

TSC Alliance
Board Meeting Notes
Monday, June 6, 2022
DRAFT

In attendance:

Voting Board Members: Peter Crino (Chair), Derek Bauer, Julie Blum, Matt Bolger, Chip Burkhalter, Lisa Carlton, Sara Chieffo, David Coit, Julian Gangolli, Tanjala Gipson, Rob Grandia, Dana Holinka, Lesley Holmes, Bill Joseph, Jessica Krefting, Laura Marks, Diane McSwain, Mustafa Sahin, Sean Shillinger, Vanessa Vazquez, Paul Waxlax

Endowment Fund Chair: Marion Adams

Staff: Cynthia Arcuri, Steve Roberds, Kari Rosbeck

Chair's Welcome and Report

Peter Crino called the meeting to order at 8:02 p.m. EST and confirmed a quorum of voting members. He expressed excitement over the upcoming World TSC Conference and the continuing challenges of the never ending COVID pandemic. Pete shared that the board has a charge to look at organizational goals for 2023. Pete reviewed the Focus Statement which he stated captured the spirit of what the TSC Alliance is all about - research, advocacy, and providing hope. He emphasized the mainstay of the organization is our community which makes the TSC Alliance so great and has been for several decades. The final point that about the Focus Statement is the importance of creating an environment where all individuals feel included.

Kari Rosbeck also shared that committees need to meet over the next several months to set organizational goals for 2023 and encouraged committee chairs to work with their staff liaisons to set meetings or discuss draft goals by August 11 in order for the staff to have full draft organization goals to work with by the staff retreat (August 15-19). The 2023 organizational goals will be reviewed and approved by the full board meeting at the next meeting on October 21.

Approval of Board Meeting Minutes from March 3, 2022

Jessica Krefting asked if board members had reviewed the meeting minutes from March 3, 2022 included in the board packet and if anyone there were any edits or changes. There being none, Krefting asked for a motion to approve. *Coit* moved and *Gangolli* seconded the motion to approve the March 3, 2022, meeting minutes. *The meeting minutes passed without dissent.*

Governance Committee Report

Matt Bolger reviewed the slate of board candidates. Board candidates being nominated for a second three-year term include: Blum, Carroll, Joseph, Marks, Vazquez, and Waxlax.

Matt also shared that there were up to 3 slots to fill for new board members and the Governance Committee were able to get 2 of the 3 new board member slots filled. The new members include: Jonathan Goldstein and Dr. Shafali Jeste. He reviewed their credentials of the new board nominees and shared they will be of tremendous help to build and move the organization to the next level.

Grandia moved and *Holinka* seconded the motion to approve the FY23 Slate of Board Candidates. Blum, Marks and Vazquez abstained from voting on returning members as they are candidates. *All other members approved the full slate of FY23 Slate of Board Candidates.*

CEO Report

Kari Rosbeck thanked Pete for his leadership and started her report with the status of the World TSC Conference and comparisons to 2018. The 2022 sponsorship goal is \$741,500 and as of June 6, the

organization had raised \$656,559 (88.5%) of the goal, compared to \$616,333 raised (104% of goal) in 2018. Though we had expected 1,000 attendees at the 2022 World TSC Conference, we will likely fall short of that goal, but this will decrease overall meal costs. There have also been increases in cost. The TSC Alliance does anticipate raising more than \$700,000 in sponsorships and at this point still believed the conference would break even. Kari shared that the TSC Alliance Board sponsorship, encouraged member to contribute, and thanked those who had already donated.

