



Consumers  
Health Forum  
*of Australia*

## **Consultation Report**

### **Providing Consumer Views on the Post-Market Review of the Life Saving Drugs Programme, Terms of Reference 4 and 7**

**May 2015**

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## Introduction and Summary

The Consumers Health Forum of Australia (CHF) is pleased to provide this report to the Department of Health to inform the Post-Market Review of the Life Saving Drugs Programme ().

CHF is the national peak body representing the interests of Australian consumers.

We were contracted by the Department in February 2015 to undertake a consumer consultation regarding two of the Review's Terms of Reference: Term of Reference 4, "Compare the subsidisation and equity principles of the Pharmaceutical Benefits Scheme (PBS) and the LSDP," and Term of Reference 7, "Establish a framework for data collection on rare diseases in Australia and assess how this could function internationally."

In order to provide broad consumer feedback, CHF looked both within and beyond its traditional member base to ensure a broad spectrum of views would be represented. We hosted two workshops – one in Melbourne on 19 March 2015, the other in Sydney on 24 March 2015 – that included consumer organisations within and outside of CHF's membership, and consumer representatives. The summary reports and list of attendees are available as [Appendices A and B](#). Workshop attendees were then invited to provide detailed feedback to questions asked at the workshops, and the summary of their comments is available as [Appendix C](#), with their unedited submissions available at [Appendix D](#).

CHF also launched an online survey for consumers broadly and promoted it through the workshop attendees, CHF's membership, and social media platforms. CHF ran the consumer survey through an online platform from 19 March 2015 through 10 April 2015.

While the survey was not designed to collect a random, representative sample of the Australian population, the respondents did have key demographic distributions that are consistent with the general population, to include geographic and income distribution.

The full survey report is available as [Appendix E](#).

This report reflects the views gathered across the workshops, survey, and supplementary submissions received.

Perhaps the most significant finding in this consultation is that consumers are deeply invested in ensuring that the health system lives up to the highest standards of equity and quality in providing for persons who are suffering from rare diseases.

The attendees at the consultation's workshop sessions expressed support for the LSDP – either in practice or in principle – but the consensus was that Australia needs to pursue a broader, more holistic strategy for tackling rare diseases and their treatments, and how we collect, maintain, and share information about them.

Through the online survey, consumers expressed strong support for the principles of equity of access, improving quality of life, and ensuring the overall costs to the consumer are mitigated.

There was a lack of consensus in both the workshops and in the survey about the standards that need to be used to assess quality of life, who ought to resource a broader rare disease strategy, and what the governing framework ought to be for the LSDP going forward. There was a general suggestion that PBAC criteria also need to be examined and perhaps revised as part of a future rare

disease scheme, but where and what those changes need to be was beyond the scope of this consultation.

For the consumer, the most important concerns are whether the medicines being subsidised by government are effective and targeting the most severe diseases, while keeping costs to consumers low. Although there was a broad recognition by survey respondents and workshop attendees of the need for Government to be mindful of overall costs, many believed that these concerns could be addressed through improved surveillance of effective treatments through better data collecting and sharing.

Moreover, there was a broad view that pharmaceutical companies need to be held to greater transparency standards so that Government could better assess whether the costs it pays to support consumers with rare diseases are fair.

On 10 April 2015, the Department released the Review's *Issues Paper* for public comment. This paper presents a number of questions under the Terms of Reference CHF was contracted for consultation, but which were not available at the time of the consultation. Where possible, we attempt to draw on the feedback we received during the consultations to offer a consensus opinion on those questions.

While we were not explicitly contracted to use the consumer consultation to provide input on the *Issues Paper*, we do attempt to align the comments and input we received from consumers with the options presented by the Review. While we note where there appears to be a consensus of opinion, we make no attempt to comment on which of the options the consumers consulted might prefer.

We welcome the Review and the Department's commitment to the importance of consulting with consumers on a matter that speaks to the heart of good health policy: how to achieve fairness, quality, and equity across the health system. We are confident that the views expressed by the Australian consumer and consumer-focused organisations in our consultation report will be valuable to the Review and Government as they consider options for the LSDP and treatment of rare diseases going into the future.

Leanne Wells

A handwritten signature in black ink that reads "Leanne Wells". The signature is written in a cursive, flowing style.

Chief Executive Officer  
Consumers Health Forum of Australia

## Consultation Report

### **Term of Reference 4: Compare the subsidisation and equity principles of the PBS and the LSDP**

Perhaps the dominating issue discussed at the consultation workshops was how an advisory panel is supposed to be expected to review a person's quality of life, which was very strongly viewed to be an important gauge of the programme's effectiveness. There seemed to be a consensus across consultations that it was not enough to just consider whether a medicine would extend a person's life if quality could not also be assured in some measure. This concept was also extended – but not by consensus – to improving the quality of life for a person's family, carers, and the community at large.

These concerns were shared by respondents to the consumer survey, where a large majority expressed support for the position that improving a person's quality of life is worth the cost of the medicine.

Despite this, there were only a few (and inconsistent) suggestions about what such an evaluation framework might look like, and serious concerns about how to apply such a framework consistently. However, major themes and suggestions included:

- Better defining efficacy of medicines, to include quality of life.
- Elevating the importance of clinical efficacy over cost effectiveness.
- Better defining the “rareness” of a disease in order to prevent subsets of common diseases being covered.
- Having multiple pathways for listing under the LSDP rather than requiring rejection from PBS listing for cost-effectiveness.

Another major concern raised by those consulted was the absence of a broader rare disease policy. Some even raised concerns with the Review process itself, believing it represented a lack of a holistic approach to rare diseases.

An often discussed and related issue was a deep concern over a lack of definition around what ought to be defined as “rare.” Several attendees raised the issue about whether there was adequate opportunity for consumers to provide input to the Pharmaceutical Benefits Advisory Committee's (PBAC) reviews and assessments of what treatments ought to be made available for rare diseases, or how existing medicines that are covered on the PBS might be approved for off-label use to treat rare diseases. Many were worried that a too strict definition of rare diseases would leave many without access to essential medicines, while a too broad definition would overwhelm a rare diseases scheme and make it too cost burdensome to continue.

Across the consultation workshops, attendees expressed the need to be mindful of the potential costs to Government if a future rare disease programme became too inclusive. Among their concerns was that rare subsets of common diseases, and medicines tailored to treat the, might be included in the scheme at the expense of the philosophy driving a rare diseases program. However, many felt that these costs could be mitigated by clear program criteria, regular reviews of medicines' efficacy, and improved transparency on how companies priced their medicines.

## Consultation questions

*What are the major equity considerations in comparing the PBS, LSDP, and other models for treating rare diseases?*

Two major themes presented themselves: defining what constitutes a rare disease, and defining the population that would be eligible to receive benefits under a future scheme. Taken together, those consulted were very concerned about inequity in access for consumers who suffer from rare diseases that require expensive medications but do not have a programme similar to the LSDP in place to assist with their treatments. That included the Highly Specialised Drugs Programme (HSDP), which was not commonly seen as an alternative programme for rare disease treatments. This disparity was often discussed in the context of how difficult (if not “arbitrary,” as some called it) it was for diseases and medications come to be listed under the LSDP.

*Are the various programme requirements for making high-cost drugs available to consumers promoting or hindering access?*

There was no strong consensus opinion about whether the multiple programmes and pathways for high cost drugs are promoting or restricting access. However, many of the consumers and stakeholders consulted said that the many and sometimes confusing pathways (eg, applying for listing under “rule of rescue” or seeking approval for either the HSDP or LSDP) spoke to the need for a more comprehensive rare diseases programme. Others alleged that the LSDP’s criteria seemed to be “out of step” with international best practices for providing consumers with rare diseases access to urgently needed medications. The McKell Institute’s submission in the Review’s public consultation period often cited as a good comparator of international practices that Australia should explore.

*Could PBAC criteria be restructured to better assess life-saving or drugs for rare diseases? Should there be one set of criteria to cover both high cost and/or specialised drugs?*

There was a general consensus that the PBAC criteria ought to be reviewed in order to promote more timely access to high cost and specialised drugs. In particular, many of those consulted believed that the requirement that a drug first had to be proved cost-ineffective before it could be considered for the LSDP was a barrier to access.

*Are there other mechanisms or programmes where drugs for rare diseases that are suited for neither PBS nor LSDP able to be made available to consumers?*

Although there was some discussion about whether the HSDP might become an umbrella for the LSDP, in general there was a consensus that there had to be particular attention paid to people with rare diseases needing highly specialised medicines.

## Issue Paper questions

*Should the LSDP be continued as a separate programme? If so, why?*

There was not a consensus view on this question, but most of those consulted were supportive of at least the objectives of the LSDP and were supportive of there being a scheme for rare disease treatments and medicines.

*Should there be one overarching subsidisation programme that applies to all high cost and/or specialised drugs? What criteria would be applied to such a programme that would distinguish them from other drugs subsidised on the PBS, for relatively common conditions?*

There was no consensus about whether there should be a single programme to cover all high cost or specialized drugs. If anything, there was wariness that such a programme in the absence of revised PBAC criteria and transparency requirements for pharmaceutical companies might buckle under cost pressures.

In the supplementary submissions received, Rare Voices Australia proposed creating a new category of medicine subsidisation regime, a so-called “Section 200,” that could be used exclusively for rare and very rare diseases.

*Should a programme that subsidises high cost and/or specialised drugs have one set of decision rules that apply to all drugs meeting those criteria?*

Many of those consulted recommended exploring a scheme that allows for multiple pathways for drugs to be considered for listing, so long as the final, deciding criteria were consistent.

## **Term of Reference 7: Establish a framework for data collection on rare diseases in Australia and assess how this could function internationally**

There was broad support for improving the collection of data on rare diseases. Moreover, there was agreement that any registry ought to be able to work in an international environment, and so it would be important to agree on standards for ensuring accuracy and consistency for the registry. Moreover, having an internationally-linked registry would permit researchers to draw on overseas evidence in assessing the efficacy of treatments or the emergence of new medicines that could be considered for use in Australia.

This broad level of agreement was also reflected in the survey results, with 96 per cent of respondents believing data collection on rare diseases to be important and overwhelming majorities believing it important for that data to be shared with researchers domestically and abroad (97 per cent on both questions). In the reverse direction, 84 per cent of survey respondents agreed with the position that international evidence should be used in Australia to more quickly approve and market treatments for rare, life-threatening diseases.

As such, most of the discussion at the workshops concerned the best practices for registries, and how to ensure they're properly resourced and "purpose-fit."

Many of those consulted raised the issue of the resource requirements for maintaining a robust data registry, ensuring the accuracy of information, reducing duplication, and its consistency across jurisdictions (domestic and international). Some said that establishing a registry first depends on determining its function (eg, for treatment monitoring, drug development, trial eligibility, disease-specific, etc.). Knowing how the data is intended to be used also has legal and privacy implications, as well as determines who would have access to the data. Some attendees noted that many clinicians were either reluctant to share their treatment information, or were simply inefficient in providing scarce information. There were suggestions that the patients themselves could and ought to be empowered to provide their information to a future registry.

However, few of those consulted thought that there would be major obstacles to ensuring the protection of privacy participants' data. A significant point of discussion at one workshop, however, was whether persons undergoing treatment for rare diseases ought to be obliged to provide their information to a registry. Although there was some support for this among the attendees, others said that it would not absolve the custodians of a registry from providing assurances about safeguarding the participants' information and being clear in how the data would be used.

On that point, there was significant discussion at both consultation sessions of who would fund and take ownership for such registries, and whether that has to be the same entity. Suggestions were varied, to include enlisting university support, but there was no consensus. There did seem to be wariness round entrusting pharmaceutical companies or private entities with registries and people's personal data. One particular point raised was the potential for legal complications over who ended up owning the information of a company-owned registry, and whether they could be compelled to share it with researchers and other stakeholders. At the end of these considerations, though, attendees conceded that pharmaceutical companies' participation would be necessary for any registry to be effective.

