

TSA Year in Review: 2022

Key projects and milestones



Welcome

The TSA's Year in Review 2022 looks back at the work of the TSA during what was a year of great transition and change. This booklet gives the TSC community, our supporters and the TSA itself a chance to reflect on the key victories from our small team in 2022.

In this booklet, you'll find:

- **TSA Impact Report 2022.** An overview of the work of the TSA in 2022 and the difference we made to the TSC community in what was a turbulent year
- **TSA Strategic Report 2022.** A more in-depth look at the TSA and what we achieved in 2022, looking at the inner-workings of our strategy and key enablers. This forms part of the annual accounts that we submit to the Charity Commission to show them what we've been doing

We welcome comments and questions about our work to improve the lives of people affected by TSC. If you've got a question for us about the Year in Review 2022, get in touch: admin@tuberous-sclerosis.org.

About Tuberous Sclerosis Complex

- Rare genetic condition, often inherited
- Every month around 10 babies are born with TSC in the UK
- Causes growths to develop in different organs around the body, such as the brain, lungs, kidneys, eyes, heart and skin
- Common problems with TSC include epilepsy, autism, learning difficulties and kidney problems. However, the way that TSC impacts on a person's life can vary considerably

About the Tuberous Sclerosis Association

The Tuberous Sclerosis Association (TSA) is the only UK charity focused on improving the lives of people affected by the rare genetic disorder Tuberous Sclerosis Complex (TSC). Founded in 1977, the TSA's mission is 'To provide help for today and a cure for tomorrow'. We do this by:

- Providing support and information to the TSC community, including the UK-wide TSA Support Line
- Organising events and opportunities for the TSC community, allowing people affected by TSC to come together, learn and be less alone
- Funding internationally-significant research into all aspects of TSC, with research focused on what will have the greatest impact on life with TSC
- Working closely with clinicians and other professionals focused on improving the lives of people affected by TSC
- Campaigning on behalf of the TSC community to ensure consistent and meaningful access to health and social care provision

“The TSA helps us understand that we are not alone, there is support, advice and help at the end of the phone. The TSA, for us, means a lifeline.”

“The TSA is a fantastic organisation. It's fabulous that they're on-hand to provide support and guidance. It's also good to know that the TSA are keeping an eye on developments and championing research into TSC, with the aim of treating the condition and ultimately finding a cure.”

“The TSA has been a very valuable support in finding help during our TSC journey.”

“The TSA Support Line was extremely helpful. I sent a long email explaining how things were for me and how I was struggling. They were fantastic and really looked into my problem and gave me several suggestions of information to read and follow up. They responded quickly, in a detailed way, and made me feel like I wasn't causing them any trouble with complex questions.”

“The amount of information from the TSA Support Line is amazing, I've gone from not having a clue where to look for information and how to learn about this condition that my daughter has, to having it right there.”

“The TSA was very helpful when my daughter was ill. They put me in touch with the right people, helping my daughter's doctor to treat her. Also, in lockdown when I was struggling, they listened to me and once again helped.”

“The TSA Support Line is a very helpful lifeline for us.”

tssa
tuberous sclerosis
association

A look back at 2022

2022 was a period of changes and challenges for the TSA and the TSC community. The charity progressed with our recovery from the Covid-19 pandemic, while battling fresh issues. But, we're confident that these experiences have left us stronger.

We all hoped that 2022 would be a year of rediscovered freedom for the TSA, TSC community and the wider world, with Covid-19 largely in the past. Yet, in many ways it felt like we replaced one crisis for multiple others, with rising energy bills and food prices making daily life harder than ever.

As the cost of living soared, things became even harder than usual for the TSC community in 2022. As well as dealing with the physical and mental impact of TSC, people were now having to think about just how to keep the electricity on and the fridge stocked. High numbers of enquiries for the TSA Support Fund were a stark reminder of why the TSA's work is so important.

Within the TSA itself, 2022 was a time of great adjustment. Multiple shifts in leadership meant that the team at the start 2022 was very different to the one that ended the year. However, the internal promotion of Dr Pooja Takhar and Luke Langlands towards the end of 2022 to Joint Chief Executives has had a big impact in steadying the charity. This shows the strength of our team and ensured an easier transition during what was a pivotal time. Pooja and Luke both continue to run the charity today in 2023.

Undeniably, all of the hurdles thrown at the TSA and TSC community in 2022 affected our fundraising capabilities, which is reflected in lower than usual income for the year. Nevertheless, the TSC community delivered an incredible amount of giving throughout the year, with participants in organised events (like the London Marathon) or DIY events (like coffee mornings) accounting for half of all of our income for the year.

Although 2022 was a very difficult year, the small TSA team worked tirelessly for the TSC community and achieved some major victories and milestones. These included:

- **Direct support and information for the TSC community.** The TSA Support Line welcomed a record number of enquiries in 2022
- **Achieving access to important medicines.** We successfully campaigned to get funding for cannabidiol (brand name Epidyolex) in the UK – an important medicine option for TSC-related epilepsy
- **Working even closer with the NHS.** We launched and continue to manage the NHS TSC Clinics Network – a UK-wide body that connects NHS TSC clinics and TSC specialist doctors and nurses
- **A return to face-to-face events.** We were delighted to welcome people to Outlook 2022, our first face-to-face event since the pandemic. Outlook is an annual event for adults mildly affected by TSC
- **Advancing the TSC research agenda.** We continued to meet all of our research commitments to improve the lives of people affected by TSC, despite major economic challenges

Looking back, 2022 forced us to adapt and innovate in ways that have set the foundation for our future growth and long-term stability. We had no choice but to rethink, reinvent and reimagine how we operate and how we can ensure that we continue to be here for the TSC community.

We're hopeful that the resilience and strength shown by our team and the TSC community in 2022 will be the foundations to future prosperous years.

A snapshot of our work in 2022

Successful change in TSA leadership, with new Joint Chief Executives

Helping people with TSC feel safe, informed and more confident in their daily lives post-pandemic

Funding important TSC research, focused on studies that will have the biggest impact

Re-introducing face-to-face TSA events (the first since the pandemic), plus continuing virtual events

Record-breaking enquiries on the TSA Support Line

Working even closer with TSC clinicians throughout the NHS

More opportunities for people to take part in fundraising, with largescale events restarting

TSA Impact Report 2022

The impact report gives readers an overview of the TSA's work in 2022 across all of our teams and everything that we achieved

Support and information

The TSA's small support team is dedicated to providing information, signposting and a listening ear to the TSC community. In 2022, we focused on widening the support that we offer, so that the TSC community can receive the best service possible

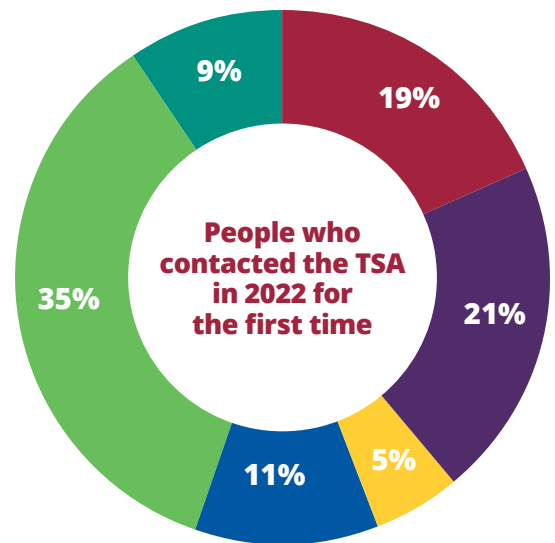
Reaching more people

In 2022, we added **456** new individuals to our database. With over 7,000 people now on the TSA database, we've almost **doubled our initial target** to be in contact with 3,700 people in the TSC community by 2023!

