LCQ16: Support for patients with rare diseases

Following is a question by the Hon Tang Ka-piu and a written reply by the Secretary for Health, Professor Lo Chung-mau, in the Legislative Council today (June 12):

Question:

Some concern groups have relayed that as the drugs provided by local medical organisations for patients with rare diseases are limited in type and the costs of prescribing such drugs are excessively high for public hospitals, some patients with rare diseases have opted to buy drugs from hospitals in the Mainland, where the prices are only 5 per cent of those at local public hospitals. Regarding the support for patients with rare diseases, will the Government inform this Council:

(1) whether it has compiled statistics on (i) the types of rare diseases already identified at present, (ii) the number of rare diseases that are curable or eligible for pharmaceutical palliative care, and (iii) the number of types of drugs provided by the Hospital Authority (HA) for use on rare diseases and, among them, of those eligible for subsidy application;

(2) whether it has compiled statistics on the types of rare disease drugs which (i) patients have to purchase at their own expenses and (ii) are subsidised by the Government, and set out in the table below a breakdown by monthly expenditure of patients with rare diseases on such drugs (i.e. (a) between $5,000 and $10,000, (b) above $10,000 and up to $20,000, (c) above $20,000 and up to $30,000, (d) above $30,000 and up to $40,000, (e) above $40,000 and up to $50,000, and (f) above $50,000);

Monthly expenditure of patients with rare diseases on relevant drugs	(i)	(ii)
(a)		
(b)		
(c)		
(d)		
(e)		
(f)		

(3) as it is reported that patients with Spinal Muscular Atrophy have to apply for subsidies for therapies with Nusinersen injections and Risdiplam, a specific oral drug for targeted therapy, before the age of □25, after which their subsidy applications will not be approved, whether it knows if the HA

will consider relaxing or removing the age limit for subsidies on those two drugs; if the HA will, of the details; if not, the reasons for that;

(4) given that, according to the Department of Health, the new mechanism for registering New Drugs ("1+" mechanism), which came into effect on November 1 last year, facilitates applications for registration of new drugs for treatment of life-threatening and severely debilitating diseases in Hong Kong, of the total number of applications received and approved under the mechanism since November last year, together with a breakdown by drug name; whether the Government will take drug price as a factor for consideration in vetting and approving applications for drug registration under the "1+" mechanism;

(5) whether the Government has formulated plans to encourage or support patients with rare diseases to go north for medical treatment and drug purchase, such as by provision of transport support, cross-boundary drug delivery services and video consultations; if so, of the details; if not, the reasons for that;

(6) as it is learnt that the Pilot Scheme for Supporting Patients of the Hospital Authority in the Guangdong-Hong Kong-Macao Greater Bay Area enables Hong Kong people with scheduled follow-up appointments at designated Specialist Outpatient Clinics or General Outpatient Clinics of the HA to receive subsidised consultations at the University of Hong Kong-Shenzhen Hospital, whether the service targets of the Scheme include patients with rare diseases; if not, whether the Government will consider including such patients as service targets of the Scheme; and

(7) as there are views that with a relatively small pharmaceutical market size, Hong Kong is no match for the Mainland in bargaining power for bulk procurement of drugs, and that pharmaceutical products are more affordably priced in the Mainland than in Hong Kong, whether the Government will encourage the HA to purchase drugs directly from the Mainland or join the Mainland's drug procurement mechanism, so as to alleviate the pressure on patients in terms of drug expenses?

Reply:

President,

In Hong Kong, public healthcare services are heavily subsidised by the Government, and drug treatment is an integral part of healthcare services. The Government and the Hospital Authority (HA) attach high importance to providing optimal care for all patients, including those with uncommon disorders, based on available medical evidence while ensuring optimal and rational use of public resources.

There is a wide range of uncommon disorders, the majority of which are currently incurable. Although new drugs for treating uncommon disorders appear on the pharmaceutical market from time to time with the advancement of medical technologies, these drugs generally are very expensive and vary

greatly in terms of evidence in both safety and efficacy as well as the clinical response of patients.

The HA has an established mechanism for regular appraisals of registered new drugs or their indications and for review of its Drug Formulary (HADF) and the coverage of the safety net. The review process follows an evidence-based approach, having regard to the safety, efficacy and cost-effectiveness of drugs and other relevant considerations, which include international recommendations and practices as well as professional views, so as to ensure equitable and effective use of public resources in providing optimal treatment for patients.

The HA will also monitor the clinical conditions of individual patients and examine the long-term efficacy of the treatments and the risks involved through an independent expert panel. In appraising new drugs, especially ultra-expensive ones, the HA will also carefully examine the long-term financial sustainability of the treatment options concerned.

