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## FOR THE MEDIA

Translational T-cell therapies for solid tumors

Prasad Adusumilli, MD; Christopher Klebanoff, MD; Katharine Hsu, MD, PhD; David Scheinberg, MD, PhD

Curative therapy for leukemia through blockade of aberrant transcription and translation

Scott Armstrong, MD, PhD; Omar Abdel-Wahab, MD; Alex Kentsis, MD, PhD; Michael Kharas, PhD; Ross Levine, MD; Yueming Li, PhD

mRNA Neoantigen Vaccines for Pancreatic Cancer

Vinod Balachandran, MD; Benjamin Greenbaum, PhD

Innovative approaches to translating CAR T cell therapies to solid tumors

Renier Brentjens, MD, PhD; Prasad Adusumilli, MD; Christopher Klebanoff, MD; Michel Sadelain, MD, PhD; David Scheinberg, MD,

## Project

PhD: Susan Slovin, MD  
**Investigator(s)**

Strategies to harness cancer cell stress for treatment and diagnosis

Gabriela Chiosis, PhD; Adriana Corben, MD; Mark Dunphy, DO; Katharine Hsu, MD, PhD; Xuejun Jiang, PhD; Shanu Modi, MD; Larry Norton, MD; Nagavarakishore Pillarsetty, PhD; Derek Tan, PhD

A nanoscale therapeutic platform to target the solid tumor microenvironment

Daniel Heller, PhD; Adriana Haimovitz-Friedman, PhD; John Humm, PhD; Charles Rudin, MD, PhD; Charles Sawyers, MD; David Spriggs, MD

Curing relapsed refractory myeloma with combined immune-therapies

Sergio Giralt, MD; Sham Mailankody, MD; Jonathan Peled, MD, PhD; David Chung, MD; Kazunori Murata, PhD

Toward the cure of adult acute lymphocytic leukemia: A personalized, genetic and epigenetic, chemo-immunotherapeutic approach

Joseph Jurcic, MD; Renier Brentjens, MD, PhD; Cyrus Hedvat, MD, PhD; Ross Levine, MD; Peter Maslak, MD; Hans Guido Wendel, MD; Scott Armstrong, MD, PhD; Michael Kharas, PhD; David Scheinberg, MD, PhD

Targeted immunotherapeutic approaches to cure multiple myeloma

Guenther Koehne, MD, PhD; David Chung, MD, PhD; C. Ola Landgren, MD, PhD; Alexander Lesokhin, MD

Multi-modality treatment of pancreatic ductal adenocarcinoma (PDAC)

Jason Lewis, PhD; Peter Allen, MD; Jan Grimm, PhD; Christine Iacobuzio-Donahue, MD, PhD; Kayvan Keshari, PhD; Steven Leach, MD; Thomas Reiner, PhD

Developing curative therapies for BRAF and NRAS mutant tumors

Neal Rosen, MD, PhD; David Solit, MD

Changing the natural history of metastatic

Neal Rosen, MD; Paul Chapman, MD; David

melanoma with multimodality therapy  
**Project**

Solit, MD; Scott Lowe, PhD; Jedd Wolchok, MD,  
**Investigator(s)**  
PhD

Development of therapeutic strategies that  
change the natural history of tumors with mutant  
RAS

Neal Rosen, MD, PhD; Scott Lowe, PhD; David  
Solit, MD; Barry Taylor, PhD; Jedd Wolchok,  
MD, PhD

Towards a cure for prostate cancer

Charles Sawyers, MD; Jason Lewis, PhD; Dana  
Rathkopf, MD; Michael Zelefsky, MD

Innovations in the structures, functions and  
targets of monoclonal antibody-based drugs for  
cancer

David Scheinberg, MD, PhD; Renier Brentjens,  
MD, PhD; Steven Larson, MD; Hans-Guido  
Wendel, MD

Curing ovarian cancer through early detection  
and personalized treatment

David Spriggs, MD; Carol Aghajanian, MD;  
Douglas Levine, MD; Paul Sabbatini, MD

## Multidisciplinary Team Research

### Project

### Investigator(s)

Quillaja saponin adjuvants in carbohydrate  
conjugate cancer vaccines

David Gin, PhD; Adam Boruchov, MD; Paul  
Chapman, MD; Govind Ragupathi, PhD and Jim  
Young, MD

Cellular target discovery and therapeutic agents  
— Proteases as targets for cancer therapy

