



FY 2020 STRATEGIC PLAN PROGRESS REPORT

OBJECTIVE

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

GOALS

1. Accelerate Research

Measures	Dec. 31, 2019	Dec. 31, 2020
Invest \$56 million in research over the next five years to drive projects, workshops, and collaborations	Goal changed to \$40 million over seven years following a campaign study and on recommendation of our capital campaign consultants. TS Alliance spent \$2,590,463 into research in 2019.	TS Alliance invested \$2,594.361 into research in 2020.
Maintain a minimum of \$6 million annually for TSCRCP	\$6 million was appropriated for the TSCRCP for FY19 and FY20.	<ul style="list-style-type: none"> \$8 million was appropriated for the TSCRCP in FY21. The CSO chairs the Programmatic Panel of the Tuberous Sclerosis Complex Research Program administered by the DOD.
Reach 75 mechanisms or combinations tested in Preclinical Consortium with working relationship with 12 industry partners	<ul style="list-style-type: none"> 35 mechanisms or combinations have been tested. 5 companies remain active members at the end of 2019. 2 companies moving into clinical trials from resources utilized from Preclinical Consortium and Biosample Repository. 	<ul style="list-style-type: none"> 44 compounds or combinations have been tested 7 companies remain active members at the end of 2019 – a total of 13 have been members since 2016 Marinus launched a Phase 2 trial in epilepsy in TSC. COVID-19 delayed launch of the second clinical trial we had anticipated to begin in 2020.

<p>Clinical Research Consortium receives \$30 million federal or industry funding for trials</p>	<p>\$7.5 million awarded to the Clinical Research Consortium for a 5-year renewal of the RDCRN grant from NIH.</p>	<ul style="list-style-type: none"> • The consortium did not receive new funding in 2020 but submitted an application to FDA to support the STOP-2 trial. • The CSO completed a four-year term on the Advisory Council for the National Institute of Neurological Disorders and Stroke.
<p>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</p>	<ul style="list-style-type: none"> • 967 biosamples • Serial blood samples on 36 individuals • 10 tissues • 12 NHD data requests, including 5 for biosamples + NHD data 	<ul style="list-style-type: none"> • 1,571 biosamples • Serial blood samples on 75 individuals • 19 tissues • Received 8 NHD data requests plus 19 additional biosample requests.
<p>\$450K paid annually on research grants, 2/3 for postdocs</p>	<ul style="list-style-type: none"> • \$314,914 paid on research grants, 2/3 of new grants awarded to postdocs • \$40,000 paid on biosample use seed grants • \$50,000 paid on Clinical Research Consortium grant • \$404,914 total 	<ul style="list-style-type: none"> • \$408,212 paid on research grants, 57% to postdocs and 7% to early-stage investigators. This proportion will increase now that we limited grants to early-stage investigators in 2020 and 2021. • \$57,842 paid on biosample use seed grants. • \$50,000 paid on Clinical Research Consortium grant • \$516,054 total
<p>Annual innovation workshop held with follow-up research funded by innovation fund</p>	<p>Biennial International TSC Research Conference held in 2019. Plan developed for first innovation workshop to be on newborn screening in June 2020.</p>	<p>Innovation Workshop on newborn screening held Nov 18 – Dec 11 using Zoom, recorded presentations, and Povernoodle. Wrap-up web meeting will be held January 29, 2021.</p>

2. Improve Access and Quality of Care

Measures	Dec. 31, 2019	Dec. 31, 2020
Grow state initiatives to four states	Active state advocacy initiatives in 2 state with secured appropriations of \$250,000 in Missouri and \$500,000 in Maryland.	State initiatives in Missouri and Maryland continued with \$250,000 in appropriations for Missouri and \$500,000 for Maryland.
Increase partnerships with specialty pharmacies, payers, industry, private foundation to support at least 200 individuals/families annually by 2023	<ul style="list-style-type: none"> • 20 individuals/families received assistance in accessing FDA approved medications. Added partnership with a major speciality pharmacy. • Ongoing relationships with 12 industry partners and four specialty pharmacy partners 	<ul style="list-style-type: none"> • 37 individuals/families received assistance in accessing FDA approved medications. • Ongoing relationships with 19 industry partners and four specialty pharmacy partners.
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	TSC Focus Group established draft guidelines for the “standards of care” part of new clinic guidelines and will present full recommendations to the Board in 2020.	Board approved on Dec 14 new TSC Clinic Guidelines and Scope of Relationship Policy, Standards of Care Metrics, Structure and Expertise Requirements for Recognition/Designation, Clinic Committee Charter, and Patient and Family Experience of Care Survey.
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	TSC Focus Group standards of care will include measurements of outcomes, but this will begin in 2021.	Gathering of baseline TSC Clinic and Center of Excellence standards of care metrics will occur in 2021.
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 evidencebased practices for TSC diagnosis, surveillance, and management	200 child neurologists participated in the Harmful and Disruptive Behaviors CME Symposium at CNS, developed by the Child Neurology Foundation with the TS Alliance as lead advocacy partner and assisting in curriculum development.	Obtained funding for and began development of CME for 2021 on clinical consensus guidelines.

