# ANNUAL REPORT 2015

































SAMUEL WAXMAN CANCER RESEARCH FOUNDATION





### ANNUAL REPORT 2015

The Samuel Waxman Cancer Research Foundation is an international organization dedicated to curing and preventing cancer. The Foundation is a pioneer in cancer research, focusing on uncovering the causes of cancer and reprogramming cancer cells. Our mission is to eradicate cancer by funding cutting-edge research that identifies and corrects abnormal gene function that causes cancer. This research is the basis for developing minimally toxic treatments for patients.

Through our collaborative group of world-class scientists, the Institute Without Walls, investigators share information and tools to speed the pace of cancer research. Since our inception in 1976, The Foundation has awarded approximately \$90 million to support the work of more than 200 researchers across the globe.

# OUR VISION

June 2016

Dear friend,

The Samuel Waxman Cancer Research Foundation (SWCRF) is marking its fortieth anniversary and we're grateful for the progress we've made over the past four decades thanks to your support. Your generosity has sustained science that has contributed to the emerging control of cancer. In 1976, the SWCRF pioneered the model of cross-institutional collaboration between world-class scientists focused on identifying and developing a targeted therapy to neutralize cancercausing abnormal gene function.

An example of how this approach can yield success is our collaboration with researchers from Shanghai on a breakthrough cure for acute promyelocytic leukemia (APL). Using differentiation therapy, we improved the five-year survival rate of APL patients from 25 percent to 95 percent. This was the first time that a cancer-causing gene product could be the target of therapy and served as a dramatic example of personalized, epigenetic therapy, proving that a once fatal form of cancer could be cured with minimally toxic treatments.

Today, targeted therapy to neutralize abnormal gene function is the buzzword in cancer therapeutics and is being used with conventional chemotherapy. We are at the forefront of this movement as evidenced by several clinical trials, based on the research of SWCRF grantees, described in this Annual Report.

The creation of novel science, its translation to the clinic and securing drug approval is a steep climb with the summit elusive and not often achieved. However, the more climbers participate in the ascent, the more chances there are of its success.

The SWCRF has funded the most talented and motivated teams of climbers who are approaching this summit. As climbers say upon reaching a summit: You have to be in it for the long trek, and you need someone to hold your safety rope. As an SWCRF donor, you hold our rope and share the culture of Team SWCRF who Collaborate for a Cure, and we thank you for your support.

Remember, we can't do this without you.

Sincerely.

Michael Nierenberg

Samuel Waxman, M.D.

Some Wapan

**Spencer Waxman**President

### MICHAEL NIERENBERG



### SAMUEL WAXMAN, M.D.



### SPENCER WAXMAN



### YEAR IN **REVIEW**



### APRIL 20

Palm Bay International teamed up with GOTHAM magazine to benefit SWCRF at its winetasting and silent auction at the New York Public Library.



### MAY 4

The SWCRF honored Dr. Siddhartha Mukherjee, author of The Emperor of All Maladies: A Biography of Cancer, and Dr. Robert Weinberg, founding member of the Whitehead Institute for Biomedical Research at MIT, at its scientific symposium at Mount Sinai.



### **MAY 20**

On May 20, the SWCRF welcomed supporters to its Collaborating for a Cure High Tea held at the New York residence of philanthropist Andrea Stark. The benefit featured an informal fashion show by honoree St. John.



### JULY 11

The eleventh annual A Hamptons Happening raised \$500,000 for research. The honorees were Marc Leder, Bobby Zarin, and St. John.



### **JULY 16**

Longtime SWCRF supporters convened at the lush Creek Club golf course in Locust Valley, NY for the 33rd annual SWCRF golf tournament and dinner hosted by Cynthia and Tony Shogren.



#### **SEPTEMBER 10**

Fashion designers Lauren Leonard Phelps and Sarah Littlejohn held Nightcaps for Cancer, a benefit for the SWCRF's brain cancer research programs.





