To: Health Panel of Legco
Hospital Authority Head Office
Food and Health Bureau
Community Care Fund

Re: Policy on and drugs for rare diseases

By invitation of the Hong Kong Alliance of Rare Disease (HKARD), we share our perspective towards current arrangements for the treatment of rare or uncommon diseases as a healthcare provider and an academic within the Hospital Authority. The following documents were provided for our reference:

1. Background brief prepared by the Legislative Council Secretariat for the meeting on 20 March 2017: Policy on and drugs for rare diseases (LC Paper No. CB (2)979/16-17(07)),
2. Legislative Council Panel on Health Services: Policy on Drugs for Uncommon Disorders. (LC Paper No. CB (2)979/16-17(06)),
3. Information Note: Rare disease policies in selected places by the Research Office Legislative Council Secretariat (IN07/16-17).
4. Reply letter penned by Director of Cluster Services of Hospital Authority, Dr. Wai Lun Cheung, to the President of HKARD, Mr. Kin Ping Tsang.

The documents will be referred by their number in the following paragraphs.

Regarding the definition of rare or uncommon disorders

Hong Kong currently has no quantifiable definition of rare or uncommon disorders. We observe that all the other countries discussed in the information note prepared by the Research Office Legislative Council Secretariat have sought a definition in order to identify patient groups for policy scoping.[3] The definition of rare diseases in different countries thus varies depending on their healthcare systems and situations.[1,3] Scoping requires assessment of the extent of the problem in the specified locality, such that resource requirements could be calculated and appropriately allocated. The stringency of the definition then appears to reflect upon the public health resources available to different localities, and also depend on the degree of reimbursement that the local government has decided to provide when they decide to do so. [3]

We agree with Dr. Cheung's premise that all patients, whether suffering from common or uncommon disorders, should receive equal opportunity to appropriate treatment.[4] However, as Legco has actually documented the need for a policy for rare diseases, it seems only reasonable to specify the scope for this policy. The need for rare disease definition and policy scoping in other countries have arisen from a need to incentivise research for an otherwise unattractive market, and identify patient groups that face unique obstacles to diagnosis and drug access that other patients with common diseases are less likely to face. Special policies entailing different arrangements of medical assistance and social care services are thus designed in order to reduce the disparities in treatment received by these patient groups when compared to patients facing a common medical diagnosis. [3]

The Legco document agrees with other countries that a policy on rare disease is necessary, although its premise on the reason this need was not obvious. Without a definition of the target service population, how is the Hong Kong government to communicate within itself, with the public, and vice versa? Without a definition, how can we compare our standards of health care with developed economies that possess the same financial capacities in public health provision for this group of patients with special needs? How can we demonstrate that our practitioner experience and competence, diagnostic capability, drug access and medical insurance practices are sufficient for uncommon disorders, when we don't know what those disorders are?

Regarding the need for public registries

The Hospital Authority (HA) aims to provide affordable multi-disciplinary care to the Hong Kong public. [1] We applaud its efforts and achievements, and acknowledge that there are real resource limitations in providing public healthcare for a small territory, especially in the challenging context of deteriorating income inequalities and a rapidly ageing population.

Yet, no matter how small or limited operations are, accounting estimations are inevitable. Without an evident data bank for diseases outside of the Hong Kong Cancer Registry, it is unclear how such accounting estimations could be made. Using the example of the Hong Kong Cancer Registry, publicly accessible information lacks data on disease severity and ultimate treatment preferences. The absence of detail in publicly accessible data leaves much to be pondered by the public regarding how current health administrators can assess its resource distribution if the extent of the problem does not appear to have been outlined from the start.

Indeed, Hong Kong may lack expertise or resources in establishing data banking for diseases, and we support suggestions to liaise with other Asian communities to achieve this basic foundational requirement for public health assessment through pooling of resources. For rare diseases, the feasibility of collaborating with the Global Rare Diseases Patient Registry Data Repository in USA can also be considered.[3] With HA's world-renowned Computer Management System and the 18-month Pilot Study on Newborn Screening for Inborn Errors of Metabolism, we believe that HA already have the baseline requirements, and intention, to collect data. It is also important to engage the public's awareness of the disease burden faced locally by providing open access to local epidemiological data.

Regarding the role of stakeholders in medical care

As practitioners within HA, we believe that HA follow core values of evidence-based medical practice, rational use of public resources, targeted subsidy, opportunity cost consideration, and treatment safety, clinical efficacy and economic effectiveness in its policy design.[1,4] Our patients and families are increasingly educated and informed, and consequently it will be unreasonable to ask them to concede that HA does comply with its core values, solely in good faith, and especially when the outcome of HA's decisions undermine their expectations. It is realistically inevitable that individual expectations of medical treatment cannot be entirely satisfied by even the most refined public health care system, as resources are limited, and personal interests easily conflict with distributive justice. Whilst it is expected that HA needs time to evaluate the feasibility of including treatments into its drug formulary, and even more time to calculate the feasibility of reimbursement, it is difficult to expect the public to accept a lack of clear timeline, especially when they are facing imminent threats of disability or death at a personal level. Drug approval is a complicated process, involving many parties at a global, regional and local level. We know that responsible parties in HA work tirelessly in the evaluation process, but the public is not aware of the numerous considerations of administration for treatment that they perceive can potentially delay death or disability. One such example is that they may not understand that a substantial proportion of drugs may be marketed as effective, but "vary greatly in safety, efficacy and clinical responses to different patients [*of different characteristics, with the same broad diagnosis*]".[2]

We know that HA does prioritize approval of treatments for diseases in where there are no existing effective treatments, akin to fast-track marketing approval process used in other countries, albeit without an official designated labelling. Currently, the Drug Advisory Committee (DAC) of HA evaluates new pharmaceuticals for listing into the HA Drug Formulary. Although the outcome of these meetings can be retrieved, the discussion process remains opaque. In Australia, such discussions can be openly accessed as a video recording, and we highly recommend this approach. Discussions can alternately be printed verbatim and openly accessed, as a form of civil education. We understand that certain information, such as drug pricing negotiations, is expected to be confidential. However, we believe a certain degree of transparency regarding the decision process and the obstacles actively faced at each step by HA is crucial for stakeholders. Without a shared genuine understanding of all perspectives, there can be no dignified or effective discussion between all stakeholders. Thus, we believe increased transparency of the DAC evaluation process

is crucial to demonstrate that HA is sincere in its intent to maintain communication with patient advocate groups and the pharmaceutical industry, with the expectation of providing long-term sustainable and appropriate treatment. [4]

We agree that *the HA needs to accumulate more experience in the use of ultra-expensive drugs with a view to formulating appropriate long-term treatment protocols for patients (sic).*[2] The Hong Kong public, through their self-informed research, also asks our administration to allow public participation to brainstorm solutions. Through the continued commitment of our healthcare administrators and Legco, we hope that together, we can minimize the functional and productive days lost, and the suffering of our patients and their caregivers brought about by illness.

Yours sincerely,

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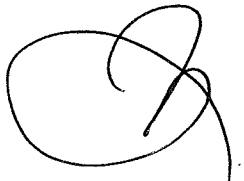

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