



2024

IMPACT REPORT



Adenoid Cystic Carcinoma
Research Foundation

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A MESSAGE FROM THE EXECUTIVE DIRECTOR



Jeffrey Kaufman, Co-Founder and Executive Director

Dear Friends of ACCRF,

Nearly 20 years ago, my wife Marnie and I founded ACCRF as a way to boost our hope in the future. Marnie had been diagnosed with ACC the year before, and we had four boys under the age of ten. We were looking for information, treatment options, and answers where there seemed to be few. So we set out to discover them.

Since then, the field of ACC research has flourished, attracting brilliant scientific advisors and researchers, committed and educated patients, and innovative biotech firms. Today, we see a research landscape and patient community that has been transformed. I am pleased to share that we have never been more confident that ACC patients will have better treatment options in the future.

ACCRF has enabled, supported, and funded so many landmark events in ACC research, including the first salivary gland tumor biorepository; the first sequencing studies of ACC tumors; the identification of MYB, MYBL1, and NOTCH as the major genetic drivers of ACC; the first validated preclinical models and drug screening platform for ACC; and updated clinical guidelines for salivary gland cancer patients that included the first targeted therapies for ACC.

Although ACCRF has accomplished so much, we are not slowing down. We are a charity started by patients, led by patients, and guided by the urgency of patients. We must continue this momentum in order to realize the effective treatments and ultimate cure that lie at the heart of our mission.

Looking back at 2024, I am thrilled to see more exciting milestones giving us hope.

Last year saw an all-time high of 20 clinical trials enrolling ACC patients, many focused on antibody-related drugs designed to kill ACC tumors. We are particularly excited about two new clinical trials of small molecule drugs that target MYB—a key driver of ACC—for the first time ever. Many of these clinical trials are direct results of research from ACCRF-funded grants, and in 2024 we awarded 14 new

A MESSAGE FROM THE EXECUTIVE DIRECTOR

grants while former grantees published more than 20 scientific papers to better understand the disease. ACCRF Director of Research Nicole Spardy Burr, PhD, has skillfully shepherded these advances toward our ultimate goals of better treatments and a cure.

Our patient community remains equally strong, vibrant, and dedicated. More patients than ever attended our annual patient meeting, participated in our webinar series, connected online, and educated themselves to build greater community together. These individuals continue to be a great inspiration and source of courage as we move forward. We are truly grateful to all of you.

I would like to make special mention of all the ACC patients who choose to participate in clinical trials. It is through your motivation and energy that learning is actualized and our community gains precious knowledge on what treatments may be worth pursuing. We thank you, and the caregivers that support you, for your enormous contributions to ACC research.

As we look to the future, Marnie, Nicole, and I grieve the many ACC friends we have lost along the way. We also rejoice in the many ACC friends we continue to make. There is still much work to be done, but it is the collective spirit of these individuals that propels us forward, and our hope is strong. We invite you to read this Impact Report in the expectation that it will provide our entire ACC community with hope and renewed energy to build a brighter future together.

Warm regards,

A handwritten signature in black ink that reads "Jeffrey Kaufman". The script is fluid and cursive, with the first letters of the first and last names being capitalized and prominent.

Jeffrey Kaufman
Co-Founder and Executive Director

HISTORIC ACHIEVEMENTS



ACCRF'S IMPACT

[Our inaugural Impact Report](#) provides an opportunity not only to highlight the accomplishments of the past year, but to take stock of how far we have come since our inception. Looking back over the last 19 years, we see the transformation of ACC research from a little-studied niche into a vibrant and vital field. The foundation's efforts have led to an explosion of knowledge about the basic biology of ACC that is now being translated into promising clinical trials. These efforts have attracted some of the world's leading oncology researchers and established ACCRF as the essential hub of ACC research. All of this serves our ultimate goal to bring more effective treatments and a cure to patients. Below are some of the strides we have made toward that goal.

