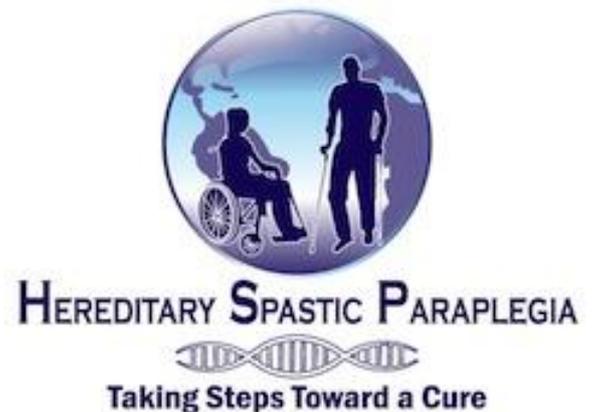


16th Annual Report and Financial Statements
of the HSP Research Foundation Inc.

2020/21



16th ANNUAL REPORT 2020/21

to members of the HSP Research Foundation Inc.

September 2021

Summary

- The COVID-19 global pandemic continues to significantly impact Foundation research and community activities, either directly or indirectly. Foundation operations were only mildly impacted however, as we do business almost exclusively electronically.
- A major focus remains on finding effective treatments for the HSPs with distinct progress on biomarker and biomarker assay development. However, circumstances have thus far precluded implementation of a Phase 1 clinical drug trial.
- \$98,000 of Foundation funding has been invested in the 'Towards a Cure' HSP Research Program in 2020/21.
- In the 2020 calendar year, \$113,000 came in through fundraising, 5% short of the \$120,000 annual target.
- Over the financial year, \$145,000 was given by 250 individual contributors - 160 community members and 90 non-members. The 160 represents 26% of families in the HSP community.
- Community member numbers have grown by net 2.7% to 732. New members are largely from Australia, but with additions as well from the US, UK, Iceland and the Philippines.
- A thorough review and assessment of risk to privacy and security of the Foundation's systems and processes is in progress, with enhancements already in place for the website. Improved security policies and procedures in the conduct of everyday business have been developed and implemented.
- A large, wide-ranging survey of community members' views, needs and interests was conducted, with initiatives that are responsive to the survey results in development.

Foundation Strategy

Mission/Purpose (what business are we in?)

To serve the interests and needs of people with Hereditary Spastic Paraplegias (HSPs) by:

- facilitating and funding research to find an effective treatment for the HSPs
- being the hub of the HSP community, creating awareness and providing information, education and support.

Vision (where do we want to be?)

To have effective treatments for the HSPs that are:

- highly effective
- widely available (globally) and
- readily affordable.

Values (what do we stand for?)

- preserve and enhance the dignity, respect for and privacy of people in the HSP community that we serve
- create and maintain opportunity and equality in participation for all community members
- be guided by evidence-based, peer-reviewed and reputable science
- be globally collaborative, facilitative and constructive with other HSP support groups, foundations, researchers and their organisations, clinicians and other medical professionals

- to maximise learning, understanding and positive impact
- maximise the funding that goes towards establishing effective treatments for the HSPs
 - be businesslike and professional in everything we do.

Distinctive competency (what do we need to be distinctively good at?)

Skilled and effective in community, relationship and strategy development, interfacing with and between the HSP community, medical researchers and clinicians, other support groups and other stakeholders to mutual benefit in implementing our mission and achieving our vision.

Foundation Focus

The major focus remains on finding an effective treatment for the HSPs with distinct progress on biomarker and biomarker assay development, but frustratingly slow progress in getting a clinical drug trial for SPG4, the most prevalent type of HSP underway.

We also continue to support the HSP community with collective and individual information and education efforts on living with HSP, covering clinical and genetic diagnosis, management and treatment, family planning, mental health and quality of life aspects.

The research program can only happen with the financial support of the HSP community. We are only as strong as our community and our Foundation. *If it is to be, it is up to us!*

This report describes the extent to which progress was made towards implementing and fulfilling our purpose over the 2020/21 financial year.

Towards a Cure HSP Research Program

The COVID-19 pandemic and attendant restrictions noticeably hampered progress, either directly or indirectly, throughout the 12month reporting period, with the proposed clinical trial and biomarker studies being the most impacted.

Clinical trial SPG4

The clinical trial aim is to test the effectiveness of a candidate drug for treating SPG4 HSP, by far and away the most prevalent form of HSP, associated with mutations in the SPAST gene. The decision was made in the first quarter of 2021 to separate the Phase 1 and 2a clinical trials in an effort to accelerate progress during the year. A documented plan or synopsis for an enhanced standalone Phase 1 trial of drug safety and tolerability at the doses to be tested was finalised and quotes obtained for all aspects of the conduct of the trial.

