OUR MISSION
The National Foundation for Cancer Research (NFCR) was founded in 1973 to support cancer research and public education relating to the prevention, early diagnosis, better treatments, and, ultimately, a cure for cancer. NFCR promotes and facilitates collaboration among scientists to accelerate the pace of discovery from bench to bedside. NFCR is committed to Research for a Cure among scientists to accelerate the pace of discovery from bench to bedside. NFCR is committed to Research for a Cure – cures for all types of cancer.

OUR VISION
NFCR is committed to fighting cancer by funding high-risk, high-impact, and potentially high-reward discoveries in the labs and transforming them into life-saving treatments for cancer patients. Through global collaboration, NFCR is making a unique impact on a new and accelerated path to cures. NFCR envisions a world without cancer!

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National Foundation for Cancer Research
5515 Security Lane, Suite 1105, Rockville, MD 20852 | www.NFCR.org
Dear NFCR Supporters,

We are pleased to present the 2022 Scientific Progress Report of the National Foundation for Cancer Research (NFCR) highlighting significant advancements in our fight together to find cures.

In 1973, NFCR was co-founded by Nobel Laureate Dr. Albert Szent-Györgyi and Franklin Salisbury, Sr., and Tamara Salisbury, to select and fund 'high-risk, high-impact' research to cure cancer – all types of cancer. We continue to stay on this course, and I am excited to share new research initiatives in 2022 that will advance our mission.

- Advanced cancer cell detection technology is being utilized by ten scientists to develop treatments, determine drug resistance mechanisms, and guide the next precision therapies for patients with advanced or metastatic cancer. (see pages 3-6)

- Collaboration is a bedrock principle of NFCR and in 2022, our Team Science Program began funding twelve teams of two expert scientists to tackle critical unmet needs of cancer patients. (see pages 7-13)

The revolutionary clinical trial, GBM AGILE, we began in 2019 for the universally fatal brain cancer, glioblastoma, is helping to accelerate cancer drug development and delivery to patients.

Our annual 2022 Global Summit and Award Ceremonies for Cancer Research & Entrepreneurship last October was a success. Both our in-person and virtual audience via our digital platform, the Oncology Metaverse, heard from our funded scientists and entrepreneurs, and other renowned cancer research leaders on their latest advancements and their vision for the future most effective care for all cancer types.

We celebrated our 2022 Szent-Gyorgyi Prize winner, the inaugural 2022 Beacon Award for Women Leaders in Oncology, and our winners in the third annual AIM-HI Women's Venture Competition. I am proud to share with you that both NFCR and its AIM-HI Accelerator Fund (AIM-HI) each received nominations for prestigious awards for non-profits.

You have enabled these research programs to begin and continue, and all of us at NFCR are deeply grateful for your trust in us and for sharing our commitment to Research for a Cure.

2023 is the 50th anniversary of NFCR in fighting cancer! I hope you enjoy the highlights here in our 2022 Scientific Progress Report. Later this year, NFCR will share with you the cumulative impact of 50 years on making cures possible - together!

Until then, thank you, and best wishes for good health!

Sujuan Ba, Ph.D.
President and CEO
2022 NFCR Innovative Research Programs

Research Funding for Discovery Research

- **Cancer Cell Diagnostic Technology Program**
  New initiative with leading researchers from across the globe focusing on liquid biopsies or live cancer cells to generate the knowledge to accelerate early detection and monitoring of advanced cancer.

- **Collaborative Team Science**
  New teams of expert scientists collaborate and exchange ideas to launch and accelerate the most innovative and impactful research.

AIM-HI Translational Research Initiative

- **Speed up the translation of discoveries from bench to bedside in order to save patients’ lives**
  - Commercialize new discoveries
  - Partner with international collaborators, Key Opinion Leaders (KOLs), and service providers

NFCR Supports Research Projects at Major Academic Institutions and Medical Centers

- Baylor College of Medicine
- Case Western Reserve University
- Harvard/DFCI
- Harvard/MGH
- Henry Ford Cancer Institute
- John Hopkins University
- Memorial Sloan Kettering
- MD Anderson Cancer Center
- MIT
- Mount Sinai Health System
- New York University School of Medicine
- Oregon Health & Sciences
- Scripps Research
- Thomas Jefferson University
- University of California, San Diego
- University of California, San Francisco
- University of Massachusetts, Worcester
- University of Kansas Cancer Center
- University of Oklahoma Health Science Center
- University of Pennsylvania
- University of Texas Southwestern
- University of Tornio
- University of Washington
- Virginia Commonwealth Univ. School of Medicine
- Weill Cornell
- Yale University

“Research is to see what everybody else has seen, and to think what nobody else has thought.”
— Dr. Albert Szent-Györgyi
New Research Program in 2022: Cancer Cell Diagnostic Technology

Advanced and metastatic cancer takes the lives of more than 90% of patients. Early detection of cancer and its progression is a dire unmet need for patients, families, and the cancer research community.

