

IMPROVING THE QUALITY OF LIFE FOR PATIENTS SUFFERING FROM HEREDITARY ANGIOEDEMA

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*The Major Improvements Necessary in Australia To Meet
World Allergy Organization's (WAO) Recommendations.*



HAE Australasia Ltd
Pre-Budget Submission for the
Australian Federal Budget 2015/2016

Prepared for the
Australian Federal Government
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EXECUTIVE SUMMARY

Hereditary Angioedema (HAE) is a rare, life-threatening, genetic medical condition which causes debilitating swellings around the body, including the airway which is life-threatening. Symptoms are often incorrectly treated as allergies or abdominal complaints which result in inappropriate treatments or unnecessary medical investigations. Where patients have an undiagnosed family history, they, on average, suffer symptoms for up to two decades before being correctly diagnosed - in the meantime suffering horrific symptoms that incorrect treatments don't resolve and wondering when the symptoms may appear next.

In recent years, there have been significant improvements in access to life-saving medication for HAE patients in Australia, however we are still far behind Europe and the USA in access to all options for optimal management. Long-term prophylactic therapy is one area where we lag behind world best practice and this creates further potential problems for patients with HAE.

Our organisation, HAE Australasia (a not for profit, health promotion charity), have reviewed Australia's performance against the World Allergy Organization (WAO) set of 20 recommendations for the management of HAE. The result of our analysis demonstrates the need for MAJOR IMPROVEMENTS.

HAE patients have been stoic for generations and just put up with debilitating swellings. However, with treatments accessible to patients overseas, ready access to information and the advances in other research and medical technologies, Australian HAE patients cannot be left behind any longer.

We are requesting that the Australian Federal Government provide our organisation, approximately **\$42,300 over a 5 year period** to partly cover the costs of projects that will provide an improved performance against the WAO recommendations. The funding requested does not take into consideration the public funding required to subsidise treatments.

This document outlines 2 recommendations of how these issues can be addressed (see summary below).

Together, HAE Australasia and the Australian Federal Government can help Australia achieve WAO standards, and drive the state of management of HAE in Australia towards world's best practice.

Recommendation 1 Support Patient, Carer, Health Care Provider (HCP) & Community Education about HAE

This will improve time to diagnosis as well as allowing patients to be in a better position to manage their condition, lead a healthy lifestyle, and participate in the community.

HAE Australasia Project	Project Details	Relevant Current Government Policy	Total Cost (\$)	Other sources of Funding (\$)	Australian Federal Government Contribution (\$)	Funding Required By:
HAE Australasia Annual Patient Meeting	Comprehensive annual conference for patients and carers. Strengthens the HAE support network. Updates attendees on current HAE research, treatment availabilities and projects underway to help improve the quality of life for HAE patients. Also discusses special topics (i.e. mental health, nutrition etc).	Department of Health Goals - Outcome 1 - Population Health	\$25,000 p.a	\$20,000 p.a	<i>(\$5,000 p.a for 5 years)</i> \$25,000	Qtr. 3 per year (commencing Qtr. 3, 2015/2016)
HAE Australasia Website Hosting	Thorough and informative website dedicated to HAE. Regularly updated with most recent news, research and events about HAE.		\$460 p.a.		<i>(\$460 p.a. for 5 years)</i> \$2,300	Qtr. 1 per year (commencing Qtr. 1, 2015/2016)

Recommendation 2 Provide Financial Support for a HAE Patient Registry

This will provide researchers with an effortless way of accessing appropriate HAE patient data. This is currently a very difficult task which inhibits HAE research from occurring.

HAE Australasia Project	Project Details	Relevant Current Government Policy	Total Cost (\$)	Other sources of Funding (\$)	Australian Federal Government Contribution (\$)	Funding Required By:
HAE Australasia Patient Registry - Development	A HAE specific Patient Registry to house HAE patient personal medical data that, where appropriate, can be made available for HAE research.	Department of Health Goal: - Outcome 2 - Access to Pharmaceutical Services	\$20,000 - \$50,000	Balance of costs	\$10,000	Qtr. 1, 2015/2016
HAE Australasia Patient Registry - Ongoing Maintenance	Regular upkeep and maintenance of the database to ensure information is complete and readily available for HAE research.	Medical Research Future Fund (MRFF)	\$1,000 - \$5,000 p.a	Balance of Costs	<i>(\$1,000 p.a for 5 years)</i> \$5,000	Qtr. 3 per year (commencing Qtr. 3, 2015/2016)

TOTAL CONTRIBUTION REQUESTED FROM THE AUSTRALIAN FEDERAL GOVERNMENT \$42,300

TABLE OF CONTENTS

Executive Summary	2
Abbreviations	5
What is Hereditary Angioedema?	6
HAE swelling attacks are usually spontaneous	7
Having HAE can severely affect a person’s way of life (2)	7
HAE Australasia – Our short history	9
HAE Australasia’s Achievements So Far	9
Recommendation 1 – Support Patient, Carer, HCP & Community Education about HAE.....	13
Project - Annual Patient Meeting	14
Project - Website Hosting	15
Where funds will be sourced after the end of the proposed Government support:	16
Current Government Policies Affected By This Recommendation	16
Recommendation 2 – Provide Long Term Financial Support for a HAE Patient Registry	17
Project - Patient Registry - Development & Implementation.....	18
Project - Patient Registry - Ongoing Maintenance	19
Where funds will be sourced after the end of the proposed Government support:	20
Current Government Policies Affected By This Recommendation	20
Team of HAE Australasia - We Make Things Happen!	22
Conclusion.....	24
Appendix 1: HAE Australasia’s Mission, Vision, Objectives.....	25
Appendix 2: How Management of HAE in Australia Compares to WAO Recommendations	26
Part 1: Australia's Achievement Level Towards the 11 WAO's HAE Management Recommendations Concerning Time To Diagnosis & Treatment Options	26
Part 2: Australia's Achievement Level Towards the 3 WAO's HAE Management Recommendations Concerning Paediatric Patients, Pregnancy & Breastfeeding	30
Part 3: Australia's Achievement Level Towards the 6 WAO's HAE Management Recommendations Concerning Patient Support & Management Considerations	32
Appendix 3: HAE Australasia’s Projects That Can Help To Meet The WAO Recommendations:	34
Appendix 4: Sample of responses from Research on the Australian HAE Treatment Landscape (2). 39	
Appendix 5: Testimonials.....	41
References	48

ABBREVIATIONS

ASCIA	Australasian Society of Clinical Immunology and Allergy
HAE	Hereditary Angioedema
HCP	Health Care Provider
LSDP	Life-Saving Drug Program
MRFF	Medical Research Future Fund
PID	Primary Immunodeficiency
WAO	World Allergy Organization

WHAT IS HEREDITARY ANGIOEDEMA?

Hereditary Angioedema (HAE) causes spontaneous and recurrent episodes of swelling of various parts of the body – the most serious being swelling in the airway, which, if not treated quickly or appropriately, the patient may die due to asphyxiation.

Studies have revealed that approximately 50 – 75% of HAE patients suffer at least one life threatening attack at some point in their life (1). Some patients suffer these airway attacks on a more frequent basis which leads to a serious fear of what the future may hold for them and if treatment will be available in time to save their life.

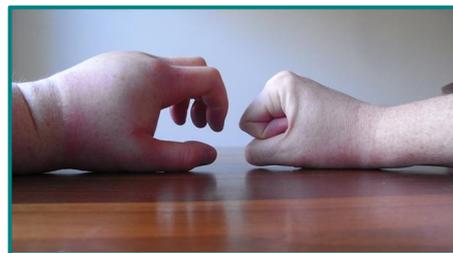
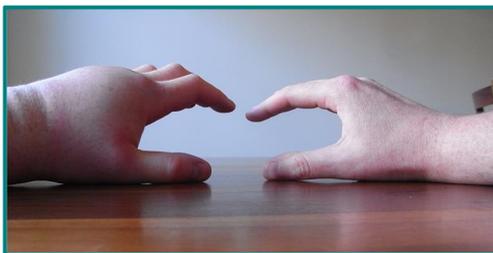
HAE attacks can be recurrent and spontaneous – there may or may not be a trigger for a swelling to occur. Even though a swelling of the airway is the most life-threatening HAE attack, swellings around other parts of the body can severely impact a HAE patient's life:

A mother suffering from an abdominal attack is in so much pain, she is bed-ridden and unable to care for her children.

A bride with facial swelling will forever be reminded in wedding photos that one of the most important days of her life was affected by her medical condition.

A dad can't play footy with his kids on the weekend because it feels as though the swelling in his foot could burst with the impact of kicking a football.

A tradesperson, office employee or medical practitioner suffering swelling of their hands won't be able to perform their pivotal role at work.



HAE is a rare condition – it may only affect approximately 480 people in Australia (1). However, these 480 people still deserve the same quality of life as those who were genetically given good health.

HAE swelling attacks are usually spontaneous

The cause of HAE is due to a deficiency in the blood protein C1 Inhibitor. Even though the cause is known, what triggers a swelling attack varies from patient to patient and also, within a patient's own experience. What may have caused an attack at one time may not the next time. Often there is no apparent trigger.

The symptoms are often mistaken for allergies or abdominal complaints and are therefore, in the first instance, treated with inappropriate medical investigations or medications that either amplify the symptoms or are not effective and the patient continues to suffer. Patients MUST receive specific HAE treatments to reduce or prevent their symptoms.