In consultation with the HA and the Department of Health (DH), the consolidated reply to the question raised by the Hon Tang Ka-piu is as follows:

(1) and (2) There is no common definition of uncommon disorders worldwide. The HA is progressively building up databases on individual uncommon disorders with a view to providing more optimal treatment to the patients concerned and targeted measures to support them. Currently, the HA has developed databases for 35 uncommon disorders (including 25 metabolic diseases, four neurological diseases and six endocrine diseases) and is consolidating information on patients with relevant disorders. It is expected that the HA will progressively build up a database covering 207 uncommon disorders. Doctors will tag the relevant patient in the record of the next consultation in the HA's Clinical Management System (CMS), and the HA will also arrange necessary information technology system enhancements to the CMS to facilitate doctors' clinical diagnosis and treatment of patients.

In view of the rising demand for ultra-expensive drug treatments for patients with uncommon disorders, the Government has allocated a designated funding to the HA to implement a special drug programme for the treatment of eligible patients with specific lysosomal storage disorders (LSDs), including Pompe disease, Gaucher disease, Fabry disease, Mucopolysaccharidosis (MPS) Type I, MPS Type II, MPS Type IV and MPS Type VI, through enzyme replacement therapy (ERT). The numbers of HA patients who received ERT and the HA's expenditure for the treatment of LSDs through ERT in the past three years (from 2021-22 to 2023-24) are set out in the table below:

Year	Number of HA patients who received the ERT	HA's drug consumption expenditure for the treatment of LSDs ($ million)

2021-22	40	82.5
2022-23	50	99.7
2023-24	59	115.9

In addition, the Government and the HA rolled out in August 2017 a Community Care Fund Medical Assistance Programme, namely "Subsidy for Eligible Patients to Purchase Ultra-expensive Drugs (Including Those for Treating Uncommon Disorders)" (CCF Ultra-expensive Drugs Programme). The HA's expert panels will assess the clinical benefits of drug treatments under these arrangements on a case-by-case basis according to each specific patient's clinical conditions and established treatment guidelines.

The following table sets out the numbers of applications approved and the amount of subsidies granted for drugs under the CCF Ultra-expensive Drugs Programme since its implementation in August 2017 and up to March 31, 2024, (including a drug repositioned from the CCF Ultra-expensive Drugs Programme to the Samaritan Fund (SF) during the said period (Note 1)):

Ultra-expensive drug and clinical indication	**Number of applications approved**	**Amount of subsidies granted ($ million)**
1. Eculizumab for Paroxysmal Nocturnal Haemoglobinuria (Note 1)	73	274.83
2. Eculizumab for Atypical Haemolytic Uraemic Syndrome (Note 2)	6	22.48
3. Nusinersen for Spinal Muscular Atrophy (SMA) (Note 3)	80	183.54
4. Tafamidis Meglumine for Familial Amyloid Polyneuropathy (Note 4)	3	2.09
5. Dinutuximab beta for Neuroblastoma (Note 5)	16	24.97
6. Tisagenlecleucel for B-cell Acute Lymphoblastic Leukaemia (Note 6)	10	19.65
7. Tisagenlecleucel for Diffuse Large B-cell Lymphoma (Note 6)	69	156.64
8. Tafamidis for Herediatary Transthyretin Amyloidosis in Adult Patients with Cardiomyopathy (Note 7)	10	8.71
9. Risdiplam for Spinal Muscular Atrophy (Note 8)	47	74.19
10. Burosumab for X-linked Hypophosphataemia (Note 9)	1	2.85
11. Ravulizumab for Paroxysmal Nocturnal Haemoglobinuria (Note 10)	11	35.74
12. Ravulizumab for Atypical Haemolytic Uraemic Syndrome (Note 10)	1	3.42

13. Onasemnogene Abeparvovec for Spinal Muscular Atrophy (Note 11)	0	0
Total	327	809.11

Note 1: From August 1, 2017, to March 31, 2024, including the number of applications approved and the amount of subsidies granted under the CCF Ultra-expensive Drugs Programme from August 1, 2017, to July 10, 2020, and under the SF since July 11, 2020, after repositioning
Note 2: From November 25, 2017, to March 31, 2024
Note 3: From September 25, 2018, to March 31, 2024
Note 4: From July 13, 2019, to March 31, 2024
Note 5: From December 29, 2020, to March 31, 2024
Note 6: From April 10, 2021, to March 31, 2024
Note 7: From December 4, 2021, to March 31, 2024
Note 8: From December 17, 2022, to March 31, 2024
Note 9: From May 26, 2023, to March 31, 2024
Note 10: From November 11, 2023, to March 31, 2024
Note 11: From December 30, 2023, to March 31, 2024

At present, cases related to undiagnosed disorders, hereditary cancers and genomics and precision healthcare are also covered by the Hong Kong Genome Project. Eligible patients and their family members are recruited at HA hospitals on a voluntary basis with informed consent, and the sequencing analysis results will allow patients to benefit from more precise diagnosis and appropriate treatment.