When we speak to someone for the first time, we ask them what their TSC connection is. In 2022, the most common connections were:

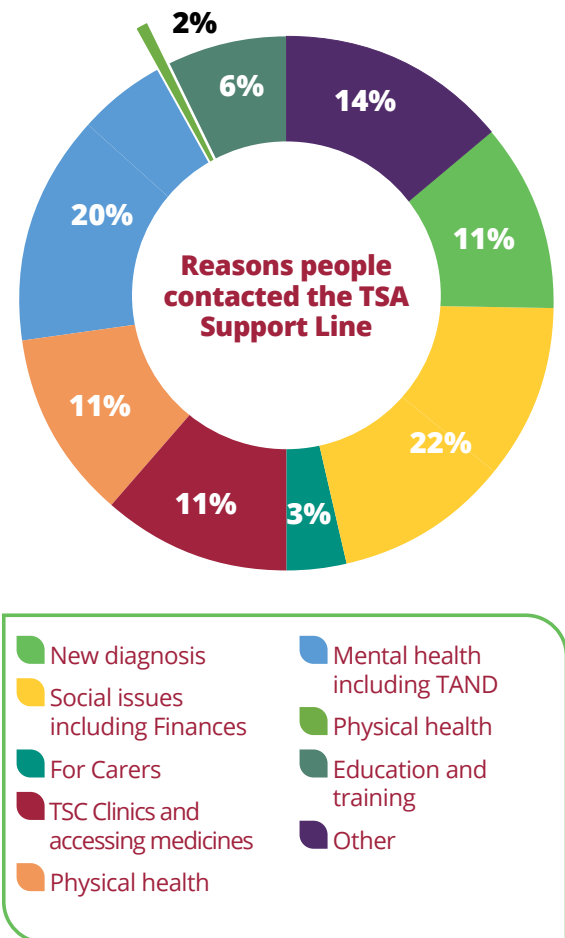
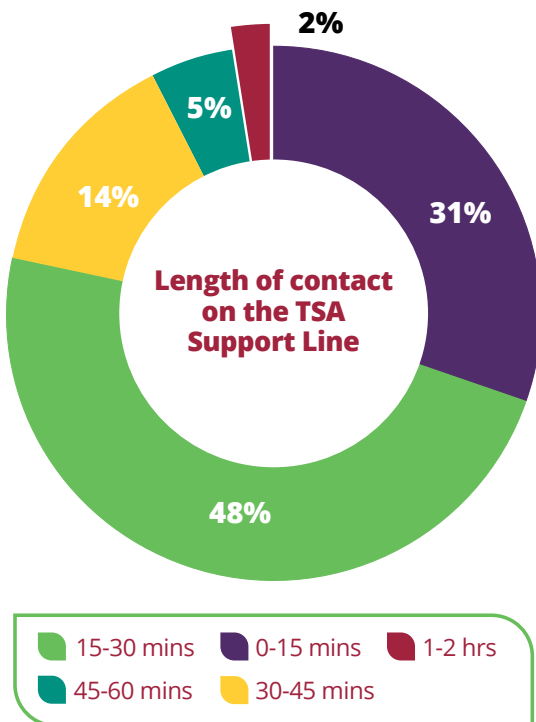
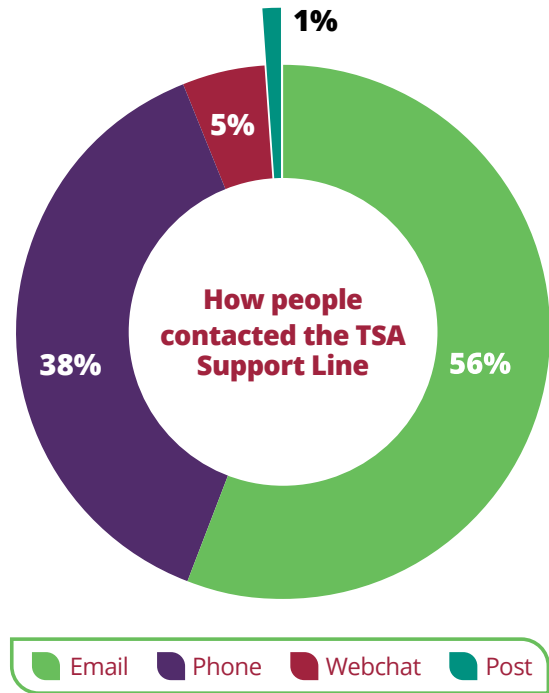
1. Professional supporting someone with TSC (doctor, teacher, social worker etc.)
2. Parent or guardian of someone with TSC
3. Wider family member (auntie, cousin etc.)
4. Someone who has TSC themselves
5. Other family members or loved ones

It's exciting to see that the TSA is now attracting TSC professionals, as **it demonstrates the belief in the TSA as a trusted and valued source of information and support.**



TSA Support Line

The TSA Support Line is completely free to the TSC community, providing a source of information and a listening ear. The TSA Support Line is available through telephone (0808 801 0700), email (support@tuberous-sclerosis.org), webchat and post. In 2022, we had 412 new enquiries, with an average of 8 enquiries every week.



TSA Support Fund

The TSA Support Fund provides small grants to people in the TSC community when they've reached financial crisis. People apply to the TSA Support Fund for things like household items, travel costs for medical checkups, or carer respite. In 2022, the TSA provided **£4,450** to the TSC community through the TSA Support Fund.

“The Support Fund enabled us to purchase equipment for my daughter’s specialist pushchair, making daily life more manageable for us all. It was an absolute lifeline. **TSA Support Fund recipient**”

335
attendees
registered for all events

2,175
views
of event recordings
on our video hub

17 **events**
in 2022

including:

- Medical talks about TSC
- Living well with TSC
- A special series of music therapy sessions

TSA events in 2022

2022 saw a welcome return to face-to-face TSA events, the first since the start of the Covid-19 pandemic. This included Outlook, our event for adults mildly affected by TSC, taking place in Durham. The planned return of Big Day in 2022, our biggest annual event for everyone in the TSC community, had to be postponed due to nationwide rail strikes. However, Big Day did take place once more in 2023.

Improving TSC care across the NHS

Throughout 2022, we continued to work very closely with NHS TSC clinics across the UK. This involved things like helping people get referrals to TSC clinics, connecting clinicians with other professionals, and working with TSC clinicians to answer medical questions all about TSC.

Other ways that we worked with NHS TSC Clinics in 2022 included:

- Running **virtual TSC clinic catch ups** in every UK nation
- Hosting the **annual NHS TSC Clinics Education Meeting**, where all TSC clinical professionals shared best practice and learnings about TSC. The meeting had a record **92 registrations**
- Applying for the TSC Clinics Network to be **recognised by the NHS as a Rare Disease Collaborative Network (RDCN)**

Fundraising and income generation

Throughout 2022, the TSA's small and dedicated team worked incredibly hard to ensure that fundraising of all sorts continued!

Everyone in the TSC community and beyond were facing major pressures in 2022. Yet, our supporters dug deep.

