aid in the development of therapies for people living with neglected ultrarare conditions, we have evolved our strategy to maximize our impact.

We have had the privilege to interact with many stakeholders in the ultrarare disease community, gaining an understanding of the challenges that they encounter while striving to bring new treatments to the clinic. With this insight, in 2023 we directed our focus toward two obstacles hindering the progress of new therapeutic options for patients living with ultrarare conditions.

First, we focused our grant-making strategy to prioritize projects that have reached, in drug-discovery parlance, the lead-optimization stage. In other words, we solicited proposals from scientists who had already obtained proof-of-principle that their chosen therapeutic modality was beneficial in an animal model of their targeted disease, as we think that these projects have a higher probability of success in transitioning into clinical trials. We were very impressed by the quantity and the quality of the proposals that we received, underscoring the profound interest that ultrarare disease research evokes among academic scientists worldwide.

Second, many ultrarare disease foundations have been established by parents of children living with these conditions. Although these foundations can be very effective

in raising funds for research, they often lack the scientific expertise necessary to deploy that capital in an effective way. We therefore launched a pilot program to provide guidance and mentorship to disease foundations interested in therapeutic development, sharing our expertise in drug discovery.

Through this program, we have been assisting disease foundations in understanding drug development processes, identifying critical gaps in understanding their pathology of interest, selecting the most suitable therapeutic modality for the condition in question, and more. Our goal is to help these disease foundations develop a roadmap that enhances their effectiveness and facilitates the delivery of therapies for their loved ones.

Therapeutic development for ultrarare diseases stands as one of the most dynamic areas in drug discovery. It demands innovation across multiple domains, including clinical trial design, financing, regulatory science, and beyond. We welcome the opportunity to contribute to this vibrant community and look towards a future free of neglected ultrarare diseases.

Our Research Strategy

Left: A co-stain of CALB1 in brown, showing Purkinje cells, and a Nissl stain in blue in the cerebellum, taken from a healthy rat, sectioned, stained, and imaged at 20x by Kajal Dahiya (a graduate student in the lab of Dr. Collin Anderson at the University of Sydney).

Therapeutic Development with our Partners

In 2023, RTW Foundation granted ~\$1.16M to 10 research teams in the United States, Canada, United Kingdom, and Australia. These teams are working to develop therapeutics for a wide range of ultrarare diseases, each of which affects no more than a few hundred people worldwide. We are passionate about our mission: supporting research on rare, neglected diseases to bring hope to people living with these conditions.



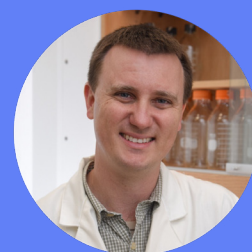
Establishing gene replacement therapy for NEDAMSS, an ultra-rare neurological disorder caused by mutations in the IRF2BPL gene

Kathrin Meyer, PhD
(Nationwide Children's Hospital, Columbus, OH, USA)



Optimizing AAV-SPL gene therapy for sphingosine phosphate lyase insufficiency syndrome

Julie Saba, MD, PhD
(University of California, San Francisco, CA, USA)



Gene therapy for SURF1-related Leigh Syndrome

Steven Gray, PhD
(The University of Texas Southwestern, Dallas, TX, USA)



Safety and toxicity assessment of AAV-based gene therapy for creatine transporter deficiency

Jagdeep Walia, MD
(Queen's University, Kingston, Ontario, Canada)



Advanced therapeutics for SELENON-related myopathy

Alan Beggs, PhD (Boston Children's Hospital, Boston, MA, USA)



Preclinical evaluation of an AAV vectorized gene therapy for Christianson syndrome in shaker rats

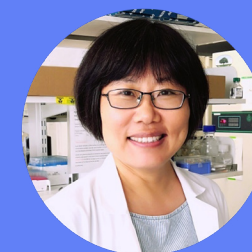
Collin Anderson, PhD
(University of Sydney, NSW, Australia)



Development of antisense oligonucleotides and creation of a humanized model of Malan Syndrome



Craig McIntosh, PhD (Murdoch University, WA, Australia) and Michael Piper, PhD (University of Queensland, QLD, Australia)



Preclinical studies of ASO therapy for SPTLC1-related hereditary sensory neuropathy

Haiyan Zhou, MD, PhD
(University College London, London, UK)



What is Antisense Oligonucleotide (ASO) therapy?

ASO uses tiny pieces of genetic material to stop or change specific instructions in cells. These genetic sequence can act as a blocker on a message to stop it from causing harm or to make it helpful instead.



What is Adeno-associated virus (AAV) vectorized gene therapy?

AAV uses a harmless virus to deliver helpful genes into the body, fixing genetic disorders by providing missing proteins to the cells that need them.