Having HAE can severely affect a person's way of life (2)

Where there is a family history of the condition, patients can be diagnosed early in life and can work with their doctor to put a medical action plan in place. It is often the case that the family doctor's only HAE experience is from other family members and may not be up to date with HAE best practice.

For the many where the condition hasn't been diagnosed, the patient may suffer decades of unexplained and debilitating swelling episodes that were either not treated correctly or resulted in unnecessary investigative procedures.

Delay in Diagnosis

On average, it takes 13 to 21 years (1) from the time a patient first experiences symptoms to the time a diagnosis is made. Even when a diagnosis is made, patients often encounter Health Care Providers (HCP's) that have limited or no knowledge of the condition and therefore do not treat the patients with HAE specific medications. Significant improvements are required in this area to ensure that patients are not suffering or enduring unnecessary medical investigations which may only worsen their symptoms.

"Doctors being unable to provide much support due to lack of awareness of HAE or expertise"

"[Patients have had] distressing experience at A & E. ... Lengthy delays, misdiagnosis, wrong treatment, anxiety"

Living with the threat of life-threatening or major attacks

Once a diagnosis is made, patients live with the threat that a life-threatening or major HAE attack is about to occur. This results in severe anxieties and other mental health issues as well as a low participation in activities. Everyday occurrences that the general population take for granted can be an issue for HAE patients.

"[There is a] negative impact on mental state Causing feelings of anxiety, depression, mood swings, inability to cope, relationship issues"

"Inability to plan in life; due to the degree of uncertainty and the unknown about attacks ... not joining clubs or classes, not planning longer term for activities or events, not taking up training or education"

"Periods of childhood or adolescence severely impact[ed] teasing, missing school, not being able to join in teams, not attending social events, minimising suffering and bravado. Detrimental impact on education"

"Restriction on [the] ability to travel to regional areas or overseas"

"Career limitation Not being able to work, taking a lesser career path"

"A curtailment of social life and an inability to participate fully in outside interest[s] Not being able to form friendships or attend functions"

HAE AUSTRALASIA – OUR SHORT HISTORY

Up until recently, the situation for Australian HAE patients was critical. However, in 2011, a group of 5 volunteers formed HAE Australia Ltd. Our vision was to enhance the quality of life for those living with HAE in Australia.

By 2013, we saw that the need of the 90 estimated New Zealand HAE patients was as urgent, if not more so, than Australia's and decided to include New Zealand in our activities. This led to a change of our organisations name – we are now known as HAE Australasia Ltd.



Our Mission:

To help Australian and New Zealand patients gain subsidised access to more treatment options, empowering patients through education about their condition, educating the medical and general community about HAE to ensure patients are recognised and diagnosed more quickly, and to connect patients and carers with our support network – they need to know that they are not alone.

HAE Australasia's Achievements So Far

Prior to HAE Australasia, there was NO representative body in existence in Australia and New Zealand for HAE. HAE patients had almost no contact with anyone else with the condition and the information that they had may have been provided by a HCP with little to no knowledge themselves of HAE. It was a very dire situation and patients were not receiving adequate care.

HAE Australasia is governed by a board of 5 women who are either HAE patients or carers. We all volunteer our time outside of work and family commitments and are very passionate about making a difference. This is evidenced by the phenomenal achievements that we have made in the short time since our organisation was formed.

Established an Australian & New Zealand support network of HAE patients & carers.

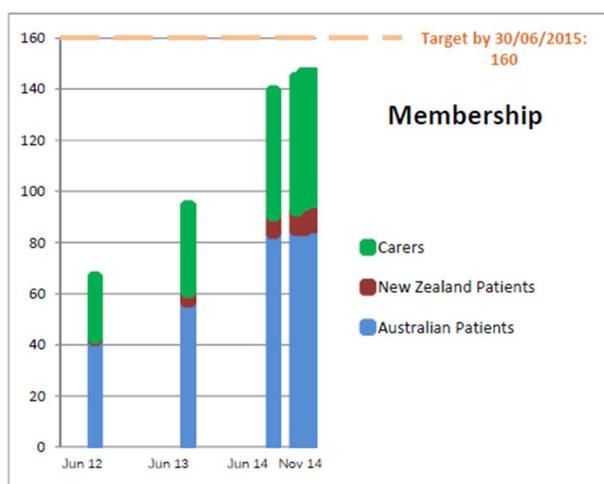


FIGURE 1: HAE AUSTRALASIA'S MEMBERSHIP SINCE AUG 2011

"I don't feel so alone or isolated knowing there is an organisation and people in that organisation that understands and has knowledge about this awful disease that I live with every day."

"We support each other, because we know what it is like to have HAE."

HAE Australasia Website - www.haeaustralasia.org.au

"When I last saw my specialist for my check up, he used the HAE Australasia website to get resources that I needed."

Developed a website with the aim of it being the go-to place for all Australian and New Zealand HAE patients, carers, health care providers and supporters.

Assisted new treatments becoming subsidised.

We were actively involved in making a new personal use, subcutaneous treatment (Icatibant) becoming subsidised for patients in Australia – this in itself was a major turning point for HAE patients – they felt that they could actually live a life!

"I can now afford to medicate myself and therefore have greatly enhanced my life. I no longer need to attend the emergency department of my local hospital, for treatment that lasts between a few hours to a couple of days, depending on the severity of the attack. I have done this at least once sometimes twice a month over the last 30 years or so. Which would of been of great cost to The Health System and the community."

"I have been involved in the introduction of new innovative therapies for HAE in Australia and negotiation with authorities can be difficult. Participation of an organized patient group facilitates such negotiation and can clarify the importance of new therapies to those with a poor understanding of the condition"

Held Annual Patient Meetings & HAE Meet Ups

Patient Meetings (annual conferences) educate patients and their carers about HAE, current research and new treatment availabilities. “HAE Meet Ups” strengthen the local HAE support networks.

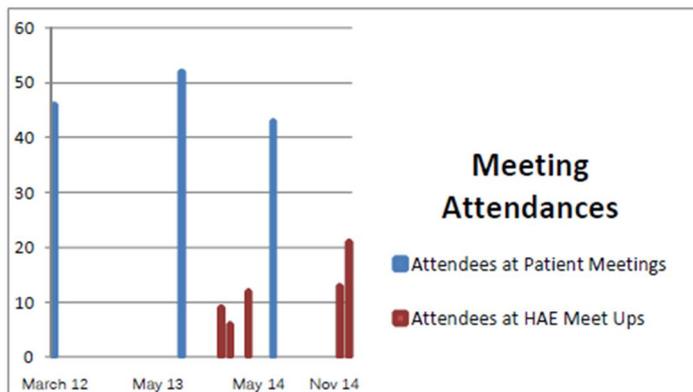


FIGURE 2: NUMBER OF ATTENDEES AT HAE AUSTRALASIA PATIENT MEETINGS & HAE MEET UPS

Patient Meetings	Budget	Actual Spend*
2012 - Melbourne	\$9,600	\$8,407
2013 - Brisbane	\$14,360	\$13,490
2014 - Adelaide	\$26,200	\$14,270
2015 - Sydney	\$25,000	

TABLE 1: BUDGET VS ACTUAL SPEND OF PATIENT MEETINGS. (* HAE AUSTRALASIA WAS SUCCESSFUL IN APPLYING FOR OTHER GRANTS TO HELP FUND THESE PROJECTS)

“Having HAE Australasia organising Patient Meet Ups has been invaluable for my self-esteem and feeling connected.”

HAE Meet Up’s*	Budget	Actual Spend
2013 - Perth	\$600	\$555
2013 - Central Coast, NSW	\$200	\$131
2013 - Brisbane	\$1,000	\$927
2014 - New Zealand	\$2,260	\$2,043
2014 - Melbourne	\$1,900	\$1,580
2015 - Hobart	\$1,200	
2015 - Perth	\$2,200	
2015 - Canberra	\$1,100	

TABLE 2: BUDGET VS ACTUAL SPEND OF HAE MEET UPS (* - HAE AUSTRALASIA HAS SECURED GRANTS TO HELP FUND THESE PROJECTS)

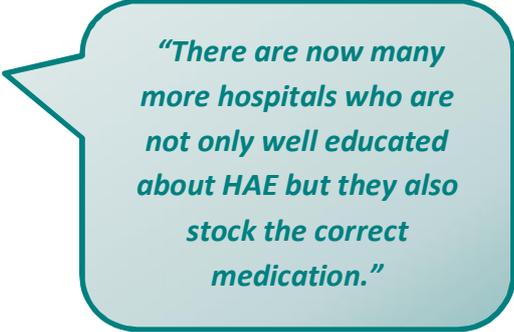
“Having been educated at HAE conferences and Forums I have attended over the years, for me knowledge is power and power is strength. Strength to carry on and live a happy, well and valuable life with this insidious disease.”

“Coming into contact with the HAE Australasia team has been life changing to say the least. They have given me the knowledge and the confidence to seek out the best medical treatment available”

“Whilst attending a HAE Australasia Patient meeting, I found out that the father of a toddler with HAE works in my building! What are the chances with so few HAE patients in Australia! It’s good to know others are close by that I can talk to”

Increased awareness in the medical community

There has been a significant increase in the number of doctors that now know about HAE due to our activities. This helps to identify potentially undiagnosed patients, or allows patients diagnosed with HAE be able to access appropriate treatments quicker.



“There are now many more hospitals who are not only well educated about HAE but they also stock the correct medication.”