The re-opening of the world following the Covid-19 pandemic meant that face-to-face fundraising events could return. DIY fundraising, such as gala dinners and bingo nights, were also very popular in 2022!

Beyond community fundraising, the TSA team planned for greater sustainability in our income, including more diversity from different sources. We also worked hard to ensure that costs were kept to reasonable levels.

Community Fundraising

Community fundraising is always a vital part of our overall income. In 2022, over half of our income was from people kindly giving to us directly or fundraising.

In 2022, community fundraising included:

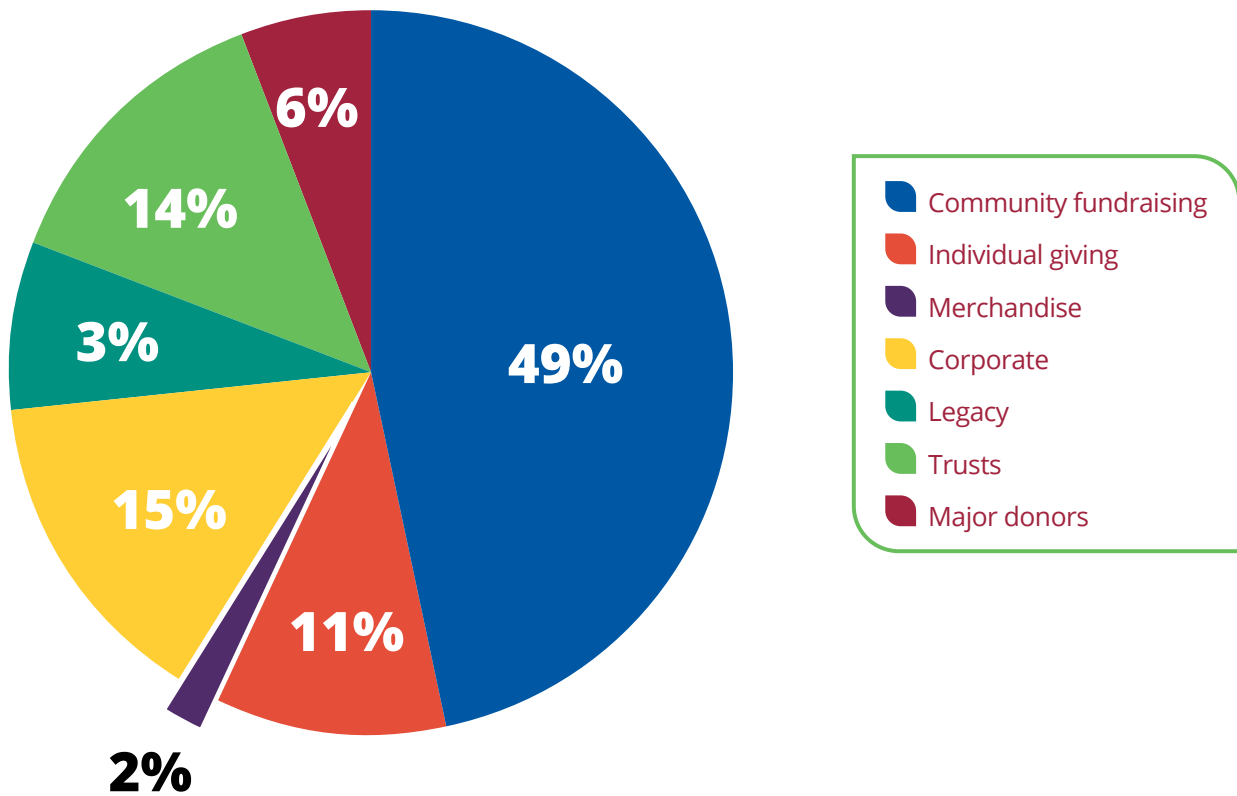
- Our incredible London Marathon runners, amounting to **£10,300**
- People giving in celebration, in memory or through legacies, raising **£19,091**
- TSC festive cards, our ever-popular campaign that amounted to **£3,642**
- Legacy and in-memory giving, with a total of **£13,091**
- The Big Give Christmas Challenge, raising **£8,000** for the TSA Support Fund. The TSA is also taking part in The Big Give 2023 - keep monitoring our channels for more information.

Sir David Suchet CBE continued to generously support the TSA throughout 2022. This included dedicating a matinee performance of his West End show 'Poirot and More: A Retrospective' to the TSC community. Sir David also kindly signed copies of his latest book, 'Behind the Lens' – this alone raised a fantastic £2,100 to support research into TSC!

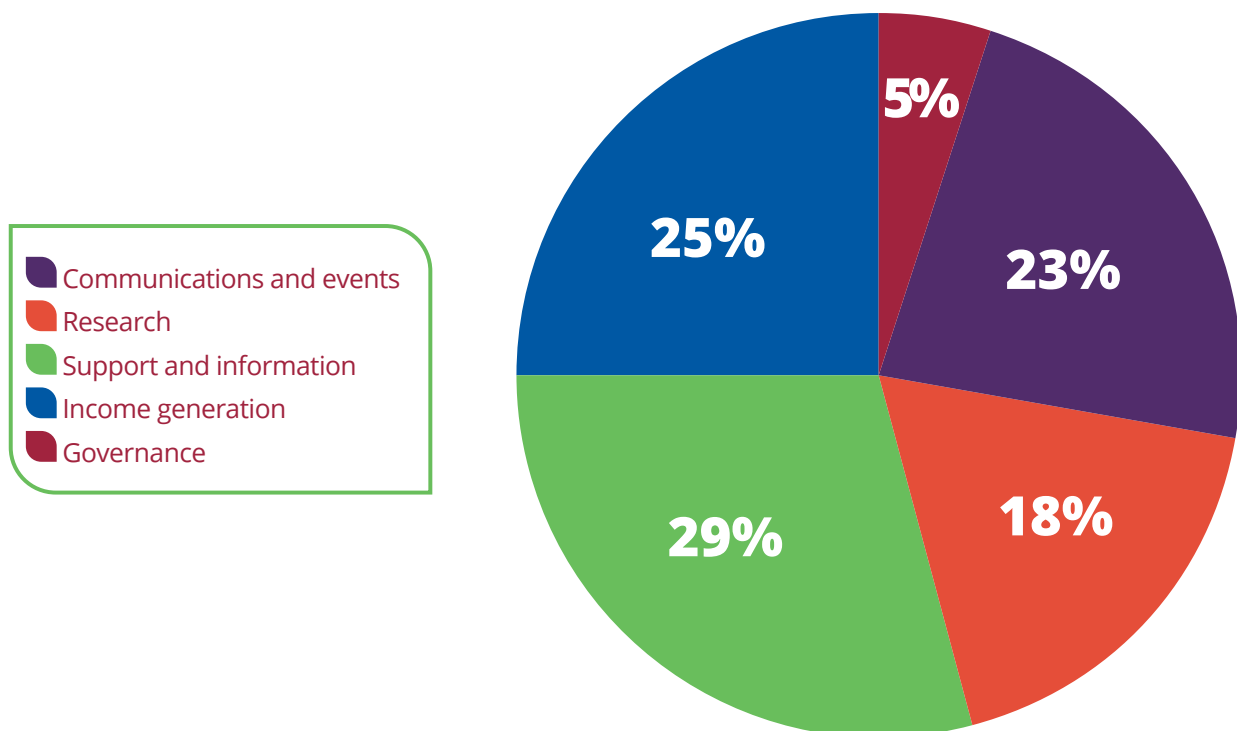
The TSA thanks all trusts, foundations and other organisations who kindly provided support to us in 2022:

- The Adint Trust
- The Thomas Sivewright Catto Charitable Trust
- The Hiscox Foundation
- The Charities Trust
- The Street Foundation
- Dunn Family Charitable Trust
- The Zedra Trust
- The Meikle Foundation
- The Harry Krish Mootsamy Foundation
- The Reed Foundation
- The Care Tech Charitable Foundation
- The Peter Sowerby Foundation
- Jazz Pharmaceuticals
- Plusultra Pharma
- Hiscox Market Operations Team
- Blinkhorns Accountancy London

Where TSA income came from in 2022



Where TSA costs came from in 2022



Driving TSC research forward

In 2022, we continued to push the TSC research agenda, with key breakthroughs across diagnosis, care and management of the condition.