Yueming Li, PhD; David Scheinberg, MD, PhD;  
Xuejun Jiang, PhD and Johanna Joyce, PhD

Non-invasive imaging and monitoring of tumor  
hypoxia

Clifton Ling, PhD; Jason Koutcher, MD, PhD;  
John Humm, PhD; Pat Zanzonico, PhD; Joseph  
O'Donoghue, PhD; and Ronald Finn, PhD

Development of active and adoptive  
immunotherapy of ovarian cancer targeting  
MUC16 and WT1

Philip Livingston, MD and Richard O'Reilly, MD

**Project**  
Development of rational strategies for anti-cancer therapy based on inhibition of signaling through the Ras and PI3kinase signaling pathways — Combined inhibition of Raf/MEK/MAPK and PI3K/AKT signaling for the treatment of cancer

Targeting tumors with genetically enhanced T lymphocytes

Functional and advanced anatomic techniques for response assessment in solid tumors

An integrated multidisciplinary approach to developing new treatments for advanced prostate cancer

Identification of cell cycle related kinase inhibitors for cancer therapy

Immunotherapy of cancer: Development of cancer vaccines, adoptive cellular therapies, immune modulation and combination immunotherapies

**Investigator(s)**  
Charles Sawyers, MD; David Solit, MD; and David Spriggs, MD

Michel Sadelain, MD, PhD; Prasad Adusumilli, MD; Renier Brentjens, MD, PhD; Vladimir Ponomarev, MD, PhD; Isabelle Riviere, PhD; Susan Slovin, MD, PhD

Lawrence Schwartz, MD and Robert Motzer, MD

Derek Tan, PhD; Gabriela Chiosis, PhD; Yueming Li, PhD; Neal Rosen, MD, PhD and Howard Scher, MD

Archie Tse, MD, PhD and David Spriggs, MD

Marcel van den Brink, MD, PhD; Ronald Blasberg, MD; Bo Dupont, MD, DSc; Alan Houghton, MD; Robert Jenq, MD; Steve Larson, MD; Jedd Wolchok, MD, PhD

## Clinical Investigations

### Project

A phase I trial of malignant pleural mesothelioma treated with autologous T cells genetically targeted to the cancer cell

### Investigator(s)

Prasad Adusumilli, MD

**Project**  
A Phase 1a/1b Trial to Assess Safety and  
Efficacy of Intrathecal Deferoxamine in  
Patients with Leptomeningeal Metastases from  
Non Small Cell Lung Cancer

Adrienne Boire, MD, PhD  
**Investigator(s)**

Experimental Therapeutics Clinical Treatment  
Unit

Richard Carvajal, MD

Vaccination of pancreatic cancer patients  
against mutated K-ras

Paul Chapman, MD

Biologic correlates in a phase I/II study of  
humanized 3F8 bispecific antibody (hu3F8-  
BsAb) in patients with relapsed/refractory  
neuroblastoma, osteosarcoma, and other  
GD2(+) solid tumors

Nai-Kong Cheung, MD, PhD

Clinical investigation of Zelavespib in  
relapsed/refractory AML with antecedent MPN  
and/or post-allo

Gabriela Chiosis, PhD

Clinical translation of PU-H71, a small molecule  
Hsp90 inhibitor

Gabriela Chiosis, PhD

Langerhans-type dendritic cell vaccination as  
consolidation for multiple myeloma after  
autologous stem cell transplantation

David Chung, MD, PhD

Circulating tumor cells in patients with metastatic  
prostate cancer: Development of predictive  
biomarkers for sensitivity to androgen receptor  
targeted treatment sensitivity

Daniel Danila, MD and Howard Scher, MD

The Development of a Novel AML-directed CAR  
T Cell Capable of Bystander Tumor Killing

Anthony Daniyan, MD

Preclinical and clinical evaluations of first-generation recombinant modified vaccinia virus Ankara (MQ710) for the treatment of anaplastic thyroid cancer and other solid tumors

Liang Deng, MD, PhD  
**Investigator(s)**

Targeting Lewis Y in small cell lung cancer using the humanized monoclonal antibody, hu3S193

Chaitanya Divgi, MD

Cardenolides as novel agents for the treatment of retinoblastoma

Hakim Djaballah, PhD and David Abramson, MD

Radioimmunotherapy, reduced-dose radiation therapy, and chemotherapy for medulloblastoma