As a result of the pandemic, the demand for and cost of running early-stage clinical trials in Australia ballooned during the year, with a price tag for the Phase 1 trial more than double an earlier estimate. The Foundation Committee decided not to proceed with funding the trial at that point. Due diligence demanded closer review of the evidence for taking the drug into clinical trial at the higher cost for the trial than the review done for what was expected to be a much less expensive trial.

Drug supply for the Phase 1 trial was secured in May 2021, although what should have been a relatively straightforward and inexpensive procurement became both costly and complicated. The drug is not available in Australia and had to be sourced overseas. Disruptions to the supply of all manner of pharmaceutical goods has occurred in Australia during the pandemic.

Biomarker development

Measuring the effect of drug treatment in a clinical trial is a major aim of the biomarker development program. To help achieve this aim, significant redesign and automation of the [manual assay](#) for the blood biomarker was needed, given the narrow time window between sampling, processing and analysis required in a clinical trial setting. Dr Wali delivered a fully automated and validated assay for measuring the effect of drug treatment on a marker of microtubule dynamics in May 2021 – a major achievement.

A second biomarker based on skin samples, also being developed by Dr Wali, is aimed at HSP diagnosis and drug testing. A novel machine-learning-guided, high-content microscopy image analysis approach to developing this biomarker is being employed.

A third biomarker study with support funding from the Foundation was finalised in Germany with the results published. This blood biomarker has unique diagnostic potential.

Movement measure

The smartphone app study to quantify and characterise a movement signature in individuals with HSP has now concluded. The hope was that changes in individual movement signature could reflect the impact of treatment in clinical trials. However, the high variability in HSP in individuals, even over short time periods, made such a benchmark impossible to establish.

Stem cell research SPG7

Dr Wali is actively engaged in research on SPG7, following a similar pathway to the SPG4 research. He and the team at the Kolling Institute in Sydney are evaluating olfactory stem cells developed from tissue samples taken from people with SPG7 mutations to help understand cell functions and mechanisms that are impaired in this HSP type. When sufficiently complete, this understanding will be used to screen for potential drug treatment candidates. The team published an [important paper](#) on a study of mitochondrial function in both SPG7 and SPG4.

Alan Mackay-Sim

Principal Investigator and head of the HSP Research Program since inception, Prof Alan Mackay-Sim, published an [important paper](#) in March proposing novel pathways for drug discovery with the potential to positively impact many forms of HSP. Alan was also honoured with [membership of the Order of Australia](#) in June for significant service to tertiary education and biomedical science.

Funding

\$98,000 of Foundation funding has been invested in the HSP Research Program in 2020/21.

Reports

Progress reports on the HSP Research Program were published on three occasions over 2020/21 and can be accessed via these links:

<https://hspersunite.org.au/hsp-research-program-update-september-2020/>

<https://hspersunite.org.au/research-program-update-december-2020/>

<https://hspersunite.org.au/research-program-update-march-2021/>

Living with HSP - Management & Treatment

Mobility maintenance and management is a major concern for people with HSP as are other manifestations of HSP that can significantly impact quality of life. Relevant issues and medical specialties include neurology, urology, rehabilitation, orthopaedics, spasticity management, pain management, psychology and specialised therapies including physiotherapy, neurophysiotherapy, podiatry / orthotics and occupational therapy.

The Foundation provides [regular, ongoing information](#) on fitness, self-managed exercise programs, assistive technologies, lifestyle and quality-of-life issues in managing symptoms. Other topics covered include genetics, family planning and mental health. Articles on the numerous aspects of HSP management and treatment can be found in the [Living with HSP](#) section of the website, representing a significant resource.

There is a lot that people with HSP can do to mitigate and alleviate the broad range of symptoms experienced. Physical fitness, especially strength and flexibility, and maintaining correct body weight can make a significant difference in the quality-of-life experienced with HSP. One of the best investments that people with HSP can make is to see a neurophysiotherapist, or a physiotherapist whose practice focuses on neurological conditions and their rehabilitation, for assessment, treatment and the development of a custom-designed, self-managed exercise program that when done regularly as prescribed, has the potential to maximise the quality-of-life achievable at any given stage of HSP progression.

