



# 25 RESEARCH BREAKTHROUGHS

FUNDED BY CYCLE FOR SURVIVAL



MEMORIAL SLOAN KETTERING | EQUINOX

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## FUNDED BY CYCLE FOR SURVIVAL

As we begin 2017, Cycle for Survival has raised over **\$105 million**. 100% of that has already been invested in research led by Memorial Sloan Kettering. Together, we are revolutionizing the way cancer is diagnosed and treated.

The following breakthroughs highlight our impact.



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## Next Generation Technology that Uncovers What Causes Cancer and Provides Insights for Treatment

Impacting the study of all cancers at MSK

### David B. Solit, MD

*Director, Marie-Josée and Henry R. Kravis Center for Molecular Oncology (CMO); Genitourinary Oncology Service; Geoffrey Beene Chair*

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**The Battle:** Researchers need next-generation sequencing machines to process tumor samples and help develop personalized treatments for individual patients, including those who may not be responding to standard treatments.

### Cycle for Survival funded...

The purchase of a million dollar state-of-the-art genome sequencer.

### And now...

This is changing the face of cancer research, a leap forward for researchers – many of whom receive Cycle for Survival funding – to develop personalized therapies that provide hope for patients with all types of cancer.

## Quicker Treatment Assessments Using a “Game-Changing” Blood Draw

Impacting the study of all cancers at MSK

### David B. Solit, MD

*Director, Marie-Josée and Henry R. Kravis Center for Molecular Oncology (CMO); Genitourinary Oncology Service; Geoffrey Beene Chair*

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**The Battle:** Traditional surgical biopsies can be invasive and aren't always comprehensive, but a new liquid biopsy technology has been recently developed to provide more comprehensive data through less invasive means. Funding is needed to invest in new lab machinery with this technology.

### Cycle for Survival funded...

A \$1.2 million state-of-the-art liquid biopsy system, which generates data from a simple blood sample.

### And now...

Doctors and researchers are calling this technology a “game changer” – leading to faster and even more targeted treatment decisions through a less invasive and more comprehensive way to research tumors.

## Genetic Discoveries Enabling Clinical Trials for Pancreatic Cancers with No Long-Term Treatment Solution

Pancreatic neuroendocrine tumors (panNETs)

**Diane Reidy Lagunes, MD**

*Gastrointestinal Oncology Service*

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**The Battle:** Surgery, chemotherapy, and radiation only briefly extend lives of panNET patients. There is a dire need for more funding and research to discover a permanent solution by better understanding this cancer and its growth.

### **Cycle for Survival funded...**

Research which revealed – through the sequencing and testing of tumors – multiple occurrences of the same genetic mutation never before identified in panNET tumors.

### **And now...**

Dr. Reidy continues her work aimed at discovering long-term treatment solutions by sequencing more and more tumors in an effort to find and target genetic mutations through clinical trials.

## Genetic Discovery Leading to a First-Ever Clinical Trial for an Ovarian Cancer that Few Women Survive

Small cell carcinoma of the ovary, hypercalcemic type

**Mrinal M. Gounder, MD**

*Sarcoma Medical Oncology Service*

**David Hyman, MD**

*Director, Developmental Therapeutics*

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**The Battle:** Treatment options for small cell carcinoma of the ovary are extremely limited and ineffective. Few women survive this aggressive form of rare cancer.

### **Cycle for Survival funded...**

A study which discovered a second universally mutated gene that, when inactive, leads to increased cancer growth.

### **And now...**

The first-ever clinical trial for small cell carcinoma of the ovary, hypercalcemic type, will begin in 2016 – focusing on activating these mutated, inactive genes and giving hope for the first time to patients of this rare cancer.

## Worldwide Training to Improve Survival Rates and Eyesight Preservation for Children with Retinoblastoma

Retinoblastoma, a form of rare eye cancer

**David H. Abramson, MD**

*Chief, Ophthalmic Oncology Service, Department of Surgery*

**The Battle:** Worldwide, half of these children die from the cancer and over 90% of survivors require eye removal. There's a need to train doctors around the globe on MSK's improved treatment techniques that lead to 99% survival rates and only 5% eye loss.