## Consultation questions

*What are the major concerns for consumers in establishing a disease registry (eg, privacy, participation, collection, etc.)?*

The major concerns identified in the consultation were safeguarding consumers' privacy and making sure that whichever entity owned the registry (or registries) could safely share the information without compromising privacy. The concern was driven not by any deficiency in laws or operating a registry, but because there can often be only a very few persons suffering from a disease that, even with de-identification methods, people might still be identifiable. Moreover, there was some concern expressed that if pharmaceutical companies became involved that there would be legal barriers concerning private ownership of information and a reluctance for companies to share potentially proprietary data.

*Is the LSDP a reasonable mechanism to collect data on rare diseases?*

There was a mixed view about this question. Many consumers with experience in the LSDP's data collection methods reported high compliance and useful information – methods which could be replicated for data collection about other rare diseases. Many of those consulted, however, expressed their belief that relying on LSDP-collected data alone would be too narrow – both for LSDP-covered diseases and rare diseases in general – and could restrict the ways in which the collected data could be used.

*Should an Australian registry go beyond LSDP-covered rare diseases?*

There was a broad consensus that there needed to be a comprehensive rare disease data collection strategy, within which LSDP-covered diseases could be captured.

*What sources of data, here or internationally, could Australia utilise to assess therapies for rare diseases?*

Attendees suggested that existing sources of data in Australia could include the Personally Controlled Electronic Health Record (PCEHR), National Disability Insurance Scheme (NDIS), NPSMedicine, and National Health and Medical Research Council (NHMRC). Existing registries which were cited as potential examples for future registries were the:

- Cystic Fibrosis Registry
- Genzyme Registry
- Australian Neuromuscular Disorders registry

## Issue Paper questions

*Should rare disease data collected internationally be used along with data collected in Australia to assess a drug's effectiveness by TGA, PBAC, or others?*

There was a strong consensus that any registry ought to be compatible with international registries, and moreover that Australia ought to be able to use information gathered abroad to support treatments and medicine reviews in Australia. This strong level of support was borne out in the consumer survey, where overwhelming majorities of respondents supported rare disease data collection, sharing of data with researchers, and interoperability with international databases.

*Should the cost of maintaining a data registry be distributed across all stakeholders? How might this be done?*

There was not as strong a consensus on which entity should bear the cost of maintaining a data registry. It was generally agreed that maintaining an effective registry was both labour and cost intensive, but because the benefits of a registry went beyond any one sector, costs might be distributed across sectors (eg, government, private, and non-government). There was not, however, any agreement on who might be best suited to make the upfront investment or maintain the registry once established.

*Drug companies often maintain their rare disease registries in order to provide regulatory agencies with additional clinical data, generally on safety, and sometimes effectiveness of the drug in the 'real world' setting. This data is not always adequate or 'fit for purpose' to answer questions raised about longer term patient benefits. Should the companies marketing these drugs be responsible for collection and maintenance of data that is 'fit for purpose'?*

Those consulted were reluctant to leave maintenance of data registries up to pharmaceutical companies, but believed strongly that the data they did collect could be useful as part of a broader scheme to collect and maintain information on rare disease treatments. Some of the leading concerns about disease registries were the lack of global data that might be available on particular diseases, making it more difficult to design an effective registry. Consumers also discussed whether there might be reluctance for multiple stakeholders to participate in a registry that they did not otherwise "own." Moreover, some raised the possibility of pharmaceutical companies claiming ownership of data that was collected during clinical trials, especially if they were given the responsibility of maintaining the registry, depriving researchers and clinicians of valuable information.

*If not the company who markets the drug, then what other effective and cost-efficient approaches are there for establishing and maintaining data registries?*

The general view in the consultation was that maintaining an effective registry would be cost or resource intensive no matter the purpose for which it was built.

## Other issues

- Obstacles to receiving therapies for rare diseases at home due to prohibitions on medicine distribution outside controlled settings.
- The quality of life implications for family members and carers.
- Changing the name of the LSDP to either better reflect the nature of the programme, which some thought was misleading, or to serve as the foundation for a larger rare diseases programme.
- Overprescribing of medicines might be unnecessarily driving up costs to the PBS, which could be skewing cost effectiveness reviews.
- Whether there has been sufficient review of medicines that were grandfathered onto the PBS, why they're maintained on the PBS, and whether there's a sufficient process in place for delisting medicines. Attendees noted that previous attempts by Government to delist medicines resulted in legal action brought by sponsoring companies.
- Could the States and Territories be responsible for funding the nursing and treatment component of rare diseases, since the Commonwealth is restricted to covering the costs of the drugs only?
- Could medicines be distributed through community pharmacies?
- Could the Department better facilitate consumer awareness about clinical trials for rare disease medicines, thus improving the amount of data available for the medicines' review?

## Issues Paper

The Department released the Review's *Issues Paper* for discussion and public consultation on 10 April 2015. While CHF was not expressly contracted by the Department to use the consumer consultation to respond to options presented in the Paper we believe that the information gathered during the consultation would be valuable to the Review and Government's deliberations.

Because CHF expects that many of the organisations and consumers it consulted for this consultation will be providing their own submissions to the Review in response to the Paper, and because CHF did not directly consult on these options, we refrain from drawing any conclusions about which option consumers might prefer. We only summarize the comments and input we received as they apply to the Paper's proposed options.

### **Option 1: LSDP continues in its current form**

The consultation found there was support for either the LSDP or an LSDP-like scheme, but there seemed to be agreement that the programme needed to be revised from its current form. Some of the more common options discussed at the workshops concerned either streamlining the listing of medicines to be covered by the LSDP through PBAC reforms, or modifying the criteria of the LSDP – and specifically the definitions around rare diseases – to make it easier for diseases and medicines to be covered. These were, however, often accompanied by discussions about not allowing the LSDP or some future rare diseases scheme to become a catch-all for variants of common diseases or tailor-made medicines.

### **Option 2: LSDP ceases and becomes an S100 programme for rare diseases**

This option was considered by workshop attendees, but generally as part of a broader strategy to address the needs of persons with rare diseases. There was some discussion about merging the LSDP with the HSDP, but the more common approach described by workshop attendees was to reform PBAC criteria for listing medicines on the PBS in such a way that might end up becoming more inclusive of medicines for rare diseases.

### **Option 3: LSDP ceases and is subsumed into state public health system**

None of those we consulted provided comments around terminating the LSDP and absorbing it into the public health system. While there was a fair amount of commentary and discussion about how to resource the LSDP or some future rare disease scheme, there were no suggestions that the responsibility for managing rare disease treatments ought to be wholly absorbed by the public health system.

### **Option 4: Other cap on total programme cost**

None of those we consulted considered formulating a cap on the LSDP or some future scheme's costs. The consumer survey found that more respondents considered it important to consider the costs of medicines on consumers rather than on the Government. Most of the comments received around containing rising costs focused on improving the transparency on how drug manufacturers set their prices, while others suggested stricter criteria to ensure that the LSDP did not become a kind of catch-all scheme for rare variations of otherwise common diseases.

## Conclusion

If there was any consensus among those we consulted, it was that while reforms to the LSDP are necessary, these must be made in concert with a more comprehensive approach towards treating rare diseases and the people who are suffering with them.

Consistent themes raised throughout the consultation concerned the quality of life for those on the LSDP, and whether government was looking at cost-effectiveness through a too narrow lens. Many consumers raised the point that providing people with rare diseases earlier and more timely access to medicines could allow them to continue to be productive and contributing members of their communities, thus lowering the long-term costs of treatment outside medicines.

Consumers in general were more concerned about ensuring that government was providing people with rare diseases, and in particular on the LSDP, with effective medicines, regardless of cost or whether a disease could be cured through treatment. Where there were concerns raised about equity principles, it was whether the process for approving medicines to be used by those covered by the LSDP respected the urgency that these medicines were needed. Moreover, there was concern that the criteria for a disease and medicine to be included under the LSDP might be too stringent and leaves out many thousands of people who are in urgent need of medicines that treat rare diseases.

Although those consulted were mindful of the potential costs to government for expanding the LSDP beyond a set of very few diseases, there was a belief that costs could be mitigated through greater transparency by pharmaceutical companies on how they price their medicines, and whether the costs they asked were justified. Some suggested that reforms to PBAC and the PBS in making medicines more readily available for off-label purposes to treat rare diseases could also keep costs low in the long-term.

There was almost no disagreement among those consulted about the importance of rare disease research, or the need for Australia to be a part of the global research community in finding effective treatments for rare diseases. There were concerns over who might be required to shoulder the burden of the costs for maintaining rare disease registries, but the support for such registries – so long as they were fit for purpose – was overwhelming in the consultation.

In summary, consumers and those consulted believed in the value of the LSDP to treat those who are afflicted with rare and life-threatening diseases. There were some calls to reform the LSDP to make it more accessible for a greater segment of Australians wrestling with rare diseases, but with an eye towards ensuring the criteria for entry were not so broad that the programme collapsed.

If the Review is to take anything away from this consultation, it's that consumers believe more value ought to be placed on the quality of life for those being treated for rare and life-threatening diseases, and that they should not have to shoulder the burden of medicines' costs. Consumers support the philosophy that drives the LSDP, and believe that whatever shape reforms take, those who suffer from rare and life-threatening diseases ought to be afforded security that their current treatments will not be adversely affected.

## Appendix A, Melbourne Workshop Summary Report

19 March 2015, 10.00am – 2.00pm

### Attendance list

Attendee	On behalf of
Anne Hunter	Fabry Support Group Australia
Clare Stuart	Tuberous Sclerosis Australia
David Menadue	National Association of People Living with HIV/AIDS
Deborah Robins	Duchenne Foundation
Diane Walsh	Consumer Representative
Esther Lim	Health Issues Centre
Helen Mikolaj	Consumer Representative
Helene Frayne	Muscular Dystrophy Queensland
Janney Wale	Consumer Representative
Joy Pettingell	Consumer Representative
Kathryn Crisell	Consumer Representative
Kathy Kendell	Health Consumers Network
Margaret Walsh	Association of Independent Retirees
Mary Jane Morales	Consumer Representative
Mary Macheras-Magias	Health Issues Centre
Megan Fookes	Rare Voices Australia
Michael Cousins	Health Consumers Association South Australia
Nicole Millis	MPS & Related Diseases Society Australia
Paula Murray	Asthma Australia
Rigoula Roussakis	Consumer Representative
Sharon Caris	Haemophilia Foundation Australia
Sharon Van der Laan	Genetic and Rare Disease Network
Simone Leyden	The Unicorn Foundation
Tricia Greenway	Consumer Representative
Varlli Beetham	Friedreich Ataxia

Attendee	On behalf of
<b>Bel Harper</b>	Consumers Health Forum of Australia
<b>Carter Moore</b>	
<b>Mehak Vohra</b>	
<b>Jo Root</b>	
<b>Adriana Platona</b>	Australian Department of Health
<b>Maria Ong</b>	
<b>Professor Paul Komesaroff</b>	LSDP Review Reference Group

## Introduction

Ms Root of CHF conducted the workshop presentation and facilitated all discussion sessions.

The introductory session provided workshop attendees with an overview of the history of the Life Saving Drugs Programme (LSDP), its costs, the drugs covered, and the nature of the consultation.

However, prior to the start of the presentation, attendees asked some clarifying questions of the Department’s representatives concerning the outcomes of the 2010 review of the LSDP. The representatives said that the major outcomes were in making the criteria for listing on the LSDP more stringent to prevent the LSDP from becoming a “catch-all” programme and emphasize the effectiveness of the medicines in treating the diseases they were intended to treat. They said that the purpose of this review was to look at assessing the values governing the LSDP.

Following the presentation, an attendee said that they felt it would be difficult to provide comments about the impact of the LSDP and its values because the benefit of medications could mean different things to different people. For some, simply arresting the symptoms of the disease could provide substantive benefits, while others need more intensive treatment. Moreover, they (and others) raised concerns about the comparative lack of evidence for the effectiveness of treatments for rare diseases compared to more common ones.

Another attendee raised concerns about the LSDP’s criteria including a mixture of drug’s efficacy and their impact on people, which the attendee felt made for a high bar for medicines to meet to be listed. The Department’s representatives said that one of the outcomes of the LSDP’s previous review was to try and prevent “perverse incentives” where pharmaceutical sponsors might artificially inflated the price of medicines so that they are rejected from listing on the PBS just to be listed on the Highly Specialised Drugs Programme (HSDP) or LSDP at inflated cost to the Government.