Assisted with HAE Research

HAE Australasia has connected research organisations with patients to conduct desperately needed HAE research and clinical trials. The quality of life and other issues surrounding HAE was discussed by patients in a recent research paper that our organisation helped locate participants for. Various responses to this research can be found in Appendix 4: Sample of responses from Research on the Australian HAE Treatment Landscape.

More still needs to be done.....

Despite these successes, there is still a long way to go.

With a relatively modest investment of funds from the Australian Federal Government, we at HAE Australasia believe we have the skills and networks to achieve significant improvements over the present situation. Funding the recommended projects will help assist our organisation to enable patients to have access to world’s best management and the opportunity to participate in various research endeavours, an activity that is highly valued among those with a hereditary condition. Future generations of HAE patients will be in a far better position than those that have endured this condition so far.

RECOMMENDATION 1 – SUPPORT PATIENT, CARER, HCP & COMMUNITY

EDUCATION ABOUT HAE

As HAE is a genetic disorder involving the deficiency of a blood protein, patients require specific treatments that either replace the deficiency or act to fix the system.

To ensure that patients receive appropriate treatment, we need to ensure that patients themselves, along with their carers, are educated about the condition, possible triggers and treatment options available - this will help them to have a voice and be their own advocate when they come across HCP's that have limited knowledge of HAE.

We also need to ensure that HCP's and the general community are educated about HAE. This will help to identify patients that are currently undiagnosed which will lead to testing for the condition and an action plan put in place so the patient can minimise the symptoms that may be endured.

Our organisation has a number of projects running concurrently in order to increase the awareness and education about the condition.

However, for HAE Australasia to assist the Australian Federal Government move towards world class standards of health care for patients with HAE, it would be greatly appreciated if the projects following could be funded.

Project - Annual Patient Meeting

Details of Project:

Comprehensive annual conference for HAE patient and carers. Provides attendees with an update on current HAE research, treatment availabilities and projects underway to help improve the quality of life for HAE patients. Special topics are also discussed, including mental health, nutrition etc. The meetings also help to strengthen the HAE support network.

Purpose of Funding:

To enable HAE Australasia to provide a quality program for this key Patient/Carer educational activity each year. Funding will also help to ensure that as many patients can attend to receive the invaluable information.

Budget for Project:

Estimated total expenses	<u>\$25,000 p.a</u>
Amount secured from other sources by HAE Australasia	\$20,000 p.a
Amount requested from the Australian Federal Government	\$5,000 p.a

We are requesting that the Australian Federal Government provides us with \$5,000 p.a for 5 years, therefore the total requested amount for this project equates to \$25,000.

Funding Required By:

Quarter 3 of each year (commencing Quarter 3, 2015/2016)

Government Legislation Changes Required?

No - This project run by HAE Australasia's simply needs to be considered as part of Government budgets

Measuring The Project's Success:

Factors that will help us determine how successful the Annual Patient Meeting is include (but not limited to):

- Number of patients/carers in attendance (including what state/country they are traveling from)
- Number of returning patients/carers
- Feedback provided in surveys
- Number of new patient/carers that become members of HAE Australasia as a result of a referral from an attendee of the Patient Meeting.

Project - Website Hosting

Details of Project:

www.haeaustroalasia.org.au. Thorough and informative website dedicated to HAE. Regularly updated with most recent news, research and events about HAE.

Purpose of Funding:

To ensure that the website remains active so that up to date HAE information can be provided to patients, carers, and supporters as well as other interested parties.

Budget for Project:

\$460 p.a

Amount requested from the Australian Federal Government

\$460 p.a

We are requesting that the Australian Federal Government provides us with \$460 p.a for 5 years, therefore the total requested amount for this project is \$2,300.

Funding Required By:

Quarter 1 each year (commencing Quarter 1, 2015/2016)

Government Legislation Changes Required?

No - This project run by HAE Australasia simply needs to be considered as part of Government budgets

Measuring The Project's Success:

We have resources in place to determine where from and how often the website is accessed - including if the website is accessed after a newsletter issue, other social media updates or other events. KPI's are in place to measure and analyse the website activity.

HAE Australasia is strongly focussed on monitoring progress and improving processes wherever possible. The organisation regularly assesses project outcomes, effectiveness and efficiencies – these projects would simply be included in the monitoring process.

Where funds will be sourced after the end of the proposed Government support:

Building secure sources of funding for HAE Australasia is currently underway. This includes strengthening relationships with grant providers and regular donors. It is anticipated that, by the end of the period of requested Government support, our organisation would be in a position to fully finance these projects from other funding sources.

Current Government Policies Affected By This Recommendation

Department of Health Goals - Outcome 1 - Population health

Due to the uncertainty of HAE, patients often lead a very sedentary and unhealthy lifestyle and may be unable to participate in the work force due to the unpredictable nature of their condition.

This sedentary lifestyle increases the patient's risk of other avoidable illnesses and chronic diseases that are listed as "National Health Priority Areas" including:

- Mental Health
 - o HAE patients are known to suffer anxieties and depression due to the affect that their condition has on their life
- Obesity
 - o Reducing physical activity to avoid triggers of HAE attacks increases the likelihood of becoming obese. Obesity may then also lead onto the following illnesses.
- Diabetes
- Cardiovascular Health Issues
- Cancer

By educating patients, carers and HCP's about the condition, treatments required, ways that patients can cope with the emotional stress of having a life-threatening condition, as well as providing comprehensive education about triggers of HAE attacks, patients will have the tools and support to lead a more active and healthy lifestyle.

These positive effects would decrease the incidence of the other avoidable illnesses and chronic diseases listed. HAE patients could also increase their participation in the community and workforce as their symptoms may be more stable. This would lead to an increase in economic activity.

RECOMMENDATION 2 – PROVIDE LONG TERM FINANCIAL SUPPORT FOR A HAE PATIENT REGISTRY

Prior to the inception of HAE Australasia, the handful of medical professionals and researchers that wanted to conduct HAE investigations in Australia had a limited patient base to contact. They may have only had contact with HAE patients known to them, or accessed patients via a Primary Immunodeficiency (PID) register. This database had 66 HAE patients, out of the potential 480 HAE patients in Australia (1) .

HAE Australasia has identified a need to develop, implement and maintain a Patient Registry that will store patient data/experiences. This information can be provided to researchers to enable their finite resources to be used more effectively.

By assisting researchers in this way, new or improved treatment options may be found, or trends in patient experiences may definitively identify triggers. Results of such research can only improve the quality of life for HAE patients in Australia and around the world.

If the Australian Federal Government would like HAE Australasia to help Australia achieve world class standards of health care and research for patients with HAE, it would be greatly appreciated if the projects following could be funded.

Project - Patient Registry - Development & Implementation

Details of Project:

A HAE specific Patient Registry to house HAE patient personal medical data that, where appropriate, can be made available for HAE research.

Purpose of Funding:

To ensure that the HAE Australasia Patient Registry can be developed to the highest standards. Knowledge gained from the development of this registry can be shared with other similar organisations to help them achieve the high standards expected of this project.

Budget for Project:

Estimated total expenses	<u>\$20,000 - \$50,000</u>
Amount requested from the Australian Federal Government	\$10,000
Amount that will be secured from other sources by HAE Australasia	Balance of Costs

Funding Required By:

Quarter 1, 2015/2016

Government Legislation Changes Required?

No - HAE Australasia's activities simply need to be considered as part of budgets

Measuring The Project's Success:

Factors that will help us determine how successful this project is include (but not limited to):

- Ease of use for patient data entry (including time taken and clarity of questions/answers)
- Accuracy rate of data input
- Ease of use for reporting details at varying levels of authority (including time taken and clarity of questions/answers)
- Relevance, reliability and accuracy rate of filtered/reported information
- User feedback on data input process, data update process, data extraction process
- Uptake by current patients and carers on our database
- Continued use of the registry by patients and carers
- Uptake by researchers to use the resource
- Number of researchers that utilise the registry for more than one project
- Awareness of patients, carers, HCP's and researchers of Patient Registry's existence.
- quantity of different reports available

Project - Patient Registry - Ongoing Maintenance

Details of Project:

Regular upkeep and maintenance of the database to ensure information is complete and readily available for HAE research.

Purpose of Funding:

To ensure that Patient Registry can be maintained in the initial years whilst momentum is gained for future support.

Budget for Project:

Estimated total expenses	<u>\$1,000 - \$5,000 p.a</u>
Amount requested from the Australian Federal Government	\$1,000
Amount that will be secured from other sources by HAE Australasia	Balance of Costs

Funding Required By:

Quarter 3 of each year (commencing Quarter 3, 2015/2016)

Government Legislation Changes Required?

No - HAE Australasia's activities simply need to be considered as part of budgets

Measuring The Project's Success:

Factors that will help us determine how successful the ongoing existence of the Patient Registry is include (but not limited to):

- Ease of use for patient data entry (including time taken and clarity of questions/answers)
- Accuracy rate of data input
- Ease of use for reporting details at varying levels of authority (including time taken and clarity of questions/answers)
- Relevance, reliability and accuracy rate of filtered/reported information
- User feedback on data input process, data update process, data extraction process
- Uptake by current patients and carers on our database
- Continued use of the registry by patients and carers
- Uptake & repeat use by researchers of the resource
- Awareness of patients, carers, HCP's and researchers of Patient Registry's existence.

HAE Australasia is strongly focussed on monitoring progress and improving processes wherever possible. The organisation regularly assesses project outcomes, effectiveness and efficiencies – these projects would simply be included in the monitoring process.