### **Cycle for Survival funded...**

Dr. Abramson's travel around the globe to train physicians from 45 countries (nearly every continent) in the more effective techniques developed at MSK that don't involve direct chemotherapy to the eye.

### **And now...**

Retinoblastoma patients across the world have renewed hope, with the dissemination of this new technique that is not only saving eyes, but also saving lives.



## A Targeted Treatment Method for a Highly Fatal Pediatric Brain Cancer

Pediatric brain cancer – DIPG (diffuse intrinsic pontine glioma)

**Ira J. Dunkel, MD**  
*Department of Pediatrics*

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**The Battle:** This is an almost always fatal disease because medications are unable to reach brain tumors directly in standard treatments typically given by mouth or IV.

### Cycle for Survival funded...

The research leading to an ongoing clinical trial which is testing a new treatment option that combines a new technique for administering medication with a new drug to more directly target a brain tumor.

### And now...

Doctors at MSK will continue trials to monitor how patients are responding over time to this new option that provides hope for children and their families impacted by such a deadly disease.

## Testing a New Chemotherapy Delivery Method for Highly Recurrent Ovarian Cancer

Ovarian, fallopian tube, and primary peritoneal cancer

**Roisin E. O’Cearbhaill, MD**  
*Gynecologic Medical Oncology Service*

**Oliver Zivanovic, MD, PhD**  
*Director, Innovative Surgical  
Technology, Gynecology Service,  
Department of Surgery*

**Dennis S. Chi, MD, FACOG, FACS**  
*Deputy Chief and Head, Section of Ovarian  
Cancer Surgery, Gynecology Service,  
Department of Surgery*

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**The Battle:** For women with recurrent ovarian cancer, the cancer tends to return more and more frequently over time despite additional chemotherapy.

### Cycle for Survival funded...

A clinical trial to test if adding heated chemotherapy (HIPEC) at the time of surgery (for recurrent ovarian cancer), followed by standard chemotherapy post-surgery, yields better results than standard chemotherapy alone.

### And now...

Researchers have been able to safely give the chemotherapy at the time of surgery. They will continue to enroll patients on the trial, further evaluate HIPEC effectiveness, and determine if patients can live cancer-free for longer periods of time.

## Discovery of Mutated Bladder Cancer Genes that Can Be Targeted for Treatment

Small cell carcinoma of the bladder

**Hikmat A. Al-Ahmadie, MD**

*Department of Pathology*

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**The Battle:** Diagnosed primarily at advanced stages, most patients see their cancer spread or return after treatment.

### **Cycle for Survival funded...**

A new area of research that uses whole genome sequencing of tumor cells to analyze the disease's genetic makeup so researchers can develop treatments to target mutated genes that drive cancer growth.

### **And now...**

Dr. Al-Ahmadie is investigating the exceptional response of one patient who was cancer-free after receiving a new treatment, and this research helped secure funding to use this sophisticated technique to analyze other rare types of bladder cancer.

## Immunotherapy Drug Successful for Melanoma Being Tested for Sarcoma

Sarcoma

**Sandra P. D'Angelo, MD**

*Sarcoma Medical Oncology Service*

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**The Battle:** Once a sarcoma patient's tumor starts spreading – which happens in 25-50% of sarcoma cases – they rarely respond to treatment. There is dire need for more effective therapies.

### **Cycle for Survival funded...**

Pre-clinical studies for a trial to see if a drug successful in treating melanoma might be effective as part of an immunotherapy drug treatment for sarcoma – an idea inspired by the growing field of research behind using patients' immune systems to fight back against cancer cells.

### **And now...**

There are nearly 80 patients enrolled in the nationwide clinical trial, as doctors and researchers continue to study patient responses and begin to develop further clinical trials for immunotherapy treatments.





