Objective

The TSC Alliance improves quality of life for everyone affected by tuberous sclerosis complex by catalyzing new treatments, driving research toward a cure and expanding access to lifelong support. ***Approved by TSC Alliance Board of Directors: October 13, 2023. Revisions approved by Board of Directors: March 6, 2025.***

Goals

Accelerate Research

Strategies

Better understand and stratify disease:

- Enable researchers to access and utilize the wide variety of clinical, omics and Patient Reported Outcomes (PRO) data collected by or shared with TSC Alliance.
- Increase the number and diversity of individuals with TSC participating in research.
- Expand quantitative clinical data collected in the Natural History Database (NHD) / Biosample Repository (BSR).
- Develop prognostic, predictive and stratification biomarkers.
- Drive development of quantitative patient-reported outcome measures for impactful aspects of TSC.

Increase diversity of technologies entering clinical trials:

- Cultivate and engage industry partners to use the Preclinical and Clinical Research Consortia.
- Enhance collaboration with academic researchers by raising funds to pay for preclinical testing of nominated compounds or other technologies (e.g., gene therapy).
- Establish a TSC Clinical Research Network, synergizing with the existing Clinical Research Consortium, TSC Clinics and COEs with membership model for industry participation and revenue generation.

Increase innovative research:

- Advocate for federal and state funding for research.
- Expand racial and ethnic diversity of research grant applicants.
- Demonstrate impact of TSC Alliance funding.

Measures

Better understand and stratify disease:

- Implement a centralized data library that researchers can access (2025). Provide tools for researchers to work with the library of data by implementing a virtual “sandbox” (2028).
- Increase the number of participants from underrepresented groups (Black or African American, Asian) by adding 5-10 in each group annually
- Collect quantitative data related to TAND and to reproductive and perinatal health outcomes from at least 300 TSC-affected individuals.
- Ensure BSR contains serial blood samples from at least 250 individuals with TSC and access to at least 100 typically-developing, non-TSC individuals and at least 100 individuals with TSC but without neurological signs or symptoms.
- Establish a blood-based data set (e.g., DNA, RNA, protein) from 200 pediatric and 200 adult individuals with TAND and appropriate controls necessary for research efforts to establish a blood-based biomarker panel for manifestations of TAND.
- Actively participate in two external collaborations to pilot newborn screening for TSC, raising awareness among our community, and when appropriate, advocate for inclusion of TSC genetic testing in newborn screening.
- Establish consensus on a Core Outcome Set of assessment tools to standardize measures of TAND manifestations in clinical research and care by 2028.

Increase diversity of technologies entering clinical trials:

- Maintain at least seven industry partners per year participating in the Preclinical or Clinical Consortia.
- Support the testing of at least 20 compounds or other therapeutic technologies in the Preclinical Consortium for industry or academia~~.~~
- Extend participation in Clinical Research Network to all TSC clinics in the U.S., attracting at least two industry or government funded clinical trials to utilize the Clinical Research Network.

Increase innovative research:

- Increase TSCRP funding from $8 million to $10 million annually.
- Obtain state funding for five TSC centers (currently three states funding four-clinics).
- Fund a minimum of three merit-based research grants per year while growing the diversity of applicants to an average of 15% of applicants from underrepresented populations over five years.
- Annually capture and report outcomes from funded grants (e.g., follow-on funding, promotion, publications, patents, etc.).

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Goals

Improve Access and Quality of Care

Strategies

Expand healthcare access:
- Refine and launch a revamped Professional Advisory Board (PAB), composed of clinicians from diverse specialties serving individuals across the age spectrum and ~~including~~ constituent representatives, who respond to and make recommendations to meet the evolving medical needs of individuals and families affected by TSC (e.g., clinical care barriers, clinical trial recruitment/enrollment/equitable representation, changes in health policy).
- Implement solutions for barriers to care (e.g., decrease clinical trial enrollment time).
- Educate the community about which clinics offer telehealth across state lines.

Improve quality and comprehensiveness of healthcare:
- Promote access to high-quality, patient-centered clinical care across the lifespan by promoting adherence to evidence-based standards of care at recognized TSC Clinics, COEs and affiliated providers.
- Integrate behavioral, mental health, social and emotional wellbeing into TSC Clinics, COEs and affiliated providers.

Build evidence base to support updates to best practice guidelines for TSC clinical care:
- Support initiatives to build evidence to address gaps in current surveillance and management guidelines of TSC care (e.g., including reproductive and perinatal health – in collaboration with The LAM Foundation).

Measures

Expand healthcare access:
- Develop framework for the PAB for review and approval by the Board of Directors in 2024.
    - Hold two meetings per year, beginning in 2025, to identify issues impacting access to care and treatments and potential solutions.
- Annually update website to indicate which clinics offer telehealth across state line.

Improve quality and comprehensiveness of healthcare:
- Develop a transition plan template based on input from Clinic Committee by 2025 and require its use by 100% of recognized clinics by 2028.
- In 2024, assess baseline number of TSC Clinics and COEs describing TAND service capabilities for both pediatrics and adults. In 2025 and annually thereafter update website to include which clinics have dedicated subspecialty services for management of TAND within their clinic or which refer out.

Build evidence base to support updates to best practice guidelines for TSC clinical care:
- Collaborate on initiatives to build evidence to address gaps on 2 understudied aspects of TSC.
    - Publish updated consensus guidelines for surveillance and management of TSC for areas previously unaddressed and identified as high priority by the community (e.g., reproductive and perinatal health, SUDEP).

