

13th Annual Report and Financial Statements
of the HSP Research Foundation Inc.
2017/18



**HSP Research
Foundation**

*A better deal for HSPers, their
children and their grandchildren*



HEREDITARY SPASTIC PARAPLEGIA



Taking Steps Toward a Cure

13th ANNUAL REPORT 2017/18

to members of the HSP Research Foundation Inc.

September 2018

Summary

- Over \$300,000 of Foundation funding has been invested in pre-clinical investigations in 2017/18. The investigations include a dose modelling study and four biomarker studies.
- Genetics testing availability and affordability has taken another big step forward. One US company is now offering testing of a significant panel of HSP types from a saliva sample, with a four-week turnaround of results at a cost of US\$250.
- In the 2017 calendar year, the \$120,000 annual target for fundraising was missed by over \$60,000 or more than 50%.
- The Joan and John Battisson Legacy, a bequest to the Foundation in the amount of \$938,500 was established during 2017/18.
- An Australian-based clinical trial is planned to launch in the coming financial year.

Foundation Purpose

Mission: to facilitate and fund research to find an effective treatment for Hereditary Spastic Paraplegia (HSP) and also, as the hub of the HSP community, to serve the interests and needs of people with HSP, creating awareness and providing support, information and education.

Vision: to have effective 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 between the HSP community, medical researchers and clinicians, other support groups and other stakeholders to mutual benefit.

Foundation Focus

Your Foundation Committee continues support of preclinical studies on the path to establishing a clinical trial to test the effectiveness of a drug treatment for HSP. We also continue to grow, foster and support the HSP community. 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 achieving our purpose over the 2017/18 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 start of the clinical trials process to test the effectiveness of a candidate drug for treating SPG4 HSP, associated with mutations in the SPAST gene.

Calculating drug dose

A major challenge in the program is working backwards from a target drug concentration in the corticospinal neurons to the type, rate and frequency that a person with HSP would ideally be taking the drug. A modelling study to address this question was undertaken in the second half of 2017 by Model Answers, the findings of which led to a dosage study in mice being initiated in early 2018. The animal study, performed by TetraQ, was completed around the end of the first quarter this year, with the development of the analytical test and the analyses of mouse brain and spinal cord tissue due for completion by QIMR at the end of 2018. The end result of this significant body of work is the establishment of drug dose levels and dosing regimen for the clinical trial.

Biomarker studies

An even bigger challenge is how to measure HSP treatment effectiveness in a clinical trial. For example, how would you know if a drug stopped disease progression given that HSP is only slowly progressive and the rate of progression can vary a lot? What about improvements in mobility, balance, walking speed and so on? Anyone with HSP can tell you that the severity of symptoms can change from day to day, depending on how well you slept, what the temperature is, how much stress you are experiencing, time of day, how regular you have been with exercise, and so on.

Unless there is a quick and radical improvement in symptoms with the drug, valid and scientific measures of HSP status need to be developed. The regulatory authorities need this sort of proof of effectiveness before they will approve any treatment being made available.

Four biomarker studies were initiated in 2017/18, the most advanced of these is a blood biomarker study being carried out in Germany; a second blood biomarker study has been initiated in Sydney, with urine also being examined for specific compounds; a specialised brain imaging study, also in Sydney, is in the advanced planning stages; and finally, a smart phone 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.

Just over \$300,000 of Foundation funding has been invested in these initiatives in the HSP Research Program in 2017/18.

Progress reports were made each quarter over 2017/18 and can be accessed here:

<https://hspersunite.org.au/hsp-research-program-update-september-2017/>

<https://hspersunite.org.au/hsp-research-program-update-december-2017/>

<https://hspersunite.org.au/hsp-clinical-trial-program-update-march-2018/>

<https://hspersunite.org.au/hsp-clinical-trial-program-update-june-2018/>

HSP Genetics

Both the availability and affordability of next-generation genetic testing for HSP and many other conditions continues to head in the right direction. Genetic testing services both within Australia and globally are maintaining and updating comprehensive HSP testing panels. A U.S.-based commercial company is now offering testing of a significant panel of HSP types from a saliva sample, with a four-week turnaround of results at a total cost of US\$250.