Moving to the Research Campaign, she shared that \$8.86 million had been raised with a majority of funds coming from individuals and private foundations. Kari reviewed the campaign by gift level and shared that 22% of the campaign goal of \$40 million had been raised to date. She also shared that there was a \$5 million prospect with a meeting scheduled the following week. David Coit stated that a \$5 million donation would be game changing. The budget for the year is \$4.8 million, and this gift would get us a third of the way to our \$40 million goal. Progress is going well and we are beginning to look at some foundations outside of the TSC community in addition to what we thought we could raise from our internal prospects. He acknowledged the staff for driving the campaign, in particular Kari and Lisa Moss. There have been a total of 71 donor meetings conducted this year for the Research Campaign - 47 ongoing conversations, 8 proposals submitted totaling \$4.16 million, 4 pledge/gift donations received for \$565,000 and 3 stewardship meetings.

Regarding events, Kari reported it had been a very productive year, including the most successful Comedy for a Cure in the history of the event, mainly due to our honorees The Dinsdale Family. The goal was \$300,000 and we raised \$583,600 with an expense ratio @ 25%. \$115,000 was raised in sponsorships (third highest in our history). Ticket Sales were \$23,150 (second highest in our history). General donation accounted for \$288,403 (highest ever by \$250,000). We also raised \$70,600 for our Fund a Cure which goes to the Research Campaign. The 20th Step Forward to Cure TSC, Global Hybrid Walk Run Ride was held over the week of May 14-15. The goal was \$850,000 and \$731,300 had been raised to date. Historically we have always raised money after the walks. In 2021, \$600,000 was raised at the end of the walk weekend and by end of the year we had raised over \$800,000. There were 1,918 registered participants, 204 teams, 29 TSC Clinic Teams, 26 National Planning Committee Members, and 20 sponsors (8 National and 12 regional). There were three live locations this year (LA, Dallas and PA) and 1 virtual action. With both of these events, we are well over goal for the year with our special events.

Finally, Kari thanked the entire TSC Alliance Team who make all of this possible.

Science & Medical Report

Steve Roberds gave the Science and Medical Report. For research grants, he reported that 18 letters of intent were received, including 5 post doc and 13 early investigators. These were currently under review and a subset will be asked to submit full proposal later this month. The 2023 International Research Conference is planned for September 7-9 and will be held at the Omni Shoreham in Washington, DC as it offered the best rate.

There have been, 2,067 samples collected in the Biosample Repository, including those from TACERN, RDCRN, PREVeNT, and buccal swabs collected early at the 2018 World TSC Conference. Gabrielle Rushing leads this project and was able to obtain 160 tissue samples, the majority of which came from the Maryland Brain and Tissue Bank. The project is on-track and we are also distributing samples to researchers.

Steve shared that there are now 3 FDA-approved drugs specifically for TSC, Afinitor, Epidiolex, and now Hyftor, the new topical sirolimus gel for facial angiofibromas. There were delays by the FDA because the pandemic made it difficult to travel to inspect Nobelpharma's facility in Japan. We expect Hyftor to be available in late 3rd quarter 2022. The TSC Alliance has been working for over a

decade to bring drugs to market, such as understanding the mTOR pathway, advocating for pharma product with regulators and access to products. Even with 3 approvals for medications, the TSC Alliance continues to address the need for new therapies with the Preclinical Consortium to facilitate drug discovery.

Since its launch in 2016, there have been 17 industry members, 60 compounds tested in the various animal models and the establishment of mouse models for epilepsy, tumors, and now TAND. Steve highlighted three success stories. The MEK inhibitor was tested in an epilepsy model, then in the tumor model, funded by donor dollars and would not have been possible without the TSC Alliance. Over the last couple of years, Dean and Steve have reached out and advocated with the company that produces the drug, which is in trials for neurofibromatosis. We now expect filing an IND in the 3rd quarter and starting Phase 2 & 3 clinical trials in TSC. Second, is the development of next generation mTOR inhibitor. The TSC Alliance agreed to co-fund the first study which was successful, and the company was able to raise venture capital because of the study and pay back the TSC Alliance. The company was then able to run multiple more studies hopefully leading to a phase I clinical study also launching in the 3rd quarter this year. Finally, gene therapy is a long time coming but further along than 10 years ago. There is ongoing testing in the Preclinical Consortium. These are 3 examples of where the TSC Alliance and Preclinical Consortium has been extremely impactful over the last several years.

