Response ID ANON-Z8YB-Y4SU-2

Submitted to **Repurposing of Prescription Medicines**
Submitted on **2021-03-30 16:58:51**

How to respond

1 What is your name?

Name:
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2 What is your email address?

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3 What is your organisation?

Organisation:
Rare Voices Australia

4 Which of the following statements best reflect your situation?

I represent a group of patients

5 Can we publish your response?

Yes, I agree my responses can be published:
Yes

Known concerns

6 What are the critical concerns and challenges/barriers to repurposing medicines in Australia?

correct critical concerns:

Rare Voices Australia (RVA) the peak body for Australians living with a rare disease, has communicated many times with the TGA on this issue of repurposing. We are disappointed that despite our clear messaging that this consultation does not seem to reflect many of the key points that RVA has repeatedly raised to the TGA. Repurposing of medicines is an important opportunity to address unmet need in rare disease but reimbursement of repurposed medicines is inequitable, uncertain and unsustainable. This is a systemic issue for rare disease where small patient numbers often result in reduced commercial incentive for pharmaceutical companies to put in an application or extend a listing, and current approval process are inappropriate and inflexible.
Off-label use is presented in this paper as a barrier to repurposing. This is misleading. In reality for rare disease off-label use is an uncertain, inequitable and last-ditch option for patients to access the treatments they need. Rare disease patients access off-label treatments due to lack of accessible treatment options.

Evidence gaps are also presented in this paper as a barrier to repurposing. Again RVA feels this is highly misleading. The consultation paper refers to and "overreliance" on "lower quality evidence" and again links this to 'off-label use'! - again failing to understand the reality facing rare disease patients. HTA prioritises evidence from randomised clinical trials yet these trials are often inappropriate for rare diseases. More flexibility and priority needs to be given to alternate types of evidence for repurposing medicines for rare diseases.

7 Are there additional challenges/barriers to repurposing that need consideration?

additional challenges:

A key barrier that does not seem to be recognised by this TGA consultation is the fact that the issue of repurposing can not just be seen as a TGA issue. To achieve any effective change, the TGA needs to work collaboratively with the PBAC, the OHTA on this issue. It is beyond frustrating and completely ineffective to be asked to provide input to to a systemic problem in such a narrow and fragmented way. As such you could argue that TGA itself is a barrier to addressing this issue by 1) not representing or misrepresenting repeated key messaging from RVA on this issue and 2) persisting with this stand alone approach (ie not recognising the relation of registration with reimbursement pathways) and which fails to respond to the systemic nature of the issue.

Potential options

Option 1. Reduce regulatory burden

8 What would be the functional impact of these options in incentivising medicines repurposing?

functional impact of regulatory burden:

This consultations focuses on adapting existing pathways for a commercial sponsor (usually a pharmaceutical company) making a submission for registration and reimbursement. Companies commonly state it is not commercially viable to pursue registration and reimbursement to repurpose medicine for a rare disease. It is unlikely that incentives, fee reductions and reduced regulatory burden will adequately address these challenges when the patient numbers are so small. The current separate processes for registration and reimbursement are complex, labour and cost intensive and completely outside the capacity of most non commercial parties such as patient groups (particularly rare disease groups) or clinicians seeing a small group of rare disease patients.

9 Are there additional options for the Department to consider to reduce the regulatory or cost burden for repurposing of medicines?

additional feasible options:

There needs to be consideration for the development of an alternate pathway for registration and reimbursement of repurposed medicines for rare diseases that is accessible for non-commercial sponsors.

Option 2. Further support the development of repurposed drugs through information access

10 Would access to data on real word use data lead to more repurposing of medicines? What sources exist and would be useful?

providing data sets:

There is no accurate real world evidence of off-label use. It is not routinely collected and monitored. Access is uncertain and not well monitored. Again I think this question is 'backward' in the context for rare disease. Registration and reimbursement of repurposed medicine in rare disease would actually enable data collection of real world evidence and lead to better care outcomes.

11 Are there other non-commercial datasets that could be obtained that would assist in facilitating repurposing?

additional datasets:

For repurposing for very small patient numbers, processes must prioritise different types of evidence. This could include things such as N=1 clinical trial, evidence from consensus clinical guidelines developed by clinical experts.

Option 3. Actively pursue registration and potential PBAC review of additional indications for medicines

12 What are the main barriers that would lead to sponsor refusal to apply to register a new indication?

barriers for commercial sponsor:

Not commercially viable due to very small patient numbers and lack of confidence that approval process would prioritise different types of evidence.

13 Would there be interest from non-commercial groups to become sponsors to enable registration and reimbursement of repurposed medicines?

interest from non-commercial groups:

There is need for a viable model for non commercial entities such as patient groups or clinicians to instigate an application for both registration and reimbursement of repurposed medicines. This pathway would have to take into account the limited experience and the constraints on time and financial resources in these cohorts.

14 Would particular measures undertaken by the Department (e.g. compelling an application or deeming a new indication) be an effective and feasible mechanism to facilitate repurposing?

extra pharmacovigilance:

This pathway in which one of these could instigate an application and receive support from the Department of Health and TGA would be ideal. This model would require investment into developing capacity of non commercial sponsors to participate in these processes and would require increased transparency around how consumers and other stakeholders can appropriately influence these processes.

Upload extra information

15 Are there any supporting attachments you would like to provide?

additional files:
No file uploaded