- Current approaches to cancer monitoring are invasive, time-consuming, and may not comprehensively represent the tumor microenvironment.

- In comparison, liquid biopsies are minimally invasive and, therefore, can be serially repeated to enable earlier detection of cancer progression. Furthermore, liquid biopsies, specifically the isolation of live cancer cells, have overcome temporal and spatial heterogeneity associated with traditional biopsies.

- NFCR’s new initiative focuses on cancer cell diagnostic technology to provide an increased understanding of the live cancer cells or circulating tumor cells (CTCs) in real-time and an inclusive understanding of the tumor microenvironment at the cellular, genomic, transcriptomic, and proteomic levels.

Ten leading researchers from across the globe have begun to generate the knowledge needed to accelerate this most critical cancer problem of early detection and monitoring of advanced cancer.

Circulating tumor cells (CTCs) detach from solid tumors and circulate in the bloodstream. Since they are live cells from a patient’s tumor, they are considered a “liquid biopsy” of the tumor and offer the possibility to assess — in real-time — the tumor’s characteristics, evolution, and response or resistance to treatments.

The National Foundation for Cancer Research wishes to thank the Sorenson Legacy Foundation for its generous support to expand on this critical research initiative.

This joint partnership enables us to increase vital circulating tumor cell research that will benefit even more patients across cancer types.
Cancer Cell Diagnostic Technology Principal Investigators

**Cancer Cell Diagnostic Technology Principal Investigators**

**Colorectal and Breast Cancer**

Cultures of circulating tumor cells (CTCs) for functional tests and drug screening is one of the most promising applications, but it presents great technical challenges such as finding the right conditions to make the cancer cells grow outside of the host.

Dr. Cristofanilli will include cells of the micro-environment to better mimic the conditions in the human body, and hopefully, increase the success rate of culturing. He will develop a pipeline for forming cultures of CTCs from patients with metastatic colorectal and breast cancer to enable high throughput drug screening (HTDS).

**IMPACT**

HTDS enables understanding the effect of multiple drugs on each patient’s tumor and predicting which treatments or combinations would be more effective or resistant and identify new biomarkers to predict response.

**Kidney Cancer**

Renal cell carcinoma (RCC), the most lethal form of kidney cancer, afflicts >70,000 patients each year in the U.S. with a rising disease incidence. Molecular biomarkers which can help clinicians with treatment decision-making have been elusive, and currently no tissue-based testing is utilized for this disease.

Dr. Kotecha will study how treatment affects the yield and profiles of the circulating tumor cells. His team will conduct genomic profiling on the circulating tumor cells and on patients’ tumor tissues and compare known gene expression signatures that are in development.

**IMPACT**

These goals will provide the essential foundation for integrating these innovative and advanced circulating cell detection technology tools into the clinic to improve the care of kidney cancer patients.

**Prostate and Kidney Cancer**

Neuroendocrine prostate cancer (NEPC) is an aggressive type of prostate cancer resistant to standard treatment and often underdiagnosed. This team will track gene expression patterns in CTCs of patients to develop an approach to detect NEPC and other subtypes.

Therapies for the most common type of kidney cancer, renal cell carcinoma (RCC), are not effective for patients with other subtypes who are often misdiagnosed.

Genomic and RNA assays on the CTCs will characterize the molecular features of the subtypes of kidney cancer.

**IMPACT**

This team is developing the methods to detect the subtypes of these cancers in patients’ CTCs. This will allow earlier detection of the subtypes and intervention with effective treatments.
Multiple Myeloma

Multiple myeloma (MM) is a blood cancer characterized by aberrant expansion of plasma cells in the bone marrow. Treatment can be very effective at controlling MM, relieving its symptoms and implications, and prolonging life. Unfortunately, MM is currently an incurable (terminal) cancer. Targeting MYC – a major cancer-causing protein in MM and many cancers - is challenging and known as "undruggable."

Dr. Kelliher will use a new compound that turns off the activation of MYC and slows growth in lab cell lines of the MYC-dependent MM.

Her team will test if the inhibitor stops the growth of circulating tumor cells isolated directly from MM patients.

**IMPACT**

This research may bring a promising new approach to treat MM and other MYC-dependent cancers, including breast, colorectal, liver and prostate cancers.

Pancreatic Cancer

Pancreatic cancer causes over 49,000 deaths each year in the US. Researchers do not have the tools to effectively detect the disease at an early stage. Moreover, the standard of care is only modestly effective and there are no biomarkers or tests to predict the patient's response.

With CTCs from pancreatic cancer patients, Dr. Huang's team will determine the expression of genes in the cells. They will use computational analyses to identify changes of gene expression patterns and correlate the expression with patients' responses to treatments.