### **OCTOBER**

The SWCRF Pay It Forward Breast Cancer Awareness Month campaign grew with in-store events held by St. John, which joined Tadashi Shoji, who returned to benefit SWCRF for a second year through sales of a limited edition floral t-shirt and other merchandise on his site.



### **OCTOBER 16**

The Angelo David Salon held Blow Out Breast Cancer, a fundraiser that sold \$25 blow outs and hair brushes to benefit SWCRF's breast cancer research programs.



### **NOVEMBER 12**

The 18th annual Collaborating for a Cure Gala honored menswear designer John Varvatos and raised \$2.5 million for cancer research on Novemeber 12 at Cipriani Wall Street. The musical guest was John Fogerty.

# PROGRESS IN PROCESS

SWCRF donations move science closer to new cancer treatments as our grantees' work leads to hopeful clinical trials.

On the path to developing new cancer drugs, clinical trials are a crucial benchmark. Trials not only vet the efficacy of drugs before receiving approval from the Food and Drug Administration (FDA) but provide critical insight to the scientific community, regardless of their outcomes. In the 1990s, trials confirmed the efficacy of a breakthrough differentiation therapy for acute promyelocytic leukemia developed by Dr. Samuel Waxman, founder and CEO of the Samuel Waxman Cancer Research Foundation (SWCRF), in collaboration with leading researchers from Shanghai – Prof. Zhu Chen and Prof. Zhen Yi-Wang. Today, research funded in part by the SWCRF is powering several trials with the potential to produce new cancer treatments.

Prior to being approved by the FDA, new drugs must be submitted to a series of studies. Phase 1 trials determine a drug's safety and its recommended dosage. In Phase 2, several hundred patients are studied to determine the drug's efficacy and side effects. Phase 3 trials study between 300 and 3,000 patients to further determine a drug's efficacy and to monitor adverse reactions.

SWCRF grantees have several trials in play at various stages, reflecting the quality – and impact -- of the research SWCRF donors are backing. "Our researchers are driving important trials for minimally toxic drugs in many cancers," said Dr. Waxman. "We are proud to support science that is moving us closer to the



JOHN D. CRISPINO, PH.D.

goal of making cancer a chronic disease that can be managed with innovative treatments."

At Northwestern University, research by John D. Crispino, Ph.D., laid the groundwork for a Phase 1 clinical study of the Aurora A kinase inhibitor Alisertib in patients with either acute megakaryoblastic leukemia or primary myelofibrosis.

The trial, which launched in October of 2015, seeks to accrue 24 patients over a two-year period. The trial will expand in the coming months to include patients at the University of Miami Cancer Center and the Mayo Clinic.

Josep M. Llovet, M.D., director of the liver cancer program at the Icahn School of Medicine at Mount Sinai, is overseeing two ongoing trials for potential treatments of two forms of liver cancer. Collaborating with Novartis, Dr. Llovet is conducting a Phase 2 study for BGJ398, an inhibitor of mutations in fibroblast growth factor receptors (FGFR), as a treatment for cholangiocarcinoma, a rare and aggressive cancer also known as bile duct cancer. The trial expects approximately 80 participants. Dr. Llovet is also studying



JOSEP M. LLOVET, M.D.

BLU-5454, another FGFR4 inhibitor, in partnership with Blueprint Medicines, in ongoing Phase 1 and Phase 2 studies involving patients with overexpression of FGF19, a protein that functions as a hormone that regulates bile acid synthesis.

At Johns Hopkins University, Stephen Baylin, M.D., Cynthia Zahnow, Ph.D., and Robert Casero,

Ph.D., are collaborating with Celgene and investigators at the UCLA Medical Center on a Phase 2 clinical trial to study immune checkpoint inhibition in women with relapsed high-grade ovarian cancer. Pending the results of a Phase 1 trial scheduled for this summer to determine optimal dosage of oral Azacytidine (AZA), the trial will involve a randomized placebo controlled study of the anti-PD1 antibody and AZA, which blocks DNA methylation and induces an anti-viral, immune mediated tumor response. The Johns Hopkins team is also conducting a Phase 2 trial testing the effects of the anti-PD1 antibody or anti-PD1 with the HDAC inhibitor Entinostat and AZA in patients with advanced lung cancer. Results

