MSSM Head and Neck Cancer Research Program
Clinical Trials

May 2012
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ACTIVE TRIALS

CURATIVE THERAPY

Phase I Study of Cabazitaxel-PF Induction Chemotherapy in Patients with Locally Advanced Squamous Cell Carcinoma of the Head and Neck
GCO # 11-0646

Study Type: Phase I
Sponsor: Sanofi
Principal Investigator: Dr. Krzysztof Misiukiewicz
Research Nurse: Patricia Strebel, RN
Coordinator: Nadia Camille

The objectives of this study are to assess the safety, the maximum tolerated dose (MTD) and the dose limiting toxicity of cabazitaxel when combined with cisplatin and FU induction chemotherapy, as well as to assess the best overall response rate after 3 cycles and the PFS and OS after 3 years. Eligible patients are 18 years or older with stage IV, previously untreated, locally advanced SCCHN (may have had previous surgery, but not chemotherapy or radiotherapy). For more information, contact Nadia at nadia.camille@mountsinai.org or (212) 241-5253.

PALLIATIVE THERAPY

A Randomized, Double-Blind Phase II Safety Study of Cetuximab, Using IMClone Versus Boehringer Ingleheim Manufacturing Processes, in Combination With Cisplatin or Carboplatin and 5-Fluorouracil in the First-Line Treatment of Patients With Locoregionally Recurrent and/or Metastatic Squamous Cell Carcinoma of the Head and Neck
GCO # 11-0970

Study Type: Phase II
Sponsor: Eli Lilly & Company
Principal Investigator: Dr. Krzysztof Misiukiewicz
Research Nurse: Patricia Strebel, RN
Coordinator: Madeleine Schrier

The objectives of this study are to prospectively compare the safety profiles of commercial cetuximab manufactured by ImClone (Arm A) and Boehringer Ingleheim (Arm B), in terms of individual all-grade toxicities occurring any time during the treatment period. This study also aims to assess the overall safety and investigate the pharmacokinetics of cetuximab, as well as
estimate overall survival, progression-free survival, and overall tumor response rate for this treatment. Eligible patients are 18 years or older with locoregionally recurrent and/or metastatic squamous cell carcinoma of the head and neck who have not received previous chemotherapy in this setting. For more information, contact Madeleine at madeleine.schrier@mssm.edu or (212) 824-7310.

A Randomized, Double-blind, Multicenter Two-Stage Adaptive Phase 3 Study of Intravenous Administration of REOLYSIN® (Reovirus Type 3 Dearing) in Combination with Paclitaxel and Carboplatin versus the Chemotherapy Alone in Patients with Metastatic or Recurrent Squamous Cell Carcinoma of the Head and Neck who have Progressed on or after Prior Platinum-Based Chemotherapy

Study Type: Phase III
Sponsor: Oncolytics
Principal Investigator: Dr. Krzysztof Misiukiewicz
Research Nurse: Patricia Strebel
Coordinator: Madeleine Schrier

The objectives of this study are to compare the overall survival for the treatment regimens in the study population, compare Objective Response (Complete Response (CR) + Partial Response (PR)) rate and duration of response for the treatment, and compare the safety and tolerability of the treatment in the study population. Patients who are eligible for second line treatment of recurrent or metastatic squamous cell carcinoma of the head and neck (R/M SCCHN) will receive a standard chemotherapy regimen of paclitaxel and carboplatin plus intravenously receive the study drug or placebo. The patient’s health status and tumor assessments will be checked at various intervals throughout the study. For more information contact Madeleine at Madeleine.schrier@mssm.edu or (212) 824-7310.