Throughout the year, the TSA and our trustees remained determined to honour our research grant commitments, despite an incredibly difficult time for the charity financially. We did this by taking funding from our savings to cover the costs.

The TSA's research portfolio of previously committed grants in 2022 consisted of 9 live studies (six of which are jointly funded with other organisations). Our portfolio of ongoing projects is currently worth **£473,073.51**.

Live Research Grants table 2022

Lead researcher	Institution	Title and purpose of research
Dr Elaine Dunlop	Cardiff University	<p>Characterising the role of exosomes in TSC</p> <p>We know that TSC growths surround and hijack healthy cells, which allows the growths to get bigger. This project aims to better understand exactly how TSC cysts do this. It's hoped that this research could reveal new ways of preventing TSC cysts from growing, which would help us to develop better ways to treat and manage TSC.</p>
Professor Andrew Tee	Cardiff University	<p>Understanding the limits of everolimus and rapamycin</p> <p>This project is investigating why not all TSC growths react in the same way when treated with the medicines everolimus and rapamycin. This knowledge would allow us to create better and more effective treatments to reduce or stop TSC growths.</p>
Dr Charlotte Tye	Kings College London	<p>Designing a protocol to measure the developmental trajectory of infants with Tuberous Sclerosis Complex</p> <p>This study is focused on a home-based assessment to better monitor and improve social behaviour linked to autism spectrum disorder in TSC. The research is ongoing and aims to provide earlier ways to improve the long-term quality of life for people affected by TSC.</p>

Lead researcher	Institution	Title and purpose of research
Dr Elaine Dunlop	Cardiff University	<p style="text-align: center;">A model of cell invasion in TSC-LAM</p> <p>One of the current priorities in TSC research is to develop a model of the human circulatory system using human cells, so that scientists can study LAM in more detail. LAM (or 'lymphangiomyomatosis') is a lung condition affecting around 1 in 3 females with TSC. This new project will help us to know why TSC-LAM happens.</p> <p style="text-align: center;">Co-funded by the US Ministry of Defence.</p>
Dr Sam Amin and Dr Ingram Wright	University of Bristol	<p style="text-align: center;">Better understanding the accessibility of mental health services for children and young people with TSC</p> <p>This project is developing more appropriate and effective ways for children and young people with TSC to access mental health services and psychological support in the UK. This research hopes to demonstrate a clear benefit of providing therapies to children and young people with TSC who are experiencing psychological or emotional distress.</p> <p style="text-align: center;">Co-funded by an anonymous major donor.</p>
Professor Petrus deVries and Professor Anna Jansen	University of Cape Town and UZ Brussels	<p style="text-align: center;">The TANDem Project</p> <p>The TANDem Project brings together a worldwide team of families affected by TSC, researchers and clinicians to provide scientific evidence for greater intervention and treatment of TSC-Associated Neuropsychiatric Disorders (TAND, which includes anxiety, depression and challenging behaviour). The two-part project will first focus on the development of a self-report TAND checklist and identification app, to measure how people are affected by TAND. The second part of the study will investigate the best ways to treat TAND.</p> <p style="text-align: center;">Co-funded by the King Baudouin Foundation.</p>
Ms Shoba Srivastava	University of Cape Town	<p style="text-align: center;">TAND in India</p> <p>This PhD project will ask families affected by TSC and their clinicians what they know about TAND, what their TAND needs are and what their priorities would be for TSC-related action in India. It'll examine how useful certain TAND identification and management strategies currently are for families in India, with the potential for findings to have worldwide importance.</p>

Lead researcher	Institution	Title and purpose of research
Dr Liesl Schlebusch	University of Cape Town	<p style="text-align: center;">Well-Beans for Caregivers Programme</p> <p>This study is investigating the feasibility, acceptability, and potential impact of a World Health Organization-developed Caregiver Skills Training programme when used in the TSC community.</p>
Dr Stacey Bissel	Birmingham	<p style="text-align: center;">Exploring Sleep experiences in people with TSC (e-Slept)</p> <p>Problems with sleep, such as daytime sleepiness and waking up during the night, are a major challenge in the TSC community. The e-Slept project aims to document sleep challenges in people with TSC, by asking their caregiver to document the experiences. It's hoped that this will ultimately help to better manage sleep problems in TSC.</p>

Our research portfolio continues to develop important results and next steps:

- **Four** live projects that we're funding are providing annual progress updates
- **Three** projects are set to be published in nine academic journals
- **Two** projects reported other forms of engagement (such as presenting their work at important academic conferences or workshops)

Working in partnership to fund TSC research

In 2022, we had two fantastic opportunities to work with external partners to drive more research into areas that are most important to our community: TSC-Associated Neuropsychiatric Disorders (TAND) with the TANDem consortium, and TSC-related epilepsy with Epilepsy Research UK (ERUK).



We are proud to be joining forces with the TSA. The research funded through this important collaboration will provide vital evidence needed to improve diagnosis, treatment and prevention of epilepsy in TSC.

Maxine Smeaton, Epilepsy Research UK Chief Executive

TSC Research Volunteer Network

Established in 2021, the TSC Research Volunteer Network is our way to encourage and empower people living with TSC and their families to help shape the future of TSC research. The official term for such a group is 'Patient and Public Involvement' (PPI), a vital way to ensure that the research we fund will have the biggest impact.

The network now has a total of **29 volunteers**, including individuals with TSC, parents and siblings. In 2022, **11 new volunteers** signed up to be a part of the network. Throughout 2022, the network's volunteers helped in many different ways including:

- Attending a research conference organised by Epilepsy Research UK
- Ensuring that the work of researchers was impactful and meaningful to people affected by TSC
- Reviewing different leaflets and other documents on behalf of TSC researchers and other organisations. This helped ensure that content was written in a way that was easy to understand for non-researchers
- Participating in workshops focused on the NHS Genomics Programme, a plan to create a pilot study that offers whole genome sequencing to all newborns

New treatments for TSC

2022 was a big year for new medicines becoming available to the TSC community across the UK. The TSA, alongside the TSC community, worked hard to ensure that decision-makers approved funding for these important medicines.

One highlight for medicine access in 2022 was when the Scottish Medicines Consortium approved funding of cannabidiol (brand name Epidyolex) to treat TSC-related epilepsy. This meant that the TSC community in Scotland had access to this new medicine from May 2022. Cannabidiol was previously approved by the All Wales Medicines Strategy Group in winter 2021. 2022 also saw decision-makers in England start the process of deciding whether to say 'Yes' or 'No' to the medicine (thanks to the TSA and TSC community, the 'Yes' decision was achieved in early 2023).

