Connecting CF Resources with Community Needs

What is Cystic Fibrosis?

Cystic fibrosis (CF) is a genetic disease that affects the respiratory, digestive and reproductive systems. Thick mucus blocks small passageways in the lungs, pancreas and other organs, while trapping bacteria that create serious infections, progressive lung damage and other complications. CF impacts people of all races and ethnicities. With the implementation of newborn screening programs, most people are diagnosed at a young age – though newborn screening often misses mutations more frequently found in people of color. Late diagnoses are becoming more common among adults who were born prior to newborn screening for CF. Early treatment, which may include nutritional and respiratory therapies, medications and exercise, has a significant impact on the quality of life of those with CF. Until recently, children with CF rarely reached adulthood. While innovative research, new medications, improved therapies and lung transplantation are prolonging and enhancing the lives of those with CF, it remains a life-threatening disease for which there is no cure.

Note from CFRI's Board President and Executive Director

Dear Friends,

On behalf of CFRI's Board of Directors, we hope this finds you safe and well. 2024 marked another year of programmatic growth for CFRI, as we continue to expand programs to address the emerging needs of the cystic fibrosis community.

As shared in this Annual Report, in 2024 we made significant progress realizing our goals and objectives. While only 40,000 people in the United States have been diagnosed with cystic fibrosis, it is our belief that many more people remain misdiagnosed or undiagnosed. Funding innovative CF research, raising awareness of the disease, creating resources to address emerging needs, and maintaining public focus on the need for increased research funding and access to care remain key goals of our strategic plan.

Cystic fibrosis impacts people of all races and ethnicities and it is known that race and ethnicity can play a role in CF health outcomes. As an organization, we hold principles of diversity and inclusion at the forefront of all that we do. We are mission-driven and determined to advance research in pursuit of a cure, while providing meaningful programs to our national – and international – CF community.

CFRI seeks to improve and enhance the lives of those with CF, now and into the future. CFRI's accomplishments are only possible thanks to the support of our community, which is passionately committed to the search for a cure.

Warm regards,

Bill

Bill Hult
President of the Board

Siri Vaeth, MSW
Executive Director & Mother of an Adult Daughter with CF

CFRI's mission is to be a global resource for the cystic fibrosis community while pursuing a cure through research, education, advocacy, and support.

Our vision is to find a cure for cystic fibrosis while enhancing quality of life for the CF community.

CFRI Cystic Fibrosis Research Institute
RESEARCH · EDUCATION · ADVOCACY · SUPPORT

2024 Annual Report

CFRI Cystic Fibrosis Research Institute
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Cystic Fibrosis Research Institute
1731 Embarcadero Road, Suite 210, Palo Alto, CA 94303
Toll Free 1.855.cfri.now (1.855.237.4669) • www.cfri.org

EDUCATION

CFRI's Strategic Plan outlines priorities that guide our program development and delivery. Our priorities are rooted in our commitment to diversity, inclusion and equity for all community members and include: ensuring research will continue toward a cure; engaging the national and global CF community in advocacy and awareness efforts that increase access to therapies, research funding and quality of life; creating innovative and responsive education and support programs for the national and global CF community; developing and strengthening national and global partnerships with community, industry and funders.

2024 Education Highlights

- Hosted the 37th National CF Education Conference as a hybrid event, where 30 speakers - including renowned experts in the field of CF, patient panelists, and CF researchers - presented the latest in research and clinical practice to over 100 in-person attendees, and to over 400 virtual attendees from our global CF community. Evaluations found that over 95% of participants improved their knowledge of CF and treatment options.
- Distributed nearly 10,000 CFRI-created informational brochures and booklets available in English and Spanish, including *CF in the Classroom*, *A Teen's Guide to Navigating Cystic Fibrosis*, *Patient Assistance Resources*, and *CF and Cancer Awareness*.
- Produced and posted 21 podcasts through our *CF Community Voices* podcast series, with over 2,000 combined views.
- Maintained our website as a dynamic resource for the global CF community. In addition, CFRI engaged its 19,000+ Facebook, Twitter, LinkedIn, and Instagram followers with breaking news, resources, and inspiring stories.
- Distributed 18,000 copies of the *CFRI Community* newsletter to homes and health centers across the globe. Distributed 6,000 copies of *CFRI Comunidad*, CFRI's Spanish-language newsletter, to CF centers nationwide.
- Provided updates on CF research, special events, advocacy efforts, and support programs through our weekly eNewsletter, distributed to over 15,000 subscribers.