Benchmarks	2005	2024
Cumulative Donations to ACCRF	\$250,000	\$22 million
Cumulative ACCRF Grants	1	181 grants to 70+ institutions globally
Genetic Causes of ACC	Unknown	MYB, MYBL1, and NOTCH identified as main drivers
Published Articles Mentioning ACC	172	363
Publicly Available Preclinical Models	0	20+
Preclinical Drug Screening	None	150+ anticancer drugs
Targeted Drugs in Clinical Guidelines	0	2 (Lenvatinib and Sorafenib)
Open Clinical Trials	1	20

RESEARCH EFFORTS

2024 OVERVIEW AND HIGHLIGHTS

“As we reflect on the progress made in 2024, it is with great pride and gratitude that I share the clinical and research milestones achieved by the ACCRF network. Our preclinical drug screening platform and the research from our grantees provides the scientific basis for the latest developments in drugs, treatments, and clinical trials. Below are some of the most promising developments from the last year.



Nicole Spardy Burr, PhD, Director of Research

- 20 clinical trials of anticancer drugs opened to recurrent/metastatic ACC patients—an **all-time high**—including trials testing drugs that target MYB (the main protein deregulated in ACC tumors) and several antibody-drug conjugates that deliver chemotherapy directly to cancer cells.
- 25 ACCRF grantees published research including work describing the ACC immune landscape and intratumor microbiome, and identifying prognostic biomarkers that may help to better inform patients about their disease journey.
- 14 new research grants were awarded by ACCRF including 4 grants to researchers exploring novel drug targets in ACC.
- 13 different laboratories utilized tumor samples provided by ACCRF to advance ACC research.
- ACCRF grantees created new tools to explore MYB levels in ACC tumor tissue and blood. The ability to detect MYB levels in the blood represents the first step towards developing a less-invasive assay to aid surveillance and assess treatment response in ACC patients.

Looking ahead to 2025, we are excited to follow the progress of early-stage clinical trials. We will continue to support projects focused on developing new ACC models and datasets for the scientific community, expand on our molecular understanding of ACC tumors, and award grants that connect talented scientists worldwide to explore multiple facets of this disease.

RESEARCH EFFORTS

CLINICAL TRIALS

2024 was a banner year for [clinical trials in ACC](#), with [a record of 20 trials of anticancer drugs enrolling ACC patients](#). Many of these clinical trials are the culmination of research efforts supported by ACCRF and represent some of the most promising novel treatments available.

Preclinical data for novel MYB inhibitors and antibody-drug conjugates (ADCs) are the strongest ever, and those drugs that have entered into clinical trials or will in the near future are poised to provide crucial data on ACC treatment options.

Antibody-drug conjugates are the most common type of drug currently in clinical trials. ADCs work by binding to specific proteins on a cell's surface and delivering chemotherapy directly into the cell in order to kill the cancer cell. This approach is beneficial because, unlike traditional chemotherapy, ADCs are able to better target cancerous cells while damaging fewer healthy cells.

Targeting MYB

MYB is a protein that controls cell proliferation and differentiation. In ACC, MYB is often deregulated and too abundant, causing cells to multiply unchecked. Drugs that target MYB are a promising approach for treatment as they have the potential to reduce or turn off this protein and therefore ACC cell proliferation.

ACCRF is proud to have collaborated with two biotech firms, providing models and screening platforms to develop and bring to clinical trials drugs designed to target MYB specifically. These promising trials are critical steps towards providing patients with more effective treatments.



Remix Therapeutics™ continued enrollment of advanced ACC patients in a multi-center [REM-422](#) trial that opened at the end of 2023. The company presented [preclinical data](#) that demonstrated tumor regressions in ACC preclinical models induced by REM-422.



Rgenta Therapeutics™ announced the first ACC patients were dosed in a Phase 1a/b clinical trial of [RGT-61159](#). This multi-center, open-label study began enrolling patients with advanced relapsed or refractory ACC. The company presented [preclinical data](#) at ASCO 2024 that demonstrated strong anti-tumor activity in ACC preclinical models that correlated with a reduction in MYB levels.

RESEARCH EFFORTS

RESEARCH PUBLICATIONS

In 2024 more than [25 articles](#) were published with findings from ACCRF-funded research. These findings help to advance the overall understanding and increase the visibility of ACC in the medical and research communities. Below are important advances published in the past year by ACCRF grantees.



Multiple ACCRF grantees published papers characterizing ACCs from rarer body sites, including the [lacrimal gland](#), [vulva](#), and [breast](#).