<ul style="list-style-type: none"> Participation goal of 136 from TSC Clinics and 80 outside TSC Clinics 		
<p>Develop a telehealth network “TSCNet” to expand access to TSC quality care by 2023</p>	<p>Not in 2019 goals.</p>	<p>Not in 2020 goals. Impact of COVID-19 led to vastly increased telehealth, as demonstrated in a TS Alliance survey:</p> <p>92% of 37 US TSC Clinics* are providing telehealth to existing patients in state/65% out-of-state</p> <p>70% of 37 US TSC Clinics* are providing telehealth to new patients in state/49% out-of-state</p> <p>* There are 65 US TSC Clinics. 37 TSC Clinics (57% response rate) completed the questionnaire by 2/16/2021.</p>
<p>Increase TSC clinics providing services to children and adults, inpatient and outpatient services, to 65% by 2023</p>	<p>54.2% (39 of 72) TSC designated clinics see individuals of all ages.</p>	<p>53.3% (40 of 75) TSC Clinics and COEs see individuals of all ages. The percentage is 76.9% (10 of 13) for COEs alone.</p>

3. Support and Empower Constituents

Measures	Dec. 31, 2019	Dec. 31, 2020
<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 600 hours of volunteer training per year 	<p>32,932 peer to peer support contacts in 2019.</p> <ul style="list-style-type: none"> 60 new volunteers added to Support Services program and 117 new volunteers overall 1,429 volunteer hours of training provided 	<p>28,447 peer to peer support contacts in 2020.</p> <ul style="list-style-type: none"> 49 new volunteers added to Support Services program with 85 new volunteers overall. 2,634 volunteers hours of training provided

<p>Increase the Education Parent Mentor program to support school meetings from 74 to 475 by 2023</p>	<p>128 school meeting supports provided.</p>	<p>115 school meeting supports provided.</p>
<p>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)</p>	<p>Built and maintained working partnership with 14 national organizations/collaborations</p> <ul style="list-style-type: none"> • TS Alliance CEO serves as Chair of the ATS-PAR. • The Improving Partnerships and Lives Grant was developed in 2019 to encourage working relationships with new 501c3 organizations. • Continued emphasis on ISAN and collaborating with multiple organizations to focus on the challenges of infantile spasms, including public awareness and access to care. • Participated in the Transition of Care Summit along with rare epilepsy partners to identify needs across the spectrum in partnership with CNF and Greenwich Biosciences. • Hosted TSCi meeting at International TSC Research Conference in Toronto. • Co-hosted 6 Regional TSC and LAM Conferences in collaboration with The LAM Foundation. • CEO and CSO served as guest presenter at Global Genes Data DIY. • Established the Bcurefull Travel Fund at the TS Alliance. 	<p>Built and maintained working partnerships with 17 national organizations/collaborations. 4 added in 2020 include: DEE-P, Faster Cures, Rare As One, Seizure Action Plan Coalition.</p> <ul style="list-style-type: none"> • TS Alliance CEO serves as the Chair of the ATS-PAR • The Associate Director of Research serves on the Board of the Epilepsy Leadership Council. • TS Alliance was a founding member of a new collaboration, the Seizures Action Plan (SAP) Coalition, promoting awareness of SAP and emergency plans. • TS Alliance became members of the newly formed Governance Committee for the Infantile Spasms Action Network (ISAN). • Hosted virtual TSCi workshop in November 2020. • Co-hosted 3 progressive Virtual TSC and LAM Conferences in collaboration with The LAM Foundation. • The CSO served on the Scientific Committee for the Tuberous Sclerosis Association's 2020 International TSC Research Conference, originally planned to be in person in London but changed to two virtual events in November 2020 and June 2021. • The CSO serves on the steering committee of the Coalition of Patient Advocacy Groups for the Rare Diseases Clinical Research Network.

Establish relationships between all Community Alliances and local Arc Chapters where existing by 2023 utilizing Associate Partnership with The Arc US	67 referrals from our Community Alliances to local Arc Chapters to build new working relationships.	21 referrals from our Community Alliances to local Arc Chapters to build new working relationships.
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	<ul style="list-style-type: none"> 23% (430 of the 1,835 attendees) of Community Alliance attendance at meetings was done virtually. TSC Now podcast series launched in May; 8 monthly episodes were produced in 2019 with 1,426 total downloads/listens. 	64% (3,171 of the 4,988 attendees) of Community Alliance attendance at meetings was done virtually.
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	TSC Academy launched on January 31, 2020 for the 2020 March on Capitol Hill.	79% of sponsored attendees took TSC Academy in preparation for advocacy on Capitol Hill. Courses developed were a Primer on TSC 101, Introduction to Research and Funding, and The Advocate's Role.
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	<ul style="list-style-type: none"> Maintained 6 global partnerships, including renewals of TS Alliance of Israel and TS Canada ST. Recognized 1 new global TSC Clinic, bringing the total number of internationally recognized clinics to 9 (3 in Israel, 3 in Canada, 2 in Mexico, and 1 in India). 	<ul style="list-style-type: none"> Continued our 6 global partnerships Recognized 1 new global TSC Clinic, bringing the total number of internationally recognized clinics to 10 (3 in Israel, 4 in Canada, 2 in Mexico, and 1 in India).

