

TUBEROUS SCLEROSIS COMPLEX RESEARCH PROGRAM



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 to benefit Service Members, their Families and the public

**Congressional Appropriations
FY02-FY24:
\$121M total**



“The TSCRP plays a unique role in funding impactful TSC research. TSCRP’s vision, mission and

focus areas are reviewed and updated annually to ensure the program is funding the most relevant and timely research. Individuals living with TSC, or their family members, are involved in annual vision setting and in prioritizing applications for funding. Additionally, the TSCRP includes representatives from the NIH and TSC Alliance in these processes, ensuring the types of research funded by TSCRP are distinct from other organizations.”

*Steve Roberds, Ph.D.,
TSC Alliance,
FY23 Programmatic Panel Member*



SCOPE OF THE PROBLEM

TSC is a rare genetic disorder caused by mutations in the TSC1 or TSC2 genes, causing tumor growth in multiple organs.

- Approximately **40,000–80,000** cases in the U.S.
- Up to **2 million** cases worldwide¹

Affected organs may include:²

- **Brain**
- **Lungs**
- **Eyes**
- **Kidneys**
- **Heart**
- **Skin**



RELEVANCE TO MILITARY HEALTH

From 2013–2022, TSC-related MHS medical encounters for DOD Beneficiaries included:³

 Average Patient Encounters	 Outpatient Encounters	 Hospital Bed Days
3,287	30,051	5,066

PROGRAM PRIORITIES

- Understanding, preventing, and treating the features of TSC-Associated Neuropsychiatric Disorders and reducing their impact
- Strategies for preventing and eradicating tumors and cysts associated with TSC
- Preventing epilepsy, improving treatment, and mitigating neurodevelopmental adverse outcomes associated with TSC-related seizures
- Developing, assessing, and testing emerging technologies to improve outcomes of TSC
- Understanding or improving outcomes of maternal-fetal health

¹ <https://rarediseases.org/rare-diseases/tuberous-sclerosis/#affected>

² <https://www.ninds.nih.gov/health-information/disorders/tuberous-sclerosis-complex>

³ Defense Medical Surveillance System, The Armed Forces Health Surveillance Branch



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

PROGRAM IMPACT AND OUTCOMES

The TSCRP invests in the full spectrum of product development.



RESEARCH BREAKTHROUGHS – MAKING A DIFFERENCE

Addressing Unmet Clinical Needs

- fMRI-guided brain surgery to reduce seizures
- Combined drug therapy: Imatinib and sirolimus
- Telehealth delivery of parent-child interaction therapy
- Novel biosensor for drug monitoring
- Behavioral intervention for TAND
- Language processing and epilepsy surgery
- TANDem2 – Longitudinal study of TAND

Developing Gene Therapies

- Gene replacement with AAV9 in TSC1 animal model
- Gene replacement with AAV9 in TSC2 animal model
- Extracellular vesicles as a platform to enhance TSC1 gene delivery

Improving Patient Care

- HYFTOR™ – FDA-approved topical medicine to treat facial angiofibroma
- JASPER – Behavioral intervention to improve social communication in children with TSC

KEY ACCOMPLISHMENTS BY THE NUMBERS



Point of Contact: CDMRP Public Affairs

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