Ira Dunkel, MD and Kim Kramer, MD

Iso-fludelone, a novel epothilone: First in human phase 1 trial

Mrinal Gounder, MD and Naiyer Rizvi, MD

Assessing the continual reassessment method as a phase I design by comparing it to the standard dose escalation scheme

Alexia Iasonos, PhD

Pilot trial <sup>89</sup>Zr-trastuzumab-PET as pharmacodynamic marker in patients with HER2-positive esophagogastric cancer

Yelena Janjigian, MD

Hyperpolarized pyruvate MR imaging of prostate cancer

Kayvan Keshari, PhD

Comparative analyses of WT1 expression and WT1 antigen-specific T-cell frequencies in patients with plasma cell leukemia following treatment with donor-derived Wilms tumor antigen-specific T lymphocyte infusions

Guenther Koehne, MD, PhD

Development of KSR phosphorothioate antisense oligonucleotides for phase I clinical

Richard Kolesnick, MD

trial

**Project**

**Investigator(s)**

A phase I study of convection-enhanced delivery of 124I-8H9 for patients with non-progressive diffuse pontine gliomas previously treated with external beam radiation therapy

Kim Kramer, MD

Phase I study of intrathecal 131-I-8H9 for central nervous system and leptomeningeal malignancies

Kim Kramer, MD

Pilot trial of a tetravalent KLH conjugate vaccine against small cell lung cancer

Lee Krug, MD

Targeting small cell lung cancer with an Ac-labeled antibody to the ganglioside GD2

Lee Krug, MD

Phase I trial of a tetravalent vaccine with escalating doses of the immunological adjuvant QS-21, in combination with oral beta-glucan for high-risk neuroblastoma

Brian Kushner, MD

A Phase I Dose Escalation Study of ESK1-Bispecific T-cell Engager (ESK-TCE) Antibody Targeting WT1 for the Treatment of Patients with Recurrent Ovarian Cancers

Chrisann Kyi, MD

Clinical and molecular-metabolic phase II trial of perifosine for recurrent/progressive malignant gliomas

Andrew Lassman, MD

Combination of targeted radiotherapy and anti-angiogenesis for resistant neuroblastoma

Shakeel Modak, MD

Lutetium-177 radiolabeled anti-prostate specific membrane antigen antibodies and its

Michael Morris, MD

relationship to tumor-absorbed radiation dose

**Project**

Quantitative PET imaging with yttrium-86 radiolabeled humanized anti-prostate specific membrane antibody J591 for dosimetric evaluation of yttrium-90-huJ591 therapy

Targeted radioimmunotherapy using Ley as a target in castrate metastatic prostate cancer

Prostate specific membrane antigen vaccine in prostate cancer

Preclinical and clinical development of 10-propargyl-10-deazaaminopterin (PDX) in relapsed or refractory aggressive non-Hodgkin's lymphomas

A phase I clinical trial of a new targeted therapy for the treatment of metastatic pancreatic cancer

Cytokine Secreting CAR T-Cell Therapy for Treatment of Leukemia

GM-CSF DNA vaccine in melanoma

Phase 1 study of humanized 3F8 monoclonal antibody (Hu3F8) when combined with interleukin-2 in patients with high-risk neuroblastoma and GD2-positive solid tumors

Augmented PSMA CAR therapy for castrate-resistant, metastatic prostate cancer

Preparation of a peptide vaccine for chronic myelogenous leukemia

**Investigator(s)**

Michael Morris, MD

Michael Morris, MD

Luke Nordquist, MD

Owen O'Connor, MD, PhD

Eileen O'Reilly, MD

Jae Park, MD

Miguel Perales, MD

Stephen Roberts, MD

Michel Sadelain, MD, PhD and Susan Slovin, MD, PhD

David Scheinberg, MD, PhD

**Project**

Biomarker development in patients with metastatic prostate cancer: Assays for androgen receptor signaling pathway in circulating tumor cells

**Investigator(s)**

Howard Scher, MD

Therapeutic response assessment in clinical trials: Development of tools for precise quantitative image analysis

