香港罕見疾病聯盟
RARE DISEASE HONG KONG

Rare Disease Hong Kong
Submission on 2020 Policy Address
（September 2020）

Established in December 2014, Rare Disease Hong Kong (RDHK) is the first patient group in Hong Kong comprising cross-rare-disease patients and their families with the support of experts and academics in the field. Representing the patients and caregivers, RDHK is committed to enhancing public awareness of rare diseases in collaboration with the stakeholders. We aim to improve rare disease policies and services, and to ensure equal respect and protection for patients in terms of fundamental rights such as healthcare, social support, education, and daily needs.

New measures stated in the previous Policy Address

In RDHK's submission on 2019 Policy Address, it was suggested that the Government should collaborate with the business sector and the community to devise and implement the "Hong Kong Rare Disease Action Plan".

The Chief Executive was responsive to the priority issued raised by RDHK. In the 2019 Policy Address, the new measures relating to rare diseases were stated as follows:

- *"To strengthen support for patients with uncommon disorders, the Government and the Hospital Authority (HA) plan to implement progressively a series of targeted measures, including examining the setting up of databases for individual uncommon disorders to facilitate clinical diagnosis and treatment, enhancing public awareness of uncommon disorders without infringing patient privacy, strengthening the support for drug treatment of patients with uncommon disorders and cancers through the Samaritan Fund and the Community Care Fund, and reviewing manpower arrangements and deploying resources to cater for the needs of the caregivers and to promote relevant scientific research and development.*

- *The Steering Committee on Genomic Medicine plans to submit a report to the Secretary for Food and Health on the study of strategies for developing genomic medicine in Hong Kong at the end of 2019. Besides, the Hong Kong Genome Institute will be set up in 2020 to take forward the Hong Kong Genome Project. The project will enhance the diagnostic rate of uncommon disorders and promote research on cancers and other hereditary diseases through genome sequencing and the establishment of a genome database."*

The above measures put forward by the Chief Executive were highly appreciated. RDHK had contacted the Food and Health Bureau (FHB) and HA time and again in the past year, hoping to exchange opinions on the implementation of the measures that can immediately bring actual benefits to patients with rare diseases, and how patient groups can support and cooperate with the Government. However, the authorities concerned have never provided any substantive replies, and the only standard reply was "under study". For instance, the Secretary for Food and Health's latest response (10 August 2020) was as follows:

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"In order to implement the series of targeted measures that support patients with uncommon disorders as stated in last year's Policy Address, the Government and HA are planning to progressively inject resources and strengthen the manpower support of Hong Kong Children's Hospital so as to facilitate clinical diagnosis and treatment for uncommon disorders, to further coordinate and take care of patients' needs, to enhance public awareness of uncommon disorders, and to examine the setting up of databases for individual uncommon disorders. The details are currently under study."

Apparently, all the new measures put forward by the Chief Executive last year are still under study. It is really disappointing and regrettable that there has been no significant progress in policy implementation so far, not to mention the outcome.

Demands to be addressed in the 2020 Policy Address

In light of the current way that the government departments deal with rare diseases, RDHK would like to put forward the following four demands, hoping that the Chief Executive will consider and respond seriously in her coming Policy Address:

(1) To establish a precise timeline and outcome indicators for implementation of the new measures

New measures stated in last year's Policy Address include:

1. *examining the setting up of databases for individual uncommon disorders;*
2. *enhancing public awareness of uncommon disorders;*
3. *strengthening the support for patients with uncommon disorders and cancers through the Samaritan Fund;*
4. *reviewing manpower arrangements and deploying resources to cater for the needs of the caregivers;*
5. *promote relevant scientific research and development.*

RDHK hereby urges the Government to instruct the relevant departments to take actions to implement the above measures, and to provide concrete detail, implementation timeline and outcome indicators for each measure. We also urge the relevant departments to communicate and exchange opinions with the patient groups about the implementation progress every three months.

The Hong Kong Genome Institute (HKGI) was established in May 2020 to take forward the implementation of the Hong Kong Genome Project. Recruitment of patients (including patients with undiagnosed disorders) for genome sequencing will begin next year. RDHK requests that the HKGI keeps communicating with patient groups and explore the possibilities of collaboration when implementing various plans, especially those directly related to patients and public education.