Steve then highlighted more about the MEK Inhibitor and upcoming trial. MEK is a critical protein that controls cell division and metabolism. MEK activity is increased in TSC brain malformations in mice and humans. Mirdametinib is a MEK inhibitor in clinical trials for NF1, glioma, and solid tumors which means TSC does not have to be the first study out of the gate. We can learn from those studies and all the TSC studies already done and get the best of both worlds. Mustafa Sahin is a key advisor over the last year in the translation of the MEK Inhibitor into clinical studies and the TSC Alliance is collaborating with Springworks. The TSC Alliance will help organize and lead a TSC clinical trial. Springworks will provide financial resources and share experiences with mirdametinib. TSC Alliance will have a contract with CROs (contract research organizations), MMS and LBR for regulatory data management. This will take the TSC Alliance time and resources for which we will be compensated. The first service agreement in place for \$49,544 is almost complete. The second service agreement is pending for \$340,345. The TSC Alliance will receive \$124,347 to cover our costs and \$215,998 will go to MMS for their services. Pete thanked Steve for all his work.

Finance Report

Bill Joseph reported on behalf of Paul Waxlax and the Finance Committee. Overall, the TSC Alliance currently in an outstanding financial position. Cash as of 4/30/22 was \$2.86 million compared to the original projection of \$2.1 million, or a positive difference of \$674,476. Pledges receivable were at \$3.1 million and reflected Kari and Lisa's efforts in the Research Campaign. Special Events net revenue as of 4/30/22 was \$1 million, with a budget of \$642,000 through April and a positive variant of \$348,000, reflecting the outstanding performance of Comedy for a Cure. Major donor contributions were at \$79,000 of a year-to-date budget of \$10,000. Grants are under budget and will likely continue throughout the year because of a double count when putting the budget in our budgeting software. Bill shared that this is being made up in other areas so we don't expect this to be an issue by year-end. General contributions were at \$1.4 million of a budgeted \$1.5 million and if you take the \$250,000 out from the grants then, in essence, we are ahead for the year. Contracts were behind currently and expect those to catch up. The Endowment Fund Transfer was double-budgeted for \$50,000 in two separate line items – Sponsorships (for World Conference) and Endowment Fund Transfer. The \$50,000 sponsorship transfer has been made and recorded in the Sponsorship line. Endowment approved an additional \$20k for Scholarships of the World Conference and this amount is also recorded in the Sponsorships line. On the expense side, Salary and Benefits are at \$781,000 vs a budget \$864,000 due to timing of new hires. However, with annual increases, we expect to be on

budget by end of the year or maybe slightly over. Bill explained that inflation and wage growth are predominant and the TSC Alliance had some additional adjustments this year for people to be on par with industry for those job responsibilities. This should not affect overall cash position. Total expenses were \$2.5 million vs budget of \$2.8 million, or a savings of \$364,000 year to date. Functional expense ratios impact our four star rating and we want to hit 78.5% in 2022. As of April 30, we were at 75.59% due to front-end loading of administrative and fundraising expenses and should migrate up to the 78.5% by December 31.

Cynthia Arcuri reported on projections. Anticipated Revenue Increases/Decreases:

- Research Investment Fund +\$430k
- Contracts +\$413k
- Comedy +\$283k
- Special Events DBD savings +\$41k
- Major Donors +\$18k
- Budget double-counting of (\$267k)
- Sponsorships (\$9k)
- Net Anticipated Impact to date +\$910k

Anticipated Expense Increases/Decreases:

- Salaries & Benefits +\$80k – true-up of salaries & taxes to be in-line with market
- LOC fee +\$3k
- Possible decrease in World Conference expense if attendance is lower than anticipated (too early to tell)

As of 4/30/22, Change in Net Assets exceeds budget by +\$511k. Full Year Projected Change in Net Assets would exceed budget by +\$812k.

