

PERSPECTIVE

A MAGAZINE FROM THE TSC ALLIANCE



WINTER 2022 • VOLUME 152



SPECIAL ISSUE: 2022 World TSC Conference

PAGE 8



With the TSC community as our strongest ally, the TSC Alliance® is a source of hope and connection for all affected by tuberous sclerosis complex (TSC). We drive research, increase care quality and access and advocate with and for people affected by the disease.

While the TSC Alliance recognizes and is closely monitoring the continuing challenges of the COVID-19 pandemic, our organization is moving full steam ahead in 2022 with plans for an array of new events, research efforts, programming and activities to support our ongoing efforts to create a future where everyone with TSC can realize their full potential – no matter how complex their journeys are to get there.

As always, we believe one of the most important things the TSC Alliance can do is bring the TSC community together – whether locally, throughout the United States or across the globe, which is crucial for inspiring our collaborators, physicians, researchers and families and individuals affected by TSC. The upcoming 2022 World TSC Conference provides the perfect opportunity to do just that, and for anyone unable to attend in person, we are very excited to offer a hybrid registration option if you want to attend virtually. You'll find complete conference details in this issue's cover

story, starting on page 8. We look forward to seeing you in Dallas!

We are also excited Comedy for a Cure® and Step Forward to Cure TSC® both celebrate 20th anniversaries in 2022! Combined, these two signature events have raised a staggering \$22 million since their founding – a true testament to the commitment and resilience of the TSC community's support and a robust volunteer army.

The TSC Alliance simply couldn't be successful without working hand-in-hand and side-by-side with volunteers, board members, physicians and scientists on our advisory boards and others – you each help our organization thrive. We want to thank our outgoing board members – Tim Dills, Bonnie Hogue Duffy, Jim Maginn, Chris Russell, Matthew Simonian – for their extraordinary service and leadership. We also want to welcome new board members Lisa Carlton, PhD; Julian Gangolli; Lesley Holmes, JD; Patroski J. Lawson, MSP; Kristi Lengyel; and Brittany Smith.

Advocacy by the TSC community is also crucial when it comes to Federal TSC research funding. Once again, this year's March on Capitol Hill be held virtually, which means anyone can easily support this effort from your home or office. Find out more on page 5.

Elsewhere in this issue, you'll find an article to help your semi-dependent or dependent loved one with TSC

Message from leadership

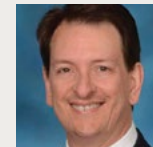
transition into adulthood on page 6. On page 20, you can read about an exciting new self-report portal for the TSC Natural History Database and the TANDem project. We've also included a summary of the 2021 Virtual International TSC & LAM Research Conference, held this past October.

People affected by TSC have more hopeful futures because of the progress we are making as a community. Together, we have raised awareness of TSC, accelerated discoveries that have led to new FDA-approved treatments and created support systems in the United States and around the world to improve TSC care and quality of life.

Join us at tscalliance.org.



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President & CEO



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If you have opinions, questions or articles for *Perspective*, we would like to hear from you. Please contact the managing editor to obtain a submissions form and guidelines.

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TSC Alliance staff updates

The TSC Alliance® is excited to announce several staff updates. First, Shelly Meitzler has been promoted to Director, Community Education & Resources. Next, we have two new staff members – Cynthia Arcuri joined our team as Chief Financial Officer and Anne Wolfe as Senior Community Programs Manager.

Shelly Meitzler joined the TSC Alliance as a Community Outreach Manager in 2017 specifically to increase community support programs. In her first year, Shelly was a critical contributor to newly formed Community Programs Department, supporting 18 Community



Alliances and 13 individual walks and serving as staff lead for all Regional TSC and LAM Conferences, while also backing up Dena Hook, then Vice President, Support Services. She also completed the Special Education Advocate Training 1.0 through the Council of Parent Attorneys and Advocates and is currently completing the Special Education Advocate Training 2.0 through the Council of Parent and Attorneys and Advocates.

In late 2020 when Dena retired, Shelly jumped at the chance to absorb the responsibilities of managing the Education Parent Mentor (EPM) and Dependent Adult Transitional Regional Coordinator (DATRC) programs, as well as managing existing and incoming TSC Connect members in her region.

In her new role as Director, Community Education & Resources, Shelly will provide education, guidance, support, resources and advocacy services to the TSC community. This position will direct peer-to-peer programs that support these efforts, including the EPM, DATRC and TSC Connect programs. Shelly will also direct planning for TSC conferences at the local, regional and international levels, including the 2022 World TSC Conference.

Cynthia Arcuri, CPA, joined the TSC Alliance as Chief Financial Officer (CFO). She previously served as Director of Finance & Administration at Louis Plung & Company in Pittsburgh, PA, overseeing all firm operations, including strategic planning initiatives, accounting, marketing and human resources.

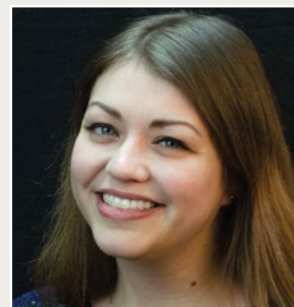
In addition, Cynthia is a nonprofit Generally Accepted Accounting Principles (GAAP) accounting expert and

spent 13 years as an auditor specializing in nonprofits at Louis Plung & Company as well as Ellin & Tucker in Baltimore. She earned a Bachelor of Science, Accounting from Pennsylvania State University and is a member of the American Institute of Certified Public Accountants.



As CFO, Cynthia will implement the infrastructure/systems needed to support the TSC Alliance's growth over the next five years. She will also build and manage effective and streamlined administrative/financial systems, including financial, accounting, legal, information technology, human resources and physical infrastructure. As a member of the Executive Management team, Cynthia will be involved in strategic planning, evaluation and professional development initiatives as well as overall management of operations as they relate to budget management, cost-benefit analysis, investing and protecting resources, forecasting needs and securing new funding.

Anne Wolfe, a passionate rare disease advocate, joined the staff as Senior Community Programs Manager. Anne comes to the TSC Alliance with more than a decade of experience in creating events and programming for diverse stakeholder audiences in healthcare, life sciences and beyond. Anne most recently served as Manager of Strategic Advancement for Global Genes, where she led the development of impactful and relevant programming and worked with the leadership team to build and sustain strategic partnerships and alliances to support community engagement and capacity building.



Anne's primary responsibility will be to assist the Community Programs team in developing and implementing programs that build, grow and advance our volunteer networks across the country with a focus on strengthening the peer-to-peer programs and Community Alliances that support to our TSC community.

With an honors degree in Peace & Justice Studies and International Relations from Tufts University, Anne will be staff lead on the TSC Alliance's Equity, Diversity & Inclusion Task Force and will help to accelerate our efforts to develop and execute strategies that reach, support and connect underrepresented TSC communities. In addition, Anne will lead all young adult- and adult-specific programming, including Regional Adult Coordinators and Future Leaders.

Special events



The **19.5 Annual Comedy for a Cure®** was wildly successful, **raising \$143,000-plus**, on October 17, 2021. The show was livestreamed to online viewers from Feinstein's at Vitello's in Studio City, CA, along with a sold-out in-person audience.

We were thrilled to present the much-deserved **Courage in Leadership Award** to **Althea Grace**, singer songwriter – who gained national



Althea Grace



Wendy Liebman

attention as a contestant on *American Idol* Season 19. We were also pleased to honor **Wendy Liebman**, who has been a significant behind-the-scenes contributor to Comedy for a Cure and our mission for many years, with a **TSC Champion Award**. The magical night included musical entertainment by Althea and a piano performance by Emily Phan, TSC Alliance Future Leader. The night's hilarious comedians included Jodi Miller, Marty Ross, Taylor Williamson and headliner Drew Lynch.

Kudos to our fabulous co-hosts Jim O'Heir and Wendy Liebman who, together with auctioneer extraordinaire Damon Casatico, presented another stellar event. And a huge thank you to event sponsors Greenwich Biosciences, Novartis, *Variety*, Horizon, Maginn Family, Neurelis, WNC Associates/Cooper Family, Upsher-Smith Laboratories LLC, Ray Chan, Tim and Kathi Dills, The Keith A. Hall Family, Marinus, Ted and Peggy Mastroianni, REGNENXBIO, Szilagyi Family and UCB.



On Sunday, November 7, 2021, eight Team TSC Champions ran in the **50th Anniversary TCS New York City Marathon** to raise awareness of tuberculous sclerosis complex and funds to support the mission of the TSC Alliance.



Maureen Elliot and Katherine Chapman

Congratulations and thank you to our dedicated Team TSC 2021 Marathon Runners: Amanda Alberts, Claire Blyth, Chip and Kristin Burkhalter, Raymond Capogna, Katherine Chapman, Maureen Elliot and Ryan Kennedy for **raising \$47,602** for the TSC Alliance.

Other community campaigns hosted by local TSC champions

Heather Joy Memorial TSC/LAM Golf Outing

Hosted by Loren and Christy Buntrock
September 18, 2021
Raised: \$5,634



Keith A. Hall Memorial Golf Tournament

Hosted by Family and Friends of Keith A. Hall
October 1, 2021, Raised \$5,275

30th Annual Cookin' Up a Cure

Hosted by Pam Sztukowski
October 3, 2021, Raised \$7,145

Facebook donations/birthday fundraisers

September 1-December 1, 2021
Raised: \$33,831

Thank you for supporting the TSC Alliance on your birthday or special occasions!

Save the dates!



The TSC Alliance's **20th Anniversary Comedy for a Cure Gala**, with Headline Sponsor Jazz Pharmaceuticals, will be held **Sunday, April 3** in Hollywood. Contact April Cooper at acooper@tscal-liance.org for sponsorship opportunities, ad sales or gift-in-kind donations. We look forward to celebrating this special anniversary with an extra special event. Stay tuned for more details!

STEP FORWARD to cure TSC

Plan now to help celebrate our **20th Anniversary of Step Forward to Cure TSC®** with a weekend-long hybrid walk-run-ride to highlight Global TSC Awareness Day and our worldwide

TSC community, **May 14-15**. Online registration opens January 24 at StepForwardtoCureTSC.org.

Step Forward to Cure TSC is the TSC Alliance's largest annual fundraiser and awareness event. You can participate in many ways:

- **Virtual auction** – donate or participate in this year's virtual auction. 100% of the funds raised from your donation gets credited to your registered team
- **Donate** – make a one-time online donation to support a participant or team
- **Register** – online, create a team and solicit team members, donors and/or sponsors

- **Sponsorship** – download a regional sponsorship opportunity packet at stepforwardtocurets.org and approach or provide contacts for potential sponsors

- **Volunteer** – join community leaders from across the country to support this year's event

Fantastic prizes and contests abound! All registered participants are automatically entered to win a grand prize randomly selected during the Wrap Up Rally on Sunday, May 15. You do not be present to win!

Contact Gail Saunders at gsaunders@tsalliance.org with any Step Forward to Cure TSC questions.

National sponsors



Government advocacy

Appropriations process stalled, \$8 million for TSCRP still pending

Last July, the House Committee on Appropriations approved the fiscal year 2022 Defense Appropriations Act, which includes an appropriation of \$8 million for the Tuberous Sclerosis Complex Research Program (TSCRP). The House had planned to bring the bill to the floor prior to its summer recess, but instead postponed action until the fall.

Unfortunately, Congress has not taken any further action on the FY22 Defense bill, or any of the remaining spending bills needed to keep the government funded. Congress nearly averted a government shutdown that would have occurred on December 3 by passing a "continuing resolution" to keep the government funded through February 18, 2022. This means final action on FY22 funding for the TSCRP will not likely occur until early 2022.

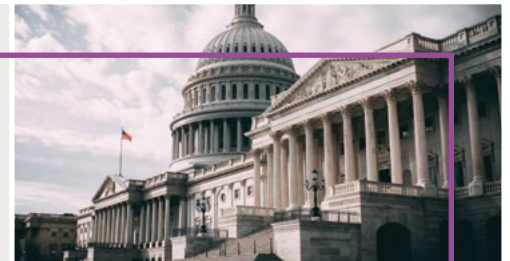
The TSC Alliance® remains confident Congress will ultimately approve the FY22 Defense Appropriations Act and continues to remain engaged with our champions in Congress.

Several bills introduced to create new ARPA-H

The President's budget proposes the creation of a new Advanced Research Projects Agency for Health, or ARPA-H. This new agency would seek to "accelerate the pace of scientific breakthroughs that have the potential to transform health care and address our most complex health challenges."

Several bills were introduced this fall to formally authorize the establishment of ARPA-H. The Cures 2.0 Act, which is an update of the 21st Century Cures Act, was introduced by Representatives Diana DeGette (D-CO) and Fred Upton (R-MI). This legislation would authorize \$6.5 billion in funding for ARPA-H and create it as a separate institute within the National Institutes of Health (NIH). The Advanced Research Project Agency-Health Act, introduced by Representative Anna Eshoo (D-CA), would authorize \$3 billion for the agency, but would establish it outside of NIH as a free-standing entity reporting directly to the Secretary of Health and Human Services (HHS).