Discovery of new pathological variants of known HSP genes accelerated over the past 12 months, with the largest number of published papers ever, from around 20 countries across the globe. Conversely, no new HSP genes were found.

Living with HSP - Management & Treatment

Mobility maintenance and management is a major concern for HSPers. Relevant issues and medical specialties include neurology, rehabilitation, orthopaedics, spasticity and pain management and treatment, 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 HSPers can do to mitigate and alleviate symptoms, and the range of symptoms experienced by HSPers is vast. 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 HSPers 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 a 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 excellent Search function available at the top of every page of the website. Articles in this section cover every imaginable aspect of living with HSP.

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 valued resource by HSPers, researchers and health professionals alike from around the world, with an ever-expanding library/archive. Statistics for the 2017/18 year were not available at the time of preparing this report. Instead, here are extrapolated

monthly figures based on August and September 2018: an average of 805 active users of the website per month, having 1,088 separate sessions per month.

- 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 clinical trial. Every email is responded to personally, either by return email or phone call or both, as 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 is both popular and active, with a total of 691 'Likes', an annual increase of 8%. There were 25 Foundation posts to the page over the 12 months with a total viewing audience of around 10,600 people. The Foundation's Facebook friends are:
 - 72% female, 28% male (almost the same as last year)
 - 42% from Australia; 33% USA; 9% UK; 3% Canada; 2% India, with the remainder from 38 countries in Europe, Asia and South America (almost the same as last year)
 - 69% are in the 25–54 age range, with 28% aged 35–44. 27% are in the >55 age group.

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 2018, the Foundation's HSP community numbered 681 members, a net increase of 1.6% over the previous year, with 38 new members joining. 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 1,775 HSPers in Australia with perhaps 25% 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 provide a source of volunteer support for the work of the Foundation.

Connecting with each other

It is a continuing challenge for the Foundation to facilitate community members connecting, interacting, and having their say in the Foundation. Communications are almost exclusively electronic with the website, e-mail and Facebook being the main methods.

Given that HSP is a rare disease and Australia is a big country, it is a huge challenge to create opportunities for personal interaction. We encourage people to initiate social contact with community members who may live near them, and we offer support to help make that happen. An Australian HSP Facebook support group that is independent of the Foundation has initiated and convened several social get-togethers, with events being held in Canberra, Perth, Adelaide, Melbourne, Brisbane and Sydney over the year – a great effort with worthwhile outcomes! The

Foundation encourages and has gladly supported these initiatives with email communications to potential attendees in the various target areas. The Foundation also reports on these activities in website updates.

Global Collaboration

With over half a million people 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.

We continue to collaborate closely with the SP Foundation in the USA, including seeking opportunities to jointly fund clinical trials studies. We highly value this relationship and continue to seek opportunities to leverage impact through joint effort.

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.

However 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, such as the Mitochondrial Foundation's "Bloody Long Walk" <https://www.bloodylongwalk.com.au/> that is now an annual event in nine locations around the country and has raised over \$2 million. This is an enviable achievement and demonstrates what is possible.

Finance

Bequest

The Joan and John Battisson Legacy in the amount of \$938,500 was established during 2017/18 by the will of the late HSP community member John Battisson of Victoria whose wife Joan, also deceased, had HSP. The Foundation is the grateful beneficiary of this thoughtful and generous bequest, with the funds "to be used for research towards a cure for Hereditary Spastic Paraplegia".

Fundraising

The December 2017 and June 2018 fundraising campaigns raised \$6,400 and \$34,182 respectively, representing our worst fundraising campaign financial year totals in many years. The total of donations for 2017/18 was \$74,168, again a very low figure comparatively and a very disappointing result, down roughly 30% from the previous year.

There was a lack of community initiated fundraising activities in the 12-month period for the third year running, which is a worrying trend. There is an ongoing need for community members to create, plan and implement independent fundraising initiatives.

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 and outer circle. There were 198 individual contributors in total over the financial year, a drop of 19% from 245 the previous year, with the amount given overall dropping from \$107k to \$74k, or roughly 30%. Giving occurs through a number of channels – by direct electronic funds transfer, PayPal, Good 2 Give (regular workplace giving), Everyday Hero and Give Now.

