

**Tuberous Sclerosis Alliance**

**Mission: To find a cure for TSC while improving the lives of those affected.** *Approved by the TS Alliance Board of Directors: December 12, 2018*

From Where (Referral Sources)	For Whom (Populations, <i>listed in order of life stage</i> )	Assumptions (Theory of Change)	Strategies/Activities (Program Interventions)	Leading Indicators	Outcomes	Impact
<p>Google / TS Alliance website</p> <p>Social media sites</p> <p>Community clinicians</p> <p>TSC Clinics</p> <p>International TSC groups</p> <p>CDC, NIH websites</p> <p>Other parents / caregivers</p> <p>Community Alliances (CAs) / walks and other local events</p> <p>Other advocacy organizations</p> <p>Industry apps / outreach</p> <p>Case managers of government benefits</p>	<p><b>1. Parents/caregivers of infants and children (under 18):</b></p> <p>a. Diagnosed with TSC</p> <p>b. Undiagnosed</p> <p><b>2. Young adults with TSC (18-26)</b></p> <p>a. Semi-independent and independent adults diagnosed with TSC and their family members</p> <p>b. Parents/caregivers/family members of dependent adults diagnosed with TSC</p> <p>c. Undiagnosed</p> <p><b>3. Adults (27+)</b></p> <p>a. Semi-independent and independent adults diagnosed with TSC and their family members</p> <p>b. Parents/caregivers/family members of dependent adults diagnosed with TSC</p> <p>c. Undiagnosed</p>	<p><b>Tuberous sclerosis complex (TSC) is a genetic disorder that causes non-malignant tumors to form in different organs, primarily in the brain, eyes, heart, kidney, skin and lungs, and affects approximately 50,000 individuals in the USA and 1 million worldwide. It is the leading genetic cause of epilepsy and autism.</b></p> <p><b>TSC is considered a linchpin disease, meaning that every advance made in our search for answers and a cure may also lead to answers and advances in other more prevalent diseases like epilepsy, autism and cancer.</b></p> <p><b>The Tuberous Sclerosis Alliance (TS Alliance) is dedicated to finding a cure for TSC while improving the lives of those affected.</b></p> <p><b>The TS Alliance believes that by using a multi-pronged approach that includes efforts directed at both research for a cure as well as support and education for the TSC community, it will maximize its impact and improve the lives of those affected with TSC. This includes:</b></p> <ul style="list-style-type: none"> <li><b>Focusing research to better understand the course of the disease and identify new treatments and a cure</b></li> <li><b>Altering the course of the disease through prenatal diagnosis and preventative treatment to improve the quality of life of those with TSC</b></li> <li><b>Assuring access to appropriate medical care and removing barriers to approved treatments</b></li> <li><b>Educating medical professionals to achieve more rapid diagnosis and better treatment aligned with evidence-based standards of care and consensus guidelines</b></li> </ul>	<p><b>Accelerate Research</b></p> <ul style="list-style-type: none"> <li>Collaborate and extend partnerships with other nonprofits, academic institutes and industry to advance TSC research</li> <li>Advocate for Federal research funding</li> <li>Increase Preclinical Consortium productivity by engaging researchers, improving models, adapting to priorities of members</li> <li>Expand capabilities of centers in the Clinical Research Consortium and fund pilot or add-on studies with pathways to outcomes.</li> <li>Increase rate of biosample collection by enrolling individuals anywhere in US</li> <li>Invest research grant funding in postdoctoral trainees and highly novel research ideas</li> <li>Convene targeted workshops on urgent research problems and follow-up with funds</li> </ul> <p><b>Improve Access and Quality of Care</b></p> <ul style="list-style-type: none"> <li>Advocate for state funding for research and/or Centers of Excellence</li> <li>Develop a support hub to navigate access to FDA approved therapies</li> <li>Build evidence for what constitutes high quality care for TSC</li> <li>Expand knowledge of evidence-based, best practices of TSC clinical care among TSC Clinic healthcare professionals and other medical providers involved in the treatment of TSC</li> <li>Expand access to care for people in regions with no TSC Clinic or TSC-knowledgeable healthcare professionals.</li> <li>Improve access and delivery of evidence-based, high quality clinical care for patients with TSC, including comprehensive adult care for inpatient and outpatient services</li> </ul>	<p><b>Accelerate Research</b></p> <ul style="list-style-type: none"> <li>Invest \$56 million in research over the next five years to drive projects, workshops, and collaborations</li> <li>Maintain a minimum of \$6 million annually for TSCRCP</li> <li>Reach 75 mechanisms or combinations tested in Preclinical Consortium with working relationship with 12 industry partners</li> <li>Clinical Research Consortium receives \$30 million federal or industry funding for trials</li> <li>Reach 2500 biosamples with serial blood samples on 250 individuals and 150 tissues, with each genetically tested &amp; 12 use requests annually with clinical data captured in the Natural History Database</li> <li>\$450K paid annually on research grants, 2/3 for postdocs.</li> <li>Annual innovation workshop held with follow-up research funded by innovation fund</li> </ul> <p><b>Improve Access and Quality of Care</b></p> <ul style="list-style-type: none"> <li>Grow state initiatives to four states</li> <li>Increase partnerships with specialty pharmacies, payers, industry, private foundations to support at least 200 individuals/families annually by 2023</li> <li>Form task group of key stakeholders (medical professionals, patients/families) to critically evaluate current TSC Clinic designation standards and recommend new organizational structure and requirements to the TS Alliance Board of Directors for approval and implementation by 2020</li> <li>Conduct 7 studies between 2019-2023 focused on advancing evidence-based standards of care for TSC and/or TSC patient/caregiver-reported outcomes, using a combination of patient survey, existing data review, and prospective study designs</li> <li>Develop bi-annual curriculum (e.g. CME/CNE or TSC workshops at professional association meetings) that exposes TSC Clinic Directors and other health care providers to current evidence-based practices for TSC diagnosis, surveillance, and management <ul style="list-style-type: none"> <li>Participation goal of 136 from TSC Clinics and 80 outside TSC Clinics</li> </ul> </li> <li>Develop a telehealth network "TSCNet" to expand access to TSC quality care by 2023</li> <li>Increase TSC clinics providing services to children and adults, inpatient and outpatient services, to 65% by 2023</li> </ul>	<p><b>Short Term Research Outcomes</b> Within 5 years:</p> <ul style="list-style-type: none"> <li>More clinical trials testing personalized/precision treatments to prevent or eliminate specific TSC manifestations.</li> <li>More distinct compounds or interventions advancing toward clinical trials through the Preclinical Consortium.</li> </ul> <p><b>Long Term Research Outcomes</b> Within 5 to 10 years:</p> <ul style="list-style-type: none"> <li>More clinical trials testing compounds with unique mechanisms of action or gene therapy/editing technologies.</li> <li>Neonatal screening with interventions implemented in clinical use to prevent the onset of irreversible, life-altering outcomes.</li> </ul> <p><b>Short Term Access/Quality of Care Outcomes</b> Within 5 years:</p> <ul style="list-style-type: none"> <li>Increase awareness of updated clinical guidelines.</li> <li>Lay groundwork for evidence-based treatment standards (including use of telehealth) through new studies and advocacy for improved patient and family care experience.</li> </ul> <p><b>Long Term Access/Quality of Care Outcomes</b> Within 5 to 10 years:</p> <ul style="list-style-type: none"> <li>Expand the number of TSC affected children and adults who have access to high quality care.</li> <li>Increase quality of clinical care at TSC Clinics and outside TSC Clinics by better adherence to clinical guidelines.</li> <li>Evidence-based treatment standards established, implemented and adopted by recognized TSC Clinics.</li> </ul>	<p><b>As a result of investing directly in TSC research and in infrastructure to support TSC research, identify treatments that improve long-term clinical outcomes and quality of life of individuals affected by TSC and alter the course of the disease for future generations through prenatal diagnosis and preventative treatments.</b></p> <p><b>As a result of expanding access to quality clinical care for TSC, improve short- and long-term clinical outcomes and quality of life of individuals affected by TSC.</b></p>

