Ex Vivo Compound Prioritization for Gastroenteropancreatic Neuroendocrine Tumors
Andrea Califano, PhD - Columbia University
$200,000 (Duration: 2 years) This grant was issued in partnership with the Falconwood Foundation
Objective: If successful, this study will be used to tailor therapy for neuroendocrine tumor patients on an individual basis using the drugs that are most effective in targeting their specific vulnerabilities.

Unfolded Protein Response in Neuroendocrine Tumors
Scott André Oakes, MD - University of California, San Francisco
$250,000 (Duration: 2 years) This grant was issued in partnership with the American Association for Cancer Research
Objective: To test whether PanNETs are reliant on elevated levels of Homeostatic UPR signaling to avoid the toxic effects of protein folding stress, and if targeted interventions to either reduce Homeostatic UPR outputs or alternatively trigger the Terminal UPR will have potent antitumor effects on the growth of PanNET cells in murine xenograft models.

Defining the Immune Landscape of Neuroendocrine Tumors – Towards Rational Combination Therapy
Professor Tim Meyer, MD, PhD - UCL Cancer Institute, University College London
$100,000 (Duration: 1 year)
Objective: To characterize the immune microenvironment of neuroendocrine tumors and understand how it responds to therapy and to develop the rationale for combination immunotherapy for patients with neuroendocrine tumors.

Enlightenment of a New Era of Cancer Therapy: Prognostication, Target Selection, and Subsequent Therapy Determined by the Dual Tumor-Immune Phenotype
Holbrook Kohrt, MD, PhD; Pamela Kunz, MD - Stanford University Cancer Center
$100,000 (Duration: 1 year)
Objective: to identify tumor-immune biomarkers that represent prognostic, predictive, and therapeutically actionable targets in patients with neuroendocrine tumors.

Molecular Analysis of the Immune Environment of Neuroendocrine Tumors and Associations with Clinical Outcomes
Matthew H. Kulke, MD - Dana-Farber Cancer Institute
$100,000 (Duration: 1 year)
Objective: To identify new ideas for treating neuroendocrine tumors based on knowledge about how they interact with the immune system.

Personalized Medicine for Neuroendocrine Tumor Patients
Diane Reidy-Lagunes, MD - Memorial Sloan Kettering Cancer Center
2015: $83,000; 2014: $88,000; 2013: $83,000; 2012: $65,000 (Duration: ongoing) Cycle for Survival Team Proceeds
Objective: Develop molecular biomarkers to predict patient response to targeted therapies for pancreatic neuroendocrine tumors.
2014 GRANTS

Developing Novel Treatments for Neuroendocrine Tumors using CAR T-Cell Technology
Carl June, MD; Xianxin Hua, MD, PhD; David Metz, MD - Abramson Cancer Center, University of Pennsylvania
$400,000 (Duration: 2 years)
Objective: To modify CAR T-cells to target and kill neuroendocrine tumor cells, a method that has had dramatic results in patients with other cancers. Success in this project could pave the way for trials of this breakthrough technology for neuroendocrine tumors.

Phase I/II Study of Intratumoral Ipilimumab with Anti-PD-L1 in Patients with Advanced, Progressive, Well-Differentiated Neuroendocrine Tumors
Pamela Kunz, MD; Holbrook Kohrt, MD, PhD - Stanford University Cancer Center
$600,000 (Duration: 2 years)
Objective: To conduct a clinical trial for carcinoid and pancreatic neuroendocrine tumor patients, combining two immunotherapy drugs that are in clinical trials for other cancers. An innovative delivery technique will also be tested to reduce the risk of adverse effects.

Multifunctional Nanomedicine for Targeted Carcinoid Cancer Therapy
Herbert Chen, MD - University of Wisconsin-Madison
$250,000 (Duration: 2 years) This grant was issued in partnership with the American Association for Cancer Research.
Objective: To study a new anti-cancer drug with a new delivery method to target the somatostatin receptors present on neuroendocrine cancer cells. The investigators will conduct preclinical experiments to establish the feasibility of this new treatment strategy for treating patients with neuroendocrine, including carcinoid cancers.

