



FY 2021 Strategic Plan Progress Report and Effectiveness Assessment

Objective

To find a cure for tuberous sclerosis complex (TSC), while improving the lives of those affected.

Goals

1. Accelerate Research

Measures	Recommended Changes to Measurement	Results as of December 31, 2021	Effectiveness Assessment
Invest \$20.4 million in research over the next five years to drive projects, workshops and collaborations	Change to: \$17.8 million assuming \$4.5 million research investment in 2023.	TSC Alliance invested \$3,936,196 into research in 2021.	In 2019-21 we invested a total of \$9.1 million into research and budgeted \$4.2 million in 2022. Because the pandemic impacted fundraising and spending in 2020, we are extending the Research Campaign and are unlikely to raise enough cash to spend \$7.1 million in 2023.
Maintain a minimum of \$6 million annually for TSCRCP	Change to: Maintain a minimum of \$8 million annually for TSCRCP.	Increased to \$8 million in FY21	Exceeding goal thanks to advocacy by community, board, staff team, and consultant

<p>Reach 75 mechanisms or combinations tested in Preclinical Consortium with working relationship with 12 industry partners</p>	<p>Change to: 75 IND-enabling studies executed by the Preclinical Consortium. IND-enabling studies include compound testing, testing of drug combinations, dose-ranging studies, and biomarker studies. Recommend increasing the measurement to reach 21 cumulative Company Members of the Preclinical Consortium.</p>	<p>Reached 57 with 17 industry partners</p>	<p>Three factors decrease likelihood of reaching this goal: (a) more experiments are being run to find the right dose of drugs and gene therapy vectors rather than testing unique mechanisms or combinations; (b) 2022 has the potential to see INDs from two prior-year Preclinical Consortium projects, requiring more internal resources to support clinical translation, which is the result of Preclinical Consortium successes; (c) Research Business Plan is being spread out over an additional year due to COVID.</p>
<p>Clinical Research Consortium receives \$30 million federal or industry funding for trials</p>	<p>No change.</p>	<p>Reached \$39 million awarded, including \$14 million awarded in 2019-2021.</p>	<p>Grants for new clinical trials depend upon generating innovative ideas with strong supporting data. Keeping the goal is achievable but a stretch since no grants are pending early in 2022.</p>
<p>Reach 2,500 biosamples with serial blood samples on 250 individuals, with each genetically tested and 12 use requests annually with clinical data captured in the Natural History Database</p>	<p>No change.</p>	<p>Reached 2000 samples collected at year end, serial blood samples from 142 individuals, and 18 biosample use requests in 2021 compared to 19 requests in 2020 and 5 in 2019.</p>	<p>Collection is going very well, with more than 100 tissues collected, too. The emphasis should shift slightly to impactful utilization of samples. Biosample seed grants have encouraged many requests, and we should look for opportunities to direct funding to utilize and share data from biosamples. Larger, more purposeful studies may be more impactful than many small studies, so 12 requests annually are appropriate.</p>

<p>\$450K paid annually on research grants, 2/3 for postdocs</p>	<p>Change to: \$450K paid annually on research grants to early-stage investigators.</p>	<p>\$450,000 in research grants were <i>awarded</i> to early-stage investigators and \$672,343 was <i>paid</i> toward research grants, including one-time innovation awards.</p>	<p>In 2020, we began limiting applicants to early-stage investigators (those within 10 years of their terminal degree or medical training). A low number of postdocs applied in 2021, and none was selected for funding. Provided all applicants are now early-stage rather than established senior investigators, the division between postdocs and non-postdocs is less important.</p>
<p>Annual innovation workshop held with follow-up research funded by innovation fund</p>	<p>Remove as a strategic goal and retain option as a tactic as needed for addressing specific challenges in research programs as needed.</p>	<p>The pandemic stretched the planned 2020 innovation workshop into a virtual effort concluding in 2021. A specific funding opportunity announcement led to funding two awards totaling \$164,856 for initiating development of TSC newborn screening assays.</p>	<p>The newborn screening workshop was effective at engaging researchers outside the TSC field and achieving the goal of determining a path forward and funding research to begin that path. However, the concept of an “innovation fund” has not resonated with major donors. It may be more effective to raise money for specific research programs, such as biomarker development, new preclinical model implementation, clinical trial design, etc., for which an innovation workshop may be important for the program but not a goal in and of itself.</p>