Advisory Services



The therapeutic journey for many ultrarare diseases starts with a diagnosis that families often have great difficulties making sense of. Parents start looking for support groups and foundations that work in the interests of their children. Unfortunately, many disease foundations lack the knowledge and expertise to decide on what might be the best therapy for the condition that affects their patient population.

At RTW Foundation, we are very sensitive to the ordeal that it means to embark in a drug-discovery project fresh from emerging from the diagnostic odyssey that many families go through. For this reason, we have launched a pilot program in which we meet with disease foundations to provide them with free, unbiased advice on drug development. The goal of this program is to make sure that the grassroots capital that these disease foundations raise looking for cures is deployed in the most effective way possible. In 2023, we had the privilege of interacting with a variety of disease foundations including:

Those at later stages may require advice related to preclinical study designs or other advice to achieve POC with a potential therapy. Those at later stages may need advice regarding manufacturing readiness, safety & toxicity studies, or advice regarding the IND submission process. The main goal of our Research Pillar is to advance the development of therapies for neglected rare diseases by meeting each foundation where they are and empowering them to advance their research objectives to the next stage. We hope that this program will provide much needed support to the many families affected by these conditions worldwide.

- The Yaya Foundation for 4H Leukodystrophy
- The FOXG1 Research Foundation
- CTNNB1 Connect and Cure

In 2024, we intend to expand this program to reach as many organizations as possible including those at different stages of development. Those foundations in the early stages of research might need advice related to the organizational structure and operations of a foundation, assistance on research reviews or landscape analyses, or tutorials in conducting a grant cycle.



Yaya Foundation Family Conference at Children's Hospital of Philadelphia, October 2023

“ Since its inception, The Yaya Foundation for 4H Leukodystrophy has navigated a complex scientific roadmap towards a cure. The RTW Foundation has provided profound insights that have equipped us with a different perspective and approach. ”

– Ben Smith, Executive Director
The Yaya Foundation for 4H
Leukodystrophy

BGTC – Bespoke Gene Therapy Consortium

RTW Foundation has been a non-profit sponsor of the Bespoke Gene Therapy Consortium (BGTC) since 2022.

Experts from our team actively serve on multiple BGTC committees, providing insights to speed the development of gene therapies for ultrarare diseases. These efforts benefit not only the commercial organizations pursuing gene therapies, but more importantly the academic researchers or patient foundations pioneering these ground-breaking therapies.

BGTC Committees on which RTW Foundation actively serves:




- **Steering:** Make key decisions on clinical program readiness, funding, and BGTC membership.
- **Preclinical:** Evaluated 62 disease programs, funding 8 for clinical development.
- **AAV Biology:** Funding 8 grants to advance our understanding of AAV viral vectors.
- **Manufacturing:** Draft recommendations to streamline AAV manufacturing and quality control.
- **Joint (clinical programs):** Coordinates across all 8 clinical programs.



And bring some magic to a world where it is rare.

Embrace your imperfections.

Join hands with others who are unique.

Disease Type	Disease Name	Affected Gene
 Ocular	Congenital Hereditary Endothelial Dystrophy (CHED)	SLC4A11
	Retinal Degeneration (NPHP5)	NPHP5
	Retinitis Pigmentosa 45 (RP45) - CNGB1	CNGB1
 Neurological	Multiple Sulfatase Deficiency (MSD)	SUMF1
	Charcot - Marie - Tooth Disease Type 4J (CMT4J)	FIG4
	Spastic Paraplegia 50 (SPG50)	AP4M1
 Systemic	Propionic Acidemia (PA)	PCCB
	Mucopolysaccharidosis IVA (MPS IVA, Morquio A Syndrome)	GALNS

Choose to Bloom – A children’s book to empower those living with rare disease

In addition to supporting research in rare disease, RTW Foundation also seeks to raise awareness about rare disorders and empower rare disease communities. The RTW Foundation is thrilled to contribute to Danielle Kinsey's endeavor in creating a children's book that empowers kids facing rare diseases. Combining her knowledge as a medical provider and her passion for poetry, she hopes to share their stories through this book. We supported the work of artist Kara Fleming to add beautiful illustrations to this children's book. Some of the captivating artwork and pieces of the story are included here. Please be on the lookout for the release of this book in 2024.



Danielle is a Surgical Resident at the University of Missouri-Kansas City, University Health Truman Medical Center.

