

# Hong Kong SMA Symposium



香港神經肌肉疾病學會  
The Hong Kong Society of  
Neuromuscular Diseases

CLINICAL AND REAL-WORLD STUDIES SHOW



IS POSSIBLE

## NUSINERSEN HELPS PATIENTS DO MORE, COMPARED WITH SHAM-CONTROL AND NATURAL HISTORY<sup>1,2</sup>

Individual results may vary from patient to patient and based on progression of the disease and duration of therapy.

In pivotal randomised controlled trials, Nusinersen demonstrated clinically and statistically meaningful improvements in motor function compared with sham-control.<sup>1</sup>

In supportive and real-world studies, pre-symptomatic patients through adults improved relative to natural history.<sup>1,2</sup>



Date - 20 Aug 2025



Tang II, Sheraton Hong Kong Hotel & Towers at 20 Nathan Road, Kowloon

Time	Topic	Speaker	Chairperson & Moderator
18:30	Registration		
19:00-19:05	Opening	Dr. Gene Gao Queen Mary Hospital	
19:05-19:40	Meeting the Urgent Need for SMA Treatment: Unmet Challenges and Nusinersen Insights	Dr. Lauren Sanders St Vincent's Hospital, Melbourne Virtual	
19:40-19:55	Q&A	All	Dr. Gene Gao Queen Mary Hospital
19:55-20:30	Enhancing Long-Term SMA Management: Real-World Insights with Nusinersen	Dr. Yuh-Jyh Jong Kaohsiung Medical University Chung-Ho Memorial Hospital Virtual	
20:30-20:45	Q&A	All	
20:45	Closing	Dr. Gene Gao Queen Mary Hospital	

Register to Participate



Add to Calendar



- Two ways to attend : Virtual or Face-to-face (dinner will be provided)
- Enrollment by registration confirmation.

### Prescribing Information: SPINRAZA® (nusinersen) 12 mg/5ml solution for injection

Please refer to the PI for further information. **Indication:** For treatment of Spinal Muscular Atrophy (SMA). **Dosage and administration:** Treatment with Spinraza should only be initiated by a physician with experience in the management of spinal muscular atrophy (SMA). The decision to treat should be based on an individualised expert evaluation of the expected benefits of treatment for that individual, balanced against the potential risk of treatment with Spinraza. Patients with profound hypotonia and respiratory failure at birth, where Spinraza has not been studied, may not experience a clinically meaningful benefit due to severe SMN protein deficiency. Recommended dose is 12mg (5ml) per administration. Initiate as early as possible after diagnosis with 4 loading doses on day 0, 14, 28 and 63. A maintenance dose should be administered once every 4 months thereafter. If a loading dose is delayed or missed Spinraza should be administered as soon as possible, with at least 14 days between doses, and continue dosing at the prescribed frequency. If a maintenance dose is delayed or missed, Spinraza should be administered as soon as possible, and dosing continued every 4 months. Information on long term efficacy is not available. Continuation of therapy should be reviewed regularly and considered on an individual basis depending on the patient's clinical presentation and response to therapy. Spinraza is for intrathecal use by lumbar puncture. Treatment should be administered by health care professionals experienced in performing lumbar punctures. Spinraza is administered as an intrathecal bolus over 1 to 3 minutes, using a spinal anaesthesia needle. The injection must not be administered in areas of the skin where there are signs of infection or inflammation. It is recommended that the volume of cerebral spinal fluid (CSF) equivalent to the volume of Spinraza to be injected, is removed prior to administration of Spinraza. Sedation and imaging techniques may be required to aid administration, particularly in younger patients and in patients with scoliosis. Aseptic technique should be used when preparing and administering Spinraza. **Special populations:** Spinraza has not been studied in patients with renal impairment nor with hepatic impairment. **Contraindications:** Hypersensitivity to nusinersen or to any of the excipients such as sodium dihydrogen phosphate dihydrate, disodium phosphate, sodium chloride, potassium chloride, calcium chloride dihydrate, magnesium chloride hexahydrate, sodium hydroxide and hydrochloric acid (for pH adjustment) and water for injections. **Special warnings and precautions:** Lumbar puncture: Adverse reactions and complications (e.g., headache, backpain, vomiting). Thrombocytopenia and coagulation abnormalities, including acute severe thrombocytopenia, have been observed after administration of other subcutaneous or

intravenous antisense oligonucleotides. Appropriate testing is recommended prior to administration if clinically indicated. Renal toxicity observed after administration of other subcutaneous or intravenous antisense oligonucleotides. Appropriate testing is recommended prior to administration if clinically indicated. Further evaluation should be considered for persistent elevated urinary protein. Hydrocephalus not related to meningitis or bleeding has been reported. Some patients were implanted with a ventriculo-peritoneal shunt. In patients with decreased consciousness, evaluation for hydrocephalus should be considered. The benefits and risks of treatment maintenance/use of ventriculo-peritoneal shunt should be carefully considered. **Drug interactions:** No interaction studies have been performed. **Pregnancy and lactation:** As a precaution, avoid use in pregnancy. A benefit-risk evaluation of the use during breastfeeding should be undertaken. There are no data on the potential impacts on fertility in humans. Spinraza has no or negligible influence on the ability to drive and use machines. **Undesirable effects:** In clinical trials, the most commonly reported side effects were associated with lumbar puncture (headache, vomiting and back pain). Meningitis have been observed with unknown frequency from the post marketing setting. Communicating hydrocephalus, aseptic meningitis and hypersensitivity (e.g. angioedema, urticaria and rash) has also been reported. The incidence of treatment-emergent anti-drug antibodies (ADA) was low (4%), yet individual safety data for the treatment-emergent ADA-positive cases were received. See PI for full list of side effects.

Legal classification: Part 1, Schedule 1 & Schedule 3 Poison. Sale Requirement: Prescription only Medicines. Pack size: SPINRAZA 12 mg solution for injection: 1 box containing 1 vial. Registration number: HK-65896. Certificate Holder: Zuellig Pharma Limited, 5/F, Berkshire House, Taikoo Place, 25 Westlands Road, Quarry Bay, Hong Kong. Date of last revision of Prescribing Information: May 2021. **Adverse events should be reported to medinfohongkong@biogen.com.** **Additional information can be found in the package leaflet.**



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SMA=Spinal muscular atrophy.

Pictures depicted are inspired by real people living with SMA and are for illustrative purposes only.

**References:** 1. SPINRAZA™ Summary of Product Characteristics. 2. Coratti G, et al. Orphanet J Rare Dis. 2021;16:430.

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