

Rare Diseases Hong Kong

Submission on the Public Consultation for the 2022 Policy Address

(September 2022)

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.

The 2022 Policy Address will be delivered in October. It will be the first policy announcement made by the Chief Executive under a new political atmosphere where the Electoral System has been improved and “patriots administering Hong Kong” has been ensured. So, RDHK is on the tiptoe of expectation.

The previous Government put forward some measures on rare diseases, which helped to address and solve the specific issues of individual diseases. However, these measures are only reliant on clinical means, sporadic, fragmented, unsystematic and unplanned, which is far from satisfactory in terms of effectiveness of tackling rare diseases. RDHK expects that the new-term Government will abandon the passive attitude adopted in the past, and take a macro perspective, proactive and forward-thinking approach to policy development so that more systematic, comprehensive and integrated short- and long-term measures can be launched. It is hoped that the current-term Government will consider and adopt the following five recommendations for combating rare diseases in Hong Kong:

- I. To set up a government-led “Steering Committee on Strategy for Rare Diseases”**
- II. To commence a study on the local definition of rare diseases**
- III. To develop and implement a five-year action plan**
- IV. To optimise and improve the access mechanism for orphan drugs by cutting the red tape**
- V. To collaborate on rare disease diagnosis and treatment in the Greater Bay Area**

The recommendations are elaborated as follows:

I. To set up a government-led “Steering Committee on Strategy for Rare Diseases”

Since 2019, RDHK has been advocating the establishment of a government-led “Steering Committee on Strategy for Rare Diseases” (Steering Committee) in its annual recommendations to the Policy Address.

In recent years, rare diseases have been receiving increasing public attention in Hong Kong. The introduction of new measures by the Government in consequent years has contributed to the boosted attention and commitment to rare diseases among various sectors such as universities, research institutions, innovation & technology enterprises, and healthcare & pharmaceutical industries; accumulation of experience in screening, diagnosis and treatment of rare diseases among the staff of the Hospital Authority (HA) from management to frontline clinical specialists; and a more comprehensive and in-depth understanding of rare disease issues among the government officials. On the existing foundation, if the passion and strength of different parties are gathered, and

professional knowledge and resources are coordinated effectively, the outcomes of tripartite collaboration among the Government, the business sector and the community can certainly be maximised, so that the efforts of the Government and stakeholders can be multiplied with half the efforts. If the current Government is determined to effectively address the challenges of rare diseases, it is imperative for it to take the lead in the establishment of the “Steering Committee”.

It is suggested that the “Steering Committee” be led by the Government and chaired by the Secretary for Health, with government officials and representatives from the HA, two medical faculties, rare diseases specialists, patient groups, the business sector, etc. as members. The core responsibilities of the committee are:

1. To formulate the policy goals and medium- to long-term strategy for rare diseases in Hong Kong based on the existing practical experience as well as the experience of Europe, the US and the Mainland;
2. To devise and implement in phases the “Hong Kong Rare Disease Action Plan” in accordance with the situation in Hong Kong, including issues of human resources, patient registries, centres of excellence, cross-border collaboration, rehabilitative support, etc.;
3. To coordinate and implement various measures for rare diseases proposed by the Chief Executive, and to monitor and report the progress of implementation to the Chief Executive; and
4. When necessary, the Steering Committee may set up sub-committees or working groups with different responsibilities and functions to bring together experts from the Government and community to tackle the challenges of rare diseases together.

II. To commence a study on the local definition of rare diseases

In the annual submissions on the Policy Address consultation presented by RDHK in the past, it was proposed that Hong Kong should have a local definition of rare diseases to facilitate the effective implementation of relevant policy initiatives. However, the Government has so far rejected the proposal for the reason that there is no universally accepted definition of a rare disease, and the service fairness will be affected after an official definition has been adopted.

In fact, the so called “reason” is only a convenient excuse based on the practical aspects of clinical services, which is in no way the mindset and perspective that a government official should have, and it reveals the slack and sitting back mentality of the officials in charge. May we ask why Hong Kong can set and continuously adjust its poverty line even though there is no uniform poverty line in the international arena? Why is the Government not afraid that setting a poverty line is unfair to those citizens with low incomes but have not yet fallen below the poverty line?

It is understandable that defining rare diseases locally is a complex task that cannot be accomplished overnight. However, the current Government should no longer evade the issue by simply saying “no” on unconvincing grounds. Instead, it should demonstrate an accountable and responsive governance style by bringing together the stakeholders to commence a study. The Government should draw on the experience of those countries and regions that have effectively dealt with rare diseases, explore the pros and cons of establishing a local definition of rare diseases and objectively weigh the positive and negative impacts on society as a whole before drawing a conclusion that is in line with the actual situation in Hong Kong.