These articles confirm that ACCs occurring outside the salivary gland possess hallmark genomic features of ACC, including MYB alterations and NOTCH1 mutations. Findings suggest that patients with non-salivary-based primary ACC tumors who develop distant metastases may benefit from consulting a head and neck medical oncologist experienced with ACC and may consider treatment strategies offered to recurrent/metastatic salivary ACC patients.

ACCRF grantee Dr. Renata Ferrarotto from MD Anderson Cancer Center published [a case report](#) describing the first ACC type II patient treated with an antibody-drug conjugate that delivers chemotherapy directly to tumor cells that express a protein called AXL on their cell surface. While on treatment, this patient experienced tumor shrinkage, a reduction of symptoms, and improved quality of life. Experiments performed at ACCRF's preclinical drug screening platform helped to provide the scientific rationale to support this clinical trial.



Scientists need accurate tools to study how normal cell processes become disturbed in tumor cells. To better understand MYB levels within ACC tumors, ACCRF grantees Drs. Adam

Fisch, Bill Faquin, John Iafrate, Joe Lennerz, and Julia Theirauf from Massachusetts General Hospital published [a paper](#) comparing several antibodies used to examine MYB levels across a patient's biopsy or tumor slide. Results from this work will aid the research community in choosing MYB antibodies that best match DNA and RNA sequencing methods of measuring MYB tumor levels.

Tumor cells often shed proteins and genomic fragments into the bloodstream, offering an opportunity to look for cancer-related biomarkers in less invasive ways than surgeries or biopsies. Using an assay that measures the amount of MYB in the blood, ACCRF grantee Dr. Daniel Pelaez from the University of Miami [showed for the first time](#) that MYB blood levels are significantly higher in ACC patients with distant metastases compared to patients with no evident disease, local disease, or without cancer. A clinical trial is being planned that will follow newly diagnosed ACC patients over time. If the test can successfully identify newly metastatic patients, it may eventually be clinically adopted to aid surveillance and monitor response to therapies.



RESEARCH EFFORTS

AWARDED GRANTS

Since founding ACCRF, we have awarded more than [\\$12 million in research grants](#) to individuals working towards new treatments and a cure, rapidly expanding the field of ACC research in the process. **In 2024 ACCRF awarded 14 grants totaling \$513,000 to field-leading scientists** to pursue the frontiers of ACC research and treatment.



John Iafrate, MD, PhD; Massachusetts General Hospital; “The ACC Tumor Immune Microenvironment: Reversing HLA/B2M Downregulation to Support Immunotherapy Efficacy”

Rajdeep Chakraborty, PhD; Macquarie University; “A Prelude towards transforming adenoid cystic carcinoma into an immune hot tumour”

Bhumsuk Keam, MD; Seoul National University Hospital; “Multicenter Validation of P63 Immunohistochemistry (IHC) as a Prognostic Marker for Adenoid Cystic Carcinoma (SNUH)”

Mridula George, MD; Rutgers Cancer Institute; “Patterns of care in the management of patients with Adenoid Cystic Carcinoma of the Breast”

Stephen Doggett, MD and **Drew Moghanaki, MD**; Mission Viejo Hospital and UCLA; “CT-Guided LDR Brachytherapy for Adenoid Cystic Carcinoma Lung Metastases: Efficacy and Safety Outcomes” (2 grants awarded)

Liron Bar-Peled, PhD; Massachusetts General Hospital Cancer Center; “Chemical proteomics to target MYB in ACC”

Miguel Rito, MD and **Isabel Fonseca, MD, PhD**; Instituto Português de Oncologia de Lisboa; “Multicenter Validation of P63 Immunohistochemistry (IHC) as a Prognostic Marker for Adenoid Cystic Carcinoma (Lisboa)”

Francois-Regis Ferrand, MD; Gustave Roussy Institute; “Pathological review of ACC in the framework of a multiomic study (REFCORomics- PRTK2024/ SPELCASTER-TRANSCAN3)”

Carla van Herpen, Prof. MD, PhD; Radboud University Medical Center; “Multicenter Validation of P63 Immunohistochemistry (IHC) as a Prognostic Marker for Adenoid Cystic Carcinoma (Radboud)”

Max Wintermark, MD and **Renata Ferrarotto, MD**; MD Anderson Cancer Center; “VEGFR inhibitors in ACC”