Other attendees raised the issue of the development of medicines that were targeted towards genetic subsets of common diseases, and whether those might either end up being listed under the LSDP or pervert the definition of rare diseases. As put by one attendee, “If everyone with lung cancer is identified with a rare, particular mutation that then gets recognized by the LSDP and treated with expensive medicines, then [the scheme] would collapse.”

## Session 1: Comparing the PBS to LSDP

This session focused on Term of Reference 4, “Compare the subsidisation and equity principles of the Pharmaceutical Benefits Scheme (PBS) and the LSDP.” The presentation covered the criteria for

medicines to be listed on the PBS, the LSDP, and other schemes. It also covered the international differences in definitions for what constitutes a “rare disease.” Attendees were then asked to discuss and report back on the following questions:

- What are the major equity considerations in comparing the PBS, LSDP, and other models for treating rare diseases?
- Are the various programme requirements for making high-cost drugs promoting or hindering consumer access?
- Could PBAC criteria be restructured to better assess life-saving or drugs for rare diseases?
- Are there other mechanisms or programmes where potentially drugs for rare disease that are neither suited for PBS (including HSDP), LSDP, able to be made available to consumers (eg, the Herceptin® Programme model)?

A few attendees raised concerns about the nature of the review itself, and why it was only the LSDP was being reviewed when Government ought to be taking a holistic approach to rare disease treatment.

Many participants said that they felt the criteria of the LSDP needed to be revised, but their reasons for wanting revision varied. Major topics included:

- Better defining efficacy of medicines, to include quality of life.
- Elevating the importance of clinical efficacy over cost effectiveness.
- Better defining the “rareness” of a disease in order to prevent subsets of common diseases being covered.
- Having multiple pathways for listing under the LSDP rather than requiring rejection from PBS listing for cost-effectiveness (the McKell Institute’s submission to the review was heavily referenced on this point).

There was considerable discussion over groups of consumers that might be “missing out” on receiving treatments owing to the restrictive criteria of the LSDP. Several attendees suggested merging the LSDP into either the PBS or HSDP. In the case of merging with the PBS, some suggested that it could help prevent pharmaceutical companies from inflating their prices for medicines by ensuring their rejection; however, other attendees countered that it might serve as a disincentive to companies from manufacturing or introducing rare disease medications entirely.

The broad view of attendees seemed to be that it was important to keep the LSDP separate from other schemes, but there were disparate views on what the LSDP should look like going forward.

There was some further discussion about whether equity principles could be among the criteria considered by PBAC on first evaluating a drug, or whether there could be broader approvals for medicines listed under the PBS to be used “off label” for the treatment of rare diseases. Some suggested that PBAC could have separate but simultaneous review streams in order to shorten the amount of time between a medicine’s application and its approval for listing under LSDP or a future rare diseases programme.

Some of the attendees expressed the need to be mindful of the potential costs to Government if a future rare disease programme became too inclusive and listed an array of specialty medicines for otherwise common diseases; however, many felt that these costs could be mitigated by regular reviews of medicines’ efficacy, improving transparency on how companies priced their medicines, and ensuring there were clear definitions for both rare diseases and the criteria for the hypothesized programme.

Other issues discussed included:

- Obstacles to receiving therapies for rare diseases at home due to prohibitions on medicine distribution outside controlled settings.
- The quality of life implications for family members and carers.
- Changing the name of the LSDP to either better reflect the nature of the programme, which some thought was misleading, or to serve as the foundation for a larger rare diseases programme.

## **Session 2: Data collection for rare diseases**

This session focused on Term of Reference 7, “Establish a framework for data collection on rare diseases in Australia and assess how this could function internationally.” The presentation covered the requirements for patients under the LSDP to provide data, international examples of rare disease databases, and the paucity of data around rare diseases in general.

Workshop attendees were asked to consider the following questions:

- What are the major concerns for consumers in establishing a disease registry (eg, privacy, participation, collection, etc.)
- Is the LSDP a reasonable mechanism to collect data on rare diseases?
- Should an Australian registry go beyond LSDP-covered rare diseases?
- What sources of data, here or internationally, could Australia utilise to assess therapies for rare diseases?
- Should rare disease data collected internationally be used along with data collected in Australia to assess a drug’s effectiveness by TGA, PBAC, or others?

There was broad consensus about the need to improve data collection on rare diseases – not just those covered by the LSDP.

Few attendees thought there would be major obstacles to ensuring the protection of privacy participants’ data, but they felt that it was important to first ensure that the nature and purpose of any rare disease registry was appropriately defined. There was a consistent view expressed that people would not mind providing their information for inclusion on a registry so long as they had assurances that their privacy was protected.

A significant point of discussion, however, was whether persons undergoing treatment for rare diseases ought to be obliged to provide their information to a registry. There seemed to be some support for this among the attendees – believing in the value of such information to others with the disease and future generations – but others said that it would not absolve the custodians of a registry from providing assurances about safeguarding the participants’ information and being clear in how the data would be used.

There was some discussion about the need to mitigate the administrative burdens of maintaining a registry, and whether it would make more sense to have multiple registries or a broader rare diseases registry – although there was no agreement among participants. The main issue seemed to fall on who ought to take responsibility for such a registry (or multiple ones) and where the funding would come from. Attendees also appeared sceptical about relying on the LSDP as being a good framework for a future rare disease registry, believing that the number of participants was too small to be meaningful outside of the narrow scope of the LSDP.

A few of the attendees expressed scepticism about relying on pharmaceutical companies to be too involved in the operations of a registry beyond clinical trials. In particular, people cited the potential for legal complications over who ended up owning the information of a company-owned registry, and whether they could be compelled to share it with researchers and other stakeholders.

There seemed to be a consensus view that any registry (or registries) ought to be able to work in an international environment, and so it would be important to agree on standards for ensuring accuracy and consistency for the registry. Moreover, having an internationally-linked registry would permit researchers to draw on overseas evidence in assessing the efficacy of treatments or the emergence of new medicines that could be considered for use in Australia.

## Appendix B, Sydney Workshop Summary Report

24 March 2015, 10.00am – 2.00pm

### Attendance list

Attendee	On behalf of
<b>Adam Johnston</b>	Consumer Representative
<b>Alison Marcus</b>	Consumer Representative
<b>Christine Walker</b>	Chronic Illness Alliance
<b>Cindy Schultz-Ferguson</b>	Consumer Representative
<b>Diana Aspinall</b>	Consumer Representative
<b>Dianne Petrie OAM</b>	Genetic Alliance Australia
<b>Doriane Ranaivoharison</b>	Genetic Alliance Australia
<b>Frank Douglas</b>	Hillcrest Rockhampton Private Hospital Consumer Reference Committee
<b>Gabrielle Quilliam</b>	Queensland Kids/Hummingbird House
<b>Gávi Ansara</b>	LGBTI Health Alliance
<b>Gen Handley</b>	Cystic Fibrosis NSW
<b>Ingo Boscheinen</b>	Australian Pompe's Association
<b>Kathryn Briant</b>	Health Care Consumers ACT
<b>Liz Pugh</b>	Federation of Ethnic Communities' Councils of Australia
<b>Lyn Whiteway</b>	Consumer Representative
<b>Margaret Knight</b>	Australian Pain Management Association
<b>Mary Potter</b>	Consumer Representative
<b>Mark de Wolf</b>	Fabry Support Group of Australia
<b>Megan Fookes</b>	Rare Voices Australia
<b>Michele Adair</b>	Cystic Fibrosis NSW
<b>Raymond Saich</b>	Australian Pompe's Association
<b>Rebecca Novacek</b>	Rare Voices Australia

Attendee	On behalf of
<b>Sam Paior</b>	Consumer Representative
<b>Sebastien Brignano</b>	Children's Health District Family Advisory Council
<b>Shelley McInnes</b>	Consumer Representative
<b>Tony Maynard</b>	National Association of People Living with HIV/AIDS

<b>Carter Moore</b>	Consumers Health Forum of Australia
<b>Fiona Walls</b>	
<b>Jo Root</b>	

<b>Adriana Platona</b>	Australian Department of Health
<b>Julie Cutts</b>	
<b>Maria Ong</b>	
<b>Professor David Isaacs</b>	LSDP Review Reference Group

## Introduction

Ms Root of CHF conducted the workshop presentation and facilitated all discussion sessions.

The introductory session provided workshop attendees with an overview of the history of the Life Saving Drugs Programme (LSDP), its costs, the drugs covered, and the nature of the consultation.

Though not included in the presentation, the bulk of the discussion following the introductory presentation, concerned the disbanding of the Disease Advisory Committees (DAC). Several of the attendees voiced their concerns about whether the loss of that independent advice would restrict future listings of drugs and diseases under the programme.

Representatives of the Department responded that there was a view that the advisory committees were essentially duplicating the work of physicians, and that it would be more valuable to the Review and the programme to release the experts on those committees to provide advice without having a potential conflict of interest.

While appreciating that the Department did not want to get involved or entangled with direct clinical care, attendees countered that the initial purpose of the DACs was to help relieve some of that burden from the Department by providing expertise to both the Department and clinicians about the efficacy of the treatments. Some felt that it was a "missed opportunity" to better utilize the DACs to inform stakeholders about the effects of drugs and treatment regimens.

A few of the workshop attendees raised the issue that the loss of expert, independent advice might also mean that consumers and persons on the programme would have one less pathway to have their concerns addressed. In particular, there was a concern that communities of persons with specific health needs would be excluded from consideration if they did not have a way to provide direct input to these committees' reviews. The Department representatives emphasized the

importance of consumer and community consultations and admitted that “visibility is limited.” One of the reasons cited for the limitations of community consultations was the proprietary nature of applications made for drugs to be listed, and they said this had been a point of resistance towards achieving a 10 week advance announcement period for PBAC meetings.

Other participants raised concerns that the loss of the DACs would limit the ability of stakeholders to assess the efficacy of the drugs being covered under the LSDP, especially with sparsely available data. The Department representatives responded to these concerns by saying that part of the purpose for Post-Market Reviews was to achieve “repeated assessments” over a drug’s lifecycle. However, they stressed that the main driver of this consultation was to examine the subsidy principles behind the LSDP.

On that point, while beyond the Terms of Reference for this consultation, several participants raised questions about whether there was enough transparency around assessing how pharmaceutical companies set their prices for medicines. The Department representatives responded that while that discussion was not explicitly within the boundaries of this consultation period, it was not off the table, and more consumer experiences and concerns were welcomed – especially if one of the outcomes of the Review would be to take on board more rare diseases and, by extension, high-cost medicines.

A last major concern for some attendees was the level of involvement of pharmaceutical companies in the Post-Market Review process. Some were taken aback by the suggestions raised in the pharmaceutical companies’ initial submissions to the Review. The Department representatives responded to these concerns by saying that it was necessary for the Review to undertake consultation with all key stakeholders.

### **Session 1: Comparing the PBS to LSDP**

This session focused on Term of Reference 4, “Compare the subsidisation and equity principles of the Pharmaceutical Benefits Scheme (PBS) and the LSDP.” The presentation covered the criteria for medicines to be listed on the PBS, the LSDP, and other schemes. It also covered the international differences in definitions for what constitutes a “rare disease.” Attendees were then asked to discuss and report back on the following questions:

- What are the major equity considerations in comparing the PBS, LSDP, and other models for treating rare diseases?
- Are the various programme requirements for making high-cost drugs promoting or hindering consumer access?
- Could PBAC criteria be restructured to better assess life-saving or drugs for rare diseases?
- Are there other mechanisms or programmes where potentially drugs for rare disease that are neither suited for PBS (including HSDP), LSDP, able to be made available to consumers (eg, the Herceptin® Programme model)?

One of the major issues attendees discussed was how a panel is supposed to review a person’s quality of life, and how to apply such standards consistently. There seemed to be a consensus in the room that it was not enough to just consider whether a medicine would extend a person’s life if quality could not also be assured in some measure. One group of attendees said that this extended beyond the person being treated and into their families, carers, and the community at large.