A Phase 2, Multi-center, Randomized, Double-blind, Placebo-controlled Clinical Trial to Evaluate the Safety and Efficacy of ALD518 in the Reduction of Oral Mucositis in Subjects With Head and Neck Cancer Receiving Concomitant Chemotherapy and Radiotherapy

Study Type: Phase II
Sponsor: Alder
Principal Investigator: Dr. Marcelo Bonomi
Research Nurse: Patricia Strebel
Coordinator: Rachel Abbott

The primary objective of the clinical trial is to evaluate the safety and efficacy of the study drug ALD518 in modifying the course of oral mucositis (OM) in subjects with head and neck cancer (HNC) receiving concurrent chemotherapy and radiotherapy (CRT). For more information, contact Rachel at rachel.abbott@mssm.edu or (212) 824-7337.
An Open-label Phase 2 Study of ACE-041 in Patients with Recurrent or Metastatic Squamous Cell Carcinoma of the Head and Neck

**Study Type:** Phase II  
**Sponsor:** Acceleron  
**Principal Investigator:** Dr. Marshall Posner  
**Research Nurse:** Patricia Strebel  
**Coordinator:** Madeleine Schrier

The primary objective of this study is to estimate the objective response rate (ORR) of ACE-041 in patients with recurrent or metastatic squamous cell carcinoma of the head and neck (SCCHN). The secondary objectives include evaluating the safety and tolerability of ACE-041, the pharmacokinetic (PK) profile of ACE-041, the progression free survival (PFS) and overall survival (OS), time to tumor progression (TTP), duration of response, and rate of disease control (ORR + stable disease [SD]). This study also seeks to explore association of the expression of ALK1 and/or other relevant markers in tumor tissue with tumor response and/or other assessments of clinical response. For more information contact Madeleine at madeleine.schrier@mssm.edu or (212) 824-7310.

**THYROID**

A Phase II Trial Using RAD001 for Patients with Radioiodine Refractory Thyroid Cancer  
**GCO # 11-0258**

**Study Type:** Phase II  
**Sponsor:** Novartis  
**Principal Investigator:** Dr. Krzysztof Misiukiewicz  
**Research Nurse:** Patricia Strebel, RN  
**Coordinator:** Madeleine Schrier

The objectives of this study are to obtain information about the effectiveness of RAD001 as first or second line treatment in patients with thyroid cancer that has shown evidence of disease progression. There are currently no established therapy options for patients with Radioactive Iodine refractory thyroid cancer who have progressed on oral tyrosine kinase inhibitors, or who are not candidates for treatment with these drugs. If promising activity is detected, larger subsequent studies can be designed based on this treatment method. Eligible patients are 18 years or older and have newly diagnosed radioiodine refractory thyroid cancer or have progressed after chemotherapy with doxorubicine or a small molecule tyrosine kinase inhibitor and who have documented disease progression within 6 months. For more information contact Madeleine at madeleine.schrier@mssm.edu or (212) 824-7310.

A Multicenter, Randomized, Double-Blind, Placebo-Controlled, Phase 3 Trial of E7080 in 131I-Refractory Differentiated Thyroid Cancer  
**GCO # 12-0007**
Study Type: Phase III
Sponsor: Eisai
Principal Investigator: Dr. Krzysztof Misiukiewicz
Research Nurse: Patricia Strebel
Coordinator: Rachel Abbott

This is a multicenter, randomized, double-blind, placebo controlled Phase 3 study to compare the progression-free survival and overall survival of subjects with $^{131}$I-refractory differentiated thyroid cancer (DTC) and radiographic evidence of disease progression within the prior 12 months, treated with E7080 24 mg by continuous once daily (QD) oral dosing versus placebo. For more information, contact Rachel at rachel.abbott@mssm.edu or (212) 824-7337.

**CORRELATIVE**

**HPV Oral Transmission Study in Partners Over Time (HOTSPOT)**
GCO # 10-1473

Study Type: Epi/biomarker
Principal Investigator: Dr. Marshall Posner
Coordinator: Nadia Camille

This study collects data on personal life style, social habits, and demographics; samples of saliva and blood; and samples of tumor to study the transmission, biology and immunity of the subjects to HPV. Eligible patients are 18 years or older with incident oropharyngeal, laryngeal, or hypopharyngeal squamous cell cancer. Partners of enrolled patients who identifies as a spouse or sexual partner of an oropharyngeal case may also participate. For more information, contact Nadia at nadia.camille@mountsinai.org or (212) 241-5253.