In pediatric cancer, research published by Shai Izraeli, M.D., of the Sheba Medical Center at Tel Aviv University, has led to the opening of a Phase 2 trial conducted by The Children's Hospital of Philadelphia with the NCI's Children's Oncology Group to



SHAI IZRAELI, M.D.

determine the impact of the JAK inhibitor Ruxolitinib combined with chemotherapy to treat a chemotherapy-resistant subtype of acute lymphoblastic leukemia (ALL). The trial protocol is awaiting final revisions pending comments from the FDA. Given positive results, the trial, co-sponsored by Incyte Pharmaceuticals and Novartis, will expand to more sites.

At Mount Sinai, breast cancer investigator Doris Germain, Ph.D., is launching a clinical trial to study fulvestrant, an anti-estrogen drug used to treat estrogen-responsive breast cancer in post-

### SWCRF UPDATES

menopausal women, as a therapeutic agent for younger women with estrogen receptor positive breast cancers who typically are treated with tamoxifen. Research by Dr. Germain and Dr. Waxman showed that the biomarker cyclin D1 is indicative of resistance to tamoxifen but sensitivity to fulvestrant. In the trial, pre-menopausal, estrogen receptor positive breast cancer patients who are also positive for cyclin D1 will be treated with either tamoxifen or fulvestrant for two to three weeks prior to their surgery to determine whether their tumors respond better to fulvestrant.



DORIS GERMAIN, Ph.D.

Last year, Drs. Germain and Waxman completed a five-year Phase 2 trial testing the combination of fulvestrant and bortezomib, a drug used to treat certain blood malignancies, as a therapy for women with metastatic breast cancer who had recurred on prior anti-estrogen therapy. Patients enrolled in the trial were randomized to receive either fulvestrant alone or fulvestrant and bortezomib. The results among 118 patients tested showed that adding bortezomib to fulvestrant doubles the number of patients that had not progressed after one year compared to the group receiving fulvestrant alone. Therefore, the results suggest that adding bortezomib to fulvestrant delays progression in this heavily pretreated patient population.



STEVEN D. GORE, M.D.



RAMON PARSONS, M.D., Ph.D.



ROBERT HROMAS, M.D., F.A.C.P.

### SCIENTIFIC ADVISORY BOARD WELCOMES NEW MEMBERS

The Samuel Waxman Cancer Research Foundation (SWCRF) added three distinguished Scientific Advisory Board (SAB) in the past year. SWCRF welcomed Steven D. Gore, M.D., Director of Hematologic Malignancies at Yale Cancer Center and Clinical Program Leader for Hematology at Smilow Cancer Hospital at Yale-New Haven, and Ramon Parsons, M.D., Ph.D., Professor and Chair of Oncological Sciences at the Icahn School of Medicine at Mount Sinai. to the SAB. The SWCRF also named Robert Hromas, M.D., F.A.C.P., Chair of Medicine at University of Florida Health, to the SAB. Drs. Hromas, Gore and Parsons join their fellow SAB members in evaluating progress reports from the foundation's funded researchers as part of SWCRF's annual grant renewal process. The SWCRF SAB includes Lorraine Gudas, Ph.D., Chair of Pharmacology at Weill Cornell Medical Center, Nancy Speck, Ph.D., Professor of Cell and Developmental Biology at the University and Tumor Immunology, City of Hope NCI

### SCIENTIFIC LEADERS PLAN WORKSHOP ON AGING AND CANCER

The SWCRF's new Aging & Cancer grants program will progress when SWCRF Founder and CEO Samuel Waxman, M.D., Chief Scientific Officer Jonathan Licht, M.D., and Chief Mission Officer Alan

Rosmarin, M.D., hold a workshop titled Aging, Hematopoiesis, and Cancer at Mount Sinai Medical Center. Co-sponsored by Syros Pharmaceuticals, the meeting will gather experts in aging and blood malignancies from across the country for scientific exchange that will set the parameters for research projects to be funded by new collaborative grants targeting new therapies for older blood cancer patients.