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Goals	**Support and Empower Constituents**
Strategies	**Develop a more collaborative and inclusive volunteer network to mentor the next generation:** • Strengthen our volunteer leadership model to address gaps in resources, support, advocacy and medical access and train our leaders to represent a more inclusive and collaborative community. **Strengthen the relationship between healthcare providers and community members at a local and regional level:** • Establish a stronger collaboration with TSC Clinics, healthcare providers and leaders to further support and provide resources to TSC families. • Improve TSC community support and resources through cultivating and growing ties with new and current partners. **Foster international relationships and development of resources to better serve the worldwide TSC community:** • Grow global expertise as a way to better support the TSC community.
Measures	**Develop a more collaborative and inclusive volunteer network to mentor the next generation:** • Fully implement **14** regions by 2024, with key leadership roles represented in each region by December of 2028. • Develop an intranet platform that facilitates effective communication, training and resource sharing among our volunteer leadership team by 2024 with 90% utilization annually 2025-2028. • Develop a benchmark for peer-to-peer support in 2024 and increase documented meetings and interactions by 10% annually in 2025-2028. • Recruit and train at least one government advocate in each state by 2028. **Strengthen the relationship between healthcare providers and community members at a local and regional level:** • Grow Clinic Ambassador/Medical Liaison program from 20% to **45%** of TSC Clinics by 2028. • Support and educate **90** SC families through the IEP and transition processes annually. • Increase the number of engagements through the TSC Support Navigators by **10%** annually from a baseline of 50 in 2023. • Maintain working partnerships with at least 15 national and international organizations/collaboratives (i.e., ARC, CNF, CTF, F/REN, LAM Foundation, ATS-PAR, ELC, Global Genes, ISAN, NORD, RDCRN, TSCi, Mind the Gap, Got Transitions, TANDem). **Foster international relationships and development of resources to better serve the worldwide TSC community:** • Grow global clinics from 13 to **25** by 2028. • Work with TSCi to increase the number of countries participating in TSC clinical trials by **25%** by 2028. • Foster partnerships in **10** low- or middle-income countries with no association representation by 2028.

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Goals	**Educate and Mobilize to Increase Investment**	**Build and Strengthen Organization**

Strategies	• Launch public phase and complete capital campaign to enable growth of research platform through partnerships with corporations, foundations and individuals.
	• Educate the community and other donors about the role and opportunities associated with Endowment, including estate planning and gift annuities.
	• Increase annual unrestricted revenue:
	  • from community-driven and corporate-sponsored events by leveraging the unique capacity of each region to meet the most pressing organizational needs.
	  • from scientific collaborations to support developing the research infrastructure
	• Broadcast TSC information, TSC community stories and research accomplishments to increase awareness and position the TSC Alliance as a leader in the rare disease space via wide-ranging, strategic communications efforts using internal tools such as news releases, social media outreach and digital platforms.
	• Create targeted strategies to better reach underserved TSC communities and educate key medical providers (e.g., ER, pediatric, family, psychiatry and primary care).
	• To address community information needs, create a social media and digital outreach plan designed to increase website utilization.
		• Adhere to financial management best practices for efficient and effective operations consistent with watchdog organization guidelines while maintaining appropriate infrastructure for organizational growth.
		• Maintain database of current, reliable information of individuals with TSC, their families and TSC Alliance supporters complying with GDPR.
		• Grow and maintain board of directors reflecting the diversity of our constituency and with skill sets needed to realize the strategic plan.
		• Invest in professional development to cultivate the next generation of staff team leadership and drive results.

Measures	• Raise $23,250,000 for research over the next five years to complete the $40 million capital campaign.
	• Grow the Endowment Fund to $7 million by 2028 and realize the maximum allowable annual contribution.
	• Increase annual net revenue from community-driven and corporate-sponsored events to 15% of total revenue.
	• Generate unrestricted revenue equal to 5% of total revenue from Preclinical and Clinical Research Consortia memberships and indirect costs of industry-sponsored studies.
	• Annually obtain a minimum of 900 million news release impressions and at least 55,000 social engagements and increase use of digital platforms (podcasts, video views and eNewsletters) by 10% each year.
	• Add 350 new families or individuals with TSC contacts to the TSC Alliance database annually
	• Obtain 1,500 physician web page visits and 200 email signups each year..
	• In 2024, create a web page visit baseline, then identify pages to target each year and increase targeted page visits 10% annually.
		• Maintain a highest ratings among watchdog organizations and meet or exceed an 78/22 program/supporting expenses ratio.
		• Engage with staff and community leaders to improve accuracy of contact information in the database, utilizing 2024 as a baseline, so there is less than 2% returned mail for appeals, annual reports and special event mailings annually thereafter.
		• Recruit three to six new Board members annually to achieve a board with:
		  • Expertise in financial investment, management and auditing; community outreach through the lifespan; global relations with sensitivity to different countries' needs; basic and translational research or clinical care with a particular expertise in adult care (e.g., nephrology, pulmonology and reproductive health); fundraising strategy; and ability to raise substantial unrestricted donations or for targeted initiatives including research or community.
		  • Grow and maintain diversity with respect to race/ethnicity, disability, age, gender, relationship to TSC, education, sexual orientation and/or geography.
		• Leverage staff core competencies and skill sets to align with mission and key objectives:
		  • Implement Career Roadmap with training and professional development plans created for each employee .
		  • Create transition plan for senior staff retirements.
		  • Maintain 80% retention outside of retirement.
		  • Build and maintain diversity with respect to language, race/ethnicity, gender, disability, sexual orientation.