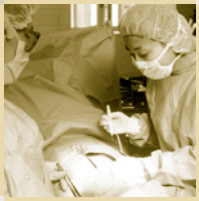






IX. Tuberous Sclerosis Complex Research Program

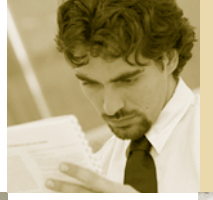
Vision: To lessen the impact of tuberous sclerosis.

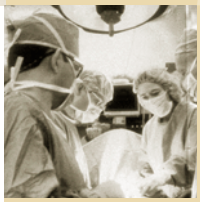
Mission: To encourage innovative research aimed at understanding the role and function of proteins produced by the TSC1 and TSC2 tumor suppressor genes.

Congressional Appropriations for Peer Reviewed Research: $1M in FY02 and $2M in FY03

Funding Summary: 3 FY02 proposals funded (2 funded with the FY02 appropriation and 1 with the FY03 appropriation); an additional 3 awards anticipated from the FY03 appropriation

...shaping the future of health care to prevent, control, and cure diseases.





"I was greatly impressed by the quality of applications received by the TSCRP, and by the professionalism and the expertise of the panel members. I strongly recommend the continuation of this exciting program under the guidance of the U.S. Army professionals currently involved."

Jeffrey DeClue, Ph.D.,
Senior Staff Fellow,
National Cancer Institute;
FY03 Ad Hoc Programmatic Reviewer

The Disease

Tuberous sclerosis is a genetic disorder that can affect any or all systems of the body. The disorder is characterized by seizures, developmental delays, kidney disease, behavioral problems, and the growth of benign tumors (tubers) on vital organs such as the brain, kidneys, and heart. These tumors typically calcify with age, becoming hard (sclerotic). Children with tuberous sclerosis may have autistic-like symptoms. Tuberous sclerosis affects as many as 25,000 to 50,000 individuals in the United States and about 1 to 2 million individuals worldwide. Although this disorder can be inherited as an autosomal dominant trait, two-thirds of cases are the result of a spontaneous genetic change on one of two genes, TSC1 or TSC2.[1] The TSC1 gene is located on chromosome 9 and produces the protein hamartin. The TSC2 gene is located on chromosome 16 and produces the protein tuberin. Hamartin and tuberin are believed to act as tumor growth suppressors. Therefore, their dysfunction may underlie the appearance of tumors that characterize tuberous sclerosis. There is currently no cure for this disease; however, surgical intervention and a number of treatments can help affected individuals.

Program Background

The Department of Defense (DOD) Tuberous Sclerosis Complex Research Program (TSCRP) was established in fiscal year 2002 (FY02) by Joint Appropriations Conference Committee Report No. 107-350, which provided $1 million (M) for tuberous sclerosis research. The mission of the TSCRP is to support innovative research directed toward a better understanding of the role and function of proteins produced by the TSC1 and TSC2 tumor suppressor genes. Appendix B, Table B-7, summarizes the congressional appropriations and the investment strategy executed by the TSCRP for FY02–03.

The Fiscal Year 2002 Program

The goal of the FY02 TSCRP was to promote research in the field of TSC. One award mechanism, Idea Development Awards, was offered to support innovative research aimed at understanding the role and function of proteins produced by the TSC1 and TSC2 tumor suppressor genes.

[1] *National Institute of Neurological Disorders and Stroke Fact Sheet, 2001; Harrison's Principles of Internal Medicine, 15th Edition, McGraw-Hill, 2001.*

Of the 13 proposals received, 3 were funded. Table IX-1 provides a summary of the FY02 TSCRP award mechanism in terms of proposals received, number of awards, and dollars invested.

Table IX-1. Funding Summary for the FY02 TSCRP

Category and Award Mechanism	Number of Proposals Received	Number of Awards	Investment
Research			
Idea Development	13	3	$1,244,002*
Total	**13**	**3**	**$1,244,002***

**$374,002 from the FY03 appropriation.*

The Vision for the Fiscal Year 2003 Program

Congress appropriated $2M to continue the TSCRP in FY03. Idea Development Awards were again offered to support innovative research aimed at understanding the role and function of proteins produced by the TSC1 and TSC2 tumor suppressor genes. A total of 13 proposals was received, as shown in Table IX-2, and approximately 3 awards are expected.

Table IX-2. Award Mechanisms Offered and Proposals Received for the FY03 TSCRP

Category and Award Mechanism	Number of Proposals Received
Research	
Idea Development	13
Total	**13**

Summary

Since FY02, the DOD TSCRP has been responsible for managing $3M in congressional appropriations. Projects funded by this program are anticipated to support research that will lead to the substantial improvement in the understanding, diagnosis, and treatment of tuberous sclerosis and enhance the quality of life of persons with the disease.

Fiscal Year 2003 Ad Hoc Programmatic Reviewers[2]

Jeffrey DeClue, Ph.D.
National Cancer Institute

Vicky Holets Whittemore, Ph.D.
Tuberous Sclerosis Alliance

[2] *FY02 TSCRP Programmatic Reviewers serve as ad hoc members to the FY03 Neurofibromatosis Research Program Integration Panel.*