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Legislative Council Panel on Health Services

Policy on Drugs for Uncommon Disorders

PURPOSE

This paper briefs Members on the policy on drug treatment of patients with uncommon disorders at public hospitals and clinics, and the proposed Community Care Fund (CCF) Programme subsidizing eligible patients to purchase ultra-expensive drugs (including drugs for treatment of uncommon disorders).

BACKGROUND

2. There is no common definition of uncommon disorders worldwide. The definition varies in different countries depending on individual country's healthcare system and situation. Given the difficulty to confirm the number of uncommon disorders cases, the lack of reliable information or data on the causes of such diseases, and the relatively recent discovery of ways to treat some of the diseases, the Hospital Authority (HA) has not defined uncommon disorders. For all patients attending public hospitals and clinics, doctors of the HA will assess their conditions in accordance with established procedures. After diagnoses have been made, doctors will provide the appropriate healthcare treatment for patients based on their clinical conditions and the treatment guidelines.

3. Drug treatment for uncommon disorders can be extremely expensive, and their efficacy varies among patients under different clinical conditions. In Hong Kong, public healthcare services are heavily subsidised by the

Government, and drug treatment is an integral part of healthcare services. The total drug consumption expenditure of the HA increased from $2.19 billion in 2005-06 to $2.68 billion in 2009-10, and further to $4.57 billion in 2015-16, representing about 8.3% of the total expenditure of the HA.

4. The HA has implemented the Drug Formulary since July 2005 with a view to ensuring equitable access by patients to cost effective drugs of proven safety and efficacy through standardisation of drug policy and drug utilisation in all public hospitals and clinics. The development framework of the Drug Formulary is underpinned by core values including evidence-based medical practice, rational use of public resources, targeted subsidy, opportunity cost consideration and facilitation of patients' choice. The HA follows an evidence-based approach in evaluating new drug applications for listing on the Drug Formulary, having regard to the three principles of safety, efficacy and cost-effectiveness, and taking into account relevant factors such as international recommendations and practices, advance in technology, disease state, patient compliance, quality of life, actual experience in the use of drugs as well as the views of relevant professionals and patient groups. The HA will, based on the above core values and principles, introduce new drugs into the Drug Formulary and regularly review the categorisation of drugs listed in the Drug Formulary.

5. The HA has been expanding the coverage of the Drug Formulary in accordance with the established mechanism in order to benefit more patients in the use of safe and efficacious drugs at standard fees and charges in public hospitals and clinics. From July 2005 to January 2017, the HA introduced a total of 188 new drugs into the Drug Formulary, including 22 general drugs, 100 special drugs, 66 self-financed drugs. In addition, 31 self-financed drugs are included in the safety net coverage of Samaritan Fund (SF) and 16 self-financed drugs are included in the coverage of the CCF Medical Assistance Programme. Existing drugs are also repositioned among categories (for example, from self-

financed or safety net category to special drug category, from special drug category to general drug category, etc.) where appropriate, whereas drugs that are obsolete or no longer in use are removed from the Drug Formulary. Moreover, with an additional recurrent allocation of over $860 million from the Government between 2009-10 and 2016-17, the HA has repositioned in phases 21 self-financed drugs (including 6 with and 15 without safety net coverage) as special drugs and widened the clinical applications of 38 therapeutic groups of special drugs in the Drug Formulary.

6. Some of the drugs for treatment of uncommon disorders are ultra-expensive drugs. The Drug Advisory Committee of HA will take into account the principles and considerations set out in paragraph 4 above in evaluating new drug applications for listing on the Drug Formulary. Moreover, the HA has put in place an independent expert panel mechanism to formulate treatment protocols for specific uncommon disorders and evaluated the efficacy of treatment on individual patient. The Government has allocated an additional annual recurrent funding amounting to $75 million to the HA in phases to meet the increasing demand for ultra-expensive drug treatments for uncommon disorders. Currently, ultra-expensive drug treatments for six types of lysosomol storage disorders (namely, Pompe, Gaucher, Fabry and Mucopolysaccharidosis Types I, II and VI) are provided for individual patients at standard fees and charges when the treatments are proven to be of significant clinical benefits to the patients concerned.

