

30 March 2017

Panel on Health Services  
Legislative Council

Dear Panel Members,

**Re: Policy on and drugs for rare diseases**

I am writing to support the proposed Community Care Fund (CCF) assistance programme for subsidizing eligible patients to purchase ultra-expensive drugs for the treatment of rare diseases; and suggest establishing a definition of and a databank on rare diseases to optimize health and social care of rare disease patients and their families.

There are new rare disease cases identified in Hong Kong every year. Most of the rare diseases pose serious threat to a patient's entire life and bring heavy burden to their families especially in financial aspect. Drug treatment for rare diseases can be extremely expensive which is unaffordable for most of the patients. Under the current assessment criteria for drug subsidies, patients from middle-income families may have to contribute to the expenses of extra-expensive drugs which may cost up to several million dollars a year. The long-term prescription of extra-expensive drugs will quickly deplete their assets and impose an immense financial burden on them that will significantly deteriorate their quality of life.

In view of the above, the financial assessment criteria for extra-expensive drugs should be modified to ensure that the level of patient contribution is affordable for average patients, and to allow early use of ultra-expensive drugs by needy patients. In the long run, it is expected that the extra-expensive drugs could also be introduced into the safety net coverage of Samaritan Fund or other funds for extending financial support to rare disease patients.

Hong Kong is internationally renowned for its high quality public health system and healthcare services. However, unlike many countries and regions such as European countries, US, Japan, Korea and Taiwan, our Government has not established any official definition of rare diseases. And currently in Hong Kong, the general public or even many healthcare professionals and social workers may not have adequate understanding and knowledge about rare diseases. Therefore, apart from drug subsidies, clearly defining rare diseases is also highly recommended.

With a clear definition the healthcare professionals, social workers and the general public would have a better and clearer understanding of rare diseases. This would definitely contribute to the prevention, diagnosis and treatment of rare diseases, as well as to enhancing support and protection for rare disease patients and their families. Besides, a territory-wide database on rare diseases or a rare disease patient registry

should also be built up to provide a profile of the common types of rare diseases in Hong Kong, to facilitate information sharing for patient care, and to foster scientific research to support patients with rare diseases.

In a nutshell, I fully support subsidizing eligible patients to purchase ultra-expensive drugs for the treatment of rare diseases. The Government is also expected to enhance the existing Drug Formulary and commit more resources to support research to enhance safety and efficacy of new drugs. Besides, Hong Kong as a World City should establish a clear definition of rare diseases and make reference to international practices in the development and implementation of health policies related to rare diseases.

Thank you very much for your kind attention.

Yours sincerely,

A handwritten signature in black ink, appearing to read "Chair Sek-ying". The signature is written in a cursive, flowing style.

Professor Sek-ying Chair  
Honorary Adviser  
Hong Kong Alliance for Rare Diseases