In 2022, England decision-makers also worked with the TSA to start their review of a topical sirolimus cream for TSC-associated facial angiofibromas (TSC growths on the face). The decision on this is due in 2023.

TSA Strategic Report 2022

The TSA Strategic Report 2022 is a deeper look into the innerworkings of the TSA's strategy and planning, not only for 2022 but also for 2023 and beyond. The strategic report includes reflections on how the TSA fulfilled its objectives for 2022, a financial review of the TSA, and a snapshot into future plans for the charity.

A message from our Chair

This report forms part of the TSA's annual report and accounts, which are submitted to UK regulators. You can obtain a full copy of the report and accounts by emailing admin@tuberous-sclerosis.org.

The full report and accounts will also be available through the TSA website (www.tuberous-sclerosis.org), Companies House (www.gov.uk/government/organisations/companies-house) and the Charity Commission (www.gov.uk/government/organisations/charity-commission).

The Independent Examiner's report does not identify any areas of concern where accounting rules were not followed, disclosures not fully made or accounting records incomplete.

On behalf of the Trustees

Best wishes,
Sanjay Sethi
TSA Chair



SANJAY SETHI, CHAIR

Objectives and activities

Purpose and Strategy

The TSA has three main charitable objectives:

- Support individuals affected by TSC, together with their families or carers
- Encourage and support research into the causes and management of TSC
- Provide education, publicity and information to promote awareness of problems caused by TSC

The TSA is in the fourth year of a five-year plan that has been disrupted by Covid-19 (2019-2023). This plan sets out a clear vision for the charity's mission to provide help for today and a cure for tomorrow

We plan to:

- REACH more people - significantly increase the number of people the TSA supports by 2023
- Drive more REseArCH - stimulate the same amount of investment in TSC research during 2019-2023 as was invested by the TSA over the previous five years
- Balance the TSA's finances by 2021 - having invested in research in recent years, we need to reduce expenditure and grow income so that the charity is sustainable in the long-term

The Aims, Objectives, Enablers and Values that deliver the strategy

Our strategic **aims** are to:

- Positively impact on the health and wellbeing of people living with TSC, families and carers
- Lead research into tools, technologies and treatments which eliminate the effects of TSC
- Lead innovation in the integration of medical and social care

Our strategic **objectives** (how we will do it) are to:

- Deliver quality, easily accessible and timely information about TSC and living with the condition
- Provide opportunities for people with TSC and their families to connect, support and learn together
- Build the knowledge and expertise of professionals working with people with TSC
- Drive the research agenda to improve outcomes and quality of life for people with TSC
- Expand our understanding of the condition to accelerate diagnosis, intervention and development of new treatments
- Disseminate and use evidence to influence NHS policy and practice
- Campaign for better access to treatments and care from the NHS
- Support the development of centres of excellence for supporting people with TSC and their families
- Pilot, then roll out, innovative approaches to whole person, family and carer support

Our strategic **enablers** that make it possible are:

- Effective teamwork by staff working together to achieve a shared vision
- High-quality, integrated financial management
- Digital transformation and smart working to reach more people
- An enterprising approach to income generation to drive more research
- Having values that are inclusive, ambitious and effective

2022 Business Plan

The first three years of our 2019-2023 strategy saw the TSA significantly reduce expenditure to address the charity's structural deficit while simultaneously improving the quality of our services and increasing our reach into the TSC community.

However, due to cost-of-living impact and working with a smaller team than originally envisaged in 2022 and beyond, we focussed our resource on a smaller number of strategic objectives in 2022 to help the TSA community and the professionals who work with them. These were:

Providing access to good quality, accessible and timely information

- Develop an integrated package of support for individuals living with TSC and their families on TSC-Associated Neuropsychiatric Disorders (TAND) which can be delivered across our support line, website, social media, and regular monthly webinars
- Continue to build and improve the TSA website and social media channels, working to achieve the Patient Information Forum accreditation as a kite-mark of service quality.

Providing the opportunity for people with TSC to connect, support and learn together

- Deliver a slimmed-down Big Day so people can connect face-to-face, as well as virtually across other events, with fewer formal sessions and more time for networking.
- Carry out a 'TSC Census' to better understand who the TSA is supporting, where there are gaps in the people we reach, and how we can reach them.

Capacity build the knowledge and expertise of professionals working with people with TSC

- Develop and launch virtual training for residential and home support workers which will help them to provide better support for individuals living with TSC and their families.
- Develop and launch virtual training for SEND leads, teachers and teaching assistants which is ready for launch in September 2022, helping those who have a child with TSC joining their new class to understand how best to support them over the coming year.
- Support the NHS TSC Clinics Network through a range of activities including a virtual annual NHS TSC clinics day and a revival of the role of medical advisers.

Driving the research agenda with the aim of improving outcomes and quality of life for people living with TSC

- Continue to support the TSA's current portfolio of funded grants, as some projects will continue into 2022 due to delays in data gathering caused by the pandemic.
- Repurpose the money ring-fenced for a TANDem workshop to open a TAND-focused flexi-grant round, open to researchers across the globe.
- Develop and deliver the UK Natural history of epilepsy and standards of care in TSC, working closely with the Chief Investigator and Co-Investigators.
- Support review of the UK clinical guidelines on identifying and managing TSC, so it is consistent with updated international guidelines and incorporates UK guidance on new treatments.

Campaigning for better access to treatments and care

- Share the views of people living with TSC and their families on newly licensed treatments for TSC-related epilepsy (Epidyolex, cannabidiol) with UK decision-makers.
- Make sure people living with TSC and their families are involved in research by manufacturers relating to topical sirolimus treatments for facial angiofibromas.
- Continue to grow the TSA's research volunteer network to drive patient-centric research and increase investment in TSA research through patient and public involvement and engagement.

Strategic enablers

We focused on:

- **High-quality, integrated financial management.** Work with Tudor John to build skills and confidence of senior managers when using the Xero accounting system for management accounts.
- **Digital transformation and smart working to reach more people.**
 - Continue our journey to regular and timely 'digital first' communications across the website and social media.
 - Put our IT provider out to tender to see if it is possible to make savings in what has become a more competitive market due to the increase in home-workers following covid.
 - Review the TSA's IT assets such as laptops and scanners which are rapidly depreciating, and identify funding for a rolling programme of replacements.
 - Invest in the development of our website so we have the technical capability to support ambitious online fundraising campaigns by other charities that we would like to emulate.

An enterprising approach to income generation to drive more research.

- Continue to generate income by delivering patient and public involvement activities across both academic and industry research projects.
- Define a 'medium gifts' programme of donations up to £3k per annum and develop a new strategy to identify a pool of donors and potential donors who will support us.
- Review whether we can generate most income by developing a new community fundraising campaign to replace Tea and Scones or by identifying and piggybacking on an external community fundraising campaign such as the Big Give.

Strategic report

The TSA's Trustees and staff continue to be ambitious about what we want to achieve for individuals living with TSC and their families. Our mission is to provide help for today and a cure for tomorrow.

The Trustees monitor performance against three headline indicators and a broader set of key performance indicators to determine the progress of our current 5-year strategy.

Reaching more people

In 2018 there were **3,394 people** in contact with the charity including people living with TSC, their families, carers and friends. By the end of 2022 the TSA had increased this number by 55 per cent with **5,253 people** in contact with the charity.

