australasian society of clinical immunology and allergy

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Policy & Advocacy
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Dear Stephanie,

Re: ASCIA feedback on the draft Australian Government's National Medicine Policy (NMP)

On behalf of the Australasian Society of Clinical Immunology and Allergy (ASCIA) I am writing to the RACP to provide feedback on the draft Australian Government's National Medicine Policy (NMP).

Whist we are pleased to note that this policy aims to set the direction and approach for the availability and use of medicines in Australia to achieve optimal health outcomes, we are concerned that **people with rare and under-recognised diseases conditions**, have been listed on pages 6 (under Equity and Access) and on pages 8-9 (under Equity) almost at the end of the list of priority groups. We believe that people with rare diseases should be listed towards the top of the priority groups, not towards the end of the list.

As stated on pages 8-9, people living with rare and under-recognised diseases often face inequities due to the scientific and technical complexities of data and its collection, and the absence of evidence for the evaluation and subsidisation of treatments for rare conditions.

Prof Connie Katelaris AM recently presented about this issue at the ASCIA 2022 Conference, using the example of primary immunodeficiencies. This is a group of individually rare diseases that have great consequences for patients, causing significant morbidity, affecting the ability to study or work, and in many instances, impacting life span, if they are not receiving appropriate treatment.

Research into these rare immune system disorders has resulted in new drug development that is very targeted, leading to great outcomes, as well as gene therapy for some of these conditions, which can be curative.

Pathways to access new therapies for rare diseases are limited:

- Reimbursement applications for rare disease treatments can take several years, due to clinical trial and health economic data being difficult to obtain, as these rely on large numbers of patients.
- At present, clinical immunology/allergy specialists rely on applications to individual committees within hospitals to allow access to these treatments for patients. This is by no means a guaranteed pathway, and access will vary across states and hospitals.
- More efficient pathways are required, to allow access to treatments for rare diseases, not based on cost effectiveness alone. When there are clear pathogenic pathways or target molecules identified, specific treatments should be accessible for patients affected by that condition, remembering that these are rare conditions with relatively few patients affected.

In summary, for rare diseases the Australian Government's National Medicine Policy should include:

- A more efficient pathway to access new drugs, to limit the delay for Australian patients with rare diseases to access state-of-the-art treatment, for which there is strong evidence for use, based on genetic analysis, target molecule identification and pathway definition; and to accept this as the evidence-base in rare diseases where clinical trial data is exceptionally difficult to obtain and health economic arguments alone are not feasible.
- Consideration for how to manage access to novel therapies such as gene therapy to ensure equity of access for Australian patients with rare diseases.

Yours sincerely,

Jill Smith

ASCIA CEO

Copy:

A/Prof Theresa Cole, ASCIA President

Prof Connie Katelaris AM, Chair, ASCIA HAE Working Party and ASCIA Past President