4. Educate and Mobilize to increase investment

Measures	Dec. 31, 2019	Dec. 31, 2020
Raise \$56 million for research over the next five years	Goal changed to \$40 million over seven years following a campaign study and on recommendation of our capital campaign consultants. TS Alliance raised \$2,650,212 in 2019.	\$4,107,146 has been raised for the Investment to Accelerate Research as of 12/31/20.

Grow the Endowment Fund to \$10 million by 2023 and realize the maximum allowable annual contribution	Total net assets for the TS Alliance Endowment Fund are \$5,915,804 as of 12/31/19.	Total net assets for the TS Alliance Endowment Fund are \$5,893,522 as of 12/31/20.
Mobilize grassroots community through budgeted special events to raise \$1.5 million average net annually over next 5 years	\$2,421,439 net was raised through special events in 2019.	\$1,227,362 was raised through special events in 2020.
Increase the number of people donating online to crowdfunded events from 1,257 (2017) to 2,514 (2023)	1,182 individuals donated online to crowdfunded events through Luminate and 4,016 donated through Facebook	4,815 individuals donated online to crowdfunded events through Classy with an additional 4,728 individual donations made through Facebook in 2020.
Secure 95 million impressions annually via multiple and varied outlets with minimum investment	Four news releases in 2019 garnered 313 million impressions.	Nine news releases in 2020 led to 694 million impressions.
Achieve additional 30 million impressions through new awareness campaign and 3,500 contacts to the database per year of the campaign	Marketing firm was contracted to conduct research on current messaging, branding and awareness with recommendations to be presented in 2020.	Marketing/communications presented findings/recommendations early 2020 and organization moved forward with planning and decisions for name change/rebranding in 2021, formally beginning May 15.

5. Build and Strengthen Organization

Measures	Dec. 31, 2019	Dec. 31, 2020
Maintain a 4 star CN rating and meet or exceed an 85/15 program/supporting expenses ratios by 2021 in accordance with the five year Research Business Plan	TS Alliance received a 4-star Charity Navigator rating, Better Business Bureau Wise Giving accreditation, and Platinum Guidestar status in 2019. Expense ratios were 72.9% program/27.1% admin and fundraising for FY19.	TS Alliance received a 4-star Charity Navigator rating, Better Business Bureau Wise Giving accreditation, and Platinum Guidestar status in 2020. Expense ratios were 73.2% program/26.8% admin and fundraising for FY20.

Implement in depth review to ensure integrity of constituent data with less than 1% duplicates by 2020 and maintain thereafter	Constituent database was reviewed and 1.2% dupes remain.	Maintaining the integrity of the database with 0.05% of duplicates noted on 12.31.20.
Participate/present at 25 professional/industry conferences per year	TS Alliance staff attended or participated in 40 professional/industry conferences.	TS Alliance staff attended or participated in 22 professional/industry conferences
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	<p>Added 8 new board members (6 elected, 2 fulfilling other terms) with expertise in financial management, global relations, IT technology, HR, outreach to Spanish speaking community, event planning, legal, nonprofit leadership/fundraising.</p> <ul style="list-style-type: none"> • 5 are women and 3 are men • 2 from diverse backgrounds • Geographically: 1 from DC; 1 from CA; 1 from the West; 2 from Midwest; 2 Northeast; 1 from the Southeast • 7 of the 8 have relatives with TSC. 1 is a corporate partner 	<p>Added 4 new board members with expertise in science and medical, neurology, TAND, clinical care, nursing, government affairs, global relations.</p> <ul style="list-style-type: none"> • 2 are women and 2 are men • 1 from diverse background • Geographically: 1 from AL; 1 from TN; 2 from VA <p>2 of the 4 are impacted or are a parent of an individual with TSC. 3 are medical professionals</p>
Grow Science and Medical team to 9.5 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	<ul style="list-style-type: none"> • Hired a new Associate Director, Research, taking the Science and Medical team to 5 FTEs. • Added Director of Digital Platforms to support communications. 	<ul style="list-style-type: none"> • Hired a new Director, Medical Affairs, taking the Science and Medical Team to 6 FTEs, including approximately 0.5 FTE dedicated to constituent support services. • 90% of staff retention outside of COVID-19 related layoffs.
Create transition plan for senior staff retirements	Transition plan created for VP of Support Services for retirement in April 2022.	VP of Support Services retiring early at the end of January 2021. Implemented transition plan in order to ensure continuity of care and service.