(3) The HA reviews medication criteria and clinical treatment guidelines in accordance with the principles of evidence-based medical practice and with reference to the clinical and scientific evidence of the drugs, and overseas medication arrangement. The HA Expert Panel on SMA (the Expert Panel) has noted and reviewed the findings of an international preliminary observational study published after adult patients with the disease had been treated with the drug Risdiplam (Evrysdi). The Expert Panel considers that, at present, the relevant scientific data and clinical evidence are still limited, and there is no empirical evidence to show that such drug can bring significant effects to adult patients with the SMA. Having made reference to overseas medication guidelines, international arrangements on disease management and on subsidies for the drug concerned, the Expert Panel considers that it is not appropriate to subsidise adult SMA patients aged above 25 to receive drug treatments through the CCF Ultra-expensive Drugs Programme at this stage.

The current medication arrangements for SMA patients in Hong Kong are similar to those in other regions. The HA's Expert Panel will continue to collect clinical evidence to assess patients' clinical conditions and treatment efficacy to review the relevant parametres. The HA will also continue to subsidise SMA patients whose drug treatments have shown significant medication efficacy through the CCF Ultra-expensive Drugs Programme. Meanwhile, the healthcare team specialising in the care of SMA patients will continue to optimise multi-disciplinary professional healthcare collaboration, with a view to providing patients with comprehensive services, including drug and surgical treatment, palliative care and rehabilitation

services, etc.

(5) The Hong Kong Special Administrative Region (HKSAR) Government has been following the principles of complementarity and mutual benefits to enhance healthcare-related co-operation with various Mainland cities of the Guangdong-Hong Kong-Macao Greater Bay Area (GBA), on the premise of benefitting the development of the healthcare systems of Hong Kong and the Mainland, so as to provide convenience to Hong Kong residents who choose to develop and reside in the Mainland. In particular, the Government supports the healthcare needs of these Hong Kong residents through collaboration with suitable healthcare organisations in the Mainland cities of the GBA. Examples include the Elderly Health Care Voucher Greater Bay Area Pilot Scheme, the Pilot Scheme for Supporting Patients of the Hospital Authority in the Guangdong-Hong Kong-Macao Greater Bay Area (Pilot Scheme), as well as the proposed new function under the five-year plan of eHealth+ to allow members of the public to keep and use their personal medical records from within and outside Hong Kong. The Government is also exploring with suitable GBA healthcare institutions the feasibility of strategic procurement of healthcare services for Hong Kong citizens.

It must be emphasised that, as the HKSAR is a member of the GBA. The Health Bureau, in formulating cross-boundary healthcare measures, would not only focus on meeting the needs of Hong Kong citizens, but would also consider the potential impact of the policies on the social resources and livelihood of people in the Mainland. Indeed, the healthcare needs, relevant laws and regulations, and regulatory regimes for healthcare professions are different in Hong Kong and the Mainland. The HKSAR Government, with the important role of protecting the health of the Hong Kong citizens, will continue to provide quality healthcare services to Hong Kong citizens with no intention to shift such responsibility to the Mainland healthcare system. In this process, the Government would also explore cross-boundary healthcare measures under the premise that these measures are feasible and mutually beneficial, with a view to providing additional and convenient access to and choices of healthcare services for Hong Kong citizens who choose to develop and reside in the Mainland.

(4) and (7) Under the principle of "one country, two systems" and with the strong support of the nation, Hong Kong has established a drug regulatory regime that is highly compatible with international standards, as well as an internationally recognised professional medical training system and an extensive and standardised medical data system.

To better leverage the medical strengths of the HKSAR, "The Chief Executive's 2023 Policy Address" announced that the Government will progress towards the long-term objective of establishing an authority that registers drugs and medical devices (medical products) under the "primary evaluation" approach, i.e. to directly approve applications for registration of drugs in Hong Kong based on clinical trial data without relying on registration approval from other drug regulatory authorities, and start approving applications for registration of medical devices. This will help accelerate the clinical use of medical products to enhance the level of healthcare,

foster the development of industries relating to the research, development and clinical trials of medical products, developing Hong Kong into an international health and medical innovation hub.

The HKSAR Government needs to give careful consideration to matters relating to the development of medical products (including the HA's drug procurement strategy) in order to maintain an independent regulatory regime for medical products while not compromising Hong Kong's institutional strengths, the long-term objective of establishing an authority that registers medical products under the "primary evaluation" approach, the future development of Hong Kong's healthcare system and services, and the recognition of healthcare technologies and clinical research by both the Mainland and the international community.