Tony Amelio, PhD; H. Lee Moffitt Cancer Center & Research Institute, Inc.; “Molecular Characterization of the Salivary Adenoid Cystic Carcinoma Tumor Immune Landscape by Anatomic Subsite”

Rajan Gogna, PhD; Virginia Commonwealth University School of Medicine; “To Study the Expression of Flower-Win and Flower-Lose in Adenoid Cystic Carcinoma (ACC)”

Damian Rieke, MD; Charité Comprehensive Cancer Center; “Identifying novel treatment options as well as predictive biomarkers for existing immune therapies in advanced ACC”

Grantees are pictured in listed order clockwise from top left

PATIENT COMMUNITY

EDUCATION & RESOURCES

ACCRF remains dedicated to providing educational resources so patients can better understand ACC and advocate for themselves. This year the foundation hosted two [webinars](#) with leading oncologists discussing current treatment options. Additionally, we created a brand new Second Opinions resource and fully updated the ACC Physicians List to provide the most current information possible for patients.

ACCRF Webinar Series

Tumor Board for Metastatic ACC

Medical Oncologist **Dr. Paul Swiecicki** (University of Michigan) and Radiation Oncologist **Dr. Jonathan Schoenfeld** (Dana-Farber/Harvard) reviewed specific cases of ACC and provided insight into multidisciplinary decision-making for the treatment of ACC metastases.

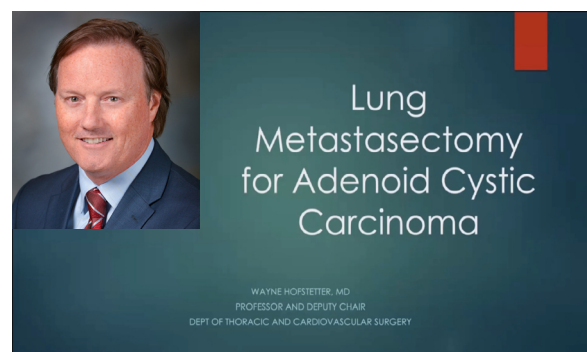
[Watch the webinar](#)



Surgical Options for ACC Lung Metastases

Dr. Wayne Hofstetter (MD Anderson Cancer Center) discussed different surgical options for treating lung metastases.

[Watch the webinar](#)



Patient Resources

ACC Physicians List

The foundation fully updated our ACC Physicians List containing information for over 850 physicians globally with experience in treating ACC. The fully searchable database allows patients to locate physicians by name, location, institution, or field of expertise to help inform their decisions in selecting doctors.

[Visit the Physicians List](#)



Second Opinions Resource

Patients should always feel empowered and comfortable in asking for additional opinions in exploring treatment options, especially for complex or unique cases. In support of this goal, ACCRF launched our new ACC Second Opinions resource offering guidance in seeking additional perspectives during treatment and a list of physicians and institutions with significant experience in ACC research and clinical trials.

[Visit the Second Opinions Resource](#)

PATIENT COMMUNITY

EVENTS

ACCRF hosted two in-person patient gatherings in 2024, our first since the pandemic. Patient gatherings build community among ACC patients and their loved ones, offering opportunities for individuals to connect, share experiences, and support one another. These gatherings also provide a chance for patients to speak directly with oncologists and researchers to better understand the ACC research landscape and available treatment options.

Needham, MA, USA - March 2, 2024

Our first patient gathering of the year brought together over 30 individuals for round-table discussions on treatment experiences, and presentations on the overall landscape of ACC research and ACCRF's efforts in the field. The day's keynote address was given by **Dr. Wayne Hoffstetter** of MD Anderson Cancer Center for a discussion of different surgical and treatment options for ACC lung metastases.

Ann Arbor, MI, USA - October 12, 2024

Our second gathering was a hub for patients in the Midwest, featuring an overview of the ACC research landscape provided by ACCRF's Director of Research **Nicole Spardy Burr, PhD** and a keynote presentation from ACCRF grantee **Dr. Paul Swiecicki** of the University of Michigan who offered insight into some of the latest developments in ACC treatments and led a discussion with patients.



PATIENT COMMUNITY

FUNDRAISING

ACCRF is proud and grateful to be supported by a dedicated community of donors whose generosity directly funds the foundation's ongoing research efforts. This year, ACCRF launched an updated and [streamlined donation system](#) including a [brand new crowdfunding platform](#) that allows individuals to create their own personalized fundraising pages. Below are some fundraising highlights from the ACCRF community.