‘Inner Circle’

This is how we describe members of the HSP community. Our sincere thanks to the community member families who contributed over the financial year. However there is a significant challenge to engage the large number of community member families who have never given, not just because it will mean more funds, but because it will reflect an informed community with a willing spirit, no matter how much they are able to give. We understand that many HSPers do it tough and struggle to make ends meet, however we are confident that a significant percentage of those who currently do not give, have the capacity to do so, even if it is a small amount.

‘Outer Circle’

This is how we describe people who are supporters or potential supporters but are not members of the HSP community. This includes people in the extended family, relatives, friends, colleagues, and in the communities in which HSPers live. We have barely scratched the surface with potential fund-raising initiatives instigated by individual community members with this outer circle. The challenge is to inspire and encourage community members to fundraise on behalf of the Foundation.

A major impediment to fundraising overall is the lack of a fundraising strategy and people with the requisite skills to implement it.

Summary

With an expenses ratio of less than 1% in 2017/18, over 99% of all incoming funds in the 2017/18 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 2017 calendar year, the \$120,000 annual target for fundraising was missed by over \$60,000 or more than 50%.

Foundation Operations

Communications

Electronic communications: 90% of the community have provided us with an e-mail address for themselves and/or their families, a decline of 1% compared to 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.

Developing our Capability & Capacity

Apart from the limitations associated with the level of funding we attract, by far and away 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. Another shortage is in potential committee members with significant experience from business or the non-profit sector and who can add significant value.

The 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 all those with whom we interact and communicate to maximise the effectiveness of our contacts management; to service the handling and processing of general inquiries, requests for information on HSP; for correspondence and for initiatives such as fundraising campaigns, donation processing and receipting.

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), Ryan Keating (IT) and Jay Seneviratne (independent accountant) and to committee members for 2017/18, Ken Price (Secretary/Treasurer), Chris Faehse and Margaret Flood, for their efforts in the interests of the Foundation and its members.

Special Thanks

A big "Thank You" to Helen MacPherson, Special Counsel at law firm Norton Rose Fulbright Australia, and Jess Gledhill PhD, Registered Patent Attorney at FB Rice. Helen and Jess and their respective firms very generously provided their full professional services on intellectual property and patent considerations relating to the HSP Research Program on a *pro bono* basis. This amounted to a considerable body of work. We are enormously appreciative, grateful and thankful not only for their dedication, energy, diligence and competence, but also for their readily approachable and considerate manner of doing business with the Foundation, given our lack of experience in this area.

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. A larger committee with additional competent professionals ensuring diverse perspectives would strengthen and enhance the sustainability of leadership of the Foundation.

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 find people with the skills, motivation, commitment and energy to make this happen as a major new strategic thrust, possibly involving a different or additional organisational form and structure.

Financial Statements

A Summary of the Financial Report of the Foundation for the 2017/18 financial year is included in this report. It has been prepared by the Foundation's Treasurer and a volunteer external accountant, and audited by an independent qualified auditor whose report is included. The Committee is 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. I welcome any comments or feedback, thoughts or ideas, or questions that you may wish to ask. E-mail me at frank@hspersunite.org.au.

A handwritten signature in black ink, appearing to read 'F. R. McKeown', with a long horizontal flourish extending to the right.

Frank McKeown
President

HSP RESEARCH FOUNDATION INCORPORATED
ABN 46 648 875 912

SUMMARY OF FINANCIAL REPORT 2017/18

PROFIT AND LOSS STATEMENT FOR THE YEAR ENDED 30 JUNE 2018

	2018	2017
INCOME		
HSP Donations received	74,168	106,670
Interest received	18,180	2,898
ATO refunds	540	129
Other income	0	999
Joan and John Battisson Legacy	938,500	0
Total Income	<u>1,031,388</u>	<u>110,696</u>
EXPENSES		
Other expenses	2,457	2,917
Research Expenditure*	301,524	56,000
Total Expenses	<u>303,981</u>	<u>58,917</u>
Operating Profit/Loss	<u>727,407</u>	<u>51,779</u>
Operating profit before income tax	727,407	51,779
Retained profits at July	192,696	140,917
Profit available for appropriation	920,103	192,696
RETAINED PROFITS	<u>920,103</u>	<u>192,696</u>
* Note - Research Expenditure breakdown:		
Consultancy fees	10,150	
Software development for a movement study	5,000	
University of Tübingen blood biomarker study	112,198	
University of Sydney blood biomarker study	24,200	
Model Answers dose modelling study US\$33,800	42,892	
TetraQ mouse dosage study	77,084	
Analytical work associated with mouse dosage study	30,000	
Total	301,524	

