2023 BOARD OF DIRECTORS

William Hult, President Jessica Martens, Vice President Zoe Davies, NP, Secretary Oscar Flamenco, CPA, Treasurer **Francine Bion** Julie Desch, MD Colleen Dunn, MS, RT, CCRD **Elyse Elconin Goldberg** Jean Hanley, MD Doug Modlin, PhD Richard B. Moss, MD Arek Puzia, CFP, CPA Ahmet Uluer, DO, MPH

2023 COMMITTEE CHAIRS

d in our thoughts the me Ann Robinson and Isa Stenzel Byrnes vital members of CFRI's organizational family whom we lost in 2023.

William Hult Audit & Compliance

Oscar Flamenco, CPA Finance

Julie Desch. MD **Research Advisory**

Isabel Stenzel Byrnes, LCSW, MPH National CF Education Conference

> Jessica Martens **CF Summer Retreat**

Bridget Barnes Newsletter

Siri Vaeth, MSW Adult Advisory Committee

Robin Modlin, MA Embrace Mothers' Retreat

Siri Vaeth, MSW CF Diversity and Inclusion Advisory Committee

> **Ann Robinson Mothers' Day Celebration**

2023 STAFF Note: We hold in our thoughts the memory of beloved staff member Danielle Mandella,

> Siri Vaeth, MSW **Executive Director**

Sue Landgraf Chief Financial Officer

Mary Convento **Programs & Finance Supervisor**

Sabine Brants, MA Programs & Outreach Associate

Leeya Kannankunni Programs Assistant

Stacie Reveles Advocacy & Outreach Associate

Danielle Mandella Social Media Outreach Associate

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. Cystic fibrosis 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. 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. 2023 marked another year of programmatic

growth for CFRI, as we continue to expand programs to address the emerging needs of the CF community.

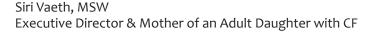
Cystic fibrosis impacts people of all races and ethnicities. As an organization, we hold principles of equity, diversity and inclusion at the forefront in all that we do. We are missiondriven and determined to advance research in pursuit of a cure, while providing meaningful programs to our national – and international – CF community.

As shared in this Annual Report, in 2023 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.

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 Hult President of the Board



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

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



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



2023 Annual Report

EDUCATION

SUPPORT

ADVOCACY



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.

2023 Education Highlights

- Hosted the 36th National CF Education Conference as a hybrid event, where 28 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 over 400 members of the global CF community virtually. Evaluations found that over 95% of participants improved their knowledge of CF and treatment options.
- Produced and posted 22 podcasts through our CF Community Voices podcast series, with over 3,400 combined views.
- Maintained our website as a dynamic resource available in multiple languages - for the global CF community. In addition, CFRI engaged its 18,000+ Facebook, Twitter and Instagram followers with breaking news, resources, and inspiring stories.
- Distributed 28,000 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.
- Distributed nearly 10,000 informational brochures about cystic fibrosis and CFRI's services.

2023 Support Highlights

- Underwrote individual therapy sessions for nearly 100 individuals with CF and/or their immediate family members from 23 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, dance, flexibility and stretching - which were attended by over 100 people with CF and their family members.
- Hosted four retreats for our national and global CF community: 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. All events provided resources, psychosocial support and connection to peers; evaluations showed measurable decreases in symptoms of depression and anxiety.
- Provided eight monthly virtual Support Groups facilitated by CF social workers: 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;

*\$1,929,355

\$96,743

\$94,720

\$28,168

\$15,045

\$2,164,031

\$728,556

\$641,900

\$109,348

\$137,950

\$1,617,754

adults with a late CF diagnosis. Participants were from across the U.S. and globe.

 Increased support for the Spanishspeaking CF community via print, social media, and website resources, including a Spanish language version of CFRI's newsletter.

INCOME

Support & Revenue

Net Special Events

Investment Income

Ed. Program & Collat

Education Programs

Fund Development

Miscellaneous

Total Income

EXPENSES

Research

Administrative

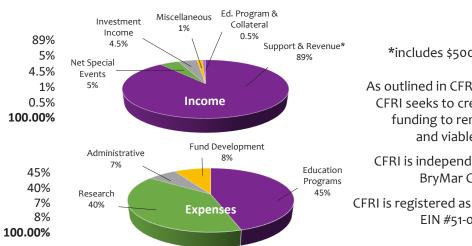
Total Expenses



2023 Advocacy and Awareness Highlights

- Advanced awareness of the CF community's racial and ethnic diversity through the creation of podcasts and films, informational sheets and resource guides in multiple languages; provided captioning and subtitles for conference attendees; made CFRI website accessible in multiple languages.
- Pursued efforts to create state Rare Disease Advisory Councils (RDACs) through coalition building with rare disease groups; sent multiple action alerts and partnered with other rare disease groups to engage our community on issues that impact the rare disease community.
- Engaged with other disease groups to advocate on potential threats to CF community access to therapies, including copay accumulator programs, surprise medical billing, and state Prescription Drug Affordability Review Boards (PDABs).
- Produced a CF Awareness and Advocacy video featuring members of the CF community to inspire greater engagement in advocacy activities.
- Created English/Spanish language informational fliers to raise awareness of the significantly higher rates of colorectal cancer among those with CF; released podcasts on this topic.
 - Held over 40 virtual and in-person meetings with state and federal legislators to discuss issues impacting the health and quality of life for those with CF, including access to care.
 - 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.
 - Strengthened 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.

FINANCIAL SUMMARY



*includes \$500,000 bequest

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

RESEARCH



2023 Research Highlights

In 2023, the following researchers conducted research with the support of CFRI:

Elizabeth Nash Memorial Fellowship Program

Paul Bollyky, MD, PhD (Principal Investigator), Kevin Qingquan Chen, PhD (Postdoctoral Fellow) - Stanford University

Targeting Bacterial Resistance to Phage Therapy in 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

Carlos Milla, MD - Stanford University Improving CF Airway Mucociliary Clearance: Toward Transition from Animals to Humans

Kenichi Okuda, MD, PhD - University of North Carolina at Chapel Hill Pathways Maintaining Basal Mucin and CFTR-Mediated Fluid Secretion in the Human Distal Airway

Zachary Sellers, MD, PhD - Stanford University Targeting IRBIT to Correct Bicarbonate Secretory Defects in Cystic Fibrosis

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

Special Circumstances Grant

Beate Illek, PhD - University of California San Diego CFTR mRNA Delivery to CF Airways