**Biomarkers of Immune Function as Predictors of Head and Neck Squamous Cell Carcinoma (HNSCC) Response to Therapy**
GCO # 10-1219

Study Type: Immune monitoring/biomarker
Principal Investigator: Dr. Andrew Sikora
Coordinator: Madeleine Schrier

This is a study of the immune response in patients with oropharyngeal cancer who undergo treatment with radiation or chemoradiation. We are testing the hypothesis that radiation-based therapy of oropharyngeal cancer is associated with activation of the endogenous HPV-specific immune response. In this study, we will collect blood at several time points before, during, and after treatment to monitor the immune response in patients with tumors positive and negative for HPV versus normal healthy volunteers. Eligible patients are 18 years or older and have biopsy-proven squamous cell carcinoma, Stage II-IV, of the oropharynxx, with a treatment plan including radiation, chemo-radiation, and/or TORS. For more information, contact Madeleine at madeleine.schrier@mssm.edu or (212) 824-7310.
**Social Support, Emotional Disclosure, and Adjustment to Head and Neck Cancer**  
GCO # 09-0557

**Study Type:** Investigator Initiated - Quality of Life  
**Principal Investigator:** Dr. Hoda Badr  
**Coordinator:** Chi Yeung

The purpose of this study is to examine factors that may affect the quality of life of and relationships of patients with head and neck cancer and their spouses/partners or family caregivers. The study involves completing questionnaires at different time points (e.g., before, after, and during treatment). Patients and their caregivers will each receive a small gift card ($10) each time they complete a questionnaire. Eligible patients are 18 years or older and are initiating radiation therapy or surgery for head and neck cancer, and have a spouse or significant other with whom he/she resides or can identify a family member that serves as his/her primary caregiver. For more information, contact Chi at (212) 659-5542.

**Improving Oral Health and Quality of Life After Oral Cancer: A Web-based Approach**  
GCO # 11-0040

**Study Type:** Investigator Initiated - Quality of Life  
**Principal Investigator:** Dr. Hoda Badr  
**Coordinator:** Chi Yeung

This study proposes a web-based intervention, called Computer Assisted Oral Health Rehabilitation and Support (CARES), which is guided by Self Determination Theory (SDT). Specific aims are to develop and evaluate the content of the CARES intervention, to evaluate the feasibility of the CARES prototype, and to pilot test the initial efficacy of CARES. For more information, contact Chi at (212) 659-5542.

**Oncologic Therapy and the Development of Obstructive Sleep Apnea in the Head and Neck Patient**  
GCO #: 11-1718

**Study Type:** Sleep Apnea  
**Principal Investigator:** Fred Lin, MD

The purpose of this study will be to determine if current cancer treatments (surgery, chemotherapy, radiation or a combination) for head and neck cancer, has any effect on developing and worsening of obstructive sleep apnea (OSA) during and after the treatments. OSA is often described by patients as being unable to breathe during sleep, including snoring and breath holding. If not diagnosed properly, one may complain about inability for a good night’s sleep, poor attention, bad moods and can possibly lead to conditions such as high blood pressure. Knowing that you have OSA can also help your doctor recommend appropriate treatments, such as a breathing mask called continuous positive airway pressure (CPAP) or another surgery. For more information, contact Fred at fred.lin@mssm.edu.
Transoral Robotic Surgery (TORS) vs. Non-Surgical Treatment for Oropharyngeal Cancer: A COST-UTILITY STUDY
GCO #11-1683

**Study Type:** Cost-Utility  
**Principal Investigator:** Eric Genden, MD  
**Research Resident/Fellow:** John de Almeida, MD and Nate Villanueva  
**Coordinator:** Nadia Camille

The objective of this study is to perform a rigorous cost-utility analysis of Transoral Robotic Surgery (TORS) compared to Chemoradiotherapy for early T-stage oropharyngeal squamous cell carcinoma. Eligible subjects are 18 years or older, cancer-free, and are non-physicians. For more information, contact Nadia at nadia.camille@mountsinai.org or (212) 241-5253.

