LCQ21: Rare diseases

Following is a question by the Hon Leung Che-cheung and a written reply by the Secretary for Food and Health, Professor Sophia Chan, in the Legislative Council today (February 24):

Question:

It is learnt that as the costs of developing drugs for rare diseases are high but the market is small, the drugs for such diseases are extremely expensive or even non-existent. Regarding rare diseases, will the Government inform this Council:

(1) whether it will consider afresh laying down a definition for "rare diseases"; if so, of the details;

(2) as the Government indicated in June last year that it was planning to progressively develop with the Hospital Authority databases for individual uncommon disorders, of the progress of the relevant work; the diseases for which databases have been/will be developed, and the respective drugs for treating such diseases (i) which are registered in Hong Kong and (ii) whose registration process is underway;

(3) whether it knows the respective numbers of applications for subsidies made to (i) the Samaritan Fund and (ii) the Community Care Fund Medical Assistance Programmes for treating rare diseases which were received and approved by the authorities in each of the past three years, with a breakdown by name of disease; the average amount of subsidies approved for each successful applicant in respect of each type of diseases;

(4) of the long-term strategies to support patients suffering from rare diseases in the following aspects: laying down a definition for such diseases, conducting clinical research, introducing or manufacturing drugs, developing databases, and stepping up public education and publicity; and

(5) as it has been reported that on the Mainland, there are currently over 20 million patients suffering from rare diseases and 61 drugs available for treating such diseases, whether the Government has plans to collaborate with the Mainland authorities in respect of the research on and treatment of such diseases; if so, of the details; if not, the reasons for that?

Reply:

President,

In consultation with the Department of Health (DH) and the Hospital Authority (HA), my reply to the various parts of the question raised by the Hon Leung Che-cheung is as follows:

(1) At present, there is no common definition of rare diseases/ uncommon

disorders worldwide. Same with other regions, Hong Kong makes arrangements on how to treat various diseases depending on the characteristics of its healthcare system and local situation. The optimal treatment for a patient hinges on professional judgement, the seriousness (not just rarity) of the disease, and the availability of clinical facilities and resources, etc.

If we lay down a definition of rare diseases, it is difficult to determine the patients' appropriate treatment and support solely based on the prevalence rate. The other more important considerations (including the severity of the diseases) may also be neglected. This may deviate from addressing the specific clinical needs of individual patients.

(2) to (4) The Government and the HA highly values provision of sustainable, affordable and optimal treatments and care for all patients (including those with uncommon disorders). Currently, mechanisms have been put in place to provide support for patients with uncommon disorders in various aspects, including clinical diagnosis, multi-disciplinary care and rehabilitation services, introduction of new drugs, as well as subsiding drug treatments.

To further support patients with uncommon disorders, the Government and the HA plan to implement progressively a series of targeted measures. These measures include developing databases for individual uncommon disorders (e.g. spinal muscular atrophy and inborn errors of metabolism covered under the existing newborn screening programme) starting from 2021-22 to facilitate clinical diagnosis and treatment; enhancing public awareness of such disorders through the HA's Smart Patient Website; strengthening support for patients with uncommon disorders through the safety net mechanism; reviewing manpower support and deploying resources to help meet the needs of patients and promote technological development and clinical research relating to uncommon disorders. We also plan to further refine the means test mechanism of the Samaritan Fund (SF) and the Community Care Fund (CCF) Medical Assistance Programmes in the first half of 2021.

The Government and the HA will continue to work closely to discuss, formulate and review the policy support for patients with uncommon disorders, while maintaining communication with stakeholders (including patient groups) to keep reviewing and enhancing relevant mechanisms and measures for supporting such patients.

On drug registration, according to the Pharmacy and Poisons Ordinance (Cap. 138), pharmaceutical products should meet the criteria of safety, efficacy and quality, and be registered with the Pharmacy and Poisons Board of Hong Kong (the Board) before they can be sold or distributed in Hong Kong. The Government has introduced various measures to expedite the registration of pharmaceutical products in recent years. Since 2015, legislative amendments relating to new chemicals or biological entities (NCEs) could be made via the negative vetting procedure. The Board also implemented the Enhanced Procedures for Registration of New Drugs in 2018, under which the time required for processing application for registration of pharmaceutical products is generally shortened by two to three months. As at February 2021, the DH had handled 68 pharmaceutical products containing NCEs since the

implementation of the Enhanced Procedures.

On drug subsidy, the HA supports needy patients (including those with uncommon disorders) to receive drug treatment through the recurrent funding from the Government, the SF and the CCF Medical Assistance Programmes.

Currently, the HA makes use of the designated funding from the Government to provide enzyme replacement therapy under a special drug programme for treatment of eligible patients with specific lysosomal storage disorders.

Taking into account the increasing demand for ultra-expensive drug treatments for uncommon disorders, the Government and the HA introduced a CCF Medical Assistance Programme in August 2017 to provide subsidy for eligible patients to purchase ultra-expensive drugs (including those for treating uncommon disorders) (the CCF Ultra-expensive Drugs Programme). The HA's Expert Panels will assess the clinical benefits of drug treatments under the relevant arrangement on a case-by-case basis according to the clinical conditions of individual patients and the established clinical guidelines. The HA will also liaise with pharmaceutical companies on providing risk sharing or capping programmes to facilitate early access to drug treatment for specific patients.

The following table sets out the number of applications approved and the amount of subsidies granted for the drugs under the CCF Ultra-expensive Drugs Programme since its implementation in August 2017 (as at December 31, 2020):

Ultra-expensive drug and clinical indication	Number of applications approved	Amount of subsidies granted ($million)
1. Eculizumab for Paroxysmal Nocturnal Haemoglobinuria (Note 1)	41	161.39
2. Eculizumab for Atypical Haemolytic Uraemic Syndrome (Note 2)	3	11.04
3. Nusinersen for Spinal Muscular Atrophy (Note 3)	31	85.35
4. Tafamidis for Familial Amyloid Polyneuropathy (Note 4)	2	1.72
5. Dinutuximab beta for Neuroblastoma (Note 5)	–	–
Total	77	259.50

Note 1: From August 1, 2017 to December 31, 2020. The drug and specified clinical indication have been repositioned to the SF safety net since July 11, 2020. The application statistics includes the applications approved under the SF since that date.
Note 2: From November 25, 2017 to December 31, 2020
Note 3: From September 25, 2018 to December 31, 2020
Note 4: From July 13, 2019 to December 31, 2020

Note 5: From December 29, 2020 to December 31, 2020

(5) The HA has all along been keeping abreast of clinical evidence and technological development on treatment options for uncommon disorders worldwide, and has exchanges with overseas and Mainland experts on issues of common concern from time to time, so as to learn from each other's experiences and promote service development.

The HA has an established mechanism for regular evaluation of new drugs and review of the coverage of the safety net. Based on scientific and clinical evidence, the process evaluates the safety, efficacy and cost-effectiveness of drugs, taking into account relevant considerations, such as the views of professionals and patient groups, so as to procure drugs that meet the statutory requirements and relevant quality standards for patients' use, and to ensure equitable and effective use of limited public resources in the provision of appropriate treatment and support for patients.

The HA will continue to pay close attention to international medical researches on uncommon disorders and the development of healthcare policies on uncommon disorders in other regions. It will also review the Drug Formulary through the established mechanism and include suitable self-financed drugs in the scope of subsidy under the safety net so as to benefit more patients in need.