## Drug Trials for a Salivary Gland Cancer with No Previously Proven Treatment

Salivary Gland Cancer - Adenoid cystic carcinoma

**Alan L. Ho, MD, PhD**

*Head and Neck Oncology Service; Geoffrey Beene Junior Faculty Chair*

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**The Battle:** No standard proven treatments are available for this cancer, which often spreads to other parts of the body.

### **Cycle for Survival funded...**

Trials for two drugs designed to interfere with tumor growth that had yet to be tested with this form of rare cancer.

### **And now...**

Dr. Ho is using several methods, including analyzing tumor tissue, to help develop for the first time viable options for patients that previously had none.



## Genetic Mutation Discoveries Leading to More Personalized Sarcoma Treatments

Sarcoma

**Mrinal M. Gounder, MD**

*Sarcoma Medical Oncology Service*

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**The Battle:** Few treatment options are available for these aggressive tumors, which require highly-tailored therapies because there are more than 50 subtypes of this cancer.

### **Cycle for Survival funded...**

The sequencing of tumors from more than 600 sarcoma patients.

### **And now...**

This sequencing is rapidly matching sarcoma patients to clinical trials for treatments that precisely target newly discovered genetic mutations.

## Improving Quality of Life for Non-Hodgkin Lymphoma Patients Through Tailored Treatment

Non-Hodgkin Lymphoma – Diffuse large B cell lymphoma (DLBCL)

**Craig H. Moskowitz, MD**

*Steven A. Greenberg Chair; Clinical Director, Division of Hematologic Oncology*

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**The Battle:** While many patients are fortunate to beat this disease, too many go on to have poor quality of life – and in some cases, die – due to side effects caused by the especially aggressive cancer treatment.

### **Cycle for Survival funded...**

A study that used a sophisticated imaging technique to more accurately evaluate how much medicine is needed for a specific patient.

### **And now...**

Doctors are able to make better informed treatment decisions for patients with this type of cancer, which decreases the likelihood of avoidable long-term side effects — thereby improving patients' quality of life.

## A New Trial Testing a Personalized Treatment for Brain Cancer Patients

Brain cancer (gliomas)

**Ingo K. Mellinghoff, MD**

*Vice Chair for Research, Department of Neurology;  
Evnin Family Chair in Neuro-Oncology*

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**The Battle:** Treatments are often ineffective, and the available drugs don't always reach the tumor.

### **Cycle for Survival funded...**

Research into a medicine that slows glioma tumor growth in patients with a particular genetic mutation.

### **And now...**

The first-ever glioma clinical trial for this drug is now under way, using an MSK-developed imaging technique that avoids an invasive brain biopsy to monitor patients' response.

## Uncovering the Genetics Responsible for Highly Lethal Pancreatic Cancer Growth

Pancreatic cancer

**Steven D. Leach, MD**

*Director, David M. Rubenstein Center for Pancreatic Cancer Research*

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**The Battle:** Only 7% of patients survive more than five years after their diagnosis, because pancreatic cancer is typically detected in its late stages when the disease has already spread to other parts of the body.

### **Cycle for Survival funded...**

A sophisticated DNA sequencing machine and state-of-the-art liquid biopsy system that have begun to uncover what genetic alterations are responsible for this cancer's growth.

### **And now...**

This research brings new hope to patients suffering from an almost unbeatable disease, and opens the door to new clinical trials that target specific mutations – one of which has seen dramatic tumor shrinkage in two-thirds of patients.

## “Reprogramming” Thyroid Cancer Cells to Become Less Resistant to Treatment

Thyroid cancer

**James A. Fagin, MD**

*Chief, Endocrinology Service*

**Alan L. Ho, MD, PhD**

*Head and Neck Oncology Service;  
Geoffrey Beene Junior Faculty Chair*

**The Battle:** Radioactive iodine (RAI) therapy has been used to treat thyroid cancer for decades, but many cancer cells become resistant to this therapy during the course of treatment.

### Cycle for Survival funded...

Extensive lab work and clinical trials to test drugs that “reprogram” thyroid cancer cells to become less resistant to RAI treatment.