It is possible one of these bills, or components of both, will be attached to the final FY22



Labor-HHS-Education Appropriations Act, the funding vehicle for NIH. The House version of this legislation appropriates \$3 billion in FY22 for ARPA-H. Once enacted and established, ARPA-H could serve as a new source of funding for research on TSC, particularly for high-risk, high-reward research that would not normally be funded through traditional NIH mechanisms.

Participate in our virtual March on Capitol Hill

You can join volunteers from across the United States by participating in this year's virtual March on Capitol Hill, scheduled for February 22-25. Securing Federal TSC research funding for the TSCRP every year is crucial, and you can easily help from the convenience of your home or office – we'll even train you on how to do it!

To find out more and get involved, simply email us at grc@tsalliance.org today.



Transition: an emotional journey for semi-dependent and dependent adults with TSC

Many families face daily struggles living with TSC, which often include multiple disabilities. The day-to-day impact of seizures, brain surgeries, TSC-associated neuropsychiatric disorders (TAND), therapies, medical appointments, ensuring access to needed medication and endless mounting piles of paperwork to ensure all appropriate supports are in place can be all consuming. It's hard to think about tomorrow let alone five or 10 years from now when children reach the age when transition to adulthood begins, but it's important to start early.

The purpose of the Individuals with Disabilities Education Act (IDEA) is "to ensure that all students with disabilities have available to them a Free Appropriate Public Education (FAPE) that emphasizes special education and related services designed to meet their unique needs and prepare them for further education, employment, and independent living" (300.1). A vital part to the foundation of this support is the Individualized Education Program (IEP). This evolving document is in place to ensure your child has obtainable annual goals and, with the appropriate supports and services, to make meaningful progress in light of the circumstances.

Regardless of their disabilities, all children eventually become adults – physically and legally. A major shift occurs for students with disabilities as they leave the structured setting of high school (when they lose the support of their entire education team) and enter the "real" world. The demands for self-determination, organization and independence are suddenly much greater. It's important to learn about the big changes that will occur in nearly every aspect of their lives and the steps in the process, especially for children with complex neurological conditions like TSC.

Part of the IEP process includes transition services and planning, which can never be started too soon. Some states begin this planning at age 14, but all states must implement at age of 16. The purpose of transition planning is to look ahead at your child's future life, set goals and decide which supports and services are needed to be successful.

The first step is writing a vision statement for your child, fully understanding many adjustments may be needed along the way. This vision forms the foundation of support and services needed to reach future goals after your child ages out of the education system. For those who have more direct care needs, there will be a concentrated focus on day programs and assisted living with significant supports in place. Some questions to address include:

1. What quality of life you envision for your child as an adult?
2. What community engagement will they be able to access?
3. What support will they need?
4. What do you want to prioritize for your child in their adult life?
5. What are their strengths and weaknesses?

Below TSC families in the community share their experience on how they approach transition:

“We hope our son can live in a home with a few others who he enjoys (or tolerates well!) that is connected to a larger community with typical adults involved since he loves attention from others. We envision this community being on or near a farm or larger property since he enjoys weeding and hoping this could lead to more

vocational skills. We also hope he is not too far from us so we can still have him home on weekends and visit him. If I could create the perfect day program it would involve a few different activities such as gardening, art, hiking and a sport such as bowling. He needs encouragement to try new things, but he is happiest when he has fun activities and can get a bit bored with too much repetition. I have spent time looking into purchasing property with others or joining with other groups who are planning this, but we have yet to find a situation that seems ideal for him. Our biggest obstacle is he doesn't love to be around others with needs greater than his (we are not even sure if he would like other individuals like himself!) so finding housemates will be a challenge." —Anonymous mother to 21-year-old with TSC

Our vision and hope for our daughter is to be engaged in local programs with weekly outings and gatherings with peers. We want to be able to manage our daughter's care outside our family home, while we are not too far into our senior years. Finding adult physicians/specialists who are informed and experienced with TSC is a real challenge and can feel like starting over with medical care. Fortunately, our TSC network includes parents who can share their own path and research. Our main goal is to continue to keep our daughter active daily with outings and things she enjoys such as dance class and gardening with her aide. She cannot form relationships or care for herself or her medical needs. Anxiety and seizure control are our biggest issues to date, so having a consistent and predictable schedule is essential for her." —Anonymous mother to 20-year-old with TSC

My child is on the severe side of the spectrum. His seizures were never controlled, even after three brain surgeries and medications. He is severely developmentally delayed and requires assistance for all basic daily activities. Every year we see improvements but for him to be a totally independent individual is ruled out. I hope government and or non-governmental organizations come up with plans to support adults who are most impacted by disability. We try to give him the best support we can and have reduced the level of expectations to a minimum. We plan to set up a special needs trust and to explore assisted living facilities, although it's hard to digest he might have to live in a facility. Planning to establish a caretaker who can assist him after we are gone, put money aside in a trust, set him up for all the care and assistance he can get are the next things to do. Independence is key and one of our main goals for our son." —Noby Joseph, father to 14-year-old Nipun

When our daughter was first diagnosed with TSC, we hoped she would be among those not severely impacted. But at 13, she has just about every manifestation of TSC. We have adjusted our plans, hopes and

dreams for her many times and will likely continue to do so as she changes. While she's made great progress, we now understand she will need significant support the rest of her life. I've heard transition planning starts in kindergarten, but what if you have a child who is constantly changing? Things like brain surgery, medication changes and seizure activity can affect the TSC individual's development and abilities. To alleviate my anxiety about her future, I started educating myself on transition planning and post-secondary options early on. The more I read, the more concerned I became I could not see my child in any of the future planning mentioned. Our friends and family with typical children talk and plan for being empty nesters, sending children off to college, attending programs abroad or starting families. Even families with less impacted children with disabilities plan for post-secondary education, living options and programs. Most of these options are likely off the table for our daughter. Where will she live? Who will be her voice? How would I assure her safety and community engagement? Will she be isolated? Abused? Neglected? Who will know my child as well as I do? It's quite terrifying!" —Lilian Ansari, mother to 13-year-old Atrina

The trust advisory committee

One way to assist and identify the areas of need for your loved one is to create a trust advisory committee. Acting as trustee for an individual with complex needs is a huge responsibility, and one person may not possess all the knowledge and skills needed for the job. Families sometimes create this committee to help guide decision making and care for their dependent adult.

Trust advisory committees typically consist of three to five individuals who can contribute a range of perspectives: a friend or relative, attorney, care manager, financial advisor and/or health care professional. Depending upon the trust's terms and the committee's composition, it may be authorized to:

- share insights concerning the beneficiary's needs
- assess trust investments
- offer advice concerning distributions
- assess suitability of various government benefit programs
- hire and fire trustees, based on responsiveness and performance
- amend the trust if laws, regulations, or the beneficiary's situation change

Making sure the individual is not isolated can be among the advisory committee's responsibilities. Be knowledgeable on your state's regulations on the age of majority, when an individual turns 18 years old, to ensure you are familiar with the appropriate steps needed for conservatorship or guardianship. Also become familiar with local resources in your community and connect with local special needs groups or agencies that can assist. The TSC Alliance has a dedicated set of volunteers who have knowledge in the transition process and are a great resource. You can contact Shelly Meitzler at smeitzler@tscalliance.org to be connected in your area.



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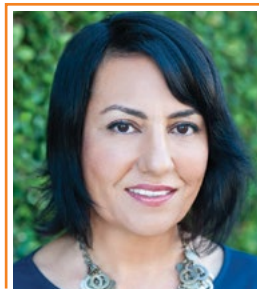
Don't miss the 2022 World TSC Conference!



The 2022 World TSC Conference is fast-approaching. With 110 experts on deck, the conference will offer a comprehensive agenda that will address the needs of any individual and family affected by TSC. We anxiously await the participation of more than 1,000 attendees in Dallas, Texas on July 28-31, 2022.

Because the TSC Alliance often hears the World TSC Conference is “life-changing,” we asked attendees from the 2018 World TSC Conference to share their experience.

“The 2018 World TSC Conference was the perfect mix of information, support, connection and fun! To be able to sit in a room with so many TSC experts and have the opportunity to hear about their latest research and ask questions was incredibly useful. Then in the evenings, sitting across the table from other families I’d only seen on social media, running into them at the pool or in the lobby and to meet new families was a truly memorable experience.



Lilian Ansari

At the end of the week, I felt empowered by the information I’d received and elated from all the personal connections I’d made. I can’t wait until the next conference in July!” — Lilian Ansari, TSC Mom

“Initially, we didn’t know what to expect from this conference. It seemed a bit overwhelming at first when we looked at the program agenda and the number of sessions happening at the same time, and we couldn’t decide which sessions to attend. However, once we settled in and met other parents with similar journeys,

the conference seemed much more manageable.

It does help if you stay at the hotel, because it allows you time to meet other parents and enjoy the site’s amenities in a more social environment. It was great to have all the experts under the same roof at one conference providing knowledge in their areas of expertise. The conference also helped us realize we are not alone in this journey.

We found the small group get-togethers with multiple physicians doing question/answer sessions to be the most valuable part of the conference. Smaller sessions allowed everyone to ask more specific questions that are better tailored to our own personal journeys. Physicians were also very generous to even sometimes answer personal questions one-on-one. “ —Noor Panjwani, TSC Dad



The Panjwani family

“The most valuable part about attending a World TSC Conference is that you can meet people from all walks of life, from all over the world, who are on the same journey as you – whether you are a parent or guardian of a child with TSC, a TSC adult or a doctor in the field. You will have the chance to listen to so many stories of hope that will inspire and empower you in your TSC journey.

Attending the 2018 World TSC Conference was quite incredible as it was my first one. From the lectures and

workshops to interactions with TSC families, young adults and more, there are so many ways we can learn from each other as one global TSC community. The formal dinner held Saturday night, which reflected on the achievements and how far the TSC community has come, was a moment I always cherish, with so many smiles by families and young adults. But most importantly, I will never forget the people I met throughout the conference.

This conference means a lot to me. It gives me the opportunity to engage with people from all over the world living with TSC just like me. Meeting people from other parts of the world with one common goal to find a cure is amazing as is seeing people from all over with so much compassion, love and understanding for one another. It's quite humbling as I want to be more involved with the global TSC community even more." —Daniel Price, Young Adult with TSC



Daniel Price

greeted and hugged others as if we knew each other our entire lives. We all felt connected on many levels.

Our family was not able to utilize the Day Camp due to our son having an increase in drop seizures during the conference. We knew other families who used the camp, and they spoke very highly of the staff members. Parents were able to attend sessions with a peace of mind with less worrying. Many TSC kids and their siblings met new friends and was able to enjoy the creative activities. One parent shared with us how her TSC child did not have friends, so to see her child so happy enjoying her new friends brought her so much joy. The camp allowed time for the whole family to attend the conference. This was also an opportunity to enjoy respite time without the kids.

The TSC Alliance works hard to offer these conferences so TSC families and TSC medical professionals can join together to share current research, knowledge and strategies to help ensure a better quality of life with support, understanding and compassion. The sessions provide invaluable information for each stage of life as we face TSC across the lifespan. The TSC Alliance is a brilliant organization that offers so much hope no matter the complexities of TSC." —Latrice Hamilton, TSC Mom

"The 2018 World TSC Conference was so amazing. The most important thing our family took from the conference was the importance of self-care. The Hamiltons relaxed



The Hamilton family

and enjoyed as best as we could. We moved at our son Terrence's pace and we did not attend every session, but we totally understand our unique family situation. Terrence's favorite part was the lazy river at the hotel's water park with an extra bonus of spending time with his favorite doctors and nurses. Now how cool is that? We really enjoyed ourselves as parents hanging out with other TSC moms and dads, and so many priceless memories were made! An extra special moment for our family was the presentation of Terrence's story during the World TSC Conference by his doctor, Dr. Tanjala Gipson of Le Bonheur Children's Hospital. Terrence had an opportunity to speak and said, 'I am not hitting Momma anymore. I ask for a hug now.' We truly hope his journey inspires others.

In my opinion, the most valuable part of attending a World TSC Conference is seeing and interacting with other TSC individuals and their family members. It was refreshing to hear their stories and individual journeys, then leaning how they coped while sharing effective strategies that made a difference in their lives. Our family realized that we were not alone in our TSC journey, so we

"I am blessed to be 65 years old and have been married for 45 years. We are parents of two children, and grandparents joyfully waiting for our fourth one due in March! I have been volunteering for more than four decades as part of our personal



The Henkel family

TSC journey, including currently as a TSC Alliance Adult Regional Coordinator. Through the years I have witnessed so many changes and so much growth in all areas with the TSC Alliance. There have always been caring and hard-working efforts every day. However, the work put into each World TSC Conference is simply unbelievable. I have heard from so many who attend, and no one who participates leaves the same. Tired yes, but in a good way!