7. With the advancement of medical technologies, new drugs including those for treating uncommon disorders appear in the market with prices significantly higher than those currently covered by the SF. While the cost effectiveness and opportunity costs of these drugs have to be taken into consideration in evaluating new drug applications, these drugs are proven to vary greatly in safety, efficacy and clinical responses to different patients.

Moreover, such ultra-expensive drugs are unaffordable for average patients, the criteria currently used in the means test for the eligibility to the SF are not completely applicable, and hence the relevant financial arrangements and sustainability in respect of these drugs need to be explored.

PROPOSED CCF ASSISTANCE PROGRAMME

8. In view of the above concerns on ultra-expensive drugs and the factors (a) to (c) below, the Government and the HA propose a new CCF assistance programme for ultra-expensive drugs (including those for treating uncommon disorders):

(a) The current financial assessment criteria for drug subsidies under the SF and the CCF Medical Assistance Programme (First Phase) are based on the principle of targeted subsidy, i.e. the level of patient contribution to drug expenses depends on the patient's household affordability. However, such criteria are not completely applicable to the ultra-expensive drugs as some patients from middle-income families may have to contribute to or pay for the drug expenses which may cost up to several million dollars a year. The long-term prescription of these drugs will deplete their assets quickly and exert a heavy financial burden on them. Therefore, the financial assessment criteria for ultra-expensive drugs should be modified in this regard;

(b) The HA needs to accumulate more experience in the use of ultra-expensive drugs, with a view to formulating appropriate long-term treatment protocols for patients. HA will keep abreast of international recommendations and practices throughout the process.

(c) There are different arrangements of medical assistance for uncommon disorders in other regions and subsidy for patients is also provided

through specific funding in some regions. HA needs time to evaluate whether Samaritan Fund is suitable to subsidize the relevant drugs.

9. The proposed Programme enables early use of ultra-expensive drugs by needy patients and allows the CCF to exercise its function to fill the gaps in the existing system and create a pioneering effect. In the meantime, adjusted financial assessment criteria will be adopted to test their feasibility and recognition.

Details of the proposed Programme and its operation

(i) Mechanism for selecting specific drugs

10. We suggest that the current drug evaluation mechanism of HA should be adopted to select the specific drugs suitable for the programme for consideration by the CCF Task Force before submitting to the Commission on Poverty (CoP) for final approval.

(ii) Target beneficiaries and application procedures

11. Under the eligibility criteria of SF and the CCF Medical Assistance Programme (First Phase), the applicant must be an "eligible person". Since the drugs funded by the proposed Programme are ultra-expensive, we propose that only Hong Kong permanent residents will be eligible to apply for its use. People studying in Hong Kong or holding work permits who are not Hong Kong permanent residents are not eligible for application.

12. As the safety and efficacy of the ultra-expensive drugs may vary among individual patients according to specific clinical conditions, the attending doctors will refer suitable cases to the expert panel for prudent examination on whether the patients can meet the clinical criteria on drug utilisation. With the

support of the expert panel, the patients can proceed to make applications and will be referred by their attending doctors to medical social workers for means test. The applications of the patients who have passed the means test will be referred to the HA headquarters for approval. Subject to the HA's approval, the hospitals will distribute the drugs to the patients.

(iii) Assessment criteria for the means test

13. The assessment criteria of the proposed Programme for the means test are formulated under the principle of targeted subsidy. Although the assessment criteria for drug subsidies under the SF are not totally applicable to the ultra-expensive drugs, the proposed assessment criteria for the means test will make reference to those for the SF subsidies, plus an annual maximum contribution which is capped at $1 million. In other words, the patient's maximum contribution to drug expenses will be either 20% of the annual disposable household financial resources of their families or $1 million (whichever is the lower) (see **Annex**). The aim is to ensure that middle-class families will not deplete their financial resources quickly for paying the drug fees which can cost up to several million dollars a year, and suffer from significant deterioration in their quality of life.

