Motion Debate on "Enacting legislation to protect the rights and interests of rare disease patients" at Legislative Council Meeting of 23 January 2019

Progress Report

At the Legislative Council meeting of 23 January 2019, the motion on "Enacting legislation to protect the rights and interests of rare disease patients" moved by Dr Hon Fernando CHEUNG as amended by Prof Hon Joseph LEE and Dr Hon Elizabeth QUAT (the Motion) was passed. The wording of the Motion is at Annex. This report sets out the latest progress of the Administration's work on the provision of services and support for patients with rare diseases (described as uncommon disorders by us).

SUPPORT FOR PATIENTS WITH UNCOMMON DISORDERS

2. It has been the long-established healthcare policy of the Government to strive to provide suitable care and treatment for all patients. Clinicians of the Hospital Authority (HA) formulate and adjust the treatment protocol for individual patients having regard to their clinical conditions and needs. In the process of delivering public healthcare services, the Government and HA are cognizant of and highly concerned about the challenges encountered by patients with uncommon disorders in their disease journey, such as challenge in diagnosis, the availability of cure and the high cost of treatment. In view of these challenges, the Government and HA have adopted a pragmatic approach in devising targeted measures to support patients with uncommon disorders, with emphasis on the provision of multi-disciplinary care.

3. Although there is no specific legislation on policy for uncommon disorders in Hong Kong at present, the Government and HA are highly attentive to the needs of patients with uncommon disorders. The comprehensive support for such patients under various mechanisms is set out below.

Multi-disciplinary care

4. The multi-disciplinary team of HA, comprising doctors, nurses, pharmacists, clinical psychologists, physiotherapists, occupational therapists and medical social workers, provides comprehensive services ranging from newborn screening, clinical diagnosis, medical and surgical treatment to palliative care and rehabilitative services for patients with uncommon disorders having regard to their clinical and physical needs. The services will be further enhanced with the phased service commissioning of the Hong Kong Children's Hospital (HKCH) since 18 December 2018. HKCH will serve as the tertiary referral centre for complex, serious and uncommon paediatric cases to offer multidisciplinary management and intensive care. Following the commencement of HKCH's specialist outpatient services on 18 December 2018, the inpatient services have begun since 27 March 2019. Patients suffering from relevant diseases, along with healthcare teams and medical equipment, are being translocated gradually from regional hospitals to HKCH.

Facilitation of diagnosis

5. We see the importance of genetic service in enabling timely diagnosis, given that many uncommon disorders are caused by genetic or congenital disorders. Over the past 30 years, the Clinical Genetic Service (CGS) of the Department of Health (DH) has been providing screening programme for certain inborn error of metabolism, as well as diagnostic and genetic counselling services for patients and families affected by genetic diseases and uncommon disorders. Up to now, the CGS has provided service to over 38 000 families, with over 1 500 new patients/families referred every year.

6. To enhance our genetic services, the CGS will move to HKCH in late 2019 so as to integrate the advanced equipment, expertise and relevant support in genetic screening. With a view to facilitating the diagnosis of genetic diseases, HKCH will keep abreast of the technological development and support advanced genetic tests such as non-invasive prenatal test for chromosomal abnormality.

7. The Hong Kong Genome Project (HKGP), as announced by the

Chief Executive in her Policy Address in 2018, would be another platform to strengthen our clinical diagnosis. The HKGP is a large-scale genome sequencing project to promote the clinical application and innovative scientific research of genomic medicine. We expect that the HKGP would enhance the diagnostic rate of uncommon genetic disorders, thereby relieving the burden generated by "diagnostic odyssey" to patients, their families and the healthcare system. The project will also enable more personalised clinical management and facilitate the reproductive decisions of parents whose children have uncommon genetic diseases.

8. We consulted the Panel on Health Services of the Legislative Council (the Panel) on the directions of the HKGP on 21 January 2019, and obtained the Panel's support for the proposal. In addition, the Financial Secretary has announced in the Budget this year that $1.2 billion would be allocated to establish the Hong Kong Genome Institute and take forward the HKGP, under which 40 000 to 50 000 whole genome sequencing will be performed in the next six years. The Government will continue to work with the Steering Committee on Genomic Medicine and the relevant expert group to discuss the project details.

