



# FAMILIES OF SMA (SPINAL MUSCULAR ATROPHY) CHARITABLE TRUST 脊髓肌肉萎縮症慈善基金

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*By Letter & Email*

26<sup>th</sup> January, 2017

Dr Ko Wing-man BBS, JP  
Secretary for Food and Health of Hong Kong  
18/F., East Wing,  
Central Government Offices,  
2 Tim Mei Avenue,  
Tamar, Hong Kong

Dear Dr. KO,

I am writing on behalf of Families of Spinal Muscular Atrophy Charitable Trust ('FSMA'), my fellow members and their parents. FSMA was founded in 1998 by professionals from various business sectors and concerned parents of Spinal Muscular Atrophy (SMA) children, who understood so profoundly the sufferings of the SMA victims and families. SMA is the number one genetic killer of children under the age of 2, and it is a rare disease affecting muscle strength and movement. Our mission is to help the SMA victims and their families to cope with the devastation of SMA by providing emotional and financial support and to find an early treatment and cure of SMA. Currently, we have over 80 members with age ranging from 1 to 60.

SMA used to be a non-treatable genetic disease. Yet, we received recent news from the U.S. on 23<sup>rd</sup> December 2016, in which the U.S. Food and Drug Administration approved Spinraza (nusinersen). This is the first drug approved to treat children and adults with SMA. We are thrilled that amongst the three patients who were on drug clinical trial last year in Hong Kong, record of success was noted. Even though we are very delighted that it can give hope to our members and families, we are extremely disappointed with the huge price which amounts to US\$125,000 per injection. The per-injection price translates to US\$750,000 for the first year of use, as a patient needs to receive six injections. For each subsequent year in the rest of a patient's life, he or she needs to receive three injections which amount to US\$375,000. The cost is undeniably unaffordable to our members and most of the SMA patients.



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Our SMA members, in particular, their parents, are facing troubling times. On one hand they are delighted that SMA becomes a treatable disease, while on the other it is hard for them to fulfill their children's needs when the price of the drug is so high. The parents of SMA patients are worried that the high price will add another obstacle to their loved ones getting Spinraza. They feel guilty and angry as they can't do anything to help their children at the present moment. The general morale of both patients and their parents is at an all-time low.

As Hospital Authority (HA) is at the forefront in tackling key health issues and promoting improved wellness among the people of our city, the SMA patients all deserve the best treatment and individual attention. To our knowledge and according to the laws of Hong Kong, medicines must be registered with the Pharmacy and Poisons Board (PPB) prior to their sale in the market. HA has also implemented the Drug Formulary (the Formulary). Through standardization of policies on drugs and drug utilization, it ensures equitable access of patients to cost-effective drugs of proven safety and efficacy. In view of the urgent need of our SMA patients and the Government's commitment in ensuring no patient will forgo treatment because of financial limitations, we earnestly hope that you can exercise your power to speed up both processes so as to save our SMA patients and their families.

I am aware that you have a lot on your plate as Secretary for Food and Health, but as the Trustee of FSMA, I am asking you to find some ways in the budget to help the SMA patient. It is heart-breaking to hear from the SMA members and parents when they reveal their worries and disappointment. Patients and families had high expectations towards the treatment, and they believed that the medicine worth the wait. Yet the breaking news has turned their hopes into devastation, and it makes no difference to them with the presence of treatment.

I appreciate all that you do for our patients, and I look forward to seeing positive feedback. Thank you for listening. For discussion or further information, please feel free to contact Ms Tina WONG, Chief Operating Officer, at 2811 1767.

Sincerely yours,

  
Ruby WAN  
Trustee