Where funds will be sourced after the end of the proposed Government support:

As discussed earlier, building secure sources of funding for HAE Australasia is currently underway. This includes strengthening relationships with grant providers and regular donors. It is anticipated that, by the end of the period of requested Government support, our organisation would be in a position to fully finance these projects from other funding sources.

Current Government Policies Affected By This Recommendation

Medical Research Future Fund

The preliminary/planning stage of the HAE Australasia Patient Registry is well underway and it is expected that the Patient Registry will be developed and implemented by December 2015.

We are proposing that, as part of the Medical Research Future Fund, the Australian Government allows support for the development/implementation and ongoing maintenance of the Patient Registry. This support will help promote critical research required into HAE.

The Patient Registry will be developed after consultation with HAE experts, the Australasian Society of Clinical Immunology and Allergy (ASCI), various universities and other research bodies, rare disease and genetic support organisations, other organisations that have successfully implemented Patient Registries and legal practitioners.

These discussions will help to ensure that the HAE Australasia Patient Registry is a world class resource that is compliant with all relevant privacy, ethical and medical codes of conduct/legislation.

HAE Australasia is in support of the Australian Federal Government's investment into medical research. However, as discussed in the McKell Institute's paper (3), even if research leads to new treatment options, it is of no use if the treatments aren't financially accessible to patients.

"[HAE] research will be greatly facilitated by HAE Australasia in terms of reaching people who might be interested in participating in research, in particular if a register were established this would be extremely effective for that purpose".

"The HAE community is diverse and dispersed and individuals can be hard to reach but a register would facilitate contact and communication."

"One can only imagine the progress this energetic and capable group could make in key areas such as patient identification, education, diagnosis, and research if they had access to more funding sources."

Department of Health Goal - Outcome 2 - Access to Pharmaceutical Services - Life Saving Drug Program

A HAE Patient Registry will enable researchers to have timely access to up to date and reliable HAE patient data. By taking away the lengthy process of trying to connect with HAE patients, researchers will be provided with more time, and better use of their resources, to further our knowledge of the condition. This will help in determining and developing new treatment options for patients.

As treatment options are limited at the moment, patients are having to endure some serious side effects in order to reduce their HAE symptoms. Having a broader range of treatment options will allow patients to find a treatment that best suits them and doesn't lead to further issues (that may increase dependency on the health system for other reason and not HAE)

The McKell Institute (3) has made some recommendations in regards to funding rare disease therapies and the Life-Saving Drug Program (LSDP). HAE Australasia strongly encourages these recommendations to be considered and implemented by the government. By implementing the recommendation, once new treatments have been developed and are available for use, the process of making them financially available to patients will become streamlined and time-efficient - allowing current and future generations of HAE and other rare disease sufferers to enjoy an improved quality of life sooner.

"When I was 24, I had to have half of my liver removed due to the side effects of the only long-term medication available that actually helped my HAE. I was in hospital for a month and then off work for another two months. Then, when I went back to work, I would be sick a few days every fortnight because we couldn't find another treatment that worked."

"I'm worried that when I get pregnant, I'll be so sick that I will be house bound and bed-ridden for the whole 9 months because I can't use any of the few medications that are currently available to me".

"I remember whenever my grandfather would have an airway attack, he would put a garden hose down his throat to make sure he could keep breathing - during those times, we didn't know where to go to get treatment or what the treatment was"

"I don't make plans with friends or family, just in case I'm sick again and I end up disappointing them - again"

"I don't travel anywhere that is more than an hour from a hospital that I KNOW has C1 Inhibitor - just in case I need it"

TEAM OF HAE AUSTRALASIA - WE MAKE THINGS HAPPEN!

Our team of 5 directors tirelessly volunteer time outside of family, work and other commitments. They co-ordinate all projects of HAE Australasia. Due to their own previous experience and enthusiasm to improve HAE patients' quality of life, they are all committed to adhering to best practice standards. We are very proud of the strong team that we have representing HAE in Australia and New Zealand.

President - Fiona Wardman (Director)

Qualifications / Experience: Area services coordinator
Over 15 years' experience in Office & Project Management

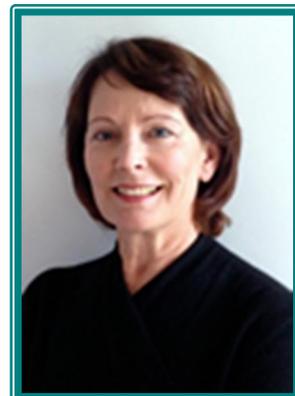
Fiona's HAE diagnosis came in her 30's after years of unexplained swellings from the age of 9 which progressively worsened as she got older. No one else in Fiona's family has HAE. Fiona is proud to be part of an organisation that is making a difference to patients with HAE in Australia and New Zealand.



Vice President - Anne Wilkinson (Director)

Qualifications / Experience: Qualified Pharmacist
Over 30 years' experience in the field

Anne is married, has four adult children and lives in Melbourne. Anne was not diagnosed as having hereditary angioedema until her late forties. Her diagnosis led to a diagnosis of family members.



Treasurer - Louise Ridout (Director)

Qualifications / Experience: CPA Qualified Accountant & Registered Financial Planner
Over 10 years in public practice

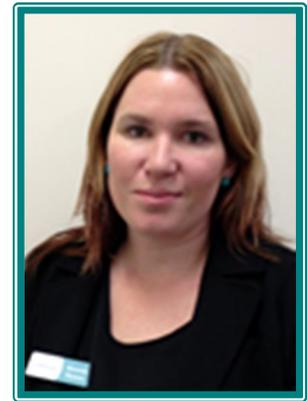
Louise lives in Melbourne with her partner and young daughter. Louise was diagnosed with HAE when she was a baby as HAE was known in her family. She is passionate about raising awareness of HAE and keen to see better treatment options available for patients.



Secretary - Amanda Tionisio (Director)

Qualifications / Experience: Office Manager for over 14 years

Amanda lives in Western Australia with her husband and 4 young children. She has a long family history of HAE and is particularly passionate about assisting children with HAE as she has two daughters with the condition.



NZ Representative - Olivia Willard (Director)

Qualifications / Experience: Part of the Digital Marketing & Communications Industry
Over 10 years' experience

Olivia is based in Auckland, New Zealand and represents the interests of our New Zealand patients. She is passionate about New Zealand patients receiving and having access to the same treatment and care as patients in Australia and the rest of the world.



Medical Advisor - Professor Connie Katelaris

Dr Katelaris is the Medical Advisor to HAE Australasia. She is Professor, Immunology & Allergy, University of Western Sydney, Head of Department and Senior Staff Specialist at Campbelltown Hospital. She is convenor of the Graduate Certificate in Allergic Diseases, the first postgraduate course in the Faculty of Medicine, University of Western Sydney. She is currently President, Asian Pacific Association of Allergology, Asthma and Clinical Immunology and is a board member of the Asthma Foundation NSW. She is a past president of ASCIA.

CONCLUSION...

Hereditary Angioedema (HAE) is a rare, life-threatening, genetic medical condition which causes debilitating swellings around the body, including the airway which is life-threatening.

After analysing WAO recommendations for HAE management, our organisation found that Australia requires major improvements in its HAE management to achieve the recommendations and be considered world class in this area.

This document discusses 2 recommendations of how the Australian Federal Government can support HAE Australasia in improving the state of management of HAE in Australia.

Recommendation 1 Support Patient, Carer, HCP & Community Education about HAE

Recommendation 2 Provide Financial Support for a HAE Patient Registry

After reviewing our resources, and with the support from the Australian Federal Government, HAE Australasia can help make some serious movement towards achieving WAO recommendations. It is estimated that a provision of only \$42,300 over a 5 year period will have a huge impact on HAE management in Australia. This in addition to public funds required to subsidise necessary treatments (which, based on McKell Institute findings (3), is only a small fraction of the Australian Health Budget).

Together, HAE Australasia and the Australian Federal Government can help Australia achieve WAO standards, and drive the state of management of HAE in Australia towards world's best practice.

APPENDIX 1: HAE AUSTRALASIA'S MISSION, VISION, OBJECTIVES



HAE Australasia Ltd is a non-government, not for profit, health promotion charity that was established in 2011 as there was a need to increase the awareness of Hereditary Angioedema (HAE) in Australia and New Zealand.

Vision of HAE Australasia

HAE Australasia Ltd.'s vision is to enhance the quality of life for those living with Hereditary Angioedema (HAE) in Australia & New Zealand.

Mission Statement

To achieve this vision, HAE Australasia Ltd aims to help Australian patients gain access to more better treatment options, empowering patients through education about their condition, educating the medical and general community about HAE to ensure patients are recognised and diagnosed more quickly, and to connect patients and carers with our support network – they need to know that they are not alone.