(iv) Proposed new drug and estimated number of beneficiaries

14. The HA proposes to firstly include Eculizumab, the drug for treating Paroxysmal Nocturnal Haemoglobinuria (PNH), in the proposed Programme for specific patients with high risks who may have serious complications. This new drug is proven effective to relieve the symptoms, reduce the risk of thrombosis complications, for example, stroke and myocardial infarction, intravascular haemolysis as well as the need for blood transfusion. It is estimated that about 10 to 16 patients will apply for the proposed Programme to use Eculizumab in the first 12 months. The estimated maximum amount of the

subsidy for the first 12 months is $67.7 million.

Future Development

15. As the evaluation of the proposed Programme may take a rather long time to complete, the proposed Programme is expected to operate for some time. During its operation, the HA will select suitable drugs on a regular basis according to the existing evaluation mechanism for consideration by the CCF Task Force before submitting to the CoP for final approval.

16. If the adjusted criteria for the means test are considered practicable and acceptable upon review, the Government and the HA will study the feasibility of introducing ultra-expensive drugs into the safety net of the SF or other funds.

17. The above proposal has been supported by the CCF Task Force and will be submitted to the CoP for consideration at its next meeting. If the proposed Programme is endorsed, the HA aims to accept applications starting from August 2017.

18. The HA will continue to keep abreast of international researches and development of health policies in other economies in respect of uncommon disorders with a view to enhancing the Drug Formulary and optimising the support for local patients. HA is open to suggestions of offering sustainable financial assistance to patients with uncommon disorders and will continue to collaborate with drug companies in providing sustainable, affordable and appropriate support for these patients in the long run.

ADVICE SOUGHT

19. Members are invited to note and comment on the proposals as set out in this paper for provision of the ultra-expensive drugs for prompt use by patients in need.

Food and Health Bureau

Hospital Authority

March 2017

Annex

Financial Assessment Criteria

1. With reference to the prevailing financial assessment criteria for HA drug items under Samaritan Fund, the means test for the proposed Programme will take into account the patients' annual disposable household financial resources (ADFR) in assessing their affordability, and the level of patient contributions will be determined based on a sliding scale. The calculation of ADFR is taken as the annual household gross income less the allowable deductions, and plus the disposable capital. Detailed formula is provided as follows:

Annual Disposable Financial Resources (ADFR) =

$$\left(\text{Monthly household gross income}^{[1]} - \text{Monthly allowable deductions}^{[2]}\right) \times 12 + \left(\text{Disposable capital}^{[3]} - \text{Deductible allowance}^{[4]}\right)$$

2. Based on the patient's ADFR and the sliding scale (Table 1), the contribution ratio and amount required from the patient to co-pay the drug expenses will be determined. Patient with lower ADFR will be required to contribute a smaller amount of the drug cost, and enjoy a higher level of subsidy from the Community Care Fund. The maximum contribution ratio will be capped at 20% of the patient's ADFR or HK$1 million (whichever is lower).

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[1] includes salary, pension, financial contributions from children, relatives and friends, income generated from the assets and properties of the patient's household and compensation received. Assistance provided by the Government such as Normal Disability Allowance, Higher Disability Allowance, Old Age Allowance, Old Age Living Allowance and grant / loan received from the Financial Assistance Scheme for Post-secondary Students are excluded from the calculation

[2] include rental or mortgage payment, rates, Government rents, property management fee of the property occupied by the patient's household (total deduction of not more than one-half of the household gross income), salary taxes, personal allowances (Table 2) for the patient's household, child care expenses, provident fund contribution, school fees of children (up to age of 21) who are at secondary level or below (other expenses, such as school activity fees, board & lodging fees, will not be counted as the allowable deductible item) and medical expenses at public hospitals/clinics (other than the drug(s) subsidized by the Samaritan Fund and /or Community Care Fund Medical Assistance Programme (First Phase) and drug payment under this application) for the last 12 months