2. Improve Access and Quality of Care

Measures	Recommended Changes to Measurement	Dec. 31, 2021	Effectiveness Assessment
Grow state initiatives to four states	Change to: Grow state initiatives to three states.	Maintained successful state initiatives in Missouri and Maryland where the TSC Centers received \$750,000 in continued funding.	This strategy increases TSC awareness on the local state level and strengthens the clinic network in those states.
Increase partnerships with specialty pharmacies, payers, industry, private foundation to support at least 200 individuals/families annually by 2023	Change to: Increase partnerships with specialty pharmacies, payers, private foundation and through TSC Navigator to decrease the number of individuals/families in medication access crisis from 76 (2021 baseline) to 45 by 2023.	76 individuals or families were helped with medication access issues in 2021.	The numbers were 20 in 2019 and 37 in 2020, with 76 in 2021. The number of people being assisted is dependent upon people contacting the TSC Alliance, so a positive aspect is people are learning we can help. However, the ultimate goal of programs like TSC Navigator is to <i>decrease</i> the number of people having access issues by helping them navigate the system proactively.
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	No change.	This goal was achieved in 2020. Implementation began in 2021 with collection of baseline metrics from 12 COEs and receipt of renewal applications from 61 clinics, and receipt of new applications from 6 clinics.	Establishing updated standards was achieved on time with input from a variety of key stakeholders over a two-year period. Implementation revealed challenges in reliable, objective collection and reporting of metrics, and some areas for improvement in clarity of the standards. These will be addressed in 2022.

<p>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</p>	<p>Change to: Conduct 3 studies by 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</p>	<p>Two trials are running (PREVeNT, TSC-STEPS) in collaboration and with partial financial support of the TSC Alliance which should result in evidence-based guidelines. PROs for TAND were added in 2021 to the NHD and should form the basis of future studies.</p>	<p>Two factors make it unlikely we would reach 7 studies by 2023. One is the lengthening of the Research Campaign, so we will have less money to invest into this. Also, we anticipated clinic metrics might be useful as a starting point, but that collection was more complicated than expected.</p>
<p>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</p> <ul style="list-style-type: none"> • Participation goal of 136 from TSC Clinics and 80 outside TSC Clinics 	<p>Change participation goal to: Participation goal of 1500 for online CME.</p>	<p>CME for updated clinical consensus guidelines launched in April 2021 in collaboration with PeerView Institute. By year-end the activity had 2,151 learners with 613 completers. PeerView has no way to tell us who participated from TSC Clinics.</p>	<p>Every other year seems to be a good cadence to generate sufficient participation. Goals for participation should vary depending on the format of the activity, as we know online CME draws more than in-person events. With online events hosted by other organizations, tracking whether attendees are TSC Clinic members is not feasible.</p> <p>Recommend adapting participation goals to format. Online participation in our last two CMEs has been 1600 (so far) to 1900, so although we always like to shoot high, 1500 would be a reasonable goal for a good participation rate; 2000 would be a stretch goal for exceptional participation. In-person participation goals will vary depend upon where they are held, the topic, and</p>