**IMPACT**

Optimizing CTCs isolation procedures to readily apply in the clinics will help guide doctors to tailor therapeutic plans for individual patients, reduce adverse effects and ultimately, improve the outcome for patients.

Breast Cancer

Antibody Drug Conjugates (ADCs) are a new class of medications composed of an antibody linked to active therapeutic agents for selective delivery of drugs preferentially to cancer cells.

ADCs, developed by Dr. Bardia, show remarkable results in triple-negative breast cancer but resistance can develop. Real-time monitoring of the changes in efficacy is needed.

To develop ADCs for other metastatic breast cancers, Dr. Bardia will use patients' CTCs and tissue biopsy to develop a diagnostic test that detects the presence of the antigens (markers) in real-time and will guide selection of the "right drug" for the "right patient."

**IMPACT**

The diagnostic test can accelerate the development of novel precision ADCs for patients with metastatic breast cancer and significantly improve their outcome.
Small cell lung cancer (SCLC) accounts for about one in seven cases of lung cancer and it afflicts over 33,000 patients per year and is rapidly fatal in 95% of cases. In most cases, chemotherapy is the only option to prolong survival, but when the cancer relapses it is resistant to most drugs. It is not known how the cancer becomes resistant, or how to overcome this, because there have only been very few living samples of the disease after relapse to study in the laboratory.

Dr. Drapkin’s laboratory has developed a method to extract SCLC tumor cells from patient blood samples (circulating tumor cells) and grow them in the laboratory. The responses of these human tumors grown in the lab models to cancer therapy mirror the responses of the patients themselves and can be used to compare different treatments.

IMPACT
By comparing SCLC cancer cells before and after patient’s experience relapse, his team can understand how SCLC becomes resistant to our current drugs and develop new drugs to fight the disease.

Dr. Bersani will collect SCLC-CTCs to study the molecular features of these metastatic cells. A first-of-its kind biobank of SCLC CTC cultures will also be generated to investigate further organ-specific pathways responsible for their metastatic seeding to distant sites. It is the first time that such an approach has applied to SCLC.

IMPACT
Understanding what drives the preferential destination of metastatic cells to certain organs may help clinicians to choose more tailored therapies and follow-up protocols, and scientists to develop better therapeutic strategies to reduce the recurrence of SCLC and improve the overall survival.

Although there are several treatment options, there are no reliable tests, or “biomarkers,” that identify tumors that will respond to specific treatments.

Dr. Rolfo’s team has developed laboratory models from SCLC patients’ CTCs, that replicate the behavior of the human tumor. This facilitates identifying specific molecular changes contributing to drug resistance. Standardizing this novel strategy of evaluating patients CTCs in the models will transform it into a reality.

IMPACT
This new approach may individualize treatment selection and develop a new approach for prolonging survival of patients with SCLC.
New Collaborative Research Initiative
NFCR Team Science Program

Collaboration is a bedrock principle of NFCR. It is how we can accelerate research advancements to realize an end to this terrible disease - through better prevention, diagnosis, and treatment. A future without cancer is possible.

NFCR has distinguished itself in the cancer research sector by emphasizing long-term, transformative research often overlooked by other major funding sources. NFCR promotes and facilitates collaboration among scientists to accelerate the pace of discovery from bench to bedside. One of the ways that NFCR promotes its mission is to provide unrestricted research support funds for outstanding cancer researchers.

In 2022, NFCR expanded its tradition and philosophy of facilitating collaborative research. Twelve teams of two experts, each team focused on a critical unmet need of patients, began collaborating and working together. The NFCR Team Science serves as a catalyst for expert scientists to work together – exchange ideas to launch and accelerate the most innovative and impactful research.

In order to maximize the impact of its support, NFCR supports novel high-risk research programs that might have the ability to dramatically change our understanding of cancer that, in turn, could lead to novel drugs, diagnostics, and therapies that will benefit cancer patients around the world.

- CAR-T Cell Therapy for Pancreatic Cancer
- Predicting Metastasis
- New Targets and Treatments for Triple-Negative Breast Cancer
- Targeted Therapies and Treating Lung Cancer Resistance
- Artificial Intelligence-based research
- Digital Spatial Profiler to better assess lung cancer biopsy tissue
- Gene Therapy and Cell Replacement
- Improving Immunotherapy for More Patients and Additional Cancer Types
Progress in Triple-Negative Breast Cancer

Dr. Coussens developed a combination of four therapies to enable immunotherapy to be effective for Triple-Negative Breast Cancer (TNBC) - the most difficult-to-treat breast cancer. In TNBC lab models, the four therapies increased immune response and caused the tumors to shrink.

New single-cell measurement technologies were applied to the tumor samples to characterize the response of specific cell types in the tumor microenvironment. Dr. Fertig, an expert computational biologist, created new artificial intelligence methods to analyze large datasets of genes and cells and identify the mechanisms causing the tumors to regress.

