

**New Jersey State Commission on Cancer Research
LAY ABSTRACT OF RESEARCH PROJECT**

NAME OF PRINCIPAL INVESTIGATOR/PROGRAM DIRECTOR: **Dale Schaar**

Project Title: **The Development of Phorbol Ester-Based Therapy for AML**

Description: **We are developing novel therapies for patients with blood-borne cancers.**

The focus of this proposal is to investigate a novel approach to treating patients with blood-borne cancers, such as leukemia, and also bone marrow diseases that have failed all of currently available treatment options. A drug, 12-O-tetradecanoylphorbol-13-acetate (TPA), has been widely studied in the laboratory regarding its ability to elicit changes in leukemic cells that are characteristic of normal blood cell maturation. Clinically, TPA has recently been evaluated as a therapeutic option in these patients with relapsed or refractory disease through an experimental trial at the Cancer Institute of New Jersey. This clinical trial has shown that TPA can be administered safely to patients with refractory malignancies and has yielded exciting observations in the laboratory. These scientific results have suggested other agents that when combined with TPA, may increase TPA's therapeutic effects as well as potentially ameliorate some of its side effects. This proposal's scientific focus will be developing novel TPA-based therapies for patients with refractory blood cell diseases. The effect of TPA in combination with other agents will be studied in abnormal human blood cells at the molecular and cellular level. The final goal will be the identification of biological signals required to convert the malignant blood cells to their normal counterparts or initiate leukemia cell suicide pathways, thereby re-establishing functional blood cell formation and function within these patients.