Developing the HSP Community

Information, Education & Support

Information, education and support for the HSP community takes on multiple forms and formats:

- The website is updated quarterly with highlights of the most important recent research studies concerning both the quest for effective treatments for HSP and for the management and treatment of the symptoms of HSP, aimed at enhancing quality of life.
- The website also contains up-to-date information on government policy and funding decisions with relevance to the HSP community, notably the National Disability Insurance Scheme (NDIS). There are also stories of social and human interest to community members.
- The website is a resource valued by the HSP community, researchers and health professionals alike from around the world, with an ever-expanding library/archive. Statistics for the 2020/21 year:
 - 3,258 repeat users
 - conducted 10,400 sessions (3.2 sessions/average repeat user over the year)
 - of 2min 41sec average duration per session
 - viewing 24,253 pages (7.4 different pages viewed/active user/year)
 - from 77 countries, with:
 - 36% of repeat users from Australia
 - 25% USA
 - 9% UK
 - 5% Canada
 - 3% India
 - with Germany, Ireland, Italy, China and the Netherlands filling out the top 10 in that order, each under 2%.
- Facebook use continues to increase. Over the course of the year, there were:

- 46 posts on the [Foundation's Facebook page](#) (an increase of 170% year-on-year)
- reaching 11,700 followers (38% increase)
- most popular post was for Rare Disease Day 2021, closely followed by fundraising, mental health issues in light of COVID-19 and a recent post from the HSP group in Japan covering people with HSP who competed in the Tokyo Paralympics.
- 993 likes (940 last year)
- with 15% in the 18-34 age bracket, 55% aged 35-54 and 30% over the age of 55 with a female to male ratio of 70/30.
- 40% of followers live in Australia, 34% in the USA, 9% in the UK, 3% in Canada and then India, Italy, Ireland, Philippines, Netherlands and New Zealand. This demographic profile is remarkably similar to that for our website.
- Email communications from members, non-members, clinicians (both specialists, and GPs) and researchers mostly include questions about diagnosis, symptoms, treatments, genetic testing, the research program, medical and allied health resources in the member's locality, requests for intervention with doctors, hospitals or healthcare systems, the NDIS and questions about participation in the proposed clinical trial. Every email is responded to personally, either by return email or phone call or both, as most appropriate. Sometimes a dialogue is necessary involving multiple communications over an extended period. This supports community members and others in getting the information and understanding required to maximise the quality of the numerous aspects of their lives that are, or have the potential to be, impacted by HSP.

For those who may have relatives or friends in the HSP community who do not use the internet, offering them the opportunity to browse or search the website and read Foundation emails using your devices and with your help, is valuable support. Another way for them to access the Foundation website or Facebook page regularly is through the local library or other council facilities, where assistance is often available for those who require it, or through clubs and groups for seniors.

Growing the Community

As of 30 June 2021, the Foundation's HSP community numbered 732 members, a net increase of 19 members or 2.7% year-on-year. The best estimate is that about half of community members have HSP, with the remainder comprising family, friends and supporters.

It is estimated that there are currently around 1,950 people with HSP in Australia with perhaps 20% of this number being community members recorded on our database. This is in line with percentages of the overall HSP population who join HSP support groups in other countries. Given our small size, growing the community is important for sustainability and to ensure continuing support, funding, the ability to take on initiatives, and to reflect HSP community confidence in the direction and work of the Foundation.

Connecting with each other

The Foundation facilitates community members connecting, interacting and having their say. Communications are almost exclusively electronic with the website, e-mail and Facebook being the main methods, supported by phone calls as appropriate. Opportunities for face-to-face social interaction have largely been denied this year due to the ongoing global pandemic.

Global Collaboration

With 580,000 globally estimated to have HSP and only about a dozen national support groups known to exist, continual development of communication and dialogue with our international colleagues is in the mutual best interests of everyone with HSP everywhere. The Foundation continues to communicate with our global colleagues every quarter, sharing news and updates, as well as any time that a need or opportunity presents itself.

Raising Awareness

Opportunities for increasing public awareness of HSP are difficult to create due to the rareness of the condition, perceived irrelevance to the bulk of the population, the large number of rare diseases (over 6,000) the lack of differentiation and distinctiveness from other conditions, the lack of regular newsworthy information, and the strategic choices to channel our limited resources elsewhere. Some support organisations for other rare diseases have been successful in establishing a significant profile, recognition, public awareness and fundraising, mostly through creative and well-resourced initiatives, demonstrating what is possible.

