



**Department of Defense
US Army Medical Research
and Materiel Command**



**Fiscal Year 2004 (FY04)
Tuberous Sclerosis Complex Research Program (TSCR)
Funded Awards List**

TABLE OF CONTENTS

Introduction

Research Awards

- ◆ Concept
- ◆ Idea Development
- ◆ Natural History Development

Participants

- ◆ Peer Reviewers
- ◆ Integration Panel Members
- ◆ Ad Hoc Programmatic Reviewers

Glossary of Terms

Introduction

The US Army Medical Research and Materiel Command (USAMRMC) is pleased to present the awards list of funded projects for the fiscal year 2004 (FY04) Tuberous Sclerosis Complex Research Program (TSCRCP). Award negotiations were completed on September 30, 2005. The awards listed in this document were selected in a competitive, two-tier review process. Funding decisions were based on evaluations of scientific excellence in the first tier, followed by determinations of programmatic relevance in the second tier. These projects represent a diverse portfolio of scientific research directed toward the program's overall goal of promoting innovative research focused on improved prevention, diagnosis, and/or treatment of tuberous sclerosis complex (TSC).

Congressional direction for FY04 specified \$3 million for TSC research. Following the receipt of funds, a programmatic strategy was developed, proposals were solicited and evaluated, award recommendations were made, and contract negotiations were completed. A total of 13 awards was made.

The FY04 programmatic strategy called for Concept, Natural History Development, and Idea Development Awards. The Concept Award, offered for the first time in FY04, is designed to fund the exploration of untested, high-risk questions or theories that could give rise to testable hypotheses. The Natural History Development Award, also new for FY04, is intended to support the development of a multi-institutional TSC natural history study, including the establishment of the research team, the development of tools for data management and research administration, and the development of the clinical protocol. The Idea Development Award is intended to fund innovative research directed toward improved prevention, diagnosis, and/or treatment of TSC.

As the funded investigators embark on these projects, the Department of Defense and the US Army gratefully acknowledge the participation of their scientific, clinical, and consumer advisors. The expertise, vision, and diversity of perspective of all individuals who contributed to this program were vital to developing a sound investment strategy on behalf of all persons affected by TSC. It is with great anticipation and excitement that we await the outcomes of this body of research.

Concept Awards

Log Number	PI Last Name	PI First Name	PI Institution	Proposal Title	Final Budget
TS043017	Sabatini	David	Whitehead Institute for Biomedical Research	Identifying Novel Drug Targets for the Treatment of Tuberous Sclerosis Complex Using High Throughput Technologies	\$100,000
TS043023	Squillace	Rachel	The Rothberg Institute for Childhood Diseases	Generation of in Vitro Cellular Models of Lymphangioliomyomatosis for the Development of Tuberous Sclerosis Therapeutics	\$96,800
TS043027	Xiao	Bo	Johns Hopkins University School of Medicine	Driving the Formation of Tuberous Sclerosis Complex by Creating Conditional Rheb Transgenic Mice	\$100,000
TS043030	Xu	Li-Hui	OncoImmune, Ltd.	Study of 2-Deoxyglucose as a Potential Treatment for TSC	\$100,000
TS043031	Sahin	Mustafa	Children's Hospital	Development of Peptide Inhibitors of Rheb Signaling Pathway	\$100,000
TS043037	Zhong	Yi	Cold Spring Harbor Laboratory	Analysis of Learning Disabilities of Tuberous Sclerosis Complex in Drosophila	\$100,000
TS043045	Guan	Kun-Liang	Regents of the University of Michigan	Regulation of TSC1/TSC2 Stability and Rheb GTP Level by Herc1	\$100,000

Idea Development Awards

Log Number	PI Last Name	PI First Name	PI Institution	Proposal Title	Final Budget
TS043006	Castro	Ariel	University of California, San Francisco	Functional Relevance of the Ras-Related GTPase Rheb in Tuberous Sclerosis	\$275,000
TS043009	Matsumoto	Tomohiro	Kyoto University	Fission Yeast Model Study for Dissection of TSC Pathway	\$420,000
TS043010	Shibley	James	Washington University	Modeling Phenotypes of Tuberous Sclerosis in the Mouse	\$307,949
TS043013	Chada	Kiran	University of Medicine and Dentistry	HMGA2 in Tuberous Sclerosis	\$444,472
TS043015	Tamanoi	Fuyuhiko	University of California, Los Angeles	A Genetic Approach to Define the Importance of Rheb in Tuberous Sclerosis	\$411,672