In purchasing drugs for the provision of optimal treatment to patients, the HA attaches great importance to patients' safety, while the quality and safety of drugs have all along been its prime consideration. The HA has put in place a robust drug procurement mechanism to purchase pharmaceutical products that meet the quality requirements through various channels, so as to ensure the safety, quality and efficacy of drugs and safeguard patients' health. Meanwhile, the HA has also been introducing market competition through centralised tendering or quotation procedures to achieve economies of scale. As the HA's centralised bulk procurement covers all hospitals and clinics under its management, the quantity of drugs purchased is relatively large, conferring upon the HA a certain degree of bargaining power in negotiating drug prices with pharmaceutical companies.

In general, the HA currently achieves cost-effectiveness at a reasonable level through the centralised bulk procurement model. With regard to drugs that are specially used or consumed in small quantities (such as drugs used to treat uncommon genetic disorders), the HA will continue to explore different cost-effective strategies to provide patients with drugs that meet the safety and quality requirements.

The HA has been closely monitoring market developments and maintaining communication with different stakeholders to promote diversification of drug supply, with a view to achieving efficient and cost-effective use of resources. From time to time, the HA also conducts mutual visits to and exchanges with relevant Mainland experts on issues of common concern, so as to facilitate the promotion of co-operation between and development of the two places through mutual learning of experience. The differences in registration systems, pricing mechanisms for pharmaceutical manufacturers, and customs mechanisms of drugs between Hong Kong and the Mainland imply that drug procurement procedures of the two places are not entirely compatible. Consequently, direct comparisons between the relevant arrangements cannot be made. As previously stated, it is important to ensure that the inherent strengths of Hong Kong's drug regulatory regime are not compromised when considering the HA’s drug procurement strategy.

It is worth noting that, to achieve the long-term development of the authority that registers medical products under the "primary evaluation"

approach, the "1+" mechanism announced in "The Chief Executive's 2023 Policy Address" came into effect on November 1, 2023. Under the newly established "1+" mechanism, applications for registration of new drugs in Hong Kong that are beneficial for treatment of life-threatening or severely debilitating diseases, and are supported with local clinical data and scope of application recognised by local experts, are only required to submit approval from one reference drug regulatory authority (instead of two originally). Under the "1+" mechanism, the relevant requirements for local clinical data and recognition by experts for application for registration (i.e. the "+" under the "1+" mechanism) will continue to ensure that all drugs approved for registration fulfil the stringent requirements of safety, efficacy and quality. It will also strengthen the local capacity of drug evaluation and enhance the developments of relevant software, hardware and expertise.

Since the commencement of the "1+" mechanism on November 1, 2023, till May 31, 2024, two new drugs for treating cancer have been approved. They are oral targeted drugs in different dosages for treating metastatic colorectal cancer, for treatment of patients for whom conventional chemotherapy has been ineffective or inapplicable. Besides, the DH has received over 210 enquiries from around 70 pharmaceutical companies. Several of these companies have expressed interest in applying for registration under the "1+" mechanism. Applications would be submitted once the necessary information is ready.

Since the implementation of the "1+" mechanism, the HA has been reviewing and exploring ways to enhance the effectiveness of updating the HADF in a proactive manner, and to shorten the time required for introducing new drugs, including them in the safety net or as Special drugs. The HA also encourages drug manufacturers or suppliers to apply for registration in Hong Kong for unregistered drugs that are in demand. Through the "1+" mechanism, the number of drugs successfully registered in Hong Kong will increase to provide patients and clinicians with more choices of drugs.

(6) As mentioned above, the HKSAR Government is committed to protecting the health of Hong Kong citizens and will continue to provide them with quality healthcare services. The implementation of cross-boundary healthcare measures is mainly intended to provide Hong Kong residents who are developing and living in the Mainland with an additional convenient access to and choice of healthcare services. Among them, the Government launched the Pilot Scheme on May 10, 2023 by making reference to the experience gained from the special support scheme during the COVID-19 epidemic, so that patients with scheduled follow-up appointments at designated Specialist Out-patient Clinics (SOPCs) or General Out-patient Clinics (GOPCs) of the HA can receive subsidised consultations at the University of Hong Kong-Shenzhen Hospital. Earlier, the Government announced the extension of the Pilot Scheme for one year till March 31 next year. There is no common definition of uncommon disorders, and their treatment may involve a range of specialist services. The scope of subsidised consultation services under the Pilot Scheme covers major SOPC and GOPC services provided by the HA, namely anaesthesiology (pain clinic only); cardiothoracic surgery; clinical oncology; ear, nose and throat; eye; gynaecology; internal medicine; neurosurgery; obstetrics; orthopaedics and traumatology (orthopaedics); paediatrics, and surgery. The Pilot Scheme

patients may consult the above specialties as needed.