TSC experts from around the world present on a wide variety of topics selected just for you. Every session is packed full for both new parents and adults with TSC like me. You'll gain a wealth of knowledge about TSC from the best of the best.

Because needs vary from person to person, these conferences offer a wide range of educational sessions and being able to speak to a TSC doctor one-on-one or during a roundtable is simply priceless. By the time of the 2018 World TSC conference, my daughter was an adult

too, and it was so great to see her meet other adults with TSC. After many years of dealing with TSC alone, the fact my husband attended with me was so needed. And after 10 years of their marriage, our son-in-law went with our daughter. Observing and being a part of this was special. So, after most of my lifetime on this journey, I have almost come full circle.

Watching all the new parents learn and bond together, which I never had the opportunity to do, was awesome of course. However, seeing the adults with TSC gain a support system of understanding and acceptance is what I treasured the most, because I spent 41 years working toward this and personally know the true need for adults to come together. It is as important as the medical and physical aspects of the day-to-day living with the challenges, struggles and obstacles of TSC into the years of adulthood.

Now, so many independent and semi-independent adults with TSC have more awareness and responsibility of their own lives. This is an extra bonus, along with one's regular life. Simply put, any extra help and knowledge derived from the World TSC Conference, combined with its social aspects, are a blessing and a win-win for everyone. If you can attend I recommend it, and if not, please take advantage of the Hybrid registration option. Take one day at a time and keep on keepin' on!" —Kathy Henkel, Adult with TSC

Plan now to join us in Dallas

The 2022 World TSC Conference is an event for everyone touched by tuberous sclerosis complex. The TSC Alliance sincerely hopes the TSC community will join us in Dallas for an unforgettable experience. We can't wait to see you at the 2022 TSC family reunion!

For more information, to register and to book a hotel room, visit tscalliance.org/2022-world-tsc-conference.

Can't attend in person?

The 2022 World TSC Conference offers a Hybrid registration option to allow those unable to attend in person to participate virtually. The cost for the Hybrid option is \$50 and registration includes the following livestreamed events: three general sessions, two Q&A sessions at the end of Friday and Saturday and three of the five different learning paths (1. panel presentations, 2. large group lectures and 3. specific topic presentations). By virtually attending, you can ask questions in real time and gain exclusive access to recordings for six months.

These sessions will also be videotaped and made available on the TSC Alliance website in January 2023.

More information about the 2022 World TSC Conference

The TSC Alliance® is excited to present the 2022 World Tuberous Sclerosis Complex (TSC) Conference, July 28-31, 2022, at the Hilton Anatole Hotel in Dallas, Texas, co-hosted by Tuberous Sclerosis Complex International (TSCi). You may wonder why we are back in Texas, and the answer is simple – the TSC Alliance signed a contract three years ago

following the hugely successful 2018 World TSC Conference, because the hotel offered us a magnificent deal to return.

The conference will bring together 1,000 participants from around the globe, including families and individuals with TSC, caregivers, healthcare professionals, researchers, a wide array of exhibitors and others.

Conference Covid-19 protocols and guidelines

The World TSC Conference planning team is keeping a close eye on recommended CDC guidelines to provide a safe environment for everyone during this experience. Specifics about protocols and guidelines will be provided on this website and will meet or exceed all CDC recommended convention and meeting protocols at the time of our gathering. The TSC Alliance's primary goal is to ensure the health and safety of our attendees, speakers, staff and volunteers, and we appreciate your partnership as we navigate the most updated news, research, and advice from our medical team.

Likely safely protocols may include the following: All those who are attending the World TSC Conference will be required to follow existing CDC guidelines in effect at the time of the conference. And, for the protection of our community members who are immunocompromised, in fragile health or battling LAM, all who are over the age of two years old will be required to wear a mask over their nose and mouth when indoors if they are medically and physically able to do so. Pursuant to the TSC Alliance's statement regarding COVID-19 (see tscalliance.org/COVID-19), we strongly encourage vaccination prior to attending the conference.

Registration and rates

Conference registration opened January 3, 2022. Please note: All-inclusive conference registration fees are per adult and cover educational sessions; breakfast, lunch and dinner; break snacks; and Exhibit Hall access. Day Camp includes meals for all participants. If you have a dinner-only guest

attending, it is \$35 per meal. If you have any questions, please call the TSC Alliance toll-free at (800) 225-6872.

A newly added Hybrid option is available for attendees who want to attend virtually. Hybrid registration is \$50 to attend the entire conference via livestreaming from your home or office.

	Full Conference	Two Days	One Day	Hybrid
Early Bird Registration Before June 1, 2022	\$275	\$225	\$125	\$50
Registration After June 1, 2022	\$375	\$300	\$165	\$50

Register online at tscalliance.org/2022-world-tsc-conference today.



Our host hotel

Our host hotel, the Hilton Anatole, will be ready and waiting to welcome our group on July 28. Located in Dallas, Texas, the Anatole is situated on 45 acres of exquisite property in the Design District. It has every amenity needed for a luxurious vacation including an on-site water park, spa, and health club. Their fine art collection has more than 1,000 pieces throughout the hotel, providing a culture outing without ever leaving the property.

Our special rate is \$183/night plus tax for a single or double occupancy room, and \$193/night plus tax for a triple or quad occupancy. Suites and room upgrades are available for an additional cost. TSC registered guests of the Hilton Anatole will enjoy amenities listed below for a daily discounted mandatory resort charge of \$12 plus tax.

This daily resort charge includes:

- Self-parking for one car
- Guest room WiFi (up to three devices)
- Access to Verandah Health Club (ages 16+only)
- 15% discount on V-Spa services
- Kids under 12 eat dinner free in Counter Offer with an adult entrée purchase
- Dessert with dinner at Counter Offer
- Two Starbucks Drip Coffees per day
- Local/toll free 1-800 phone calls
- Access to Jade Waters

The location allows for easy access to two airports: Dallas Love Field (10 minutes) and Dallas-Fort Worth International (20 minutes), simplifying travel if you are flying.

The staff at the Anatole will be equipped in emergency preparedness and briefed on the special concerns facing our group.

Scholarships

Thanks to the generous support from the Foglia Family Foundation and the TSC Alliance Endowment Fund to the Bcureful Travel Fund, the TSC Alliance will provide a limited number of scholarships in memory of Ken Johnson to individuals or families who might otherwise not be able to attend the 2022 World TSC Conference.



Scholarship applications are available online at tscalliance.org/2022scholarships. All applications are due by March 1, 2022. Scholarships will be awarded March 15, 2022. Scholarship funds can be used to cover the cost of registration, hotel, day camp and one-on-one aides. Travel expenses are not eligible and must be covered by the recipient. International applications are welcome.

Day camp

Campers will be registered during the online registration process. You must complete all camper information with your registration. Cost is \$100 per camper, which averages to \$40 per day per camper for a full day and \$20 for the half day on Sunday and includes all meals (breakfast, lunch, dinner, and breaks.)

Corporate Kids Events (CKE) will be running the Day Camp. CKE successfully ran the day camps at the 2006 National TSC Conference in Chicago, 2014 World TSC Conference in Washington, DC and the 2018 World TSC Conference in Dallas. They have also operated similar camps for other organizations like the TSC Alliance over the years



and regularly provide childcare at the Hilton Anatole. Please visit conferencechildcare.com for more information.

Camp options

Camp Discovery: This camp is for children with TSC who are functioning at an age-appropriate level as well as unaffected siblings of a TSC child.

Ratio of campers to staff in Camp Discovery:

Infants 0 to 2 Years	1:2
Children 3 to 5 Years:	1:4
Children 6 to 12 Years:	1:6
Children 13 to 17 Years:	1:8



Camp Explorer: This is the special needs camp for TSC-affected children with moderate to significant needs. Children who require assistance with ADLs, communication, mobility, behavior management or seizure control all will be best served through Camp Explorer.

Camp Explorer is staffed at a 2:1 ratio for all ages. 1:1 staffing is available for an additional fee of \$225 per camper for the full conference or \$90 per camper per day.

Research room

The 2022 World TSC Conference will feature a Research Room dedicated to providing attendees a place to learn about ongoing research studies or clinical trials and how they can participate. As part of this new feature, the TSC Alliance will offer on-site blood collections for the TSC Biosample Repository and Natural History Database projects. Blood draws will be performed for individuals with a TSC diagnosis.

Attendees interested in participating will be able to note their interest in participating on the conference registration form. A TSC Alliance research staff member will follow up with further details. If you are not currently participating in the TSC Biosample Repository and Natural History Database project, our research staff will arrange to complete the consent process prior to your arrival at the conference.

A total of 17 hours will be available for attendees to complete their blood draws. If you have questions about the 2022 TSC World Conference Research Room, please email Gabrielle Rushing, PhD, Associate Director, Research, at grushing@tscalliance.org.

Dependent Adult Camp: This is a camp for dependent adults with TSC with moderate to significant needs. Activities will be individually established based on the camper's interests and will include as much as the camper is able to try.

The Dependent Adult Camp is staffed at a 2:1 ratio. 1:1 staffing is available for an additional fee of \$225 per camper for the full conference or \$90 per camper per day.

Sponsors

The 2022 World TSC Conference is grateful to the event's supporters, including Title Sponsor Jazz Pharmaceuticals and Presenting Sponsor Nobelpharma.

Questions

Each staff member plays a crucial role in conference preparations; main contacts are listed below.

- **General Conference Questions (Including Day Camp)**
Shelly Meitzler, smeitzler@tscalliance.org, (800) 225-6872 x 232
- **Sponsorship & Exhibitor Inquiries**
Lisa Moss, lmoss@tscalliance.org, (800) 225-6872 x 222
- **Media Inquiries**
Jaye Isham, jisham@tscalliance.org, (800) 225-6872 x 213
- **Research Room Inquiries**
Gabrielle Rushing, PhD, grushing@tscalliance.org, (800) 225-6872 x 150

Visit tscalliance.org/2022-world-tsc-conference for more information and to register. We look forward to seeing you in Dallas!

Conference agenda

Draft (All speakers invited and subject to change.)

The 2022 World TSC Conference will offer general sessions with keynote speakers each day, panel presentations, workshops, large group lectures, specific topic presentations and discussion groups. This multi-pronged approach will allow the TSC community to select engagement opportunities, whether you are parents of a newly diagnosed infant or an adult with TSC. Sessions will cover topics such as medical challenges, behavioral management, life skills, community support, transition issues and educational resources as well as financial considerations.



Five different learning paths

Large group lectures (livestreamed) will feature three to four speakers with a moderator and include formal presentations on topics that impact individuals across the lifespan with some time at the end for Q&A.

Panel presentations (livestreamed) will feature three to four speakers with a moderator who will ask questions to engage interactive conversation among the panel and attendees to address specific areas of need.

Specialty topic lectures (livestreamed) will feature two or more speakers with a moderator to focus on specialty topics around accessing, navigating and securing resources and information required for the complex needs of an individual across the life span.

Workshops (non-recorded) will include an interactive working group featuring three to four speakers, a moderator and a key take-away learning objective with supporting materials with a specialized focus on the complex needs in the TSC community.

Discussion groups (non-recorded) will feature 2-3 speakers and a moderator to discuss more personal topics that affect a subset of the TSC community in a closed setting.

In addition to the learning paths above, three general sessions will be offered for all in-person and virtual attendees as well as meet-the-experts (Q&A sessions with expert physicians).

WEDNESDAY, JULY 27, 2022

6:00 p.m. – 9:30 p.m.

TSCi Reception, by invitation only
Fleur de Lis A & B

THURSDAY, JULY 28, 2022

8:00 a.m. – 2:00 p.m.

Nursing Symposium, by invitation only
Plum Blossom

9:00 a.m. – 2:00 p.m.

TSCi Workshop, by invitation only
Fleur de Lis A & B

12:00 p.m. – 5:00 p.m.

Biosample Repository Blood Draw
Inverness

12:00 p.m. – 6:00 p.m.

General Conference Registration and Day Camp Registration
Conference Registration Room and Atrium II

2:30 p.m. – 4:00 p.m.

TSC Clinic Directors Meeting, by invitation only
Plum Blossom

3:00 p.m. – 6:00 p.m.

Exhibit Hall Set Up
Grand Ballroom C

3:00 p.m. – 5:00 p.m.

