ANNEX C

Criteria for inclusion of medicines in the RDF

All of the following criteria must be met for a medicine to be considered for inclusion in the RDF:

i. Medicine is registered by Health Science Authority (HSA) 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);

ii. 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 those with the condition;

iii. 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;

iv. There is no cheaper alternative option (including non-drug therapy) for the condition;

v. 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

vi. The annual cost of the medicine would constitute an unreasonable financial burden on the patient and/or their family or caregiver.

The medicine should also be fairly priced relative to other countries to be considered for listing in the RDF.