Lawrence Schwartz, MD

Synthesis of 150 grams of PDX

Francis Sirotnak, PhD

Phase I trial of mouse TRP-2 DNA vaccine for patients with melanoma

Jedd Wolchok, MD, PhD

Alemtuzumab treatment of steroid-refractory acute graft-versus-host disease

James Young, MD

## Drug Discovery and Development

**Project**

Synthetic introns to enable mutation-dependent targeting of cancer cells

**Investigator(s)**

Omar Abdel-Wahab, MD

Imaging the efficacy of TRAIL-enhanced cancer immunotherapy

Prasad Adusumilli, MD and Vladimir Ponomarev, MD, PhD

Small molecule degrader of HMGCS1 to harness the mevalonate pathway in cancer

Heeseon An, PhD

Self-immolative tumor-targeted nanogenerators

Christophe Antczak, PhD

Imiquimod analogs as new cancer immunotherapy agents

Daniel Bachovchin, PhD

**Project**

New DPP8/9-binding ligands for the treatment of acute myeloid leukemia

**Investigator(s)**

Daniel Bachovchin, PhD

Induction of pyroptosis for cancer monocytic/macrophage lineage

Daniel Bachovchin, PhD

Peptidase Inhibitors as New Cancer Immunotherapy Agents

Daniel Bachovchin, PhD

ID protein degradation as a therapeutic strategy for advanced cancers

Robert Benezra, PhD; James Harding, MD

Tumor endothelial cell-specific inhibitor for Id-proteins

Robert Benezra, PhD

Over expression of asmasse in endothelial stem cells by lentiviral vector delivery

Nira Bloom, PhD

Expression and modulation of Fcγ receptors on human dendritic cells for targeted immunotherapy

Adam Boruchov, MD

Mesoporous silica nanoparticles as drug delivery vehicles in a PDGFB-driven murine high grade glioma model

Michelle Bradbury, MD, PhD

Construction and characterization of retroviral vectors encoding genes capable of generating both primary and efficient co-stimulatory signals to T cells for use in the adoptive immunotherapy of B cell malignancies

Renier Brentjens, MD, PhD

Development and characterization of novel inhibitors of JAK kinases

Jacqueline Bromberg, MD, PhD

Induction of natural EGFR inhibitors  
**Project**

Luca Cartegni, PhD  
**Investigator(s)**

Therapeutic modulation of alternative splicing

Luca Cartegni, PhD

Overcoming CDK4/6 inhibitor resistance

Sarat Chandarlapaty, MD, PhD

Therapeutic targeting of Gαq pathway in uveal melanoma

Yu Chen, PhD

Development of small molecule inhibitors of MCL-1 for cancer therapy

Emily Cheng, PhD

Using Self-Assembling DisAssembling (SADA) Platform in Pretargeted Radioimmunotherapy and DNA Damage Repair Inhibitors to Enhance Immunogenic Cell Death

Nai-Kong Cheung, MD, PhD

Dual specific human monoclonal antibody targeting IGF-I and IGF-II for treating cancer in children

Nai-Kong Cheung, MD, PhD

Multistep targeting of GD2 using DOTA hook

Nai-Kong Cheung, MD, PhD

Optimizing radioimmunotherapy delivered through cerebrospinal fluid: Testing affinity-matured scFv constructs in xenograft models

Nai-Kong Cheung, MD, PhD

Humanized antibody targeting CSPG4 on melanoma

Nai-Kong Cheung, MD, PhD

SADA DOTA-PRID and SADA DOTA-PRIT for incurable cancers

Nai-Kong Cheung, MD, PhD; Darren Veach, PhD

T cell-engaging bispecific antibody (TCE bsAb) therapeutics in DLK1-positive cancer

Ping Chi, MD, PhD

**Project**

Therapeutic and diagnostic targeting of DLK1 in cancer

**Investigator(s)**

Ping Chi, MD, PhD

Development of the epichaperome inhibitor LSI-137

Gabriela Chiosis, PhD

Novel compounds inhibiting Hsp90

Gabriela Chiosis, PhD and Neal Rosen, MD, PhD

Development of novel Hsp70 ATPase activity modulators

Gabriela Chiosis, PhD

PU24FCI, a novel selective Hsp90 inhibitor

Gabriela Chiosis, PhD

Bcr-Abl substrates and inhibitors

Bayard Clarkson, MD

Leptin receptor as a target to eradicate CML tumor initiating cells

Bayard Clarkson, MD

Design of molecular glue degraders to selectively target ERK2 over ERK1

Arvin Dar, PhD

Development of D-peptide inhibitors of oncogenic KRAS mutants

Samuel Danishefsky, PhD

Testing the immunogenicity of a new unimolecular hexavalent vaccine in combination with the novel synthetic saponin adjuvant SQS-1-0-5-5

