Funding of treatments for rare diseases in Singapore

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1 INTRODUCTION

- The Rare Disease Fund (RDF) was established in Singapore in July 2019 to provide long-term, financial support to patients with rare genetic diseases who require high cost treatments.
- It is a national multi-stakeholder charity fund that combines community donations with 3-for-1 government matching, and is intended to be a last-line of support after government subsidies, insurance and other financial assistance.

2 METHODS

- The local prevalence of "rare" and "ultra-rare" conditions was defined in line with overseas rates (≤4 in 10,000 and <2 in 50,000 respectively) to identify patients likely to benefit from the RDF.
- Public healthcare institutions (PHIs) were invited to propose new medicines for inclusion, and each potential topic was prioritised for evaluation by ACE (the national HTA agency) in consultation with the Rare Disease Expert Working Group (RDEG).
- Evaluation, pricing and stakeholder engagement processes were established in line with international best practice. External price referencing from five overseas jurisdictions (Australia, New Zealand, UK, South Korea and Taiwan) was conducted for each medicine (where available) to ensure that local prices proposed by companies were fair.
- Criteria were defined for medicines eligible for inclusion in the RDF and to improve consistency and transparency of decision-making (Fig. 1).
- A voluntary RDF Committee was formed to approve the medicines covered under the RDF based on ACE/RDEG's evaluation, and determine the amount of financial support for each eligible patient according to their needs on a case-by-case basis.
- Workflow processes in the PHIs were developed to assist patients and their clinicians work with medical social workers (MSW) to complete clinical and financial eligibility assessments and apply for funding (Fig. 2).

Medicines supported under the RDF should meet **all** of the following criteria:

- ✓ Medicine is registered by the Health Sciences Authority (HSA) Singapore or a reputed international regulatory authority (Food and Drug Administration (US FDA) and/or European Medicines Agency (EMA)) for the condition assessed (i.e. medicine has proven therapeutic modality);
- ✓ Medicine treats a rare, but clinically defined genetic condition that is chronically debilitating or life-threatening;
  - ✓ There is acceptable evidence that the condition causes a significant reduction in either absolute or relative age-specific life expectancy or quality of life for patients with the condition;
- ✓ There is acceptable evidence that the medicine is likely to substantially extend a patient's lifespan and improve their quality of life as a direct consequence of its use;
- ✓ There is no cheaper alternative option (including non-drug therapy) for the condition;
- ✓ The medicine is not indicated for the treatment of other conditions, or if it is, the cumulative prevalence across all indications still falls within the definition of rare (<1,600 patients across all indications); and
- ✓ The annual cost of the medicine would constitute an unreasonable financial burden on the patient and/or their family or carer.

Medicines should also be fairly priced relative to other countries to be considered for inclusion in the RDF.

Figure 1: Eligibility criteria for RDF medicines

3 RESULTS

- ~2,500 people are estimated to have a rare disease in Singapore. Specific treatments are not available for most conditions.
- High cost medicines that are required to be taken life-long were prioritised for RDF listing. Medicines for rare cancers, and blood products (e.g. for haemophilia) were excluded from evaluation, as there are already other financial mechanisms in place to support patients requiring these treatments.
- 6 medicines for 4 rare diseases were listed in the RDF in 2019 (Tab. 1) in line with specific initiation/continuation clinical criteria.
- None of the treatments were cost effective at the prices proposed, which was expected given the small number of patients requiring treatment. Prices proposed by companies were considered acceptable and fulfilled external price referencing rules.
- Singapore citizens at any PHI who require RDF medicines can apply for financial assistance and will be subject to a review of their clinical and financial eligibility annually. To date, 7 patients have applied for financial assistance through the RDF.
- As more funds are raised, additional medicines will be listed in the RDF; prioritised according to clinical need.

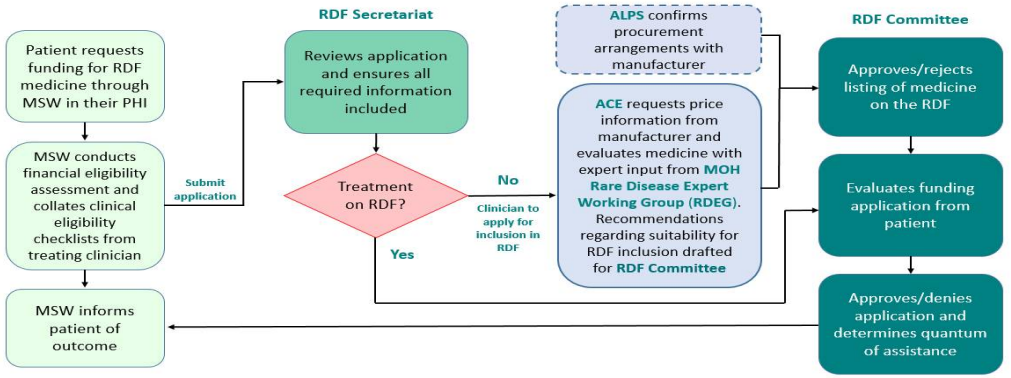

Figure 2: High level process for patient applications for financial support under the RDF

Key: MSW, medical social worker; PHI, public healthcare institution; RDF, Rare Disease Fund; ALPS, agency responsible for national supply chain and procurement in the public healthcare sector; ACE, Agency for Care Effectiveness (national HTA agency of Singapore); MOH RDEG, Ministry of Health Rare Disease Expert Group

Table 1: Medicines listed in the RDF (as of June 2020)

Condition	Number of patients in Singapore	Medicine(s)
Gaucher disease (Type 1 or 3)	2	Imiglucerase, velaglucerase alfa, taliglucerase alfa
Hyperphenylalaninaemia due to tetrahydrobiopterin (BH4) deficiency	4	Sapropterin dihydrochloride
Pompe disease	7	Alglucosidase alfa
Primary bile acid synthesis disorder	1	Cholic acid

4 CONCLUSION

- Wider considerations of disease and treatment experiences from a multi-stakeholder standpoint should be considered to inform RDF listings as evaluation processes evolve.
- There will also be a need to balance the long-term sustainability of the fund with the number of emerging medicines that may require coverage in the future.

References: Further information relating to the Rare Disease Fund in Singapore is available at www.kkh.com.sg/giving/Documents/Rare-Disease-Fund/index.html#clinical-criteria

www.ACE-HTA.gov.sg