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

Annual Scientific Meeting 2025 of The Hong Kong Society of Neuromuscular Diseases

7 December 2025 (Sun)

Program Book



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The Hong Kong Society of Neuromuscular Diseases
Annual Scientific Meeting 2025

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Welcome Message

Dear Colleagues and Friends,

On behalf of the Hong Kong Society of Neuromuscular Diseases (HKSNMD), I warmly welcome you to our Annual Scientific Meeting (ASM) 2025. I would also like to express my sincerely gratitude to the organizing committee for their dedicated effort in bringing this meeting to fruition.

This year, we have curated an engaging and comprehensive program for ASM 2025, featuring distinguished international and local experts who will share their insights and the latest advancements in neuromuscular diseases. Additionally, our case-sharing sessions will include interactive discussions, encouraging active participation from both speakers and attendees.

2025 is a particularly special year as we celebrate the 10th Anniversary of HKSNMD. I take great pleasure to congratulate all our members on reaching this significant milestone. We are excited to celebrate this occasion with all participants and guests at ASM 2025. We are grateful to have you with us to witness this memorable and meaningful moment of the society.

In conclusion, I wish the HKSNMD continued success and numerous accomplishments in the years to come. The fight against neuromuscular diseases remains a vital effort across clinical practice, research, hospital services, and community care both in Hong Kong and worldwide. I hope that this meeting will make a meaningful contribution toward our shared goals.



Dr Sophelia Chan
President
The Hong Kong Society of Neuromuscular Diseases

Organizing Committee of The Hong Kong Society of Neuromuscular Diseases Annual Scientific Meeting 2025

Chair:
Dr Sophelia Chan

Committee Members (in last names' alphabetical orders)
Ms Nerita Chan
Dr Mark Cheung
Dr Michael Fu
Dr Sharon Fung
Dr Gene Gao
Dr Hencher Lee
Dr June Wong
Dr Winnie Wong

Council of The Hong Kong Society of Neuromuscular Diseases

(In last names' alphabetical orders)	
President	**Dr Sophelia Chan**
Vice-President	**Dr Amanda Kan**
Hon Secretary	**Dr Sharon Fung** **Dr Gene Gao**
Hon Treasurer	**Dr Mark Cheung**
Council Members	**Ms Nerita Chan** **Dr CN Lee** **Dr Hencher Lee** **Dr June Wong** **Dr Winnie Wong**
Council Co-opted Members	**Dr Sandy Cheng** **Dr Michael Fu** **Dr Shun Wong** **Prof Ella Yeung**

List of Invited Speakers *(In last names' alphabetical orders)*



Dr Gene Gao
Associate Consultant
Queen Mary Hospital
Hong Kong SAR



Dr Blaz Koritnik
Head of the Institute of Clinical Neurophysiology
The University Medical Centre Ljubljana
Slovenia



Prof Anna Kostera-Pruszczyk
Department of Neurology
Medical University of Warsaw
Poland



Dr Min-Yu Lan
Attending Doctor
Department of Neurology, Kaohsiung Chang Gung Memorial Hospital
Taiwan



Prof Jan Lünemann
Professor of Neurology
University of Münster
Germany



Dr Rodrigo de Holanda Mendonça
Neurologist
Hospital das Clínicas of FMUSP
Brasil

Scientific Program

Time	Program	Speaker	Chairperson
09:00 - 09:15	**Opening Remarks**	*Dr Sophelia Chan* *President of HKSNMD*	
09:15 - 10:00	**Symposium 1** *(Sponsored by AstraZeneca)* A New Era in Generalized Myasthenia Gravis: How Targeted Complement Inhibition Improves Patient Outcomes	*Prof Jan Lünemann (Germany)*	*Dr Michael Fu* *Dr Hencher Lee*
10:00 - 10:30	**Case Discussions 1*** 3 Local case presentations	*Dr Timothy Yeung (QMH, HK)* *Dr Iris Tang (HKU, HK)* *Dr WY Lau (KWH, HK)*	
10:30 - 10:50	**Tea Break 1**		
10:50 - 11:45	**Symposium 2** *(Sponsored by Biogen)* Antisense Oligonucleotide Therapy in Amyotropic Lateral Sclerosis	*Dr Gene Gao (HK)*	*Dr June Wong*
	What Meaningful Change Looks Like for Adults Living with SMA?	*Prof Anna Kostera-Pruszczyk (Poland)* *(Virtual Presentation)*	
11:45 - 12:30	**Lunch Time / Case Discussions 2*** 1 Local case presentation	*Dr Nelson Mak (PMH, HK)*	
12:30 - 13:15	**Symposium 3** *(Sponsored by Novartis)* SMA Today and Tomorrow: The Potential for Intrathecal Gene Therapy in Closing Current Treatment Gaps	*Dr Rodrigo de Holanda Mendonça (Brasil)*	*Dr Sharon Fung* *Dr Shun Wong*
13:15 - 13:30	**Case Discussions 3*** 1 Local case presentation	*Dr Lisa Au (CUHK, HK)*	
13:30 - 14:15	**Symposium 4** *(Sponsored by Roche)* How the Early Diagnosis of NMDs led to Better Care and Accessibility to Treatment with Case Illustrations	*Dr Blaz Koritnik (Slovenia)*	*Dr Mark Cheung* *Dr Sandy Cheng*
14:15 - 14:45	**Case Discussions 4*** 3 Local case presentations	*Dr Anthony Leung (QMH, HK)* *Dr Nicholas Fok (TMH, HK)* *Dr Samuel Hui (KWH, HK)*	
14:45 - 15:00	**Tea Break 2**		
15:00 - 15:45	**Symposium 5** *(Sponsored by Medison Pharma)* Hereditary Transthyretin Amyloidosis – More than Just A Peripheral Neuropathy	*Dr Min-Yu Lan (Taiwan)*	*Dr Winnie Wong* *Dr CN Lee*
15:45 - 16:15	**Case Discussions 5*** 3 Local case presentations	*Dr Jonathan Chu (Private)* *Dr Sandy Cheng (Gleneagle Hospital HK)* *Dr HW Cheung (UCH, HK)* *Dr Florinda Chu (QMH, HK)*	
16:15 - 16:30	**Closing Remarks**	*Dr Sophelia Chan* *President of HKSNMD*	

***Please refer the next page for local case presentation details**

Case Discussions Details

Time	Topic	Speaker
10:00 -10:30	**Case Discussions 1**	
	Solving a Diagnostic Conundrum with Genetic Analysis : A Case of Familial Weakness	*Dr Timothy Yeung (QMH, HK)*
	Anti-synthetase Syndrome Myopathy	*Dr Iris Tang (HKU, HK)*
	A Lump on the Limb	*Dr WY Lau (KWH, HK)*
11:45 - 12:00	**Case Discussions 2**	
	Difficult IT access in a SMA patient	*Dr Nelson Mak (PMH, HK)*
13:15 - 13:30	**Case Discussions 3**	
	A Young Woman with Weakness of the Legs	*Dr Lisa Au (CUHK, HK)*
14:15 - 14:45	**Case Discussions 4**	
	From MSA to DM : Unravelling Diagnostic Challenges in Neuromuscular Disorders	*Dr Anthony Leung (QMH, HK)*
	A Young Woman with Breathlessness	*Dr Nicholas Fok (TMH, HK)*
	Is It Neuropathy or Myopathy	*Dr Samuel Hui (KWH, HK)*
15:45 - 16:15	**Case Discussions 5**	
	A Case with Refractory CIDP in a Renal Dialysis Patient	*Dr Jonathan Chu (Private)* *Dr Sandy Cheng (Gleneagle Hospital HK)*
	Adult-Onset CMTX1 Due to the Non-Coding GJB1 c.-103C>T Variant : A Case and Cohort Observation from Hong Kong	*Dr HW Cheung (UCH, HK)*
	A Man with Slowly Progressive Unsteadiness	*Dr Florinda Chu (QMH, HK)*

Abstracts

A New Era in Generalized Myasthenia Gravis: How Targeted Complement Inhibition Improves Patient Outcomes

Prof Jan Lünemann
Professor of Neurology
University of Münster
Germany

Generalized myasthenia gravis (gMG) is an autoimmune disorder of the neuromuscular junction marked by fluctuating, fatigable weakness driven by pathogenic autoantibodies—most commonly against the acetylcholine receptor (AChR)—that trigger classical complement activation, postsynaptic membrane injury, and impaired transmission. The disease poses huge burden on patients, impairing their daily activities, quality of life and ability to work.

