LCQ21: Spinal muscular atrophy

Following is a question by the Hon Michael Tien and a written reply by the Secretary for Health, Professor Lo Chung-mau, in the Legislative Council today (June 26):

Question:

It is learnt that spinal muscular atrophy (SMA) is a group of hereditary neuromuscular disorders and that the common forms of SMA can be classified into SMA type 1, type 2 and type 3. Some patient groups have indicated that the Hospital Authority (HA) has imposed restrictions on the eligibility for the drugs "Nusinersen" and "Risdiplam", which are for the treatment of SMA, under the Community Care Fund Medical Assistance Programmes (the Assistance Programmes), and that most of the SMA patients who can receive treatment under the Assistance Programmes are patients with SMA type 1 or type 2, resulting in a considerable number of other SMA patients being unable to apply for drug subsidies or undergo treatment for years. In this connection, will the Government inform this Council:

(1) whether it knows the number of persons who were granted subsidies under the Assistance Programmes for the purchase of (i) Nusinersen and (ii) Risdiplam and their average age in each of the past three years, and set out in the table below a breakdown by type of SMA suffered by persons who were granted subsidies (i.e. (I) type 1, (II) type 2 and (III) type 3) and by age group (i.e. (a) minors and (b) adults);

Year: _____

Drug	Age group	Number of persons who were granted subsidies		Average age of persons who were granted subsidies
ï¼ˆiï¼‰	ï¼ˆaï¼‰	ï¼ˆIï¼‰		
		ï¼ˆIIï¼‰		
		ï¼ˆIIIï¼‰		
	ï¼ˆbï¼‰	ï¼ˆIï¼‰		
		ï¼ˆIIï¼‰		
		ï¼ˆIIIï¼‰		
ï¼ˆiiï¼‰	ï¼ˆaï¼‰	ï¼ˆIï¼‰		
		ï¼ˆIIï¼‰		
		ï¼ˆIIIï¼‰		
	ï¼ˆbï¼‰	ï¼ˆIï¼‰		
		ï¼ˆIIï¼‰		
		ï¼ˆIIIï¼‰		

(2) given that the age limit for applying for the Assistance Programmes is not set out on HA's website, and in the reply to a question raised by a Member of this Council on the 12th of this month, the Government indicated that having reviewed the findings of a drug treatment study on Risdiplam and made reference to overseas medication guidelines and international arrangements on disease management and on subsidies for the drug concerned, the HA Expert Panel on SMA considers it inappropriate to subsidise drug treatment for adult SMA patients aged above 25 to receive drug treatments, whether the Government knows if the Assistance Programmes have set the same age limit for applying for Nusinersen; if they have, of the reasons for that; if not, the reasons for the difference in the eligibility criteria for these two types of drugs; and

(3) as some patient groups have indicated that the Mainland currently has no age limit in its medication standards for SMA patients, who are provided with drug subsidies for treatment regardless of the type of SMA they suffer from, and that some Mainland experts have found through their actual treatment experience that Nusinersen and Risdiplam could have significant efficacy on patients with SMA type 3, whether the Government knows the reasons for the discrepancy in the medication standards between HA and Mainland organisations?

Reply:

President,

In consultation with the Hospital Authority (HA), the consolidated reply to the question raised by the Hon Michael Tien is as follows:

(1) The Community Care Fund "Subsidy for Eligible Patients to Purchase Ultra-expensive Drugs (Including Those for Treating Uncommon Disorders)" (CCF UED Programme) subsidises needy and eligible patients to purchase ultra-expensive drugs, including the two drugs for the treatment of Spinal Muscular Atrophy (SMA), i.e. "Nusinersen" and "Risdiplam". The average amount of subsidy approved for each application under the CCF UED Programme was about $2 million in 2023-24.

The number of approved applications in the past three years (from 2021-22 to 2023-24) who started using the above two drugs, i.e. "Nusinersen" and "Risdiplam" after submitting the first application for drug use under the CCF UED Programme is shown in the following table:

		2021-22	2022-23	2023-24
Nusinersen				
Patients aged 18 or below	pre-symptomatic SMA	0	2	1
	infantile-onset SMA	4	4	3
	childhood-onset SMA	9	12	9

Patientsaged over 18	pre-symptomatic SMA	0	0	0
	infantile-onset SMA	0	0	0
	childhood-onset SMA	0	0	0
Total (mean age of the patients at the start of the treatment: 6)		13	18	13
Risdiplam (Note)				
Patients aged 18 or below	pre-symptomatic SMA	–	0	0
	infantile-onset SMA	–	0	1
	childhood-onset SMA	–	0	2
Patientsaged over 18	pre-symptomatic SMA	–	0	0
	infantile-onset SMA	–	0	0
	childhood-onset SMA	–	0	4
Total (mean age of the patients at the start of the treatment: 17)		–	0	7

Note: Covered under the CCF UED Programme since December 2022.

