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Legislative Council Panel on Health Services

The Administration's Response to the Rare Diseases Bill proposed by Dr Hon Fernando CHEUNG

PURPOSE

This paper sets out the Administration's response to the Rare Diseases Bill (the Bill) proposed by Dr Hon Fernando CHEUNG.

THE BILL

2. Dr Hon Fernando CHEUNG's Bill seeks to provide a legislative framework for policy on supporting patients with rare diseases. The Bill seeks to provide for –

(a) a Commission on Rare Diseases Policy;
(b) an Evaluative Committee on Rare Diseases;
(c) recognition of a disease or malfunction as rare disease;
(d) register of rare disease drugs, treatments or products;
(e) a statutory subsidy scheme for rare diseases; and
(f) a Rare Disease Information System.

THE GOVERNMENT'S POSITION AND THE EXISTING SUPPORT FOR UNCOMMON DISORDERS

3. It has been the Government's long-established healthcare policy to strive to provide suitable care and treatment for all patients, regardless of their illnesses. The Hospital Authority (HA) would formulate and adjust treatment protocol for patients in light of their individual clinical conditions and needs. Nonetheless, 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 rare diseases

(described as uncommon disorders by us) in their disease journey, such as possible delay 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.

4. Although there is no specific legislation on policy for uncommon disorders in Hong Kong at present, the Government and HA have in practice been providing comprehensive support for such patients as set out below.

Multi-disciplinary care

5. The multi-disciplinary team of HA, comprising doctors, nurses, pharmacists, clinical psychologists, physiotherapists, occupational therapists and medical social workers, has all along been providing holistic care to patients with uncommon disorders. In light of clinical and physical needs of individual patients, the multi-disciplinary team would provide suitable comprehensive services ranging from newborn screening, clinical diagnosis, medical and surgical treatment to palliative care and rehabilitative services. The support given is not limited to the provision of suitable drug treatments, and aims to ensure that individual needs of each patient can be properly addressed.

Introduction of new drugs

6. Provision of suitable and affordable drug treatment is one of the important aspects of our support for patients with uncommon disorders. Established mechanisms and subsidy programmes have been put in place to evaluate new drugs and provide financial assistance to patients in need, including patients with uncommon disorders. The Bill provides for a designated registration system and subsidy scheme for the relevant drugs, which would overlap with our work on this front. 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.

7. Regarding the registration of new drugs, the Government has rolled out various measures to expedite the drug registration process in recent years. To enable the pharmaceutical products to be available in the market as early as possible, the Pharmacy and Poisons Board (the "Board") agreed to implement the "Enhanced Procedures for Registration of New Drugs" (Enhanced Procedures) in June 2018. 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 Board will initiate the legislative procedures of amending the Pharmacy and Poisons Regulations (Cap. 138A), aiming to shorten the time required for registration of the pharmaceutical product. As at January 2019, the Department of Health (DH) has handled the registration of 17 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.

8. As for the listing of new drugs on the HA Drug Formulary for use in public hospitals and clinics, HA has an established mechanism to evaluate new drugs quarterly with a view to widening the Drug Formulary. The evaluation process follows an evidence-based approach, having regard to the principal considerations of safety, efficacy and cost-effectiveness. The Budget for 2019-20 will provide an additional recurrent subvention of $400 million for HA to widen the scope of the Drug Formulary, particularly the coverage of General and Special Drugs in the Formulary. It is estimated that HA's total expenditure on drugs will increase to $6 billion in 2019-20. In addition, HA has devised procedures for clinicians to use non-Formulary drugs and unregistered drugs under special circumstances for specific patients to ensure that patients would receive the most appropriate clinical care and drug treatment.

9. Some of the drug treatments for uncommon disorders can be ultra-expensive with preliminary medical evidence and diverse clinical responses among patients. As such, 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 the drugs for treatment of uncommon disorders when evaluating these drugs for listing.

Furthermore, 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 Drug Formulary, and to appraise the actual benefit of drug treatments on individual patients. 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.

Provision of drug subsidy

10. Under the current healthcare policy, we strive to ensure that all patients will not be denied appropriate and adequate medical treatment due to lack of means, and therefore our public healthcare services are heavily subsidised and available to members of the public at low rates. 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.

11. In view of 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. For the period from August 2017 to March 2020, the CCF has reserved a total of about $380 million as the budget limit for this programme. As of January 2019, 21 applications have been approved with a total subsidy of around $71 million and average subsidy of around $3.4 million per application. HA will continue to evaluate and propose suitable new drug for inclusion into the coverage of the CCF Ultra-expensive Drugs Programme.

12. To expedite the introduction of new drugs into the safety net, 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.

SERVICE ENHANCEMENTS FOR SUPPORT FOR PATIENTS WITH UNCOMMON DISORDERS

13. The Government and HA have formulated a number of targeted measures to enhance the support for patients with uncommon disorders on the basis of the existing mechanisms. In particular, the service enhancements will focus on facilitating the diagnosis of uncommon disorders and improving the access to drug treatments.

