香港罕見疾病聯盟
Hong Kong Alliance for Rare Diseases

Press Release **For Immediate Release**

Patients Alliance Urges HK Government to Squarely Face Medical Evidence and Treat Adult SMA Patients Promptly

(3 May 2020 – Hong Kong) The Hong Kong Government introduced treatment for Spinal Muscular Atrophy (SMA) more than two years ago. Only one adult SMA patient, Josy Chow, has been prescribed for treatment. Other adult patients still face an indefinite wait. The Food and Health Bureau has rejected patients' request for access on the grounds that, "There is no evidence to show a significant treatment efficacy among childhood-onset adult SMA patients." Yet, evidence exists. The Lancet, an authoritative medical journal, published an independent study in April 2020, demonstrating that an SMA treatment, Nusinersen, positively improves the motor function of adult patients, and is safe to use. Hong Kong Alliance for Rare Diseases (HKARD) urges the Government to squarely face study data and treat adult SMA patients.

Medical evidence proves SMA treatment is safe and effective in improving patients' mobility

The Lancet has recently published an independent study which has no funding support from any pharmaceutical company. Its results prove Nusinersen's efficacy in improving motor functions of adult SMA patients. The study was conducted in Germany and compares the differences between patients' conditions before and after treatment.

Major results are:

- 28% of patients had a clinically meaningful improvement at 6 months of treatment
- 35% of patients had a clinically meaningful improvement at 10 months of treatment
- 40% of patients had a clinically meaningful improvement at 14 months of treatment

No study subject had any serious treatment side effect. Among those who completed 14 months of treatment, the most commonly reported side effects were headache (35%), backpain (22%) and nausea (11%).

HKARD is concerned about their adult SMA members' disease progression. Many of their symptoms have worsened and their motor functions are declining. Most members are highly educated, have a successful career or are breadwinners – and they are deeply worried about losing their career and their ability to support their family, should their disease progress.

HKARD wrote to the Government in January 2020 to ask for early access to treatment for adult SMA patients, so that they can maintain their motor functions, improve self-care ability, continue to work

hard in their career and be self-reliant. On 22 April 2020, the Food and Health Bureau replied, rejecting HKARD's request based on the grounds that, "There is no evidence to show a significant treatment efficacy among childhood-onset adult SMA patients."

Mr. Tsang Kin Ping, President, Hong Kong Alliance for Rare Diseases said, "Most adult SMA patients are childhood-onset cases. They have overcome countless challenges brought by SMA. Without extraordinary determination and perseverance, they would not have achieved today's success. These patients have only a humble wish – to be treated, so that they can continue to work, take care of their family and contribute to society. In view of Josy's positive improvement and the new Lancet study which specifically addresses the safety and efficacy of a specific treatment for adult SMA patients, I strongly urge the Food and Health Bureau to squarely face the medical evidence and treat SMA patients."

Self-reliant patients contribute to society

SMA is characterised by loss of motor neurons in the spinal cord and lower brain stem, resulting in severe and progressive muscular atrophy and weakness. Ultimately, individuals with the most severe type of SMA can become paralyzed and have difficulty performing the basic functions of life, like breathing and swallowing. Most adult SMA patients have type 2 or 3 SMA and share a similar life expectancy with healthy people.

YAU Ka Man, Carmen, a registered social worker, NGAN Chiu Ming, a programmer, and HO Yuen Kei, a Hong Kong Boccia Athlete who is going to represent Hong Kong and compete in the Tokyo 2021 Paralympic Games, are all childhood-onset adult SMA patients. All of them wish to have access to treatment soonest so as to halt their muscle strength loss and maintain their current mobility.

When the Hong Kong Government introduced Nusinersen into Hong Kong in 2017, Mrs. Carrie Lam, Chief Executive of the HKSAR Government, visited Josy Chow and personally conveyed the good news to her. Currently, only 14 patients are treated with Nusinersen. Those who have access to treatment have enjoyed various positive improvements. For example, Josy has reduced the duration of ventilation support from the whole day to only at bedtime. She can breathe unaided during the daytime. From being completely unable to swallow anything, Josy can now swallow soft textured food like steamed egg and fish. These changes have brought significant improvements to her quality of life. She is the only adult patient to have received the treatment.

According to The Lancet, patients with SMA type 2 or 3 reach adulthood with varying levels of motor dysfunction and with slow but ongoing disease progression. This study provides evidence for the safety and efficacy of Nsinersen among a real-world cohort of adult SMA patients. Numerous patients showed clinically meaningful improvements in motor function and/or stabilisation of the disease, independent of age.

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About Hong Kong Alliance for Rare Diseases (HKARD)

The Hong Kong Alliance for Rare Diseases ("HKARD"), established in December 2014, is the first patients' group in Hong Kong comprising cross-rare-disease patients and their families with the support of experts and academics in the field. Its objectives are to spearhead and improve related policies and services, promote public education on rare diseases, and strengthen the community's support for patients, in order to ensure respect and protection for patients in terms of such fundamental rights as healthcare, social support, education and daily needs equal to other citizens.