### SWCRF NAMES NEW BOARD MEMBERS

The SWCRF named new directors to its board and elevated longtime board members to new officer positions in recent months. Lewis Rubin, Chief Operating Officer of Davis & Gilbert LLP, Kenneth Fishel, principal of Renaissance Properties, and Baron Silverstein, Managing Director of Bank of America Merrill Lynch, were welcomed as directors Spencer Waxman, principal of Shannon River Fund Management, was named board president while Howard Kurz, founder of Lily Pond Management LLC, was appointed a vice president.

The SWCRF board includes Chairman Michael Nierenberg, Vice President Dena K. Weiner, Treasurer Thomas A. Conway, Secretary Laurie L. Schaffran and directors James E. Frankel, Gary Gladstein, Clifford Greenberg, Dennis A. Herman, Linda Herman, David Hinkelman, Gary Jacob, Robert Kantor, Matthew Lyons, Riva Ariella Ritvo-Slifka, Howard Shlafmitz, Tony M. Shogren, David S. Taub† and Samuel Waxman, M.D.

# FORTY YEARS OF COLLABORATING

### **FOR A CURE**



The founding supporters of the SWCRF at Mount Sinai in 1976. From left: Barry Finn, Shelly Finn, Miriam Alpert, Dr. Samuel Waxman, Marion Waxman and Irving Alpert, whose \$10,000 donation helped Dr. Waxman found the SWCRF.

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Dr. Waxman was published in the op-ed section of The Wall Street Journal in 2007 for his insightful essay on the growing impact of epigenetics and targeted therapies in the world of cancer research and treatment.



Philanthropists Sidney and Mary Kantor (pictured here in 1995) launched the SWCRF's first gala benefit and fostered the foundation's fundraising support within the fashion industry. Mrs. Kantor was instrumental to building the SWCRF's collaborative work with Shanghai's leading cancer researchers.



After nearly 20 years, the annual Collaborating for a Cure Gala has become one of the premier benefits in New York City. The 2015 SWCRF Gala honored men's wear designer John Varvatos, continuing its tradition of collaborating with fashion luminaries to support research for a cure.





Dr. Waxman has mentored many scientists during his 45-year tenure at Mount Sinai Medical Center. He is pictured here with the members of his lab team in the 1980s.



Dr. Waxman's collaboration with distinguished researchers from Shanghai --- Prof. Zhu Chen and Prof. Zhen-Yi Wang --- led to their discovery of a successful differentiation therapy for acute promyelocytic leukemia (APL). The therapy improved the five-year survival rate of APL patients from 25 percent to 95 percent and remains the standard of care for the disease



The network of SWCRF-funded researchers convened at Mount Sinai in May 2015 for the annual Scientific Review and Symposium. Since 1976, the SWCRF has awarded approximately \$90 million to more than 200 researchers around the world.

#### **BLOOD MALIGNANCIES**

Ravi Bhatia, M.D., UAB Comprehensive Cancer Center
Sai-Juan Chen, M.D., Ph.D., Shanghai Institute of Hematology
John D. Crispino, Ph.D., Northwestern University
Margaret Goodell, Ph.D., Baylor College of Medicine
Yongkui Jing, Ph.D., Mount Sinai Medical Center
David A. Jones, Ph.D., University of Oklahoma
Ross Levine, M.D., Memorial Sloan Kettering
Jonathan D. Licht, M.D., University of Florida Health Cancer Center
Ari Melnick, M.D., Weill Cornell Medical Center
Ruibao Ren, M.D., Ph.D., Brandeis University
Samuel Waxman, M.D., Mount Sinai Medical Center
Arthur Zelent, Ph.D., University of Miami

### **BRAIN CANCER PROGRAM**

Albert S. Baldwin, Jr., Ph.D., University of North Carolina, Chapel Hill Kevan Shokat, Ph.D., University of California, San Francisco William Weiss, Ph.D., University of California, San Francisco