Role of the Hong Kong Children's Hospital

14. The Hong Kong Children's Hospital (HKCH), which has commenced its service by phase since 18 December 2018, will play an essential role in enhancing our support for patients with uncommon disorders, particularly given that many rare diseases are caused by genetic or congenital disorders. HKCH will serve as the tertiary referral centre for complex, serious and uncommon paediatric cases which require multidisciplinary management. Specifically, it will support genetic testing such as non-invasive prenatal test for chromosomal abnormality and develop newer genetic tests. To further enhance our genetic services, the Clinical Genetic Service (CGS) of the Department of Health will move to HKCH in late 2019 with a view to pooling together advanced equipment, expertise and relevant support in genetic screening.

15. Over the past 30 years, the Genetic Screening Unit (GSU) of the

CGS has been operating the territory-wide Neonatal Screening Programme, targeting two conditions, namely, glucose-6-phosphate dehydrogenase deficiency and congenital hypothyroidism for all newborn babies delivered at the hospitals under HA. On the other hand, the Genetic Counselling Unit (GCU) of CGS has been providing diagnostic and genetic counselling services to patients and families affected by genetic diseases and uncommon disorders. The Genetic Laboratory of CGS is equipped with both conventional and advanced genetic testing technologies to provide diagnostic support to patients of GCU. Up to now, GCU has provided service to over 38 000 families, with over 1 500 new patients/families referred every year. Moreover, GSU and HA have been in collaboration since 2015 to provide newborn screening programme for 24 metabolic diseases of inborn errors of metabolism in HA's hospitals to enable early detection, diagnosis and intervention.

16. In terms of research and development, dedicated infrastructure such as research laboratories and clinical trial centre have been planned in HKCH to facilitate close collaboration with the medical faculties of the University of Hong Kong and the Chinese University of Hong Kong for pursuing basic and translational research in paediatric and genetic diseases.

Information System for Uncommon Disorders

17. 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.

18. 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.

Hong Kong Genome Project

19. The Steering Committee on Genomic Medicine (the Steering Committee), as set up by the Government in end 2017 to advise on the development of genomic medicine in Hong Kong, is examining the genomic medicine development strategies for Hong Kong from various perspectives, including the role of genomic medicine in uncommon disorders. One of its preliminary recommendations was the Hong Kong Genome Project (HKGP), which is a large-scale genome sequencing project to promote the clinical application and innovative scientific research of genomic medicine.

20. The Chief Executive announced in her Policy Address last year to introduce the HKGP. 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. We have consulted the Panel on Health Services of the Legislative Council (the Panel) on the directions of the HKGP on 21 January, and obtained the Panel's support for the proposal. 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 and the relevant expert group to discuss the project details.

Enhanced means test mechanisms for SF and CCF Medical Assistance Programmes

21. For the purpose of alleviating the financial burden of patients' families arising from drug expenses, the Government and HA have introduced measures to enhance the means test mechanism for SF and CCF Medical Assistance Programmes in early 2019. The enhancement measures include modifying the calculation of annual disposable financial resource in drug subsidy application by counting only 50% of the patients' household net assets, so as to offer asset protection to patients' families;

and refining the definition of "household" adopted in financial assessment.

22. Based on the data of drug subsidy applications approved from June 2017 to February 2018, it is estimated that the enhancement measures will lower patient contribution for around 1 005 existing applications per year. Apart from increase in drug subsidy among existing cases, there will also be additional financial implications due to new applications which may potentially be made by those who may become eligible for subsidy as a result of the enhancement measures. After enhancing the means test mechanism and increasing the frequency of including suitable drugs in the safety net, we aniticipate that the total subsidy under SF and CCF Medical Assistance Programmes will increase from around $700 million in 2017- 18 to $1.5 billion in 2019-20.

23. We expect that the enhancement measures can significantly lower the patient contribution to drug cost, provide financial protection to patients' families, and enable more patients to receive drug subsidy.

PROPOSED BILL

24. 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 proposals in the Bill. Legislating on uncommon disorders would not necessarily be conducive to helping patients with uncommon disorders.

25. The statutory regime as proposed 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. A definition based on prevalence rate would 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.

26. The proposed Bill will also have policy and resource implications on the Government.

THE WAY FORWARD

27. The Government and HA have been very concerned about the well-being of patients with uncommon disorders. Mechanisms are in place to provide support to such patients in various aspects, including clinical diagnosis and assessment, multi-disciplinary care and rehabilitation services, introduction of new drugs, as well as subsidising drug treatments. The initiatives proposed in the Bill will overlap with the work of the Administration and HA on this front without promising better treatment for patients concerned. In light of the above considerations, the Government does not support legislating on uncommon disorders at this stage. The Government and HA will continue the on-going dialogue with stakeholders including patient groups to review and strengthen the support for patients with uncommon disorders.

Food and Health Bureau
Hospital Authority
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