Community Survey

130 HSP community members responded to 50 questions in a survey covering a wide range of community-related topics. Here is a summary of the main findings:

- Around 80% of respondents have HSP, are satisfied with the Foundation, which they hold in good repute and would recommend to others for membership.
- Respondents are aged from 18 to over 75 (68% over 55). The female:male mix is 55-45. The metro:regional mix is 2:1.
- 75% are aware of the Foundation's fundraising, but only just over 50% are aware of the role of the Foundation in facilitating and funding research.
- Just over 50% are aware of the Foundation's community, education and information activities.
- 26% feel well-informed by the Foundation with information relevant to them or family members, with a further 62% feeling somewhat informed, with the majority of that group saying that they would like to know more.
- Almost 40% have mentioned the Foundation to a health professional, but fewer than 20% of survey respondents heard mention of the Foundation from a health professional.
- The [Foundation website](#) is the primary preferred source of information on HSP for 40%, with neurologists the primary preferred source for 20%.
- The website is generally accessed by community members at each quarterly update.
- The website content is regarded as both highly reliable and reputable.
- 80% of respondents think that fundraising activities are pretty good or better with 66% of respondents having contributed at least once.
- 52% of those who have never given say they can't afford to; 17% prefer not to say why; 13% give to other charities.
- 50% think that the Foundation should do more to help people connect with each other, with 40% unsure.
- Respondents confirmed the Foundation focus on education and information.

Read the [full report](#).

Finance

Fundraising

Over the financial year, \$145,000 was given by 250 individual contributors - 160 community members and 90 non-members. The 160 represents 26% of families in the HSP community. The December 2020 and June 2021 fundraising campaigns raised \$35,000 and \$57,000 respectively, much the same as the previous year. The combined total from the fundraising campaigns represents 63% of the \$145,000 financial year total. Over \$39,000 came in through birthday fundraisers and other events, where people engaged their social media networks to get behind them and help raise funds for HSP research.

This highlights both the importance of these two annual campaigns and also of non-fundraising campaign related income, such as through regular giving and individually inspired and undertaken initiatives to raise funds for HSP research, including utilising social media networks to attract funds in lieu of gifts for birthdays or other notable personal events; and sporting/physical challenges.

Who gives, how, and how much?

One of the larger challenges we face is expanding the base of contributors to the Foundation. Contributors are categorised in two groups - inner circle (members of the HSP community) and outer circle (supporters or potential supporters outside the HSP community). There were 250 different contributors over the financial year, significantly higher than the average of previous years. It is difficult to draw inferences, but a concern is that some community members may not be sufficiently engaged to be motivated to give. Giving occurs through a number of channels – mainly by direct electronic funds transfer, PayPal, Good 2 Give (regular workplace giving), Everyday Hero and Give Now.

A major impediment to fundraising overall is the lack of a fundraising strategy and people with the requisite skills to implement it. This is an area of Foundation activity currently under review.

Summary

With an expenses ratio of under 0.1% in 2020/21, 99.9% of all incoming funds in the financial year is directly available to fund HSP research. We are proud of the fact that this can happen only because we are a 100% volunteer organisation that is committed to professionalism in all that we do. In the 2020 calendar year, \$113,600 was raised, missing the nominal \$120,000 annual target for fundraising by \$6,400 or 5%.

Foundation Operations

Communications

Electronic communications: We have a current, working email address for 94% of the community who choose to receive email communications for themselves and/or their families, up 3% from last year. We utilise e-mail communications with community members where possible. Everyone is encouraged to provide us with an e-mail address, and for those community members for whom e-mail is not feasible, we encourage them to explore the possibility of receiving e-mails via family, friends or carers. It is possible that a significant percentage of email addresses on file are defunct. A campaign to update email addresses for community members is warranted.

Developing our Capability & Capacity

Apart from the limitations associated with the level of funding we attract, the major impediment to developing our capability and capacity is the lack of skilled volunteers to develop and manage strategic portfolios, notably in fundraising and promotion, and in communications/community development. It is hoped that community focused initiatives reflecting the results of the major recent survey will address some of these needs.

An ongoing challenge, like all small non-profit organisations, is how to be effective and businesslike so that minimal resources can be effectively employed, given that we are run totally by volunteers. We are maintaining and continuously improving systems and processes:

- to maintain an accurate and complete database of community members with whom we interact and communicate to maximise the effectiveness of our contacts management
- to service the handling and processing of general inquiries and requests for information on HSP
- for correspondence and for initiatives such as fundraising campaigns and financial transaction processing.

Security & Privacy

Maintenance of the integrity of the Foundation's systems and processes to ensure the security of data and guard the privacy and confidentiality of community member and other information is a matter of the highest priority. With the ever-expanding volume and type of threats to cyber security being universally experienced by individuals and organisations everywhere, a thorough review and assessment of risk was initiated late in the financial year and is ongoing. The Foundation website has had both a visual makeover and a strengthened security framework with new software, a new host providing enhanced security including systematic backups. The reviews and assessments cover not just structures and systems, but also internal processes, procedures with documented policies aimed at implementing and maintaining best-in-class practices to minimise risk and strengthen security. This initiative will likely run until the end of calendar 2021.