Treating Neuroendocrine Tumors via Synthetic Lethality
Michael German, MD - University of California San Francisco
$250,000 (Duration: 2 years) This grant was issued in partnership with the American Association for Cancer Research.
Objective: To test an FDA approved inhibitor of Mek1/2 (FDA approved for melanoma) in preclinical models of neuroendocrine tumors. The results will provide insight into the unique mechanisms that drive neuroendocrine cancer growth. Positive results of an already approved drug could speed the path from the bench to the bedside to pave the way for clinical trials.

2013 GRANTS

Implication of Heterogeneous Innate Immune Cells in Pancreatic Neuroendocrine Tumor Resistance
Gabriele Bergers, PhD - University of California San Francisco
$250,000 (Duration: 2 years) This grant was issued in partnership with the American Association for Cancer Research.
Objective: To develop a next generation anti-angiogenic treatment strategy for neuroendocrine tumor patients by studying resistance mechanisms.

Identifying Altered Epigenetic States and Drivers in Intestinal Carcinoid and Pancreatic Neuroendocrine Tumors
Bradley Bernstein, MD, PhD - Broad Institute, Massachusetts General Hospital
Daniel Chung, MD - Massachusetts General Hospital
Matthew Kulke, MD; Ramesh Shivdasani, MD, PhD - Dana-Farber Cancer Institute
$620,000 (Duration: 2 years)
Objective: To identify altered epigenetic states and drivers in carcinoid and pancreatic neuroendocrine tumors to identify new treatment strategies for patients. In parallel the team will also work to develop carcinoid and pancreatic neuroendocrine tumor models. These models will be used to assess potential new biomarkers and will also facilitate future research projects.
2013 GRANTS

Epigenomic Analysis of Intestinal Neuroendocrine Cells and the Epigenetic Basis of Neuroendocrine Tumors
Ramesh Shivdasani, MD, PhD - Dana-Farber Cancer Institute
$431,959 (Duration: 4 years) Pan Mass Challenge Proceeds
Objective: To study how epigenetic regulation controls the process by which a stem cell becomes a neuroendocrine cell and to identify how changes in epigenetic regulation can promote development of neuroendocrine tumors.

Theragnostics of Neuroendocrine Tumors with Somatostatin Antagonists
Wolfgang Weber, MD, PhD; Diane Reidy-Lagunes, MD - Memorial Sloan Kettering Cancer Center
$250,000 (Duration: 2 years)
Objective: To conduct a clinical trial for patients with carcinoid cancer for both treatment with the radionuclide, Lutetium-177 and imaging with the radionuclide, Gallium-68. This trial may provide proof of concept data to assess the potential for peptide receptor radionuclide therapy with somatostatin antagonists as a new treatment strategy for neuroendocrine tumor patients. This could pave the way for development of a new treatment and diagnostic imaging strategy for patients with neuroendocrine tumors in the US.

2012 GRANTS

Understanding the TumorSuppressor Activities of ATRXDaxx through Epigenomic Profiling and Animal Models
Peter Lewis, PhD - University of Wisconsin-Madison (Previously C. David Allis, PhD, The Rockefeller University)
$450,000 (Duration: 2 years) Mary Terese Hartzheim Award for Neuroendocrine Tumor Research
Objective: To create models to understand the role of ATRX and Daxx in neuroendocrine tumor development with the ultimate goal of developing new therapies for patients by targeting these processes and to establish the precise changes in chromosome structure resulting from mutations in ATRX and Daxx.