The TSA Support Line continued to provide vital support and information by telephone and email to people living with TSC across the UK. **413 people** contacted the TSA Support Line in 2022 (408 in 2021), and **26,598 users** visited our website for information (23,458 in 2021).

Driving more research

The TSA continued to drive the research agenda by running the 2022 TANDem Seed Grant Funding Round jointly with TAND Consortium. The purpose of the grant round was to offer small 'seed' grants to international researchers that will address priority research areas identified in a recent scoping review of all TAND research to date. In order to ensure the most direct benefit to people with TSC and their families, the grant call specifically encouraged small projects that would be complementary to the TANDem project (a project already co-funded by the TSA and the King Baudouin Foundation) and/or that could 'bolt on' to the TANDem project. Following the review process, the TAND review panel awarded funding to Dr Liezl Schlebusch (University of Cape Town), Ms Shoba Srivastava (University of Cape Town) and Dr Stacey Bissell (University of Birmingham). 2022 also saw the TSA join forces with Epilepsy Research UK to jointly fund a research fellowship with a focus on TSC-related epilepsy. The selected researcher will highlight an area of epilepsy research that will

benefit not only epilepsy as a whole, but also TSC-related epilepsy. This is an exciting opportunity for TSC-related epilepsy research to extend beyond the 'walls' of TSC, into researchers and experts who may not have otherwise encountered or focused on the condition previously.

Balancing the books

Enabling and funding world-leading research into the diagnosis, treatment and management of TSC is a central pillar of the TSA purpose. However, the Trustees have made the difficult but ultimately responsible decision that further research commitments will only be made from operating surpluses to ensure that the charity is able to continue to deliver its mission from a position of financial stability. All existing research commitments continue to be met, and the charity continues to drive TSC research more broadly.

2022 was a year of transition for the TSA team with key individuals leaving the charity which impacted overall income generation. The new leadership team is committed to the 5-year strategy including the plan to balance income and expenditure to ensure the charity is sustainable in the long term. The TSA continues to see the deficit between income and outgoings reduce. This remains the primary focus of the current Joint Chief Executives, to ensure the long-term longevity and survival of the charity.

Objectives and activities

Support services and communications

The TSA remains focused on flexibly reaching more people impacted by TSC respecting geographical and cultural diversity.

We continued to provide the TSA Support Line, which enables people across the UK to contact our small team of professional advisers confidentially by telephone, email, webchat and post (9am to 5pm on Monday to Friday). We were contacted by around 32 people each month in 2022 asking for help and support. Some callers wanted practical information, others needed a listening ear and emotional support.

We also provided proactive information and resources through our website (www.tuberous-sclerosis.org) and signpost to them through our popular social media channels (we use Facebook for people living with TSC and their families, Twitter for researchers, clinicians, and opinion formers in the media and Parliament, and LinkedIn for other professionals).

The TSA has always been proud to offer support and information to everyone in the TSC community, including the training of professionals focused on the condition. To further improve our capabilities and impact of training professionals, in 2022 we developed online training packages for social and education professionals. The modules cover vital areas of TSC, to allow these professionals to offer the best support possible to people with TSC. The modules, which will be eligible for Continual Professional Development points (CPD, ongoing education that many professionals must do every year) are expected to launch in 2023.

During 2022 we continued to host our popular virtual events, giving the TSC community the opportunity to virtually come together in order to network, receive support and information, and learn more about TSC as a condition.

We organised regular virtual events focused on the concerns that people were expressing on the TSA Support Line. These events were recorded and shared online so they were accessible for families with caring responsibilities. Virtual events in 2022 included:

- Talks on the physical and medical impact of TSC, such as an overview of TSC and its relationship to the lung condition lymphangioleiomyomatosis (LAM), the kidneys, and women's health
- Meetings with specific NHS TSC clinics, allowing people to better understand their role and how to access such clinics
- Virtual get-togethers, such as our festive event in December 2022 for Outlook – our group for adults mildly affected by TSC

In 2022, we returned to hosting face-to-face events for the first time since the covid-19 pandemic. In April 2022, we welcomed adults mildly affected by TSC to the longstanding Outlook meeting. Hosted in Durham,

the event gathered **21 attendees**. The TSA had planned to re-introduce Big Day, our annual face-to-face meeting for everyone in the TSC community, in 2022. However, Big Day needed to be postponed to 2023 due to rail strikes.

During 2022 we awarded **£4,500 across 19 grants** from the TSA Support Fund to individuals and families. The TSA Support Fund provides small discretionary grants confidentially to our community, offering a lifeline to individuals and families when the financial implications of TSC overwhelm them. We prioritise community members in receipt of benefits or on low incomes who are unlikely to have savings.

The TSA worked hard with all decision-makers across the UK in discussions regarding access to cannabidiol (brand name Epidyolex) for TSC-related epilepsy. Following a hard series of campaigning, the TSA was delighted to secure funding agreements in Scotland and Wales for cannabidiol in 2022, with Northern Ireland also following. Indication from England decision-makers that they were likely to reject funding of the medicine led to the TSA spearheading an in-depth campaign to change their mind – this subsequently led to a 'Yes' for England, in early 2023.

We continued to build new and solidify existing relationships with TSC clinicians and NHS TSC clinics throughout the UK. Regular contact with TSC clinicians helps to:

- Act as a trusted partner to the clinics supporting people with TSC and their loved ones
- Provide support and information to the TSC community outside of clinics, including practical information as well as a listening and empathetic ear
- Act a central pillar between clinicians, other professionals and the TSC community
- Ensure that as many people as possible are registered with an NHS TSC clinic

We formed successful alliances with other charities and worked with them on a range of activities and initiatives with the common aim to raise the profile and importance of rare disease with parliamentarians and policy makers.

During 2022, we continued to work closely with colleagues from the Specialised Healthcare Commissioning Alliance (SHCA) and Genetic Alliance to raise awareness of the need to the views of patients and families when making decisions about treatments for rare diseases. We continued our membership of the Embracing Complexity Coalition to raise awareness of the need for joined up support from health and social care for complex conditions, and the Council for Disabled Children to improve access to services for children living with TSC and their families.

Two issues of the TSA's popular community magazine, Scan, were published in 2022. The magazine is an important element of the TSA's support and information, providing an update to households on key TSC news and TSA projects. Scan was delivered to around **2,000 households in 2022**.

Research

Our research team is committed to driving more research to give people living with TSC and their families hope for the future. The TSA is the only UK charity dedicated to supporting TSC research, with an **in-year research grant commitment in 2022 of £473,073.51**.

The charity's Trustees remain determined to honour the research grant commitments that we have made and decided that the Designated Endowment Fund would be used to cover the cost of our ongoing research grants programme over 2020-22.

The TSA's research portfolio of previously committed grants in 2022 consists of **9 live grants (6 of which are jointly funded)**.

Our portfolio of ongoing projects is worth **£473,073.51**.

TSC Research Volunteer Network

In 2022, **11 new volunteers** signed up to be a part of the TSC Research Volunteer Network – the TSA’s patient and public involvement and engagement programme. Established in 2021, the network aims to encourage and empower people living with TSC and their families to help shape the future of TSC research. The network now has a total of **29 volunteers**, including individuals with TSC, parents and siblings.