(2) To set clear guidelines on the review mechanism of inclusion of orphan drugs into the Safety Net

The government policies always lag behind patients' needs and clinical practices. In response to patients' demand, the authority concerned did approve to make clinical use of several expensive orphan drugs in Hong Kong in the past few years. However, whenever there are new medicines available in Europe and America, the patients, caregivers, patient groups and stakeholders in Hong Kong have to run around to seek help, praying for an early access to the new medications. On one occasion, the Chief Executive promised to get the rare disease patients access to a new orphan drug only when a paralysed patient handed the petition to her in a rare disease patient group's rally calling for more support from officials. But this is not an uncommon phenomenon which reflects the fact that Hong Kong is lacking a clear and feasible review mechanism of inclusion of orphan drugs for early access to patients in need, which makes the clinical experts, patients and pharmaceutical industry all at a loss. Although the financial assessment criteria for eligibility for safety net coverage were improved in 2018, the current drug review and selection mechanism cannot address the clinical needs of the patients, and the path to inclusion of orphan drugs into the safety net is still a thorny one.

As several orphan drugs were approved for use in the past few years, it is believed that the clinical experts and management had gained experience in approving orphan drugs, which to some extent should lay the foundation for the development of clear selection guidelines. In fact, it is doubtless that the drug safety and efficacy of medicines registered in Hong Kong are all recognised by the drug regulatory authorities of European countries and the United States. However, sufficient local clinical data can never be collected if a drug is not used. Besides, the HA and FHB mainly consider the cost-effectiveness in the drug approval process, they do not have health economics experts to advise them, however. So other essential factors like health care effectiveness and efficiency, and social impacts are neglected. RDHK recommends that more scientific health economic analysis should be adopted in the review mechanism of orphan drugs. For instance, contexts of specific type of rare diseases, "Patient-Reported Outcome" and "Risk Sharing" protocols should be considered when setting drug review guidelines for the clinical physicians, patients, pharmaceutical companies and decision makers.

(3) To set up the "Steering Committee on Strategy for Rare Diseases" as soon as possible

Despite the Chief Executive's sincere intention to address patients' demands last year, all new measures to combat rare diseases have not been practically implemented. The key problem is lack of coordination and supervision. So last year, RDHK requested the Government to set up a steering committee on strategy for rare diseases which is necessary to solve the bottleneck and propose feasible ideas to the Chief Executive regarding rare disease policies.

It is suggested that the "Steering Committee on Strategy for Rare Diseases" be chaired by the Secretary for Food and Health and comprises rare disease experts and representatives from the

Government, HA, two medical faculties, patient groups and the business sector. The main responsibility of the Committee is to formulate and implement the "Action Plan" in phases by taking reference from the "APEC Rare Disease Action Plan" and the current situation and conditions of Hong Kong. The Committee is also responsible for coordinating the implementation of various rare disease measures put forward by the Chief Executive, monitoring the implementation progress and reporting to the Chief Executive. Sub-committees or working groups with different duties and functions may also be set up if necessary, so as to bring together different experts from the Government and the community to tackle the challenges of rare diseases.

(4) To formally define "rare disease" and compile the rare disease List

Currently, there is no formal definition of rare diseases in Hong Kong. In recent years, the term "uncommon disorder" has been used in government official documents. However, the FHB has neither defined nor given any examples of "uncommon disorders". It is really hard to understand why the Government adopts such an evasive and vague attitude towards this issue. As a consequence, different stakeholders have their own definition of rare diseases. Knowing that it is not that easy to work out a definition that is scientifically acceptable and complies with the situation in Hong Kong, RDHK does not expect the Government to finish the job overnight. Nevertheless, the Government cannot avoid this problem forever. RDHK hopes that the Government will be determined to take the first step by setting up the "Steering Committee on Strategy for Rare Diseases" and giving the task of defining rare diseases high priority.

Meanwhile, the Government can take the first step in compiling the rare disease list by recognizing approximately 40 "uncommon disorders" or indications that are treated with special drugs, drugs supported by the Care Community Fund, Samaritan Fund and some compassionate programmes as listed in the Drug Formulary. In the future, more and more rare diseases can be added to the list after the official definition of rare diseases has been established, which can facilitate compliance by all stakeholders.

Conclusion

Since the current-term Government took office, the Chief Executive has made substantial commitment that no previous government has ever had in response to rare disease issues. If the new measures put forward last year can be effectively implemented in the coming years, it will not only meet the immediate needs of the rare disease patients, but also lay a solid foundation for formulating long-term strategies and plans.

RDHK strongly believes that the challenges of rare diseases can be tackled more effectively through tripartite collaboration among the Government, business sectors and the community. Meanwhile, RDHK is more than willing to communicate and cooperate with the Government in relation to rare disease issues.

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