HTA Policy and Methods Review: Consultation 2 Options Paper Response

Takeda wishes to highlight the importance of recognising medicines for rare diseases in the HTA review

Rare diseases pose unique challenges in HTA that have been recognised in HTA policies and methodologies around the world. The challenges associated with conducting clinical studies, including low patient numbers, treatment outcome heterogeneity and limited understanding of the condition can translate into uncertainty in HTA. This uncertainty must be balanced against the urgent need for effective interventions to improve patient outcomes for people living with a rare disease.

The options paper includes proposals that have potential to enhance speed and equity of access to orphan drugs for Australians living with rare disease. However, the paper lacks detail regarding how these options may be designed with the flexibility to accommodate HTA for orphan drugs, and has the potential for unintended consequences as a result of HTA process and method changes. A more detailed consideration of the implications of these options for rare diseases is required.

Options that may provide benefit for rare disease medicines:

Four options Takeda support to achieve faster access for rare disease medicines are:

- 2.2 Single HTA point of access
- 3.2 Explicit qualitative framework (MCDA or equivalent) for rare diseases
- 3.2 & 4.3 Guidance on including RWE in HTA for rare diseases.
- 4.1 Explore patient level warranties to address population uncertainty.

Four options Takeda supports to improve equity of access for patients living with a rare disease:

- 3.1 Establishing a PICO pathway.
- 1.2 & 3.1 Guidance for early clinician and patient engagement
- 4.1 Establishment of a time-limited/ interim fund
- 2.1 One HTA committee for review of LSDP items (could be PBAC or MSAC)

Unintended consequences or challenges for rare disease medicines from the options paper

- **To ensure equitable access, greater transparency** is needed regarding the **definition of HATV and HUCN** and how this will be applied for orphan drugs and medicines for rare diseases; as well as clarification on **who determines** these categories and at **what stage** in the process they are **decided.** Clear guidance will enable companies to better engage with the HTA process, thereby facilitating faster access to medicines for patients.
- Description of HUCN should be **independent** of regulatory process; and should be **consistent** for repurposed medicines.
- **Clarity on the criteria for each HTA pathway** will require **more transparency** on **acceptable cost-effectiveness** for medicines classified as **HATV, HUCN, LSDP, PBS/CE** and **PBS/CM** (diagram p.41).
- The intent of the **LSDP to fund medicines for ultra-rare diseases** must be retained. An option for MSAC review for LSDP items to link with hospital administration could also be considered.
- Many options are **not mutually exclusive** and so the **sequencing** of **implementation** will impact on the **success** of achieving **faster** and more **equitable** access, and must be carefully considered.

Addressing the challenges of rare diseases in HTA requires a collaborative effort involving stakeholders from various sectors, including patients, healthcare providers, researchers, and policymakers. A flexible and patient-centric approach to HTA is required to facilitate faster access to life-changing treatments for Australians living with rare diseases.

MCDA = Multiple criteria decision analysis, RWE = Real world evidence, HATV = High added therapeutic value, HUCN = high-unmet clinical need, PBS/CE = Pharmaceutical benefit scheme/ cost-effectiveness, PBS/CM = Pharmaceutical benefit scheme/ cost-minimisation, LSDP = lifesaving drugs programme, PBAC = Pharmaceutical benefits advisory committee