Throughout 2022, the TSA offered a range of opportunities to volunteers, including:

- Attending a conference organised by Epilepsy Research UK at the Frances Crick Institute in London. The event covered a range of research topics, with network attendees also raising awareness of TSC.
- Helping TSC researchers apply for funding from major funders. This was done by reading summaries of the researcher’s work, to ensure that the work was impactful and meaningful to people who are affected by TSC on a daily basis.
- Giving feedback on draft information leaflets written by pharmaceutical companies conducting a trials into TSC medicine. The leaflets will help explain the studies and how to be a part of them.
- Reviewing different leaflets and other documents on behalf of TSC researchers working on TSC projects. This helped ensure that content was written in a way that was easy to understand for non-researchers.
- Participating in workshops focused on NHS Genomics Programme. The Newborn Genomes Programme is a plan to create an NHS pilot study to offer whole genome sequencing (WGS) to all new-borns. It is hoped that WGS of newborns in NHS England will make it quicker and easier to identify and also treat rare conditions like TSC.

New treatments for TSC

In early 2022, Scottish Medicines Consortium approved Epidyolex (generic name ‘cannabidiol’) to treat TSC-related epilepsy. The news means that the TSC community in Scotland have been able to access this important new medicine from May 2022. The drug was approved by the All Wales Medicines Strategy Group in winter 2021.

2022 also saw decision makers in England start the appraisal process for Epidyolex and topical sirolimus cream for TSC-associated facial angiofibromas with final outcomes to be announced in 2023.

Income generation

The last few years have been unprecedented for many charities, with the TSA being no exception. UK businesses and charities were optimistic that 2022 would be a year of replenishment and returning to normality following the devastating consequences of covid-19. However, in reality the long-term financial impact of the covid-19 pandemic continued to devastate the TSA in 2022. This was further exacerbated in fresh challenges through rapidly rising inflation and a major cost of living crisis.

The result of this three-pronged challenge for income generation in 2022 led to: Reduced means for people to give to charities (either directly through donations due to personal financial challenges, or in fundraising due to resourcing challenges of this); Greater numbers of applications to trusts and foundations, diluting this important income stream; and charities being unable to take greater risks in fundraising, reducing innovation. As a rare disease charity whose supporter base is smaller than others, the TSA felt these financial challenges even harder than most charities in 2022.

The TSA’s small and dedicated team has continued to work incredibly hard to mitigate risks to the charity’s income whilst minimising expenditure.

We are extremely grateful to the following grant-making trusts and foundations for their crucial unrestricted support towards core costs throughout 2022:

- The Zedra Trust (£3,000)
- The Thomas Sivewright Catto Charitable Trust (£750)
- The Hiscox Foundation (£3,320)

- The Adint Trust (£5,000)
- The Charities Trust (£1,000)
- The Street Foundation (£3,000)
- The Dunn Family Charitable Foundation (£3,000)

In addition, we received unrestricted grants from Jazz Pharmaceuticals (£12,000) and Plusultra Pharma (£10,000) which was a welcome boost for the charity.

We would also like to thank the following trusts who have supported us with restricted funding to enable us to continue to develop specific areas of our work:

- The Meikle Foundation (£2,000) for our ongoing work in Scotland
- The Harry Krish Mootosamy Foundation (£2,000) for giving us the opportunity to deliver virtual music therapy sessions
- The Reed Foundation (£1,500) who generously contributed funding to assist with discretionary grants through The TSA Support Fund
- The Care Tech Charitable Foundation (£5,000) and the Peter Sowerby Foundation (£10,500) both kindly enabled us to establish a social care education training programme for professionals to enhance their knowledge and understanding of Tuberous Sclerosis Complex and the impact it has on individuals and families

The generosity of grant-making trusts and foundations who supported us in 2022 enabled us to continue to deliver our vital services to the individuals and families impacted by TSC.

Our sincere thanks go to our corporate supporters. Hiscox Market Operations Team (London) fundraised during 2021/22 and their efforts were kindly match funded by the Hiscox Foundation, bringing the total raised by the group to **£6,640**. We are also hugely grateful to Blinkhorns Accountancy (London) for their generous donation of **£27,696**.

The TSA would like to thank the amazing people who took part in the October 2022 TCS London Marathon on behalf of the charity. We are in awe of the dedication and time they all gave to train for such an iconic event, and we are thrilled that our team of runners raised a fantastic **£10,300**. In addition, a 'virtual runner' completed the 26.2-mile distance on his own and raised a further **£1,165**.

Sir David Suchet CBE continued to generously support the TSA throughout 2022 through a range of means including a West End matinee performance of 'Poirot and More, A Retrospective' that he dedicated to the TSA community. In addition, as a part of the 750-year celebrations of the Fishmongers Company, Sir David kindly signed copies of his latest book, raising a fantastic **£2,100** to support research into TSC.

Leaving a gift in a will is a wonderful and powerful gesture that enables a person to support the TSA even when they are no longer here. For the family left behind, they are able to take great comfort that their loved one has left behind a legacy to be proud of. We would like to formally record a huge thank you to everyone who supported us in this way. In memory donations and legacies raised **£13,091** for the TSA this year.

Facebook donations have become popular with fundraisers as they are an easy way to support the TSA and raise money for milestone celebrations, such as birthdays and anniversaries. Throughout 2022, this income stream raised over **£6,000** for the TSA.

In December 2022, the TSA's Big Give Challenge raised vital funds for our TSA Support Fund. The fund offers a lifeline to individuals and families affected by TSC when the financial implications of the condition become too much to carry and they need financial assistance. We met this target to raise **£4,000** for the TSA Support Fund through the Big Give Christmas Challenge in seven hours. This was then doubled by the Big Give and the Reed Foundation to **£8,000**.

The response to our 2022 Christmas cards was fantastic, raising **£3,642** for the TSA. The winner of our Christmas card competition was talented illustrator Annaliese Howard (who is mildly affected by TSC) with her 'Snowman and turkey' card.

Despite the challenges so many people faced throughout 2022, our fundraisers dug deep and went above

and beyond for the TSA. We have a growing number of individuals who raised money by taking part in outdoor activities such as fun runs, other walking and running events and cycle rides. Other fundraisers enjoyed organising activities such as bingo nights and gala dinners. We hope 2023 will provide a year of steady growth and stability within the TSA as we look to sustainably generate income from more diverse and varied sources.

Financial review

Grant making policy

Research grants are given for purposes directly concerned with furthering an understanding of TSC and its treatment. The TSA is a member of the Association of Medical Research Charities (AMRC) and follows AMRC policy in the conditions attached to awarding grants. All applications are peer reviewed.

Benevolent grants through the TSA Support Fund are given to individuals and families facing the financial impact of TSC. The TSA Support Fund provides a grant for a wide-range of items and experiences, such as household items, respite care and travel costs to hospital visits. All applications for assistance are reviewed by a senior lead and grants awarded confidentially where deemed appropriate, based on the Association's guidelines. Trustees are eligible to apply for such grants and are dealt with in the same way as any other applicant.

Statement on reserves and review of financial position as of 31st December 2022

At the end of the year, the TSA had total funds of **£829,711** consisting of:

- A Designated Fund of **£649,711** (further details as below) which are anticipated to be spent down in the coming years
- Non designated reserves of **£180,000** representing six months of expenditure excluding any research commitments

About the Designated Fund

The Designated Fund largely arises from a bequest in 1993 by the late Mr H I Leech which does not form part of the TSA's normal reserves.

