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Key Talking Points

Our Ask: Funding for the Tuberous Sclerosis Complex Research Program (TSCRCP) at \$10 Million in FY25 Appropriation

Why is the TSCRCP important and unique?

- This line item is the only source of dedicated **federal** funding for tuberous sclerosis complex (TSC) research.
- Awards are peer-reviewed by scientific experts and consumers (individuals with TSC and caregivers/family).
- It has had bipartisan support since the program's inception in FY02. Congress has appropriated \$113 million between FY02 and FY23. The most recent appropriation was \$10 million.
- In FY23, 12 applications were selected for funding, but an additional 17 applications scored as Outstanding or Excellent were not funded (totaling \$8.6 million in unfunded research). Renewing the appropriation to \$10 million would enable funding of additional high-scoring applications.
- To be sure no project receives overlapping funding from multiple agencies, program officers from NIH, TSCRCP, and the TSC Alliance participate on program panels and develop funding strategies together.

What is the impact of the TSCRCP and what are some potential benefits?

- TSCRCP-supported research examined the role TSC genes play in cell growth and proliferation—specifically in controlling the mechanistic Target of Rapamycin (mTOR) signaling pathway in cells. This research rapidly led to clinical trials, resulting in the first drug approved by the FDA specifically for treatment of individuals with TSC in 2010.
- In 2022, the first rapamycin topical gel was FDA-approved for treatment of facial angiofibromas in TSC. TSCRCP funding in FY10 funded a clinical trial of topical rapamycin, which demonstrated effectiveness of this approach.
- An FY18 award to Aeovian enabled development of novel, highly mTORC1-selective Inhibitors for the treatment of epilepsy associated with TSC. Its candidate drug should enter Phase 1 clinical trials this year.
- Two TSCRCP awards in FY12 and FY15 enabled generation of a potential approach for gene therapy of TSC, which has shown promising results in a mouse model of TSC

Hope no matter how complex

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tumors in the brain. These awards led to gene therapy companies entering the TSC-related epilepsy space.

- Dr. Steven Sparagana at Texas Scottish Rite Hospital for Children received an FY05 TSCRP award to initiate the TSC Natural History Database (NHD) with the TSC Alliance. The NHD is still operating and growing with now more than 22 participating clinics. NHD data is also linked to biosamples collected in the Biosample Repository (BSR), which allows TSC researchers to link clinical data in the NHD to the analysis of blood, DNA, or TSC-affected tissue. As of February 2024, the NHD has 2,600-plus participants world-wide with more than 2,000 unique biosamples collected.
- Dr. Gina Lee received an award in FY20, and she has started her own TSC-focused lab at UC Irvine and continues to mentor new TSC researchers. Her lab's dedication to TSC and tumor signaling has led to important advances in understanding how tumor cells communicate.

Recent awards with potential for longer-term impact

- One FY23 award was to measure the risk and impact of lung and kidney complications in women with TSC of child-bearing age, and the impact of pregnancy. The occurrence of lung and kidney issues during pregnancy have been observed, but no quantitative data exists to guide healthcare at this critical point for mother and baby. This is one of the first steps to improve safe pregnancy recommendations for those with TSC/LAM.
- Another FY23 award was to understand the impact of caregiver wellbeing on behavioral and other neuropsychiatric issues in those with TSC for whom they are caring.

What is the relevance of TSC research to the military?

- Epilepsy research is important to the US military to better understand the link between traumatic brain injury (TBI) and epilepsy. There are similarities between seizure activity in TSC and in TBI.
- TSC biology is relevant to common diseases affecting military personnel and their families, including traumatic brain injury; hyperactivity and difficulties focusing; autism spectrum disorder, which affects 1 in 36 children; and diabetes and cancer, which impact many service members every year. Research into TSC heightens overall scientific understanding of TSC and its related disorders.
- TSC research may lead to new interventions for preventing the development of seizures in high-risk military and civilian individuals.

Which Congressional committees address the TSCRP funding request?

- The **Appropriations Committees** make recommendations for discretionary spending appropriations in the federal budget. They set aside funding for discretionary spending in military services, like the Department of Defense, which administers the TSCRP.
- The **Armed Services Committees** are responsible for the oversight and authorization of the Department of Defense (DoD), United States Armed Forces (Army, Navy, Marine Corps, Air Force, and Coast Guard), and certain aspects of the Department of Energy. Together, the House and Senate Armed Services Committees write and propose the National Defense Authorization Act (NDAA) for each fiscal year. Once signed into law, the NDAA for the fiscal year sets the specifics in the budget and authorizes expenditures for the DoD.