Natural History Development Award

Log Number	PI Last Name	PI First Name	PI Institution	Proposal Title	Final Budget
TS043004	Sparagana	Steven	Texas Scottish Rite Hospital for Children	Tuberous Sclerosis Complex National Database	\$141,307

**Fiscal Year 2004 Tuberos Sclerosis Complex Research Program
Peer Reviewers**

Peer Reviewers	Degree	Institution/Affiliation
Baraban, Scott	Ph.D.	University of California, San Francisco
Bidichandani, Sanjay	M.D., Ph.D.	University of Oklahoma Health Sciences Center
Bissler, John	M.D.	Cincinnati Children's Hospital Medical Center
Bradlyn, Andrew	Ph.D.	West Virginia University
Fernandez-Valle, Cristina	Ph.D.	University of Central Florida
Gambello, Michael	M.D., Ph.D.	University of Texas Medical School at Houston
Kwiatkowski, David	M.D., Ph.D.	Brigham and Women's Hospital
Mastbaum, Celia		Tuberous Sclerosis Alliance
Miller, Douglas	M.D., Ph.D.	New York University School of Medicine
Moore, Bartlett	Ph.D.	University of Texas M.D. Anderson Cancer Center
Newburgh, M. Janet	Ph.D.	Scientific Review Administrator
Reilly, Karlyne	Ph.D.	Mouse Cancer Genetics Program, NCI-Frederick
Sheffield, Patrick		Tuberous Sclerosis Alliance
Sherman, Lawrence	Ph.D.	Oregon Health Sciences University
Short, Marion Priscilla		The National Neurofibromatosis Foundation
Sims, Katherine	M.D.	Massachusetts General Hospital and Harvard Medical School
Stubbs, John	Ph.D.	Howard University College of Medicine
Sybert, Virginia	M.D.	University of Washington
Viskochil, David	M.D., Ph.D.	University of Utah
Vogel, Kristine	Ph.D.	University of Texas Health Science Center at San Antonio

**Fiscal Year 2004 Tuberous Sclerosis Complex Research Program
Integration Panel Members**

Reviewers	Degree	Institution/Affiliation
Adamson, Peter	M.D.	University of Pennsylvania School of Medicine
Crino, Peter	M.D., Ph.D.	University of Pennsylvania School of Medicine
Finkelstein, Robert	Ph.D.	National Institute of Neurological Disorders and Stroke
Gibbs, Jackson	Ph.D.	Merck Research Laboratories
Holets Whittemore, Vicky (Chair)	Ph.D.	Tuberous Sclerosis Alliance
Johnson, William	M.D.	University of Medicine and Dentistry of New Jersey, Robert Wood Johnson Medical School
Korf, Bruce	M.D., Ph.D.	University of Alabama, Birmingham
Legius, Eric	M.D., Ph.D.	Catholic University of Leuven, Belgium
Petri Henske, Elizabeth (Chair-Elect)	M.D.	Fox Chase Cancer Center
Small, Judy	Ph.D.	The National Neurofibromatosis Foundation, Inc.

**Fiscal Year 2004 Tuberous Sclerosis Complex Research Program
Ad Hoc Programmatic Reviewer**

Ad Hoc Reviewer	Degree	Institution/Affiliation
Dabora, Sandra	M.D., Ph.D.	Brigham and Women's Hospital/Harvard Medical School

Glossary of Terms

Concept Award: The intent of this award mechanism is to fund the exploration of an initial concept or theory that could give rise to a testable hypothesis. This award is intended to encourage the exploration of untested, high-risk questions relevant to tuberous sclerosis complex (TSC) and is not intended to support the next step in an established research project. Presentation of preliminary data is not consistent with the intent of this mechanism. Concepts from complementary areas of science such as chemistry, biophysics, mathematics, and engineering are encouraged as are research proposals involving consumer-scientist collaborations.

Idea Development Award: The intent of this award mechanism is to encourage innovative research directed toward improved prevention, diagnosis, and/or treatment of TSC. To be eligible for an Idea Development Award, the applicant must be an independent investigator at the level of Assistant Professor (or equivalent) or higher. All Idea Development Award proposals must include preliminary data relevant to TSC research and the proposed project.

Natural History Development Award (NHDA): The intent of this award mechanism is to fund the development of a multi-institutional natural history study that will provide quantitative data on TSC-related tumor growth and/or other manifestations of TSC. Proposals must include plans for the establishment of the research team, the development of tools for data management and research administration, the definition of recruitment strategies, and the development of the clinical protocol. Products of the NHDA mechanism include a detailed clinical protocol and a Natural History Study Award submission in the following fiscal year (pending receipt of funds by the Tuberous Sclerosis Complex Research Program).