Transoral Robotic Surgery (TORS) vs. Non Surgical Treatment for Oropharyngeal Cancer: A Retrospective and Prospective Multi-Institutional Comparative Study
GCO #11-1428

**Study Type:** Retrospective/Prospective Multi-Institutional  
**Principal Investigator:** Eric Genden, MD  
**Research Resident/Fellow:** John de Almeida, MD and Nate Villanueva  
**Coordinator:** Nadia Camille

The objectives of this study are to evaluate the oncologic and functional outcomes of Transoral Robotic Surgery (TORS) for oropharyngeal squamous cell carcinoma and to compare outcomes between TORS and chemoradiotherapy by means of a retrospective and prospective stage matched comparative study. Eligible subjects for the retrospective component are patients with resectable primary oropharyngeal squamous cell carcinoma (T1-T4), who were treated with TORS or conventional chemotherapy from January 1, 2006 to December 31, 2011. Eligible subjects for the prospective component are patients with primary oropharyngeal squamous cell carcinoma (T1-T4) that are considered within the indications for robotic surgical treatment or conventional chemotherapy and are treated sometime from January 1, 2012 to the closure of the study.

**IMAGING/DIAGNOSTIC**

Evaluation of a Miniaturized Microscope Device for the Detection of Esophageal Squamous Cell Cancer
GCO # 10-0982

**Study Type:** International, Multicenter Clinical Trial  
**Principal Investigator:** Dr. Sharmila Anandasabapathy  
**Coordinator:** Josephine Mitcham
The overall objective of this multicenter study is to determine whether high-resolution-imaging during routine diagnostic upper endoscopy can assist clinicians in detecting and discriminating dysplastic (pre-cancerous) areas. We are conducting a clinical trial of a new high-resolution microendoscope (HRME) in high-risk subjects undergoing endoscopic screening for suspected squamous cell neoplasia in both the United States and northern China. Eligible patients are 18 years or older and are undergoing endoscopic screening for esophageal squamous cell neoplasia. For more information, contact Josephine at josephine.mitcham@mountsinai.org or (212) 241-6293.

**In Vivo Multimodal Imaging of Upper Aerodigestive Epithelium**

**GCO # 09-2057**

**Study Type:** Investigator Initiated - Imaging/Device Study  
**Principal Investigator:** Dr. Andrew Sikora  
**Coordinator:** Lauren Levy

The overall objective of this study is to evaluate whether noninvasive fluorescence and reflectance imaging of the upper aerodigestive tract can help clinicians more accurately determine intraoperative margins during ablative cancer surgery. This is an *in vivo* study designed to evaluate the feasibility of using prototype optical imaging technology to enhance the discrimination between areas of noncancerous “normal” and cancerous mucosa. Eligible patients have biopsy-proven squamous cell carcinoma of the oral cavity, oropharynx, larynx, and/or hypopharynx and are receiving surgical treatment. For more information, contact Lauren at lauren.levy@mssm.edu or (212) 241-5951.

**A Prospective, Multicenter Study, to Evaluate the Efficacy and Safety of [18F]-ML-10, a PET Imaging Radiotracer, in Early Detection of Response of Non-Hematological Tumors to Concurrent Chemoradiotherapy (ApoSense).**

**GCO # 10-0926**

**Study Type:** Phase II  
**Sponsor:** ApoSense  
**Principal Investigator:** Dr. Lale Kostakoglu, Department of Nuclear Medicine  
**Coordinator:** Lena Marra

This is a multicenter study to evaluate the efficacy and safety of [18F]-ML-10 in its ability to image apoptosis and predict treatment response of non-hematological tumors in patients treated with concurrent chemoradiotherapy. [18F]-ML-10 is a novel, low molecular-weight probe, rationally-designed for targeting cells undergoing apoptosis. Eligible patients are 18 years or older with newly diagnosed locally advanced squamous cell carcinoma of the head and neck, with a primary tumor size of >2. For more information, contact Yasir at yasir.qureshi@mountsinai.org (212) 241-2209 or Lena at lena.marra@mountsinai.org (212) 241-9369.
POST-ACCRUAL ONGOING TRIALS