Cynthia reviewed our budgeted milestones to staged spending. Our first milestone was to have a cash balance of \$2 million and \$2.95 million gross revenue on April 30 Actuals were \$2.8 million cash and \$3.1 million in gross revenue. Had milestones not been met, expenses would have been reduced as outlined in the plan. For July, milestones include cash balance of \$1.63 million and \$4.07 million gross revenue. The bigger concern for this milestone is gross revenue, particularly with the double count of \$267,000 in grants. There is a chance we may be off by about \$15,000 and will need to revisit at that time. Cynthia reported that the Executive Team reviewed numbers through May and projections in June and hope to be at \$4.08 million as of June 30. Paul Waxlax thanked Bill for reporting and shared we are very close when you look at \$15,000 out of \$4.07 million. He also shared he agreed with Bill and Cynthia to see where numbers come, especially cash, but just wanted to be sure to give the board advanced notice in case a discussion needed to take place.

On cash flow, Cynthia reported that Inflows assumptions include:

- May – Walk Run Ride revenue came in, plus some larger contract payments/preclinical memberships, anticipating more sponsorships for World TSC Conference, anticipating World TSC Conference registrations to be lower than budgeted
 - *Total WRR raised was \$726k of \$850k goal*
- June – quiet month, most registrations and sponsorships have already been received, may experience some receivables being collected for special events
- July – quiet month, \$238k Endowment Transfer scheduled
- August-December – collect payments on multi-year pledges

On the outflows side:

- May & June – travel booked for World Conference scholarships, speakers, TSCi affiliates, preclinical expenses paid (after collection)

- July & August – final World Conference expenses paid out (highest month of expenses)
- Sept, Nov & Dec – budgeted grants to payout

The cash flow reserve is \$1,134,492. Estimates are well above that with the low point in the current cash projections is in October at \$1.93 million and no concerns currently.

Cynthia shared the projections for the World TSC Conference. Total revenue estimated at \$803,600. Revenue was still looking good even with less registrations than we originally planned most likely due to COVID and travel concerns. Expenses estimated at \$792,246 and still anticipate an \$11,000 surplus for the conference. Kari mentioned that the scholarship income is really at \$90,000 because of the generosity of the Endowment Fund and Foglia Family Foundation. Cynthia explained we budgeted for \$50,000 and that the other is in some of the other travel expenses (food). Pete inquired about a late June push for registrations and Kari stated early bird prices were extended to June 30 with social media and email push planned since some people may be waiting to make a game day decision.

Endowment Fund Report

Marion Adams presented the endowment fund report. As of 5/31/22, total investments were at \$6.1 million with a decrease from 12/21 of \$800,000 or 8.8% decrease due to the stock market, which is down overall by 12%. Transfers were approved by the Endowment Fund, \$70,000 for the World TSC Conference (which has been transferred) and the annual \$238,000 for general operations (which may or may not need but is ready to transfer).

Committee Reports

Compensation Committee

Bill Joseph gave the Compensation Committee report. He shared that the board would not be voting on the Employee Handbook at this meeting. The Committee is still in the process of reviewing. There was an extensive rewrite including state specific information for each state where employees are located. The Committee needs to get back together to discuss some of the wording and the way it is structured and hopefully by the October board meeting the full board can take a vote on it. Joseph confirmed that Attachment K is the updated DRAFT Employee Handbook.