Introduction of new drugs

9. The Government and HA place high importance on providing suitable and affordable drug treatment to patients with uncommon disorders. Established mechanisms and subsidy programmes are put in place to evaluate new drugs and provide financial assistance to patients in need, including patients with uncommon disorders. In view of the public concern over the lead time for introducing new drugs in Hong Kong, the Government and HA have been making strenuous efforts to enhance the existing mechanisms, particularly taking into account the possible difficulties faced by patients with uncommon disorders.

10. In respect of the registration of new drugs in Hong Kong, the Government has rolled out various measures to expedite the drug registration process in recent years. Under the "Enhanced Procedures for Registration of New Drugs" (Enhanced Procedures) as introduced in June 2018, the Pharmacy and Poisons Board (the Board) will initiate the legislative procedures of amending the Pharmacy and Poisons Regulations

(Cap. 138A) upon receipt of an application for registration of a new pharmaceutical product by a pharmaceutical company, or when a new pharmaceutical product is covered under HA's early access programme or other relevant government-subsidised drug programmes. The Enhanced Procedures aim at shortening the time required for registration of the pharmaceutical products so that they are made available in the market as early as possible. As at March 2019, DH has handled the registration of 19 pharmaceutical products containing new chemicals or biological entities under the Enhanced Procedures. The time required for processing applications for registration of pharmaceutical products are generally shortened by two to three months after the implementation of the Enhanced Procedures.

11. As regards the inclusion of new drugs in the HA Drug Formulary (HADF) for use in public hospitals and clinics, HA has been conducting on-going evaluation for new drugs and clinical indications regularly with the support of 21 expert panels to ensure timely and equitable access to suitable drugs by patients under the HADF. The evaluation process follows an evidence-based approach, having regard to the principal considerations of safety, efficacy and cost-effectiveness. HA has all along been expanding the HADF with the recurrent resources provided by the Government. An additional recurrent subvention of $400 million has been allocated to HA in 2019-20 to widen the scope of the HADF. With the additional funding, HA will incorporate eight new drugs into the HADF under the special drug category for provision to patients under standard fees and charges. Furthermore, HA will also extend the therapeutic application of 11 special drugs / drug classes in the HADF for various diseases including multiple sclerosis and tuberous sclerosis complex. It is expected that around 53 000 patients will be benefited from the expanded scope of the HADF in 2019-20.

12. In the process of evaluating drugs for treating uncommon disorders, HA's relevant committee would take into account special factors including the lack of large-scale scientific research data in support of the efficacy of these drugs and their diverse effectiveness on individual patients. To appraise the actual benefit of drug treatments on individual patients, HA has put in place an independent expert panel mechanism to formulate treatment protocols for uncommon disorders using ultra-

expensive drugs, including drugs not in the HADF. The expert panel has reviewed the application for use of ultra-expensive drugs on individual patients with specific uncommon disorders on a case-by-case basis in accordance with established treatment guidelines, having regard to the patients' clinical conditions and making reference to overseas treatment guidelines and the latest available clinical evidence. In addition, HA has devised procedures for clinicians to use non-HADF drugs and unregistered drugs under special circumstances for specific individual patients to ensure that patients would receive the most appropriate clinical care and drug treatment.

Provision of drug subsidy

13. Our public healthcare services are heavily subsidised and available to members of the public at low rates under the prevailing healthcare policy to ensure that no one will be denied appropriate medical care due to lack of means. At present, HA makes use of the recurrent funding provided by the Government, Samaritan Fund (SF), and the Community Care Fund (CCF) Medical Assistance Programmes to provide drug subsidy to patients suffering from different diseases, including those with uncommon disorders.