While corticosteroids, conventional immunosuppressants, IVIg, plasma exchange, and thymectomy remain foundational, substantial unmet needs persist. Patients may still experience uncontrolled symptoms or MG crisis despite these treatment. In recent years, there are newer biologic therapies available, bringing a better care for MG patients.

In this lecture, Prof. Jan Lünemann will examine how targeted complement inhibition is reshaping gMG management by directly interrupting complement-mediated damage at the motor endplate. He will also share patient cases to illustrate how complement inhibitor could fit in the treatment paradigm and improve patient management.

Abstracts

Antisense Oligonucleotide Therapy in Amyotropic Lateral Sclerosis

Dr Gene Gao
Associate Consultant
Queen Mary Hospital
Hong Kong SAR

The presentation by Dr. Gene Gao highlights the real-world experience of treating SOD1-associated amyotrophic lateral sclerosis (ALS) in Hong Kong using antisense oligonucleotide therapy. It covers the genetic landscape of ALS, local diagnostic practices, and the clinical use of tofersen. Preliminary data suggest that tofersen may help stabilize disease progression in selected patients. The talk also discusses challenges in treatment timing and emphasizes the importance of multidisciplinary care and early diagnosis of neuromuscular diseases (NMDs).

Abstracts

What Meaningful Change Looks Like for Adults Living with SMA?

Prof Anna Kostera-Pruszczyk
Department of Neurology
Medical University of Warsaw
Poland

The presentation by Professor Anna Kostera-Pruszczyk shares real-life experiences and long-term outcomes of teenagers and adults living with spinal muscular atrophy (SMA), a rare genetic condition that affects muscle strength and movement. Using data from Polish registry and case studies, the talk highlights how people with different types of SMA manage their daily lives and how treatment with nusinersen has helped improve their mobility and independence. The presentation also discusses the safety of the treatment and emphasizes the importance of ongoing care and support for people with SMA as they grow older.

Abstracts

SMA Today and Tomorrow: The Potential for Intrathecal Gene Therapy in Closing Current Treatment Gaps

Dr Rodrigo de Holanda Mendonça
Neurologist
Hospital das Clínicas of FMUSP
Brasil

Intrathecal gene therapy is redefining treatment options for older patients with spinal muscular atrophy (SMA), whose needs remain incompletely met by current therapies. As newborn screening and early intervention reshape SMA's natural history, clinicians encounter increasingly diverse, evolving phenotypes in adolescents and adults, often with prior exposure to disease-modifying treatments. In the presentation, Dr. Rodrigo Holanda will address unmet needs, characterize changing phenotypes, and summarize emerging efficacy and safety data on intrathecal onasemnogene abeparvovec. He will explore realistic expectations for outcomes, implications for long-term management, and how intrathecal gene therapy may integrate into multidisciplinary, lifespan SMA care pathways.

How the Early Diagnosis of NMDs led to Better Care and Accessibility to Treatment with Case Illustrations

Dr Blaz Koritnik
Head of the Institute of Clinical Neurophysiology
The University Medical Centre Ljubljana
Slovenia

The presentation by Dr. Blaz Koritnik aims to enhance the diagnostic skills of neurologists, focusing on the differential and early diagnosis of several neuromuscular diseases: Amyotrophic Lateral Sclerosis (ALS), Pompe disease, and Spinal Muscular Atrophy (SMA). The session will be divided into two main parts. The first section will cover the diagnostic approaches for those conditions, while the second will specifically delineate the features and methods needed to accurately distinguish between each disease. The talk will utilize case examples to illustrate key diagnostic principles in a practical context.

Abstracts

Hereditary Transthyretin Amyloidosis – More than Just A Peripheral Neuropathy

Dr Min-Yu Lan (Taiwan)
Kaohsiung Change Gung Memorial Hospital and Chang Gung University
College of Medicine, Kaohsiung
Taiwan

Hereditary transthyretin amyloidosis (hATTR) is an inherited and progressive disease caused by mutations in the TTR gene, leading to the production of misfolded variant transthyretin protein and the deposition of its amyloid fibril in various tissues including nerves. Peripheral neuropathy related to hATTR is the most common form of familial amyloid polyneuropathy (FAP). Characterized by a progressive, length-dependent polyneuropathy, hATTR-FAP leads to neurological dysfunction with sensory loss, paresthetic pain, muscle atrophy and weakness.

However, hATTR is a multisystemic disease, with peripheral neuropathy being a prominent but not the sole clinical manifestation. The autonomic nerve system and the heart are often affected. Infiltrative cardiomyopathy resulting from cardiac deposition of transthyretin amyloid may cause heart failure and cardiac rhythm or conduction abnormalities. Autonomic dysfunction in hATTR may present with orthostatic hypotension, gastrointestinal dysmotility and genitourinary dysfunction. Sometimes the kidneys, the ocular vitreous and the meninges may be involved. It should be noted that the clinical presentation including onset ages and phenotypic constellation may vary among TTR mutations, probably ascribed to variance in the stability of composed transthyretin tetramers.

Systemic involvement of hATTR has major impact on quality of life of the patients and is strongly associated with morbidity and mortality. Acknowledgement of the multisystemic nature is not only the key diagnostic clue for differentiating hATTR-FAP from other etiologies of peripheral neuropathy, but also mandates a comprehensive, multidisciplinary approach to care, including specialized pharmacological treatment and ongoing monitoring of various organ systems. The advances in disease-modifying treatment, in conjunction with the integrated specialty care, may optimize quality of life and long-term outcome for the patients with hATTR.

Solving a Diagnostic Conundrum with Genetic Analysis : A Case of Familial Weakness

Dr Timothy Yeung
Queen Mary Hospital
Hong Kong SAR

A 27-year-old lady presented with episodic proximal upper limbs and neck weakness starting from 10 years of age. The patient's elder sister and father also experienced episodic weakness.

Physical examination noted multidirectional ophthalmoplegia, ptosis, winging of scapula, and symmetrical proximal upper limb fatigable weakness (MRC grade 4/5). Reflexes, sensation, coordination and gait were unremarkable.

In view of strong family history, myopathic findings, and winging of scapula, inherited conditions including limb girdle muscular dystrophy, facioscapulohumeral muscular dystrophy were suspected.

Germline genetic testing for neuromuscular gene panel by next generation sequencing was conducted, which identified heterozygous NM_000079.4 (CHRNA1):c.737C>T p.(Ser246Phe), a likely pathogenic variant.

The detection of a likely pathogenic CHRNA1 variant, in vitro functional data, and characteristic electrophysiology findings established the diagnosis of CHRNA1-related slow-channel congenital myasthenic syndrome in the patient.

