

15th Annual Report and Financial Statements
of the HSP Research Foundation Inc.

2019/20



15th ANNUAL REPORT 2019/20

to members of the HSP Research Foundation Inc.

September 2020

Summary

- Detailed planning for a Phase 1/2a clinical drug trial commenced towards the end of the financial year, made possible by a second drug dose range finding study that was successfully completed with conclusive results.
- Three biomarker studies continue to progress - two are focused on compounds in blood and skin as potential markers of HSP status and progression; the third is a smartphone app study capturing signature gait/mobility data of individuals with HSP and comparing results over time.
- \$24,000 of Foundation funding has been invested in preclinical investigations as part of the *Towards a Cure* HSP Research Program in 2019/20.
- In the 2019 calendar year, \$117,000 came in through fundraising, just short of the \$120,000 annual target.
- Over the financial year, \$105,000 was given by 150 community members, representing 24% of families in the HSP community.
- Community member numbers have net declined by 4% from 743 to 713, with 21 new members joining and 51 no-longer-active community members (contact details no longer current). New members are largely from Australia, but with additions as well from France, Chile, the Philippines, the USA, Qatar, the UK and Lebanon.
- Natural disasters have played a role in Foundation operations with two committee members facing prolonged disruption from bushfire threat over the 2019/20 summer and another two being somewhat impacted as well. The COVID-19 global pandemic is hampering and delaying research activities, however Foundation operations were only mildly impacted by the pandemic, a distinct advantage of being a virtual organisation without physical premises, doing business almost exclusively electronically.

Foundation Purpose

Mission: To serve the interests and needs of people with Hereditary Spastic Paraplegia (HSP) by facilitating & funding research to find an effective treatment and by being the hub of the HSP community, creating awareness and providing information, education and support.

Vision: to have treatments for the HSPs that are:

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

Values:

- the dignity, respect for and privacy of people in the HSP community that we serve are of the utmost importance
- opportunity and equality in participation for all community members
- to maximise the funding that goes towards establishing effective treatments for the HSPs

- to be globally collaborative and facilitative with other HSP support groups, foundations, researchers and their organisations, clinicians and other medical professionals to maximise learning, understanding and positive impact
- to be businesslike and professional in everything we do.

Distinctive competency: effectiveness interfacing with and amongst the HSP community, medical researchers and clinicians, other support groups and other stakeholders, to mutual benefit.

Foundation Focus

The focus on finding an effective treatment for the HSPs continues with ongoing preclinical studies and detailed planning underway for a clinical trial to test the effectiveness of a drug treatment for SPG4, the most prevalent type of HSP. We also continue to foster and support the HSP community with collective and individual information and education efforts. The research and clinical trials 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 fulfilling our purpose over the 2019/20 financial year.

Towards a Cure HSP Research Program

The Foundation funds and facilitates an HSP Research Program aimed at establishing an effective treatment for HSP. Currently this comprises preclinical studies as the initial phase of the clinical trials process to test the effectiveness of a candidate drug for treating SPG4 HSP, by far and away the most prevalent form of the disease, associated with mutations in the SPAST gene.

Calculating drug dose

Following inconclusive results of the dose range finding study in mice that was completed in March 2019 after 22 months and at a cost of \$186,000 to the Foundation, a second, redesigned mouse study was successfully completed earlier this year to provide the basis of evidence for a clinical trial dosing regime.

Clinical trial planning

Detailed planning for a Phase 1/2a clinical trial, which hinged on success in the second dose range finding study, finally got underway in earnest late in the financial year. Rigorous documentation of plans is required to satisfy ethical and regulatory requirements in clinical trials. To this end, initially a Synopsis, to be followed by a Protocol, to document the clinical trial plans is being developed. Phase 1 of the clinical trial will test the safety and tolerability of the proposed drug treatment over a range of levels not previously approved for human use. The Phase 2a study is designed to test the preliminary effectiveness of the candidate drug at various dose levels in people with SPG4 HSP over a six-month period. The exact design of the Phase 2a study will be dependent on the outcomes and results of the Phase 1 study.

Biomarker studies

A major challenge is how to measure HSP treatment effectiveness in a clinical trial. For example, how would we know if a drug stopped disease progression given that HSP is only slowly progressive and the rate of progression can vary a lot even for the same person over their

lifetime? What about improvements in mobility, balance, walking speed or even levels of fatigue? Anyone with HSP can tell you that the severity of symptoms can change from day to day, depending on how well they slept, what the temperature is, how much stress they are experiencing, time of day, how regular they have been with exercise, and so on.

