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Key Talking Points

Our Ask: Funding for the Tuberous Sclerosis Complex Research Program (TSCRCP) at \$10 Million in FY24 Budget

Why is the TSCRCP important?

- 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 \$8 million
- In FY22, 12 applications scored as Outstanding or Excellent were not funded (totaling \$6.5 million in unfunded research). Increasing the appropriation to \$10 million would enable funding of additional high-scoring applications.

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

Past impact

- TSCRCP-supported research examined the role TSC genes play in cell growth and proliferation—specifically in controlling the mammalian 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.
- Effectiveness of a behavioral intervention strategy, JASPER, to improve outcomes in children with autism is being tested in a large, NIH-funded clinical trial. This breakthrough trial would not be possible without data obtained from an FY10 TSCRCP clinical research award to define early autism predictors in TSC and an FY14 TSCRCP award for a pilot clinical trial.
- 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 tumors in the brain.

Hope no matter how complex

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Possible near-term impact

- An award made with FY19 funding for a pilot clinical trial called “TSC Remote Assessment and Intervention (TRAIN)” aims to demonstrate the equivalent efficacy of telehealth-delivered training for behavioral interventions to greatly decrease the need for in-person visits by patients and caregivers.
- Two FY22 awards address near-term needs of the TSC community—one to develop a point-of-care biosensor platform to monitor therapeutic drugs in the blood, and another to understand the nature of emotion dysregulations and optimize a behavioral intervention to treat emotion dysregulations in TSC.

Possible longer-term impact

- One FY22 award was to develop a novel gene therapy using extracellular vesicles as a platform to deliver the TSC1 protein into cells.
- Another FY22 award was to develop a T-cell based therapy to treat TSC.

What’s the relevance 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 more common diseases affecting military personnel and their families, including autism spectrum disorder, which affects 1 in 54 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.

Why is it important to fund the TSCRP?

- Grants awarded by the TSCRP provide an opportunity for researchers to get projects started and be competitive down the line when they scale projects up for NIH funding opportunities.
- The research funding fills a gap between the private funding awarded by the TSC Alliance (competitive grants have been given for more than 45 years, totaling more than \$34 million in funding) and larger awards from the NIH.
- To be sure the awards are not duplicative, program officers from NIH, TSCRP, and the TSC Alliance participate on respective program panels and deliberate strategies together.

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.