Using Patient Perspectives in Health Technology Assessment for Rare Conditions: A Singapore Case Study

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RATIONALE

- **Patient experiences explain what living with the condition and treatment means in real life**

Evidence gaps

Small populations can limit trial data and generalisability.

Lived experience

Patients describe symptoms, uncertainty, daily trade-offs and care burden.

Context for decisions

Input complements clinical and economic evidence, supporting a more balanced assessment.

- Patients and carers have provided their lived experiences about medical conditions and treatments to inform ACE's technical evaluations since May 2022

OBJECTIVE

Pulmonary arterial hypertension (PAH)

- Rare but serious progressive condition
- Fewer than 500 people in Singapore
- Narrowing of blood vessels in the lungs makes the heart work harder
- Symptoms may include breathlessness, fatigue, chest pain, increased heart rate and fainting

What this presentation covers

1. How ACE collects and uses patient input for HTAs, using PAH as a case study

2. Key factors that support patient input from rare disease communities, to inform future patient engagement efforts in HTA

METHODS

Key steps for patients to provide their lived experiences

Call for patient input into ACE's evaluations

Patient organisations distribute the survey to members

CEE collates responses and includes patient input in evaluation report

Patient input summary is published in the technology guidance

CEE provides feedback to patient organisations to close the feedback loop

The process is designed to make participation feasible, transparent and useful for decision-making.

METHODS

Developing the instrument

- In 2024, ACE conducted a technical evaluation of selexipag for the treatment of PAH to inform funding decisions
- To incorporate patients' voices into the HTA evaluation, ACE developed a structured questionnaire to capture lived experiences of patients with PAH, their current treatments, unmet needs and expectations of selexipag

METHODS

Targeted engagement strategy

Channels

- Online survey
- Word document by email
- Distribution to patients by two local patient organisations

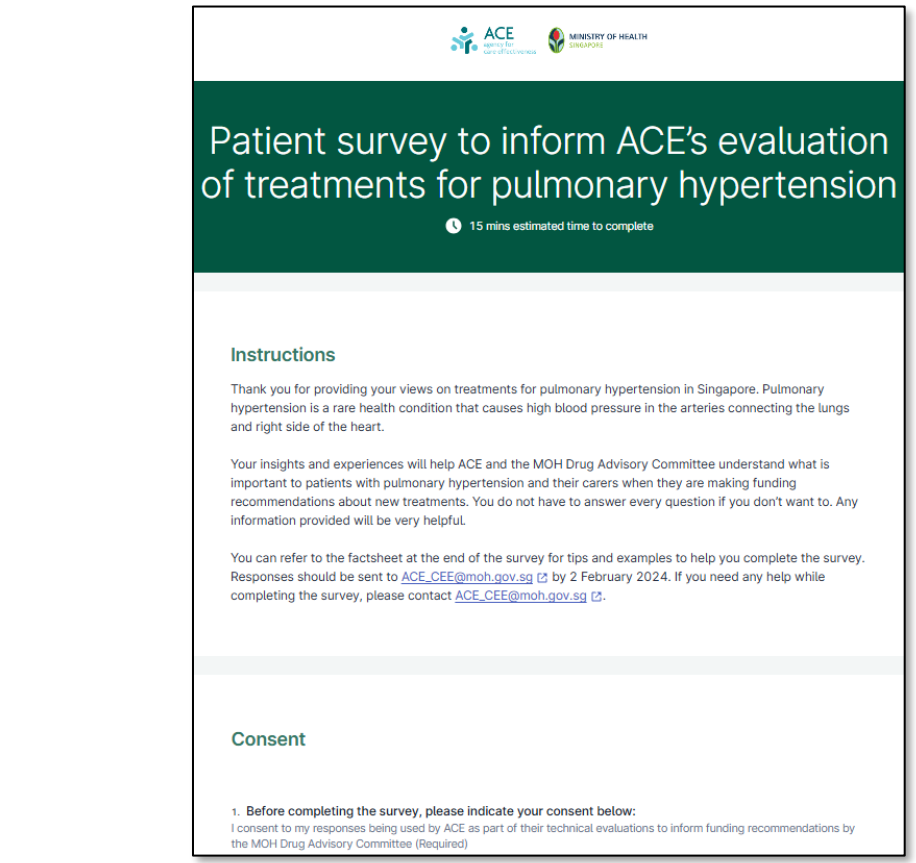
Respondents

- Individual patients
- Carers
- Combined organisation submission

Survey length

- Comprehensive version
- Truncated version

METHODS



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Patient survey to inform ACE's evaluation of treatments for pulmonary hypertension

