SUBMITTED BY: **STEPHEN M. LANIER, PH.D., VICE PRESIDENT FOR RESEARCH**

REPORT ON WAIVERS APPROVED TO BOARD STATUTE 2.41.01.140

BACKGROUND

Four research projects required a petition to waive Statute 2.41.01.140 because of review restrictions on publishing the research results. These waivers have been approved by the Vice President for Research to allow acceptance of the relevant grant or contract, acting in accordance with the University's research policy on restricted and proprietary research.

OVERVIEW

CENTERS FOR DISEASE CONTROL AND PREVENTION AND NATIONAL INSTITUTE OF OCCUPATIONAL SAFETY AND HEALTH

This waiver allowed acceptance of a contract with the Centers for Disease Control and Prevention and the National Institute for Occupational Safety and Health to Youcheng Liu, Ph.D., associate professor, Department of Family Medicine and Public Health Sciences, School of Medicine. The study, "Assessment of elastomeric respirators in healthcare delivery settings," aims to determine the feasibility and capability of U.S. healthcare delivery organizations to routinely use reusable respirators and/or rapidly convert to their use during a public health emergency such as a large respiratory infectious disease outbreak. These respirators are not routinely used in healthcare delivery organizations and most healthcare personnel have little or no experience using them. This study will determine the feasibility and capability of U.S. healthcare delivery organizations to routinely use reusable respirators and/or rapidly convert to their use during a public health emergency when there is an increase demand for them.

This project required a waiver to Board Statute 2.41.01.140 because the sponsor requires that all resulting publication submissions and presentations are reviewed and approved by the sponsor prior to submission in an effort to protect all aggregate data analysis from participating sites and to assure that the CDC's study objectives are adhered to.

VERTEX PHARMACEUTICALS

This waiver allowed acceptance of a clinical study agreement with Vertex Pharmaceuticals Incorporated to Dana Kissner, M.D., associate professor – clinical, Department of Internal Medicine, School of Medicine. The study, "A Phase 3, Randomized, Double-blind, Controlled Study Evaluating the Efficacy and Safety of VX-445 Combination Therapy in Subjects With Cystic Fibrosis Who Are Homozygous for the F508del Mutation (F/F)," is one of several studies being performed by Dr. Kissner to evaluate the efficacy and safety of VX-445 in triple combination with Tezacaftor (TEZ) and Ivacaftor (IVA). This study is focusing on subjects with a specific DNA mutation, different from the other related studies. The participating patients have

continuing unmet needs despite the currently available cystic fibrosis transmembrane conductance regulator modular therapies. If this drug combination is found to be safe and effective it will be a new treatment for cystic fibrosis.

This project required a waiver to Board Statute 2.41.01.140 because the sponsor requires that all resulting publication submissions and presentations are delayed up to 120 days in order to protect the potential patentability of any technology.

VERTEX PHARMACEUTICALS

A second waiver for a study Dr. Kissner has with Vertex Pharmaceuticals Incorporated allowed acceptance of a clinical study agreement, "A Phase 3, Open-label Study Evaluating the Long-term Safety and Efficacy of VX-445 Combination Therapy in Subjects With Cystic Fibrosis Who Are Homozygous or Heterozygous for the F508del Mutation." The project is in parallel with several other studies conducted by Dr. Kissner using the same combination of drugs. This specific study will evaluate the long-term efficacy and safety of these drugs in participants with cystic fibrosis who carry a specific DNA mutation. The safety and tolerability of the long-term treatment is based on adverse events (AEs), clinical laboratory values, ECG's, vital signs and pulse oximetry with favorable results.

This project required a waiver to Board Statute 2.41.01.140 because the sponsor requires that all resulting publication submissions and presentations are delayed up to 120 days in order to protect the potential patentability of any technology.

VERTEX PHARMACEUTICALS

A third waiver for a study Dr. Kissner has with Vertex Pharmaceuticals Incorporated allowed acceptance of a clinical study agreement, "A Phase 3, Randomized, Double-blind, Controlled Study Evaluating the Efficacy and Safety of VX 445 Combination Therapy in Subjects With Cystic Fibrosis Who Are Heterozygous for the F508del Mutation and a Minimal Function Mutation (F/MF)." The project will evaluate the efficacy and safety of VX-445 in triple combination with two other drugs (Tezacaftor (TEZ) and Ivacaftor (IVA)) in subjects with a specific DNA mutation who have been non-responsive to other therapies including TEZ or IVA. Patients with this genotype usually have severe disease and lack an approved therapy. In vitro and clinical data for other studies using VX-445 suggest that the triple combination being used for this study will be greatly beneficial for these types of patients.

This project required a waiver to Board Statute 2.41.01.140 because the sponsor requires that all resulting publication submissions and presentations are delayed up to 120 days in order to protect the potential patentability of any technology.