Meet-and-Greet Opportunities

Adults with TSC

Ballroom A

Hosts: Derek Bauer, MD; Rob and Shannon Grandia; Kathy Henkel; Jake Irby; Melyssa McDonough, MS, BCBA; Mary Ann Scroggins



Adult Siblings

TBD

Hosts: Jocelyn Cenna, Carlie Russell

Grandparents

CounterOffer

Hosts: Bill and Deb Joseph, Mary Ann Lamb

International Attendees

Fleur de Lis A & B

Hosts: Carla Fladrowski, Mica Rozenberg

Parents of Adults with TSC

CounterOffer

Hosts: April Cooper; Dana Holinka; John Hulbert, MD; Leslie Russell

Parents/Caregivers of Transitioning Young Adults

Jade Room

Hosts: Lisa Carlton, PhD; Steve Peters

Parents/Caregivers of Young Children with TSC (ages 0-13)

East Atrium

Hosts: Ryan and Andrea Beebe, Lesley and Alex Holmes, Phil Tallman

Social Networking Groups (Inspire, Instagram, Facebook)

Jade Pavilion

Hosts: Danielle Clark, Carly Kaye, Lauren Shillinger

Teens and Young Adults (ages 13-27),

Lalique

Hosts: Preston Fitzgerald, Jonathan Goldstein, Rylee Grandia, Celie Johnson, Kirk McCreary, Presley Mylar, Emily Racenet



4:00 p.m. – 5:00 p.m.

TSC 101 for Newly Diagnosed Individuals or Families

Edelweiss

Moderator: Debora Moritz

Speakers: Angela Peron, MD; Rajsekar Rajaraman, MD

6:30 p.m. – 8:30 p.m.

Conference Welcome and Opening Dinner

Imperial Ballroom

TSC Alliance President & CEO Kari Luther Rosbeck; TSC Alliance Board Chair Peter Crino, MD, PhD; and Local TSC Clinic Directors Steve Sparagana, MD and M. Scott Perry, MD

8:30 pm

Gathering for International Attendees

Media Grill + Bar

Adults with TSC Evening Activity

Plum Blossom

TSC Dad's Outing

Gossip Bar

Event Leader: Sean Shillinger

8:30 p.m. – 11:00 p.m.

Teen Lounge Open

Lalique

FRIDAY, JULY 29, 2022

7:00 a.m. – 8:30 a.m.

Breakfast

East Atrium

Biosample Repository Blood Draw

Inverness

Research Room Open

Fleur de Lis B

7:30 a.m. – 5:30 p.m.

Day Camp

Atrium I

Check-In: Stemmons Ballroom

8:00 a.m. – 5:00 p.m.

Exhibit Hall and TSC Resource Center

Grand Ballroom C

8:00 a.m. – 12:00 p.m.

Conference Registration

Conference Registration Room and Atrium II

8:30 a.m. – 10:00 a.m.

Opening General Session:

Global Collaborations (TANDem) and Updated Consensus Guidelines

Grand Ballroom D & E

Welcome: Peter B. Crino, MD, PhD, Chair, TSC Alliance Board of Directors
Moderator: Pradnya Gadgil, MBBS, MRCPCH

Family Speaker: Eva Schoeters

Speakers: Petrus de Vries, MBChB, MRCPsych, PhD; Hope Northrup, MD

10:00 a.m. – 10:30 a.m.

BREAK

10:00 a.m. – 2:00 p.m.

Biosample Repository Blood Draw Inverness

Research Room Open

Fleur de Lis B

10:30 a.m. – 12:00 p.m.

First Break Out Session

PANEL: Navigating the Complexities for Special Needs Care in TSC (SSI, Waivers, Medicaid, Access, Financial Planning and Guardianship)

Grand Ballroom A

Moderator: Lisa Szilagyi

Panelists: Kelly Piacenti; Nadia Vann, JD; John Hulbert, MD

SPECIALTY: Using Technology to your Advantage - Seizure Monitoring, Communication Devices and Futuristic Medical Technology

Grand Ballroom B

Moderator: M. Scott Perry, MD

Speakers: Debora Moritz, Rob Moss, Rahul Viparathi

LECTURE: Seizure Types and Treatments

Grand Ballroom D & E

Moderator: Rohini Coorg, MD

Speakers: Mark Keezer, MDCM, PhD; Elizabeth Thiele, MD, PhD; Jim Wheless, MD

WORKSHOP: Organizing Your Thoughts and Emotions and Managing Relationships (TSC individual)

Plum Blossom

Moderator: Mary Kay Koenig, MD

Adult with TSC Panelists: Danielle Clark, Emilie Hyde, Foster Hyde, Phil Tallman
Speakers: Jamie Capal, MD; Petrus de Vries, MBChB, MRCPsych, PhD



DISCUSSION: Preparing Your Family for Epilepsy Surgical Evaluation and Surgery

Edelweiss

Moderator: Brenda Porter, MD, PhD
Speakers: Bridget Britt; Gerald Grant, MD; Dave and Rachel Johnson

GROUP SESSION: Sibling Workshop
Cooper

12:00 p.m. – 1:00 p.m.

Box Lunches
Ballroom Foyer

LUNCH AND LEARN: TSC 101 Part II – Caregiver Guide to Care for Your Loved One for Newly Diagnosed

Plum Blossom

Panelists: Debora Moritz, Kelly Piacenti, Leslie Russell

LUNCH AND LEARN: Caregiver Mental Health and Wellbeing

Ballroom A

Moderator: Tanjala Gipson, MD
Panelists: David Dunn, MD; Carla Fladrowski; Mica Rozenberg

12:00 p.m. – 1:00 p.m.

Global Alliance Meeting, by invitation only

Fleur de Lis A

12:00 p.m. – 5:00 p.m.

Teen Lounge Open
Lalique

1:00 p.m. – 2:30p.m.

Second Break Out Session

PANEL: Surgical Approaches and Assessments for Epilepsy and SEGA
Grand Ballroom A

Moderator: Dan Curry, MD
Panelists: Jeffrey Blount, MD; Gerald Grant, MD; Asim Choudhri, MD; Howard Weiner, MD

SPECIALTY: Accessing Managed and Palliative Care Throughout Your TSC Journey

Grand Ballroom B

Moderator: Ashley Pounders, MSN, FNP-C
Speakers: Sarah Friebert, MD; Nishant Gupta, MD; Josh Samuels, MD, MPH

LECTURE: Skin and Dental Manifestations and Treatment Options

Grand Ballroom D & E

Moderator: Klaus Werner, MD, PhD
Speakers: Elizabeth Gosnell, DMD, MS; Joyce Teng, MD, PhD

WORKSHOP: Preparing for Transitions - Childhood to Adulthood (Medical Care)

Plum Blossom

Moderator: Michael Frost, MD
Speakers: Peter Crino, MD, PhD; Dana Holinka; Mustafa Sahin, MD, PhD; Rebecca Schultz, PhD, APRN, CPNP

DISCUSSION: Women's Health Issues for TSC Adults

Edelweiss

Moderator: Brenda Porter, MD, PhD
Speakers: Kathy Henkel; Lisa Henske, MD; Monika Madan, MD, MSc, CCFP; Hope Northrup, MD; Lara Friel, MD, PhD

2:30 p.m. – 3:00 p.m.

BREAK

3:00 p.m. – 4:30 p.m.

Third Break Out Session

PANEL: Accessing Early Childhood Services and Non-Medical Therapies
Grand Ballroom A

Moderator: Rohini Coorg, MD
Panelists: Joanna Lomas-Mevers, PhD, BCBA; Shelly Meitzler, Ashley Pounders, MSN, FNP-C; Kim Street, LMSW, LPN, CCM

SPECIALTY: LAM, Treatment Options and Clinical Trials

Grand Ballroom B

Moderator: Sue Sherman, MHA

Speakers: Nishant Gupta, MD; Lisa Henske, MD; Joel Moss, MD, PhD

LECTURE: Genetics Made Simple
Grand Ballroom D & E

Moderator: Steve Sparagana, MD
Speakers: Jennifer Glass, MS, CGC, LGC; Hope Northrup, MD; Zacil-Ha Vilchis Zapata, MD

WORKSHOP: TAND - Aggressive Behavior Management

Plum Blossom

Moderator: Peter Davis, MD
Speakers: Nathan Call, PhD, BCBA-D; David Dunn, MD; Tanjala Gipson, MD; Leslie Russell

DISCUSSION: Men's Health Issues for TSC Adults

Edelweiss

Moderator: Steve Roach, MD
Speakers: Derek Bauer, MD; Rob Grandia; Billy Holcombe, PhD, Jake Irby

4:30 p.m. – 4:45 p.m.

BREAK

4:65 p.m. – 5:30 p.m.

GENERAL QUESTIONS AND ANSWERS (Panel of Presenters)

Grand Ballroom D & E

Moderator: Kari Luther Rosbeck
Panelists: Nathan Call, PhD, BCBA-D; Peter Crino, MD, PhD; Dan Curry, MD; Tanjala Gipson, MD; Nishant Gupta, MD; Chris Kingswood, MD; Hope Northrup, MD; Josh Samuels, MD, MPH; Joyce Teng, MD, PhD; Howard Weiner, MD; Jim Wheless, MD

5:30 p.m. – 7:00 p.m.

Spanish Educational Meeting and Gathering

Plum Blossom

Moderator: Darcy Krueger, MD, PhD
Speakers and Event Leaders: Ary Agami; Joao Garcia Martínez, MD; Vanessa Vazquez; Zacil-Ha Vilchis Zapata, MD

6:30 p.m. – 8:00 p.m.

Dinner

Imperial Ballroom

8:30 p.m.

Movie Night

Grand Ballroom D & E

Adults with TSC Outing

Plum Blossom

Multicultural Social, for people interested in multi-cultural issues

Gossip Bar

Hosts: Lisa Carlton, PhD; Danielle Clark; Latrice Hamilton; Vanessa Vazquez

8:30 p.m. to 11:00 p.m.

Teen Lounge Open

Lalique

Saturday, July 30, 2022

7:00 a.m. – 8:30 a.m.

Breakfast

East Atrium

Biosample Repository Blood Draw

Inverness

Research Room Open

Fleur de Lis B

7:30 a.m. – 5:30 p.m.

Day Camp

Atrium I – Check-In: Stemmons Ballroom

8:00 a.m. – 10:00 a.m.

Conference Registration

Conference Registration Room and Atrium II

8:00 a.m. – 5:00 p.m.

Exhibit Hall and TSC Resource Center

Grand Ballroom C

8:30 a.m. – 10:00 a.m.

GENERAL SESSION: TSC Community Driven Research Network (TSC Alliance Research Model)

Grand Ballroom D & E

Moderator: Martina Bebin, MD, MPA

Family Speaker: Latrice Hamilton

Speakers: Dean Aguiar, PhD; Sara Chieffo; Zoë Fuchs; Steve Roberds, PhD; Gabrielle Rushing, PhD

10:00 a.m. – 10:30 a.m.

BREAK

10:00 a.m. – 2:00 p.m.

Biosample Repository Blood Draw

Inverness

Research Room Open

Fleur de Lis B

10:30 a.m. – 12:00 p.m.

Fourth Break Out Session

PANEL: TAND – ADHD, Anxiety, Autism (Research)

Grand Ballroom A

Moderator: Mustafa Sahin, MD, PhD

Panelists: Nathan Call, PhD, BCBA-D; Jamie Capal, MD; Anna Jansen, MD, PhD; Shafali Jeste, MD; Melyssa McDonough, MS, BCBA

SPECIALTY: Caregiver Resources – Self and Respite Care

Grand Ballroom B

Moderator: Debora Moritz

Speakers: Kelly Piacenti; Karina Stormfeldt Helboe; Kim Street, LMSW, LPN, CCM

LECTURE: Kidney Tumors, Cysts and Treatment Options

Grand Ballroom D & E

Moderator: Chris Kingswood, MD

Speakers: John Bissler, MD; Elahna Paul, MD, PhD; Oded Volovelsky, MD, PhD

WORKSHOP: Seizure Action Plans and Rescue Meds

Plum Blossom

Moderator: Michael Frost, MD

Speakers: Peter Davis, MD; Pradnya Gadgil, MBBS, MRCPCH; Rob Moss; Rebecca Schultz, PhD, APRN, CPNP

DISCUSSION: Navigating Education Systems for a High School Certificate

Edelweiss

Moderator: Shannon Grandia

Speakers: Kate Crossett, Shelly Meitzler

12:00 p.m. – 1:00 p.m.

Box Lunches

Ballroom Foyer

LUNCH AND LEARN: Government Advocacy

Ballroom B

Moderator: Sara Chieffo, Debora Moritz, Katie Smith

LUNCH AND LEARN: Peer-to-Peer Support Programs

Ballroom A

Moderators: Shelly Meitzler

LUNCH AND LEARN: Special Events and Fundraising

Plum Blossom

Moderators: April Cooper, Gail Saunders

12:00 p.m. – 5:00 p.m.

Teen Lounge Open

Lalique

1:00 p.m. – 2:30 p.m.

Fifth Break Out Session

PANEL: Genetics, Reproductive Issues and Impact on Relationships

Grand Ballroom A

Moderator: Josh Samuels, MD, MPH

Panelists: Jennifer Glass, MS, CGC, LGC; Hope Northrup, MD; Angela Peron, MD; Lara Friel, MD, PhD

SPECIALTY: Refractory Epilepsy – Now What?