Equity, Diversity & Inclusion (ED&I) Update

Laura Marks gave the update on the ED&I. On May 21, the ED&I Task Force met with 23 attendees including staff, community and some board members. Laura shared the excitement for the World TSC Conference and multicultural gathering. In order to prepare for that gathering, the ED&I Task Force are hosting a virtual meet and greet to involve more community members before the World TSC Conference and who are hopefully attending. Laura reported on some positive results from 2021 including: an increase in the diversity of speakers at World TSC Conference; scholarship awards for the World TSC Conference aimed to increase equity for those who applied; Spanish translation of key brochures/documents and update on Spanish webpage. Work is underway to look at gaps in the Natural History Database/Biosample Repository and outreach to the currently under-represented Black and Asian community. Gabrielle Rushing, Ashley Pounders and Dr. Tanjala Gipson are collaborating on a project and publication, focusing on different skin manifestations in black people and correlation to delayed diagnosis. They are also working on an infographic to raise awareness of differences in darker skinned individuals and their skin manifestations. Zoe Fuchs co-chairs the Diversity Committee for the Health Research Alliance and from that work recently published an article. And the TSC Alliance has applied for a fellowship through the Rare Disease Diversity Coalition. Overall, the task force is taking a step back to prioritize what we need and approach, whether on three subgroups like we had last year or to focus more on specific projects.

Government Relations Report

Sara Chieffo provided the Government Relations Committee report. The March on Capitol Hill once again was held virtually and with some record breaking numbers for the TSC Research Program (TSRP) at the Department of Defense (DoD). This did extend for several months given the current climate in Washington, DC. Overall, 197 people from 42 states registered to participate. Holding the March virtually has allowed more people to be involved. Our ask, as in the past, was for Congressional representatives to sign on to the Dear Colleague letter in support of the TSCP. FY23 House Dear Colleague Letter sponsored by Representatives Mullin (R-OK) and Raskin (D-MD) closed with 205 signers (record for virtual), exceeding last year's number of 202. FY23 Senate Dear Colleague Letter sponsored by Senators Cramer (R-ND) and Heinrich (D-NM) closed with 35 signers, exceeding last year's number of 34 and matching a previous high of 35. It is huge to have a third of the Senate signing the Dear Colleague as they don't like to sign things. We did advocate for an increase this year from \$8 million to \$10 million. Sara offered a huge thank to those who participated and also to Katie Smith for all her hard work.

State funding is also proceeding. In MO, the TSC Alliance requested \$350,000 for TSC Centers of Missouri. It was in the MO state budget for \$250,000 and our MO advocates contacted key appropriators offices to ask for an increase. TSC Centers of MO received 250,000 last year. No final update was available as of the board meeting. In MD, we requested continued funding of \$500,000 for the TSC Center of Maryland and University of Maryland Clinical Center for Adults with Neurodevelopment Disorders. TSC Center of Maryland also received \$350,000 in supplemental funding for a psychiatric nurse practitioner (PNP) and RN to help with mental health screening evaluations and care. MD also issued a proclamation for TSC Global Awareness Day on May 15. In AL, Margaret Cox, Marion Adams, and Dr. Martina Bebin are exploring TSC Awareness and if there's a possibility for state funding in AL. As an initial step, they met with Governor Ivey's office on April 4 and had an AL proclamation recognizing TSC Awareness. Pete offered his appreciation to Sara and shared how thrilled he was for the TSC funding in MD. He also thanked Sean and Lauren Shillinger for all their hard work, including a new seizure safe schools bill, called Brynleigh's Act after their daughter.

Global Relations and Ukraine Task Force

Kari Rosbeck provided the report on behalf of Darcy Krueger and with an update provided by Katie Smith. For TSCi, Kari shared that we are glad to be co-hosting the World TSC Conference and looking forward to the TSCi Workshop which will focus on global collaboration, including a look at global clinical trials, international partnerships and TAND. There are still concerns from some countries around travel regarding visa complications and Katie Smith as been encouraging anyone who can't come to the World TSC Conference to participate online. Regarding Global Alliances, Kari thanked Vanessa Vasquez for helping with Spanish translations, including the *Intro to TSC*, *Intro to Clinical Trials*, *Infantile Spasms*, and *TSC Alliance Place of Hope* Brochures. As reported previously, the TSC Alliance is now revamping the Spanish web page. Additionally, we are collaborating with Canada and India on the Natural History Database. Hoping to have good Global Alliance representation from Canada, Hungary, India, Israel, and Mexico at the World TSC Conference.