Despite this point of conversation, there were no suggestions about what such an evaluation framework might look like.

Another major concern raised by attendees was that, in the absence of a rare disease policy, rare subsets of otherwise common diseases might end up being defined as “rare” and overwhelm either the LSDP or the Highly Specialised Drug Programme (HSDP). This discussion included concerns about how more drugs were being increasingly “tailor made,” which in turn increased their potential cost. This might create the position of drugs being excluded from listing from the PBS due to cost consideration, but then not being brought under either the LSDP or HSDP because the disease was not rare – or was just a subset of a common disease.

That led to discussion about whether it was improper to base criteria for listing under the LSDP on an initial consideration for listing on the PBS. A few attendees raised the idea of having special access provisions to allow PBAC to immediately consider a drug for the LSDP, or whether PBAC’s criteria concerning “cost effectiveness” needed to be revised more broadly.

One attendee raised the concern that some populations more prone to rare or uncommon diseases than others might feel that they were being denied access to medicines not due to any objective cost considerations, but because of discriminatory attitudes towards their population.

Several attendees raised the issue about whether there was adequate opportunity for consumers to provide input to PBAC reviews and assessments of what ought to be covered as a “rare disease.” As one attendee put it, “[We] shouldn’t have to have a lobbyist in the ear of the Minister.”

The last major point of discussion was the lack of available data for assessing the efficacy of treatments and for anticipating medicines to treat additional rare diseases. Many attendees expressed concerns about how difficult it was for people with rare diseases to receive an initial diagnosis due to a lack of data, which led to prolonged and unnecessary suffering while they awaited diagnosis and treatment. This rekindled discussion of the DACs’ disbanding.

Side discussions of whether the LSDP was appropriately named yielded only one recommended suggestion that seemed to have broad agreement:

- Rare Disease(s) Programme

This seemed to be consistent with attendees’ concerns that Australia lacked a truly comprehensive rare diseases programme, and the need for Government to take a “whole of care” approach towards rare diseases.

Other issues raised included:

- Overprescribing of medicines might be unnecessarily driving up costs to the PBS, which could be skewing cost effectiveness reviews.
- Whether there has been sufficient review of medicines that were grandfathered onto the PBS, why they’re maintained on the PBS, and whether there’s a sufficient process in place for delisting medicines. Attendees noted that previous attempts by Government to delist medicines resulted in legal action brought by sponsoring companies.
- Could the States and Territories be responsible for funding the nursing and treatment component of rare diseases, since the Commonwealth is restricted to covering the costs of the drugs only?
- Could medicines be distributed through community pharmacies?
- Could the Department better facilitate consumer awareness about clinical trials for rare disease medicines, thus improving the amount of data available for the medicines’ review?

## Session 2: Data collection for rare diseases

This session focused on Term of Reference 7, “Establish a framework for data collection on rare diseases in Australia and assess how this could function internationally.” The presentation covered the requirements for patients under the LSDP to provide data, international examples of rare disease databases, and the paucity of data around rare diseases in general.

Workshop attendees were asked to consider the following questions:

- What are the major concerns for consumers in establishing a disease registry (eg, privacy, participation, collection, etc.)
- Is the LSDP a reasonable mechanism to collect data on rare diseases?
- Should an Australian registry go beyond LSDP-covered rare diseases?
- What sources of data, here or internationally, could Australia utilise to assess therapies for rare diseases?
- Should rare disease data collected internationally be used along with data collected in Australia to assess a drug’s effectiveness by TGA, PBAC, or others?

Two existing registries anchored most of this session’s discussions: the Cystic Fibrosis Registry and the Transplant Registry. In the case of the former, an attendee noted that it is a national database that spans 15 years, which provides important longitudinal information and has been used by consumer advocates to successfully lobby for more resources. For the latter, attendees noted that there have been problems with linking datasets, but it is still a vital tool for improving practices and examining quality of life outcomes.

Several attendees raised the issue of the resource requirements for maintaining a robust data registry, ensuring the accuracy of information, reducing duplication, and its consistency across jurisdictions (domestic and international). Some said that establishing a registry first depends on determining its function (eg, for treatment monitoring, drug development, trial eligibility, disease-specific, etc.). Knowing how the data is intended to be used also has legal and privacy implications, as well as determines who would have access to the data.

This led into a conversation of who would fund and take ownership for such registries, and whether that has to be the same entity. Suggestions were varied, to include enlisting university support, but there was no consensus. There did seem to be wariness round entrusting pharmaceutical companies or private entities with registries and people’s personal data, but attendees conceded that their participation would be necessary for any registry to be effective.

Many suggestions for establishing a framework for rare disease collection in Australia included borrowing from existing international registries, both to ensure consistency with international researchers and to prevent having to reinvent the wheel for successful models.

When the conversation became specific to the LSDP, some attendees acknowledged that it was a good source of data, and that patients could be relied upon to provide consistent and useful information, but it was too narrow in scope to be the foundation for a larger rare diseases registry. Attendees also noted that, even under the best practices for de-identification, in the cases of truly rare diseases, people could still be personally identified in a database.

Attendees suggested that existing sources of data in Australia could include the PCEHR, NDIS, NPSMedicine, and NHMRC. However, some attendees noted that many clinicians were either reluctant to share their treatment information, or were simply inefficient in providing scarce information.

## Appendix C, Supplemental Responses to Consultation Questions

### Submissions Received

<b>Organisation</b>
<b>MPS and Related Diseases Society</b>
<b>Australian Pompe's Association</b>
<b>Duchenne Foundation</b>
<b>Rare Voices Australia</b>
<b>Cystic Fibrosis Australia</b>
<b>Anonymous</b>

<b>Individual</b>
<b>Kathryn Crisell</b>
<b>Eddie Iwanowski</b>
<b>Louise Duffy</b>
<b>Darren Carmichael</b>
<b>Mario Fici</b>
<b>Anonymous</b>
<b>Anonymous</b>
<b>Anonymous</b>

## Appendix D, Organisation Submissions to Consultation Questions

### MPS and Related Diseases Society

*What are the major equity considerations in comparing the PBS, LSDP, and other models for treating rare diseases?*

Model needs to allow for timely reimbursement of treatments unlike current system which sees a drug needs to go through PBAC and deemed unsuitable for PBS (due to cost) than recommended for listing for LSDP) which then awaits decision by Cabinet.

Positive of PBS is that it considers [quality of life] unlike the LSDP, however rare disease treatments will never be cost effective due to small patients numbers. The need to prove treatments are life extending is not appropriate due to limited data in rare disease and the fact that patients limited ability to wait for longitudinal data. While this data should be collected, patients should not have to wait for it before they can access treatment.

All patients should have access to the best possible treatment available at any particular time. Funding treatments for only a subset of patients is unfair. Telling patient groups they need to compromise is unfair and does not acknowledge that for patients 'compromise' is never a win-win situation but for many it means simply no access to treatment. A more equitable situation would be if instead pharma and Department of Health reached a compromise with more sustainable pricing arrangements/ risk sharing arrangements that also encourage development of better, less intrusive treatments.

*Are the various programme requirements for making high-cost drugs promoting or hindering consumer access?*

No response.

*Could PBAC criteria be restructured to better assess life-saving or orphan drugs for rare diseases?*

Threshold for clinical effectiveness should be lower for a treatment when there is no other comparable treatment available as untreated patients will deteriorate. Once there is a funded treatment any newer treatments can be compared to existing treatment when assessing clinical effectiveness.

*Are there other potential mechanisms or programmes where drugs for rare diseases that are suited for neither PBS (including HSDP) nor LSDP could be made available to consumers (eg, the Herceptin® Programme model)?*

No response.

*What are the major concerns for consumers in establishing a disease registry (eg, privacy, participation, collection, etc.)?*

No response.

*Is the LSDP a reasonable mechanism to collect data on rare diseases?*

No response.

*Should an Australian registry go beyond LSDP-covered rare diseases?*

No response.

*What sources of data, here or internationally, could Australia utilise to assess therapies for rare diseases?*

No response.

*Should rare disease data collected internationally be used along with data collected in Australia to assess a drug's effectiveness by TGA, PBAC, or others?*

No response.

*Do you have any other feedback related to these consultation questions, or any of the discussion that took place at the workshop?*

No response.

## Australian Pompe's Association

*What are the major equity considerations in comparing the PBS, LSDP, and other models for treating rare diseases?*

Due to their rarity, it is very difficult to collect large volumes of data for rare diseases. It is particularly provide Australian based evidence to the level required by the PBS given the small patient populations of rare diseases. For this reason treatments for rare diseases need their own programme of assessment to ensure equitable access is provided to Australians with rare conditions.

One recommendation we have that could provide further benchmarking for the programme would be for the Department of Health to undertake an annual review of treatments for rare diseases available under a comparable system – the UK's National Institute for Health and Care Excellence (NICE). This could be developed into a comparative report for guidance in Australia.

*Are the various programme requirements for making high-cost drugs promoting or hindering consumer access?*

The LSDP requirements operation here in Australia are hindering consumer access to medications for rare diseases. The system is too slow meaning patients are being left without treatment for years. These delays can mean that patients are becoming avoidably disabled or being left to deteriorate with life threatening conditions despite the fact that a registered treatment exists which is widely used internationally.

*Could PBAC criteria be restructured to better assess life-saving or orphan drugs for rare diseases?*

There are a number of potential ways to restructure criteria to better assess treatments for rare diseases:

- Better definition within the existing programme: One recommendation we have is to update the wording of Criterion 4 to remove the subjective word 'substantially.' It may provide more clarity to put a quantifiable goal for life extension, such as an expectation that life may be extended for two or more years.
- Removing impediments to applications: For example, direct applications for listing on the LSDP need to first have an application to PBS rejected – which is time consuming and unnecessary.
- Evaluations taking in the broader context of the impacts of rare diseases and benefits of treatment: The programme should take into account the benefits of treatment on quality of life, as well as the broader social and economic improvements in productivity of providing treatment.

Rare diseases don't just affect the patient they affect the whole family including careers and livelihoods of so many. Treatments keep people productive and engaged in the community not dependent on it and taking further family members out of the workforce to become carers. There is a need to focus not just on the costs but on the benefits of keeping patients at work or parents and loved ones from becoming full time carers.

*Are there other potential mechanisms or programmes where drugs for rare diseases that are suited for neither PBS (including HSDP) nor LSDP could be made available to consumers (eg, the Herceptin® Programme model)?*

Currently many people depend on compassionate access schemes. However these present a challenge for a company if they develop a drug, have done all the clinical trials and registrations but then find that it will take years to get funded. Compassionate access provides an opportunity while TGA and PBS approvals are sought to obtain local patient experience and data testing is normally done overseas but there needs to be more certainty of funding in a reasonable period.

It would be wonderful for the Department of Health and Federal Government to look into alternative programmes to provide access to treatments for rare diseases. Perhaps a system for negotiated or managed entry providing treatment to orphan drugs for those in need while further evaluations are taking place could be provided.

*What are the major concerns for consumers in establishing a disease registry (eg, privacy, participation, collection, etc.)?*

The majority of APA members are very happy to participate in providing their information to a registry. This registry could collect data on the condition and link clinicians with other treating doctors as well as holding clinical data.

The key areas that APA would like to make suggestions are around the sustainable funding and planning for an independent and secure registry and database of details for rare diseases:

- **Funding:** The patient registry should be funded by the Department of Health to ensure that it is available to all relevant stakeholders and is sustainable long term. It is also important that healthcare professionals are incentivised to enter data into the database (such as Medicare funding for appropriate testing and record keeping to ensure consistent information for each disease)
- **International comparisons:** It is critical that Australian based disease registries should be compatible with the current International and industry based disease registries to ensure that
- **Patient information and data entry:** An online survey such as that used by the Erasmus Pompe Patient Annual Survey could be utilised, with patients inputting their data directly where possible to reduce administration times.

The patients are a great resource. Providing patients themselves with surveys to complete with their own information reduces the impact on healthcare professionals and minimizes privacy issues. Other registries rely on nurses to complete the data entry. APA would like to suggest that the patients who are able (and their carers) are requested to complete a medical update each year.