Grand Ballroom B

Moderator: Jim Wheless, MD

Speakers: Peter Crino, MD, PhD; Sergiusz Józwiak, MD, PhD; Elizabeth Thiele, MD, PhD; Mike Wong, MD, PhD

LECTURE: Access to TSC Clinical Care

Grand Ballroom D & E

Moderator: Steve Roach, MD

Panelists: Carla Fladrowski; Pradnya Gadgil, MBBS, MRCPCH; Joao García Martínez, MD; Darcy Krueger, MD, PhD;



Mica Rozenberg; Michal Tzadok, MD;
Oded Volovelsky, MD, PhD

WORKSHOP: Advocacy in the Education System at All Levels

Plum Blossom

Moderator: Kate Crossett

Speakers: Jennifer Flinn, EdD; Billy Holcombe, PhD; Shelly Meitzler; Nadia Vann, JD

DISCUSSION: TAND – Mental Health Issues in Living with TSC as an Independent and Semi-Independent Adult

Edelweiss

Moderator: Derek Bauer, MD

Speakers: Jamie Capal, MD; Petrus de Vries, MBChB, MRCPsych, PhD

2:30 p.m. – 3:00 p.m.

BREAK

3:00 p.m. – 4:30 p.m.

Sixth Break Out Session

PANEL: Finding your Way Home – Creative Housing Options/ Microboards

Grand Ballroom A

Moderator: Kelly Piacenti

Panelists: Deanna Ebel, Esq.; John Hulbert, MD; Nicole Jorwic; Lisa Szilagyi

SPECIALTY: Managing Progressive Kidney Disease

Grand Ballroom B

Moderator: John Bissler, MD

Speakers: Chris Kingswood, MD; Josh Samuels, MD, MPH

LECTURE: Gene Therapy – Hopes and Hurdles

Grand Ballroom D & E

Moderator: Mike Wong, MD, PhD

Speakers: Julian Agosto-Martinez, MD; Mustafa Sahin, MD, PhD; gene therapy company representative

WORKSHOP: TAND – Feeding, Elopement and Potty Training

Plum Blossom

Moderator: Nathan Call, PhD, BCBA-D

Speakers: Joanna Lomas-Mevers, PhD, BCBA; Mindy Scheithauer, PhD, BCBA-D; Valerie Volkert, PhD, BCBA-D

DISCUSSION: What's Next? Tools to Empower You on Your Medical Journey to an Adult Neurologist

Edelweiss

Moderator: Klaus Werner, MD, PhD

Speakers: Derek Bauer, MD; Elizabeth Thiele, MD, PhD

4:30 p.m. – 4:45 p.m.

BREAK

4:45 p.m. – 5:30 p.m.

GENERAL QUESTIONS AND ANSWERS (Panel of Presenters)

Grand Ballroom D/E

Moderator: Steve Roberds, PhD

Panelists: Derek Bauer, MD; John Bissler, MD; Petrus de Vries, MBChB, MRCPsych, PhD; David Dunn, MD; Gerald Grant, MD; Lisa Henske, MD; Shafali Jeste, MD; Sergiusz Józwiak, MD, PhD; Darcy Krueger, MD, PhD; Angela Peron, MD; Elizabeth Thiele, MD, PhD

6:30 p.m. – 8:30 p.m.

Blue Night Dinner

Imperial Ballroom

Host: Kari Luther Rosbeck

8:30 p.m. – 11:00 p.m.

Teen Lounge Open

Lalique

8:30 p.m.

Adults with TSC Outing

Plum Blossom

Event Leader: TBD

9:00 p.m.

TSC Mom's Outing

Gossip Bar

Event Leader: Lauren Shillinger

SUNDAY, JULY 31, 2022

7:00 a.m. – 8:30 a.m.

Breakfast

East Atrium

7:30 a.m. – 12:00 p.m.

Day Camp

Atrium I – Check-In: Stemmons Ballroom

8:30 a.m. – 10:00 a.m.

Seventh Break Out Session

PANEL: Other Organ Manifestations - Liver, Pancreas, Heart and Eyes

Grand Ballroom A

Moderator: Rajsekar Rajaraman, MD

Panelists: Mary Aronow, MD; Darcy Krueger, MD, PhD; David Rodriguez-Buritica, MD

SPECIALTY: Transition into Adulthood – Employment and Higher Education

Grand Ballroom B

Moderator: Julie Scroggins

Speakers: Deanna Ebel, Esq.; Preston Fitzgerald; Jonathan Goldstein; Celie Johnson; Kirk McCreary; Presley Mylar; Emily Racenet

LECTURE: TAND – Behavioral Interventions and Overview of Medications

Grand Ballroom D & E

Moderator: Billy Holcombe, PhD

Speakers: Petrus de Vries, MBChB, MRCPsych, PhD; David Dunn, MD; Tanjala Gipson, MD; Shafali Jeste, MD

WORKSHOP: Navigating Health Care System to Better Access Medication

Plum Blossom

Moderator: Mary Kay Koenig, MD

Speakers: Jessica Krefting, BSN, RN; Wynne Bird BSN, RN, CPN, CNRN; Ashley Pounders, MSN, FNP-C

DISCUSSION: Sexual Development for Dependent and Semi-Independent TSC Adults

Edelweiss

Moderator: Shelly Meitzler

Speaker: Monika Madan, MD, MSc, CCFP

10:00 a.m. – 10:30 a.m.

BREAK

10:30 a.m. – 12:00 p.m.

CLOSING SESSION: Driving the Future of TSC – Clinical Trials and Clinical Care

Grand Ballroom D & E

Moderator: Anna Jansen, MD, PhD

Family Speakers: Dave and Rachel Johnson

Speakers: Martina Bebin, MD, MPA; Sergiusz Józwiak, MD, PhD; Darcy Krueger, MD, PhD; Joel Moss, MD, PhD; Mustafa Sahin, MD, PhD

Closing Remarks: Kari Luther Rosbeck

**TAND stands for TSC-associated neuropsychiatric disorders.*

For complete details and online registration Visit tscalliance.org/2022-world-tsc-conference for more information, to register online and/or to make hotel reservations.

2021 Virtual International TSC & LAM Research Conference attendees focus on “driving discoveries beyond boundaries”



The 2021 Virtual International TSC & LAM Research Conference, held October 28-30, 2021, welcomed 179 people from 18 countries. Co-sponsored by the TSC Alliance® and The LAM Foundation, the conference featured three plenary sessions with 17 oral presentations and two poster sessions with 14 posters. Additionally, two half-day sessions of “topic-based discussion” brought together clinical and basic science researchers to discuss cross-cutting topics, including big data and clinical translation.

The keynote address was delivered by Daphne Koller, PhD, Founder and CEO of Insitro, Inc. Dr. Koller is best known for her work as a pioneer in the artificial intelligence (AI) field and as a thought leader in precise, targeted medicine. Her presentation, “Transforming drug delivery using digital biology,” introduced the concept of machine learning as an approach to drug discovery and development. Dr. Koller’s talk highlighted the benefits of machine learning for rare disorders like TSC, as biological data (MRI scans, pathology data, genetics) comprise massive datasets that are uninterpretable by humans because of their scale. While humans have a difficult time parsing these integrated datasets, computers can interpret subtle, previously unknown drivers of disease that might prove to be useful therapeutic targets.

This article summarizes a selected subset of presentations, and we regret not all talks and posters could be described in this overview.

Discovering new ideas for therapeutic targets and human cell-based models

Conference plenary sessions began with Kathrin Thedieck, PhD, whose lab at the University of Innsbruck (Austria) has been studying a protein crucial to understanding the similarities between cells lacking functional TSC proteins and normal cells reacting to external stress stimuli. This protein—G3BP—plays a role in the TSC-mTOR signaling cascade; it helps the TSC protein complex tether to lysosomal proteins so it is in the proper position to inactivate mTOR.

Dr. Vivi Heine and her lab at Amsterdam UMC have been studying human-derived samples and creating induced pluripotent stem cells (iPSCs) to examine neuronal activity. This research has shown it is possible to recapitulate TSC-associated phenotypes in cell-based models. This is important for understanding human disease biology and testing possible new drugs on cells derived directly from people with TSC.

Dr. Helen Bateup presented her work using organoids, which are three-dimensional assemblies of iPSC-derived cells composed of a mixture of the different types of cells found in the brain, to understand the early-developmental changes that lead to the formation of cortical tubers. Dr. Bateup’s work shows how dysregulation of mTOR signaling during early cortical development contributes to altered cellular differentiation and ultimately, the formation of tubers.

Predicting and preventing manifestations of TSC and LAM

Dr. Simon Johnson shared data from a retrospective analysis that utilized machine learning to examine LAM patient records and biospecimens. The methods he discussed could be used to potentially stratify care for individuals at risk for developing LAM, reduce uncertainty in predicting disease severity and improve overall outcomes.

Dr. Jamie Capal described predictors of autism from the TSC Autism Center of Excellence Research Network (TACERN) clinical study. Dr. Capal shared data showing a correlation between seizure burden and developmental outcomes. These

data, in tandem with other studies of early development and seizure onset, have provided a foundation for several clinical studies, including the currently enrolling TSC-STEPS trial (NCT04595513).

Breakout discussion groups

Conference attendees were invited to join afternoon topic-based discussions. Conference Co-Chairs Dr. Rebecca Ihrie and Dr. Nishant Gupta moderated panel discussions on Thursday and Friday, respectively. Following presentations and group Q&As, attendees were randomly assigned to breakout rooms to discuss the topics on a deeper level and ensure all attendees had an opportunity to engage in small-group discussions.

- **Extracting meaning from multi-omics datasets:** Presentations by Drs. Ihrie, Krymskaya, Xu and Davis focused on many different aspects of big data – ranging from introducing new tools researchers might find useful in their analyses, sharing datasets and open science and validating results using patient samples. Breakout discussions following these presentations focused on the need for a centralized repository of datasets specific to TSC and LAM researchers. Participants also suggested single-day workshops to teach researchers how to use and analyze big data, which would not only contribute to accelerating research but also would help develop early career researchers' skills in an accessible, practical way.
- **Informing and enabling clinical trials, from bench to randomized clinical trials:** Participants heard presentations from Drs. Aguiar, Henske and Ess focusing on current resources available to investigators interested in translating their findings from bench to bedside. Dr. Ajamete "Aj" Kaykas from Insitro joined for the panel discussion and Q&A, which centered on uncovering some of the general barriers to translation researchers are facing. Breakout discussions focused on how the TSC Alliance and The LAM Foundation may assist researchers in navigating the complicated translation process, including potentially funding high-risk, high-reward biomarker studies, improving clinical trial design and prioritizing the use of existing data (e.g., from the TSC Natural History Database) for longitudinal projects.

Early-career researcher symposium

To close the conference, Co-Chairs Charilaos "Harry" Filippakis, PhD and Gerta Hoxhaj, PhD developed an engaging and practical Early-Career Researcher Symposium. This meeting brought together a Career Development Panel (Rebecca Ihrie, PhD; Mark Keezer, MD, PhD; Gina Lee, PhD; Dario Lemos, PhD; and Alexander Valvezan, PhD) to provide guidance and advice to researchers who are early in their trajectories – be it academia, industry, advocacy or anything in between. Drs. Filippakis and Hoxhaj also invited early-career researchers who submitted abstracts to present and receive feedback on their oral presentations.

Acknowledgments

The TSC Alliance sincerely thanks our conference co-host, The LAM Foundation, for its vision and help in planning and executing the conference. Conference support was generously

provided by Greenwich Biosciences; The Rothberg Institute for Childhood Diseases; Nobelpharma; UCB; PsychoGenics, Inc; Upsher-Smith Laboratories, LLC; Noema Pharma; Seizure Tracker; BridgeBio; and Novartis.

Bcureful travel and lodging assistance



The Bcureful Travel Fund at the TSC Alliance® underwrites the TSC Travel and Lodging Patient Assistance Program, which is administered by the National Organization for Rare Disorders (NORD). This travel assistance program provides people diagnosed with TSC with financial assistance for travel and lodging related to participation in a clinical trial and/or furtherance of treatment at a TSC Alliance-recognized Center of Excellence or TSC Clinic.

- **Airfare** is available for the patient and one caregiver with a 3-hour or greater driving distance from study or treatment site. Travel arrangements are scheduled and prepaid.
- **Hotel** is available for participants with a 3-hour or greater driving distance from study or treatment site. Hotel stay will be booked and prepaid (not to exceed \$200 plus tax/night).
- **Ground transportation** between the airport and hotel and/or to the study site will also be a covered expense up to \$50 per trip.
- **Hospital/clinic parking** up to \$50 per trip will be reimbursed with the submission of receipt.
- **Mileage reimbursement** at the current IRS rate will be reimbursed for those participants who are traveling by car and not utilizing airfare assistance.

"When Owen's most recent seizures did not stop with medications, surgery was our only option left to rid his brain of them," explained Mary Garnett. "This required multi-stage surgery in a state 15 hours away. With assistance from the Bcureful's assistance with travel and lodging, Owen was able to get this surgery and has thrived ever since. We owe a great deal to this wonderful organization that made the impossible possible."