SUPPORT

2024 Support Highlights

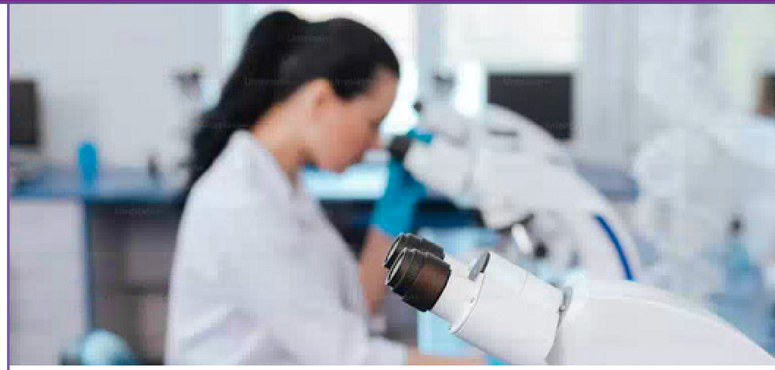
- Provided nine monthly facilitated virtual Support Groups with over 600 participants from across the U.S. and globe. Groups are provided to: adults with CF; caregivers of children with CF; parents/spouses/partners of adults with CF; people who lost a loved one to CF; adults with CF post-transplant; teens with CF; Spanish-speaking CF community members; adults with a late CF diagnosis; those who cannot use modulators.
- Underwrote individual therapy sessions for 85 individuals with CF and/or their immediate family from 20 states, resulting in a statistically significant reduction in depression and anxiety symptoms.
- Offered free weekly online CF-specific wellness classes, including strength training, yoga, pilates, and dance, attended by over 100 CF community members.
- Hosted four retreats for the national and global CF community. These included a virtual CF Spring Retreat and hybrid Summer Retreat for adults with CF and an in-person Spring Retreat and virtual Fall Retreat for mothers of children/adults with CF. These events provided resources, research updates, psychosocial support, and connection to peers; evaluations showed measurable decreases in symptoms of depression and anxiety.
- Increased support for the Spanish-speaking CF community via print, social media, and website resources, including a Spanish language version of CFRI's newsletter.

ADVOCACY

2024 Advocacy and Awareness Highlights

- Participated in 45 virtual and in-person meetings with state and federal legislators and policy makers regarding issues impacting the health and quality of life for those with CF, incl. access to care.
- Pursued efforts to create state Rare Disease Advisory Councils (RDACs) through coalition building with rare disease groups. Led efforts to pass legislation to create an RDAC in California.
- Advanced awareness of the racial and ethnic diversity of the CF community through the creation of podcasts and films. Expanded informational sheets in multiple languages; expanded Hindi and Spanish language offerings on our YouTube channel; provided captioning and subtitles for conference attendees; made CFRI website accessible in multiple languages.
- Engaged with other disease groups to advocate for pharmacy benefit manager (PBM) reform, copay accumulator bans, the prohibition of co-pay accumulator programs and surprise medical billing, while raising awareness of potential risks posed by state Prescription Drug Affordability Review Boards (PDABs) to CF community access to therapies.
- Produced an impactful CF Awareness and Advocacy video featuring members of the CF community to inspire greater engagement in advocacy activities.
- Partnered with other rare disease groups to engage our CF community on issues that impact the rare disease community. Sent multiple action alerts to 15,000 community members, facilitating communication between them and their representatives.
- Participated in diverse activities to raise awareness of the discriminatory use of Quality Adjusted Life Year (QALY) to assess drug pricing and value for rare disease drugs.
- Continued alliances with our CF organizational partners in the Cystic Fibrosis Engagement Network, with national rare disease organizations, disability groups, and with our partner organizations in the American Thoracic Society Public Advisory Roundtable.