The patient was started on Fluoxetine 40mg Daily, and was noted to have full power and no ophthalmoplegia upon follow up 1 month later, with sustained improvement during subsequent follow ups.

Anti-synthetase Syndrome Myopathy

Dr Iris Tang
The University of Hong Kong
Hong Kong SAR

Demographic: F/55. Non-smoker non-drinker. Works in bank.

Past medical history:
1. Stage I CA Left breast with mastectomy and RT, on letrozole
2. Hyperlipidaemia

HPI:
Insidious onset of elevated CK upon routine blood test in general out-patient clinic
CK: 263 in 3/2024 from baseline 157 (7/2023) and myalgia. Stopped atorvastatin but CK still increasing trend to 1059 in 8/2024.
No skin rashes
Also with one year history of dry cough
Low-dose CT in private showed bilateral lower zone sub-pleural reticular shadow and minimal bronchiectasis, plan for CT monitoring

Admitted to medical ward in 10/2024 for increasing myalgia and dysphagia. New onset Raynaud's phenomenon
Palpitations+

PE:
Shoulder abduction 4/5. Knee flexion 4/5
Mechanic's hands
No synovitis.
No gottron's papule / heliotrope rash

Ix:
CK :2217
MSA: anti-PL7 and anti-Ro52 positive
EMG early recruitment, no fib / fasciculation. Low amplitude polyphasic MUAP
Lung function: FVC 1.77 64%, FEV1 1.42 64% DLCO/VA 3.22 70%
Repeat HRCT: Reticular densities and patchy GGO, in favour of NSIP PET-CT: interstitial lung changes, mild hypermetabolic LN at mediastinum MRI heart: marginal elevated T1 value of myocardium

Diagnosis: Anti-synthetase syndrome (PL7+/Ro52+) with myositis, ILD, mechanic's hands, raynaud's phenomenon +/- cardiac involvement

Treated with IVIg due to bulbar symptoms, started Prednisolone 1mg/kg/day
Followed by rituximab 1g x 2 with good clinical response

Discussion point: - Recognition of extra-muscular features in anti-synthetase syndrome - Treatment of Anti-synthetase syndrome associated ILD

A Lump on the Limb

Dr WY Lau
Kwong Wah Hospital
Hong Kong SAR

Summary: This is a 50yrs lady complained of lumps over calf since 2022 progressively increase in size and numbers. Initially worry about sarcoma and she has been referred to ortho tumor clinic. Subsequent biopsy shows non-caseating granuloma and bronchoscopy with EBUS has been performed with LN biopsy also showing granuloma. Diagnosis of sarcoidosis was then substantiated and Prednisolone has been started. The case pathological slides will be presented and brief discussion on granulomatous myositis will be follow.

Abstracts for Case Discussions 2

Difficult IT access in a SMA patient

Dr Nelson Mak
Princess Margaret Hospital
Hong Kong SAR

We present a case of spinal muscular atrophy Type II in a 14-year-old female patient. She was born in Baptist Hospital with an uneventful antenatal course. She presented with bilateral lower limb weakness at 5 months of age, and was diagnosed to have spinal muscular atrophy at 6 months. Genetic investigations confirmed Exon 7 deletion with 3 copies of SMN2. She was previously followed up in Queen Elizabeth Hospital, with multiple episodes of respiratory failure during intercurrent illnesses, requiring intensive care support. Nocturnal non-invasive positive pressure ventilation was initiated at 6 years. She was enrolled into the HK Paediatric SMA treatment program, starting nusinersen at 8 years of age initially in the Queen Mary Hospital. After nusinersen treatment, her motor function has improved, and her respiratory performance has been well maintained. She also has much less hospitalizations due to intercurrent illness. As she developed progressive severe kyphoscoliosis, she required posterior spinal fusion at 12 years. After the spinal surgery, her motor performance fluctuated. She preferred to continue nusinersen treatment. The lumbar puncture for the intrathecal nusinersen was guided by 3D dynamic CT guidance. We noted increasing difficulty in lumbar puncture in recent attempts for intrathecal therapy. We hope to discuss the value of a portacath insertion for easier access in future administrations of intrathecal Nusinersen, and the potential benefit of using higher dose of nusinersen in her case.

Abstracts for Case Discussions 3

A Young Woman with Weakness of the Legs

Dr Lisa Au
The Chinese University of Hong Kong
Hong Kong SAR

A 38-year-old lady was admitted for insidious onset and progressive weakness of lower limbs for 3 years. She had first-trimester miscarriage 3 years ago after a traffic accident when she was nearly hit by a taxi and she fell back. She felt her lower limbs' strength were decreased after the accident, but she was still able to walk unaided with normal gait. Condition started to deteriorate after delivery of her second pregnancy, when husband noticed limping gait and slowness in movements.

She thought her symptoms were the sequelae of the traffic accident which caused musculoskeletal problem to her. Thus, she sought help from various private orthopedic surgeons and neurologists with multiple investigations performed. MRI brain and whole spine, nerve conduction test were all normal. She began to seek help from chiropractor and traditional Chinese medical practitioner, and she felt her motor symptoms improved with treatment.

On the day of admission, she complained of pain over her right arms and legs after bloodletting treatment by traditional chinese medical practitioner. She was worried about stroke and so she attended A&E. Physical examination showed full limbs power and normal cranial nerves, but prominent lower limb hypertonia and hyperreflexia. She was able to volunteer history herself but was noted to be mentally slow. Blood tests including autoimmune markers, anti-aquaporin 4 and anti-MOG were negative. CSF examination showed no signs of infection or inflammation. HK-MoCA scored 21/30. Concerning her family history, she reported for mother was diagnosed Parkinson's disease in her 50s and her elder sister was diagnosed bipolar affective disorder in her 50s.

She was initially suspected to have hereditary spastic paraplegia. HSP panel test was arranged but did not reveal any positive molecular findings. Her condition continued to deteriorate with increasing parkinsonism, expressive dysphasia and dysarthria. Further detailed family history taking revealed her mother suffered from concurrent cognitive impairment and parkinsonism in her 50s, and her elder sister was diagnosed early onset dementia with behavioral and psychological symptoms in her 50s. ALS and FTD panel test was arranged and revealed a heterozygous likely pathogenic variant in PSEN1.

From MSA to DM : Unravelling Diagnostic Challenges in Neuromuscular Disorders

Dr Anthony Leung

Queen Mary Hospital
Hong Kong SAR

Background
The association between anti-transcriptional intermediary factor 1 gamma (anti-TIF1-γ) autoantibody positivity and cancer-associated dermatomyositis (DM) is well recognized. We present a case of anti-TIF1-γ dermatomyositis that developed two years after the remission of malignancy.

Case Presentation
A 78-year-old Chinese woman presented with a two-year history of recurrent falls and progressive dysphagia. She had a history of bilateral metachronous breast cancer, diagnosed in 2000 and 2020, respectively, for which the disease was in remission since 2021 after completion of adjuvant chemotherapy and mastectomy. Since 2023, she experienced progressive weight loss, unsteady gait, and repeated falls. Due to generalized muscle rigidity and bradykinesia, multiple system atrophy-parkinsonian type (MSA-P) was suspected, and dopaminergic agents were ineffective. The patient's condition further deteriorated within 1 year, leading to progressive dysphagia and the need for percutaneous endoscopic gastrostomy for nutritional support. The later emergence of heliotropic rash on the face, Gottron's papules and mild proximal myopathy 3 months after the onset of rapidly progressive dysphagia supported the diagnosis of dermatomyositis. Laboratory tests revealed positive serum anti-TIF1-γ antibodies, and electromyography along with muscle biopsy confirmed the diagnosis. Notably, creatine kinase (CK) levels were within normal limits. Following treatment with intravenous immunoglobulin, the initiation of oral prednisolone and mycophenolate mofetil, the patient exhibited a gradual improvement in muscle strength. A positron emission tomography scan repeated showed no evidence of malignancy recurrence.