Reliable measures of HSP status and changes in status are needed to measure treatment effectiveness in a clinical trial. Sufficiently sensitive measures for a clinical trial do not currently exist. The regulatory authorities, as well as good science, demand high confidence in measures and measurement before granting approval to conduct a clinical trial.

Three biomarker studies were in progress during the year. The most advanced of these is a blood biomarker study being carried out in Germany with results expected soon. A second biomarker study of compounds in blood and skin samples is being undertaken in Sydney is also showing promise and progressing well. A smartphone app is in testing and development to quantify and characterise individual mobility with the potential for being able to measure changes that might occur with treatment. No testing was possible in the financial year, being scheduled for March but postponed due to the pandemic.

\$24,000 of Foundation funding has been invested in these studies in the HSP Research Program in 2019/20.

Progress reports were made each quarter over 2019/20 and can be accessed here:

<https://hspersunite.org.au/hsp-clinical-trial-program-update-september-2019/>

<https://hspersunite.org.au/hsp-clinical-trial-program-update-december-2019/>

<https://hspersunite.org.au/hsp-clinical-trial-program-update-march-2020/>

<https://hspersunite.org.au/hsp-clinical-trial-program-update-june-2020/>

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.

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.

Articles on multiple aspects of HSP management and treatment can be found in the [Living with HSP](#) section of the website. This is a significant resource with specific interests served by the Search function available at the top of every page of the website. Articles in this section cover numerous aspects of living with HSP.

Communicating effectively with members of the HSP community on Living with HSP could be significantly enhanced. The Foundation has never had people with professional competence in this area and is a topic for review.

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 every quarter 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 2019/20 year:
 - 6,400 active users (down 17% year-on-year)
 - conducted 15,250 sessions (2.4 sessions/average active user over the year)
 - of 3 min 27 sec average duration per session
 - viewing 43,700 pages (7 different pages viewed/active user/year)
 - from 153 countries, with:
 - 36% of all users in the USA
 - 26% Australia
 - 9% UK
 - 5% Canada
 - 3% India
 - with Germany, Netherlands, South Korea, Italy and France filling out the top 10 and each under 1.5%.
- Email communications from members arrive in a steady stream, mostly with questions about diagnosis, symptoms, treatments, genetic testing, 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 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 their HSP.
- The Foundation's Facebook page remains popular and active, with a total of 940 'Likes', an annual increase of 15%. There were 17 Foundation posts to the page over the year with a total audience of around 8,500 people.

For those who may have relatives or friends in the HSP community without computers, offering them the opportunity to browse or search the website and read Foundation emails represents valuable and tangible support. Alternatively or additionally, a way for them to access the website regularly is through the local library, where assistance is often available for those who require it, or through over 50's clubs and groups.

Growing & Developing the Community

As of 30 June 2020, the Foundation's HSP community numbered 713 members, a net decrease of 30 members or 4%. 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. Given our small size, it is important to continue the focus on growing the community with the aim of achieving critical mass and sustainability, thus ensuring 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 happen thanks to the independent Australian HSP Facebook support group that initiates and convenes get-togethers in multiple locations around the country. Here is the list for 2019/20 with plans suspended for many months due to the pandemic:

- 8 July 2019 - Newcastle/Central Coast NSW; (venue Cardiff RSL Club)
- 20 July - Perth WA (Principal Bar & Restaurant Midland)
- 25 August - Sydney NSW (Campbelltown Catholic Club)
- 13 September - Brisbane Qld - cancelled due to member affected by bushfires
- 29 September - Newcastle/Central Coast NSW (Everglades Country Club Woy Woy)
- 10 October - Melbourne Vic (Zagame's Berwick Hotel)
- 24 October - Adelaide SA (The Republic Hotel Norwood)
- 2 November - Sydney NSW (Club Palm Beach)
- 10 November - Brisbane Qld (Warner Tavern)
- 29 March 2020 - Perth WA - cancelled due to COVID-19
- 31 March - Melbourne Vic - cancelled due to COVID-19
- 3 April - Brisbane Qld - cancelled due to COVID-19.

We lend support with communications about the events to community members in the various locations. The social events are reported in articles that are amongst the most popular on the website.

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. We collaborate most closely with the SP Foundation in the USA, including seeking opportunities to jointly fund research studies.

Raising Awareness

Opportunities for increasing public awareness of HSP are limited 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 choice to channel the limited resources available towards research to find an effective treatment. 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.

Review

With the changing nature of communications and their role in community development, these various aspects of Foundation activity under the umbrella of community development now especially warrant review.

