立法會
Legislative Council

LC Paper No. CB(2)979/16-17(07)

Ref : CB2/PL/HS

Panel on Health Services

Background brief prepared by the Legislative Council Secretariat for the meeting on 20 March 2017

Policy on and drugs for rare diseases

Purpose

This paper provides background information and summarizes the concerns of members of the Panel on Health Services ("the Panel") on issues relating to the policy on and drugs for rare diseases.

Background

2. According to the Administration, there is currently no common definition of rare disease available worldwide. The health policy in the management of uncommon disorders also varies among countries. Locally, there is no definition of or comprehensive database on rare disease. At present, the Clinical Genetic Service of the Department of Health ("DH") provides clinical diagnosis, counselling and prevention services for those families possibly affected by genetic-related diseases, whereas the Hospital Authority ("HA") provides medical services for patients suffering from uncommon disorders.

3. In providing drug treatments for its patients, HA follows the core values including evidence-based medical practice, rational use of public resources, targeted subsidy and opportunity cost consideration. Since drug treatments for uncommon disorders can be extremely expensive and their efficacy varies among patients under different clinical conditions, HA has set up an independent expert panel, which comprises specialist physicians, paediatricians, clinical pharmacologists and pharmacists, to formulate treatment protocols for specific uncommon disorders and evaluate the benefits of individualized treatments.

4. At present, HA provides drug treatment through enzyme replacement therapy for individual patients with specific lysosomal storage disorders. The drugs in use will be categorized as Special Drugs in the Drug Formulary of HA ("the Formulary")[1] for provision at standard fees and charges for those patients meeting the specific clinical conditions and with specific specialist authorization. In addition to drug treatments, HA provides multi-disciplinary care and other treatments, including rehabilitative care, pain alleviation, surgical treatment and bone marrow transplant, for patients with uncommon disorders where appropriate. From 2008-2009 to 2015-2016, the Government has allocated a total recurrent funding of $75 million in phases to meet the increasing demand and sustain the provision of the ultra-expensive drug treatments for uncommon disorders in HA.

Deliberations of the Panel

5. The Panel discussed issues relating to the policy on and drugs for rare diseases in the context of discussing the Formulary and receiving briefings from the Secretary for Food and Health on the policy initiatives at a number of meetings held between March 2014 and December 2016. The deliberations and concerns of members are summarized in the following paragraphs.

6. Many members considered that the Administration should provide a clear definition and policy on rare diseases to support patients suffering from these diseases and their families. There was also a suggestion of developing a territory-wide data bank for rare diseases to provide a profile of the common types of rare diseases in Hong Kong, so as to foster scientific research and facilitate support for patients with rare diseases.

---

[1] At present, there are around 1 300 drugs listed on the Formulary. These drugs are classified into four categories. The General Drugs and Special Drugs are provided within the standard fees and charges at public hospitals and clinics when prescribed under specified clinical conditions. General Drugs have well-established indications and cost-effectiveness, and are available for general use as indicated by patients with relevant clinical conditions. Special Drugs have to be used under specified clinical conditions with specific specialist authorization. For patients who do not meet the specified clinical conditions but choose to use Special Drugs, they will have to pay for the drugs. Other drugs in the Formulary are self-financed items whereby patients have to purchase at their own expense. These self-financed items are categorized into those with safety net and those without safety net. The former are drugs which are proven to be of significant benefits but extremely expensive for HA to provide as part of its subsidized services. Patients who need these drugs but have financial difficulties may apply for assistance from the Samaritan Fund or the Community Care Fund Medical Assistance Programme to fully or partially cover their expenses on these drugs.