The Effect of Prophylactic Swallowing Exercises on Head and Neck Cancer Patients.
GCO # 07-0462

**Study Type:** Prospective Randomized Clinical Trial  
**Principal Investigator:** Tamar Kotz, MS  (212) 241-8452  
**Coordinator:** Nadia Camille  (212) 241-5253

The objective of this study is to investigate the effects of intensive prophylactic swallowing exercises on the swallowing outcomes of head and neck cancer patients treated with radiation therapy with and without chemotherapy.

Concurrent and Maintenance Sunitinib and Image-Guided Radiation Therapy for Oligometastases
GCO # 06-0906

**Study Type:** Phase II  
**Principal Investigator:** Max Sung, M.D.

This is the first published study of combined sunitinib and radiation in humans. This is also the first attempt to combine drug therapy with radiation in patients with distant metastases.

A Double-Blind, Randomized Phase III Study Evaluating the Efficacy and Safety of Sorafenib Compared to Placebo in Locally Advanced/Metastatic RAI-Refractory Differentiated Thyroid Cancer
GCO # 10-1607

**Study Type:** Phase III  
**Sponsor:** Bayer  
**Principal Investigator:** Dr. Krzysztof Misiukiewicz  
**Research Nurse:** Patricia Strebel, RN  
**Coordinator:** Nadia Camille

This Phase III study is designed to evaluate the efficacy and safety of sorafenib vs. placebo in patients with locally advanced/metastatic RAI-refractory differentiated thyroid cancer. Subjects will be randomized to receive either sorafenib or placebo in a blinded fashion. It is estimated that enrollment will require approximately 18 months. Eligible patients are 18 years or older with locally advanced/metastatic RAI-refractory differentiated thyroid cancer. For more information, contact Nadia at nadia.camille@mountsinai.org or (212) 241-5253.
SATELLITE STUDIES

Plasticity of a CD49fhigh Transiently Quiescent Population Directs Stochastic Tumor-Initiating Capacity in HNSCC.
GCO # TBD
Study Type: Stem Cell Research
Principal Investigator: Julio Aguirre-Ghiso, PhD (212) 241-9582
Coordinator: Paloma Brag

Detection of Trm9 levels in Colon and Head and Neck Tumors
GCO # 08-0960
Study Type: Correlative
Principal Investigator: Julio Aguirre-Ghiso, PhD (212) 241-9582
Coordinator: Paloma Brag

Retrospective Analysis of Dormancy and Progression Markers in Head and Neck Squamous Cell Carcinoma.
GCO # HSD-09-00060
Study Type: Retrospective IHC
Principal Investigator: Julio Aguirre-Ghiso, PhD (212) 241-9582
Coordinator: Paloma Brag

Detection of Dormant HNSCC Cells in Bone Marrow
GCO # 09-0770
Study Type: Prospective
Principal Investigator: Julio Aguirre-Ghiso, PhD (212) 241-9582
Coordinator: Paloma Brag

Prognostic Significance of p16 and Immune Infiltration in Head and Neck Cancer
GCO # 11-0097
Study Type: Retrospective Analysis of HNSCC Specimens
Principal Investigator: Andrew Sikora, M.D.
Coordinator: Charles Tong (212) 659-9454

TRIALS IN DEVELOPMENT

The Quarterback Trial: A Randomized Phase III Clinical Trial Comparing Reduced and Standard Radiation Therapy Doses After Induction Chemotherapy for Locally Advanced HPV 16 Positive Oropharynx Cancer
GCO: TBD
Study Type: Phase III Investigator Initiated
Principal Investigator: Marshall Posner, MD (212) 659-5567
Coordinator: Nadia Camille (212) 241-5253