Finance

Fundraising

Over the financial year, \$105,000 was given by 150 community members, representing 24% of families in the HSP community. The December 2019 and June 2020 fundraising campaigns raised \$28,000 and \$32,000 respectively, not much different from the previous year. The combined total represents 57% of the \$105,000 financial year total.

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 sporting/physical challenges; selling goods specifically designed for charitable fundraising; and utilising social media networks to attract funds in lieu of gifts for birthdays or other notable personal events.

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 150 different contributors over the financial year, significantly lower than the average of previous years. It is difficult to draw inferences, but a concern is that 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 also an area of Foundation activity for review.

Summary

With an expenses ratio of just 0.3% in 2019/20, over 99% 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 2019/20 financial year, the nominal \$120,000 annual target for fundraising was missed by \$15,000 (14%).

Foundation Operations

Communications

Electronic communications: 91% of the community have provided us with an e-mail address for themselves and/or their families, the same as 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. 9% of community members not receiving emails, the main form of Foundation communications with the community, is too high and warrants attention.

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. Three new committee members came on Board in late 2019, however bushfires and the COVID pandemic have slowed the implementation of potential community development initiatives and the full realisation of their added value at this point. An exploration of alternative business models and organisation design is still warranted.

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.

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) and Ryan Keating (IT) and to committee members for 2019/20, Ken Price (Secretary/Treasurer) Margaret Flood, Diane Thompson, Anthony McInnes and Julian Pereira for their volunteer efforts in the interests of the Foundation and its members.

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 records are full, accurate and complete. Expenditure outside of research funding is tightly controlled, amounting to less than 1% of total income in the financial year. Security of the member database and confidentiality of its contents are a high priority with both being actively managed to professional standards. The Foundation strategy is pursued with singular focus. Succession continues to represent an issue of risk that needs to be addressed.

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 is to make this happen as a major new strategic thrust, possibly involving a different or additional organisational form and structure.

Various areas of Foundation activity have been identified for review, including communications – enhancing accessibility and adoption of technical information on the management and treatment of HSP, and in sophistication of communications channels to enhance community development; fundraising – aimed at increasing net revenue especially from sources outside the HSP community; and the all-volunteer business model and current organisation design – aimed at addressing structural, chronic limitations that have been identified.

The unswerving focus on doing what it takes to have clinical drug trials for SPG4 HSP is unchanged, while research activities investigating SPG7 HSP are expanding.

Financial Statements

A Summary of the Financial Report of the Foundation for the 2019/20 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 2019/20

PROFIT AND LOSS STATEMENT FOR THE YEAR ENDED 30 JUNE 2020

	2020	2019
INCOME		
HSP Donations received	105,367	126,766
Bank Interest received	26,231	21,042
ATO refunds (GST)	3,590	1,490
Joan and John Battisson Legacy	0	17,158
* Refund	2,893	0
Total Income	<u>138,081</u>	<u>166,456</u>
EXPENSES		
Other expenses	307	1,101
** Research Expenditure	23,567	100,202
Total Expenses	<u>23,874</u>	<u>101,303</u>
Operating Profit/Loss	<u>114,207</u>	<u>65,153</u>
Operating profit before income tax	114,207	65,153
Retained profits	985,256	920,103
Profit available for appropriation	1,099,463	985,256
RETAINED PROFITS	<u>1,099,463</u>	<u>985,256</u>

Notes

*** Refund**

Refund of portion of consulting fee deposit paid that was not expended

**** Research Expenditure**

Consultancy Fees - preclinical investigations and clinical trial	4,042	8,112
Blood biomarker study - University of Sydney	19,525	55,025
Consultancy Fees - regulatory application (USA)	0	3,519
Drug dose range finding study	0	33,546
Total Research Expenditure	<u>23,567</u>	<u>100,202</u>

HSP RESEARCH FOUNDATION INCORPORATED
ABN 46 648 875 912

BALANCE SHEET FOR THE YEAR ENDED 30 JUNE 2020

	2020	2019
CURRENT ASSETS		
Cash Assets	1,099,463	985,256
Total Current Assets	<u>1,099,463</u>	<u>985,256</u>
TOTAL NET ASSETS	<u>1,099,463</u>	<u>985,256</u>
EQUITY		
Retained profits	1,099,463	985,256
TOTAL EQUITY	<u>1,099,463</u>	<u>985,256</u>

A copy of the full Financial Report is available to members on request by emailing: admin@hspersunite.org.au or by writing to us at:

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