Samuel Danishefsky, PhD

Cell migration inhibition: synthesis and evaluation of migrastatin and analogues for the identification of a novel chemotherapeutic

Samuel Danishefsky, PhD

Preparation of multiple drug reversal agent 5-N-

Samuel Danishefsky, PhD

Preparation of multiple drug reversal agents for  
Project: acyl ardeemin and some active analogues

Synthesis and evaluation of a novel  
unimolecular multiantigenic vaccine against  
breast cancer

Telomerase inhibition: Synthesis and evaluation  
of UCS1025A and analogues for the  
identification of a novel chemotherapeutic

Development of an AML-directed dual-targeted  
CAR T cell

Targeting DJ-1 for cancer therapy

Synthesis of a multi-scaffold library for high-  
throughput screening for novel inhibitors of  
androgen receptor signaling

Synthetic study on proteasome inhibitor  
salinosporamide A

Novel enhancement strategies for cancer DNA  
vaccines: Epitope optimization and fusion  
vectors

Development of reagents to antagonize  
ceramide-mediated raft clustering in vivo

High throughput screening of specific inhibitors  
for kinase suppressor of Ras1 (KSR1)

ASMase gene therapy for radiosensitization

Preclinical development of anti-ceramide

Samuel Danishefsky, PhD

### Investigator(s)

Samuel Danishefsky, PhD

Samuel Danishefsky, PhD and Malcolm Moore,  
D.Phil.

Anthony Daniyan, MD

Yael David, PhD

Christine DiBlasi, PhD

Atsushi Endo, PhD

Manuel Engelhorn, PhD

Zvi Fuks, MD

Zvi Fuks, MD

Zvi Fuks, MD and Richard Kolesnick, MD

Zvi Fuks, MD

monoclonal antibodies

**Project**

Preclinical evaluation of KSR phosphorothioate antisense oligonucleotides in treating gf Ras-dependent human malignancies

The development of highly concise routes to epothilones in the context of the chemical synthesis of the promising antitumor agent 4-desmethyl-12,13-desoxyEpothilone B

L1CAM targeting 1xx CAR T cells for advanced solid tumors

Targeting hematopoietic stem cells with lipid nanoparticles to potentiate cancer immunotherapy

Design of small molecules that trigger secretion of neoantigen: Chaperone complexes

Mechanism of TEM8 in increasing CD8 T cell responses

ROS-activatable prodrug of Doxazoline

Clinically approved nanoparticles as environmental-responsive self reporting drug delivery system

Multimodality theranostics of pancreas cancer

Gene expression profiling to identify therapeutic targets in chronic lymphocytic leukemia

In Situ CAR T cell engineering for treatment of

**Investigator(s)**

Zvi Fuks, MD and Richard Kolesnick, MD

Ana Gabarda Ortega, PhD

Karuna Ganesh, MD, PhD

Alexander Gitlin, MD, PhD

Jonathan Goldberg, PhD

Polly Gregor, PhD

Jan Grimm, MD, PhD

Jan Grimm, MD, PhD

Jan Grimm, MD, PhD

Mark Heaney, MD, PhD

Daniel Heller, PhD

... and CRISPR-Cas9 engineering for treatment of  
**Project** high grade serous ovarian cancer

Daniel Heller, PhD  
**Investigator(s)**

Targeted nanoformulation and delivery strategy  
to improve the utility of PROTACS

Daniel Heller, PhD

Nanoscale bio-imaging

Daniel Heller, PhD

ZIC1: Therapeutic target and predictor of  
outcome in adult male germ cell tumors

Jane Houldsworth, PhD

Biodistribution and radiobiological effectiveness  
of <sup>225</sup>Ac and <sup>213</sup>Bi for alpha-particle  
radioimmunotherapy

John Humm, PhD

Targeting isocitrate dehydrogenase mutations by  
enzyme hyperactivation

Andrew Intlekofer, MD, PhD

Novel combination therapies for ER+/HER2-  
breast cancer

Xuejun Jiang, PhD

Identification of chemical inhibitors of the ULK1  
kinase complex

Xuejun Jiang, PhD and Derek Tan, PhD

Development of novel therapies for endometrial  
carcinomas

Xuejun Jiang, PhD; Neal Rosen, MD, PhD

Identification and characterization of small  
molecule inhibitors of Apaf-1/Cytochrome c-  
mediated apoptosis