Over 80% of APA members receiving treatment voluntarily complete the Erasmus patient survey each year and we have over 90% compliance each year. Some APA members currently completing three registries LSDP, Erasmus and Pompe Registry. There is currently no Medicare funding for testing of Pompe patients not on treatment or on the International Compassionate Access Programme (ICAP) on which many of our members rely for treatment.

- Management and administration of the registry: An independent expert body should be engaged to manage the registry. The APA would like to see the registry tendered to see what organisations universities and commercial companies who are currently doing data base management can offer. Possible managers for the programme would be universities or consumer organisations such as the CHF or Rare Voices Australia (RVA) funded and managing the programme on behalf of the Department of Health.

*Is the LSDP a reasonable mechanism to collect data on rare diseases?*

The APA believes that data collection should go beyond collection for those currently receiving treatment for a rare disease, and thus while collection through the LSDP is reasonable a registry or database should go beyond the existing parameters for the programme. APA has 34 patients distributed over 20 hospitals with a significant variability of treatment from site to site, which should be corrected. Centres of excellence in each state should be established for annual assessment with the sophisticated equipment required (for example, BioDex) to ensure consistent reproducible results. There is currently no protocol or Medicare funding for testing and monitoring of patients who are not currently receiving treatment either through the LSDP or compassionate access, which means data is incomplete.

*Should an Australian registry go beyond LSDP-covered rare diseases?*

Yes. This is important also for connecting patients and doctors of patients with ultra-rare diseases, and could aide in addressing the challenges of multiple misdiagnoses experienced by people with rare diseases by improving information sharing. APA currently has 34 patients with members in every state and territory but others have maybe 4 or 5 what do they do and who do they go to understand the system and disease they have. Australia has a complex health system PBS LSDP and it's hard to come to terms with at a time when you or your child are critically ill. A registry could assist in overcoming some of the barriers faced.

*What sources of data, here or internationally, could Australia utilise to assess therapies for rare diseases?*

As referenced earlier in relation to the collection of patient information and data entry - Patients themselves are a great resource! Over 80% of APA members receiving treatment voluntarily complete the Erasmus patient survey each year and we have over 90% compliance each year. Some APA members currently completing three registries LSDP Erasmus and Pompe Registry. Australians are included in a number of international studies, and these should be taken into account for assessing therapies. Assessment should go beyond this however, and take into account international data and assessments of treatments, particularly given the low patient numbers and challenges collecting data for rare diseases.

*Should rare disease data collected internationally be used along with data collected in Australia to assess a drug's effectiveness by TGA, PBAC, or others?*

Yes. Australia is too small to rely solely on local data for Ultra rare diseases. The APA has only 34 patients Pompe is a spectrum disorder and we need to include overseas data to improve our understanding of the condition and treatments. With such low numbers impacted by specific rare diseases, there is potential for Australia to suddenly be affected by a rare disease that has had no or very limited presence before, making the international experience of this disease essential. Due to the genetic nature of many rare conditions, including Pompe disease, environmental factors in other countries are unlikely to affect the data. It is critical that Australian data collected is compatible with the International data and a local rare disease registry should take this into account.

*Do you have any other feedback related to these consultation questions, or any of the discussion that took place at the workshop?*

Is there a better Name for LSDP? The name of the programme should be reflective of the intent of the programme – supporting Australians' access treatment for serious rare diseases. Alternative names could better represent this programme such as changing the name to the 'Rare Diseases Programme.'

## Duchenne Foundation

*What are the major equity considerations in comparing the PBS, LSDP, and other models for treating rare diseases?*

- 1- Disproportion of representation for rare diseases - who are small in population for each disorder but a large number when combined. Thus the thinking that LSDP should not be capped and that it should be an arm of PBS rather than a separate programme.
- 2- The original purpose seems to have produced a small club of largely enzyme disorders. A renaming and redefining of purpose would make the programme more encompassing - particularly when compared to [overseas] approvals and reimbursements for innovative new therapies. Programme coverage for all rare diseases needs to be spread better amongst efficacious treatments without which society pays a high burden of care through disability, adaptive housing, aids and therapy, hospitalizations, loss of contributions to the workforce.
- 3- The application process must be clearer and equitable since there shouldn't be more ambiguity and barriers particularly for manufacturers and consumers with rare diseases. One example is in the risk/benefit analysis...whilst many of these disorders are 100% fatal, the acceptable therapy outcome alternatives to years of total paralysis and truncated lifespan could be less demanding than life/death or full recovery because an improvement in Quality of Life could very well mean the difference between a life and a living hell.
- 4- The process could involve local acceleration where approvals have been granted by overseas counterparts - saving time, cost and lives, because there are reciprocal standards that are agreed upon by international collaboration. Australians feel on a par with the developed world and expect equity with patients in USA & Europe for instance. In our community there have been cases where parents will clutch at straws and travel to the 3rd world where standards are not so high to pursue dangerous or ineffectual experimental treatments. Having timely access to proven treatments will impact upon such unsafe and wasteful practices.

*Are the various programme requirements for making high-cost drugs promoting or hindering consumer access?*

No specific analysis recalled but from reading the submissions from industry and hearing fellow consumer groups reps who display more experience discuss it, we'd have to agree that there needs to be more clarification of terms and a more lucid process which will not only incentivize new drug development to supply to smaller markets like Australia, but more importantly, expedite access to those who will be saved from debilitating, truncated lives.

*Could PBAC criteria be restructured to better assess life saving or orphan drugs for rare diseases?*

Many consumers seemed to recommend the case-by-case decision making model cited in the McKell Institute report, where submissions could be ranked on all manner of criteria with less weighting given to cost effectiveness because a whole range of factors would be considered and rated. Validating a wide range of rankings on each scale and aggregation of scores could help balance the fine line between good medical outcomes and compassion in a more efficient manner.

*Are there other potential mechanisms or programmes where drugs for rare diseases that are suited for neither PBS (including HSDP) nor LSDP could be made available to consumers (eg, the Herceptin® Programme model)?*

Once again, no knowledge of such programmes for Duchenne or other rare diseases; but I sense these are special programmes to address a common lifestyle disease? The size of the population to benefit would be higher and warrant a special programme to stop an epidemic of drug abuse or loss of life from an aggressive cancer? But if every application were reviewed case-by-case as more or less a separate "programme" which comes under PBS, then the best conditions for assessment and implementation could be decided based on all factors, but most importantly each drug programme would be based on the outcomes for the target patient group. Flexibility and adaptation is how mankind progresses - PTC for instance have recently announced an extremely ethical siblings programme for all candidates participating in an open label trial of their DMD drug, Translarna. Under the programme, siblings can automatically have access to their brother's medicine so that the disease will not be abated in one sibling more than the other. This initiative from a first-in-class drug company is naturally welcome news for consumers but it shows that we are not only all breaking new ground but we can rewrite the rules if we are keeping patients as our core business.

*What are the major concerns for consumers in establishing a disease registry (eg, privacy, participation, collection, etc.)?*

None, beyond the standard privacy considerations as set out in ethics approvals required. De-identified data is our legacy and the diagnostic and treatment odyssey so arduous, I believe people with rare diseases would consider it their duty to provide their statistics and records for the benefit of mankind in the future. Data should be kept beyond death or the "research" value of the registry will be severely diminished. A registry is akin to research and it is an understanding that research is not for the benefit of today's patient but of future patients.

*Is the LSDP a reasonable mechanism to collect data on rare diseases?*

I don't believe so, although it should access, curate and add data about the patients it serves. Such a huge database for many thousands of diseases needs to be delimited quickly from national medical records in order to make a start. It affects so many people, that it would take decades and billions to build from the ground up. Technology and creative consents could be utilized to stop the unnecessary duplication of effort and cost in our health system.

*Should an Australian registry go beyond LSDP-covered rare diseases?*

Definitely.

*What sources of data, here or internationally, could Australia utilise to assess therapies for rare diseases?*

Not a lot of therapies have had the time to be independently reviewed. Perhaps UPPMD post marketing surveillance will result in very objective data collection one day. However peers of published research and other international regulatory agencies like the EMA & FDA examine the research sponsored by the drug company for objective proof of efficacy and are very stringent and so we might expect reciprocal approvals based on the assessments of either of these agencies.

*Should rare disease data collected internationally be used along with data collected in Australia to assess a drug's effectiveness by TGA, PBAC, or others?*

Yes, this is the concept being investigated by the TREAT-NMD network into which the Australian Neuromuscular Disorders registry feeds. It is hoped that these databases will be extended and used for post market surveillance.

*Do you have any other feedback related to these consultation questions, or any of the discussion that took place at the workshop?*

No response.

## **Anonymous**

*What are the major equity considerations in comparing the PBS, LSDP, and other models for treating rare diseases?*

No response.

*Are the various programme requirements for making high-cost drugs promoting or hindering consumer access?*

They are hindering consumer access. The criteria for some of the already approved drugs are hard to meet, in that, it usually means a consumer needs to be showing signs of irreversible damage before treatment can begin.

*Could PBAC criteria be restructured to better assess life saving or orphan drugs for rare diseases?*

Yes, the consumer needs to be able to assess these drugs before irreparable damage is done to vital organs.

*Are there other potential mechanisms or programmes where drugs for rare diseases that are suited for neither PBS (including HSDP) nor LSDP could be made available to consumers (eg, the Herceptin® Programme model)?*

No response.

*What are the major concerns for consumers in establishing a disease registry (eg, privacy, participation, collection, etc.)?*

Privacy is one of the main concerns. Also who will own the information and enter the data? Who will fund the registry?

*Is the LSDP a reasonable mechanism to collect data on rare diseases?*

No, the LSDP only has a limited number of drugs for a limited number of diseases available. The data collected should come from a wider group of diseases, then as new drugs become available in the future, the data for getting these drugs onto the LSDP will already be there.

*Should an Australian registry go beyond LSDP-covered rare diseases?*

Yes, as new drugs become available in the future, the data for getting these drugs onto the LSDP will already be there.

*What sources of data, here or internationally, could Australia utilise to assess therapies for rare diseases?*

No response.

*Should rare disease data collected internationally be used along with data collected in Australia to assess a drug's effectiveness by TGA, PBAC, or others?*

Yes, as these diseases generally have low numbers of patients, the more information that can be obtained the better the decision that can be made to help those who really need it.

*Do you have any other feedback related to these consultation questions, or any of the discussion that took place at the workshop?*

No response.



Rare Voices Australia (RVA) welcomes opportunity to attend and give its perspective to the Life Saving Drugs Program (LSDP) Consultation Meetings facilitated by Consumer Health Forum held in both Melbourne and Sydney in March 2015.

Rare Voices Australia has circulated the CHF survey to members to respond.

***Is there a better Name for Life Saving Drugs Program (LSDP)?***

Rare Voices Australia does not believe the name 'life saving' serves the program well. The term 'life saving' is often misinterpreted. The treatments that are listed on the program are by no means 'cures' of disease. It is more reasonable and accurate to have a program with a name that is reflective of its purpose. As was discussed within the context of the consultation meetings, a better name could be 'Rare Disease Treatment Program.'

***What are the major equity considerations in comparing the PBS, LSDP, and other models for treating rare diseases?***

Equity considerations that need to be considered when comparing a program designed to list drugs and treatments for common conditions that treat thousands of people and thousands of conditions throughout the country as opposed to a program that talks to a very small population of people with very rare diseases are;

1. The PBS is a model that is designed and accommodates literally thousands of treatments and medicines for conditions that can be prescribed and accessed readily via a local General Practitioner and local pharmacy. The condition is commonly known, understood with excellent data to justify the use of the medicine administered.
2. The LSDP is a very specific program whereby the treatments are specific to very rare diseases. Therefore these rare diseases will not be clearly understood by the average local GP or local pharmacy. Rare Diseases require a multidisciplinary approach in their management. The treatments listed on the LSDP are highly specialised and require specific management around their storage and handling. Therefore additional care is required to actually dispense and administer the infusions. The ERT programs are co-ordinated by Centres of Expertise and administered by experienced nurse co-ordinators in hospitals and experienced hospital in home services in the case of home infusion programs. Therefore these treatments require a different model that is more tailored to fit a particular need.
3. A model that could be adopted that acts similarly to Section 100 – Highly Specialised Drugs Model i.e. a new Section 200 model could be tailored for Rare and Very Rare Disease Therapies. This model could be established specifically for the listing of these therapies. The new model would enable the PBS to recognise the high costs of the therapies but enable them to be funded and managed appropriately in an existing structure removing the cumbersome administrative burden currently experienced with the LSDP.