### **BREAST CANCER PROGRAM**

Albert S. Baldwin, Jr., Ph.D., University of North Carolina, Chapel Hill
Stephen Baylin, M.D., Johns Hopkins University
Robert A. Casero, Ph.D., Johns Hopkins University
Jayanta Debnath, M.D., University of California San Francisco
Eduardo Farias, Ph.D., Mount Sinai Medical Center
Doris Germain, Ph.D., Mount Sinai Medical Center
Wilson Miller, M.D., Jewish General Hospital
Garry Nolan, Ph.D., Stanford University
Samuel Waxman, M.D., Mount Sinai Medical Center
Robert A. Weinberg, Ph.D., Whitehead Institute for Biomedical Research
Cynthia Zahnow, Ph.D., Johns Hopkins University
Arthur Zelent, Ph.D., University of Miami
Ming-Ming Zhou, Ph.D., Mount Sinai Medical Center

### CANCER STEM CELL PROGRAM

Julio Aguirre-Ghiso, Ph.D., Mount Sinai Medical Center
Nabeel Bardeesy, Ph.D., Massachusetts General Hospital
Stephen Baylin, M.D., Johns Hopkins University
Ravi Bhatia, M.D., UAB Comprehensive Cancer Center
Robert A. Casero, Ph.D., Johns Hopkins University
Margaret Goodell, Ph.D., Baylor College of Medicine
Robert A. Weinberg, Ph.D., Whitehead Institute for Biomedical Research
Cynthia Zahnow, Ph.D., Johns Hopkins University

### CHILDHOOD AND YOUNG ADULT CANCERS

Albert Baldwin, Jr., Ph.D., University of North Carolina, Chapel Hill
John D. Crispino, Ph.D., Northwestern University
Christopher A. French, M.D., Brigham and Women's Hospital
Shai Izraeli, M.D., Tel Aviv University
Mark Lemmon, Ph.D., University of Pennsylvania
Yang Shi, Ph.D., Boston Children's Hospital & Harvard Medical School
Kevan Shokat, Ph.D., University of California, San Francisco
William Weiss, M.D., Ph.D., University of California, San Francisco

### **COLON CANCER PROGRAM**

Ronald Evans, Ph.D., Salk Institute
Reuben Shaw, Ph.D., Salk Institute
David A. Jones, Ph.D., University of Oklahoma





#### LIVER CANCER PROGRAM

Stephen Baylin, M.D., Johns Hopkins University
Josep M. Llovet, M.D., Mount Sinai Medical Center
Samuel Waxman, M.D., Mount Sinai Medical Center

### **LUNG CANCER PROGRAM**

Duane Compton, Ph.D., Dartmouth Medical School Ethan Dmitrovsky, M.D., MD Anderson Cancer Center Cynthia Zahnow, Ph.D., Johns Hopkins University

### MOUNT SINAI MEDICAL CENTER - SWCRF LABORATORY (MSMC)

Julio Aguirre-Ghiso, Ph.D., Mount Sinai Medical Center Eduardo Farias, Ph.D., Mount Sinai Medical Center Doris Germain, Ph.D., Mount Sinai Medical Center Yongkui Jing, M.D., Mount Sinai Medical Center Boris Leibovitch, Ph.D., Mount Sinai Medical Center Samuel Waxman, M.D., Mount Sinai Medical Center