Xuejun Jiang, PhD and Derek Tan, PhD

Targeting the PTEN ubiquitin ligase NEDD4 with  
a novel electrophilic fragment library

Xuejun Jiang, PhD and Derek Tan, PhD

Protein corona engineered lipid nanoparticle  
(PRO-LNP) for targeted macromolecular drug

Alex Kentsis, MD, PhD

delivery  
**Project**

**Investigator(s)**

A modular platform for cell specific  
macromolecular drug delivery

Alex Kentsis, MD, PhD

Chemical library screen for musashi inhibitors

Michael Kharas, PhD

Inhibition of MSI translation in MSI driven  
leukemias

Michael Kharas, PhD

Tunable peptidomimetic nanoconjugates for  
cancer gene control

Alex Kentsis, MD, PhD

Metabolic engineering to enhance CAR T cell  
therapy

Kayvan Keshari, PhD

IL10 blockade as a novel immunotherapy across  
solid cancers

Danny Khalil, MD, PhD

Molecular glues targeting stem cell/immuno  
oncologic programs in cancer

Michael Kharas, PhD

Developing MUSASHI RNA binding protein  
inhibitors to target cancer

Michael Kharas, PhD

Raman nanostar-triggered automated laser  
ablation device

Moritz Kircher, MD, PhD and Ricardo Toledo-  
Crow, PhD

ENT1 requires ceramide-rich platforms to  
transport nucleoside chemotherapeutics

Richard Kolesnick, MD

C16:0 ceramide nano-liposomes reverse multi-  
drug resistance

Richard Kolesnick, MD

Sphingolipid-based anti-angiogenic  
chemosensitization

Richard Kolesnick, MD

chemosensitization

**Project**

**Investigator(s)**

Acid sphingomyelinase is obligate for gemcitabine action

Richard Kolesnick, MD

Small animal imaging and spectroscopy

Jason Koutcher, MD PhD

Leveraging living drugs for tumor-targeted radiotherapy

Simone Krebs, MD

Pre-clinical development of a novel class of proteasome inhibitors

Marc Ladanyi, MD

Improved detection of abl, c-kit, src and PDGFR $\alpha$  expressing tumors using a combination of positron labeled EGFR tyrosine kinase inhibitors and “stealth” nanocarriers

Steve Larson, MD

Ac-225 DOTA Proteus for Pre-targeted Radioimmunotherapy (PRIT) of solid tumors

Steven Larson, MD

Alpha and Beta Pre-targeted Radioimmunotherapy for Peritoneal Carcinomatosis

Steven Larson, MD

Targeting FGFR3 oncogenic mutation for theranostics

Jason Lewis, PhD

Statins to Enhance Pretargeted Radioimmunotherapy of HER2-expressing Esophagogastric Tumors

Jason Lewis, PhD

Amplifying the efficacy of pretargeted radioimmunotherapy (PRIT) in pancreatic cancer

Jason Lewis, PhD

Radiosensitization of malignant gliomas by gene

Gloria Li, PhD and Philip Gutin, MD

therapy  
**Project**

**Investigator(s)**

Targeted delivery of attenuated interleukin-15 for ILC1-mediated cancer immunotherapy

Ming Li, PhD

Targeting TGF- $\beta$  signaling in CD4+ T cells for cancer immunotherapy

Ming Li, PhD

Development of lysosomal inhibitors for pancreatic cancer treatment

Yueming Li, PhD

Development of the autophagy-lysosome pathway blockers for pancreatic cancer treatment

Yueming Li, PhD

Development of sulfonamide  $\gamma$ -secretase inhibitors for cancer therapy

Yueming Li, PhD

Preclinical studies of sulfonamide  $\gamma$ -secretase inhibitors for cancer therapy

Yueming Li, PhD

$\gamma$ -Secretase: Target validation and inhibitor development

Yueming Li, PhD

Target notch signal pathway in B-cell neoplasm

Yueming Li, PhD

Identification and characterization of small molecule enhancers of GTPase activity

Piro Lito, MD

Advancing uPAR CAR T cells towards clinical trials in cancer

Scott Lowe, PhD

Harnessing senolytic CAR T cell based therapies for the treatment of solid tumors