Conclusion
This case illustrates the possibility of normal CK levels in patients with anti-TIF1-γ-associated dermatomyositis. Clinicians should maintain a high level of suspicion, particularly in patients presenting with rapidly progressive dysphagia and a history of malignancy.

Abstracts for Case Discussions 4

A Young Woman with Breathlessness

Dr Nicholas Fok
Tuen Mun Hospital
Hong Kong SAR

Ms. Lee, a previously healthy 35-year-old non-smoker, was referred from a general practitioner to the emergency department for bilateral lower limb edema and low peripheral oxygen saturation.

Exertional breathlessness, orthopnea, mild sinus tachycardia, and apparent bilateral proptosis initially raised concern for thyroid storm with heart failure.

Baseline investigations—including blood tests, chest X-ray, and electrocardiogram—were unremarkable. Arterial blood gas analysis, however, revealed severe decompensated type II respiratory failure with metabolic compensation, necessitating ICU admission and non-invasive ventilation.

The subsequent identification of proximal limb weakness and variable bilateral ptosis shifted the working diagnosis to myasthenic crisis. Despite treatment with intravenous immunoglobulins, her condition deteriorated—marked by reduced consciousness and severe dysphagia—prompting intubation, plasma exchange, and high-dose corticosteroids.

Ms. Lee responded well over the following week and was transferred to the neurology ward, where a detailed history uncovered critical longstanding features: bilateral ptosis since childhood; insidious shoulder and back muscle wasting over a decade; inability to lift her torso during yoga for the past four years; and, over the preceding six months, progressive neck flexion weakness and exertional dyspnea. Symptoms had remained subtle enough that she was preparing for an overseas vacation at the time of admission. She had visited her GP solely to request diuretics for anticipated flight-related lower limb edema.

Neurological examination confirmed fixed partial ptosis with preserved saccades, mild neck and limb-girdle weakness, scapular winging, deltoid and supraspinatus atrophy, generalized hyporeflexia, and fatigable upper limb strength.

Extensive testing—including autoimmune (AChR and MuSK antibodies), endocrine (thyroid function, HbA1c), metabolic (vitamin B12, folate, thiamine, lactate), and toxicology screens—was unremarkable. Electrophysiology showed myopathic changes without neuromuscular junction dysfunction. Lower limb MRI revealed T2-hyperintensities in the posterior and lateral thigh compartments and soleus muscles. Muscle biopsy revealed ragged red fibers and COX-negative fibers. Genetic testing identified the pathogenic mitochondrial DNA variant m.3243A>G in MT-TL1, confirming a diagnosis of mitochondrial myopathy.

Ms. Lee was tapered off the immunotherapy, achieved good functional recovery, and remained stable on home nocturnal BiPAP without recurrent respiratory failure.

This case illustrates an atypical neuromuscular syndrome: respiratory failure was the dominant, initial manifestation—preceding significant limb weakness—contrasting with the classic neuromuscular disease trajectory in which progressive, disabling limb weakness culminates in late respiratory involvement.

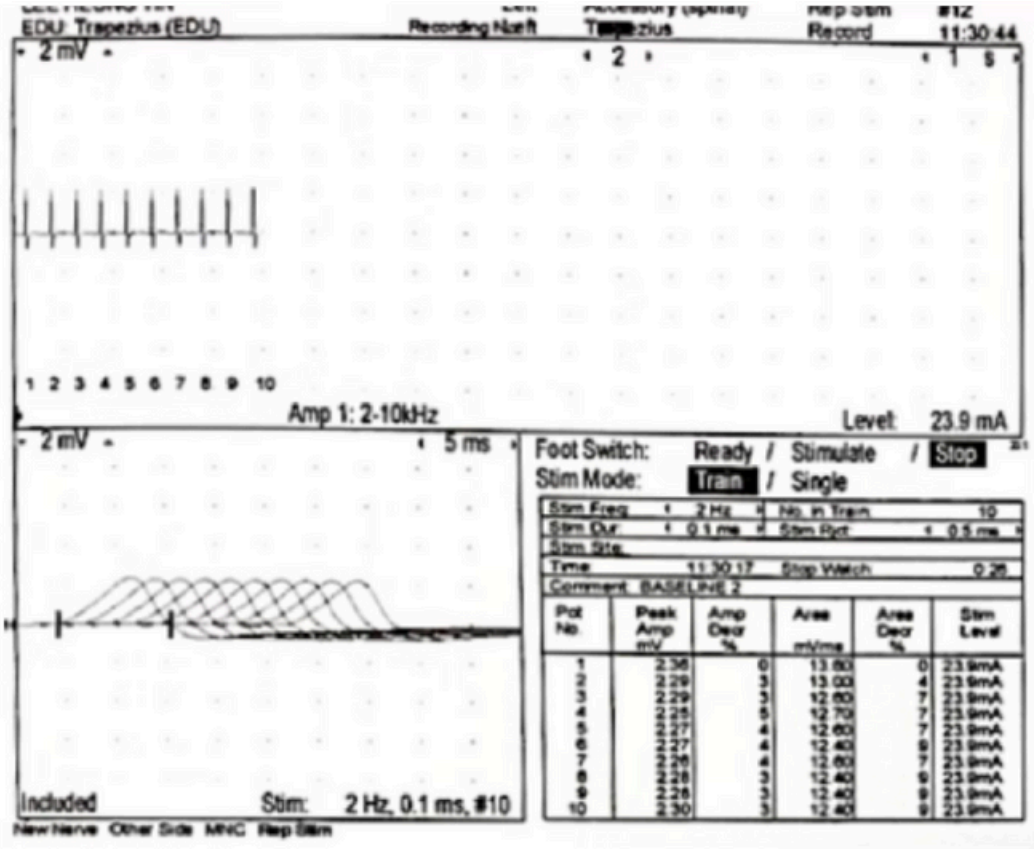


Figure 1: Normal repetitive nerve stimulation.

Abstracts for Case Discussions 4

Is It Neuropathy or Myopathy

Dr Samuel Hui
Kwong Wah Hospital
Hong Kong SAR

78-year-old gentleman with known DM, HT, lipid and gout, complained of progressive 4 limb distal numbness, bilateral hand clumsiness, unsteady gait, dysphagia and episodes of muscle spasms and vocal cord spasms. P/E found LMNL type of weakness +/- fasciculations. Blood tests found ANA 320, anti-Ro positive. anti-ganglioside Ab/ anti-achR -ve. NCS showed no significant neuropathy. Anti-neuronal Ab, pareneoplastic Ab all -ve. Myositis specific Ab: anti-Ro52 equivocal, other Ab -ve. In view of worsening of symptoms, a course of IVIG was given, and patient showed improvement afterwards. Muscle biopsy was also done and showed mild fibre atrophy, focal fibre degeneration and mild inflammatory infiltrate present. In view of clinical response to IVIG, he is now on regular IVIG infusion. Imaging was also booked for malignancy screening. Current working diagnosis is inflammatory neuropathy/ myositis responding to IVIG.

A Case with Refractory CIDP in a Renal Dialysis Patient

Dr Jonathan Chu
Private

Dr Sandy Cheng
Gleneagles Hospital Hong Kong
Hong Kong SAR

Unmasking a Novel Trigger: Peritoneal Dialysis as a Potential Initiator of Chronic Inflammatory Demyelinating Polyneuropathy?