			whether we do it alone or with several partners.
Develop a telehealth network "TSCNet" to expand access to TSC quality care by 2023	Change to: Identify TSC specialists to be resources for the 27 largely rural states participating in the interstate medical licensure compact.	No activity given that the pandemic revolutionized the practice of telehealth.	<p>As reported in 2020, 92% of TSC Clinics responding to our survey provided telehealth to existing patients and 70% to new patients.</p> <p>The corresponding strategy for this goal is "Expand access to care for people in regions with no TSC Clinic or TSC-knowledgeable healthcare professionals." Also, we need to advocate for legal and regulatory changes to reduce barriers to telehealth.</p>
Increase TSC clinics providing services to children and adults, inpatient and outpatient services, to 65% by 2023	No change.	60% as of December 31.	The goal is to improve transition of care. Other ways to attempt to accomplish this goal is developing a hybrid model in which different pediatric and adult institutions work seamlessly for transition of care, cultivating new clinics at institutions which treat all ages, or developing programs to facilitate transition of care to nearby adult TSC specialists.

3. Support and Empower Constituents

Measures	Recommended Changes to Measurement	Dec. 31, 2021	Effectiveness Assessment
<p>Grow peer to peer support from 20,246 (2017) to 35,867 (10% annually)</p> <ul style="list-style-type: none"> Recruit 225 new volunteers into Support Services programs by 2023 Provide 1,200 hours of volunteer training per year 	<p>Change to: Grow peer to peer support to 10,578 by 2023</p> <ul style="list-style-type: none"> Recruit 225 new volunteers into support services by 2023 Provide 600 hours of volunteer training hours per year 	<p>In 2021, Community Programs refined how they counted peer to peer supports and used the year as a new baseline. In total, there were 8,742 peer supports.</p> <ul style="list-style-type: none"> 349 total volunteers recruited; 12 new community leaders were recruited 511 training hours in 2021 	<p>Peer supports were redefined at the beginning of 2021 for clarity in how these were counted. We increased reporting by volunteers and feel we are capturing more accurate information. Therefore, we want to adjust the goal to utilize 2021 as a baseline and growing by 10% each of the next two years. Also, we are reducing training hours back to 600 due to the challenges caused by the pandemic in engaging in face-to-face training.</p>
<p>Increase the Education Parent Mentor program to support school meetings from 74 to 475 by 2023</p>	<p>Change to: Increase the Education Parent Mentor program to support school meetings from 74 to 150 by 2023.</p>	<p>Held 55 school meetings in 2021.</p>	<p>The pandemic added many challenges in how we advocated and interacted with the school districts. The end of the year we did see a big uptick as students went back to in person learning. We also reviewed and revised what we count as a school meeting.</p>
<p>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)</p>	<p>No change.</p>	<p>Maintained 17 partnerships in 2021</p>	<p>We have been able to amplify our voice, raise greater awareness, advocate for public policy and organize conferences in collaboration with other organizations. This has been highly effective.</p>

<p>Establish relationships between all Community Alliances and local Arc Chapters where existing by 2023 utilizing Associate Partnership with The Arc US</p>	<p>Remove this measurement.</p>	<p>Local ARC Chapters were greatly impacted by the pandemic, many closed and have not been responsive to partnerships on a local level.</p>	<p>No longer effective. We completed our agreement as an Associate Member on December 31, 2021, with the national ARC and found it very difficult to get responses from local ARC chapters, especially during the pandemic. This Associate Member agreement was very effective from 2016-2020 when we had a need to stay on top of national policy changes impacting social services.</p>
<p>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</p>	<p>No change</p>	<p>440 attended peer support meetings. Additionally, we conducted 30 webinars for the community with total live audience of 1,642 and total cumulative recording views is 6,636. 9 community leadership webinars were also conducted.</p>	<p>The Community Programs team has effectively been able to adapt to all virtual meetings utilizing technology. The pandemic has created a new way for engaging and interacting with the community from the comfort of their homes.</p>
<p>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.</p>	<p>No change.</p>	<p>135 participants attended the first Virtual March on Capitol Hill. 100% of new advocates took the TSC Academy courses required/requested for MOTH participants.</p>	<p>Content for the TSC Academy course on Clinical Trials was created and laid out. The Consensus Guidelines were delayed in completion and publication and therefore the production of both of these courses moved to 2022 when the organization had more bandwidth.</p>
<p>Expand to 10 Global Alliances by 2023, growing each countries' constituencies by 15%, increase</p>	<p>Change to: Expand to 7 Global Alliances by 2023, growing each countries' constituencies</p>	<p>Currently working with the Philippines to add them as a Global Alliance and implemented succession plan in Thailand to</p>	<p>The pandemic has impacted our global partners, their ability to meet in person and their capacity. We</p>

