LCQ20: Policies and measures on rare diseases

Following is a question by the Hon Elizabeth Quat and a written reply by the Secretary for Food and Health, Professor Sophia Chan, in the Legislative Council today (May 5):

Question:

Regarding the policies and measures on rare diseases, will the Government inform this Council:

(1) as the Chief Executive (CE) indicated in the 2019 Policy Address that, in order to strengthen the support for patients with uncommon disorders, the Government planned to implement progressively a series of targeted measures, which included developing databases for individual uncommon disorders, enhancing public awareness of such disorders, strengthening the support of drug treatment for patients with such disorders through the Samaritan Fund and Community Care Fund Medical Assistance Programmes (the two Medical Assistance Programmes), reviewing the manpower support and deploying resources to help take care of the needs of patients, and promoting relevant technological development and clinical research, of the specific details, implementation timetables and performance indicators of the various measures;

(2) whether it will set up a steering committee on strategies for rare diseases to be tasked with formulating relevant strategies and plans as well as coordinating the work on implementing the various measures mentioned in (1); if so, of the details; if not, the reasons for that;

(3) whether it will launch a scheme of short-term trial of drugs for rare diseases under which (i) a panel, to be formed by clinical experts and representatives of the Hospital Authority (HA), patient groups and pharmaceutical companies, is to be tasked with selecting drugs for free trials by patients and drawing up relevant details (such as clinical criteria, trial periods and indicators of curative effect), and (ii) patients whose conditions have substantially improved after the end of the trial period are to be subsidised, through the existing drug treatment support mechanism, to continue the medication; if so, of the details; if not, the reasons for that;

(4) whether it knows the respective average, longest and shortest time taken by the authorities in each of the past three years for vetting and approving the applications under the two Medical Assistance Programmes; if such figures are not available, whether it will compile such statistics; of the measures in place to enhance the efficiency of vetting and approving applications;

(5) whether it knows, in respect of each type of drugs for rare diseases, (i) the total number of patients receiving subsidies under the two Medical

Assistance Programmes and, among them, the respective numbers of those receiving full and partial subsidies, (ii) the average amount of subsidy received by each subsidised patient, (iii) the average amount of drug cost contributions made by each subsidised patient, and (iv) the total subsidy amount, in each of the past three years;

(6) as CE indicated in the 2020 Policy Address that the Government would, in accordance with the established mechanism, continue to increase the drugs covered by the two Medical Assistance Programmes and relax the clinical criteria for inclusion of drugs, of the details and latest progress of the relevant work;

(7) as HA indicated in September last year that it had, through the annual plan mechanism, sought additional resources from the Government for taking measures to cope with uncommon disorders, of the amount of the relevant funding and a breakdown by the uses; whether it has assessed if the additional resources concerned are sufficient for the provision of appropriate support for patients of rare diseases on a long-term basis; and

(8) given that the Hong Kong Genome Institute, which is wholly owned by the Government, is implementing a large-scale genome sequencing project called "Hong Kong Genome Project" (HKGP), the pilot phase of which is focusing on those patients with undiagnosed disorders and hereditary cancers as well as their family members, of the following details of the pilot phase of HKGP: (i) whether any rare diseases have been included in HKGP (if so, the names of the diseases), (ii) the progress of recruitment of patients and the screening arrangements, and (iii) the procedure for sample taking and genome sequencing?

Reply:

President,

My reply to the various parts of the question raised by the Hon Elizabeth Quat is as follows:

(1), (2) & (7) The Government and the Hospital Authority (HA) highly value the provision of sustainable, affordable and optimal treatment and care for all patients (including those with uncommon disorders) and have been working closely to discuss, formulate and review the policy support for patients with uncommon disorders, while maintaining communication with relevant stakeholders including patient groups to keep reviewing and enhancing relevant mechanisms and measures. Currently, mechanisms have been put in place to provide support for patients with uncommon disorders in various aspects, including clinical diagnosis and assessments, multi-disciplinary care and rehabilitation services, introduction of new drugs, as well as subsiding drug treatment. We therefore do not see a need to set up a steering committee on strategies on top of existing mechanisms.

To strengthen support for patients with uncommon disorders, the Government and the HA are progressively rolling out a series of measures. For

instance, the Government has increased the recurrent funding for the HA by $25 million from 2021-22 onwards to support the Hong Kong Children's Hospital (HKCH) in providing more comprehensive services to patients with uncommon disorders. These include gradually increasing the manpower for clinical genetic service and the number of relevant clinical and supporting staff; launching a case manager programme for the co-ordination of patients' treatment and care; boosting the capacity of genetic and genomic testing service to enhance the capability to diagnose uncommon disorders; developing databases for individual uncommon disorders (including spinal muscular atrophy as well as inborn errors of metabolism covered under the existing newborn screening programme in the beginning phase) to facilitate clinical diagnosis and treatment; and establishing information platforms (e.g. by utilising the HA's Smart Patient Website) to raise the awareness of uncommon disorders among the public and healthcare professionals. These measures will be taken forward by the HKCH progressively from 2021-22.

Besides, the Government will continue to deploy resources to promote scientific development and clinical research relating to uncommon disorders, which includes the provision of funding for new related research projects under the Health and Medical Research Fund. These projects will commence progressively.