Uremic neuropathy is the most common neurologic complication of end-stage renal disease (ESRD) patients. On contrary, Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) specifically related to peritoneal dialysis (PD) has been seldom reported before but gaining attention.

Here we present the case of a 40s-year-old male with end-stage renal disease (ESRD) secondary to IgA nephropathy for two decades. Four years ago, he commenced peritoneal dialysis (PD) following a rise in creatinine to the 600s (μmol/L). Notably, 1-2 months after initiating PD, he developed progressive bilateral lower limb weakness and numbness. His condition deteriorated over the ensuing year, rendering him chairbound.

Neurophysiological studies revealed a severe sensorimotor polyneuropathy with unequivocal demyelinating features, consistent with CIDP. Cerebrospinal fluid analysis was normal, while a subsequent nerve biopsy confirmed primary demyelination. He demonstrated a partial response to intravenous immunoglobulins (IVIG) and a more robust, significant improvement with corticosteroid therapy. The temporal relationship strongly suggested PD as a potential trigger for this secondary CIDP.

A clinical turn occurred when he was switched to haemodialysis and subsequently underwent a successful renal transplantation. Following transplantation, his neurological function improved remarkably, and he regained the ability to walk unaided, albeit slowly. This case highlights PD as a potential precipitant for CIDP, a phenomenon described in limited case reports. It underscores the importance of recognizing this treatable complication in dialysis patients and suggests that renal transplantation may offer the best chance for profound and sustained neurological recovery.

Adult-Onset CMTX1 Due to the Non-Coding GJB1 c.-103C>T Variant : A Case and Cohort Observation from Hong Kong

Dr HW Cheung
United Christian Hospital
Hong Kong SAR

Background:
The GJB1 c.-103C>T 5′UTR variant is a non-coding mutation causing X-linked Charcot–Marie–Tooth disease (CMTX1), likely constituting the largest proportion of GJB1 neuropathies in Hong Kong.

Case Presentation:
A 36-year-old woman presented with progressive bilateral hand weakness and distal limb wasting, later requiring bilateral ankle–foot orthoses. Examination revealed distal symmetrical weakness and hyporeflexia. Her father and younger sister were similarly affected, suggesting X-linked inheritance.

Investigations:
Nerve conduction studies showed a demyelinating neuropathy with an ulnar-sparing pattern— compound muscle action potentials were elicited in the ulnar but not in the median nerve. Sural nerve biopsy confirmed demyelination. Initial GJB1 exon sequencing was negative; subsequent noncoding analysis detected the pathogenic c.-103C>T variant.

Cohort Findings:
Among 24 primary patients identified with the same variant in a local cohort, about half demonstrated ulnar sparing, and most had median motor conduction velocities below 38 m/s, with some slightly above this threshold.

Conclusion:
The GJB1 c.-103C>T variant represents a prevalent local cause of CMTX1 in Hong Kong. This highlights the importance of sequencing of the non-coding regions of this gene.

Abstracts for Case Discussions 5

A Man with Slowly Progressive Unsteadiness

Dr Florinda Chu
Queen Mary Hospital
Hong Kong SAR

This patient is a 63 year old man, who presented with progressive four limb numbness and weakness for over 10 years. He has a known history of IgM MGUS, with nerve conduction studies in 2016 showing distal demyelinating changes.

The numbness has further deteriorated since mid 2024, with numbness, unsteady fait and easy falls. Physical examination demonstrated reduced pinprick sensation from bilateral mid forearm and hands, bilateral hyperalgesia up to mid dorsal foot. There is generalized hyporeflexia of bilateral upper and lower limbs.

Nerve conduction studies in 8/2025 showed sensorimotor demyelinating polyneuropathy, with interval deterioration compared with previous parameters.

Further investigations show plasma M-protein IgM kappa positive. Anti-MAG antibiotic came back >70,000 BTU. Hence the diagnosis of anti-MAG PN can be substantiated.

Patient was then started on Rituximab, and has just completed a course of Rituximab of 4 weekly doses, and plan for follow up with monitoring of symptoms.

Acknowledgements

This Conference is sponsored by:

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ZOLGENSMA® is a single-dose IV gene therapy designed **to address the genetic root cause of SMA**[1]

Across 5 clinical studies*, and supported by real-world evidence†, ZOLGENSMA® has demonstrated[2–10]:

Unprecedented survival[2–7,11]

Improved bulbar function[2,4,5,8,9,12]

Rapid and sustained improvements in motor function[2–10]

Achievement and maintenance of key motor milestones[2–8,12]



ZOLGENSMA® is indicated for the treatment of patients with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the *SMN1* gene and a clinical diagnosis of SMA Type 1, or patients with 5q SMA with a bi-allelic mutation in the *SMN1* gene and up to 3 copies of the *SMN2* gene.[1]

* Data from the completed Phase 1 START trial, the Phase 3 STR1VE-US, STR1VE-EU, and SPR1NT trials, and the ongoing START LTFU therapeutic-dose cohort (n=10), as of the June 11, 2020 data cut.[3–7]

† Data from a real-world observational study of ZOLGENSMA® treatment in 76 older and heavier patients with SMA (mean age at infusion was 16.8 months [range, 0.8–59.0 months], and the mean weight was 9.1 kg [range, 4.0–15.0 kg]), including those who previously received nusinersen (Weiß C, *et al.* 2021).[8] Data from a real-world, retrospective study of ZOLGENSMA® in 21 patients infused at 1–23 months of age in Ohio (Waldrop MA, *et al.* 2020); and a retrospective chart review of 7 patients older than 7 months treated with ZOLGENSMA® at the Children's Hospital of Philadelphia between July 2019 and February 2020 (Matesanz SE, *et al.* 2021).[9,10]

IV=intravenous; LTFU=long-term follow-up; SMA=spinal muscular atrophy; SMN=survival motor neuron.

References: 1. Novartis. ZOLGENSMA® (onasemnogene abeparvovec) Prescribing Information (EU Nov 2022). Revised on 01 Feb 2023. **2.** Mendell JR, *et al. N Engl J Med.* 2017;377(18):1713–22. **3.** Mendell JR, *et al. JAMA Neurol.* 2021;78(7):834-841. **4.** Day JW, *et al. Lancet Neurol.* 2021;20(4):284–93. **5.** Mercuri E, *et al. Lancet Neurol.* 2021;20(10):832–41. **6.** Strauss KA, *et al. Nat Med.* 2022;28(7):1381–9. **7.** Strauss KA, *et al. Nat Med.* 2022;28(7):1390–7. **8.** Weiß C, *et al. Lancet Child Adolesc Health.* 2021;6(1):17–27. **9.** Waldrop MA, *et al. Pediatrics.* 2020;146(3):e20200729. **10.** Matesanz SE, *et al. Pediatr Neurol.* 2021;118:1–5. **11.** Finkel RS, *et al. Neurology.* 2014;83(9):810–7. **12.** Al-Zaidy S, *et al. Pediatr Pulmonol.* 2019;54(2):179–85.