TSC Alliance Board Member Diane McSwain donates, raises funds and champions this important initiative. Be on the lookout for more information on the upcoming Valentine's Day 2022 fundraising event to benefit the Bcureful Travel Fund. For more information and to apply for funds, visit tscalliance.org/travelfund.

TSC Alliance launches new self-report portal

The TSC Alliance® has added a new dimension in connection with the TSC Natural History Database. A new portal will permit the collection of patient-reported outcomes (PROs) on a variety of topics related to tuberous sclerosis complex (TSC). PROs typically:

- are directly reported by the person with TSC,
- pertain to the affected individual's health, quality of life or functional status associated with health care or treatment, and
- are not monitored in real-time (i.e., not to be used for reporting emergencies).

In the case of individuals with TSC who are unable to speak or report for themselves, PROs can be submitted by their parent, guardian, or caregiver.

This initiative's purpose is to help the TSC Alliance and TSC researchers better understand the perspective of those affected by TSC so we can begin to track changes and measure improvement in areas most important to the TSC community, whether that be in the context of general care, treatment or something else. These outcomes can be measured in absolute terms (e.g., rating severity of pain on a numerical scale) or used to report changes from a previous measure (e.g., pain rating that changes over time or new symptom onset after starting a new drug).

PROs may also provide additional useful information. For example, a new drug may demonstrate beneficial clinical outcomes in terms of seizure reduction, while PROs may identify that people are not taking medications due to adverse side effects, the complexity of the dosing schedule or something else. PRO data are captured using an instrument with questions and documentation about its use. In addition, PROs may be helpful for regulatory bodies such as the United States Food and Drug Administration (FDA) to assess risk-benefit measures when evaluating a drug or medical device or clinical trial.

The TSC Alliance's first use of the self-report portal as a PRO instrument is through collaboration with the TANDem project (Empowering Families through Technology: a mobile-health project to reduce the TAND identification and treatment gap) by allowing the community to complete the

self-quantified TAND checklist (TAND-SQ). The TSC Alliance is helping the TANDem project group validate the utility of the TAND-SQ for future use in a mobile application. You can learn more about the TANDem at TANDconsortium.org.

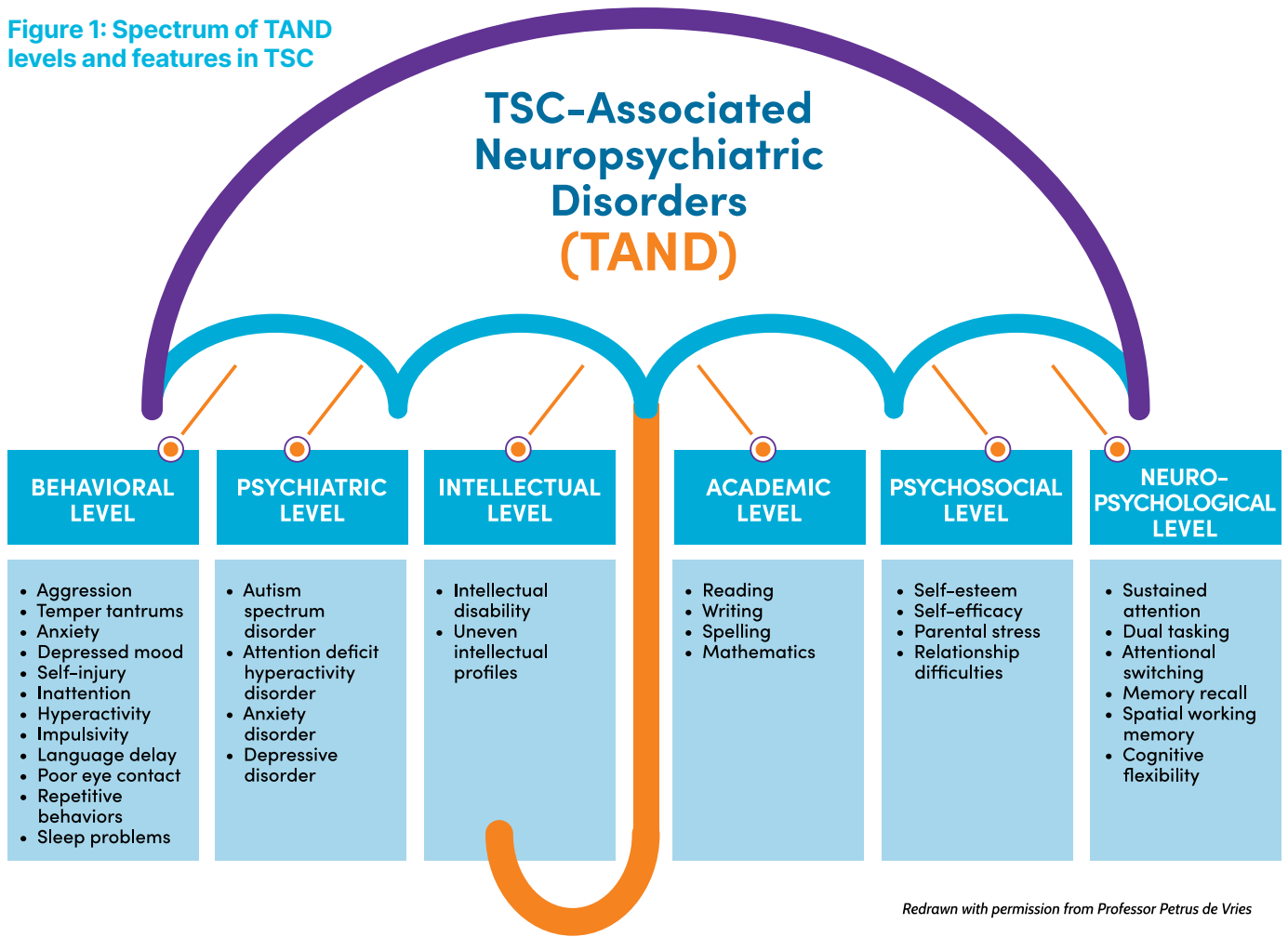
"We have listened to moms and dads who asked for a technology platform, making sure we do what is really needed in the community," Petrus de Vries, MBChB, FRCPPsych, PhD, who's overseeing the TANDem Project.

For many TAND symptoms (see Figure 1), no objective outcome measurements or ways to quantify severity currently exist. We hope this initiative will help us better understand TAND and how to treat it by learning directly from those affected by TSC or their caregivers.

The Self-Report Portal is built in Studytrax, a secure database used since 2014 for collecting medical data in the TSC Natural History Database (NHD). Participants in the NHD will be able to connect their self-reported data with their existing ID number. Similar to the NHD, no personal identifiers will be included, allowing de-identified data to be exported for research projects. It should take between 20-30 minutes to complete the TAND-SQ. Participants will be able to download their answers as a PDF for personal use. In the future, the TANDem team plans to develop clinical guidelines for the identification and treatment of TAND Clusters to be incorporated as a toolkit that will allow participants to readily share with their healthcare team. The TSC Alliance plans to incorporate additional PRO instruments in the self-report portal in the future. For more information or to sign up to participate, please email biosample@tscalliance.org.

"Studytrax is honored to be a part of the long history of TSC Alliance initiatives that improve the lives of those with TSC," explained John Putzke, PhD, MSPH, President of Studytrax. "What's most exciting is that the platform will help to organize and gather information about the full range of what those affected by TSC experience, including development, mental health and learning abilities. There will also be a secure messaging system to ask questions. New development will bring more ways to empower those with TSC and their families through technology, and bring a network of people, resources and investigators together to better coordinate and advance TSC research."

Figure 1: Spectrum of TAND levels and features in TSC



TANDem in a nutshell: A summary of the TANDem initiative

TSC-associated neuropsychiatric disorders (TAND) represent the number one concern to families and individuals around the world, yet they are highly under-identified and under-treated – referred to as an “identification gap” and a “treatment gap.” In 2012 the term “TAND” was introduced, and in 2015 the TAND Checklist was created to reduce the “identification gap.” Research using the TAND Checklist helped identify seven natural TAND Clusters that may be useful to reduce both the identification and treatment gaps for TAND further.

Following the initiative’s earlier research, community-based participatory research with families and individuals as well as a range of TSC stakeholders identified three next steps for action:

- Creation of a self-report and quantified version of the TAND Checklist.

- Creation of a digital tool such as an app for the TAND Checklist.
- Generation of evidence-based guidelines and a toolkit for next-step management of TAND Clusters.



TANDem
Empowering families through technology

The TANDem project was a direct result of the feedback from our TSC stakeholders and has three main goals:

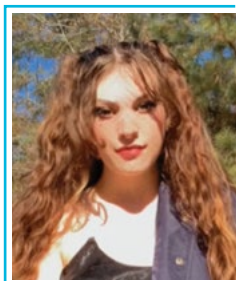
- Development and validation of a quantified, self-report TAND Checklist (TAND-SQ), built as a mobile app.
- Generation of consensus clinical guidelines for identification and treatment of TAND Clusters to be incorporated as a toolkit into the app.
- Establishment of a global TAND Consortium through a range of networking, capacity-building and public engagement activities.

Meet the 2022 TSC Alliance future leaders

Launched in 2016, the Future Leaders Program (formerly the Junior Leaders program) was designed with post-high school education in mind – whether it’s attending trade school, obtaining certification or earning an associate’s or bachelor’s degree. The TSC Alliance offers this opportunity for young adults with TSC, or their siblings, to volunteer and offer support while raising awareness for TSC. Throughout the school year, program participants will complete a series of virtual awareness and support activities, concluding with an exit interview.

Laura Loveless **Hattiesburg, MS** **Freshman, Pearl River** **Community College**

I was diagnosed at three weeks old after they found a rhabdomyoma. I wanted to be a Future Leader because it’s important to me to raise awareness about how TSC affects people. TSC is such a diverse disorder that can be complicated for some people to understand, so I wanted to be able to be a resource for others. I also wanted to be able to connect to others that share the same disorder. As a leader, I hope to be able to bring more awareness to TSC.



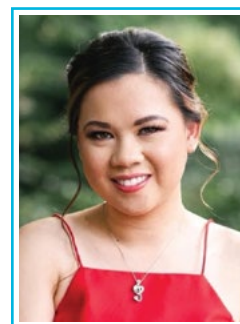
Guinevere Pandolfi **New York, NY** **Student at NYU, Class of 2023**

I’m a young adult with TSC. I plan on studying genetics in my future career, so I wanted to be a future leader to engage and build a relationship with the TSC community. The more familiar I am with the TSC community, the more I’ll be able to incorporate it into my studies in genetics, and hopefully, I can help create a larger platform for TSC. I hope to spread awareness about TSC as well as learn more about and become more involved with the clinical research of TSC.



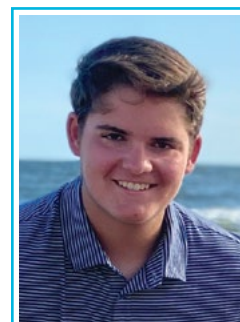
Emily Phan **Orange County, CA** **Music Director, Pepperdine** **University 2019**

At the age of 6 months old, I was diagnosed with TSC after being rushed to the hospital due to infantile spasms. After starting vigabatrin through a clinical trial at UCLA, all my seizures stopped. This afforded me the opportunity to become who I am today and share my experiences battling TSC. As a TSC future leader, I get to speak in my love language, acts of service, by sharing my gift of music as a classical concert pianist and build heartfelt connections within a wonderful community I am able to call home.



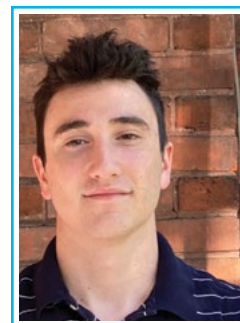
Jack Quintana **Summerfield, NC** **High School Senior**

My connection to TSC is my younger sister. I plan to attend medical school and become a pediatric ophthalmologist to treat patients with TSC in my practice. As a Future Leader, I want to spread TSC awareness in any possible way so we can fight this disease together.



Jacob Saadoun **Philadelphia, PA** **Peri-Operative Assistant at Penn** **Medicine**

My connection to TSC is my older sister. I wanted to become a Future Leader to learn more about TSC and connect more with the TSC community. I hope to continue spreading awareness and learning from the community about their experiences



with TSC. I also hope to assist in discovering creative ways to advocate and increase awareness for TSC.

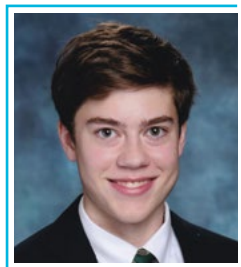
**Robert Seymour
Holliston, MA
Paraprofessional/Fred. W
Elementary School in Holliston
Massachusetts**

I wish to be a Future Leader because I want to offer advice and guidance to other young adults like me who are living with TSC. I hope to formulate strong connections with others and use my voice in a positive manner that contributes to some sort of social change within our country and the world at large.