III. To develop and implement of a five-year action plan

In a document submitted to the Legislative Council years ago, the Government expressed that “legislating for purposes that can be achieved through an administrative route is neither necessary nor desirable”. Having been concurring with the Government’s stance, RDHK proposed to the then Chief Executive in 2019 that the Government should take the lead in formulating and implementing in phases the “Hong Kong Rare Disease Action Plan” with the tripartite collaboration among the Government, the business sector and the community. However, no positive response has been received in the past few years. So RDHK would like to put forward this proposal again to the current Government, expecting that there are goals for the 5-year tenure as well as implementation schedule for each year in the action plan.

The action plan should include but not limited to the following aspects:

1. Manpower allocation and professional training

Specially trained professionals (e.g. clinical geneticists, genetic counsellors, allied nurses, etc.) are essential for effective screening, diagnosis, treatment, rehabilitation and research relating to rare diseases. To effectively enhance patient services, relevant positions should be established in the HA to provide the aspiring professionals with career development opportunities.

2. Patient registries

The previous Government pledged to establish patient registries for certain types of rare diseases. The current Government should carry on with the work and expand the list of rare diseases covered by patient registries within the five-year tenure.

3. One-stop follow-up consultation and centres of excellence

The diagnosis and treatment of rare diseases often involve multiple specialists, and therefore some patients need to visit eight to ten different specialists for follow-up consultations, which has long been criticised as a time-consuming and exhausting process. A few years ago, Hong Kong Children’s Hospital began to pilot one-stop follow-up care for individual rare diseases, which has been well received by patients. This patient-oriented service model should be gradually extended to more rare diseases, and on this basis, the establishment of rare disease centres of excellence should be planned.

4. Newborn screening

Newborn screening is an effective method for early diagnosis and timely treatment. It is known that individual rare diseases have been included in the local newborn screening programme for further studies and trials. The Government should step up its efforts in the coming years to expand the scope of newborn screening for rare diseases with effective treatment and reliable testing methods available.

5. Cross-border collaboration

In recent years, the Government has been vigorously promoting cross-border collaboration in various fields, especially integration with the Greater Bay Area. In fact, cross-border collaboration is also needed for and will be beneficial to the diagnosis and treatment of rare diseases (please refer to point V of this document for details). The Government should make specific plans for cross-border collaboration to facilitate the diagnosis and treatment of rare diseases, and make sure that those plans can be effectively implemented.

IV. To optimise and improve the access mechanism for orphan drugs by cutting the red tape

For those rare disease patients with declining physical function, their greatest concern is whether they can receive timely treatment. According to the usual process of drug registration and inclusion, patients usually wait for years before having the opportunity to take new drugs that have already come onto the market. Fortunately, the Government has begun to recognise and propose new measures dealing with rare diseases in recent years. For instance, since August 2017, eligible patients have been provided with subsidy to purchase ultra-expensive drugs (including those for treating uncommon disorders). Two of the ultra-expensive drugs (Tisagenlecleucel and Tafamidis) currently funded by the Community Care Fund (CCF) were incorporated into the CCF assistance programme in 13 to 14 months on average after registration; whereas Nusinersen was included in the Safety Net in only 4 days after registration, so that the patients in need could receive treatment as soon as possible.

As a patient group, RDHK welcomes the flexible approach adopted by the HA. The precedent cases in recent years have shown that accelerating the inclusion of orphan drugs in the HA Drug Formulary and Safety Net is feasible. Therefore, RDHK handed in a submission on “Optimising and Improving Access Mechanism for Orphan Drugs by Cutting the Red Tape” to the Government and HA, recommending the seven measures below to optimise and regularise the access mechanism for orphan drugs:

1. Optimise the documentation requirements for registration and registration process of orphan drugs;
2. Strengthen collaboration between the Chief Pharmacist’s Office (CPO) of the HA and the pharmaceutical industry;
3. Omit the non-essential work of the Drug and Therapeutics Committee (DTC) in the drug inclusion process;
4. Adjust the schedule and mode of the Drug Management Committee (DMC) meetings;
5. Solve the chicken-and-egg dilemma by coordinating the two mechanisms of medical testing and drug inclusion;
6. Adopt early awareness and alert systems (also known as horizon scanning) to set priorities for the allocation of resource in advance; and
7. Adopt “Orphan Drug Trial” policy and systematically collect and use real-world data to generate empirical evidence which helps to accelerate patient access to orphan drugs.

So far, RDHK has not received any response from the authorities. It is hoped that the current-term Government will seriously consider and adopt the above recommendations.

V. To collaborate on rare disease diagnosis and treatment in the Greater Bay Area

The Central Government and various provinces and municipalities in the Mainland have launched many policy measures to improve the diagnosis and treatment of rare diseases in recent years. For instance, in 2015, the National Health and Family Planning Commission established the “Expert Committee on Rare Diseases Diagnosis, Treatment and Medical Security”, reflecting that rare diseases have entered the field of national policy; in 2016, the National Rare Diseases Registry System was established to consolidate national rare disease data; in 2018, the National Health Commission (NHC) released its first list of rare diseases, including 121 rare diseases; and in 2019, the NHC established a national collaborative network for rare disease diagnosis and treatment which

consists of 324 hospital nationwide (13 of which are located in the Greater Bay Area), and published the first “*Guidelines for Diagnosis and Treatment of Rare Diseases*”.