Zolgensma®

Important note: Before prescribing, consult package insert. **Presentation Solution for intravenous infusion**: Each mL contains onasemnogene abeparvovec with a nominal concentration of 2×10^{13} vector genomes (vg). Vials will contain an extractable volume of not less than either 5.5 mL or 8.3 mL. **Indications** Zolgensma is indicated for the treatment of: patients with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the *SMN1* gene and a clinical diagnosis of SMA Type 1, or patients with 5q SMA with a bi-allelic mutation in the *SMN1* gene and up to 3 copies of the *SMN2* gene. **Dosage and administration** Should only be infused by a healthcare professional. Patients should not be re-dosed with Zolgensma. For single-dose intravenous infusion only. The recommended dose is 1.1×10^{14} vector genomes (vg)/kg. The appropriate dose and kit is determined by patient body weight. Zolgensma should be postponed in patients with infections until the infection has resolved and the patient is clinically stable . Clinical signs or symptoms of infection should not be evident at the time of Zolgensma administration. Prior to Zolgensma infusion, AAV9 antibody testing (retesting may be performed if AAV9 antibody titers are reported as above 1:50), liver function (alanine aminotransferase [ALT], aspartate aminotransferase [AST], total bilirubin, albumin, prothrombin time, partial thromboplastin time [PTT], international normalised ratio [INR]), creatinine, complete blood count (including hemoglobin and platelet count), and troponin-I should be conducted at baseline. After Zolgensma infusion, liver function (ALT, AST, total bilirubin), platelet counts, and troponin-I should be conducted on a regular basis. All patients should receive systemic corticosteroids before and after dosing Zolgensma. For complete instructions, refer to the package insert. **Special populations:** *Renal impairment:* The safety and efficacy of Zolgensma have not been established in patients with renal impairment. A dose adjustment should not be considered. *Hepatic impairment:* Zolgensma therapy should be carefully considered in patients with hepatic impairment. A dose adjustment should not be considered. *OSMN1/SMN2 genotype & anti-AAV9 antibodies:* No dose adjustment should be considered in patients with a bi-allelic mutation of the *SMN1* gene and only one copy of *SMN2* or in patients with baseline anti-AAV9 antibody titres above 1:50. *Pediatric patients:* Administration of Zolgensma to premature neonates before reaching full-term gestational age should be carefully considered. There is limited experience in patients 2 years of age and older or with body weight above 13.5 kg. The safety and efficacy of Zolgensma in these patients have not been established. **Method of administration:** For single-dose intravenous infusion only. **Intravenous infusion instructions:** Administer Zolgensma as a slow infusion approximately 60 minutes. It must not be administered as an intravenous push or bolus. For complete preparation and intravenous infusion instructions, refer to the package insert. **Contraindications:** Hypersensitivity to the active substance or to any of the excipients listed in section 6.1 of package insert. **Warnings and precautions** ◆*Advanced SMA:* The benefit/risk profile of Zolgensma in patients with advanced SMA kept alive through permanent ventilation and without the ability to thrive is not established. ◆*Hepatotoxicity:* Administration of AAV vector may result in aminotransferase elevations, which may be serious. Acute serious liver injury and acute liver failure have occurred with Zolgensma. Cases of acute liver failure with fatal outcomes have been reported. Patients with pre-existing hepatic impairment or acute hepatic viral infection may be at higher risk of acute serious liver injury/acute liver failure. A systemic corticosteroid should be administered to all patients before and after Zolgensma infusion. Liver function should be assessed prior to infusion and monitored for at least 3 months after infusion, and at other times as clinically indicated. Promptly clinically assess and closely monitor patients with worsening liver function test results and/or signs or symptoms of acute illness. In case hepatic injury is suspected, further testing is recommended (e.g., albumin, prothrombin time, PTT and INR). The risks and benefits of Zolgensma therapy should be carefully considered in patients with pre-existing hepatic impairment. ◆*Systemic immune response:* It is recommended that patients are clinically stable in their overall health status (e.g., hydration and nutritional status, absence of infection) prior to Zolgensma infusion. Zolgensma should be postponed in patients with infections until the infection has resolved and the patient is clinically stable. Increased vigilance in the prevention, monitoring, and management of infection is recommended before and after Zolgensma infusion. Seasonal prophylaxis against respiratory syncytial virus (RSV) is recommended and should be up-to-date. ◆*Immunogenicity:* Patients should be tested for the presence of AAV9 antibodies prior to infusion with Zolgensma. An immune response to the adeno-associated viral vector serotype 9 (AAV9) capsid will occur after infusion of Zolgensma. ◆*Thrombocytopenia:* Platelet counts should be obtained before Zolgensma infusion and should be closely monitored for significant decreases within the first three weeks following infusion and on a regular basis afterwards; at least weekly for the first month and every other week for the second and third months until platelet counts return to baseline. ◆*Thrombotic microangiopathy (TMA):* Prompt attention to signs and symptoms of TMA is advised, as TMA can result in life-threatening or fatal outcomes. If clinical signs, symptoms and/or laboratory findings occur, a specialist should be consulted immediately to manage TMA as clinically indicated. ◆*Elevated troponin-I:* Troponin-I levels should be obtained before Zolgensma infusion and monitored for at least 3 months following Zolgensma infusion or until levels return to within normal reference range for SMA patients. Consider consultation with a cardiac expert as needed. ◆*Theoretical risk of tumorigenicity as a result of vector integration:* There is a theoretical risk of tumorigenicity due to integration of AAV vector DNA into the genome. **Pregnancy, lactation, females and males of reproductive potential Pregnancy:** There are no available data regarding Zolgensma use in pregnant women. No animal fertility or reproduction studies have been conducted with Zolgensma. **Lactation:** There is no information available on the presence of Zolgensma in human milk, the effects on the breastfed infant or the effects on milk production. **Adverse drug reactions *Very common (≥10%)*:** Aspartate aminotransferase increased, alanine aminotransferase increased, ammonia increased, gamma-glutamyltransferase increased, hepatic enzyme increased, liver function test increased, transaminases increased. ***Common (≥1 to <10%)*:** Thrombocytopenia, platelet count decreased, vomiting, hepatotoxicity, hepatic steatosis, hypertransaminasaemia, pyrexia, troponin increased, troponin-T increased, troponin-I increased. ***Uncommon (≥0.1 to <1%)*:** Thrombotic microangiopathy, acute liver failure. **Interactions:** No interaction studies have been performed. Interactions with antiviral medicinal products are not expected. Live vaccines, such as MMR and varicella, should not be administered to patients on an immunosuppressive steroid dose. Where feasible, the patient's vaccination schedule should be adjusted to accommodate concomitant corticosteroid administration prior to and following Zolgensma infusion. **Legal classification:** P1St1S3. **Reference:** EMA Mar 2024 (CDS 1300e + 1351s + 1373s + 1415i) **Revision date:** 17 Nov 2025

For healthcare professionals only.

FA-11565308 Nov 2025

zolgensma® (onasemnogene abeparvovec)

NOVARTIS

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Taikoo Shing, Hong Kong
Tel: +852 2882-5222 Fax: +852 2577-0274

SPINRAZA®
(nusinersen) injection 12 mg/5 mL

The First SMA Treatment for All Ages and Subtypes[1,2]

3 Maintenance Doses Annually[2]

good adherence:
95.7% on-time doses[3^]

Consistent Drug Concentration

no dosage adjustment across different ages and weights[4]

Extensive Real-World Evidence

✓ 2-72 years of age[5]
✓ across a wide spectrum of SMA severity[5]

14000+
PATIENTS TREATED WORLDWIDE[6]



1.Biogen News, https://investors.biogen.com/news-releases/news-release-details/us-fda-approves-biogens-spinrazatm-nusinersen-first-treatment. Accessed October 2025. **2.**Spinraza Hong Kong Product Insert. **3.**Peng J, et al. BMC Pediatr. 2025 Mar 27;25(1):239. PMC11948911. ^ A dose was considered adherent if the inter-dose interval followed the standard dosing regimen, with a grace period of ± 7 days for Dose 2 to 4 and ± 28 days thereafter. **4.**Finkel RS, et. al., Ann Clin Transl Neurol. 2022 Jun;9(6):819-829. **5.**Coratti G, et al. Orphanet J Rare Dis. 2021;16(1):430. **6.**Based on commercial patients, Early Access patients, and clinical trial participants as of 31 December 2022.Biogen Press Release. June 2023. Available at: https://investors.biogen.com/news-releases/news-release-details/new-data-cure-sma-highlight-potential-benefit-spinrazar. Accessed: October 2023.