Results indicate that the four therapies alter the microenvironment of breast cancer so immune cells can both reach and attack the tumor cells.

**IMPACT**
The artificial intelligence approach to combine datasets from mouse models with those from human clinical trials enables identifying patients in which these same mechanisms occur, guiding doctors to identify patients who would respond to this new immunotherapy.

Combatting Brain Metastasis in Breast Cancer

Patients with HER2-positive breast tumors (abnormal amounts of HER2 growth protein) are treated with targeted therapies but a subset of patients do not respond to treatment and develop progressive disease, including brain metastases associated with dismal outcomes.

Drs. Polyak and Weaver, pioneers in the tumor microenvironment, are exploring how the brain environment enables breast cancer cells to grow and survive.

Their data shows the sugar or glycosylation molecules in primary and metastatic cancers are distinct and are due to the microenvironment mechanical stress response.

- Cells in the brain interact with cancer cells and confer their resistance to HER2-targeting therapies.
- Cell interaction altered gene expression and changed the sugar coating of the cancer cells in the experimental models with similar changes also detected in patients samples.

**IMPACT**
This research in glycosylation suggests that targeting the sugar coating and cancer cells could overcome resistance to treatment and improve patient outcomes.

**Collaborative Research Principal Investigators**

- Lisa Coussens, Ph.D. Oregon Health & Science University
- Kornelia Polyak, M.D., Ph.D. Dana Farber Cancer Institute
- Elana Fertig, Ph.D. Johns Hopkins University
- Valerie Weaver, Ph.D. University of California, San Francisco
Collaborative Research Principal Investigators

Oral Cancer & Immune Resistance

Dr. Davoli previously showed that loss of a specific “p” region of chromosome 9p is more frequent in patients that do not respond to immunotherapy and can be utilized to stratify patients and decide clinical treatment. She and Dr. Gutkind want to understand the molecular mechanism underlying this effect.

During this first year, the Davoli team engineered with the gene-editing tool, CRISPR, several human cancer cell lines to contain or not the loss of genomic regions on chromosome 9p. Furthermore, Dr. Gutkind’s team conducted experiments in mouse models to start dissecting the reason why tumors with 9p are less responsive to immunotherapy.

**IMPACT**
These studies will allow a better understanding how tumor cells evade the recognition and attack by the immune system. Since 9p loss is a common feature of human solid tumors (especially common in oral, lung, melanoma and bladder cancers), these findings have the potential to help us identify patients who will respond or not to immunotherapy as well as improve current immunotherapeutic strategies.

How & Why Metastasis Occurs

Metastasis (spread of cancer) is responsible for over 90% of cancer deaths and loss of quality of life.

Some people are more predisposed to metastasis than others. Among the metastasis-controlling genes discovered by Dr. Welch are some found in the cell part called the mitochondrion. The mitochondrial genes have sequences that differ by race. This research on mitochondrial genes that regulate metastasis has identified a genetic explanation for why some people (ancestries) develop more aggressive (metastatic) cancers than other people/races.

Short RNA products or tRNA-derived fragments (tRF) have been identified from one mitochondrial gene in the mouse. This research aims to understand how tRF work and develop critical tools - a MINTmap - to compare data from the mouse with humans. Dr. Rigoutsos is developing the mouse version of a MINTmap to identify tRF in mouse tissues.

**IMPACT**
This research has high potential to lead to (1) new therapies to stop or treat metastasis, (2) new tools for doctors to identify which tumors are most dangerous and plan treatments, and (3) an explanation of racial disparities observed in cancer outcomes. Once metastasis is blocked, survival and quality of life will improve dramatically.
While 50% of patients with Merkel cell carcinoma (MCC) respond well to immune-based therapies with long-lasting benefits, others do not. As with most cancers, the basis for different outcomes is unknown.

Approximately 80% of MCCs are caused by the Merkel cell polyomavirus, while 20% are caused by sunlight-induced DNA mutations. Each MCC subtype may be ‘seen’ in different ways by the immune system.

Using cutting-edge approaches to study gene expression in TILs (tumor-infiltrating cells), mechanisms of T-cell immunity will be identified within a single cancer type.

The research has begun to study TILs in MCC specimens from patients receiving immunotherapy. Computer modeling also revealed a gene expression profile derived from lung cancer mutation-specific T cells can also identify tumor-specific T cells in MCC specimens.

**IMPACT**
A deeper understanding of the extent and type of immune cells in tumors responding or not to immunotherapy is now possible. This research will allow combinations of existing and emerging therapies to help patients overcome MCC and other virus-driven cancers.

Lung cancer patients with mutations in the ALK gene (or ALK-positive or ALK+) eventually become resistant to the 1st, 2nd, and 3rd line of standard therapies with no other available life-sustaining therapy.