Volunteers & Committee

Members of the HSP community are indebted to a few key people who are instrumental in the smooth running of the Foundation. Sincere thanks to our long-term volunteers Isaac Nakhla (website, email, filing system) and Ryan Keating (Contacts Management System) for their tireless efforts over many years, including central roles in the security review underway. Sincere thanks also to committee members for 2020/21, Ken Price (Secretary/Treasurer) Margaret Flood, Diane Thompson, Anthony McInnes, Greg Emery and Julian Pereira for their diligence and effort in ensuring that the interests and needs of the Foundation and members of the HSP community are being well served.

Governance

Members of the HSP community can be confident that the Foundation and the business it conducts is being well managed and well done. Financial and other business records are full, accurate and complete. Expenditure outside of research funding is tightly controlled, amounting to a record low this financial year of just 0.08% of total income. The Foundation mission/purpose is pursued with singular focus. Succession continues to represent an issue of risk that needs to be addressed soon with increased priority.

The Foundation is registered with the Australian Charities and Not-for-profits Commission (ACNC) set up under the Federal Department of Treasury as the national regulator of charities. They also provide support to charities to assist with their compliance obligations in the form of online tools, of which the Foundation takes full advantage to not only ensure compliance but also to implement enhancements.

Future Strategic Direction

Awareness and fundraising focused outside the HSP community would be good for the sustainability of the Foundation's dual mission – facilitating and funding research to find effective treatments for the HSPs; and providing information, education and support to the HSP community. The challenge to make this happen is being considered as an integral part of initiatives to significantly enhance community engagement.

Various areas of Foundation activity are the subject of review and planning for new initiatives, including enhancing:

- engagement with, accessibility to and adoption by the HSP community of technical information on the management and treatment of HSP
- use of communications channels differently to increase engagement and social activity
- fundraising aimed at increasing net revenue especially from sources outside the HSP community.

The research program focus remains on facilitating and funding the development of effective treatments, currently clinical drug trials for SPG4 HSP; stem cell investigations of SPG7 HSP; development of biomarkers; promising cell morphomics studies; and gene therapies for rarer forms of the HSPs.

Financial Statements

A Summary of the Financial Report of the Foundation for the 2020/21 financial year is included in this report. The Office Bearers of the Foundation are satisfied about the report's quality and reliability in every respect. We have the capacity to meet our current and foreseeable commitments and are confident of our continuing financial good standing into the future.

Comments or Feedback

I submit this Annual Report to you, the members of the HSP community and the Foundation, in good faith and with optimism and confidence that we are currently fulfilling our mission and achieving worthwhile goals, and expect to continue that into the future. Comments or feedback, thoughts or ideas, or questions that members may wish to ask are welcome. E-mail to: admin@hspersunite.org.au.



Frank McKeown
President

HSP RESEARCH FOUNDATION INCORPORATED
ABN 46 648 875 912

SUMMARY OF FINANCIAL REPORT 2020/21

PROFIT AND LOSS STATEMENT FOR THE YEAR ENDED 30 JUNE 2021

	2021	2020
INCOME		
HSP Donations received	145,316	105,367
Bank Interest received	11,231	26,231
ATO refunds (GST)	1,671	3,590
Joan and John Battisson Legacy	3,043	0
Refund	0	2893
Total Income	<u>161,261</u>	<u>138,081</u>
EXPENSES		
Other expenses	136	307
Research Expenditure	98,783	23,567
Total Expenses	<u>98,919</u>	<u>23,874</u>
Operating Profit/Loss	<u>62,342</u>	<u>114,207</u>
Operating profit before income tax	62,342	114,207
Retained profits	1,099,463	985,256
Profit available for appropriation	1,161,805	1,099,463
RETAINED PROFITS	<u>1,161,805</u>	<u>1,099,463</u>

HSP RESEARCH FOUNDATION INCORPORATED
ABN 46 648 875 912

BALANCE SHEET FOR THE YEAR ENDED 30 JUNE 2021

	2021	2020
CURRENT ASSETS		
Cash Assets	1,161,805	1,099,463
Total Current Assets	<u>1,161,805</u>	<u>1,099,463</u>
TOTAL NET ASSETS	<u>1,161,805</u>	<u>1,099,463</u>
EQUITY		
Retained profits	1,161,805	1,099,463
TOTAL EQUITY	<u>1,161,805</u>	<u>1,099,463</u>

A copy of the full Financial Report is available to members on request.