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Submitted to Adoption of international scientific guidelines in Australia
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About you

1 What is your name?

Name:
Nicole Millis

2 What is your email address?

Email:
nicole.millis@rarevoices.org.au

3 What is the name of the organisation you represent? (Write 'Individual' if your submission is not on behalf of an organisation)

Organisation:
Rare Voices Australia

4 Where are you located?

VIC

5 Which of the below best describes you? (Select all that apply)

Consumer, Organisation

Guideline on registry-based studies (EMA/426390/2021)

6 Do you support the adoption of this guideline and associated proposed TGA annotation?

Yes, I support adoption

Do you have any relevant information you wish to include?:

Australian HTA processes utilise models that are designed primarily for more common diseases. This presents challenges for reimbursement decisions for medicines/technologies for rare diseases. Smaller patient numbers impact
cost effectiveness, and there is often less clinical evidence available due to the challenges of conducting large-scale clinical trials. This highlights the importance of fit-for-purpose approaches to both research and HTA models for rare diseases. Rare Voices Australia supports the adoption of this guideline as part of addressing current inequities/challenges experienced by rare disease therapies in current HTA processes.

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Appendix 2 to the guideline on the evaluation of anticancer medicinal products in man. The use of patient-reported outcome (PRO) measures in oncology studies (EMA/CHMP/292464/2014)

7 Do you support the adoption of this guideline?

Yes, I support adoption

Do you have any relevant information you wish to include?:

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