7. According to the Administration, the definition of rare diseases in different countries varied depending on their healthcare systems and situations. HA placed high importance in providing optimal care for all patients while ensuring rational use of public resources. There were currently a number of HA patients suffering from six lysosomal storage disorders, namely Pompe disease, Fabry disease, Gaucher disease, Mucopolysaccharidosis Type I, Mucopolysaccharidosis Type II and Mucopolysaccharidosis Type VI. Enzyme replacement therapy ("ERT"), which provided patient with enzyme that was deficient or defective in their bodies, was one of the treatments for these diseases. Since the efficacy of ERT varies among patients under different clinical conditions and ERT was extremely expensive, the expert panel would assess annually the suitability of individual patients for receiving ERT and the efficacy of such treatment on a case-by-case basis. Six ERT drugs were currently positioned as Special drugs in the Formulary[2]. For applications approved by the expert panel, HA would provide ERT for the patients concerned at standard fees and charges.

8. Members were concerned about the provision of drug treatments for, and the financial burden so incurred by, patients suffering from the rare disease of Paroxysmal Nocturnal Haemoglobinuria ("PNH"). They called on HA to include those ultra-expensive drugs for treating rare diseases (such as Eculizumab for PNH patients) in the Formulary. Members were advised that HA had been actively liaising with the relevant drug suppliers from time to time with a view to formulating a sustainable financial arrangement to support the patients concerned. A mechanism was in place for HA to provide the ultra-expensive drug treatments for individual patients (including PNH patients) at standard fees and charges in emergency situations. HA undertook to update the Panel on its progress of liaison with the drug supplier on the arrangement to provide Eculizumab for PNH patients.

9. In view of the latest development of medical technology, there was a suggestion of providing in the public healthcare system non-invasive prenatal DNA test for screening of uncommon disorders of fetus. According to the Administration, DH and HA had launched in October 2015 an 18-month Pilot Study on Newborn Screening for Inborn Errors of Metabolism ("the Pilot Study") in the Queen Elizabeth Hospital and the Queen Mary Hospital. The first phase of the Pilot Study (from October 2015 to March 2016) would cover 21 Inborn Errors of Metabolism ("IEM"). In the second phase (from April 2016 to March

---

[2] These drugs include Alglucosidase alpha for Pompe disease, Algalsidase beta for Fabry disease, Imiglucerase for Gaucher disease, Laronidase for Mucopolysaccharidosis Type I, Idursulfase for Mucopolysaccharidosis Type II and Glasulfase for Mucopolysaccharidosis Type VI.

2017), the Pilot Study would be extended to cover 24 IEM in total. It was estimated that about 10 000 babies could be screened for the disease each year.

Recent developments

10. At the Panel meeting on 28 January 2017 to receive a briefing from the Secretary for Food and Health on the 2017 Policy Address in relation to health matters, members were advised, among others, that the Community Care Fund would implement a new pilot scheme to provide drug subsidies for eligible patients with uncommon disorders such as PNH. Separately, more than 11 000 babies were screened for IEM under the Pilot Study as at the end of November 2016. DH and HA would extend the screening service to all public hospitals with maternity wards in phases starting from the second half of 2017-2018.

11. The Administration will brief the Panel on the policy on drug treatment of patients with uncommon disorders at public hospitals and clinics, and the proposed Community Care Fund assistance programme for subsidizing eligible patients to purchase ultra-expensive drugs (including drugs for treatment of uncommon disorders) on 20 March 2017.

Relevant papers

12. A list of the relevant papers on the Legislative Council website is in the **Appendix**.

Council Business Division 2
Legislative Council Secretariat
14 March 2017

Appendix

Relevant papers on the policy on and drugs for rare diseases

Committee	Date of meeting	Paper
Panel on Health Services	17.3.2014 (Item III)	Agenda Minutes CB(2)2053/13-14(01)
	19.1.2015 (Item III)	Agenda Minutes
	15.6.2015 (Item V)	Agenda Minutes
	18.1.2016 (Item IV)	Agenda Minutes
	19.12.2016 (Item III)	Agenda

Council Business Division 2
Legislative Council Secretariat
14 March 2017