The TSC Alliance continues to closely monitor the situation in Ukraine. We are in regular contact with TSC Ukraine and their local medical advisor based in Lviv. The organization and medical advisor have been keeping us updated on their access to antiseizure medications and mTOR inhibitors. We are facilitating connections with our industry partners and Ukrainian contacts to help with medication access and have been working with Mustafa Sahin, MD, PhD to assist with TSC medication needs identified by Boston Children's Hospital (BCH) and the Ukraine Neurology Initiative (UNI). They have been very helpful to get medication overseas.

The TSC Alliance has also been working with Upsher-Smith for donations of vigabatrin. Upsher-Smith is in contact with a team of individuals from BCH who are working with a third-party, non-profit partner, Brothers Brother Foundation (BBF) in Pittsburgh, PA to facilitate a donation of vigabatrin to

Ukraine. There have been challenges around regulations and it has been a multi-step process as Upsher-Smith products are only approved in the United States and they can only deliver vigabatrin to a US-licensed pharmacy. Brother's Brother Foundation received a donation and is working with the Neurosphaera Epilepsy Therapy Center (led by Piotr Zwolinski, MD) and the Emergen Foundation in Warsaw, Poland who will deliver the medication to Ukraine for distribution to patients. UNI recently reported their shipment of anti-seizure medications through BBF (~30,000 units of product) has safely reached Dr. Tychkivska, the medical advisor in Lviv. This shipment included a donation of vigabatrin from Upsher-Smith.

Dr. Sahin confirmed that there is only about a 2-week supply of vigabatrin left in the country and that really vigabatrin seems to be the bottle neck for medication making it to Ukraine. There is still additional need for vigabatrin, so BCH are working with their European colleagues to identify a possible pharmaceutical company in Europe for future vigabatrin needs. Dr. Sahin reported that they have been introduced to a contact at Novartis Oncology Ukraine. Novartis Oncology Ukraine has long supported patients with TSC and is well acquainted with the only patient organization in Ukraine representing the interests of these patients. With the outbreak of war in Ukraine, Novartis Oncology Ukraine donated "Western Ukrainian Specialized Children's Medical Center" the necessary amount of everolimus (Afinitor) for patients in need of it. According to the information available to Novartis Oncology Ukraine, at the moment in Ukraine pediatric patients with TSC are provided with the necessary amount of Afinitor. They are now working to provide adult TSC patients with Afinitor. There was discussion about what more TSC Alliance could do and general agreement that with the heavy REMS for vigabatrin creates a very challenging situation. Kari shared that she and Katie will keep the board updated if any new information becomes available.

Outreach Report

Dana Holinka gave the Outreach report with an update on the goals. The TSC Navigator had a goal of 1,000 views and as of April 30 was already at 785 views. Another goal was to provide support for 50 encounters regarding medication issues and as of April 30, the TSC Alliance has provided support for 21 medication issues. The goal is to increase peer to peer supports annually by 10% and motivate a new generation of volunteers/advocates for the TSC Alliance, by adding 100 new volunteers and provide 600 volunteer training hours. To date, we have provided 482 training hours. For the March on Capitol Hill, 100% of new participants completed the training through the TSC academy.

Pete thanked all the committee members for their updates.

Other Business

Pete asked if there were any other committee reports or business to be discussed. There being none, he thanked the board, sharing it is a group effort to operate effectively during the pandemic. Overall, he was very optimistic with the financial report and Science and Medical's potential for new therapies over the next 5 years.

The next board meeting will be held in-person on October 20-21 at the TSC Alliance HQ. Rachel Wojnilower, Executive Assistant, will be reaching out to board members about attendance. The last board meeting of the year takes place on December 12 via Zoom at 8pm EST.

Bauer moved to adjourn and *Blum* seconded. *The motion passed without dissent.* The meeting was adjourned at 9:16pm EST.

Respectfully submitted,
Jessica Krefting, Secretary