On drug subsidy, we have further refined the means test mechanism of the Samaritan Fund (SF) and the Community Care Fund (CCF) Medical Assistance Programmes in late April 2021, which includes modifying the calculation of annual disposable financial resources and the validity period of financial assessment for recurrent applications.

(3), (5) & (6) Provision of suitable drug treatment is one of the important aspects of support for needy patients (including those with uncommon disorders). With the advancement of medical technology, the HA has been keeping abreast of international developments in the medical field and new drugs introduced into the pharmaceutical market from time to time. The HA has an established mechanism for regular appraisal of registered new drugs or their clinical indications and review of its Drug Formulary (HADF) and the coverage of the safety net with the support of multiple expert panels of different specialities, in order to cater for prevailing and evolving service needs. 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 is providing drug treatment for needy patients (including those with uncommon disorders) through the recurrent funding from the Government, the SF and the CCF Medical Assistance Programmes. On the other hand, the HA will, based on service needs, liaise with pharmaceutical companies concerned from time to time to discuss and formulate risk sharing or capping programmes for individual suitable self-financed drugs. Moreover, the HA will liaise with pharmaceutical companies on providing special or compassionate programmes, having regard to the exceptional circumstances and clinical needs

of specific individual patients, so as to facilitate their early access to drug treatment. The HA will also look into the long-term arrangements for drug treatment to patients with specific uncommon disorders.

On increasing the drugs covered by the SF and the CCF Medical Assistance Programmes and relaxing the clinical criteria for existing drugs, the HA's Drug Management Committee (DMC) will regularly call for submissions from clinicians on self-financed drugs proposed for inclusion into the safety net. Upon professional deliberation by the DMC, the list of drugs recommended for inclusion into the safety net will undergo governance approval process by relevant committees.

As at March 2021, the numbers of drugs covered by the SF and the CCF Medical Assistance Programmes were 51 and 37 respectively. The number of drugs included in the safety net over the past three years is as follows:

Number of drugs included (Note)	2018-19	2019-20	2020-21
SF	6	10	9
CCF Medical Assistance Programmes	10	4	11

Note: Including new drugs repositioned from the CCF Medical Assistance Programmes to the SF, as well as those which are originally covered by either the SF or the CCF Medical Assistance Programmes and subsequently introduced to the other source of funding for different clinical indications.

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 statistics of approved applications for drugs covered by the Programme since its implementation is set out at Annex.

The HA will formulate clinical treatment criteria for drugs to be included in the HADF or the safety net based on the principle of evidence-based medical practice, published scientific research and clinical data as well as international practices. Review of drugs listed on the HADF and the safety net as well as related clinical indications and clinical treatment criteria is an ongoing process driven by evolving medical evidence, the latest clinical developments and market dynamics.

The HA will continue to keep abreast of the latest developments of clinical and scientific evidence, listen to the views and suggestions of patient groups, and review the HADF and the scope of subsidy under the safety net through the established mechanism so as to benefit more patients in need.

(4) The HA does not keep information on the required time from submission of application by an applicant to completion of vetting process for the SF or the CCF Medical Assistance Programmes. Nonetheless, upon receipt of referrals

from the doctors concerned as well as the required information or documents from the patients, medical social workers will process the applications as soon as possible to provide timely assistance for needy patients.

Some cases might take longer to process as patients or household members have not submitted all required documents. As such, the HA has stepped up publicity to remind patients or applicants of the information and supporting documents required for processing their applications. Video clips about the application procedures, points to note and post-approval check mechanism of different types of medical assistance are being produced to enhance public understanding of how to apply for assistance under the SF and the CCF Medical Assistance Programmes. Meanwhile, the HA is developing a mini mobile application in "HA Go" to facilitate communication with patients on the financial assessment as well as applicants' enquiry on application status and funding utilisation.

Moreover, measures taken by the Government and the HA in recent years to enhance the means test mechanism of the SF and the CCF Medical Assistance Programmes have also helped streamline the application process. For example, since early 2019, the definition of "household" adopted in financial assessment has been revised to cover only core family members living under the same roof and having direct financial connection with the patient concerned. Statistics have shown that the number of drug subsidy applications with household size of one to two persons increased by about 30 per cent in the first 12 months after the implementation of the enhancement measures. In addition, further refinements have been implemented since late April 2021, including extending the validity period of the financial assessment of the first application from recurrent applicants to 18 months on the condition that the patient contribution is not more than $2,000, as well as waiving the requirement for all applicants to submit financial documents if they have been referred a second application within one to two months after the first application.

The Government and the HA have sought to streamline and expedite the processing of subsidy applications by simplifying the application procedures, stepping up publicity, as well as enhancing the transparency of both the application and vetting procedures. We will also continue to study the issues relating to drug subsidy applications with a view to providing appropriate support for needy patients.

(8) Under the pilot phase of the Hong Kong Genome Project (HKGP), around 5 000 whole genome sequencing will be performed, which will cover cases of undiagnosed genetic diseases and hereditary cancers, many of which involve patients with uncommon disorders. The Hong Kong Genome Institute is proactively discussing with the three partnering centres at the HKCH, the Li Ka Shing Faculty of Medicine of the University of Hong Kong and the Faculty of Medicine of the Chinese University of Hong Kong on the recruitment of participants, and the projects such as testing and genetic counselling. According to the current progress, recruitment of patients for the HKGP will start later this year.