Brave Like Gabe 7th Annual 5K

This year's race raised \$40,000 for ACCRF's research efforts, and with 1,100 participants worldwide and nearly 400 at the main event in St. Paul, MN, USA, this was one of the most successful 5Ks in the organization's history.

As part of this year's 5K, Sue and Dave Morse held their annual satellite race in Winchester, MA, USA—their fifth year participating in the Brave Like Gabe 5K. Their truly special event brings people together to support ACC research and celebrate the community.

Memorial Bike Ride

Douglas Meyer biked over 180 miles across New York State, single-handedly raising over \$4,000 in research funding for ACCRF. Douglas raised money for the event using ACCRF's new [crowdfunding platform](#).

Social Media Fundraisers

Thank you to the many people who hosted fundraisers for ACCRF on social media, including on our [Facebook page](#). Their combined efforts and generosity raised over \$8,000 in support of ACCRF's programming.



2024 Fundraising Match Challenge

Diana and Ogie Yanakiev generously created a \$50,000 fundraising match challenge to encourage donations by matching any gift made in December up to \$50,000. We are proud to say that the match was reached, and helped ACCRF raise well over \$100,000 to fund our research efforts in 2025 and beyond.

“

ACCRF has, from the beginning, been an organization created by and for patients, and those same individuals have continually supported the foundation and its goals of effective treatments and a cure. Through boundless generosity of time, effort, and funding, the members of the ACCRF community make our work possible. We are incredibly grateful to everyone who supports ACCRF and we sincerely thank you for all that you do.

Marnie Kaufman, Co-Founder and Director



FINANCIALS

OVERVIEW

ACCRF derives funding from three main sources:



Direct Public Support

ACCRF is supported by generous donations from the public, all of which go directly to supporting our research efforts. Public donations have comprised **80% of all ACCRF's funding** since the foundation's inception.



Preclinical Screening

ACCRF manages and makes available preclinical ACC models for studying disease biology and screening drugs. Fees paid to ACCRF to access these models were unusually high in 2024 due to strong biotech interest in the disease.

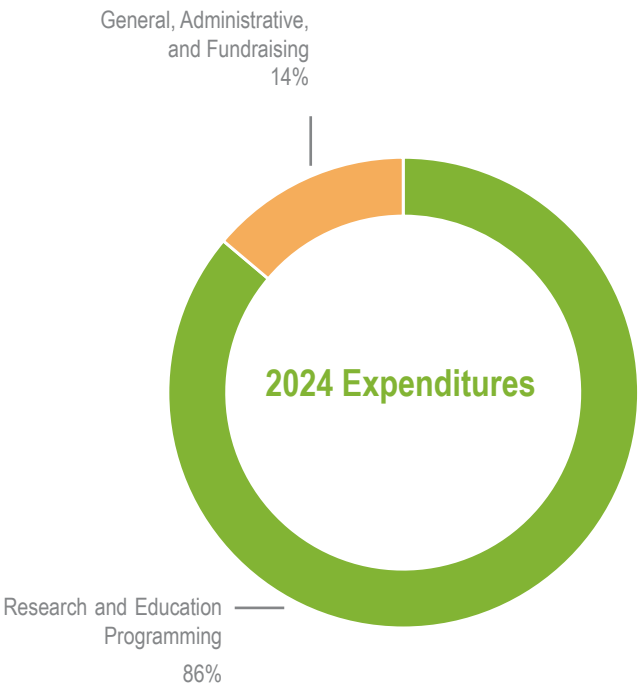


Investment Income

Any funds not immediately directed to research are invested and the income is applied to future research programs.

2024 Revenue and Expenditures*

Funds Raised	\$ 1,644,000
Direct Public Support	\$ 636,000
Preclinical Screening	\$ 911,000
Investment Income	\$ 97,000
Total Expenditures	\$ 1,531,000
Research and Education Programs	\$1,319,000
General, Administrative, and Fundraising	\$212,000



* preliminary cash basis financial results



ACCRF is proud to hold a four-star “Give With Confidence” rating from Charity Navigator, and to meet Charity Watch’s “grade A” efficiency criteria, reserved for organizations allocating exceptionally high levels of funds towards their programs.