[3] includes cash owned by the patient's household at the time of the application and such which have been accrued through past savings from any sources or which have just been acquired, investments in stocks and shares, insurance (refer to investment-linked insurance policies and dividend provided by life insurance policies, but cash value under a life insurance policy should not be counted), valuable possessions, property (for example, land, car park and flat owned in Hong Kong and outside Hong Kong), lump-sum compensation and other realizable assets. The first flat (self-owned or rented) resided in together by the patient's household and the tools of trade owned by the patient's household at the time of application are excluded from the calculation

[4] Deductible Allowance (Table 3) is provided when calculating the total value of disposable capital of patient's household. The amount of deductible allowance depends on the patient's household size. If the disposable capital of the patient's household is below the deductible allowance, the amount of deductible will be capped at the disposable capital of the patient's household. It is set with reference to the prevailing asset limit in assessing eligibilities for applications for the Public Rental Housing (PRH). The level of allowance will be regularly reviewed with reference to the PRH's asset limit which is subject to annual review under an established mechanism.

Annex

Table 1: Sliding scale

	Contribution ratio and amount for the proposed Programme		Contribution from patient under prevailing Samaritan Fund assistance for HA drug items
(A) Annual disposable financial resources (ADFR) ($)	**(B) Contribution ratio (%)**	**(C) Maximum annual contribution from patient ($) (C) = (A) x (B)**	
0 – 20,000	**-**	**0**	**0**
20,001 – 40,000	**-**	**1,000**	**1,000**
40,001 – 60,000 #	**-**	**2,000**	**2,000**
60,001 – 100,000	**5**	**3,000 – 5,000**	**3,000 – 5,000**
100,001 – 140,000	**10**	**10,000 – 14,000**	**10,000 – 14,000**
140,001 – 180,000	**15**	**21,000 – 27,000**	**21,000 – 27,000**
180,001 – 2,180,000	**20**	**36,000 – 436,000**	**36,000 – 436,000**
2,180,001 – 4,180,000	**20**	**436,000 – 836,000**	**436,000 – 836,000**
4,180,001 – 6,180,000	**20***	**836,000 – 1,000,000**	**836,000 – 1,236,000**
6,180,001 – 8,180,000	**20***	**1,000,000**	**1,236,000 – 1,636,000**
8,180,001 – 10,180,000	**20***	**1,000,000**	**1,636,000 – 2,036,000**
10,180,001 – 12,180,000	**20***	**1,000,000**	**2,036,000 – 2,436,000**
12,180,001 – 14,180,000	**20***	**1,000,000**	**2,436,000 – 2,836,000**
14,180,001 – 16,180,000	**20***	**1,000,000**	**2,836,000 – 3,236,000**
16,180,001 – 18,180,000	**20***	**1,000,000**	**3,236,000 – 3,636,000**
18,180,001 – 20,180,000	**20***	**1,000,000**	**3,636,000 – 4,036,000**
20,180,001 – 22,180,000	**20***	**1,000,000**	**4,036,000 – 4,436,000**
≥ 22,180,001	**20***	**1,000,000**	**as calculated**

Fixed contribution amount is required from patients whose household's ADFR are $60,000 or below, the formula calculating the applicant's annual contribution in the above table is not applicable

* Capped at a flat contribution rate of 20% or HK$1 million, whichever is lower

Annex

Table 2: Personal Allowances (updated on 28 February 2017)

Number of Household Member(s) (including the Patient)	Total Personal Allowances[#] (HK$)
1 Person	6,150
2 Persons	10,740
3 Persons	15,290
4 Persons	19,820
5 Persons	26,380
6 Persons	24,750
7 or more Persons	27,600

[#] The figures are adjusted every year in line with the Consumer Price Index A, and every five years in line with the latest Household Expenditure Survey conducted by the Census and Statistics Department.

Table 3: Deductible Allowance (updated on 1 April 2016)

Number of Household Member(s) (including the Patient)	Allowance to be deducted from Disposable Capital^ (HK$)
1 Person	242,000
2 Persons	329,000
3 Persons	428,000
4 Persons	500,000
5 Persons	556,000
6 Persons	601,000
7 Persons	643,000
8 Persons	674,000
9 Persons	744,000
10 or more Persons	801,000

[^] The figures are subject to annual review.