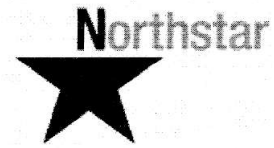
HSP RESEARCH FOUNDATION INCORPORATED
ABN 46 648 875 912

BALANCE SHEET FOR THE YEAR ENDED 30 JUNE 2018

	2018	2017
CURRENT ASSETS		
Cash Assets	920,103	192,696
Total Current Assets	<u>920,103</u>	<u>192,696</u>
TOTAL NET ASSETS	<u>920,103</u>	<u>192,696</u>
 EQUITY		
Retained profits	920,103	192,696
 TOTAL EQUITY	<u>920,103</u>	<u>192,696</u>

A copy of the full Financial Report 2018 is available to members on request by emailing admin@hspersunite.org.au or by writing to us at:

HSP Research Foundation
PO Box 4064
WARRIMOO NSW 2774



**INDEPENDENT AUDIT REPORT TO THE MEMBERS OF HSP RESEARCH
FOUNDATION INCORPORATED**

Report On The Financial Report

We have audited the accompanying financial report, being a special purpose financial report of HSP Research Foundation Incorporated, which comprises the Balance Sheet as at 30 June 2018, the profit and loss statement for the 12 months ended 30 June 2018, the Subsidiary Financial Statement Joan and John Battisson Legacy 30 June 2018, note comprising a summary of significant accounting policies and other explanatory information, and the committee's report.

Committee's responsibility for the financial report

The committee of the association is responsible for the preparation of the financial report, and has determined that the basis of preparation described in Note 1, is appropriate to meet the requirements of the Associations Incorporation Act (NSW). The committee's responsibility also includes such internal control as the committee determines is necessary to enable the preparation of a financial report that is free from material misstatement, whether due to fraud or error.

Auditor's Responsibility

Our responsibility is to express an opinion on the financial report based on our audit. We have conducted our audit in accordance with Australian Auditing Standards. Those standards require that we comply with relevant ethical requirements relating to audit engagements and plan and perform the audit to obtain reasonable assurance whether the financial report is free from material misstatement.

An audit involves performing procedures to obtain audit evidence about the amounts and disclosures in the financial report. The procedures selected depend on the auditor's judgement, including the assessment of the risks of material misstatement of the financial report, whether due to fraud or error. In making those risks assessments, the auditor considers internal control relevant to the association's preparation of the financial report that gives a true and fair view, in order to design audit procedures that are appropriate in the circumstances but not for the purpose of expressing the appropriateness of accounting policies used and the reasonableness of accounting estimates made by the committee, as well as evaluating the overall presentation of the financial report.

We believe the audit evidence we have obtained is sufficient and appropriate to provide a basis for our audit opinion.



CHARTERED ACCOUNTANTS
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Independence

In conducting our audit, we have complied with the independence requirements of the Australian professional ethical pronouncements. As auditor of HSP Research Foundation Incorporated for the financial year ended 30 June 2018, we declare that, to the best of our knowledge and belief, there have been no contraventions of the code of conduct relating to independence in APES 110 Code of Ethics for Professional Accountants issued by the Accounting Professional and Ethical Standards Board.

Audit Opinion

In our opinion the financial report of the association has been prepared in accordance with the Association Incorporation Act NSW including:

- a) Giving a true and fair view of the entity's financial position as at 30 June 2018 and of its performance for the year ended on that date; and
- b) Complying with Australian Accounting Standards as referred to in Note 1 to the financial statements.

Basis of accounting and restriction on distribution

Without modifying our opinion, we draw your attention to Note 1 to the financial report, which describes the basis of accounting. The financial report has been prepared to assist HSP Research Foundation to meet its statutory requirements under the Associations Incorporation Act NSW. As a result the report is not suitable for another purpose and as such may not be relied upon.



Peter Lever CA

27 September 2018

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