Biogen

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

Please refer to the PI for further information. **Indication:** For treatment of 5q 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 12 mg (5 mL) 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. For maintenance dose is delayed or missed, see full PI for detailed administration schedule.Spinraza is for intrathecal use by lumbar puncture. Treatment should be administered by health care professionals experienced in performing lumbar punctures. Spinraza is administrated 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. **Special populations:** Nusinersen 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). Thrombocytopaenia and coagulation abnormalities, including acute severe thrombocytopaenia, 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: The most common adverse reactions (ADRs) associated with the administration of Spinraza were headache, vomiting and back pain. 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 Ltd, 5/F Berkshire House, Taikoo Place, 25 Westlands Road, Quarry Bay, Hong Kong. Version: September 2024
Adverse events should be reported to medinfohongkong@biogen.com. Additional information can be found in the package leaflet.**©2025 Biogen. All rights reserved.**

Biogen-276114-HK-11/2025

ULTOMIRIS® is indicated as an add-on to standardtherapy for the treatment of adult patients with gMG who are anti-acetylcholine receptor (AChR) antibody-positive[1]

ULTOMIRIS®
(ravulizumab)
injection for intravenous use



RAPID ACTION THAT DELIVERS LONG-LASTING CONTROL[1-3,a]

for your adult **patients with gMG** who are anti-AChR antibody-positive

ULTOMIRIS® is the first and only long-acting C5 inhibitor that delivers:[1,4]

Immediate, complete and sustained complement inhibition

- Targeting C5 and preventing generation of MAC, a key driver of destruction at the NMJ[1,5-7]

Rapid and long-lasting symptom control

- Improvements in MG-ADL total scores were observed within 1 week of ULTOMIRIS® treatment, and maintained through 60 weeks of treatment in the open-label extension study[2,3]
- ULTOMIRIS® demonstrated more than 2x greater improvement in MG-ADL total score through Week 26 vs placebo (ULTOMIRIS®: -3.1 vs placebo: -1.4, [P<0.001])[1,2,a]

Significant reduction in rate of clinical deterioration events[c]

- Reduction of 59.8% (p=0.0019) for ravulizumab vs pre-study values[3]
- Reduction of 71.1% (p=0.0011) for ravulizumab vs placebo[3]

8 WEEKS Once-every-8-week maintenance dosing[d]

- Predictable dosing, with each infusion lasting around an hour[1,d,e]

Safety profile assessed in a randomised, placebo-controlled, multicentre Phase 3 study in adult patients with gMG

- Headache, diarrhoea and nausea were the most frequent adverse reactions in ≥10% of patients taking ULTOMIRIS®[2]

ULTOMIRIS® Offer your patients symptom control for the journey ahead[a]

[a] Based on the Myasthenia Gravis Activities of Daily Living (MG-ADL), a categorical scale that assesses the impact on daily function of 8 signs or symptoms that are typically affected in gMG.2,4 Improvements in MG-ADL total scores from baseline were observed within 1 week of treatment, for 26 weeks, and were maintained through 60 weeks of treatment in the open label extension study.2,3 ULTOMIRIS® dosing regimen consists of once-every-8-week maintenance dosing, starting 2 weeks after loading dose administration.1 Please refer to the ULTOMIRIS® Hong Kong Prescribing Information for dosing instructions.
[b] Based on the MG-ADL, a categorical scale that assesses the impact on daily function of 8 signs or symptoms that are typically affected in gMG.[2]
[c] Clinical deterioration was defined as: an MG crisis (weakness severe enough to necessitate intubation or to delay extubation following surgery); significant symptomatic worsening (worsening to a score of 3, or a 2-point worsening from baseline, on any of the individual MG-ADL items other than double vision or eyelid droop, which in the investigator's assessment was associated with significant symptomatic worsening); or use of rescue therapy for health in jeopardy (administration of rescue therapy to a patient whose health, in the opinion of the investigator, would be in jeopardy if rescue therapy were not given)
[d] Once-every-8-week dosing starting 2 weeks after loading dose.[1]
[e] Minimum infusion time for ULTOMIRIS® 100 mg/mL maintenance doses ranges from 30 minutes to 75 minutes, depending on body weight.[1]

AChR, acetylcholine receptor; gMG, generalised myasthenia gravis; MAC, membrane attack complex; MG-ADL, Myasthenia Gravis Activities of Daily Living; NMJ, neuromuscular junction

References: 1. ULTOMIRIS® Hong Kong Prescribing Information. August 2023. **2.** Vu T, et al. N Eng J Med Evid. 2022;1(5) doi:10.1056/EVIDoa2100066. **3.** Meisel A, et al. J Neurol. 2023;270:3862-3875. **4.** Vanoli F & Mantegazza R. Expert Opinion on Biologic Therapy. 2023;23(3):235-241. **5.** Howard JF Jr. Ann NY Acad Sci. 2018;1412(1):113-128. **6.** Conti-Fine BM, et al. J Clin Invest. 2006;116(11):2843-2854. **7.** Vu T, et al. J Neurol. 2023;270(6):3129-3137.

Abbreviated Prescribing Information
Presentation: 300mg/3mL and 1,100mg/11mL concentrate for solution for infusion. Indications: 1) Adult and pediatric patients with a body weight of 10 kg or above with paroxysmal nocturnal haemoglobinuria (PNH). 2) Adult and pediatric patients with a body weight of 10kg or above with atypical haemolytic uremic syndrome (aHUS). 3) Adult patients with generalised myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody-positive. 4) Adult patients with neuromyelitis optica spectrum disorder (NMOSD) who are anti-aquaporin 4 (AQP4) antibody-positive Dosage: (Adult PNH, aHUS, gMG & NMOSD, Pediatric PNH & aHUS with body weight ≥40kg) loading dose (IV infusion): 2,400mg (≥40 to <60kg) / 2,700mg (≥60 to <100kg) / 3,000mg (≥100kg), followed by maintenance dose (IV infusion): 3,000mg (≥40 to <60kg) / 3,300mg (≥60 to <100kg) / 3,600mg (≥100kg) starting 2 wks after loading dose administration and then every 8 wks. (Paediatric PNH & aHUS with body weight <40kg) loading dose (IV infusion): 600mg (≥10 to <20kg) / 900mg (≥20 to <30kg) / 1,200mg (≥30 to <40kg), followed by maintenance dose (IV infusion): 600mg (≥10 to <20kg) / 2,100mg (≥20 to <30kg) / 2,700mg (≥30 to <40kg) starting 2 wks after loading dose administration and then every 4 (≥10 to <20kg) / 8 (≥20 to <40kg) wks. Switching from eculizumab to ravulizumab: Loading dose of ravulizumab administered 2 wks after the last eculizumab infusion, and then maintenance doses administered per regimen above, starting 2 wks after loading dose administration. Administration: Diluted to a final concentration of 50 mg/mL prior to administration by IV infusion using a syringe-type pump or an infusion pump over a minimal period of 25 to 75 minutes depending on body weight. Contraindications: hypersensitivity to ravulizumab or any excipients; unresolved Neisseria meningitidis infection; unvaccinated against Neisseria meningitidis unless receiving prophylactic antibiotics until 2 wks after vaccination. Precautions: Meningococcal Infection/Immunisation - Vaccinate all patients at least 2 wks prior to initiating ravulizumab. If initiate <2 wks, administer prophylactic antibiotics until 2 wks after vaccination. Patients <18 yrs of age must be vaccinated against Haemophilus influenza and pneumococcal infection. Laboratory Monitoring: (PNH) Closely monitor patients for at least 16 wks to detect haemolysis and other reactions. (aHUS) Closely monitor patients for severe TMA complications. (gMG) Monitor symptoms of gMG. (NMOSD) Monitor symptoms of NMOSD relapse. Use in Pregnancy/Lactation: Women should use effective contraception during treatment with ravulizumab, and for up to 8 months after treatment; breastfeeding should be discontinued during treatment, and up to 8 months after treatment. Adverse Reactions: Very common (≥10%): upper respiratory tract infection, nasopharyngitis, headache, diarrhea. Common (≥1% to <10%): urinary tract infection, dizziness, vomiting, abdominal pain, nausea, dyspepsia, urticaria, pruritus, rash, arthralgia, back pain, myalgia, muscle spasms, pyrexia, influenza-like illness, chills, asthenia, fatigue, infusion related reaction. Full local prescribing information is available upon request. API-HK.ULT.0823