### And now...

Numerous clinical trials are under way, which are testing a variety of drugs that reprogram cells and improve RAI therapy – providing thyroid patients with new promise after years of having few options.



## Clinical Trials Launched After Genetic Mutations Uncovered in Ovarian Cancer

Ovarian cancer

**Carol Aghajanian, MD**

*Chief, Gynecologic Medical  
Oncology Service*

**Rachel N. Grisham, MD**

*Gynecologic Medical Oncology Service*

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**The Battle:** Low-grade serous ovarian cancer is a rare type of ovarian cancer, which often affects young women and does not respond to chemotherapy.

### **Cycle for Survival funded...**

The molecular profiling of this disease, a process that uncovered new genetic mutations in certain patients with this specific cancer.

### **And now...**

A national Phase I clinical trial and an international Phase III clinical trial have been completed, using a drug that combats these mutations. MSK is analyzing the data to help as many patients as possible with this targeted therapy.

## Genetic Mutation Identified that Drives Bladder Cancer

Bladder cancer, plasmacytoid variant carcinoma type

**David B. Solit, MD**

*Director, Marie-Josée and Henry R. Kravis Center for Molecular Oncology (CMO); Genitourinary Oncology Service; Geoffrey Beene Chair*

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**The Battle:** This aggressive cancer spreads early, recurs quickly after surgery, and is often resistant to chemotherapy.

### **Cycle for Survival funded...**

The genetic sequencing that detected a disease-causing mutation.

### **And now...**

Doctors will use this information to correctly classify the disease earlier, giving patients the best chance of survival through personalized treatment – which includes a promising new immunotherapy drug.

## Molecule Discovered That Limits the Growth of Pancreatic Cancer

Pancreatic cancer, gastrointestinal tumors, sarcoma, and other cancers

**Marilyn Resh, PhD**

*Cell Biology Program*

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**The Battle:** No drugs exist to block the Hedgehog acyltransferase (Hhat) protein which is known to drive cancer growth in several rare cancers.

### Cycle for Survival funded...

The study that discovered a molecule that blocks this protein – thus inhibiting the growth of these cancers.

### And now...

Chemists are working with pharmaceutical companies to improve a drug that uses this molecule to stop cancer growth.

## The First-Ever Trial of a Treatment Specifically for Appendix Cancer

Tumors of the appendix, colon, and rectum that have spread to the abdominal lining

**Andrea Cercek, MD**

*Gastrointestinal Oncology Service*

**Garrett M. Nash, MD, MPH,**

**FACS, FASCRS**

*Colorectal Service*

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**The Battle:** Doctors treat appendix cancer with the same chemotherapy as colon cancer, but it has been ineffective for many patients.

### Cycle for Survival funded...

- The first-ever randomized trial comparing two types of intra-abdominal chemotherapy after surgery to determine which is more effective for appendix cancer
- Genetic studies of the tumors to better define appendix cancer

### And now...

Investigators continue to add more patients and new tests to clinical trials – providing insight and hope for patients who previously had little.





## Research Leading to New Drug and Immunotherapy Trials for Gastrointestinal Tumors

Gastrointestinal stromal tumors (GIST)

### Ronald DeMatteo, MD, FACS

*Vice Chair, Department of Surgery; Head, Division of General Surgical Oncology;  
Leslie H. Blumgart Chair in Surgery*

**The Battle:** Current treatments don't kill every cancer cell, so tumors return despite treatment.

### Cycle for Survival funded...

Key initial research into two new strategies:

- Finding drugs that block cancer-promoting proteins
- Using a patient's own immune system to fight GIST (immunotherapy)

### And now...

Multiple clinical trials are being launched and developed based on this research, combining both immunotherapy and cancer-blocking drugs to stop GIST growth.



## A Powerful New Immunotherapy Combination to Boost Myeloma Patients' Immune System After Stem Cell Transplant

Multiple Myeloma

**Alexander M. Lesokhin, MD**

*Myeloma Service*

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**The Battle:** Patients' immune systems are weakened by the stem cell transplant procedure, making it extremely difficult to recover and fight cancer at the same time.