This Fund has been broadly treated as an "endowment" and invested with professional advisers to provide a resource which supports the TSA in delivering its charitable objectives. In particular, it enables the Trustees to:

- Fund or co-fund out of capital (and, if need be, at short notice) major medical research projects which are determined by the Trustees to be crucial for the treatment of TSC
- Help fund the charitable activities from investment income, thereby helping to secure the future of the charity and provide continuing support to future and current people affected by TSC

In recent years, the balance on the Designated Fund has reduced significantly as a result of both additional research commitments and the provision of additional services, the cost of which have not been covered by the income of the Association.

The trustees are of the opinion that the Designated Funds should be retained to a minimum of **£300,000**.

This allows the TSA to have at minimum sufficient funds equivalent to 10 months of outgoings, as well as the means to instigate major medical research projects, co-funded by third parties, at short notice if necessary.

Financial result for the year

The Association's total income for the year was **£284,639** (2021 **£443,454**).

Total investment loss was **£87,971** (2021 **£52,835** gain). To fund deficits and payments relating to research commitments entered into during the year, some investments needed to be sold to provide the Association with additional working capital.

Adjusting for the investment loss, the overall deficit for the year was **£170,183**. This represents a significant decline on the surplus of **£19,922** in 2021. Pre-investment deficit in 2022 was **£75,688**, compared to **£32,913**

in 2021. This decline of **£42,775** was primarily driven by the net and non-recurring benefit of hosting the International TSC Research Conference in 2021, which resulted in additional income of **£73,399**. Excluding the international conference, pre-investment net expenditure for 2021 is **£106,312** which provides a better view of underlying performance and allows a more meaningful comparison with other years including 2022. On this basis, the 2022 pre-investment deficit of **£75,688** is **£30,624** better than the adjusted 2021 pre-investment deficit of **£106,312**. This reflects the continued efforts of the leadership team and their management of resources.

Political and macro-economic uncertainty in 2022 contributed to higher inflation and interest rates which in turn had an adverse effect on equity and bond markets. This resulted in a reported loss of on investments of **£94,495** in 2022 which was **£147,330** worse than 2021 which saw a gain of **£52,835**.

After adjusting for the investment loss, the overall deficit for the 2022 was **£170,183**, which is **£190,105** lower than the **£19,922** surplus in 2021.

Future plans

The 2022 Business Plan focuses on the following strategic objectives:

Ensuring that the TSA is on solid ground across all teams, with a view to long-term stability

- Recruit a new fundraising team (Head of Fundraising and Fundraising Officer) and establish a clear fundraising strategy across all income streams for the immediate and long-term
- Recruit new hires across TSA teams, providing stronger foundations and resilience for a healthier and happier charity that also mitigates risk of future exits
- Ensure that the TSA's internal practices and structures are up-to-date and easy to follow, including access to all banking and other procedures

Providing access to good quality, accessible and timely information

- Develop further support materials and resources for the TSC community. Namely, an improved 'TSA Welcome Pack' to make the first steps of a TSC diagnosis easier
- Continue to build and improve the TSA website and social media channels, working to achieve the Patient Information Forum accreditation as a kite-mark of service quality
- Undergo audit of TSC website, updating and removing content as needed to better represent the digital-first TSA and TSC community

Providing the opportunity for people with TSC to connect, support and learn together

- Explore opportunities for virtual peer-to-peer support, such as in the form of virtual events, one-to-one discussions or other forums
- Deliver a slimmed-down Big Day which had to be postponed from November 2022 to May 2023 due to rail strikes. Big Day will allow the TSC community to connect face-to-face, with the focus on networking and feeling like part of a community
- Deliver face-to-face Outlook event, connecting adults mildly affected by TSC
- Continue to deliver ambitious calendar of virtual events, ensuring that geographical location is not a barrier to the TSA

Capacity-build the knowledge and expertise of professionals working with people with TSC

- Launch the NHS England-recognised TSC Rare Disease Collaborative Network (RDCN). If application unsuccessful, work on appeal process to ensure the right decision is made
- Further solidify relationship with NHS TSC clinicians through regular educational communications focused on improving diagnosis, care and management of TSC
- Support the NHS TSC Clinics Network through a range of activities including a virtual annual NHS TSC Clinics Education Day

Driving the research agenda with the aim of improving outcomes and quality of life for people living with TSC

- Work with Epilepsy Research UK on the successful application to the joint-funded 'Emerging Leader' award
- Support review of the UK clinical guidelines on identifying and managing TSC, so it is consistent with updated international guidelines and incorporates UK guidance on new treatments
- Continue to grow TSA's research volunteer network to drive patient-centric research and increase investment in TSA research through patient and public involvement and engagement

Campaigning for better access to treatments and care

- Continue to work with pharmaceutical partners and foster strong relationships in anticipation of pipeline product launches (ie, topical sirolimus treatment for facial angiofibromas)
- (Should NICE reject Epidyolex (cannabidiol) funding application for TSC-related epilepsy after second appraisal), work with key stakeholders – including the TSC community – to appeal to NICE in order to get the decision that the TSC community deserve
- (Should NICE accept Epidyolex (cannabidiol) funding application for TSC-related epilepsy), work with stakeholders to ensure that access to medicine and awareness of its availability is done efficiently

**Our mission remains unchanged:
To provide help for today and a cure for tomorrow**

The TSA Summary Statement of Financial Activities

For year ended 31 December 2022

	Unrestricted funds £	Restricted funds £	2022 Total funds £	2021 Total funds £
INCOME AND ENDOWMENTS FROM				
Donations and legacies	236,732	4,500	241,232	344,236
Charitable activities				
Research	22,000	-	22,000	77,969
Other trading activities	3,668	-	3,668	6,290
Investment income	17,740	-	17,740	14,959
Total	280,140	4,500	284,640	443,454
EXPENDITURE ON				
Raising funds	84,558	-	84,558	127,006
Charitable activities				
Family Care & Support	163,661	4,500	168,161	162,472
Research	106,330	-	106,330	186,889
Total	354,549	-	359,049	476,367
Net gains/(losses) on investments	(87,971)	-	(87,971)	52,835
NET INCOME/(EXPENDITURE)	(162,380)	-	(162,380)	19,922
RECONCILIATION OF FUNDS				
Total funds brought forward	992,091	-	992,091	972,169
TOTAL FUNDS CARRIED FORWARD	829,711	-	829,711	992,091

The Tuberosus Sclerosis Association Summary Balance Sheet

For year ended 31 December 2022

	Unrestricted funds £	Restricted funds £	2022 Total funds £	2021 Total funds £
FIXED ASSETS				
Investments	642,707	-	642,707	906,723
CURRENT ASSETS				
Debtors	3,385	-	3,385	90,740
Cash at bank	270,842	-	270,842	202,171
	274,227	-	274,227	292,911
CREDITORS				
Amounts falling due within one year	(87,223)	-	(87,223)	(207,543)
NET CURRENT ASSETS	187,004	-	187,004	85,368
TOTAL ASSETS LESS CURRENT LIABILITIES	829,711	-	829,711	992,091
NET ASSETS	829,711	-	829,711	992,091
FUNDS				
Unrestricted funds			829,711	992,091
TOTAL FUNDS			829,711	992,091

**Everyone at the TSA
would like to give thanks
to our Board of Trustees
for all of their work:**

Sanjay Sethi - Chair

Dr Frances Elmsli - Vice Chair

Rajan Khullar - Treasurer

Annemarie Cotton

Chris Kingswood

Jane Rogers

Martin Short

Thomas Carter

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association