



TUBEROUS SCLEROSIS COMPLEX RESEARCH PROGRAM (TSCRCP)

ACCELERATING TSC RESEARCH TOWARD A CURE

MISSION: Support innovative and high-impact research that promotes discoveries in TSC, from mechanistic insights to clinical application across all ages, by fostering new ideas and investigators for the benefit of Service Members, their beneficiaries, and the American public

FY22
Appropriation
\$8M

TSCRCP Fiscal Year 2022 (FY22) Funding Mechanisms



Exploration - Hypothesis Development Award (EHDA)

\$150K



Idea Development Award New-to-the-Field Investigator (IDA-NFI)

\$500K



Idea Development Award Established Investigator (IDA-EI)

\$500K



Clinical Translational Research Award (CTRA)

\$1M

Clinical Translational Research Award

- The CTRA supports studies that will move promising, well-founded preclinical and/or clinical research findings closer to clinical application, including diagnosis, prognosis, or treatment of Tuberous Sclerosis Complex (TSC).
- *Collaborations between clinicians and research scientists are strongly encouraged.*

Deadlines

30 June 2022

Letter of Intent

28 July 2022

Full Applications Due

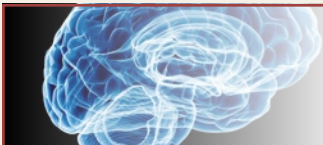
September 2022

Peer Review

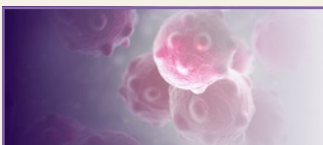
December 2022

Programmatic Review

TSCRCP FY22 Focus Areas



Understanding and **treating** the features of TSC-associated neuropsychiatric disorders and reducing their impact, including pharmacological, behavioral, and surgical interventions



Strategies for **eradicating tumors** associated with TSC and TSC-associated **lymphangiomyomatosis (LAM)**, including gaining a deeper mechanistic understanding of TSC signaling pathways and tumor microenvironment



Preventing epilepsy, improving treatment, and **mitigating** neurodevelopmental outcomes associated with TSC-related seizures

TSCRP-Funded Research Laid the Groundwork for Recent Food and Drug Administration (FDA) Drug Approval

> 2011

The TSCRP funded an early trial led by Dr. Mary Koenig to study topical rapamycin (also known as sirolimus) to treat facial angiofibromas in TSC patients. The goal of the **TREATMENT** trial was to develop a form of rapamycin that could provide a safe, effective treatment for facial angiofibromas in patients with TSC.

> 2014

The **TREATMENT** trial was completed with a final enrollment of 179 patients.

> 2018

Dr. Koenig's team published their results, "Efficacy and Safety of Topical Rapamycin in Patients with Facial Angiofibromas Secondary to Tuberous Sclerosis Complex: The **TREATMENT** Randomized Clinical Trial" in *JAMA Dermatology* in 2018.

> 2022

The FDA approved **HYFTOR™** for facial angiofibromas. **HYFTOR™** is the first FDA-approved topical treatment for facial angiofibromas in adults and children 6 years of age or older who have TSC.

Bench to Bedside - Recently Funded CTRAs



Toward Chimeric Antigen Receptor Transgenic T Cell Therapy for TSC, led by Dr. Isabelle Le Poole at Northwestern University. The project investigates whether immunotherapy can be used to treat TSC through the adoptive transfer of T cells. (FY17)



Resting State Functional MRI (RS) Finds Correct Surgical Target to Stop Seizures in TSC, led by Dr. Varina Boerwinkle at the University of North Carolina. This study evaluates whether RS can identify where seizures are coming from in children with TSC and whether targeted removal improves their overall prognosis. (FY19)



Mapping of Brain GABA Levels in TSC Using High-Resolution Proton MR Spectroscopic Imaging, led by Dr. Doris Da May Lin at Johns Hopkins University. This is a pilot study to test the hypothesis that brain GABA levels are abnormal in patients with TSC and are related to the severity of seizure activity. (FY19)



TSC Remote Assessment and Intervention (TRAIN), led by Dr. Connie Kasari at University of California, Los Angeles. The primary goal is to determine whether joint engagement and social communication in children with TSC can be improved by using remotely administered caregiver training. (FY19)

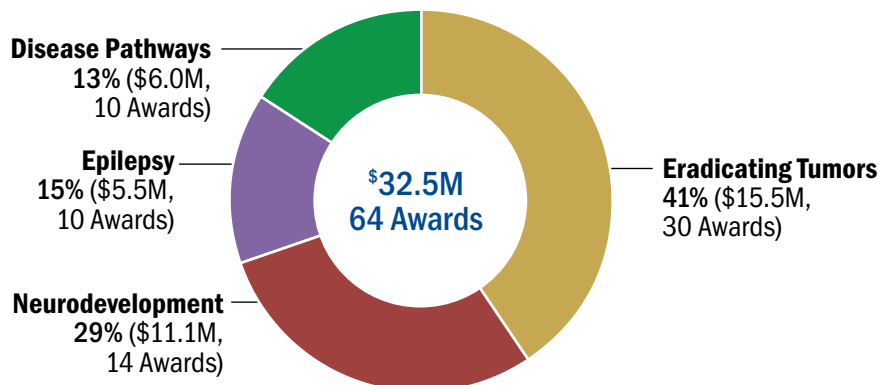


LAM Pilot Study with Nilotinib LAMP-2, led by Dr. Jeanine D'Armiento at the Columbia University Medical Center. This project evaluates the safety and tolerability of nilotinib (tumoricidal therapeutic) in patients with LAM. (FY20)



Assessment and Treatment of Behavior Problems in TSC at Preschool Age: A Telehealth Approach, led by Dr. Nicole McDonald at the University of California, Los Angeles. The goal of this project is to provide families of children with TSC and behavior problems access to an effective and tailored approach to helping their child. (FY21)

FY15-FY20 Awards



For more information, visit:
<https://cdmrp.army.mil/funding/tscrp>