Operational Objectives

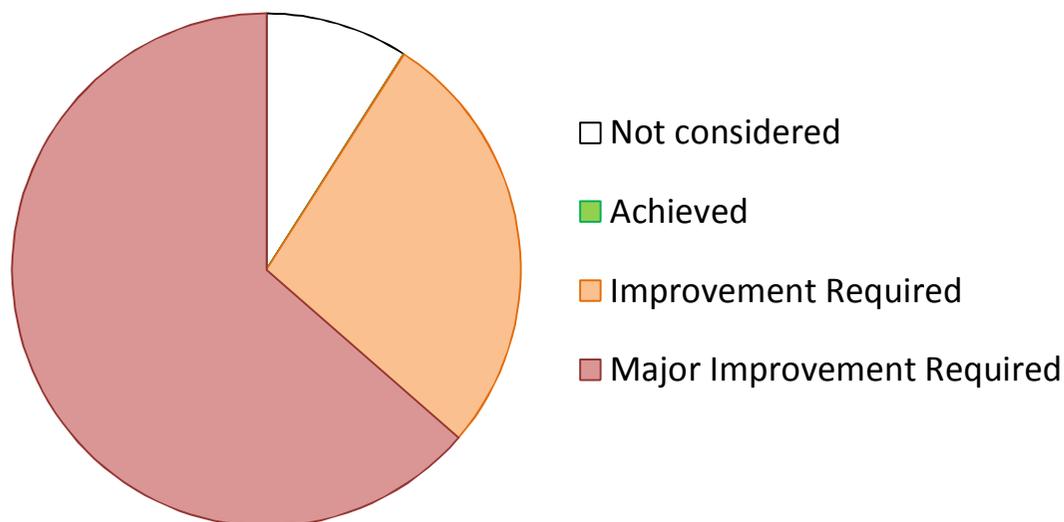
The objectives of HAE Australasia Ltd are to:

- Enhance the support and quality of life for people with HAE & their carers
- Provide a support group for people affected by HAE and allow patients and carers to communicate with each other to discuss their experiences. They need to know that they are not alone.
- Provide prompt and reliable responses in a professional manner to any HAE query
- Provide up to date and broad based education to:
 - o HAE patients to help them better understand their condition and treatment options available.
 - o HAE Carers to enable them to appropriately support individuals suffering from HAE
 - o Health care providers to enable them to appropriately recognise & support individuals suffering from HAE
- Engaging in activities to raise community awareness of HAE (to help undiagnosed patients access appropriate treatment and to help the community understand what it's like to live with the life-threatening and debilitating condition)
- Look at ways to make treatments and medications more accessible and subsidised for patients with HAE
- Assist research organisations with medical research into the causes, prevention and treatment of HAE
- Be considered by patients, carers, health care providers and the wider community to be a professional, reliable, courageous and compassionate organisation.

APPENDIX 2: HOW MANAGEMENT OF HAE IN AUSTRALIA COMPARES TO WAO RECOMMENDATIONS

HAE Australasia has analysed Australia's current performance against the WAO's recommendations (4) for the management of HAE. Comparative data was obtained from ASCIA (1) and reported experiences from HAE patients (2)

Part 1: Australia's Achievement Level Towards the 11 WAO's HAE Management Recommendations Concerning Time To Diagnosis & Treatment Options



What issues do these recommendations include?

- Testing patients with suspected HAE
- Symptoms requiring treatment
- Time to treatment is given
- Treatment options and supportive therapies
- Treatments to be avoided in specific circumstances
- Availability of treatments
- Monitoring of side effects

Delay to diagnosis

	World Allergy Organisation Recommendation	Australia's Current Situation	Australia's Achievement Level Achieved	HAE Australasia's Objectives Aimed At Reaching WAO Recommendations
1	All patients suspected to have HAE-1/2 (i.e. recurrent angioedema in the absence of a known cause) should be assessed for blood levels of C4, C1-INH protein, and C1-INH function, and these tests, if abnormally low, should be repeated to confirm the diagnosis.	Average delay to diagnosis of 13 to 21 years	 Major Improvement Required	Provide education to Health care providers.

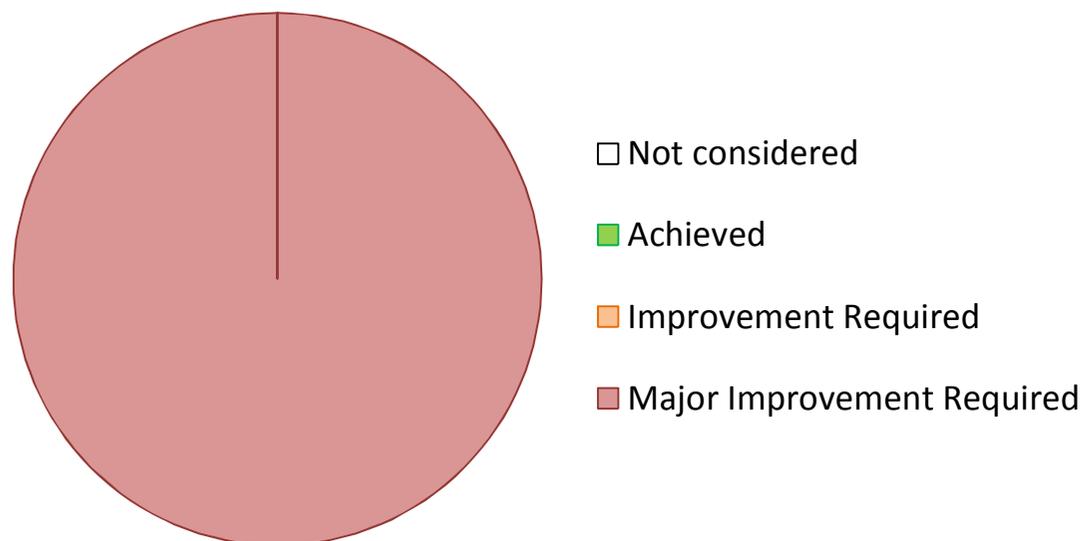
Treatment Options

	World Allergy Organisation Recommendation	Australia's Current Situation	Australia's Achievement Level Achieved	HAE Australasia's Objectives Aimed At Reaching WAO Recommendations
2	All attacks that result in debilitation/dysfunction and/or involve the face, the neck, or the abdomen should be considered for on-demand treatment. Treatment of attacks affecting the upper airway is mandatory.	These recommendation are detailed in the ASCIA Action Plan for HAE (5), however, not all patients have been prescribed the only subsidised, on-demand treatment (Icatibant). Also, if a patient were to present at an emergency department requiring treatment, they are sometimes faced with delays getting treatment. Some patients hesitate to treat themselves with available on demand treatment	 Major Improvement Required	Look at ways to make treatments and medications more accessible. Assist research organisations with medical research
3	We recommend that attacks are treated as early as possible.		 Major Improvement Required	Provide education to Health care providers. Provide education to HAE patients Provide education to HAE Carers

4	We recommend that HAE attacks are treated with C1-INH, Ecallantide, or Icatibant.	C1-INH is available in Australia, not subsidised and only approved for a limited number of uses. Icatibant can be prescribed to patients with PBS authority (6) however, even when prescribed Icatibant, there is a hesitation by patients to use it. Ecallantide is not available in Australia.	 Major Improvement Required	Look at ways to make treatments and medications more accessible.
5	We recommend that intubation or tracheotomy is considered early in progressive upper airway edema.	Tracheostomies are rarely required when appropriate treatment is given in a timely manner.		Assist research organisations with medical research Provide education to Health care providers.
6	We recommend that patients with attacks receive adjuvant therapy when indicated (pain management, intravenous fluids, and supportive care), but specific therapies should be used without delay when indicated.	The ASCIA action plan confirms this recommendation (5) however, some patients still have delays in receiving appropriate specific treatments	 Major Improvement Required	Provide education to HAE patients Provide education to HAE Carers
7	We recommend that oral antifibrinolytics are not to be used as on-demand treatment.	The only options in Australia for long term prophylaxis are Danazol (attenuated androgen) and Tranexamic acid (antifibrinolytics)	 Major Improvement Required	
8	We recommend that all patients should have on-demand treatment for 2 attacks.	C1 Inhibitor is available in very limited situations for at home use. Icatibant can be prescribed to patients with PBS authority (6) however, even when prescribed Icatibant, there is a hesitation by patients to use it.	 Improvement Required	Look at ways to make treatments and medications more accessible Provide education to Health care providers. Provide education to HAE patients Provide education to HAE Carers

9	We recommend that all patients should carry their on-demand treatment at all times.		 Improvement Required	Look at ways to make treatments and medications more accessible. Assist research organisations with medical research
10	The administration of short-term prophylaxis should be considered before surgeries, especially dental/intraoral surgery, where endotracheal intubation is required, where upper airway or pharynx is manipulated, and before bronchoscopy or endoscopy.	Short term prophylaxis is considered, however, it is common for HAE patients to have difficulty accessing C1 inhibitor for major surgeries/dental procedures due to hospitals & insurance funds debating whose budget the cost of the treatment should come from. Occasionally, HAE patients have had to cover the cost personally, or delay necessary treatment until the cost is covered by hospital/insurance company.	 Major Improvement Required	Provide education to Health care providers. Provide education to HAE patients Provide education to HAE Carers
11	Before the initiation of long-term prophylaxis with androgens, measurements of complete blood count, urine analysis, liver function tests, lipid profile, assessment of cardiac risk factors, and liver ultrasound should be performed. While using androgens for long-term prophylaxis and for 6 months after stopping therapy, complete blood count, urine analysis, lipid panel, liver function tests, and blood pressure should be monitored every 6 months and an ultrasound of the liver should be done yearly to asses for adverse events associated with androgens and contraindications to androgens.	Similar testing for side effects is recommended in Australia, however, it has been found that some patients have not experienced the regular follow up required and have suffered severe (& potentially life threatening) side effects as a result	 Improvement Required	Provide education to Health care providers. Provide education to HAE patients Provide education to HAE Carers

Part 2: Australia's Achievement Level Towards the 3 WAO's HAE Management Recommendations Concerning Paediatric Patients, Pregnancy & Breastfeeding



What issues do these recommendations include?