### **Cycle for Survival funded...**

A clinical trial evaluating a new treatment that strengthens immune cells—helping patients rebound after a stem cell transplant, fight the cancer, and prevent it from returning. Patients are given immunotherapy drugs to train T-cells (immune cells) to recognize the cancer as foreign—and attack.

### **And now...**

Drs. Alexander Lesokhin and David Chung will continue enrolling patients into the trial, and measure how their T-cells and cancer cells react to the treatment.

## A New Radiation Technique to Destroy Cancer and Protect the Lungs of Patients with Mesothelioma

Malignant Pleural Mesothelioma

**Andreas Rimner, MD**

*Radiation Oncologist*

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**The Battle:** Comprehensive radiation therapy is too dangerous for mesothelioma patients whose lungs are intact.

### **Cycle for Survival funded...**

A clinical trial offering patients with mesothelioma a groundbreaking radiation technique called intensity modulated radiation therapy (IMRT). It specifically targets the outside lining of the lung—which contains the cancer—while minimizing damage to the lung itself.

### **And now...**

MSK researchers are teaching other hospitals how to use IMRT, and working with them to coordinate treatment and monitor results for patients in the multi-center clinical trial.

## A Study of Millions of Genetic Variants to Predict How Chemotherapy Will Affect Patients

Advanced Bladder Cancer

**Helena Furberg, MSPH and PhD**

*Associate Attending Epidemiologist*

**Via Joseph, PhD**

*Assistant Attending Geneticist*

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**The Battle:** The platinum-based chemotherapy drugs used to treat advanced bladder cancer fail to help about half of patients, and they cause life-threatening heart problems in more than 20 percent of those receiving the treatment.

### Cycle for Survival funded...

The world's first genome-wide pharmacogenetic study of bladder cancer. Researchers are looking at millions of different genetic variants to find characteristics that determine if someone will respond to chemotherapy. If they predict a poor response or high risk of a life-threatening heart problems, patients would be offered a different treatment, sparing them from the side effects of the platinum-based chemotherapy.

### And now...

Genotyping has been completed for 1,000 patients. Drs. Vijai Joseph and Helena Furberg are analyzing these data to find the link between a person's genetic makeup and their response to drugs—all in an effort to give patients the safest, most effective treatment to beat their cancer.

## Research on Why Brothers and Sons of Men with Testicular Cancer Are More Likely to Get the Same Cancer

Bilateral and Familial Testicular Cancer

**Zsafia Stadler, MD**

*Gastrointestinal Oncology Service*

**Darren Feldman**

*Genitourinary Oncology Service*

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**The Battle:** Men at risk for bilateral and familial testicular cancers typically don't know they're likely to get the disease. Scientists believe that there is a genetic connection that could help predict and prevent cancer in future generations.

### Cycle for Survival funded...

Next-generation DNA sequencing looking at thousands of genes to find genetic mutations linked to cancer risk.

### And now...

The study is ongoing, with Drs. Zsafia Stadler and Darren Feldman continuing their analysis of the data and results. Their ultimate goal is to use these genetic findings to counsel patients and their family members about their risk for these types of cancer.

## Powerful Computers That Can More Accurately Predict Patients' Survival Rate

Pancreatic Cancer

**Richard Kinh Gian Do, MD, PhD**

*Radiologist*

**Amber Simpson, PhD**

*Computational Biologist*

**The Battle:** Pancreatic cancer is an incredibly aggressive disease: the average five-year survival rate is only five percent.

### Cycle for Survival funded...

A sophisticated computer algorithm that can estimate an individual's survival rate with 70 to 80 percent accuracy—picking up on patterns in a CT scan that a human eye cannot detect.

### And now...

With further validation, doctors will one day use the survival probability algorithm to guide treatment options and help patients make informed decisions. Researchers are also working to advance this type of computational biology—investigating if the CT scan patterns are linked to the genetic mutations differentiating types of pancreatic cancer.





**JOIN THE BATTLE**