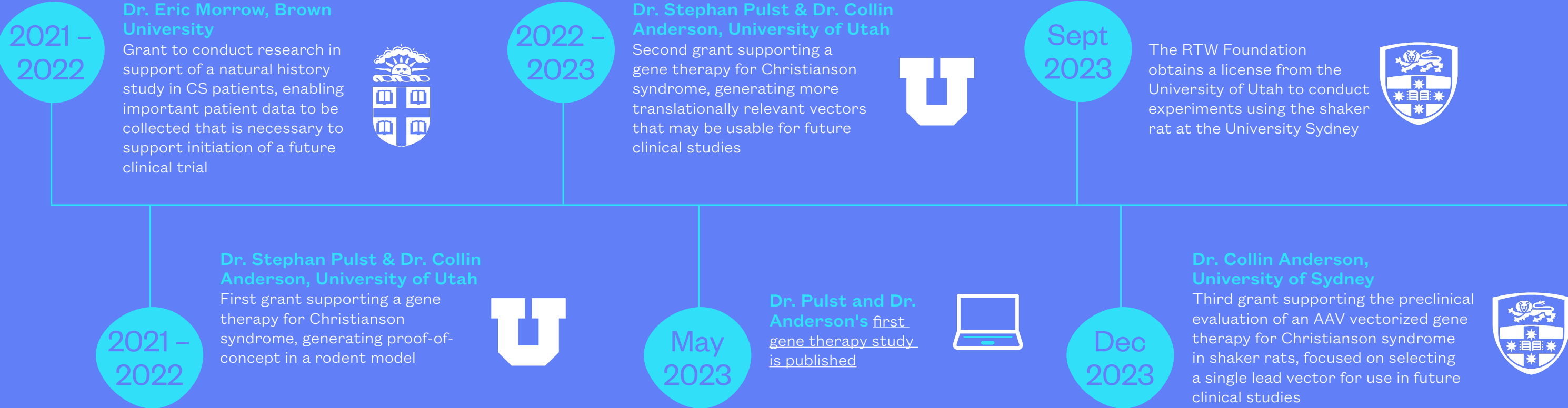
Christianson Syndrome Research

Christianson syndrome (CS) is a rare X-linked disease that affects brain development primarily in boys. Symptoms, including epilepsy, ataxia, and developmental delays, usually appear between 2 and 4 years of age, and progressively worsen during the first decade of life. The RTW Foundation continues to support efforts to develop and test more translationally relevant versions of gene therapy, working toward a treatment for CS patients.

In 2023 our primary collaborator Dr. Collin Anderson relocated to the University Sydney in Australia where he took on a role as a lecturer (associate professor) and began establishing his own laboratory. The RTW Foundation supported the rederivation and transfer of the shaker rat, a rodent model for CS, to the University of Sydney so that research could continue there. Additionally we also funded a third CS grant, focusing on selecting a lead gene therapy vector that could potentially be used in a future clinical trial.

“ I opened my research laboratory at University of Sydney after an international move to Australia in 2023, and The RTW Foundation has been instrumental in helping me continue my work on developing novel therapeutics for Christianson syndrome. In addition to providing funding that has allowed us to import our animal model and continue this work, The RTW Foundation has continued to be an exceptionally valuable partner in both providing resources and strategically planning experiments. Thank you to The RTW Foundation for the continued partnership in the Christianson syndrome space. ”

– Dr. Collin Anderson
University of Sydney, NSW, Australia



Team



David Pan
Chief Financial Officer



Juan Carlos López, PhD
Managing Director, Research Grants



Joe Katakowski, PhD
Director of Research



Sarah Garwood, MA
Director, Community Engagement



Christina Mastrangelo
Executive Assistant

*We're grateful to **Nate Pelsma** for his contributions to the Foundation in 2023.

Board of Directors



Roderick Wong, MD
Chairman



Stephanie A. Sirota, MA
President

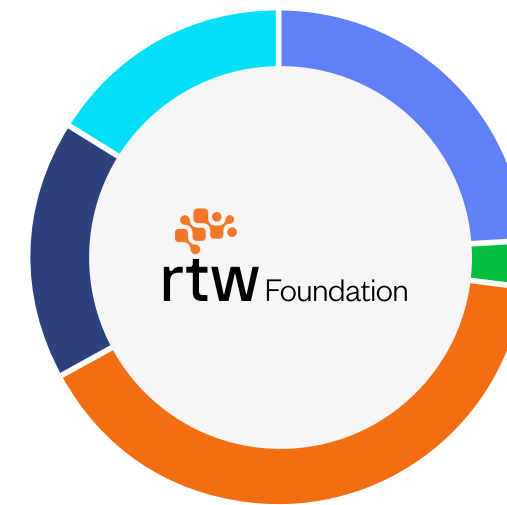


Marti Speranza Wong, MBA
President, Community Engagement

Financial Report

2023 Functional Expenses As of December 31, 2023

Community Program	799,739.29
Research Grants Program	1,350,597.57
Research Institute	565,176.13
Policy Institute	95,822.73
Management & General	588,948.52
Total	3,400,284.24



- Community Program - **23%**
- Research Grants Program - **40%**
- Research Institute - **17%**
- Policy Institute - **3%**
- Management & General - **17%**



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