RESEARCH



2024 Research Highlights

In 2024, the following researchers conducted research with the support of CFRI:

Elizabeth Nash Memorial Fellowship Program

Steven Jonas, MD, PhD (Principal Investigator), Ruby Sims, PhD (Postdoctoral Fellow)
University of California Los Angeles
Designing a Cystic Fibrosis Gene Therapy Nanocarrier Platform to Target and Modify Airway Stem Cell-Derived Ionocytes

Ron Kopito, PhD (Principal Investigator), Celeste Riepe, PhD (Postdoctoral Fellow)
Stanford University
Pharmacogenomic Discovery of Therapeutic Targets for Corrector-Resistant Cystic Fibrosis

Matthew Porteus, MD, PhD (Principal Investigator), Anais Amaya Colina, PhD (Postdoctoral Fellow) - Stanford University
A Hybrid Gene Correction Strategy for Cystic Fibrosis

New Horizons Program

Nadia Ameen, MD - Yale University School of Medicine
Elucidating The Ion Transport Functions of CFTR High Expresser Cells (CHEs) And Its Relevance To Intestinal Disease In Cystic Fibrosis

Benjamin Chan, PhD - Yale University School of Medicine
Optimization of Phage Therapy to Reduce Pseudomonas-Induced Inflammation in Cystic Fibrosis

Daria Van Tyne, PhD
University of Pittsburgh
Optimization Of Activity and Improved Delivery of Bacteriophages Targeting Burkholderia Spp

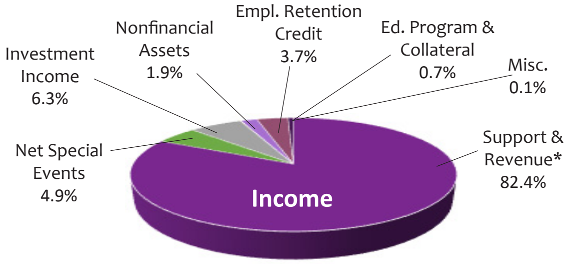
Katrine Whiteson, PhD - University of California Irvine
Targeting Recalcitrant CF Pathogens with Phages, Antibiotics, And Small Molecule Adjuvants

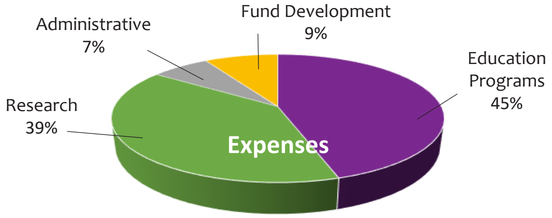
Feng Yuan, PhD
University of Alabama at Birmingham
Dissecting Pulmonary Ionocyte Subtypes and Their Functional Roles in Cystic Fibrosis

FINANCIAL SUMMARY

INCOME		
Support & Revenue	*$1,707,551	82.4%
Net Special Events	$102,649	4.9%
Investment Income	$130,211	6.3%
Empl. Retention Credit	$76,143	3.7%
Ed. Program & Collateral	$13,707	0.7%
Nonfinancial Assets	$39,652	1.9%
Miscellaneous	$1,663	0.1%
Total Income	**$2,071,576**	**100.00%**

EXPENSES		
Education Programs	$715,133	45%
Research	$630,708	39%
Administrative	$114,300	7%
Fund Development	$144,087	9%
Total Expenses	**$1,604,228**	**100.00%**





*includes bequests

As outlined in CFRI's Strategic Plan, CFRI seeks to create sustainable funding to remain a strong and viable agency.

CFRI is independently audited by BryMar, CPA, LLP.

CFRI is a registered 501 (C)(3) charity: EIN #51-0169988.