14. In response to the emergence of ultra-expensive drugs in the market and the rising demand for patients with uncommon disorders to receive ultra-expensive drug treatments, the Government and HA rolled out in August 2017 a CCF Medical Assistance Programme, namely "Subsidy for Eligible Patients to Purchase Ultra-expensive Drugs (Including Those for Treating Uncommon Disorders)" (the CCF Ultra-expensive Drugs Programme). The scope of this programme has been expanding to provide patients with subsidies for specific drug treatments according to their individual clinical needs.

15. Following the expansion its coverage to include the drug for treating Spinal Muscular Atrophy in September 2018, the Commission on Poverty has just approved at its meeting on 9 April 2019 the introduction of Tafamidis under this programme to provide subsidy for treatment of Transthyretin Familial Amyloid Polyneuropathy. For the period from August 2017 to March 2019, 23 applications have been approved with a

total subsidy of around $76 million and average subsidy of around $3.3 million per application. For the financial year 2019-20, the CCF has reserved a total of about $225 million as the budget limit for the CCF Ultra-expensive Drugs Programme. HA will continue to evaluate and propose suitable new drug for inclusion into the coverage of the CCF Ultra-expensive Drugs Programme.

16. To expedite the introduction of new drugs into the coverage of SF and CCF Medical Assistance Programmes, HA has, since 2018, increased the frequency of the relevant prioritisation exercise from once to twice a year, so as to provide more timely support to patients. Apart from that, HA set up a new mechanism in July 2018 designated for evaluating the application for special drug programmes by individual patients, including patients with uncommon disorders, so as to enable their early access to novel drug treatments. A negotiation team has also been formed to liaise with pharmaceutical companies on formulation of special drug programmes and long-term financial arrangements for use of specific ultra-expensive drugs. HA will put forth the drugs recommended under this mechanism to the Commission on Poverty for consideration of inclusion in the CCF Ultra-expensive Drugs Programme to provide subsidy for patients.

17. With a view to alleviating the financial burden of patients' families, the Government and HA have introduced measures in early 2019 to enhance the means test mechanism for SF and CCF Medical Assistance Programmes. The enhancement measures include modifying the calculation of annual disposable financial resources for drug subsidy application by counting only 50% of the patients' household net assets; and refining the definition of "household" adopted in financial assessment. After enhancing the means test mechanism and increasing the frequency of including suitable drugs in the safety net, we estimate that the total subsidy under SF and CCF Medical Assistance Programmes will increase from around $728 million in 2017-18 to $1.5 billion in 2019-20. We will closely monitor the effectiveness of the enhancement measures in lowering patient contribution to drug expenses and provide financial protection for patients and their families.

An Information system for uncommon disorders

18. HA's clinical management system (CMS) maintains the clinical data of all patients, including patients with uncommon disorders, and information of different diseases. In the process of acquiring more local experiences in treating uncommon disorders, HA has been accumulating relevant clinical data with an aim to progressively enrich the data of patients with uncommon disorders in its CMS. The CMS would assist clinicians in diagnosing and treating patients, as well as to facilitate research and policy formulation in this regard.

19. In view of the centralised genetic services to be provided by HKCH, a data centre has been built in HKCH for housing associated IT systems and servers, providing a scalable capacity for processing massive volume of genetic data with future resources injection. In the long run, HA will take reference from the model of HKCH with an aim to developing a centralised service centre with relevant clinical data for providing care and treatment to all patients with uncommon disorders.

THE GOVERNMENT'S STANCE ON LEGISLATING FOR RARE DISEASES

20. We welcome proposals that can improve the quality, efficiency and efficacy of care for all patients, including but not limited to patients with uncommon disorders. However, legislating for purposes that can be achieved through an administrative route is neither necessary nor desirable. The Government and HA are committed to providing most suitable care and treatment for all patients, including those with uncommon disorders. Measures are already in place to support patients with uncommon disorders, along the lines of some of the legislative content proposed in the Motion. Legislating on uncommon disorders would not necessarily be conducive to helping patients with uncommon disorders.

21. Every legislative proposal has to take into account the local context and public healthcare policy. The proposed statutory regime for rare disease would introduce an unnecessary legal divide between rare and non-rare disease patients and would complicate clinical treatment processes.