While some advances have been made to address the resistance and re-induce control of cancer, the knowledge is far from complete mainly due to the limit of how much can be assessed using samples of tumors obtained from patients progressing on these ALK inhibitors.

This team is using a breakthrough technology, NanoString GeoMx Digital Spatial Profiler, that identifies and quantifies the genes in standard preserved tissue samples, a method not previously possible. In the past year, they have:

- Determined changes in gene expression in multiple individual compartments of cancer (tumor epithelium, fibroblasts, endothelium)
- Changes suggest novel mechanisms driving ALK inhibitor resistance not previously detected using available methods

**IMPACT**
This new approach can now be extended to larger cohorts of patients with ALK-positive or other types of lung cancer treated with targeted therapies. A deeper understanding of how cancers evolve with targeted therapies can potentially nominate new ways to treat resistant cancers and save lives.
New Targets for Novel Treatments for Triple-Negative Breast Cancer

Dr. Daniel Haber is collaborating with Dr. Esther Rheinbay to confirm a target to develop a new treatment for the most deadly subtype of breast cancer, triple-negative breast cancer (TNBC). Overexpression or amplification of the Androgen Receptor (AR) occurs in 10% of TNBC. However, unlike prostate cancer, AR expression abnormalities in TNBC do not respond to anti-androgen treatment.

Dr. Haber’s team obtains sequencing and DNA or genomic data and RNA or transcriptome data from patients’ CTCs with and without AR abnormalities. Dr. Rheinbay’s lab analyzes the ‘omic’ data to identify genes, transcription factors, and DNA regulators that contribute to the lack of response of anti-androgens.

In the past year, they discovered:
- AR directs a completely different set of cellular pathways in breast cancer cells, compared with the estrogen receptor
- Therapeutic targeting of the AR in breast cancer must consider these new pathways.

**IMPACT**
They expect their studies will reveal a unique role for manipulating androgen signaling in TNBC and could lead to a treatment option, giving patients with TNBC the hope they need.

Immune Activating Therapies for Breast Cancer

Most women in the earlier stages of breast cancer choose a surgery that minimizes the amount of normal breast tissue removed. However, to prevent the cancer from returning, it is important that all the cancer is removed, and none is left behind. To address this unmet clinical need, this team has developed a dual-purposed targeted molecule that allows tumor visualization and treatment during surgery.

In 2022, the team developed a mouse breast cancer model and demonstrated a selective biomarker expressed on the model’s primary and metastatic breast cancer. With a chemical probe that recognizes the biomarker, the scientists demonstrated activation of the probe after bound to the breast cancer slows the progression of cancer and decreases metastasis.

**IMPACT**
Probe activation will turn on an immune response to attack the cancer. Future studies will test the potential benefit of combining the activation of this unique chemical probe with immune-activating therapies to provide a more durable response and permanent tumor immunity.
Collaborative Research Principal Investigators

**CAR-T Cell Immunotherapy for Pancreatic Cancer**

The research team is applying the latest genetic engineering technology to patient-derived disease models to develop a novel CAR-T cell therapy for pancreatic cancers.

There are currently no effective CAR T cell therapies (or T cell-based therapies) for pancreatic cancer. This team designed new CAR molecules targeting CEACAM6, a tumor-associated antigen that contributes to immune suppression. In 2022, they discovered:

- Two CAR molecules that maintain efficacy against a range of CEACAM6-expressing cell lines.
- The CARs to some extent also target CEACAM5 – which contributes to immune suppression.

**IMPACT**

Targeting both CEACAM antigens may enhance the anti-tumor efficacy of these CAR T cells, preventing tumor escape. This work will proceed with an evaluation of CAR T cell performance in tumor-bearing mouse models to identify promising candidates for future clinical studies.

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**Novel Approach to Suppress the Spread of Cancer**

Seryl-tRNA synthetase (SerRS), an essential protein that can regulate many different processes in our cells, can prevent cancer growth and metastasis in lab models. Drs. Schimmel and Yang, renown experts in the tRNA synthetase proteins, hypothesize that SerRS boosts the activity of the immune system against cancer or that SerRS directly stops cancer in its tracks by blocking key processes for cancer to survive and spread.

The team is uncovering exactly how SerRS interacts with immune cells and immune responses. If this hypothesis turns out to be correct, SerRS could potentially be developed as a novel biological therapy that stimulates the immune system against cancer.

Other results indicate a relationship between SerRS and cancer cells consistent with inhibiting cancer growth and metastasis. If adding SerRS to cancer cells is all it takes to prevent them from growing and spreading, then SerRS could be a unique biologic drug to directly target and suppress cancer in patients.