***Are the various program requirements for making high-cost drugs promoting or hindering consumer access?***

- The LSDP program requirements are hindering consumer access. The criteria to access the LSDP have been stated as ‘arbitrary’.
- The criteria appear to keep patients out of the program as long as possible and do not seem to promote bringing new patients onto the program.
- The fact that patients are diagnosed with disease early and can’t access the treatment until symptoms are in life threatening stage (when symptoms are irreversible) seems to be counterproductive and go against basic human right principles.
- The program needs to do better. When it works - it works well.
- However there are reported cases whereby people are simply not accessing therapy. This is unacceptable. What hope do they have with such a process?

***Could PBAC criteria be restructured to better assess lifesaving or drugs for rare diseases?***

Yes there are a number of ways in which the PBAC criteria could be restructured to better assess lifesaving or drugs for rare diseases.

**Process/ logistics around process;**

1. Shorten the application process to fit within a shorter and ‘typical PBAC cycle’ the first time to ensure limited delay. Remove the process in which a direct application for listing on the LSDP needs to first have application to the PBS rejected – which is time consuming and unnecessary.
2. A solution may be to adopt a more formal pre-submission process. A process that may include the sponsor, Pharmaceutical Benefits Advisory Committee (PBAC) Chair or delegate, representatives from the Department of Health, a clinician with specialty expertise, a health consumer representative and potentially an evaluator. This group would meet to formally discuss the submission and determine its eligibility for a Section 200 listing.
3. To assist the difficult task of assessing these products, a new Rare Disease Subcommittee (RDS) of the PBAC could be formed with the specific remit of assessing submissions for Section 200 listing:
  - The RDS should include standing representatives from the PBAC.
  - The RDS should include additional clinical experts with specific rare disease experience, to provide a report to the PBAC to allow them to make a determination on the subsidisation of products seeking listing under Section 200.
  - The RDS should include consumer representation from the rare disease community.

Evaluations and assessments currently do not take into account quality of life and broader benefits to the person living with the rare disease and the implications to their immediate family and immediate environment. The program fails to consider their broader social and economic context and improvements in productivity of providing treatment. The focus needs to shift towards the benefits of treatment as opposed to keeping patients off treatment altogether.

***Are there other mechanisms or programs where potentially drugs for rare disease that are neither suited for PBS (including HSDP), LSDP, able to be made available to consumers (e.g., the Herceptin® Program model)?***

RVA would like to see a program adopted specifically for rare diseases that fits the people it is serving. An improved LSDP renamed and restructured is long overdue that enables patients to access rare disease treatments.

As discussed in earlier points a fit for purpose model based on the Section 100 model that has been adopted and formatted to serve rare disease treatments is RVA's preferred model when considering a change of model.

***What are the major concerns for consumers in establishing a disease registry (e.g., privacy, participation, collection, etc?)***

Without actually reaching out formally to the RVA members by a survey, RVA makes comment based on anecdotal references. Collection of data is a 'hot topic' and is receiving a lot of attention across the rare disease space.

The RVA Roadshow meetings indicated that some interesting facts;

- a. People living with a rare disease have limited capacity to contribute genotypic and phenotypic data on their rare disease. This limits capacity to develop diagnostics and treatments for their rare disease. The vast majority of these people do not access hospital services.
- b. Australia has multiple rare disease data registries with duplicated effort across the different registries. Streamlined and integrated approaches are required. More efficient systems and tools are needed to collect, share and analyse patient data, and to manage data entry, access, consent and privacy issues. Data registries need to be harmonised nationally and internationally.
- c. A national registry or linked up registries is required to collect phenotype data that can be matched to genetic data that can be used for research into better diagnostics and treatments, and to direct policy and service delivery. For ultra-rare diseases a registry, resources and the right international linkages are required to support clinical trials.
- d. Many questions need answering to progress a national registry for example around feasibility, costs, benefits, access, usability, quality control, recruiting participants, gaining consent, and ongoing maintenance.

***Is the LSDP a reasonable mechanism to collect data on rare diseases?***

1. The LSDP has been collecting data to justify treatment. Why? Is this done anywhere else?
2. The justification for data collection and the intent without speaking with the founders of the LSDP, is to have a co-ordinated approach to treating such rare diseases. To further understand and manage these very rare diseases. If it is not managed by the LSDP – where will it be managed?

3. RVA believes the data collection needs to extend beyond those on the current program. Each State/ Territory jurisdiction could support funded centres of expertise to co-ordinate assessment.
4. There needs to be a National approach and co-ordinated approach to testing. At the moment it varies greatly from State to State.

***Should an Australian registry go beyond LSDP-covered rare diseases?***

1. Yes, an Australian registry should go beyond LSDP-covered rare diseases but only if the disease in its entirety is defined as a rare disease. If the disease is a common disease with a rare sub-set, this is not classed by definition as a 'rare disease'. The definition RVA defers to is the European definition.
2. Collection of data for people on treatment and not on treatment is equally important and aids research.
3. Natural history data can assist in research for future therapies and assist with new knowledge. This is so important and crucial in assisting clinicians with managing the patients and for patients themselves to make crucial family planning decisions and decisions about future wellbeing.
4. Registries can assist the issue of multiple misdiagnoses experienced by people with rare diseases by improving information sharing.

***What sources of data, here or internationally, could Australia utilise to assess therapies for rare diseases?***

The sources that RVA defers to in the absence of local information being co-ordinated at this point and time are as follows;

1. Orphanet is the reference portal for information on rare diseases and orphan drugs, for all audiences. Orphanet's aim is to help improve the diagnosis, care and treatment of patients with rare diseases. The Australian Orphanet page still being set up is managed by the Department of Health in Western Australia. <http://www.orpha.net> There is also an 'app' now available to patients.
2. National Organisation of Rare Disorders (founded in the United States 30 years ago) funded by NIH. <https://www.rarediseases.org/>
3. But ultimately the patients themselves are the best experts and have the most knowledge of their particular rare disease.
4. Many folk living with a rare disease are already in international registries that are set up by Australians – such as the TREAT NMD international registry for the numerous Neuromuscular disorders, the Fabry Disease Registry and the Cystic Fibrosis Registry to name a few.

When assessing therapies – the Australian Department of Health needs to work with such international registries to support their work. Especially as many of the rare diseases impact very small cohorts of patients. The larger international registries have much larger data sets for analysis.

***Should rare disease data collected internationally be used along with data collected in Australia to assess a drug's effectiveness by TGA, PBAC, or others?***

RVA does believe that a combination of international and local data is needed especially as many of our patients come from other countries and most of these diseases are genetic. Therefore it is crucial we collaborate across borders and work with international data. In the case of very small cohorts of patients it is difficult to assess treatments using local data. Working with international data can provide additional confidence when undergoing assessments. It is critical that Australian data collected is compatible with the International data and a local rare disease registry should take this into account.

Thank you again Carter and Jo Root for your work behind the scenes managing the consultation meetings with very short notice. It was an interesting discussion at both the Sydney and Melbourne meetings. Despite the varied delegates with vast range of experience in and around the LSDP, the conversations and questions raised were excellent and many people came very well prepared and well-read. I congratulate CHF for its facilitation. Rare Voices Australia is happy to contribute and collaborate further as needed.

Kindest Regards,

Megan Fookes  
Executive Director  
Rare Voices Australia

Fabry Support Group Australia attended the Life Saving Drugs Program (LSDP) Consultation Meetings facilitated by Consumer Health Forum held in both Melbourne and Sydney in March 2015. Mrs Anne Hunter; FSGA National Office Manager attended the Melbourne meeting and Mr Mark De Wolf; FSGA Board of Directors, attended the Sydney meeting. Fabry Support Group Australia has circulated the CHF survey to members to respond. Below are some further points to consider from Fabry Support Group Australia, in relation to the questions asked by the Consumer Health Forum concerning the review of the LSDP.

***Is there a better Name for Life Saving Drugs Program (LSDP)?***

Fabry Support Group Australia does not believe the name 'life saving' drugs program as appropriate. The treatments and therapies listed for Fabry disease on the program are not claiming to be 'life saving', but rather 'life stabilising' or 'life extending'. The program name needs to reflect this. All the treatments listed on the program are for very rare diseases and are specifically tailored to work for those particular diseases. The name needs to consider these points.

A better name may be 'Rare and Very Rare Disease Treatment Program'

***What are the major equity considerations in comparing the PBS, LSDP, and other models for treating rare diseases?***

The major equity considerations that FSGA believes need to be considered when comparing models for treatment of diseases, such as the PBS and LSDP include; Common disease and rare disease data is very different. Unlike common conditions whereby the data is accessible from a large cohort of patients, the same cannot be applied to rare disease treatments, with studies limited by smaller sample sizes. This limitation of the data needs to be considered.

The treatments listed on the LSDP for Fabry disease are lifelong therapies that are needed fortnightly. The average GP lacks knowledge of this condition, the many symptoms of Fabry disease, and a diagnosis is often delayed. A recent survey of the Fabry Community in Australia, conducted by Stollznow research, found that in 15% of the cases surveyed, a diagnosis took 10 years or longer after the onset of symptoms. Most GP's are not equipped to deal with the challenges of the average Fabry sufferer, necessitating Fabry patients to be managed at Centres of Excellence for children and adults in each jurisdiction.

***Are the various program requirements for making high-cost drugs promoting or hindering consumer access?***

The LSDP program requirements, such as the criteria to qualify to receive therapy, seems to be 'out of step' with global best practice. In other countries, those diagnosed with Fabry disease needing to commence therapy, do so without delay. Accessing essential therapy is inappropriately delayed in Australia, and there are very few children on ERT in Australia. This contrasts to other parts of the world.

The FSGA questions the program's efficiency, and is concerned that the current program may be hindering patient's access, denying essential treatment to those who need it.

The LSDP is not a program that delivers cures for Fabry disease. Enzyme Replacement Therapies (ERT) are life extending and life stabilising, and do not claim to cure the disease. ERT is limited in its ability to reverse the organ damage once already done. This was demonstrated in the early clinical trials in the year 2000, where the patients receiving the treatments were already in late stages of disease progression with existing major organ damage. Therapies should be administered earlier to patients (including children), when there are early signs and symptoms of the disease, prior to permanent organ damage. Fabry patients should not have to wait to become 'sick enough' to commence treatment, when permanent organ damage has already occurred. Studies into the benefits of treatment of paediatrics should be reviewed and conducted, and the program should consider the best practice guidelines of other countries in regards to the treatment of the paediatric Fabry patient. Commencement of therapy in Australia seems delayed, with the criteria to access therapy insisting on certain organ damage, and evidence of disease progression. Patients are not receiving therapy until they exhibit major organ involvement, and the process in which the program lists patients to access therapy, seems inconsistent and arbitrary.

Access to therapy has been largely the responsibility of the Advisory Committees. This decision making process has been largely unknown to the patient community, with a lack of transparency of process. Now that the Medical Advisory Committees have been disbanded, the FSGA questions who is now responsible for making the decision to review patients' access to treatment, and what is the process by which a patient can appeal a decision they believe to be unjustified? The FSGA believes it to be unethical to deny patients access to treatment, and that most GPs would lack the specialist understanding to manage and represent such cases.

The treatments listed on the LSDP are specific to the diseases that are listed for them. There are no alternative treatments for those particular diseases. The treatments are highly innovative and are 'tailor made' for those particular conditions. They are therapies for life long progressive diseases.

***Could PBAC criteria be restructured to better assess lifesaving or drugs for rare diseases?***

The FSGA believes the PBAC criteria could be better restructured. The treatments are listed as safe and effective by the TGA, and should not require reassessment by the PBAC? This process is inefficient and costly.