Each disease has its uniqueness and individual patients would require different clinical attention and care. If only patients with certain diseases falling under the definition would receive the statutory entitlements, the focus of our support would be diverted to how the line is being drawn and how to review the defined coverage, thereby obscuring the crucial mission of addressing the specific clinical needs of individual patients. Grouping diseases by their rarity does not necessarily serve the purpose of improving the relevant diagnosis and treatment. A definition based on prevalence rate would also overlook other significant factors to be considered such as the severity of the disease and the availability of treatments. Indeed, the optimal treatment for a patient – whether struck by known or rare disease, hinges more on professional judgement, the seriousness (not just rarity) of the disease, and availability of expertise and resources, etc. than on the presence of a statutory definition of and statutory register on the disease.

THE WAY FORWARD

22. The Government and HA will continue the on-going dialogue with stakeholders including patient groups, as well as accumulating more experiences in treating uncommon disorders under established mechanisms, so as to review and strengthen the support for patients with uncommon disorders.

Food and Health Bureau
Hospital Authority
April 2019

(Translation)

Motion on
"Enacting legislation to protect the rights and interests of rare disease patients"
moved by Dr Hon Fernando CHEUNG
at the Council meeting of 23 January 2019

Motion as amended by Prof Hon Joseph LEE and Dr Hon Elizabeth QUAT

That as rare disease patients in Hong Kong have all along suffered neglection of their well-being, they have to face numerous difficulties in their living; quite a number of countries have laid down definitions, enacted legislation and formulated evidence-based and regularized long-term policies on rare diseases as early as the 1980s, making it more convenient for rare disease patients to apply for approval of drugs, subsidy, and so on, and enabling them to receive more efficient and more proper treatment and care; and the United States, member states of the European Union and the neighbouring countries and regions of Hong Kong, such as Singapore, Japan, Australia, Taiwan and Korea have long laid down definitions of rare diseases, formulated support policies, established a database of rare disease cases, etc.; however, the Hong Kong Government has yet to lay down any definition and formulate any concrete policy on rare diseases to provide support for rare disease patients; in this connection, this Council urges the Government to enact legislation on rare diseases, in order to protect and promote the rights of rare disease patients and enable them to receive proper diagnosis, treatment and care, thereby fulfilling the requirements of the United Nations Convention on the Rights of Persons with Disabilities; the relevant contents include:

(1) establishing a policy committee on rare diseases to advise on a strategic development direction for a policy on rare diseases, monitor the implementation of the policy on rare diseases by government departments and statutory bodies, report on the implementation of the policy on rare diseases, etc.;

(2) establishing an evaluative committee on rare diseases to evaluate whether a disease meets the definition of rare diseases;

(3) defining a disease which affects no more than 1 in 10 000 individuals in Hong Kong and is clinically definable as a rare disease;

(4) introducing a registration system for rare disease drugs whereby rare disease patients, medical practitioners and pharmaceutical companies

can apply for the inclusion of new drugs in the list of drugs for rare diseases;

(5) introducing a subsidy system to ensure that rare disease patients will receive safe, quality, effective and affordable drugs and treatment, instead of laying emphasis only on cost-effectiveness;

(6) introducing a rare disease information system which contains a list of rare diseases and their prevalence rates, the demographic information of patients and usage statistics of rare disease drugs;

(7) establishing a dedicated medical team and stepping up health care training to centralize the handling of suspected rare disease cases and expedite the testing and diagnosis of rare diseases, thereby raising the cost-effectiveness of health care; and

(8) establishing an inter-disciplinary group to provide support for promoting the physical and mental health of rare disease patients and their families;

and the Government should also allocate additional resources for introducing genetic tests in the public health care system, including the provision of free prenatal non-invasive fetal trisomy testing services to pregnant women for early identification of the risk of rare diseases in foetuses, so that appropriate treatment can be provided as early as possible, and provision of preconception trisomy testing services to those who wish to have children, such that they can understand the risk of giving birth to babies suffering from rare diseases and the treatment required, thereby making appropriate decisions on childbearing.