- Screening children of affected parents
- Treatment options for children
- Treatment options for pregnant and breastfeeding patients

Special Circumstances – Paediatric Patients

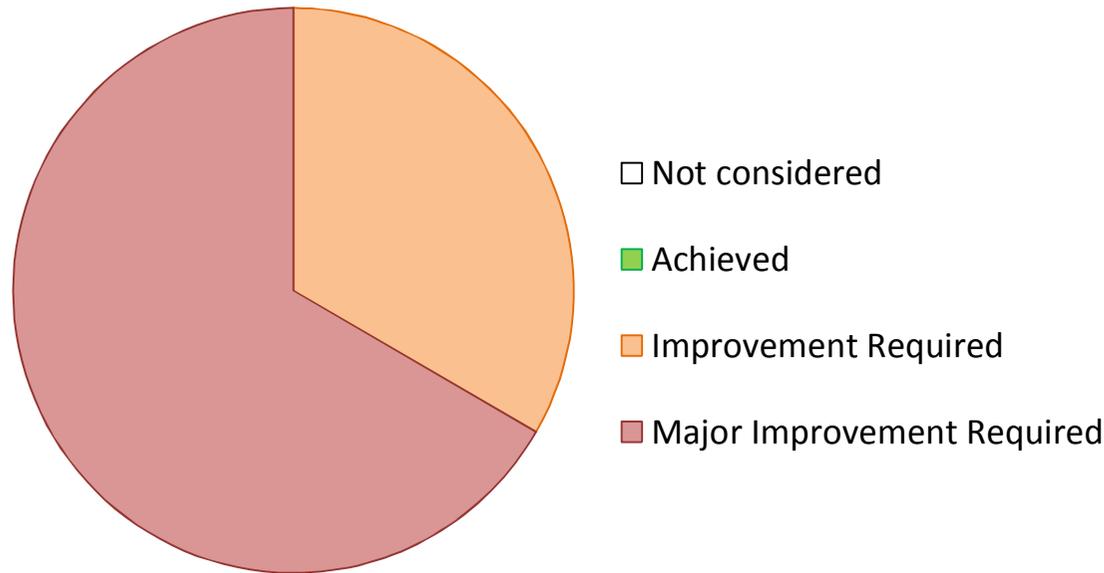
	World Allergy Organisation Recommendation	Australia's Current Situation	Australia's Achievement Level Achieved	HAE Australasia's Objectives Aimed At Reaching WAO Recommendations
12	Screening children for HAE-1/2 should be deferred until the age of 12 months, and all offspring of an affected parent should be tested.	From discussions with our patient base, some families have ensured all children of an affected parent are tested. Children of other families have not been tested as they may not show symptoms and the family will "deal with it if anything happens".	Major Improvement Required	<ul style="list-style-type: none"> Provide education to Health care providers. Provide education to HAE patients Provide education to HAE Carers

13	The preferred on-demand therapy for HAE-1/2 attacks in children is pdC1INH.	The ASCIA action plan confirms this recommendation (5), however, as C1 Inhibitor is not subsidised (6), it is difficult for families to access.	 Major Improvement Required	Look at ways to make treatments and medications more accessible. Assist research organisations with medical research Provide education to Health care providers.
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Special Circumstances – Pregnancy & Breastfeeding

	World Allergy Organisation Recommendation	Australia’s Current Situation	Australia’s Achievement Level Achieved	HAE Australasia’s Objectives Aimed At Reaching WAO Recommendations
14	During pregnancy and lactation, pdC1-INH is the preferred therapy.	The ASCIA action plan confirms this recommendation (5), however, as C1 Inhibitor is not subsidised (6), it is difficult for families to access. Also, some patients have experienced difficulties with hospitals/insurance providers debating who should cover the cost of the treatment required on hand at the time of delivery.	 Major Improvement Required	Look at ways to make treatments and medications more accessible. Provide education to Health care providers.

Part 3: Australia's Achievement Level Towards the 6 WAO's HAE Management Recommendations Concerning Patient Support & Management Considerations



What issues do these recommendations include?

- Patient action plans to be in place and have ready access to treatment
- Training patients in self-administration of on-demand treatment
- HAE identification cards
- Regular assessments of patients
- Screening family members
- Vaccinations

Patient Support and Management Considerations

	World Allergy Organisation Recommendation	Australia's Current Situation	Australia's Achievement Level Achieved	HAE Australasia's Objectives Aimed At Reaching WAO Recommendations
15	All patients with HAE should have an action plan and product available to treat an attack of HAE.	Not all patients have been provided an Action Plan from their specialist. C1-INH is available in Australia, not subsidised and only approved for a limited number of uses. Where appropriate, Icatibant can be prescribed to patients with PBS authority (6), however, even when prescribed there is a hesitation by patients to use it. Ecallantide is not available in Australia	 Major Improvement Required	Look at ways to make treatments and medications more accessible. Assist research organisations with medical research Provide education to Health care providers. Provide education to HAE patients Provide education to HAE Carers Raise community awareness of HAE

16	We recommend that all patients who are provided with on-demand treatment licenced for self-administration should be taught to self-administer.	It is recommended that patients prescribed Icatibant are trained for self-administration (6). C1 Inhibitor is TGA approved for self-administration (6), however, as this treatment is not subsidised, very few patients have access to the treatment for use for self-injection.	 Improvement Required	Look at ways to make treatments and medications more accessible. Provide education to Health care providers. Provide education to HAE patients Provide education to HAE Carers
17	We recommend that all patients should be provided with an HAE identification card.	MedicAlert bracelets are recommended for travelling patients.	 Major Improvement Required	Provide education to Health care providers. Provide education to HAE patients Provide education to HAE Carers
18	All patients with HAE should have at least 1 annual assessment by an HAE specialist.	Annual review for patients is recommended, including an annual review to determine the patients' competence to administer injections. However, there are some patients that don't have symptoms that do not follow this recommendation.	 Improvement Required	Provide education to HAE patients Provide education to HAE Carers
19	Family members of patients with HAE should be screened so that appropriate therapy can be available for treatment, especially because the first event may be of the upper airway and fatal without appropriate therapy.	From discussions with HAE Australasia's patient base, some families have ensured that all blood relatives of an affected patient are tested, and in some other families, individuals have not been tested as they may not show.	 Major Improvement Required	Provide education to Health care providers. Provide education to HAE patients Provide education to HAE Carers Provide a support group for people affected by HAE
20	Hepatitis A and B vaccination should be administered to HAE-1/2 patients receiving blood products, including pC1-INH. All patients should receive influenza vaccine.	Screening for Hepatitis and HIV is recommended, but there is no specific recommendation for Hepatitis or influenza vaccination.	 Major Improvement Required	Provide education to Health care providers.

APPENDIX 3: HAE AUSTRALASIA'S PROJECTS THAT CAN HELP TO MEET THE WAO RECOMMENDATIONS:

HAE Australasia's Objective: Provide education to Health care providers to enable them to appropriately recognise & support individuals suffering from HAE

- Event: **Attend Seminar & Conferences**
 - o Regular attendance at key industry events to increase the awareness amongst health care providers (HCP's) of HAE, including:
 - the symptoms
 - correct treatments
 - the difficulties that patients face when correct treatment is delayed
 - the need for patients to have an appropriate Action Plan in place
 - the need for patients and carers to have an understanding support network specific to HAE.
- Project: **Educate and Communicate with HCP's**
 - o Regular dissemination of information to HCP's that are likely to have an interaction with a patient with HAE (for example, emergency physicians, allergists, immunologists, haematologists, GP's) to:
 - ensure that they are becoming more educated about the condition so that they can identify any possible undiagnosed patients and screen those patients for HAE
 - ensure correct treatment options are provided in a timely manner to reduce symptoms (especially of life threatening attacks)
 - increase awareness of current subsidised treatments that may be an option for their patients for on-demand use
- Project: **HAE Australasia's Website to become the "go-to" place for all HAE information for HCP's**
 - o Regular review of website content by HAE experts to ensure up to date information
 - o Regularly communicating website resources to HCP's

HAE Australasia's Objective: Look at ways to make treatments and medications more accessible and subsidised for patients with HAE

- Project: **Participate in Government Assessments for Treatment Subsidisation**
 - o Be actively involved with any submission to Government Departments proposing subsidisation of HAE treatments. This includes, C1 Inhibitor, which is currently under review for subsidisation. C1 Inhibitor is the preferred treatment option for paediatric patients, pregnant patients, or patients where other treatments are contraindicated.

- Project: **Approach Pharmaceutical Companies for New Treatments**
 - o Active interaction with producers of HAE treatments that may not currently be available in the Australian market, to encourage new treatment options for Australian patients. Patients need to have options when it comes to treatments as what treatments may be suitable/tolerable for one, may not be for another.
- Event: **HAEi Global Conference**
 - o Attend the bi-annual conference to educate ourselves (and then patients) about world best standards of HAE management to determine where Australia and New Zealand need to improve - then put steps in place to attain best practice

"Since the availability of Firazyr [Icatibant] for me which was October 2012 I have not attended the hospital for any medical treatment connected to my HAE. Thanks to the assistance of HAE Australasia."