**IMPACT**

SerRS research may lead to novel therapeutic applications to suppress the growth and metastasis of breast, brain, esophageal, kidney, rectal, stomach, and thyroid cancers.
Brain swelling or cerebral edema is a hallmark of glioblastoma, (GBM), a uniformly deadly brain tumor. Drs. Jain and Suva found that the new type of immunotherapy, known as immune checkpoint blockade - which has revolutionized the treatment of many cancers, increases cerebral edema by approximately 20%.

This is not only neurologically detrimental to GBM patients; it can even be lethal. Most patients who experience edema receive steroids to reduce brain swelling; however, these drugs are highly immunosuppressive and thus counteract the effects of immunotherapy. Therefore, an alternative to steroids is urgently needed.

Using cutting-edge techniques, the team has also identified losartan - a widely prescribed, safe, and inexpensive anti-hypertensive drug, as a pharmaceutical option for edema control. Losartan addresses the underlying cause of immunotherapy-induced edema and also sensitizes the tumors to immune checkpoint blockade therapy.

**IMPACT**
Losartan is safe, effective, and affordable – so it can be readily prescribed along with immunotherapy to patients with glioblastoma.

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Cancer-associated retinopathy (CAR) is a rare complication of cancer in which the body’s immune response to cancer inadvertently also attacks the eye, resulting in permanent blindness. CAR is diagnosed in different types of cancer: lung, breast, gynecologic, colon, pancreatic, skin, and prostate cancer.

Since the human retina cannot regrow itself after injury, this immune attack on the retina results in the permanent death of the rod and cone photoreceptors, the vision cells.

These two ophthalmologists worked closely with scientists who developed the first gene therapy for a genetic disease (type of childhood blindness). They aim to transplant healthy rod and cone cells in the retina to restore vision for CAR patients.

In 2022, their preliminary work has:
- Identified several new populations of developing photoreceptor cells in the mouse
- Begun transplanting the healthy photoreceptor cells into a mouse model of retinal disease mimicking CAR in patients to better understand the ideal cell type to restore vision.

**IMPACT**
This project may lead to the development of new treatments to restore precious vision to cancer patients with retinopathy.
Our Efforts to Advance New Brain Cancer Treatments

The fatal brain cancer, glioblastoma (known as GBM) takes the lives of most patients after 8-15 months after their diagnosis. Sadly, there have been no new effective treatments in several decades to change this dismal outcome.

To address this unmet need, NFCR helped to initiate a collaboration of knowledge networks of world-class cancer researchers and oncologists to meet this challenge. An innovative, patient-centric trial platform—GBM AGILE—was founded with funding from NFCR and other non-profit organizations. GBM AGILE efficiently tests new treatments, advancing effective ones faster and rejecting ineffective ones quickly - vastly different than standard clinical trials.

Now that GBM AGILE is launched and operational with multiple arms of drugs being tested simultaneously, NFCR is focusing on supporting more candidate treatments to get through pre-clinical research and Phase I clinical trials so that they can enter GBM AGILE, and ultimately, benefit GBM patients. NFCR's most dedicated and talented scientists in our network are working on several lead drug candidates in the pre-clinical research stage. Pre-clinical research includes:

- **Demonstrate** in cancer models that the drug has the intended anti-cancer effect
- **Monitor** in cancer models any adverse side effects
- **Determine** how the drug will be metabolized in patients
- **Conduct** toxicity profiles
- **Validate** methods in drug manufacturing
- **Develop** a step-by-step plan of how the new drug will be evaluated in patients
- **Submit** the Initial New Drug Application (IND) to the U.S. FDA (Food and Drug Administration)

NFCR In the News: Addressing GBM and Sharing Awareness of GBM AGILE

On April 20, 2022, NFCR CEO Sujuan Ba and Scientific Advisory Board Chair Web Cavenee joined NBC4 Washington’s Doreen Gentzler to talk about the passing of beloved journalist Wendy Rieger from GBM.

“GBM AGILE is hundreds of researchers and physicians who want to provide hope to these patients and to make this terrible situation maybe a little bit better. Hopefully, we will find something that will solve this problem,” said Dr. Cavenee.
The 3rd Annual Global Summit was held in Washington, D.C., at The National Press Club on October 22, 2022. NFCR CEO and President, and AIM-HI Accelerator Fund, CEO and Co-Founder, Sujuan Ba, Ph.D., welcomed research leaders from academia, government, industry, and the investment community, and cancer research advocates and patients — to come together around the shared mission to end the suffering and loss of life from cancer.

For our virtual attendees, the day and evening programs were live-streamed through the NFCR Oncology Metaverse, our digital conference platform.

During the day, NFCR scientists and entrepreneurs, and other renowned cancer research leaders presented the highlights of their latest discoveries and breakthroughs and their visions for their research to change the cancer research landscape.

Dr. Web Cavenee, Chair, NFCR Scientific Advisory Board, moderated the panel on Molecular Approaches to Understand and Treat Cancer. One NFCR team is using immunotherapy to save more patients with Merkle Cell skin cancer. Another team is pioneering gene and cell replacement therapy to restore vision loss occurring in some cancer patients.