**William Suringa
Northern VA
High School Senior**

I have a sibling with TSC. During my t-shirt fundraiser for Step Forward to Cure TSC®, many of my classmates asked me “what on earth is TSC?” I had the usual scientific response queued to answer, but



I knew there was so much more. Stories I heard at the 2017 DC LAMposium Regional Conference and 2018 World TSC Conference in Dallas, cheers from other teams during the walk-run-ride and Art for a Cure competition, applause after presenting my paper at a young researcher’s conference and helping my brother struggle through school all came to mind. I wanted to become a Future Leader to give more people the whole truth of TSC.

**Natalia “Nat”
Zarazua
Mexico City, Mexico
Psychology Degree
Candidate**

I am a young adult with TSC who wanted to become a Future Leader to learn more about the disease, meet other young adults with TSC and have an impact by give my personal experience living with TSC. I am excited to be part of the program because I want to help other young adults and their families affected by TSC. Also, I want to share my own experiences and want people to know that TSC doesn’t define us! We are more than that and can achieve everything we want, whether by ourselves or with help of others. TSC mustn’t be an obstacle.



Based on your request, the TSC Alliance® will host monthly Zoom calls throughout 2022 for adults with TSC. Our recent e-Webinar series featured a Life Stages track to address the needs of the adult with TSC. Because of its success, we will continue to provide adult-specific “topic” calls mixed with open discussion, as well as “open forums” with no predetermined topics. This year’s schedule includes:

- January 19 – Topic Call
- February 16 – Topic Call
- March 16 – Open Forum
- April 20 – Topic Call
- May 18 – Topic Call
- June 15 – Open Forum
- July 20 – Topic Call
- August 17 – Topic Call
- September 21 – Open Forum
- October 19 – Topic Call

- November 16 – Topic Call
- December 21 – Open Forum

You must register for each Topic/ Open Forum call separately to receive the specialized Zoom links. Please note: Using your computer camera is optional and not required for participation.

To subscribe to our *TSC Waves* adult newsletter, register for any monthly call or submit a “topic” suggestion, visit tscalliance.org/adults. You may also contact April Cooper, Vice President, Community Programs, at acooper@tscalliance.org with any questions or ideas.

Connect with an Adult Regional Coordinator

The TSC Alliance’s Adult Regional Coordinator (ARC) program acts as a resource to help connect any adult

Adults with TSC corner



with TSC with others within his or her region. ARCs are knowledgeable about resources, events, support meetings, conferences and any new research on TSC. ARCs also support adult involvement within our Community Alliances and are involved with government advocacy. To contact an ARC in your area, visit tscalliance.org/ARCprogram.

TSC Clinics “Up Close”

Centre Hospitalier Universitaire Sainte-Justine Centre and Hospitalier de l’Université de Montréal



The TSC Alliance® recognizes four TSC Clinics in Canada. This article continues an interview series about the healthcare professionals and staff at the two clinics in the French-speaking city of Montréal, Quebec.

Dr. Philippe Major directs the clinic at the Centre Hospitalier Universitaire Sainte-Justine (CHU Sainte-Justine), which received its TSC Clinic designation in Fall 2018. Less than 5 miles away is the Centre Hospitalier de l’Université de Montréal (CHUM), where Dr. Mark Keezer directs its clinic for adults with TSC, which was recognized in Fall 2020.

The TSC Alliance, in collaboration with Tuberous Sclerosis Canada Sclérose Tubéreuse (TSC Canada), is pleased to announce CHU Sainte-Justine and CHUM will be the first two international TSC Clinics to enroll children and adults with TSC in the TSC Alliance’s Biosample Repository and Natural History Database projects beginning in 2022.

What motivated you to choose your career path?

Dr. Benoit: I always knew I wanted to work with children and be involved in education. Once in medical school, I really enjoyed understanding the complexity of renal physiology: pursuing a career as a pediatric nephrologist and professor at the Université de Montréal was my way to go! Having also an interest in genetics, I completed a fellowship in Paris on inherited kidney diseases and kidney development. It is therefore with great pleasure that I joined the TSC Clinic at CHU Sainte-Justine.

Dr. Dahdah: Tough question indeed. Motivation in the medical field does not come with one item or two, or a short list for that matter. It does not result from a deep reflection neither, at least in my perspective. The real motivation for me has always been innate to my beliefs in advancing humanity’s wellbeing. As for my motivation toward the care of children with TSC, I would say I’ve always considered that rare and orphan diseases merit more attention than what is provided or available. Being engaged in “the obvious” (diseases that are always in the news and on billboards) is readily available and well served. Caring for the “lonely” should be left to the hazard. In medicine, like in many aspects of life, there are lesser known diseases, and TSC is one.

Nurse Lucie: I decided to be a nurse since a young age to help and care for others. I have worked with several patient populations during my career, but for the past 15 years, I have had the opportunity to obtain a position in neurology and I fell in love with working with the patients and families living with tuberous sclerosis complex. Since Dr. Major founded the TSC Clinic, this allowed me to become actively involved with this clientele.

Dr. Haffaf: Since my childhood, I was fascinated by my older brother. He has always been a model and a mentor to me. Seeing him grow, learn and become the admirable physician he is today influenced my career choice. I became a doctor because I like helping people and listening to them. For me,

it was important to answer the question of how to give the best patient care.

I am lucky to be able to combine my experiences as a pediatrician, oncologist and medical geneticist. It has helped me a lot in my career by allowing me to have an integrated view of the different needs of patients with complex conditions. The transversality of genetics and genomics offers an interesting platform for the personalization of care.

Dr. Lattouf: I always felt helping people was a noble occupation, and I had an inclination to listen and try to be helpful from an early age. From there, my interest in science drew me naturally to the medical profession. And once I was accepted in medical school, I merely needed to find the path I felt would generate the most impact. Oncology seemed just right from a clinical and research perspective.

Nurse Vanessa: I have always been interested in the medical field but also by the psychosocial aspects of clinical care. As a nurse, I feel I am able to have a direct impact on patient health and wellbeing.

Dr. Major: When I was in medical school, I became fascinated by the nervous system and by the brain in particular. I decided to become a neurologist when I realized the knowledge of brain anatomy and function could help people by localizing lesions and identifying the diseases. I subsequently worked with pediatric neurologists who inspired me and confirmed my idea that this field was for me. I was impressed by their passion for neurology, by their dedication, and by their depth of knowledge. During my residency in pediatric neurology at the CHU Ste-Justine, I developed a particular interest for epilepsy,

especially refractory epilepsy. Therefore, it was logical for me to pursue a fellowship in tuberous sclerosis complex, as it is often associated with severe epilepsy.

Dr. Major, after you completed your pediatric neurology residency at CHU Sainte-Justine, Université de Montréal in 2006, you did additional training in pediatric neurology and TSC at the Massachusetts General Hospital (MGH), Harvard University, where you worked with Dr. Elizabeth Thiele. What were some of the highlights from your two years there?

Dr. Major: My two years of fellowship at the MGH, under Elizabeth Thiele's mentorship, were among the most memorable of my life. I had the opportunity to work with one of the top TSC experts in the world, in an institution with a rich history of excellence in the field of neurology.

Dr. Thiele had been able to build a comprehensive multidisciplinary TSC Clinic – a model I subsequently wanted to emulate. I was impressed by Dr. Thiele's warm approach with the patients, by her leadership in the fields of TSC and by her constant desire to better understand the disease. In 2006, it was the beginning of the mTOR treatments. It was particularly exciting to participate to the mTOR clinical research projects that ultimately changed the management of TSC. From a genetic disease for which only symptomatic treatments were offered, we could finally aim at treating the specific defect causing some of the clinical manifestations.

Another highlight of my fellowship is I met with great residents and physicians with whom I still keep contact and collaborate with when I can.



Philippe Major, MD, pediatric neurologist, Chief of Pediatric Neurology, Departments of Neurosciences and Pediatrics, **Université de Montréal**



Geneviève Benoit, MD, pediatric nephrologist, Associate Professor, Pediatrics Department, **Université de Montréal**



Nagib S. Dahdah, MD, MBA, pediatric cardiologist, Professor of Pediatrics, Division of Pediatric Cardiology, **Université de Montréal**



Lucie Côté, RN, nurse clinician coordinator, Pediatrics Department **Centre Hospitalier Universitaire Sainte-Justine**



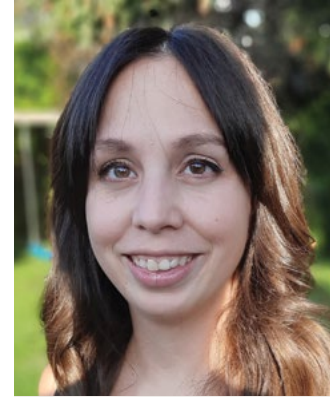
Mark R. Keezer, MDCM, PhD, Associate professor (clinical), Department of Neurosciences and the School of Public Health of the Université de Montréal



Zaki El Haffaf, MD, medical geneticist, Associate Professor; Director Genetic Department



Jean-Baptiste Lattouf, MD, FRCS, urologic oncologist, Department of Surgery-Urology



Vanessa Léger, RN, nurse clinician for the CHUM epilepsy clinic and the CHUM TSC clinic, Department of Neurology

Dr. Keezer, your undergraduate education was at Georgia State University (GSU) in history and anthropology. What motivated you to choose your career path, which includes a PhD in translational neuroscience from Leiden University, The Netherlands?

Dr. Keezer: After finishing my undergraduate degree at GSU, I returned to my home city of Montreal, unsure of what type of career I wanted to pursue. I spent a summer volunteering at a hospital and fell in love with medicine. I had a few incredible mentors as a medical student, resident and graduate student. These mentors led to my fascination with neurology, epilepsy and finally epidemiology. There are many to mention, but Drs. Fred Anderman and Christina Wolfson along with Professor Ley Sander were particularly central.

What I most loved about anthropology/archeology was the process of reconstructing human behavior based upon the incomplete data at our disposal. Neurology and epidemiology are similar, where you begin with an incomplete knowledge set, having to use attention to detail and logic to arrive at reasonable conclusions, always knowing the truth is just beyond your grasp, and so always ready to revisit your hypotheses if faced with new and conflicting data. In a sense, there remains an art to all of what I do, which I especially enjoy.

How many languages are you fluent in Dr. Keezer?

Dr. Keezer: Only two: English and French. I am part of the English-speaking minority of Quebec, Canada. Out of 8.5 million inhabitants, there are about 600,000 of us. My fluency in French has certainly improved since beginning to work at

a French-language university. I am grateful to my students who politely correct their “anglo” supervisor when I make yet another grammatical error. Otherwise, I know a few words in Arabic, but only those my “sittu” (“grandmother” in Arabic) would say to a grandchild.

What are the most challenging aspects of your clinical work or research as it relates to those affected by TSC?

Dr. Major: In my practice, the most challenging aspect is the management of mental health issues related to TSC. We have the ability to screen for these issues, but I feel the health system should provide more services to patients facing these challenges.

Dr. Benoit: Although renal involvement is common in TSC, many patients have few or no symptoms related to kidney disease. Thus, most patients with TSC-related kidney lesions come to attention on surveillance imaging. We therefore do our best to provide comprehensive information to the patients and their families about a kidney condition for which they are often asymptomatic. We also have a role in determining which patients may benefit from medical interventions to slow the renal progression.

Some patients with TSC may develop chronic kidney disease, usually in adulthood. Transitional care is an important step in our patients’ care; preparing a safe and smooth transition from adolescence to adult care is a primordial part of our role.

Dr. Dahdah: Resources are always on the list. Convincing non-profit organizations and governmental granting agencies

to fund clinical research has been extremely tough, especially for orphan diseases. Pharmaceutical companies could be generous, but only when there is an advantage to their products. I think large non-profit organizations that were extremely successful in raising huge amounts of funds for their target diseases could work in unison with those lesser-known diseases and assist in their efforts. Are we not in a society where we cover the essential needs of our family members? There is a deep though to give here.

Nurse Lucie: One of the biggest challenges is to offer individualized and optimal services to all patients within a reasonable timeframe, given the lack of resources in the Quebec health system.

Dr. Keezer: I am fortunate in that I am generally able to obtain the necessary clinical resources for my patients. I have fantastic colleagues who work with me and our patients toward a common goal. Access to social services is a challenge at times. It is especially stressful for parents of children with severe intellectual disability, where entering the age of majority comes with a host of financial and legal considerations.

Dr. Haffaf: There are three exciting challenges in my clinical practice:

1. Ensuring the best transition of care from pediatric to adult care. This step is essential in the continuity of care and to catch up patients who have escaped diagnostic technological advances.
2. Helping to resolve difficult TSC diagnoses, such as those with mosaicism. Indeed, helping families and patients understand the mechanism of the condition and solving mosaic cases helps to better understand the heterogeneous presentation of the disease
3. Supporting patients and their families. Genetics provides family management of the condition. From preconception to old age through prenatal issues and finally, at all ages. It is exciting for teams to adopt a multidisciplinary approach in order to provide all the answers to families.