Hong Kong has excellent healthcare facilities and human resources, but the Government has always lacked proactive and holistic strategy and long-term planning for the diagnosis and treatment of rare diseases. Cross-border collaboration, especially with the Greater Bay Area, will bring about complementary benefits to local healthcare services and patients.

The following are the preliminary recommendations for close collaboration between Hong Kong and the Greater Bay Area on rare disease diagnosis and treatment:

1. Designate some hospitals in Hong Kong (Hong Kong Children’s Hospital, Queen Mary Hospital or Princess Margaret Hospital may be considered) to participate in the national collaborative network for rare disease diagnosis and treatment, and work closely with the 13 designated rare disease hospitals in the Greater Bay Area in clinical diagnosis and treatment. There can be cross-border referrals as necessary and when conditions permit. (Action to be taken by: Health Bureau, Hospital Authority)
2. Include HA’s rare disease cases in the National Rare Diseases Registry System, and encourage intensive cooperation and exchange of data and scientific research with the 13 designated rare disease hospitals in the Greater Bay Area. (Action to be taken by: Health Bureau, Hospital Authority, Faculties of Medicine of HKU and CUHK)
3. The internationally recognised Clinical Trial Centres of the two faculties of medicine in Hong Kong should collaborate with the 13 designated rare disease hospitals in the Greater Bay Area to attract multinational and Mainland pharmaceutical companies to conduct clinical trials for innovative drugs in Hong Kong. (Action to be taken by: Faculties of Medicine of HKU and CUHK)
4. Review the differences between the drug regulatory systems in Hong Kong and the Mainland to facilitate effective coordination and promote integration. The Department of Health of Hong Kong accepts the National Medical Products Administration as a recognised drug regulatory authority. (Action to be taken by: Health Bureau, Department of Health)
5. Adopt strategic procurement and initiate cross-border collaboration on medical insurance system in the Greater Bay Area so that more reasonable orphan drug prices can be achieved. (Action to be taken by: Hospital Authority)

As cross-border collaboration between Hong Kong and the Greater Bay Area on rare diseases is a new thing, it is expected that the government departments and relevant experts will discuss and study the above recommendations as soon as possible, and launch initiatives as far as they are ready.

If the diagnosis and treatment of rare diseases in Hong Kong and the Greater Bay Area are integrated, it is expected that local industry and patients will benefit in the following ways:

1. The number of clinical cases will be largely increased by real-time clinical exchange through the collaborative network for rare disease diagnosis and treatment. Consequently, the experts can gather more data and clinical experience which helps to accelerate diagnosis and treatment planning process.
2. If more and more clinical trials for innovative orphan drugs are conducted in Hong Kong, the local patients will gain early access to new drugs, whereas local cumulative data on drug use can be collected.

3. Since the biomedical industry in the Mainland has advanced by leaps and bounds, recognising its drug regulatory authorities can facilitate early patient access to new drugs from the Mainland.
4. Under the national medical insurance system, the Mainland has strong bargaining power. Therefore, the adoption of strategic procurement in the Greater Bay Area will help the patients in Hong Kong gain access to high-quality and more affordable drugs.

Conclusion

In his important speech delivered at the inaugural ceremony of the current Government, President Xi Jinping put forward “four proposals” and called for an earnest address to people’s concerns and difficulties in daily life. As he said, the newly inaugurated HKSAR Government should be pragmatic, live up to what the people expect of it, and consider the expectations of the whole society, particularly ordinary citizens, as what it should accomplish foremost. It should be more courageous and adopt more efficient measures to overcome difficulties and forge ahead. It should make sure that all citizens in Hong Kong share more fully and fairly in the fruits of development.

At the session on “Spirit of the President's Important Speech” held by the Health Bureau, the Secretary for Health, Professor Lo Chung-mau stated, “Government dominating; be bold and committed; leading the Greater Bay Area; be creative and innovative; improving the healthcare system; caring for all patients; seeking common ground while allowing differences; and winning mutually in the reforms”. He encouraged colleagues of the Health Bureau to incorporate the “four proposals” raised in the important speech of President Xi into their own work, with a view to safeguarding citizens' health and enhancing their happiness in daily life, building up a “Healthy Hong Kong” which can merge into the “Healthy China”.

RDHK has high expectations for the work of the current-term Government, and hopes that the policy makers and executive departments will put words into action and turn knowledge into practice. We look forward to turning a new page for Hong Kong together and turning a new page for the rare disease patients as well!

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