The process of listing new treatments needs to be transparent and time efficient. Any delays in this process, results in further unnecessary disease progression. New therapies, once trialed, should be promptly listed. We now have a third generation of Fabry patients within our support group, and since 1995, little change has occurred in regards to the LSDP process, despite advances in research and diagnosis. The policies need to step up and match the changes. Time to respond!

***What are the major concerns for consumers in establishing a disease registry (e.g., privacy, participation, collection, etc?)***

This question is raised from time to time with the FSGA members, and many Fabry patients are willing to sign up for research with most recognising the benefits to their family and future generations.

For Fabry Disease, with the introduction of the first Enzyme Replacement Therapies (ERT), both respective companies started registries. Now the two are harmonised into one registry. However this registry is for patients receiving treatment. Some Centres of Excellence collect data for both patients on and off treatments, however there is opportunity to co-ordinate and provide incentive to collect data. FSGA knows that the Centres are poorly resourced and administration of data collection is time consuming. FSGA could partner in such a process, and there are opportunities whereby Patient Organisations could contribute to managing data entry to registries.

In Adelaide DNA testing is done and collected for Fabry patients and has been going on for many years. All the tests are sent to Adelaide to verify results. There is great opportunity for establishing an independent registry to assist with valuable research and data collection, assisting to develop care plans and guidelines for people living with Fabry disease.

***Is the LSDP a reasonable mechanism to collect data on rare diseases?***

People living with Fabry Disease on treatment find collecting data part of the routine but it isn't always well co-ordinated and the results are not always shared. There is inconsistency throughout the country. Fabry patients need to know how their disease is progressing and this can be done in consultation with the State Centres of Excellence for both patients on treatment and off treatment. Why is it a requirement of the LSDP? Why do patients living with Fabry disease need to justify their use of a therapy for their ongoing care?

***Should an Australian registry go beyond LSDP-covered rare diseases?***

An Australian registry should go beyond LSDP-covered rare diseases. Collecting data for all rare diseases will assist research and assist new knowledge to better equip the medical professions and companies investing in new treatments but most importantly to the people living with such rare diseases.

***What sources of data, here or internationally, could Australia utilise to assess therapies for rare diseases?***

In Australia, Fabry patients use the Genzyme Registry but this is only for people living with Fabry disease on treatment. An independent registry is something the Fabry International Network (FIN) has been trying to establish, and this effort should be supported.

***Should rare disease data collected internationally be used along with data collected in Australia to assess a drug's effectiveness by TGA, PBAC, or others?***

FSGA does believe that using a combination of international and local data is important as our Fabry population is small and making decisions based on small cohorts is difficult. Combining data internationally is also important, as there are many mutations of Fabry Disease. Treatment responses are varied, often due to the responsiveness of the particular mutation to the treatment. Combining data internationally allows for better analysis of results of treatment, allowing for further understanding.

Kindest Regards,

Sheridan Campbell.  
FSGA Board of Directors Chair

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## Appendix E, Consumer Survey Report

### Introduction

CHF ran a consumer survey through an online platform from 19 March 2015 through 10 April 2015. It was promoted through the CHF membership, social media and publications, and to attendees at the consultation's workshops for promotion through their networks. There were 174 respondents to the survey, with 89.7 per cent (157) completing the survey's core questions.

### Respondents' Demographics

While the survey was not designed to collect a random, representative sample of the Australian population, the respondents did have key demographic distributions that are consistent with the general population. The survey takers were diverse across age groups, with all adult age groups represented (Table 1). However, persons aged 56 to 75 were more represented among the survey respondents than in the general population<sup>1</sup>, with persons aged 18 to 25 under represented.

All Australian States were represented in the group of respondents. The respondents' geographic distribution generally followed that of the Australian population at large<sup>2</sup>, although the ACT was strongly over-represented, while New South Wales was under-represented (Table 2). Respondents' reported household income was very consistent with the general population's<sup>3</sup> (Chart A).

Less than seven per cent of respondents spoke a language other than English at home – a measure of cultural and linguistic diversity – which is less than the estimated 20 per cent of Australians who are culturally and linguistically diverse. Just less than three per cent of respondents indicated Aboriginal or Torres Strait Islander heritage, which is slightly below estimates of the Aboriginal and Torres Strait Islander community's representation in the general population<sup>4</sup>.

The survey respondents were higher users of the health system than Australians in general<sup>5</sup> (Table 3). The average number of GP visits in the last year among survey respondents who self-reported their GP visits in the last year was 7.9, versus 5.6 for the general population. 98.7 per cent of respondents who provided GP visit information had at least one visit in the last year, versus 84.7 per cent of the general population. Even if we're to assume that all survey respondents who did not provide GP visit information did not visit one in the last year, it would mean 90.2 per cent of respondents had at least 1 GP visit in the last year – well above the general population.

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<sup>1</sup> Australian Bureau of Statistics, 3235.0 - *Population by Age and Sex, Regions of Australia, 2013*.

<sup>2</sup> Ibid., 3101.0 - *Australian Demographic Statistics, Sep 2014*.

<sup>3</sup> Ibid., 6523.0 - *Household Income and Income Distribution, Australia, 2011-12*.

<sup>4</sup> Ibid., 3238.0.55.001 - *Estimates of Aboriginal and Torres Strait Islander Australians, June 2011*.

<sup>5</sup> National Health Performance Authority 2015, *Healthy Communities: Frequent GP attenders and their use of health services in 2012–13*.

## Summary of Results

Consumers were very strongly supportive of the LSDP's guiding philosophy – providing those with rare and life-threatening diseases with urgent medicines.

Their primary concerns are not with costs or bureaucracies, but ensuring that effective medicines reach those who need them the most – even if those medicines cannot rid someone of disease. They were concerned with whether those being treated for rare and life threatening diseases could be afforded a good quality of life, and ensuring that their experience could be used by researchers to improve the lives of others.

In order to better understand the survey's results, CHF asked respondents a number of questions about their income, utilisation of the health system, and knowledge of the costs of certain medicine programmes.

These questions were designed to help determine whether there might be significant differences in respondent cohorts based on whether they were more or less likely to support certain principles based on their assumptions about its potential costs or the frequency with which they use the health system.

The only significant indicator we found in the results to separate the respondents into cohorts was their self-reported use of the health system by how many times they estimated visiting a GP in the last year. We categorized these cohorts in the same manner as the National Health Performance Authority did in their report, *Healthy Communities: Frequent GP attenders and their use of health services in 2012–13*, replicated in Table 3.

But even dissecting by these cohorts, there was not a significant variation in their opinions or perceptions from the overall respondent view, and so is not reported.

## Equity Principles

One of the primary drivers behind the equity concerns of the LSDP falls on how PBAC reviews applications for medicines, and whether cost-effectiveness too grossly outweighs what consumers feel ought to be more important considerations. However, one of the challenges in surveying consumers about these issues is to present the very technical process of PBAC's review in terms that are accessible by all consumers, regardless of their familiarity with the health system and nuances of the PBAC process.

The questions CHF designed asked respondents to rate the importance to them of PBAC's major thematic considerations when determining whether to list a medicine on the PBS. These were:

- How effective the drug is in treating the disease
- The severity of the disease being treated
- The drug's cost to the consumer
- The availability of other treatments
- The rarity of the disease being treated
- The drug's cost to government

The survey randomised these options in order to prevent bias in more strongly preferring the first option. Respondents were asked to rate the importance of these criteria using a Likert Scale.

Overwhelmingly, respondents were more concerned that the medicine was effective (92.4 per cent rating 4 or 5), followed by ensuring medicines that treated severe diseases were accessible (83.5 per cent rating 4 or 5). The least concerning criterion was the medicine's potential cost to Government, with only 30.6 per cent considering that important (4 or 5) versus 45.9 per cent considering it unimportant (1 or 2). (Chart B)

The next major segment of the survey asked for respondents' opinions regarding some of the more significant issues CHF expected to identify ahead of the consultations. While these were developed in consultation with the Review's medical ethicist, we did not have the benefit of the issues paper going into the survey's design. We framed these as values statements, and asked respondents to rate how strongly they agreed or disagreed with the propositions. The questions were:

- People with rare, life-threatening diseases should have access to all possible forms of treatment, including medicines.
- Government should subsidise medicines for rare, life-threatening diseases, even if few people require them.
- People with rare, life-threatening diseases should have to pay at least part of the cost of the medicine, even if it's very expensive.
- Government should only subsidise treatments for rare, life-threatening diseases that are proven to be effective.
- Australia should consider using evidence from overseas regulators to more quickly approve and market treatments for rare, life-threatening diseases.
- Improving a person's quality of life is worth the cost of a medicine, even if the medicine cannot totally rid that person of their illness or condition.

The survey randomised these options in order to prevent bias in more strongly preferring the first option. Respondents were asked to rate the importance of these criteria using a Likert Scale.

There was strong support in favour for Government to provide or promote access to medicines for those in urgent need, and for people to be able to maintain quality of life even if their disease was incurable. There was strong opposition to the proposal that persons with rare or life-threatening diseases should bear part of the costs of their medicines.

Respondents were most divided on the question of whether Government subsidy should be tied to a medicine's effectiveness. While a majority of respondents agreed with the proposal (62 per cent), in the open-ended section of the survey, respondents questioned how one might define "efficacy," especially when it comes to considering a person's quality of life.

After asking these values statements, the survey provided respondents with the true costs of the LSDP, and asked if knowing those costs affected their support for the values positions. This was done based on a popular line of argument that if consumers knew the costs of these programmes, they might urge Government restraint. Overwhelmingly, though, respondents indicated that knowing the costs of the LSDP did not affect their support (84.0 per cent).

Of the few respondents who indicated that their opinions were changed (8.3 per cent), when they were asked the questions again, we found that two respondents' indicated levels of support had not changed. Moreover, most people's support only changed for one or a few questions, not on the whole. Where there were changes, the average change was less than one point on the Likert Scale.

The questions that had the highest net positive changes were for persons with rare or life threatening diseases to pay a portion of the cost of their medicines (5 positive, 1 negative, 5 no change; average change +0.45) and that Government should only subsidise medicines proven to be effective (5 positive, 2 negative, 4 no change; average change +0.18). The largest decline in support was for the position that a person's quality of life was important regardless of the cost of the medicine or its efficacy (3 positive, 4 negative, 4 no change; average change -0.45).

However, so few respondents indicated that their positions had changed in light of the cost data, and because their changes in support rarely moved them from agreeing to disagreeing with any particular statement, the overall effect on the strength of agreement with the statements was negligible (Chart C).

### **Rare Disease Data Collection**

There was very strong support among the survey's respondents for collecting data on rare diseases, to include using data collected overseas and sharing Australian data with international researchers.

When asked the values statement, "Australia should consider using evidence from overseas regulators to more quickly approve and market treatments for rare, life-threatening diseases," 74.7 per cent of respondents agreed (4 or 5).

When asked directly, "How important do you believe it is that the Government collect and maintain data on persons undergoing treatment for rare diseases for research purposes?," 96.8 per cent of respondents felt it was very (82.5 per cent) or somewhat (14.3 per cent) important. These levels of support for research were consistent across sharing data with researchers and engaging in post-market surveillance for efficacy

### **Other Issues identified**

The end of the survey allowed for respondents to provide open-ended feedback on issues raised in or related to the survey's themes. Analysing the responses received, the following major issues were identified, although not always consistently nor with a consensus of how to resolve them.

- Ensuring that funding of common and chronic diseases is not subsidised.
- Assisting people with rare and life-threatening diseases in finding clinical trials that might aid in their treatment and overall wellbeing.
- Pressuring pharmaceutical companies to be transparent about the costs incurred in developing their medicines.
- Ensuring that Australia's treatments and research for rare diseases are "benchmarked" to best-practice standards so that it becomes an international leader in the field.