The Mechanistic Underpinnings of Pancreatic Neuroendocrine Tumors
Guillermina Lozano, PhD; MD - Anderson Cancer Center
$300,000 (Duration: 2 years)
Objective: To create the mouse models necessary to identify the cellular changes that occur with loss of Daxx and ATRX to determine the impact of Daxx and ATRX mutations on tumor growth. The mouse models that the team creates will both define the importance of the p53 pathway in the maintenance of pancreatic neuroendocrine tumors and be useful to test potential new therapies.

Overcoming Resistance to mTOR Inhibition in Pancreatic Neuroendocrine Tumors
Eric Nakakura, MD, PhD - University of California San Francisco
$250,000 (Duration: 2 years) This grant was issued in partnership with the American Association for Cancer Research.
Objective: To identify therapeutic strategies to overcome resistance to currently available mTOR inhibiting treatments using a mouse model.

Octreotide Targeted Treatment of Neuroendocrine Tumors of the Pancreas
Renata Pasqualini, PhD - MD Anderson Cancer Center
$250,000 (Duration: 2 years) This grant was issued in partnership with the American Association for Cancer Research.
Objective: To develop components of a virus that will be designed to kill neuroendocrine cancer cells. The virus will be modified to attach to somatostatin receptors on pancreatic neuroendocrine tumor cells, limiting negative effects on healthy tissues.
Mouse Model Project Using Forward Genetics
David Tuveson, MD, PhD - Cold Spring Harbor Laboratory
$300,000 (Duration: 2 years)
Objective: To use forward genetics to establish mouse models of neuroendocrine cancer that will enable biological and pre-clinical studies.

The Impact of MEN1, Daxx, ATRX, and PTEN in Pancreatic Neuroendocrine Cancer Pathogenesis
Kwok-Kin Wong, MD, PhD - Dana-Farber Cancer Institute
$300,000 (Duration: 2 years)
Objective: To create mouse models to determine the impact of the genes: MEN1, Daxx, ATRX, and PTEN in pancreatic neuroendocrine tumor development and determine the epigenetic and expression profiles of the mouse pancreatic islet cells derived from these mouse models.

Suppression of Neuroendocrine Tumors via Epigenetic Regulation
Xianxin Hua, MD, PhD - Abramson Cancer Research Institute, University of Pennsylvania
$250,000 (Duration: 2 years) This grant was issued in partnership with the American Association for Cancer Research.
Objective: To uncover a new treatment strategy for patients with neuroendocrine tumors by studying menin.

Molecular Analysis of Neuroendocrine Tumor Survival
Matthew Kulke, MD - Dana-Farber Cancer Institute
$265,000 (Duration: 2 years) 2011 Pan-Mass Challenge Proceeds
Objective: To evaluate whether genetic variation and protein expression in key molecular pathways affect survival in patients with neuroendocrine tumors, and to identify new treatment targets for patients with neuroendocrine tumors.

Transcriptome and Methylome of Pancreatic Neuroendocrine Tumors with and without ATRX/Daxx Mutations
Nickolas Papadopoulos, PhD - Sidney Kimmel Comprehensive Cancer Center, Johns Hopkins University
$300,000 (Duration: 2 years)
Objective: To determine the epigenetic landscape of pancreatic neuroendocrine tumors by studying tumors with and without DAXX and ATRX mutations.

Oncolytic Viral Therapy for Neuroendocrine Cancers
Charles Rudin, MD, PhD - Johns Hopkins University School of Medicine
$250,000 (Duration: 2 years) This grant was issued in partnership with the American Association for Cancer Research.
Objective: To define components of the Seneca Valley Virus (SVV-001) viral entry pathway, and to explore determinants of productive cytolytic infection with SVV-001.
**2010 GRANTS (continued)**

**Mouse Model Project Using Forward Genetics**
David Tuveson, MD, PhD - Cancer Research UK  
$300,000 (Duration: 2 years)  
Objective: To use forward genetics to establish mouse models of neuroendocrine cancer that will enable biological and pre-clinical studies.

