Australian Government

Department of Health

General Guidance for Providing Feedback to the Contracted Assessment

Immunoglobulin Review

MSAC Referral 1592

Primary Immunodeficiency Diseases (PID) with Antibody Deficiency

Public consultation closes 9.00 am 10 September 2020

COVER SHEET FOR ALL FEEDBACK SUBMISSIONS

This cover sheet must be included with your feedback (submission). If completing by hand, please ensure your writing is clear and legible.

Details	
Individual name/group name/organisation name[1]	Dr Theresa Cole, ASCIA Immunodeficiency committee chair
Please delete categories that do not apply	Medical Officer / NGO

CONTACT DETAILS	
We would like to collect your contact details should further information or clarification be required on your submission. If you agree, contents of your submission may be included in subsequent documents accessed by the Department of Health, MSAC and any documents published during the review process (refer to permissions to publish below). Please provide at least one contact address. If you are making a submission for a group or organisation, please provide contact information for one member of your group or organisation. If you would like to remain anonymous, please leave this section blank.	
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INTERNET PUBLICATION	
Please tick this box if you wish for your submission to remain confidential and **do not consent** to having information from your submission published on the internet or as part of the MSAC public summary document.	
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THIRD PARTY PERSONAL INFORMATION	
Please tick this box if your submission contains **personal information of third party individuals.**	

EVIDENCE OF CONSENT	
You should not include personal information about a third party unless you are able to provide evidence of written consent. Please tick this box if you have **attached evidence of written consent**.	

[1] Please leave blank if you would like to remain anonymous. A pseudonym may be provided if preferred.

N.B. Feedback will be accepted up to a maximum of 4 pages using font no smaller than size 11 Calibri.

GENERAL INSTRUCTIONS (ALL RESPONDENTS)

The purpose of the consultation is to obtain feedback on the draft Contracted Assessment on Immunoglobulin for Primary Immunodeficiency Diseases (PID) with Antibody Deficiency.

The information collected will be used to inform the Medical Services Advisory Committee (MSAC) process to ensure that the use of government-funded immunoglobulin in Australia is patient focused and seeks to achieve best value.

You are invited to provide feedback on the following questions:

1. The context for the Contracted Assessment is outlined in the executive summary on pages 9-13, and the body of the report on pages 22-37. Do you agree or have any comments on this section?
   This review appears to be focused on intravenous immunoglobulin (IVIg) in patients with common variable immunodeficiency (CVID). This is a subpopulation of all patients requiring immunoglobulin and is not representative of all patients. Most CVID is diagnosed in adulthood whereas other primary immunodeficiencies (PIDs) are more common in children. The search terms for the literature review are not exhaustive and do not include a number of PIDs that result in hypogammaglobulinaemia. There is also a large body of literature around use of subcutaneous immunoglobulin (SCIg) that does not appear to have been included. SCIg is generally better tolerated than IVIg, with fewer side effects and this has been entirely missed in the review. It should also be noted that patients requiring SCIg need training by registered nurses with experience in administering and running SCIg programs, not technicians.

   As such, this review cannot be extrapolated to other PID patients and the conclusions are unable to be generalised. There are unlikely to be further studies in this field as immunoglobulin replacement therapy (IRT) is an internationally accepted standard for PIDs and it would be unethical to withhold treatment.

   There is already an Australian PID database. However, it is not likely we can achieve any subgroup analysis as numbers are currently too small. International registries already exist with far larger numbers of patients.

   Again, it will be difficult to assess impact of other therapies such as antibiotics due to the heterogeneity of the patient cohort.

2. The benefits (effectiveness) of immunoglobulin therapy are provided in the executive summary on pages 14-15, and the main body of the report on pages 62-70. Is this a reasonable interpretation of the evidence?
   This review does not adequately assess the benefit of immunoglobulin replacement therapy (IRT) in preventing life threatening infections. Historical data clearly shows that, for example, patients with x-linked agammaglobulinemia died in childhood before the advent of IRT. It would be entirely unacceptable to perform a placebo-controlled trial in any patient group with risk of life-threatening infections or long term organ damage as a result of infection.

