

From Where (Referral Sources)	For Whom (Populations, listed in order of life stage)	Assumptions (Theory of Change)	Strategies/Activities (Program Interventions)	Leading Indicators	Outcomes	Impact
<p>Google / TSC 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 TSC Alliance is dedicated to finding a cure for TSC while improving the lives of those affected.</p> <p>The TSC 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 <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 \$17.8 million in research over the next five years to drive projects, workshops, and collaborations • Maintain a minimum of \$8 million annually for TSCRP • Reach 75 IND-enabling studies executed by Preclinical Consortium with 21 cumulative company members • Clinical Research Consortium receives \$30 million federal or industry funding for trials • Reach 2500 biosamples with serial blood samples on 250 individuals with each genetically tested & 12 use requests annually with clinical data captured in the Natural History Database • \$450K paid annually on research grants to early stage investigators <p>Improve Access and Quality of Care</p> <ul style="list-style-type: none"> • Grow state initiatives to three states • Increase partnerships with specialty pharmacies, payers, industry, private foundations and through TSC Navigator to decrease the number of individuals/ families in medication access crisis from 76 (2021 baseline) to 45 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 TSC Alliance Board of Directors for approval and implementation by 2020 • Conduct 3 studies between 2021-2023 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 11500 from online CME • Identify TSC specialists to be resources for the 27 largely rural states participating in the interstate medical licensure compact • 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 to 10,578 by 2023 <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 150 by 2023 • Build and maintain working partnerships with 15 national organizations/collaboratives (ARC, CNF, CTF, EF/REN, LAM Foundation, ATS-PAR, ELC, Global Genes, ISAN, NORD, RDCRN, TSCi) • 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 75% of sponsored March on Capitol Hill advocates engaging with course content. 100% of new, sponsored advocate engagement 2021-2023. Increase # of courses available for learning by 100% and number of courses accessed by 50% between 2020-2023 • Expand to 7 Global Alliances by 2023, growing each countries' constituencies by 10%, increase global TSC Clinics to 15, and launch NHD internationally 	<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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