global TSC Clinics to 18, and develop country-specific strategic plans	by 10%, increase global TSC Clinics to 15, and successfully launch NHD internationally.	strengthen Southeast Asia outreach. Participated in Rare Disease International and the development of a Collaborative Global Network for Rare Diseases. Developed implementation plan for expanding NHD globally.	recognize this has been a stressor and have worked to adapt to address the needs in each country.
--	---	---	---

4. Educate and Mobilize to Increase Investment

Measures	Recommended Changes to Measurement	Dec. 31, 2021	Effectiveness Assessment
Raise \$29.26 million for research over the next five years	Change to: Raise \$18.95 million for research over the next five years.	\$8,212,256 raised through 2021.	The TSC Alliance was impacted by the pandemic, particularly in 2020, and our ability to aggressively increase fundraising for the capital campaign. We in effect lost one year and will be adding an additional year to the seven-year campaign.
Grow the Endowment Fund to \$10 million by 2023 and realize the maximum allowable annual contribution	No change.	\$6,711,748 in total net assets of the Endowment Fund and an annual contribution of \$226,000 was made to the TSC Alliance.	There was a \$100,000 gift annuity to the Endowment Fund in 2021 and we continue to cultivate donors from those who expressed interest from the campaign survey. We also continue to market gifts and bequests to the Endowment Fund in Perspective and at major events.
Mobilize grassroots community through budgeted special events to raise \$1.5 million average net annually over next 5 years	No change.	\$1,143,605 net in community fundraising events in 2021.	The pandemic has changed the way we fundraise and engage our community and made us much more efficient. For instance, instead of 37 separate walks, there is now one

			Walk Run Ride weekend with everyone focused on the same goal and coming together to increase awareness of TSC. It's also held near TSC Global Day.
Increase the number of people donating online to crowdfunded events from 1,257 (2017) to 2,514 (2023)	No change.	7,519 transactions raising \$912,859.	Crowdfunding has become the go to in fundraising during the pandemic while live events are on hold. The number of transactions increased dramatically in 2021. We will assess how returning to a combination of hybrid and live events may impact crowdfunding..
Secure at least 200 million impressions annually via multiple and varied outlets with minimum investment	Change to: Increase annual impressions to at least 400 million.	Cumulative impressions in 2021 totaled 809 million.	We had a very high response to our 2021 press releases and announcements and would therefore recommend increasing the baseline.
Achieve additional 30 million impressions through new awareness campaign and 3,500 contacts to the database per year of the campaign	Change to: Increase additional awareness campaign impressions to at least 100 million.	The 2021 awareness campaign implemented by Geben Communication secured 24 media placements (6 podcasts, 4 national outlets, 9 trade publications and 5 local outlets) leading to 179.3 million impressions and 5,200+ social media engagements.	Due to the success of Geben Communication efforts in 2021, we recommend increasing the impression baseline for this category. We still need to determine the database's baseline number(s) or remove this particular measurement altogether.