Dr. Jennifer Grandis, Prof. Head and Neck Surgery, at the University of California, San Francisco, was the moderator of New Approaches to Treat Breast, Pancreatic, and Head and Neck Cancers. Highlights included novel targets for a new treatment for triple-negative breast cancer and CAR-T Cell therapy for pancreatic cancer.

Dr. Raju Kucherlapati, Prof. Genetics of Harvard, led a panel of leaders of several AIM-HI portfolio companies. Some of the latest results included multi-functional antibodies for gastrointestinal cancer and the advanced cancer detection technology used in research hospitals globally.
At the 2022 Global Summit, the AIM-HI Accelerator Fund (AIM-HI) introduced a first-of-its-kind award to elevate the impact of women leaders in cancer research - the **Beacon Award for Women Leaders in Oncology**.

The **Award** recognizes outstanding women leaders in all sectors of the health and life sciences industry who have made a significant impact on advancing cancer treatment, detection, and diagnosis for patients worldwide through the development and commercialization of novel technologies, advocacy, and/or implementation of public policy.

The Beacon Award Selection Committee awarded the **inaugural Beacon Award for Women Leaders in Oncology** to the distinguished **Anna D. Barker, Ph.D.**, Chief Strategy Officer at the Lawrence J. Ellison Institute for Transformative Medicine.

Dr. Barker is honored for her accomplishments in championing, designing, and implementing innovative, often unprecedented, scientific, and clinical programs that have enabled advances in the field of cancer research and provided critical support for the development of precision oncology.

These programs, notably, **The Cancer Genome Atlas (TCGA)** and **GBM AGILE**, have engaged numerous institutions, clinicians, researchers, and patients to catalyze new thinking and novel approaches to solve major scientific and clinical problems in cancer.

- **TCGA** impacts virtually every area of cancer research, for example, 33 tumor types, including 10 rare cancers that were molecularly characterized by TGCA teams, and provided an unprecedented data resource for precision oncology.

- **GBM AGILE**, the adaptive clinical trial effectively changes how the most promising treatments will efficiently be evaluated for patients with the fatal brain cancer, glioblastoma (GBM). - See page 14 for updates in **GBM AGILE this year by NFCR** - one of the knowledge networks.
2022 AIM-HI Women's Venture Competition

The annual AIM-HI Women's Venture Competition initiative advances women-led enterprises in the oncology field, supports female researchers, and recognizes their scientific achievements.

The competition provides critical early-stage funding for women entrepreneurs in oncology.

“Too few medical breakthroughs and innovative technologies ever become new treatments due to lack of funding. It is more challenging for women-led companies and researchers to get funded. We need to change that.”

– Sujuan Ba, Ph.D.
President and CEO, NFCR
Co-Founder and CEO, AIM-HI Accelerator Fund

It also provides other essential resources to participating startups, including mentoring visibility, or developing time-efficient, strategic connections, and networking with AIM-HI’s global network of innovators, investors, and influencers in the oncology subsector of the life sciences industry.

At the 2022 Global Summit and Award Ceremonies for Cancer Research & Entrepreneurship, the AIM-HI Accelerator Fund congratulated the 2022 Women’s Venture Competition top finalists.

An esteemed judging committee consisting of world-class life sciences industry professionals, business leaders, investors, and entrepreneurs initially selected six semi-finalists before deciding the final top three 2022 Finalists.

First Place with distinction: Johanna Webb, President & CEO of RiverWalk Therapeutics; Second Place: Revital Mandil-Levin, Ph.D., CEO & Co-Founder of Nanocarry Therapeutics; and Third Place: Rosa Hwang, M.D., Co-Founder of Stellanova Therapeutics.

RiverWalk Therapeutics has novel inhibitors of a key metastasis signaling pathway and immune suppressor found in triple-negative breast cancer and kidney, ovarian, and non-small cell lung cancer.
Dr. Rakesh Jain discovered that tumors have structurally and functionally abnormal blood vessels resulting in leaky blood vessels that cause edema, lack of oxygen, and immunosuppression. He 'normalized' the abnormal vessels using anti-angiogenic approaches, allowing drug delivery and efficacy of anti-cancer medicines.

His research laid the foundation for clinical trials and approval of seven combinations of anti-angiogenic drugs with checkpoint blockade immunotherapy to enhance their efficacy in lung, liver, endometrial, and kidney cancer patients.

Attendees of the Global Summit honored the recipient of NFCR’s 2022 Szent-Györgyi Prize for Progress in Cancer Research, Dr. Rakesh Jain of Harvard and Massachusetts General Hospital.

The blue-ribbon Prize Selection Committee of renowned leaders in cancer research elected Dr. Jain for his pioneering research on overcoming barriers posed by the tumor microenvironment (TME) which led to the improved delivery and efficacy of anti-cancer medicines.