"Pregnancy and breastfeeding a newborn is hard enough, let alone dealing with HAE and not being able to take regular medication to help it go away for a little while! If only C1 Inhibitor was subsidised so that I could get through this time in my life"

"It's amazing that in countries as affluent as Australia & New Zealand, we are still so far behind the rest of the world with treatments for HAE"

"Having Firazyr available has lifted any anxieties I had about travelling away from a hospital that I knew had other emergency treatment. Thank you so much HAE Australasia for being my voice!"

HAE Australasia's Objective: Assist research organisations with medical research into the causes, prevention and treatment of HAE

- Project: **Patient Registry**
 - o Develop and maintain a registry of HAE patient data that, once de-identified, can assist in the advancement of HAE research.
- Project: **Connect Medical Researchers with Patients and Carers**
 - o Encourage active participation in current research that may lead to an improvement of treatment options or screening in the future

HAE Australasia's Objective: Provide education to HAE patients to help them better understand their condition and treatment option available.

HAE Australasia's Objective: Provide education to HAE Carers to enable them to appropriately support individuals suffering from HAE

- Event: **Annual HAE Patient & Carers Meeting**
- Event: **Local "HAE Meet Up's"**
- Project: **Newsletters and Social Media Updates to Regularly Communicate With & Educate Patient & Carers:**
 - o Discuss current treatment options available that must be discussed with their specialist physicians, including the need for a specific action plan based on their circumstances. HAE attacks in pregnant patients may not follow the "normal" trend for that patient (1), and therefore, a thorough action plan is required.
 - o Emphasise the need to follow the ASCIA Action Plan (5) for the management of their attacks.
 - o Emphasise the need for screening all blood relatives of a HAE patients. As HAE is spontaneous, the first attack a paediatric (or adult) patient may have may be a life-threatening one (1). Therefore, it is better to know if the condition is present and if so, to have an Action Plan in place.
 - o Alter patient mindset from stoically "putting up" with debilitating attacks to treating attacks early to help improve their quality of life. See Appendix 0 for more patient feedback and hesitations about self-administration of treatment.
 - o Broaden patient and carer knowledge on techniques to deal with their condition or ways to reduce triggers (for example, presentations by dietitians, psychologists, meditation therapists)
- Project: **HAE Australasia's Website to become the "go-to" place for all HAE information for Patients and Carers**
 - o Regular review of website content by HAE experts to ensure up to date information
 - o Regularly communicating website resources to patients and carers

"Without the help, support and guidance that HAE Australasia has given my family, I would not be equipped with the information required to help my daughter and she probably would not be with us today."

HAE Australasia's Objective: Provide a support group for people affected by HAE and allow patients and carers to communicate with each other to discuss their experiences. They need to know that they are not alone.

- Event: **Annual HAE Patient & Carers Meeting**
- Event: **Local "HAE Meet Up's"**
 - o Face to face interactions amongst HAE patients and carers. The only other HAE patients that they may have previously known could have just been other family members.
 - o Encourage experience sharing between patients to help them cope with their situations
- Project: **Patient and Carer Private Facebook Forum**
 - o Patients and carers can provide understanding to others that may have encountered similar issues or have had the same experience.
 - o Ability for patients and carers to ask questions of each other

HAE Australasia's Objective: Engaging in activities to raise community awareness of HAE (to help undiagnosed patients access appropriate treatment and to help the community understand what it's like to live with the life-threatening and debilitating condition)

- Project: **Increase Awareness via Social Media**
 - o Regular updates on the HAE Australasia Awareness & Support Facebook page (www.facebook.com/HAEAustralasia)
 - o Regular updates on Twitter @HAEAustralasia
 - o Encourage patients, carers and supporters to like, share, re-tweet updates, posts and links to broaden the reach of our awareness activities.
- Events: **Activities Surrounding International hae day :-)**
- Events: **Increase Awareness via Competitions and Fundraising Events**
 - o Brings new audiences to our awareness activities and broadens the community knowledge of HAE.
- Project: **HAE Australasia's Website to become the "go-to" place for all HAE information**
 - o Regular review of website content by HAE experts to ensure up to date information
 - o Regularly communicating website resources to other rare disease, genetic support and community organisations



"HAE Australasia is an important organization for the support and education of people with HAE and those associated with them, and for the furtherance of research and understanding of this condition"

"I really don't know how I got on without HAE Australasia."

"One can only imagine the progress this energetic and capable group could make in key areas such as patient identification, education, diagnosis, and research if they had access to more funding sources."

"I would like to give my thanks and sincere appreciation to those involved with HAE AUSTRALASIA, as you have given me hope and an expectation for a better future."

"I hold the HAE Australasia team in very high regard and I have absolutely no doubt that they saved my life, in more ways than one."

"It is remarkable that this intrepid group of dedicated volunteers has made such great strides in transforming the lives of a very sick and neglected patient population with relatively scarce financial resources."

"The progress HAE Australasia has made in serving the HAE patient community since its relatively recent inception is nothing short of extraordinary."

APPENDIX 4: SAMPLE OF RESPONSES FROM RESEARCH ON THE AUSTRALIAN HAE TREATMENT LANDSCAPE (2)

- Patients reported that the top 3 sources of information were their specialist, online sources (including the HAE Australasia Facebook Page) and other family members
- There was a severe negative impact on the quality of life over the long term reported by HAE patients. Some of the issues that were identified included:
 - o “Periods of childhood or adolescence severely impact e.g. teasing, missing school, not being able to join in teams, not attending social events, minimising suffering and bravado. Detrimental impact on education”
 - o “Distressing experience at A & E. e.g. Lengthy delays, misdiagnosis, wrong treatment, anxiety”
 - o “Doctors being unable to provide much support due to lack of awareness of HAE or expertise”
 - o “Career limitation e.g. Not being able to work, taking a lesser career path
 - o “A severe impact on other family members. E.g. anxiety, impact on ability to work, disruption of childhood, necessity to care for grandchildren when a parent is ill”
 - o “The restriction on ability to travel to regional areas or overseas”
 - o “A curtailment of social life and an inability to participate fully in outside interest e.g. Not being able to form friendships or attend functions”
 - o “The inability to plan in life; due to the degree of uncertainty and the unknown about attacks e.g. not joining clubs or classes, not planning longer term for activities or events, not taking up training or education”
 - o “The decision not to have children (2 patients)”
 - o “Negative impact on mental state (not a discussion point but did emerge) e.g. Causing feelings of anxiety, depression, mood swings, inability to cope, relationship issues”
- “Stoicism is likely to have contributed to:
 - o Some limitations by some patients on their use of treatment e.g. because they have grown accustomed to dealing with attacks and one’s behaviour can be difficult to change.
 - o They may not feel an attack is severe enough in relation to other attacks they can have, even though most of the population might consider the same attack to be severe.
 - o They may not have discussed the true impact on life of their less severe attacks with specialists”
- Research found that, when determining the severity of attacks, not only were patients considering things like the impact on their airway, pain level, the location of the attack, the need to attend the emergency department, but they would also consider the impact that the attack had on their daily life, if they were able to continue looking after children or go to work. There were also instances where their specialist advice was in contradiction to the ASCIA action plan and therefore wouldn’t treat an attack that may have been recommended by ASCIA (or vice versa).

- “HCP’s were thought to be very supportive of self-administered treatment but some reported that their specialist was concerned about the costs and that they thought that this might be impacting them to discourage its “over use””
- The primary non product related barriers (to self-injecting Firazyr) were:
 - o “Perception of an attack not being severe enough”
 - o “Perception that the cost of Firazyr is high and so should be retained for the worst type of attack.”
- Secondary barriers:
 - o “Risk of running out of Firazyr for a really bad attack”
 - o ““Overuse” of Firazyr might lead to it’s being rationed or withdrawn”
 - o “Frequent use might reduce a patient’s pain threshold”
- The research found that, between specialists, nurses, other HCP’s and patients, there was a discrepancy between how the severity of an attack was defined – this would impact when an attack was treated. For example:
 - o Location of the attack
 - o Mild v moderate v severe
 - o Mild v severe
 - o Non-significant v significant effect on quality of life
 - o Good v bad
 - o Making life “normal” for patient
- “Most HCP’s feel some pressure or responsibility to limit Firazyr recommendations”
- “Those (HCP’s) that recommend Firazyr only for airways/severe abdominal most cost conscious and more likely to talk about cost with patients”
- “Conversely, small number are not impacted by cost at all: These HCP’s think overall cost of treatment is low given low incidence of disease; cost savings given fewer hospital admissions and therapeutic benefit is high for patient cohort that has had inadequate treatment prior. Tends to be HCP’s that are more relaxed about patients using Firazyr to treat peripheral episodes”
- “Patients have learned to be stoic and to normalise symptoms”

APPENDIX 5: TESTIMONIALS

1. Patient - Shane

HAE Australasia and me

January 2014 my daughter sent me an email saying she was going to apply for a travel grant to the HAE conference in Washington DC, and would I like to go too.

I looked at the application and found I was still in time to apply for a travel grant, and was able to get one. Without the grant, I would not have been able to attend. My daughter didn't attend due to other issues.

At the conference, I was sought out by the HAE Australasia people, as they knew I was attending, but I had not joined HAE Australasia. I would meet one person and then they said "you must meet this person". They were very passionate and made me feel special.

The conference was really an eye opener for me and I learnt so much.