5. Build and Strengthen Organization

Measures	Recommended Changes to Measurement	Dec. 31, 2021	Effectiveness Assessment
<p>Maintain a 4-star Charity Navigator rating and meet or exceed an 80/20 program/supporting expenses ratios by 2022 in accordance with the five-year Research Business Plan</p>	<p>Change to: Maintain a 4-star Charity Navigator rating and meet or exceed an 80/20 program/supporting expenses ratios by 2023</p>	<p>The TSC Alliance will qualify to return to a 4-star rating on Charity Navigator for FY2021. Currently rated as a Platinum Charity by Guidestar, 2021 Top-Nonprofit GreatNonprofits and an accredited charity by the Better Business Bureau Wise-Giving Program.</p> <p>Current program ratios are: 79.17% (\$5,465,789) of expenses going toward programs, and 20.83% for supporting services (\$919,902 for fundraising and \$517,901 for management and admin). The \$919,902 represents 11.18% of total revenues raised.</p>	<p>Due to the pandemic, the TSC Alliance made business decisions to retain cash in order to preserve our organization. As a result, we slowed and reduced some program spending in 2020 which led to a 3-star Charity Navigator rating. In 2021, we had tremendous success in raising and spending funds, particularly for research which will have a positive impact. In 2022, we are growing staff to meet our programmatic growth which had been put on hold the prior two years. Our 2022 budget plans for 78.5/21.5 program/supporting ratios. We are running about a year behind on the Research Business Plan and will be revising by adding a year to 2026.</p>
<p>Implement in depth review to ensure integrity of constituent data with less than 1% duplicates by 2020 and maintain thereafter</p>	<p>No change.</p>	<p>The database's current constituent total is 154,259 with 321 suspected duplicate records, putting the total percentage of duplicate records well under 1%.</p>	<p>We are on target for this measurement and continue to monitor it annually.</p>
<p>Participate/present at 25 professional/industry conferences per year</p>	<p>No change.</p>	<p>Participated in 37 professional/industry conferences in 2021</p>	<p>This is highly effective. Since we have grown our staff, finding the balance between external commitments and completing</p>

			internal goals has improved greatly, especially when almost all meetings were held virtually. This has allowed us to build our reputation as a leader in the rare disease space while learning from others' experiences.
Recruit 3-6 new Board members annually, reflecting the diversity of our constituency, with financial management, global relations, technology, scientific/medical expertise and ability to raise substantial unrestricted donations or for targeted initiatives including research or community initiatives	No change.	<p>Added 6 new board members with expertise in regulatory affairs, executive pharmaceutical leadership, commercial legal and litigation, government relations, global marketing and patient advocacy, and finance and audit.</p> <ul style="list-style-type: none"> • 4 are women and 2 are men • 2 from diverse backgrounds • Geographically: 2 from DC, 3 from CA, 1 from Chicago <p>3 of 6 are parents of individuals with TSC. 3 are from corporate partners.</p>	Recruitment of members with identified skill sets was a priority along with those who represented the diversity of our community.
Grow Science and Medical team to 8.5 FTEs to accelerate research and improve quality of care by 2021 and corresponding staff (e.g., financial, fundraising, communications, support services) and infrastructure to support growth while maintaining 80% retention outside of retirements	No change.	<p>6 FTEs in Science and Medical Staff in 2021. 3 additional positions added: consultant Grant Writer to Development to assist with the research campaign; Executive Assistant to support CEO and executive team; and Director of Community Education and Resources to supplement Community Programs (Shelly Meitzler promoted to this position in November).</p> <p>Overall staff retention at 95% outside of retirements.</p>	The TSC Alliance continues to emphasize the importance of staff retention, succession and transition planning. A Sr. Community Programs Manager was hired at the end of FY21. For FY22, adding a Director of Annual Giving and Partnerships to Development and hiring a new Community Programs Manager in first quarter.

<p>Create transition plan for senior staff retirements</p>	<p>No change.</p>	<p>The transition plan for VP Support Services was executed in early 2021. A transition plan for the CFO position was created.</p>	<p>In 2021, the Vice President of Support Services (Dena Hook) left one year earlier than anticipated and the transition plan that was put in place in 2020 was expedited. A transition plan for the CFO position was created, a new CFO hired in December and an overlap of three months will allow for adequate training to take place in 2022.</p>
--	-------------------	--	---