## Charts and Tables

**Table 1**

<b>Age Distribution of Adult Survey Respondents by Respondents' Self-Reported Age</b>			
<i>Age Range</i>	<i>% of Respondents</i>	<i>% of Australians</i>	<i>Diff.</i>
18 to 25	5.3	14.0	<b>-8.7</b>
26 to 35	15.4	17.9	<b>-2.5</b>
36 to 45	22.5	18.6	<b>+3.9</b>
46 to 55	17.2	17.8	<b>-0.6</b>
56 to 65	22.5	14.8	<b>+7.7</b>
66 to 75	14.8	9.4	<b>+5.4</b>
Older than 75	2.8	7.7	<b>-4.9</b>

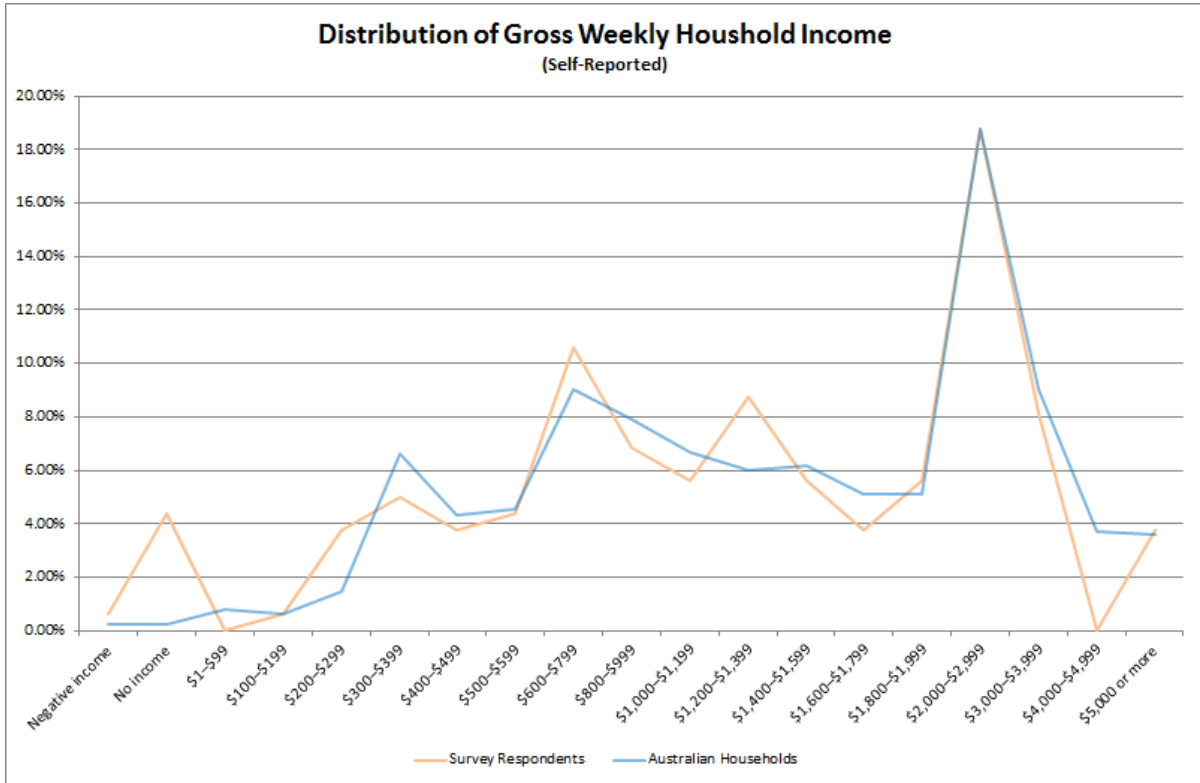
**Table 2**

<b>State and Territory Distribution of Survey Respondents by Respondents' Self-Reported State of Residence</b>			
<i>State</i>	<i>% of Respondents</i>	<i>% Australians</i>	<i>Diff.</i>
VIC	29.6	24.9	<b>+4.7</b>
NSW	23.7	32.0	<b>-8.3</b>
QLD	19.5	20.1	<b>-0.6</b>
ACT	9.5	1.6	<b>+7.7</b>
WA	8.9	11.0	<b>-2.1</b>
SA	5.9	7.2	<b>-1.3</b>
TAS	1.8	2.2	<b>-0.4</b>
NT	1.2	1.0	<b>+0.2</b>

**Table 3**

<b>Distribution of Survey Respondents by Respondents' Self-Reported Number of GP Visits in the last year</b>			
<i>Category</i>	<i>% of Respondents</i>	<i>% Australians</i>	<i>Diff.</i>
Very high (20+ visits)	9.4	3.8	<b>+5.6</b>
Frequent (12-19 visits)	10.7	8.7	<b>+2.0</b>
Above average (6-11 visits)	30.8	22.8	<b>+8.0</b>
Occasional (4-5 visits)	25.2	15.8	<b>+9.4</b>
Low (1-3 visits)	22.6	33.6	<b>-11.0</b>
Did not attend (no visits)	1.3	15.3	<b>-14.0</b>

**Chart A**



**Chart B**

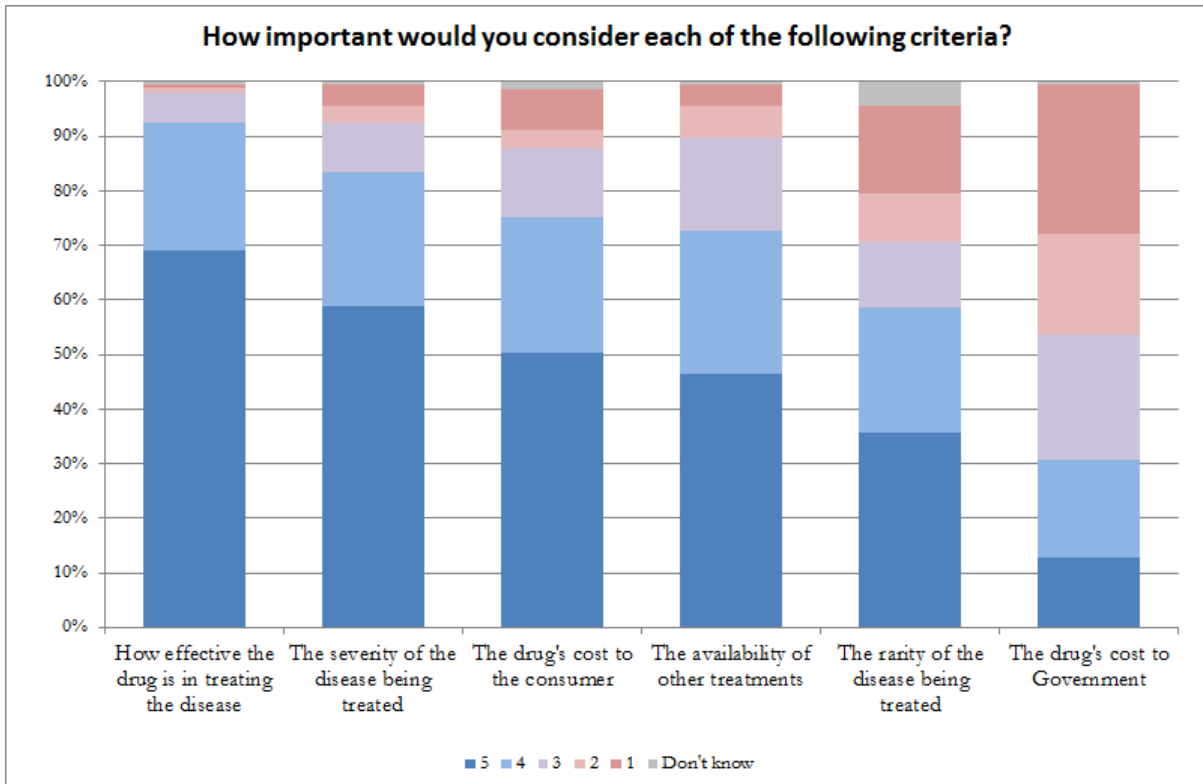
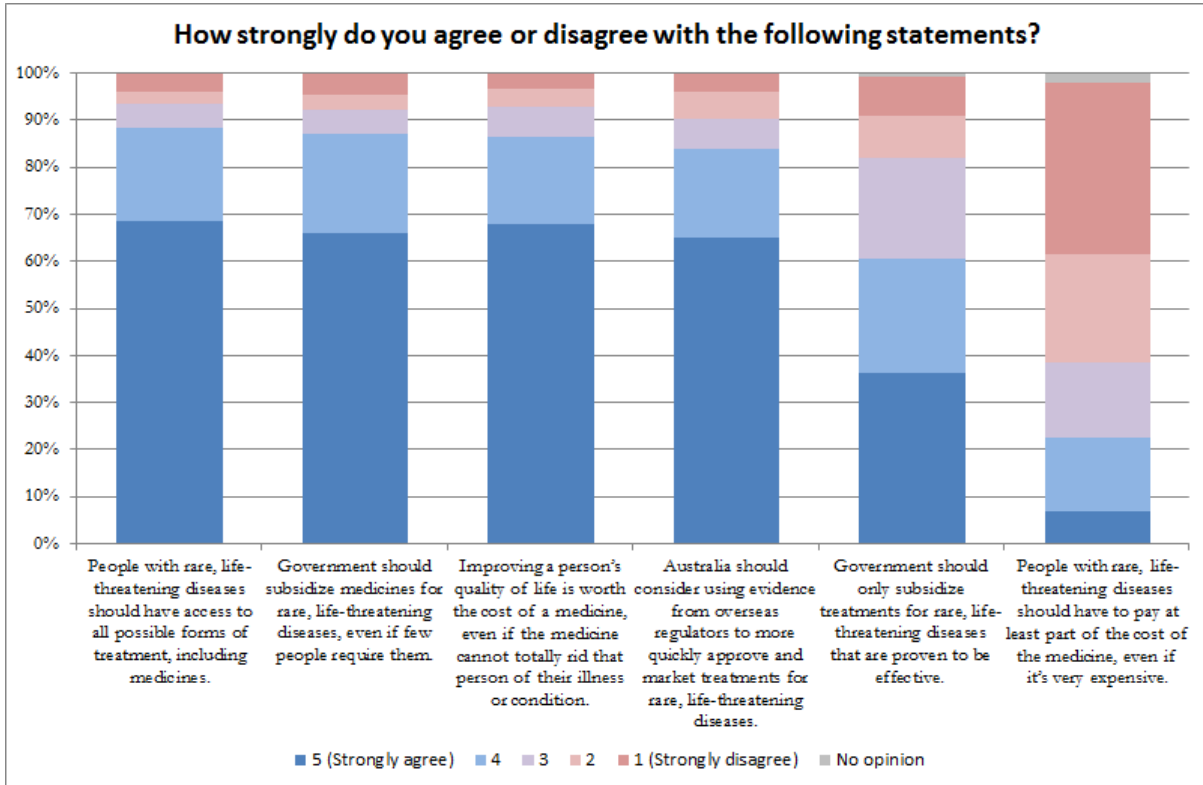


Chart C





## **Background information**

The Consumers Health Forum of Australia Inc (CHF) is the national voice for health consumers. As an independent non-government organisation, CHF helps shape Australia's health system by representing and involving consumers in health policy and programme development.

Health consumers have a unique and important perspective on health as the users and beneficiaries of health care and, ultimately, those who pay for it. CHF takes consumers' views to government and policy makers, providing an important balance to the views of health care professionals, service providers and industry to achieve a health system that reflects the needs of all stakeholders.

CHF member organisations reach millions of Australian health consumers across a wide range of health interests and health system experiences. Health policy is developed through wide consultation with members, ensuring a broad, representative, health consumer perspective.

Current priorities include safety and quality in health care, safe and appropriate use of medicines and health care for people with chronic conditions. CHF also facilitates the appointment of consumer representatives on over 200 national health-related committees.

CHF believes all consumers should receive affordable, safe, good quality health care at the time they need it. The best outcomes are achieved when consumers are involved in decisions about and management of their own health care. Consumers should receive health care information when they need it in a form they can understand, particularly about using medicines.

Established in 1987, CHF receives funding from the Australian Government Department of Health and Ageing and membership fees. It seeks external funding for priority projects.

With its ability to access a variety of health consumer networks and extensive knowledge of consumer issues, CHF is a respected and influential contributor to the Australian health debate.

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