Dr. Lattouf: Coordination with multiple specialties in patients living with TSC and having various multisystem affections have always been a challenge. Clinical knowledge is another unanswered need, as data from the literature always pertains to limited series of patient with questionable generalizability. This is why I believe a multi-institutional/multi-centric approach is the best way to generate meaningful clinical information to treat these patients. Such work is just starting to take place, and the future holds many promising possibilities.

Nurse Vanessa: The most challenging aspect of my clinical work is because TSC is a multisystem condition, requiring the coordination of multiple medical specialists. Over time, we have recruited a dedicated physician/healthcare professional for each specialty. This has made one of my important tasks a little easier.

What are the most fulfilling aspects?

Dr. Major: The most fulfilling aspects of running a TSC clinic is I have the opportunity to build long-lasting bonds with the patients and their families. I get to be part of their “extended” families. I feel this high level of trust helps me better guide the patients in terms of treatments and life choices.

Another aspect of my work I particularly enjoy is working with dynamic and generous colleagues who share common values. Despite the challenges we face as health professionals, we can always rely on the team for support and good humor.

Dr. Benoit: Definitely the trust and confidence we develop on a long-term basis with patients and their families. Working in a multidisciplinary team allows us to treat the person as a whole. The approach is also family-centered, because at some point, any disorder affects the entire family; the emotional needs of our patients and their families are taken into account. I am always touched by the love and dedication of families of children with TSC to ensure their children's needs are being met.

Dr. Dahdah: Making the difference in kids' lives, bringing smiles to surface despite the heavy weight of the disease on the families trigger the tingling feeling of fulfillment. From a purely scientific angle I would say sometimes a relatively little effort can put to light a major source of useful information which will enable advances in healthcare.

Nurse Lucie: Working with families is very fulfilling in many ways. The privileged and close contact I have with families, the privilege of witnessing a precious moment of seeing their children grow and develop and above all, to feel my work is making a difference in their lives.

TSC Clinic days are often the most rewarding, because I have the chance to work with a multidisciplinary team and with exceptional professionals who have the children and families at heart, which allows me to grow and offer the best patient care possible. All of this is very satisfying..

Dr. Keezer: I find being able to reassure patients that whatever happens, we will work to find a solution is the most fulfilling. Being able to reassure a parent who has just transitioned with his or her dependent child, a parent who is nervous about being without the support of the pediatric team, that all is not lost. Also being able to reassure an older adult, perhaps floundering without proper follow-up for years/decades, that together we will ensure everything that needs to be addressed is done.

Managing the complications of TSC is an ongoing process. Working with patients with TSC is very unlike my work with my average patient with epilepsy. There are generally so many other systemic issues that must continuously be considered. This aspect feeds into my interest in the comorbidities of epilepsy. I find working with people with TSC a special challenge, but one that motivates me, as I continue to learn how to be a better physician to them.

Dr. Haffaf: The most rewarding aspect of this approach is the exchange of expertise among many professionals around the care needs of patients with TSC. I promote multidisciplinary and interdisciplinary integrated medicine to increase the relevance of team interventions.

In addition, the education we provide to patients and their families about the condition by giving some demystification of the disease and by offering them the opportunity for pre-implantation diagnosis.

Dr. Lattouf: The gratefulness coming from families of patients who are heavily affected by TSC, when they find a multidisciplinary setting in an adult care facility, where their loved ones are fully taken care of in a one-stop-shop type of clinic, is priceless. These people are often burdened with the task of being caregivers with very little resources. They oftentimes have to juggle a day job and a personal life along with their caregiver role. Having access to multidisciplinary TSC clinics for their loved one makes life so much easier for them. I believe the plus value is tremendous in this setting.

Nurse Vanessa: The feeling that we are taking care of patients from head to toe.

I recently had the mother of a patient tell me it was very reassuring for her to know who to call if she had any questions regarding the health of her daughter. It made a big difference in her life to have someone like me who is easily reachable. Such comments are always very rewarding.

TSC is considered a linchpin disease – meaning every advance made in TSC research may benefit more prevalent diseases like epilepsy, autism and cancer. What would be your priority if you were a \$1 million grant to support clinical or laboratory research in TSC?

Dr. Major: I strongly think research about rare diseases like TSC should nowadays involve a multidisciplinary team, because clinical manifestations are more difficult to comprehend in isolation. From bench to bedside, specialists in epilepsy, autism, cancer, etc. should collaborate together. If I had \$1 million, fostering collaboration would be my priority. This would hopefully lead to advances in TSC, as well as other related conditions.

Dr. Keezer: There is so much we do not understand about TSC in general, but I believe this is especially the case for adults with TSC. I am very interested in questions regarding optimal methods of screening and following comorbidities in adults with TSC. These are individuals who will live with TSC for 60-plus years or more. There must be better ways to predict who is at greater risk of developing complications such as renal angiomyolipomas.

The TSC community also continues to have many questions as to who should be treated with mTOR inhibitors early versus those for whom we can still wait, how long to treat and whether intermittent strategies are appropriate in certain people.

Dr. Haffaf: This is a very interesting question of current interest. There are probably two avenues of research. The first one relies on gene therapies (Crisper), which asks the question: how can we correct the molecular defect while ensuring the best possible efficiency?

The second one involves establishing a better knowledge of the other factors determining the expression of the disease. Why is the presentation so heterogeneous? What are the modifying factors? Several analogies could be made with oncogenesis for example.

Dr. Lattouf: I would focus on my field and invest a \$1 million grant in the oncologic aspects of TSC, but I think patients with this condition are best served by investing in research on gene therapy for TSC, which would attack this ailment at its roots and address all affected systems at once.

How do you balance your time between clinical and personal and what do you enjoy doing outside of work?

Dr. Major: Work is definitely a significant part of my life, but it is as essential for me to dedicate time and energy to my family. As father of three active teenagers, I particularly enjoy supporting them in all their projects and activities, like volleyball, swimming, soccer, karate and chess. On a more personal note, my hobbies are running, cycling and volleyball. Last fall, I had the opportunity to run my first marathon, an event that required quite a lot of preparation, but one I enjoyed very much.

Dr. Benoit: I am a proud mother of three wonderful boys. Juggling the demands of career and personal life is an ongoing challenge! I have an interest in photography, and I try to spend some time playing piano. I am also a foodie. I find joy in little things, and I feel blessed to have a dynamic career and be surrounded by love at home.

Dr. Dahdah: I don't; at least I don't think about it this way. If I am not in peace with what I do, then I simply won't do it. There comes the balance, by simply doing what we love and loving what we do.

Nurse Lucie: Being the mother of two teenagers, I am also a volleyball mom and a hockey mom, so I spend some of my time going to encourage them in their respective sports. I also enjoy going for walks in nature and recharging my batteries at the chalet, where a discovered paddleboarding in the summer and snowshoeing in the winter allows me to disconnect and find my zen and calm.

My favorite expression is: *carpe diem* or "seize the day"!

Dr. Keezer: I try to not work on weekends, leaving me with time dedicated toward family and friends. My free time most recently has been spent playing strategy board games with one son (*Rome vs. Carthage* is his favorite) and building plastic scale models with the other. I must admit, I have as much fun doing these activities as they do.

Dr. Haffaf: My time away from work is devoted to my family (parents, my fantastic wife and two splendid daughters), meditation and spirituality on the meaning of life and music. I play the luth and I am part of an Arab-Andalusian music association. It brings me serenity.

I also work on setting up a virtual library dedicated to Arab-Andalusian music. It is my way of contributing to the cultural heritage of the young generations. It is based on the same professional philosophy.

Dr. Lattouf: This is a tricky one, and my wife would say it is work in progress. I have to say I have three magnificent boys who keep me quite busy most my time off work. Trying to

keep them busy in a constructive way is a challenge in itself; but without knowing, they give back so much, and they make work every next day even easier. When I have time for myself, I enjoy among other things reading, sketching and listening to music.

Nurse Vanessa: I work full time at CHUM, and it's not always easy to balance my clinical and personal time. I think it's important for my mental health to make a break between my professional life and my private life. Knowing that while at work I gave the best of me to my patients and that my family deserve the same. I currently have a three-year-old daughter and a baby on the way, so my families take up all my free time.

Afinitor® loss of exclusivity: what you need to know

As of October 2021, all dose formulations of Afinitor® lost exclusivity, and generic versions (everolimus) can now be marketed for sale at pharmacies. The TSC Alliance® wants to ensure the TSC community understands what this means and offer some action steps to prevent any delay in medication access.

Losing exclusivity simply means the brand-name drug is no longer protected from generic drug competition. In this scenario, FDA-approved generic equivalents (everolimus) of name brand Afinitor® can legally be prescribed with a prescription. It is important to understand a generic designation does not mean lesser value or efficacy. Generic medications must be biologically similar to the name brand formulations and therefore be interchangeable for treatment management.

Insurance companies will then negotiate drug prices from pharmaceutical manufacturers through third party companies known as pharmacy benefit managers (PBMs). Common PBMs are Express Scripts, CVS Caremark and OptumRx. Once an agreement has been reached, insurance companies will decide

what medication (brand, generic or both) to include on the insurance plan pharmacy drug list (PDL) known as the formulary (preferred) medication. This simply means your insurance plan will list the preferred medication available to you on your PDL. Because additional options are available, it is not uncommon for insurance plans to adjust what medication is covered; therefore, if your plan once covered the brand (Afinitor®), it might now only cover generic form (everolimus). Since we are in the new year, it is essential to understand what option is listed as your preferred medication. Most plans have online portals that will allow you to confirm which available option is preferred.

The TSC Alliance understands abrupt medication changes are never ideal; however, we also want to make sure our community feels comfortable knowing that interchanging this medication between name brand versus generic is a safe option. We recommend you discuss which option is best for you or your loved one, but ideally it is reasonable to stick with the medication covered under your plan. Therefore, if your insurance no longer covers brand Afinitor® but covers the generic option, then switching to your medication over should be seamless.

Because these drugs are equivalent, drug tapering or drug vacation

prior to switching over to the generic option, does not seem to be necessary. Furthermore, the name brand and generic dosages are equivalent; thus, if you were taking Afinitor® 10 mg you would take everolimus 10 mg. Side effects will be similar between brand and generic, and there is no clinical evidence for "new" side effects during the transition. However, we understand a small population of individuals can be sensitive to even small changes in medication and might experience different side effects. If you or your loved one has had specific issues regarding this in the past, we strongly encourage you to discuss it with your prescribing healthcare provider.

As with any change, some difficulties in co-pay assistance with drug coverage is to be expected. At this time, those enrolled in Afinitrac as of December 31, 2021, will continue to receive co-pay assistance for undisclosed amount of time. Generic drugs tend to be less expensive, but we understand cheaper does not always make it more affordable to the consumer. Unfortunately, the TSC Alliance is not aware of any assistance programs currently for generic options. GoodRx.com is a coupon app that may help reduce costs. Please contact your local healthcare provider if you need additional assistance with options during these changes.

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 Anonymous
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 Mr. Juan G. Carrion
 Mr. Lars Holmquist
 Mr. Tim Kanter
 Ms. Joanne Math
 Mr. and Ms. Robert Moss
 Mr. and Ms. Christopher Rosbeck
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The benefits of legacy giving

Everyone has something they are passionate about. You may be passionate about a place that holds an important spot in your heart, a cause that's near and dear to you or a charitable organization for which you frequently volunteer.

But, did you know you can ensure your support of the places you're passionate about, like the TSC Alliance® Endowment Fund, continues even after your lifetime while also receiving tax benefits for yourself?

You can – with a legacy gift to TSC Alliance Endowment Fund.

Here are some popular opportunities:

Include a gift to TSC Alliance Endowment Fund in your will or living trust.

Your benefits: Retain control of your assets and have the flexibility to change your mind at any time.

Name TSC Alliance Endowment Fund beneficiary of your retirement account.

Your benefit: These assets pass to TSC Alliance Endowment Fund tax-free, allowing us to use the entire amount to support our mission. If passed to your loved ones, they would have to pay income tax when distributions are made from the account.

Transfer appreciated stock to TSC Alliance Endowment Fund.

Your benefits: You qualify for a federal income tax charitable deduction based on the current fair market value of



the securities and are exempt from paying capital gains tax on any increase in their value.

Create a life income gift.

Your benefits: Depending on the type of gift you choose, you can receive stable income for life or for a period of up to 20 years. You may also qualify for a federal income tax charitable deduction in the year you make the gift.

Need help?

There are many ways you can ensure your support for TSC Alliance Endowment Fund continues for years to come. Contact Lisa Moss, Vice President, Donor Relations, at (301) 562-9890, (800) 225-6872 or lmoss@tscalliance.org today for help finding the perfect gift for you.

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