Scott Lowe, PhD

Targeting p53-regulated metabolism for anti-leukemia therapy <b>Project</b>	Scott Lowe, PhD <b>Investigator(s)</b>
Advancing CARM1 Inhibitors as Preclinical Candidates Against Metastatic Breast Cancer	Minkui Luo, PhD
Developing mechanism-based protein arginine methyltransferase inhibitors as anti-cancer reagents	Minkui Luo, PhD
Develop protein methyltransferase inhibitors as anti-cancer drugs	Minkui Luo, PhD
High-throughput screening for SETMAR inhibitors: Develop protein methyltransferase inhibitors as anticancer drugs	Minkui Luo, PhD
High throughput screening for SETMAR inhibitors	Minkui Luo, PhD
TREX1 exonuclease inhibitor to improve antitumor immunity	John Maciejowski, PhD
Synthesis of polyketide-like small molecule libraries	Daniel Macks, PhD
Effect of histone deacetylase inhibitors on normal and transformed prostate cells	Paul Marks, PhD
Monitoring molecular biomarkers in human cancer	Peter Maslak, MD
Targeting CDH5 and simultaneously treating PSMA-positive and PSMA-negative prostate cancer with alpha particle therapy	Michael McDevitt, PhD

Project	Investigator(s)
Camelid antibodies armed with alpha particles and directed at enzalutamide-treated prostate cancer	Michael McDevitt, PhD
A self-assembling synthetic clot to specifically infarct tumor tissue in vivo	Michael McDevitt, PhD
RNAi prophylaxis of chemotherapy-induced nephrotoxicity	Michael McDevitt, PhD
Optimizing EGFR Blockade in Human Glioblastoma	Ingo Mellinghoff, MD
Evaluation of epothilone analogs, synthetic microtubule stabilizing agents with potent anti-myeloma and anti-ovarian cancer action	Malcolm Moore, D.Phil.
Murine tumor xenograft models for preclinical drug development	Malcolm Moore, D.Phil.
Targeting activated ADAM17 for the treatment of epithelial ovarian cancer	Dimitar Nikolov, PhD
Function blocking antibodies against ADAM metalloproteases for inhibition of Eph, EGFR and Notch-dependent tumorigenesis	Dimitar Nikolov, PhD
Small-molecule inhibitors of Eph receptor signaling	Dimitar Nikolov, PhD
Single-chain anti-EphA2 antibodies for treatment of lymphoma and leukemia	Dimitar Nikolov, PhD
Real time polymerase chain reaction (PCR) for clinical and correlative studies	Stephen Nimer, MD

**Project**  
The role of arginine methyltransferases in the pathogenesis of myeloid malignancies

**Investigator(s)**  
Stephan Nimer, MD

DNA vaccines against breast cancer: Synergy through targeting both tumor cells and tumor stroma

Francesca Orlandi, PhD

Chemical synthesis in carbohydrate-based cancer vaccines

Ouathek Ouerfelli, PhD

Overcoming acquired resistance to targeted therapy in lung cancer

William Pao, MD, PhD

Development of Gboxin, a benzimidazolium compound, as an antitumor reagent

Luis Parada, PhD

Protein chip biology system for high-throughput screening

Gavril Pasternak MD, PhD

Sigma receptors: a novel anti-proliferative target for tumor therapy

Gavril Pasternak, MD, PhD

Structure activity of the sigma<sub>1</sub> antagonist binding pocket

Gavril Pasternak, MD, PhD

Development of potent small molecule inhibitors of the cGAS-STING dsDNA-sensing immune pathway

Dinshaw Patel, PhD

Development of small molecule inhibitors of METTL3-METTL14 RNA methyltransferase complex as drugs against acute myeloid leukemia

Dinshaw Patel, PhD

T-cell precursors and allogeneic hematopoietic

Miguel Perales, MD

stem cell transplant  
**Project**

**Investigator(s)**

SKI-N69: A candidate radiosensitizer

John Petrini, PhD

Modulation of DNA damage signaling pathways

John Petrini, PhD

Targeted synthetic immunity for activating site specific anti-tumor innate immune response

Nagavarakishore Pillarsetty, PhD

Site specific activation of  $\gamma\delta$  T cells for cancer immunotherapy applications

Nagavarakishore Pillarsetty, PhD

Targeted nuclear delivery of EGF-Auger emitting radioconjugates for cancer therapy