3. Are there other benefits not captured in the report?
   With no significant review of the SCIg literature there is a large body of evidence missing demonstrating fewer side effects and better patient tolerability.

4. The safety or side effects associated with immunoglobulin therapy are provided in the executive summary on page 14, and the body of the report on pages 57-61. Are there other adverse effects that are not captured in the report?
   The side effects presented are true for IVIg but not SCIg and therefore conclusions are over stated by not reviewing the SCIg literature.

5. Overall, has all the relevant evidence been taken into account?
   No, this is a suboptimal review in terms of patient cohorts and immunoglobulin products.

6. What comments do you have on the inputs and outcomes of the economic evaluation (executive summary pages 16-17, and body of the report pages 82-99)?
   I do not understand the calculations of healthcare cost per patient ass demonstrated in table D2.4 as this combines both SCIg and IVIg. Patients receive one or the other so these should be presented as two separate tables. It is also inaccurate to say patients need months of training for SCIg administration. This can often be achieved within a few weeks. Therefore, the cost of Ig administration in table D 3.1 may be incorrect.

   Development of a national model of care for SCIg should be considered, to ensure that patients are appropriately supported and that the governance of Ig products is secured with home based IRT. Initial SCIg training (and the ongoing support for patients to ensure they can continue to give SCIg successfully at home) is most often provided by specialist registered nurses (very rarely by medical staff). Therefore, models of care around the provision of SCIg should include the input of specialist nurses to ensure the success of SCIg programs, otherwise patients can end back on IVIg in hospital.

7. Do you have any comments on the assumptions and estimates of the overall financial costs for governments in the Contracted Assessment (executive summary pages 17-18, and body of the report pages 100-109)?
   Again by focusing on CVID and therefore mostly adults, this does not assess costs to children with other PIDs which may be different.

8. Do you have any comments on potential areas for future research (see Other Relevant Considerations) identified in the Contracted Assessment (executive summary pages 18-19 and body of the report on page 110)?
   There will be limited ability to perform research in this area within Australia. We have a relatively small population of patients with PIDs and within that there is a range of different groups. Many international registries exist and we should look to them for information on larger cohorts.

ADDITIONAL QUESTIONS FOR PATIENTS, CARERS AND CONSUMER ORGANISATIONS

Patients, carers and consumer organisations are invited to provide feedback **on all areas** of the draft Contracted Assessment as outlined above.

In particular, feedback on the following areas are considered of value from a patient and carer perspective:

1. The evidence presented in the assessment report suggests that immunoglobulin (Ig) therapy has poorer safety (more adverse effects) and may have superior effectiveness than no Ig therapy. Based on your knowledge and experience do you agree with this? What are the benefits of Ig therapy and do they outweigh any adverse effects you may have experienced?

2. Where do you receive your immunoglobulin infusions (e.g. public hospital, private hospital, or home)? Do you have any issues accessing this therapy?

3. What out-of-pocket costs are there for patients when receiving Ig therapy (e.g. specialist fees, tests required for review not fully covered by Medicare or health insurance)?

4. Do you receive antimicrobial prophylaxis (e.g. ongoing treatment with antibiotics to prevent infections) in addition to Ig therapy?

5. Do you have any other comments you would like to make in relation to this review?

If you require any further guidance please contact the MSAC IG secretariat.

Privacy

Unless otherwise requested, all submissions on the draft contracted assessment to MSAC Ig Referral 1592 will be provided to the Evaluation Sub-Committee of MSAC and the MSAC.

Responsibility for copyright in submissions resides with the author(s), not with the Department of Health.

Your submission and contact details will be stored in accordance with the Australian Privacy Principles from Schedule 1 of the *Privacy Amendment (Enhancing Privacy Principles) Act 2012* and the *Archives Act 2012*. Should you have any concerns about the storage of your submission, or if you wish to gain access to make a correction, please contact the MSAC Ig Review Secretariat. A copy of the Department's privacy policy is available on request. If you wish to make a complaint about the handling of your private information, you may contact the Department of Health Privacy Contact Officer on 02 6289 5773 and, if unsatisfied with the response, you may submit a complaint to the Office of the Australian Information Commissioner.