"I have had the good fortune to collaborate with so many talented students, clinicians, other world leaders, and of course, patients who participated in the trials. Therefore, being recognized by NFCR for contributions to basic and translational oncology is an enormous honor."

-Rakesh K. Jain, Ph.D.
Professor of Tumor Biology, Harvard Medical School
Director of the Edwin L. Steele Laboratory for Tumor Biology, Boston Massachusetts

"Dr. Rakesh Jain’s seminal discoveries in basic and translational research have guided numerous fields in cancer research with the promise of saving lives," remarked Sujuan Ba, Ph.D., Co-chair of the 2022 Prize selection committee and President and CEO of NFCR.

"These are the pillars of the Szent-Györgyi Prize. Incidentally, Dr. Jain has been continuously funded by the NFCR since 1998. We are delighted and proud that he is receiving the 2022 Szent-Györgyi Prize."

2018 Szent-Györgyi Prize Winner, Douglas Lowy, M.D., Principal Deputy Director of the National Cancer Institute, introduced Dr. Rakesh Jain to the audience, and together with Sujuan Ba, presented the Szent-Györgyi Prize award to him.
Accolades for NFCR and the AIM-HI Accelerator Fund

In 2022, both NFCR and the AIM-HI Accelerator Fund were nominated by local and international prestigious programs, respectively. Each nomination recognizes the many significant ways our non-profits advance our missions. We want to thank all of our donors and supporters for your trust and partnership with us to make cures possible.

**NFCR - A Moxie Award Finalist for Boldness in Business.**

The Moxie Award honors the achievements of growing businesses, nonprofits, and associations in the Washington, DC metro community. Organizations are recognized for having demonstrated boldness and innovation as an integral part of their growth strategy. 2022 marks the first time NFCR is a finalist for this honor.

- In 2022, NFCR welcomed three new Board Directors to help us amplify our impact on cancer patients.
- NFCR distinguishes itself from other cancer charities by funding research others won’t, such as early-stage metastasis research. As a result, this research often leads to the development of critical new fields of cancer research.

**A PRIX GALIEN USA Nominee for Best Incubator, Accelerator, and Equity**

The US Prix Galien is an international awards program dedicated to progress through innovative medicines development.

In 2022, the newest Prix Galien Award category, "Incubators, Accelerators and Equity," acknowledges the role played by organizations in guiding the next wave of innovators, by offering a range of mentoring skills including clinical trial design expertise, legal counsel, lab space, professional development, peer-to-peer support, free access to leading advisors and economic support to advance life science innovation.

- At AIM-HI, we bridge the gap between innovative, early-stage cancer research and successful development of high-impact oncology products.
- Our integrated approach combines essential funding with development and business expertise, leading to successful oncology companies that bring forth new biomedical discoveries to benefit society.

- Our Oncology Metaverse connects experts and individuals in the cancer community
- GBM AGILE will serve as a more efficient, cost-effective, and accelerated drug development model, helping patients survive even the deadliest cancers
- The Women’s Venture Competition, in partnership with the AIM-HI Accelerator Fund empowers women scientists-entrepreneurs in oncology

To be nominated alongside other esteemed nominees is a testament to our non-profit approach to impact underserved and underfunded sectors in cancer through the hard work and dedication of our staff, founders, advisory members, directors, and volunteers.
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WAYS TO CONTINUE TO SUPPORT NFCR

Here are some ideas about how you can help support the NFCR community—but we’d love to hear your ideas as well!

**Champion for a Cure**
Join our monthly giving program. This saves on fundraising costs, freeing up funds to sustain our scientists. For more information, visit: nfcr.org/monthly.

**Honor & Memorial Giving**
In honor of a cancer survivor or in memory of a loved one, your gift provides a meaningful tribute to someone whose life has been impacted by cancer.

**Create a Legacy**
Remember NFCR in your will or living trust. It’s easy to arrange and may be changed at any time you choose through a provision or amendment prepared by your attorney.

You may also want to consider a Charitable Gift Annuity, which guarantees an income for life for a donor and/or a donor’s spouse, with a portion eligible for tax deduction.

**Stock Gifts**
Donating with long-term securities, including stocks and bonds, can offer significant tax benefits.

**Charitable IRA Rollovers**
Donate directly from your traditional or ROTH IRA. Donors must be at least 70 ½ years old. Check with your attorney on the benefits of your IRA contribution.

**Corporate Matching Gifts**
Does your employer have a matching gift program? It is a great way to maximize or even double your impact! Check with your HR Department for guidelines and gift matching forms. You can also discover more by visiting: nfcr.org/employermatch

NFCR gratefully accepts donations via cash, credit cards, donor advised funds (DAF), PayPal, Apple Pay, or Electronic Funds.

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