**2009 GRANTS**

**Caring for Carcinoid Foundation Neuroendocrine Tumor Bioconsortium**  
A collaboration among:  
- Dana-Farber Cancer Institute (Matthew Kulke, MD)  
- MD Anderson Cancer Center (James Yao, MD)  
- Massachusetts General Hospital Cancer Center (Daniel Chung, MD)  
- Memorial Sloan Kettering Cancer Center (Diane Reidy-Lagunes, MD)  
- Stanford University Cancer Center (Pamela Kunz, MD)  
$640,000 (Duration: 2 years) Includes Pan-Mass Challenge Proceeds  
Objective: To build a Neuroendocrine Tumor Biospecimen Consortium to enable robust studies to: illuminate the etiology and molecular epidemiology of neuroendocrine tumors; enable early detection and diagnosis of neuroendocrine tumors; identify predictors of patient outcome and toxicity; and identify novel drug targets.

**Cellular Reprogramming of Enteroendocrine Cells and Neuroendocrine Tumors**
Michael Choi, MD - Massachusetts General Hospital  
$150,000 (Duration: 2 years) Mary Terese Hartzheim Award for Neuroendocrine Tumor Research  
Objective: To use cellular reprogramming to understand the biology of enteroendocrine cells and neuroendocrine tumors.

**Carcinoid Cancer Genome Study**
Matthew Meyerson, MD, PhD - Dana-Farber Cancer Institute  
$1,500,000 (Duration: 3 years) Includes Pan Mass Challenge Proceeds  
Objective: To identify mutated genes that cause carcinoid cancer by sequencing ileal carcinoid tumors.

**2006 GRANTS**

**Identify and Validate Molecular Targets for Therapy in a Newly Developed Human Midgut Carcinoid Tumor Cell Line**
Lee Ellis, MD - MD Anderson Cancer Center  
$250,000 (Duration: 2 years)  
Objective: To identify and validate molecular targets for therapy in a newly developed human midgut carcinoid tumor cell line as well as validate other neuroendocrine tumor cell lines.

**Generate Mouse Models of Neuroendocrine Cancer**
Seung Kim, MD, PhD - Stanford University School of Medicine  
$250,000 (Duration: 2 years)  
Objective: To generate mouse models of neuroendocrine cancer.
**2006 GRANTS (continued)**

**Origin and Differentiation of a New Class of Serotonin-Expressing Enteroendocrine Cells**
Andrew Leiter, MD, PhD - University of Massachusetts Medical School
$300,000 (Duration: 4 years)
Objective: To identify and characterize precursor cells that differentiate into serotonin-expressing enteroendocrine cells and to identify a distinct pathway controlling their differentiation.

**Elucidate the Role of the MEN1 Gene in Carcinoid Tumors and Determine a Functional Connection between the Genes MEN1, Rbp2 and p27**
Matthew Meyerson, MD, PhD - Dana-Farber Cancer Institute
$250,000 (Duration: 2 years)
Objective: To elucidate the role of the MEN1 gene in carcinoid tumors and to determine a functional connection between the genes MEN1, Rbp2 and p27.

**2005 GRANTS**

**Determining whether there are Unique Protein Expression Patterns in Gastrointestinal Neuroendocrine Tumors**
Daniel Chung, MD - Massachusetts General Hospital
$300,000 (Duration: 3 years)
Objective: To use proteomics to study carcinoid cancer and pancreatic neuroendocrine cancer.

**Discover New Treatments for Carcinoid Cancer and Pancreatic Neuroendocrine Cancer**
Matthew Kulke, MD - Dana-Farber Cancer Institute
$250,000 (Duration: 5 years)
Objective: To discover new treatments for carcinoid cancer and pancreatic neuroendocrine cancer.

**Understanding the Genetic and Cellular Origins of Carcinoid Cancer**
Ramesh Shivdasani, MD; PhD - Dana-Farber Cancer Institute
$500,000 (Duration: 5 years)
Objective: To understand the genetic and cellular origins of carcinoid cancer.