- *The president of HAEi (the international parent body), personally went around meeting everyone.*
- *Doctors gave me their contact details should I have issues (I went there on a fact finding mission to help my daughters' condition).*
- *I spoke to other patients from Australia and NZ...and around the world*

Fiona [Wardman] told me I should be under an immunologist to manage my condition. I didn't fully understand this at the time as I have been on the same medication for over 30 years and had no issues. I followed her advice and discovered that one of the side effects of Danazol, is ischaemic heart disease and liver damage. I knew I had to get my liver functions tested, but never knew why. I now take a third of my original medication and have access to [Firazyr] in the event of an attack.

The information I have obtained, directly as a result of being a member of HAE Australasia is amazing. I have not only passed this onto my immediate family, but also other lines of my family.

My dad has seen a specialist and his medication has been reduced by half. He has recently had a triple bypass. I can't help thinking that maybe if we had made contact with HAE Australasia earlier and learnt more about HAE, then this may not have been necessary.

Now getting back to my fact finding mission for my daughter. She lives in Christchurch and up until last year, was a 2nd year law student. She has had her life turned upside down due to HAE. She is a member of HAE Australasia and has been supported by them. It wasn't until August last year that I realised how dire her situation was. She was depressed and had given up on life. I was so concerned that I flew to Christchurch from Melbourne to sort out her issues. Olivia Willard, HAE Australasia NZ Director, put me in touch with Dr Jordan, an immunologist in Auckland. I arranged for a doctors referral for my daughter to see him. Olivia arranged a patient meet-up for NZ, in Auckland. Dr Jordan organised an appointment a few days before the meet-up and HAE Australasia gave her a travel grant to fly up to Auckland. She stayed with my dad in Hamilton and they both travelled to the meet-up together. At the meet-up she met a young lady, also treated by Dr Jordan, and they compared notes.

It became apparent that my daughter's HAE management is not being managed well (at all).

At a recent visit to Christchurch hospital, she woke up in ICU with a target on her throat showing the location for an emergency tracheostomy. She was given the wrong medication, and not enough of the medication she needed. I am on my way to Christchurch to meet with the department heads at Christchurch hospital to sort this out.

Without the help, support and guidance that HAE Australasia has given my family, I would not be equipped with the information required to help my daughter and she probably would not be with us today.

I am able to suggest that the hospital emergency staff get some training on HAE, and offer the help and resources of HAE Australasia to do this.

HAE Australasia, via their face book page, gives me access to patient forums where I can compare notes with other patients. We support each other, because we know what it is like to have HAE.

Via the HAE Australasia website, there are a wealth of resources to help us deal with any situation. We can get instant help via the forums or by emailing one of the team from HAE Australasia.

As you can imagine the worry I have about my daughters' condition, but HAE Australasia has been with me all the way with advice and support.

I really don't know how I got on without HAE Australasia.

*Regards
Shane*

2. Patient - Maureen

To whom it may concern,

The following is a part of how my life has been improved by being a member of HAE AUSTRALASIA.

I don't feel so alone or isolated knowing there is an organisation and people in that organisation that understands and has knowledge about this awful disease that I live with every day.

The fact that HAE AUSTRALASIA has been involved in the process of getting my medication on the PBS has greatly improved my life.

I can now afford to medicate myself and therefore have greatly enhanced my life.

I no longer need to attend the emergency department of my local hospital, for treatment that lasts between a few hours to a couple of days, depending on the severity of the attack. I have done this at least once sometimes twice a month over the last 30 years or so. Which would of been of great cost to The Health System and the community.

Since the availability of FIRAZYR For me which was October 2012 I have not attended the hospital for any medical treatment connected to my HAE. Thanks to the assistance of HAE AUSTRALASIA.

Having been educated at HAE conferences and Forums I have attended over the years, for me knowledge is power and power is strength. Strength to carry on and live a happy, well and valuable life with this insidious disease.

Having HAE AUSTRALASIA Organising Patient Meet Ups has been invaluable for my self-esteem and feeling connected.

Being one of the senior members of the HAE Community, I have been able to pass onto the younger members some of the knowledge and awareness I have gained through these meetings.

For individuals we have no hope of consulting with government departments, health bodies and drug companies regarding research, development and funding for potential new treatments.

Without the Doctors, committee and supporters of HAE AUSTRALASIA who give their time willingly our lives would be very different.

I would like to give my thanks and sincere appreciation to those involved with HAE AUSTRALASIA, as you have given me hope and an expectation for a better future.

Yours sincerely,

Maureen

3. Patient - Cindy

Just a few years ago I was a patient in a very remote location living with a rare, life threatening disease, had no effective medication on hand, knew very little information about my disease and my health care providers didn't seem to be able to give me any answers either. I felt isolated, helpless and was a ticking time bomb waiting to go off. It was very much a case of "you don't know what you don't know". Coming into contact with the HAE Australasia team has been life changing to say the least. They have given me the knowledge and the confidence to seek out the best medical treatment available. I have learned that when you have a rare condition like HAE it is vital to be an expert on your own condition and not rely on health care providers to know what's best, as this is not always the case. The education of the patients has had a massive flow on effect to many medical professionals as well. There are now many more hospitals who are not only well educated about HAE but they also stock the correct medication. This is all thanks to the power of an organisation doing the talking, rather than coming from just one person. As a group they have been able to assist in the progress of new HAE medications being considered and also subsidised. The development of the website has made important information and documents such as the Action Plan more readily accessible. The online support group and yearly patient meet ups play an important role of making patients feel understood and that they are not alone, this in itself is a very powerful thing as many patients have never met another person with the same condition.

I hold the HAE Australasia team in very high regard and I have absolutely no doubt that they saved my life, in more ways than one.

4. Anonymous Patient Testimonials & Quotes

“Having Firazyr available has lifted any anxieties I had about travelling away from a hospital that I knew had other emergency treatment. Thank you so much HAE Australasia for being my voice!”

“Pregnancy and breastfeeding a newborn is hard enough, let alone dealing with HAE and not being able to take regular medication to help it go away for a little while! If only C1 Inhibitor was subsidised so that I could get through this time in my life”

“It’s amazing that in countries as affluent as Australia & New Zealand, we are still so far behind the rest of the world with treatments for HAE”

“When I last saw my specialist for my check up, he used the HAE Australasia website to get resources that we needed for my HAE management plan.”

“I remember whenever my grandfather would have an airway attack, he would put a garden hose down his throat to make sure he could keep breathing - during those times, we didn’t know where to go to get treatment or what the treatment was”

“I don’t travel anywhere that is more than an hour from a hospital that I KNOW has C1 Inhibitor - just in case I need it”

“I’m worried that when I get pregnant, I’ll be so sick that I will be house bound and bed-ridden for the whole 9 months because I can’t use any of the few medications that are currently available to me”.

“When I was 24, I had to have half of my liver removed due to the side effects of the only long-term medication available that actually helped my HAE. I was in hospital for a month and then off work for another two months. Then, when I went back to work, I would be sick a few days every fortnight because we couldn’t find another treatment that worked.”

“Whilst attending a HAE Australasia Patient meeting, I found out that the father of a toddler with HAE works in my building! What are the chances with so few HAE patients in Australia! It’s good to know others are close by that I can talk to”

5. HAEi (International) President / HAEA (USA) President - Anthony Castaldo,

My name is Anthony Castaldo and I serve as the President of HAE International—the umbrella organization that represents the world’s HAE patient group. I am also the President of the United States HAE Association.

I have the great privilege of interacting with leaders and members of HAE patient organizations from throughout the world. I must say that the HAE Australasia group and their leadership stand out as one of the most well organized, energetic, and effective of all our member organizations. The progress HAE Australasia has made in serving the HAE patient community since its relatively recent inception is nothing short of extraordinary. With limited resources, this “can do” organization has made great strides in identifying patients and helping otherwise sick people receive desperately needed treatment. This is no small accomplishment because HAE is a rare, difficult to diagnose disease that is not only debilitating, but potentially fatal. It is remarkable that this intrepid group of dedicated volunteers has made such great strides in transforming the lives of a very sick and neglected patient population with relatively scarce financial resources. One can only imagine the progress this energetic and capable group could make in key areas such as patient identification, education, diagnosis, and research if they had access to more funding sources.

Sincerely.

Anthony J. Castaldo

President



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6. Immunology Senior Consultant – Dr William Smith



Government of South Australia
SA Health

29 January 2014

To Whom It May Concern:

RE: HAE Australasia

I am a medical immunologist working at the Royal Adelaide Hospital and in private practice. I am consulted by people who have HAE to provide medical management and medical advice.

I have found HAE Australasia to be an excellent organization, well organized and enthusiastically run. It has been very useful in providing high-quality information and support for people with HAE. The newsletters have been very effective in providing information on developments and events in the world of HAE. HAE Australasia seems to be well linked and integrated with equivalent international organisations.

I and my colleagues have from time to time carried out research with the purpose of improving management and conditions for people with HAE. This research will be greatly facilitated by HAE Australasia in terms of reaching people who might be interested in participating in research, in particular if a register were established this would be extremely effective for that purpose. The HAE community is diverse and dispersed and individuals can be hard to reach but a register would facilitate contact and communication.

I have been involved in the introduction of new innovative therapies for HAE in Australia and negotiation with authorities can be difficult. Participation of an organized patient group facilitates such negotiation and can clarify the importance of new therapies to those with a poor understanding of the condition.

In my opinion, HAE Australasia is an important organization for the support and education of people with HAE and those associated with them, and for the furtherance of research and understanding of this condition.

Yours sincerely

A handwritten signature in black ink, appearing to read 'W. Smith'.

Dr William Smith
Immunology Senior Consultant

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