Nagavarakishore Pillarsetty, PhD

Genetically engineered CAR T cells for antigen-heterogeneous prostate cancer

Vladimir Ponomarev, MD, PhD

Tumor antigen-directed molecular imaging to strategize T-cell immunotherapy

Vladimir Ponomarev, MD, PhD

Evaluation of endothelial cell apoptosis in human cancer specimens in response to ionizing radiation

Elizabeth Poyner, MD, PhD

Novel structure derivatives of trehalose dimycolate as cancer vaccine adjuvants

Vivek Rao, PhD

Imaging of the Peripheral Nervous System

Thomas Reiner, PhD

Preclinical Testing of Hedgehog Inhibitors in Pancreatic Cancer

Marilyn Resh, PhD

Identification of hedgehog palmitoylation inhibitors

Marilyn Resh, PhD

**Project**

Efficacy testing of TDI-3410, a Hedgehog Acyltransferase inhibitor, in mouse models of lung, breast and pancreatic cancer

Targeting eIF4A in metastatic estrogen receptor positive breast cancer

Development and in vivo evaluation of clinical retroviral vectors designed to express high levels of mutant HSV thymidine kinase to increase the safety of T lymphocyte infusions

Development of improved TORC1 inhibitor-based therapies for the treatment of breast cancer and other carcinomas

Development of improved TORC1 inhibitor-based therapies for the treatment of breast cancer and other carcinomas

Development of combined inhibition of ERK and PI3K/AKT signaling as a strategy for the treatment of mutant RAS-dependent tumors

DLL3 radioimmunoconjugates for high-grade neuroendocrine tumor imaging and therapy

Design of combinatorial therapeutics

Preclinical development of the anti-androgen A52 for prostate cancer therapy

Therapeutic self-assembling nanodevices

Radiotherapy with targeted multivalent  $\alpha$ -particle

**Investigator(s)**

Marilyn Hesh, PhD

Ezra Rosen, MD, PhD

Isabelle Riviere, PhD

Neal Rosen, MD, PhD; Daniel Heller

Neal Rosen, MD, PhD; Daniel Heller

Neal Rosen, MD, PhD

Charles Rudin, MD, PhD

Chris Sander, PhD

Charles Sawyers, MD

David Scheinberg, MD, PhD

David Scheinberg, MD, PhD

generators  
**Project**

**Investigator(s)**

Development of novel inhibitors of SCCRO

Bhuvanesh Singh, MD

A novel class of peptidomimetic agents targeting tumor growth, angio-genesis, and metastases

Francis Sirotnak, PhD and David Scheinberg, MD, PhD

Modulation of chemotherapy sensitivity by alterations in PTEN/AKT signaling

David Solit, MD

Characterization of an RNA binding protein targeted by thalidomide analogues

David Spriggs, MD

MUC16 synthetic lethal screening in ovarian cancer

David Spriggs, MD

Development of novel menin inhibitors for glioma therapy

Viviane Tabar, MD

pdfSEAKER: Development of CAR T cells that express human peptide deformylase (hsPDF) to activate synergistic small-molecule prodrugs locally at tumors

Derek Tan, PhD

onSEAKER: Engineering CAR T cells to biosynthesize synergistic small molecule drugs in situ

Derek Tan, PhD

Targeted delivery of epigenetic small-molecule drugs by SEAKER cells

Derek Tan, PhD

A novel CAR T-cell platform for targeted, local biosynthesis of small-molecule therapeutics

Derek Tan, PhD

Studying Chk1-mediated signaling pathways using chemical genetics and functional imaging

Archie N. Tse, MD, PhD

using chemical genetics and functional imaging

**Project**

Identification of novel small molecules with anti-proliferative action towards human lung cancer cell lines harboring oncogenic EGFR or KRAS

ESSENCE compounds as tools for specific modulation of alternative splicing patterns

Development of NDR1/STK38 inhibitors in collaboration with the Tri-I TDI

The oncogenic action of NRF2 depends on de-glycation by Fructosamine-3-kinase

Defining the mechanism underlying GITR agonist activity

Isolation TCRm modalities with broad reactivity to KRAS-HLA complex

TMC-95A analogues as potential antitumor agents

Controlled rate freezer for preclinical laboratory studies

Janus kinase 2 (JAK2) as a therapeutic target in graft-versus-host disease

Reversal of indoleamine 2,3-dioxygenase (IDO)-mediated suppression of tumor immunity stimulated by human dendritic cells

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