### **DEVELOPING EPIGENETIC THERAPY**

Stephen Baylin, M.D., Johns Hopkins University Robert Casero, Ph.D., Johns Hopkins University Sai-Juan Chen, Ph.D., Shanghai Institute of Hematology John D. Crispino, M.D., Northwestern University Ronald Evans, Ph.D., Salk Institute Alan Friedman, M.D., Johns Hopkins University Margaret Goodell, Ph.D., Baylor College of Medicine Shai Izraeli, M.D., Tel Aviv University Yongkui Jing, Ph.D., Mount Sinai Medical Center Jonathan D. Licht, M.D., University of Florida Health Cancer Center Alexander MacKerell, Ph.D., University of Maryland Ari Melnick, M.D., Weill Cornell School of Medicine Wilson Miller, M.D., Ph.D., Jewish General Hospital Gilbert Privé, Ph.D., University of Toronto Ruibao Ren, M.D., Ph.D., Brandeis University Samuel Waxman, M.D., Mount Sinai Medical Center Yue Xiong, Ph.D., University of North Carolina, Chapel Hill Arthur Zelent, Ph.D., University of Miami Ming-Ming Zhou, Ph.D., Mount Sinai Medical Center

### PANCREATIC CANCER PROGRAM

Nabeel Bardeesy, Ph.D., Massachusetts General Hospital Gregory David, Ph.D., NYU School of Medicine

### SIH/SWCRF CO-PI PROGRAM

Sai-Juan Chen, Ph.D., Shanghai Institute of Hematology Zhu Chen, M.D., Ph.D., Shanghai Institute of Hematology

### **TUMOR DORMANCY PROGRAM**

Julio Aguirre-Ghiso, Ph.D., Mount Sinai Medical Center Emily Bernstein, Ph.D., Mount Sinai Medical Center Eduardo Farias, Ph.D., Mount Sinai Medical Center

# HONOR ROLL

SWCRF collaborators are ascending to leadership roles in science, business and beyond, reflecting the foundation's heritage of funding excellence in cancer research.



JONATHAN D. LICHT, M.D., SWCRF Chief Scientific Officer, was appointed Director of the University of Florida Health Cancer Center.



DOUGLAS LOWY, M.D., a former member of the SWCRF's Scientific Advisory Board, was named Acting Director of the National Cancer Institute.



BENJAMIN G. NEEL, M.D., PH.D., SWCRF collaborator at the Ontario Cancer Institute at Princess Margaret Cancer Center, was named Director of The Laura and Isaac Perlmutter Cancer Center at NYU Langone



ETHAN DMITROVSKY, M.D., former Associate Director of the SWCRF, was appointed Provost of MD Anderson Cancer Center.



RAVI BHATIA, M.D., SWCRF funded researcher at City of Hope, was appointed Director of the Division of Hematology and Oncology and Deputy Director of the University of Alabama's Comprehensive Cancer Center.



The Salk Institute promoted SWCRF collaborator **REUBEN SHAW**, **PH.D.**, to Director of Salk's National Cancer Institute-designated Cancer Center.



SWCRF collaborator **JAMES E. BRADNER, M.D.,** Dana-Farber Cancer
Institute, was named President of the



PROF. ZHU CHEN, M.D., former Director of the Shanghai Institute of Hematology, is now Vice Chairman of the Standing Committee of the National People's Congress of the People's Republic of China.



### THANK YOU

The following donors have shared The Samuel Waxman Cancer Research Foundation's vision and belief in collaboration by generously funding the Foundation's Institute Without Walls in 2015.

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We have carefully reviewed all gifts made to the Samuel Waxman Cancer Research Foundation to ensure each gift is properly recognized. The Thank You list includes all gifts made between January 1, 2015 and December 31, 2015. Gifts mentioned exclude in-kind donations and multiple year pledges.

We make every effort to follow the donor's preferences when compiling the annual report list. If you would prefer to have your name listed differently in the future, please let us know. If we have made a mistake, we sincerely apologize for our error and ask you to bring it to our attention. Please contact Dan Orvieto at 646-398-5260 so the record may be corrected. Thank you.

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# FINANCIAL STATEMENT

(July 1, 2014 - June 30, 2015 audited)

### **REVENUE**

\$235,000
1,069,000
1,566,000
5,000

Total Revenue \$2,875,000

### **EXPENSE**

Program Services	\$2,364,000
Fundraising	277,000
Management and General	251,000

Total Expense \$2,892,000

### SURPLUS/(DEFICIT)

(\$17,000)

# SAMUEL WAXMAN

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