(2) and (3) The HA strives to provide all patients with optimal care and treatments, and has all along been supporting patients with uncommon disorders (including those with SMA) on various fronts, including clinical diagnosis, multi-disciplinary care and rehabilitation services. In addition, the HA has set up independent expert panels to formulate treatment guidelines for individual uncommon disorders and to assess the clinical efficacy of drugs for individual patients.

The most common forms of SMA can be classified as SMA Types I, II, and III, with SMA Type IV being less common. The classification of the types is based on the age of symptom onset and the highest motor milestone achieved. SMA Type I is the most common and severe form of SMA, with symptoms that are evident before 6 months of age. Without any treatment, the affected children cannot sit or stand unaided, and most of them succumb before the age of 2 due to respiratory failure. SMA Type II usually has symptom onset between 6 and 18 months of age. The affected children can sit without support but are unable to stand or walk unaided. Life expectancy is often reduced but most individuals live to reach adulthood. SMA Type III has symptom onset after 18 months of age up till adolescence. The affected children can sit, stand and walk on their own, but because of weak muscle strength, they walk limply. Their symptoms may deteriorate slowly to the point where they lose the ability to walk at adolescence, and most of them have a normal life expectancy.

The HA reviews medication criteria and clinical treatment guidelines in accordance with the principle of evidence-based medical practice and with reference to the clinical and scientific evidence of the drugs as well as overseas drug administration arrangements. Under the prevailing mechanism, patients with pre-symptomatic, infantile-onset (Type I) and childhood-onset (Types II and III) SMA may use the drugs "Nusinersen" and "Risdiplam" if they

meet specific clinical criteria and receive approval from the Expert Panel on SMA (the Expert Panel). Therefore, the current use of the above drugs in Hong Kong already includes patients with SMA who are pre-symptomatic, Types I, II and III.

The HA has been closely keeping in view the medication guidelines as well as arrangements on disease management and subsidies on the use of drugs for SMA patients in other places. The Expert Panel noted the findings of an international study published after adult patients with the disease had been treated with the drug "Nusinersen". The Expert Panel considered that, medically, the relevant scientific data and clinical evidence of the drug in question can so far only prove that the treatment effect is more pronounced in affected infants and children if treatment is started at an early age, while the empirical evidence showing that the use of the drug can bring significant benefits to patients with SMA who are treated in adulthood is still limited. Having made reference to overseas guidelines, arrangements on disease management and on subsidies for the drug concerned among different places internationally, the Expert Panel considers that it is not appropriate to subsidise adult SMA patients to receive drug treatments through the CCF UED Programme at this stage.

In addition to drug treatments, the support provided by the HA to adult patients with SMA also includes multi-faceted holistic healthcare services, such as clinical diagnosis and assessment, surgery, interventional therapy, palliative care, and rehabilitation services. For instance, in respect of rehabilitation services, the HA provides support that encompasses the assessment of home and work environments, wheelchair fitting, and the temporary lending of assistive devices to enhance patients' physical functionality and self-care abilities in the domestic setting, while alleviating the burden on family members or caregivers in taking care of patients.

The HA's Expert Panel will continue to closely monitor the development of drug administration and relevant clinical and scientific evidence on SMA in other places, and review the relevant treatment guidelines from time to time in accordance with the established mechanism, so as to ensure that clinical services and the use of drugs can keep pace with the latest development of medical technology and scientific evidence. The HA will also continue to adhere to the principles of prudent use of limited public resources while providing treatments to the greatest number of needy patients, and to provide subsidises through the CCF UED Programme in a timely manner to more SMA patients to whom drug treatments would show clinical efficacy. Meanwhile, the healthcare team specialising in the care of SMA patients will continue to optimise multi-disciplinary professional healthcare collaboration, so as to provide patients with comprehensive services, including drug and surgical treatments, palliative care and rehabilitation services.