Please visit contactazmedical.astrazeneca.com, for (1) enquiring Medical Information (MI), (2) reporting Individual Case Safety Report (ICSR) and/or (3) reporting Product Quality Complaint (PQC) to AstraZeneca Hong Kong Limited.
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AstraZeneca

Further information is available on request:
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Unit 1-3, 11/F, China Taiping Finance Centre,
18 King Wah Road, North Point, Hong Kong
Tel: (852) 2420 7388 Fax: (852) 2422 6788

HK-10134 31/05/2024

Evrysdi® (risdiplam)▼ is indicated for the treatment of 5q spinal muscular atrophy (SMA) in patients 2 months of age and older[1]

4+ years of long-term safety and efficacy[2]

The ONLY SMA therapy with non-invasive at-home dosing[1]

15,000+ patients treated worldwide*



Evrysdi® risdiplam

Important Safety Information

Special warnings and precautions for use: Potential embryo-foetal toxicity: Embryo-foetal toxicity has been observed in animal studies. Patients of reproductive potential should be informed of the risks and must use highly effective contraception during treatment and until at least 1 month after the last dose in female patients and 4 months after the last dose in male patients. The pregnancy status of female patients of reproductive potential should be verified prior to initiating Evrysdi® therapy. Potential effects on male fertility: Based on observations from animal studies, male patients should not donate sperm while on treatment and for 4 months after the last dose of Evrysdi®. Prior to initiating treatment, fertility preservation strategies should be discussed with male patients of reproductive potential.

▼ This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions. Please consult your local product information for details on how to report adverse reactions.

* Based on commercial patients, pre-approval patient access programs, and clinical trial participants as of February 2024. Source: Data on file. The Roche Group, 2024.

References: 1. Evrysdi (Risdiplam) Hong Kong Prescribing Information, Date: March 2024. **2.** Oskoui M et al, MDA Clinical & Scientific Conference; Dallas, Texas, USA March 19-22, 2023.

M-HK-0001587 approved in March-2024 is valid for 2 years or until change is required in accordance with regulatory requirements, whichever comes first.

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Roche Hong Kong Limited Unit 1-2, 24/F, Central Plaza, 18 Harbour Road, Wan Chai, Hong Kong Tel: +852 2723 2832



amvuttra (vutrisiran) injection 25 mg/0.5 mL

Going beyond polyneuropathy symptoms to improve lives*[1]

AMVUTTRA® (vutrisiran) is indicated for the treatment of hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis) in adult patients with stage 1 or stage 2 polyneuropathy[2]

The decision to continue treatment in those patients whose disease progresses to stage 3 polyneuropathy should be taken at the discretion of the physician based on the overall benefit and risk assessment.[2] *AMVUTTRA® demonstrated improvements from baseline in quality of life.[1] Healthcare professionals are asked to report any suspected adverse reactions by contacting safety.hongkong@medisonpharma.com.

ATTRAD-HK-27112025

Abbreviation: hATTR, hereditary transthyretin amyloidosis. **References:** 1. Adams D, et al. *Amyloid.* 2023;30(1):1-9. 2. AMVUTTRA® Hong Kong Prescribing Information.
Prescribing Information: • **Indication:** Amvuttra is indicated for the treatment of hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis) in adult patients with stage 1 or stage 2 polyneuropathy. • **Posology:** The recommended dose of Amvuttra is 25 mg administered via subcutaneous injection once every 3 months. Vitamin A supplementation at approximately, but not exceeding, 2,500 IU to 3,000 IU vitamin A per day is advised for patients treated with Amvuttra. The decision to continue treatment in those patients whose disease progresses to stage 3 polyneuropathy should be taken at the discretion of the physician based on the overall benefit and risk assessment. *Missed dose:* If a dose is missed, Amvuttra should be administered as soon as possible. Dosing should be resumed every 3 months, from the most recently administered dose. • **Special Populations:** *Elderly patients:* No dose adjustment is required in patients ≥ 65 years of age. *Hepatic impairment:* No dose adjustment is necessary in patients with mild hepatic impairment (total bilirubin ≤ 1 x upper limit of normal (ULN) and aspartate aminotransferase (AST) > 1 x ULN, or total bilirubin > 1.0 to 1.5 x ULN and any AST). Vutrisiran has not been studied in patients with moderate or severe hepatic impairment and should only be used in these patients if the anticipated clinical benefit outweighs the potential risk. *Renal impairment:* No dose adjustment is necessary in patients with mild or moderate renal impairment (estimated glomerular filtration rate [eGFR] ≥ 30 to < 90 ml/min/1.73 m^2). Vutrisiran has not been studied in patients with severe renal impairment or end-stage renal disease and should only be used in these patients if the anticipated clinical benefit outweighs the potential risk. *Paediatric population:* The safety and efficacy of Amvuttra in children or adolescents < 18 years of age have not been established. No data are available. • **Method of Administration:** Amvuttra is for subcutaneous use only. Amvuttra should be administered by a healthcare professional. • **Contraindications:** Severe hypersensitivity (e.g., anaphylaxis) to the active substance or to any of the excipients as listed in the full Hong Kong Prescribing Information. • **Important Safety Information:** By reducing serum transthyretin (TTR) protein, Amvuttra treatment leads to a decrease in serum vitamin A (retinol) levels. Serum vitamin A levels below the lower limit of normal should be corrected and any ocular symptoms or signs due to vitamin A deficiency should be evaluated prior to initiation of treatment with Amvuttra. Ophthalmological assessment is recommended if patients develop ocular symptoms suggestive of vitamin A deficiency, including reduced night vision or night blindness, persistent dry eyes, eye inflammation, corneal inflammation or ulceration, corneal thickening or corneal perforation. Pregnancy should be excluded before initiating Amvuttra and women of childbearing potential should practice effective contraception. If a woman intends to become pregnant, Amvuttra and vitamin A supplementation should be discontinued and serum vitamin A levels should be monitored and have returned to normal before conception is attempted. In the event of an unplanned pregnancy, Amvuttra should be discontinued. • **Adverse Events:** The most frequently occurring adverse reactions reported in Amvuttra-treated patients were pain in extremity (15%) and arthralgia (11%). Other common adverse reactions include dyspnoea, injection site reactions and increased blood alkaline phosphatase. *Please refer to the full Hong Kong Prescribing Information for additional information, or email medinfo.hk@medisonpharma.com.*

MEDISON

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Alnylam® PHARMACEUTICALS