		<ul style="list-style-type: none"> <li>• <b>Community building to empower those living with and caring for those with TSC</b></li> </ul>	<p><b>Support and Empower Constituents</b></p> <ul style="list-style-type: none"> <li>• Create a premiere support services volunteer corps through Clinic Ambassadors, Junior Leaders, Dependent Adult Transition Resource Coordinators, Adult Regional Coordinators, Spanish Support Network, Education Parent Mentors and Community Alliance leadership</li> <li>• Expand skills of parents/caregivers and young adults to advocate for and access appropriate educational services to improve quality of life</li> <li>• Grow collaborations with other nonprofit organizations internationally, nationally and locally to ensure access to resources, support services, transition tools and information</li> <li>• Expand reach of education and support through use of technology</li> <li>• Increase scientific knowledge in the TSC community</li> <li>• Grow and advance the Global Alliance Program</li> </ul>	<p><b>Support and Empower Constituents</b></p> <ul style="list-style-type: none"> <li>• Grow peer to peer support from 20,246 (2017) to 35,867 (10% annually) <ul style="list-style-type: none"> <li>▪ Recruit 225 new volunteers into Support Services programs by 2023</li> <li>▪ Provide 600 hours of volunteer training per year</li> </ul> </li> <li>• Increase the Education Parent Mentor program to support school meetings from 74 to 475 by 2023</li> <li>• Build and maintain working partnerships with 15 national organizations/collaboratives (ARC, Bcureful, CNF, CTF, EF/REN, LAM Foundation, ATS-PAR, ELC, Global Genes, ISAN, NORD, RDCRN, TSCi)</li> <li>• Establish relationships between all Community Alliances and local Arc Chapters where existing by 2023 utilizing Associate Partnership with The Arc US</li> <li>• Utilize technology or support services app to increase participation by 25% from 2,029 (Community Alliance meetings) attendees (2017) through live virtual meetings monthly in three major time zones, and create a podcast library by 2023</li> <li>• Implement TSC Academy by 2020 with 100% of sponsored March on Capitol Hill advocates completing required units for certificate by 2021 (avg score of 80% on post-test for required advocacy units/70% for required science units). Increase # of units accessed by 100% per year between 2020-2023</li> <li>• Expand to 10 Global Alliances by 2023, growing each countries' constituencies by 15%, increase global TSC Clinics to 18, and develop country-specific strategic plans</li> </ul>	<p><b>Short Term Support Outcomes</b> Within 5 years:</p> <ul style="list-style-type: none"> <li>• Increase in training for volunteers and targeted peer support for TSC-affected individuals and their families / caregivers.</li> <li>• Increase engagement in educational advocacy and improve tailoring of educational services to needs of individuals with TSC.</li> <li>• Expand transition resources for parents and young adults.</li> </ul> <p><b>Long Term Support Outcomes</b> Within 5 to 10 years:</p> <ul style="list-style-type: none"> <li>• Increase knowledge of procedures of self-advocacy in clinical and educational settings.</li> <li>• Increase self-reported social support experienced by individuals and families affected by TSC.</li> <li>• Expand scientific knowledge among the global TSC community to increase access to government supported research and medical care.</li> </ul>	<p><b>As a result of providing educational, peer and social support, improve the cognitive outcomes and quality of life of individuals affected by TSC and their family members.</b></p>
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