

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>1. Parents/caregivers of infants and children (under 18):</p> <p>a. Diagnosed with TSC</p> <p>b. Undiagnosed</p> <p>2. Young adults with TSC (18-26)</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>3. Adults (27+)</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>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.</p> <p>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.</p> <p>The Tuberous Sclerosis Alliance (TS Alliance) is dedicated to finding a cure for TSC while improving the lives of those affected.</p> <p>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:</p> <ul style="list-style-type: none"> Focusing research to better understand the course of the disease and identify new treatments and a cure Altering the course of the disease through prenatal diagnosis and preventative treatment to improve the quality of life of those with TSC Assuring access to appropriate medical care and removing barriers to approved treatments Educating medical professionals to achieve more rapid diagnosis and better treatment aligned with evidence-based standards of care and consensus guidelines 	<p>Accelerate Research</p> <ul style="list-style-type: none"> Collaborate and extend partnerships with other nonprofits, academic institutes and industry to advance TSC research Advocate for Federal research funding Increase Preclinical Consortium productivity by engaging researchers, improving models, adapting to priorities of members Expand capabilities of centers in the Clinical Research Consortium and fund pilot or add-on studies with pathways to outcomes. Increase rate of biosample collection by enrolling individuals anywhere in US Invest research grant funding in postdoctoral trainees and highly novel research ideas Convene targeted workshops on urgent research problems and follow-up with funds <p>Improve Access and Quality of Care</p> <ul style="list-style-type: none"> Advocate for state funding for research and/or Centers of Excellence Develop a support hub to navigate access to FDA approved therapies Build evidence for what constitutes high quality care for TSC 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 Expand access to care for people in regions with no TSC Clinic or TSC-knowledgeable healthcare professionals. Improve access and delivery of evidence-based, high quality clinical care for patients with TSC, including comprehensive adult care for inpatient and outpatient services 	<p>Accelerate Research</p> <ul style="list-style-type: none"> Invest \$56 million in research over the next five years to drive projects, workshops, and collaborations Maintain a minimum of \$6 million annually for TSCRCP Reach 75 mechanisms or combinations tested in Preclinical Consortium with working relationship with 12 industry partners Clinical Research Consortium receives \$30 million federal or industry funding for trials Reach 2500 biosamples with serial blood samples on 250 individuals and 150 tissues, with each genetically tested & 12 use requests annually with clinical data captured in the Natural History Database \$450K paid annually on research grants, 2/3 for postdocs. Annual innovation workshop held with follow-up research funded by innovation fund <p>Improve Access and Quality of Care</p> <ul style="list-style-type: none"> Grow state initiatives to four states Increase partnerships with specialty pharmacies, payers, industry, private foundations to support at least 200 individuals/families annually by 2023 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 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 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"> Participation goal of 136 from TSC Clinics and 80 outside TSC Clinics Develop a telehealth network "TSCNet" to expand access to TSC quality care by 2023 Increase TSC clinics providing services to children and adults, inpatient and outpatient services, to 65% by 2023 	<p>Short Term Research Outcomes Within 5 years:</p> <ul style="list-style-type: none"> More clinical trials testing personalized/precision treatments to prevent or eliminate specific TSC manifestations. More distinct compounds or interventions advancing toward clinical trials through the Preclinical Consortium. <p>Long Term Research Outcomes Within 5 to 10 years:</p> <ul style="list-style-type: none"> More clinical trials testing compounds with unique mechanisms of action or gene therapy/editing technologies. Neonatal screening with interventions implemented in clinical use to prevent the onset of irreversible, life-altering outcomes. <p>Short Term Access/Quality of Care Outcomes Within 5 years:</p> <ul style="list-style-type: none"> Increase awareness of updated clinical guidelines. Lay groundwork for evidence-based treatment standards (including use of telehealth) through new studies and advocacy for improved patient and family care experience. <p>Long Term Access/Quality of Care Outcomes Within 5 to 10 years:</p> <ul style="list-style-type: none"> Expand the number of TSC affected children and adults who have access to high quality care. Increase quality of clinical care at TSC Clinics and outside TSC Clinics by better adherence to clinical guidelines. Evidence-based treatment standards established, implemented and adopted by recognized TSC Clinics. 	<p>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.</p> <p>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.</p>

		<ul style="list-style-type: none"> • Community building to empower those living with and caring for those with TSC 	<p>Support and Empower Constituents</p> <ul style="list-style-type: none"> • 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 • Expand skills of parents/caregivers and young adults to advocate for and access appropriate educational services to improve quality of life • Grow collaborations with other nonprofit organizations internationally, nationally and locally to ensure access to resources, support services, transition tools and information • Expand reach of education and support through use of technology • Increase scientific knowledge in the TSC community • Grow and advance the Global Alliance Program 	<p>Support and Empower Constituents</p> <ul style="list-style-type: none"> • Grow peer to peer support from 20,246 (2017) to 35,867 (10% annually) <ul style="list-style-type: none"> ▪ Recruit 225 new volunteers into Support Services programs by 2023 ▪ Provide 600 hours of volunteer training per year • Increase the Education Parent Mentor program to support school meetings from 74 to 475 by 2023 • 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) • Establish relationships between all Community Alliances and local Arc Chapters where existing by 2023 utilizing Associate Partnership with The Arc US • 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 • 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 • 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 	<p>Short Term Support Outcomes Within 5 years:</p> <ul style="list-style-type: none"> • Increase in training for volunteers and targeted peer support for TSC-affected individuals and their families / caregivers. • Increase engagement in educational advocacy and improve tailoring of educational services to needs of individuals with TSC. • Expand transition resources for parents and young adults. <p>Long Term Support Outcomes Within 5 to 10 years:</p> <ul style="list-style-type: none"> • Increase knowledge of procedures of self-advocacy in clinical and educational settings. • Increase self-reported social support experienced by individuals and families affected by TSC. • Expand scientific knowledge among the global TSC community to increase access to government supported research and medical care. 	<p>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.</p>
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