15 mins estimated time to complete

Instructions

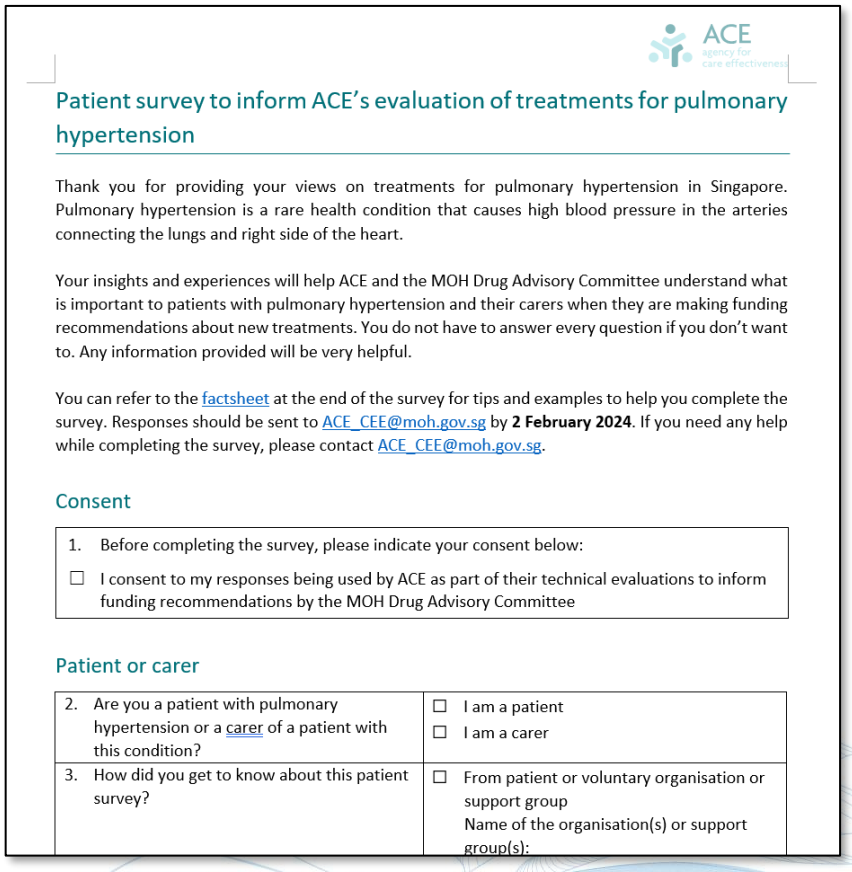
Thank you for providing your views on treatments for pulmonary hypertension in Singapore. Pulmonary hypertension is a rare health condition that causes high blood pressure in the arteries connecting the lungs and right side of the heart.

Your insights and experiences will help ACE and the MOH Drug Advisory Committee understand what is important to patients with pulmonary hypertension and their carers when they are making funding recommendations about new treatments. You do not have to answer every question if you don't want to. Any information provided will be very helpful.

You can refer to the factsheet at the end of the survey for tips and examples to help you complete the survey. Responses should be sent to ACE_CEE@moh.gov.sg by 2 February 2024. If you need any help while completing the survey, please contact ACE_CEE@moh.gov.sg.

Consent

1. **Before completing the survey, please indicate your consent below:**
I consent to my responses being used by ACE as part of their technical evaluations to inform funding recommendations by the MOH Drug Advisory Committee (Required)



ACE agency for care effectiveness

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Patient or carer

2. Are you a patient with pulmonary hypertension or a carer of a patient with this condition?	☐ I am a patient ☐ I am a carer
3. How did you get to know about this patient survey?	☐ From patient or voluntary organisation or support group Name of the organisation(s) or support group(s):

METHODS

Analytical approach

- Patient organisations were given four weeks to respond to the survey

1. **Collate**
   Responses received through patient organisations
2. **Analyse**
   CEE conducts thematic analysis
3. **Incorporate**
   Findings included in HTA report
4. **Close the loop**
   Outcome shared with patient organisations

RESULTS

Participation outcome

- A total of 17 respondents, comprising 15 patients and 2 carers, shared their lived experiences of PAH, including unmet treatment needs and expectations of selexipag

Why this was meaningful

- Contributions added real-world context that is not captured in clinical data alone
- Depth of qualitative insight helped build a fuller picture of the disease burden

RESULTS

Patient responses

Physical health

Breathlessness, fatigue and limitations in everyday activities

Mental / emotional wellbeing

Uncertainty, anxiety and the burden of living with a progressive condition

Work and social life

Reduced ability to work, participate socially and plan activities

Pregnancy and family

Effects on family decisions and reliance on carers

- These insights provided context to the clinical and economic evidence in the HTA report and informed funding deliberations by the Ministry of Health Drug Advisory Committee

RESULTS

Factors that supported meaningful patient input

1	**Partnership with patient organisations is essential**	They can reach small communities and support trust
2	**Prioritise depth of insight**	Qualitative detail can be highly informative and provides critical context when numbers are small
3	**Offer flexible and accessible participation**	Multiple response modes and shorter options reduce burden and consultation fatigue
4	**Capture diverse perspectives**	Symptoms, treatment response and caring needs can vary widely
5	**Patient priorities help decision-makers understand acceptable trade-offs**	Patients can explain what benefits, risks and treatment burdens they may be willing to accept

CONCLUSION

This case study showed that patient perspectives can strengthen HTA even when patient numbers are small.

Complement evidence

Lived experience provides insights not captured in traditional clinical or economic data

Support patient-centred decisions

Input helps decision-makers understand real-world burden and treatment priorities

Provide a model

The approach can support other rare disease communities to engage in HTA

For rare conditions, the goal is not to count voices — it is to understand what those voices reveal.

THANK YOU

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