Specialist Working Group for Neurology

Proposed changes to the *Criteria for the clinical use of intravenous immunoglobulin in Australia, Second Edition*

ITEM	CRITERIA FOR THE CLINICAL USE OF INTRAVENOUS IMMUNOGLOBULIN IN AUSTRALIA, SECOND EDITION (CRITERIA)	PROPOSED REVISIONS TO THE CRITERIA	SWG RATIONALE FOR PROPOSED CHANGE (A) Administrative) (B) Progressive (C) Programmed
Condition Name	Opsoclonus-myoclonus ataxia (OMA)	Opsoclonus-myoclonus ataxia (OMA)	
Specialty	Neurology	Neurology	
Chapter	6		
Specific Conditions		• Idiopathic • Paraneoplastic associated neuroblastoma • Paraneoplastic associated small cell lung cancer • Paraneoplastic associated breast cancer • Paraneoplastic associated other tumour type	
Level of Evidence	Small case studies only; insufficient data (Category 4a).	Small case studies only; insufficient data (Category 4a).	

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Justification for Evidence Category	The Asia–Pacific IVIg Advisory Board (2004) consensus statement summarises several case reports suggesting that IVIg is useful in idiopathic OMA and childhood paraneoplastic OMA associated with neuroblastoma.	The Asia–Pacific IVIg Advisory Board (2004) consensus statement summarises several case reports suggesting that intravenous immunoglobulin (IVIg) is useful in idiopathic OMA and childhood paraneoplastic OMA associated with neuroblastoma.			No change. While there have been further case series showing benefit, there will be no other evidence for this rare disease.
Description and Diagnostic Criteria	OMA is an immune‐mediated monophasic or multiphasic disorder consisting of opsoclonus (conjugate chaotic eye movements), cerebellar ataxia, and arrhythmic myoclonus affecting the trunk, the head and the extremities. OMA may be either paraneoplastic or idiopathic, presumably para-infectious (e.g. post-viral). In children, OMA complicates about 2–3% of neuroblastomas. In adults, it may occur in association with several cancers, most commonly small-cell lung cancer and breast cancer.	OMA is an immune‐mediated monophasic or multiphasic disorder consisting of opsoclonus (conjugate chaotic eye movements), cerebellar ataxia, and arrhythmic myoclonus affecting the trunk, the head and the extremities. OMA may be either paraneoplastic or idiopathic, presumably para-infectious (e.g. post-viral). In children, OMA complicates about 2–3% of neuroblastomas. In adults, it may occur in association with several cancers, most commonly small-cell lung cancer and breast cancer.			No change.
Diagnosis is required	Diagnosis of OMA by a neurologist **Note**: Given the rarity of OMA and its devastating effects, IVIg should be used where it is considered appropriate by a neurologist.	Yes	By which specialty	Neurologist	No change - Diagnosis and prescribing must be undertaken by a neurologist.
Diagnosis		No	By which		

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must be verified		specialty	
Exclusion Criteria	Adult paraneoplastic OMA.		The SWG could not find any evidence as to why this exclusion existed. The incidence was very rare, and was treated in the same way as Idiopathic OMA in adults, that is, with Ig as second line treatment after steroids.
Indications	Long-term maintenance therapy of OMA in association with other tumour therapies.	**Child with OMA.** **Second-line treatment of OMA in adults following the use of corticosteroids.**	The use of 2 indications allows adults with idiopathic OMA to be treated after a course of steroids. Children are eligible for Ig as first line therapy.
Qualifying Criteria	Diagnosis of OMA by a neurologist: In children; OR As second-line treatment following the use of adrenocorticotrophic hormone or corticosteroids.	**Child with OMA.** Patient with OMA is younger than 18 years AND Clinical assessment indicates significant disability, as measured by the Cerebellar Functional System Score with a value of at least 2 points.	SWG confirmed the revision of the second qualifying criteria by removal of the reference to ACTH given that ACTH is rarely used in Australia. The cerebellar functional system score was chosen to demonstrate initial disability and response.

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		As there is no validated measure for OMA, the Cerebellar Functional System Score has been selected from the Expanded Disability Status Scale (Kurtzke 1983). **Second-line treatment for OMA in adults following the use of corticosteroids.** Adult with OMA in whom a standard course of steroid therapy has been undertaken (or steroids are contraindicated). AND Clinical assessment demonstrates disability as measured by the cerebellar functional system score with a value of at least two points.	Values of the cerebellar functional system score are: 0. -Normal – no evidence of cerebellar dysfunction 1. Abnormal signs without disability 2. Mild ataxia 3. Moderate ataxia - 4. Severe ataxia - all limbs or gait 5. Unable to perform co-ordinated movements due to ataxia Changes in opsoclonus symptoms will be rated as: i. Deterioration in symptoms ii. Symptoms stable iii. Mild improvement iv. Moderate improvement Significant improvement
Review Criteria	**Review** Regular review by neurologist is required; frequency as determined by clinical status of patient.	**Child with OMA.** Regular review by a neurologist is required; frequency as determined by the clinical status of	

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	For stable patients on maintenance treatment, review by a neurologist is required at least annually. **Effectiveness** Objective indicators of relief of symptoms of OMA and improvement or stabilisation of scores of ADLs.	the patient. For stable patients on maintenance treatment, review by a neurologist is required at least annually. Efficacy of immunoglobulin (Ig) treatment is demonstrated by improvement in symptoms of OMA and improvement in or no deterioration of disability. **On review of the initial authorisation period** Patient demonstrates clinical improvement in, or stabilisation of, opsoclonus symptoms after six months treatment. AND There has been no further deterioration or some improvement in the degree of disability as measured by the Cerebellar Functional System Score.	

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		For stable patients on maintenance treatment, review by a neurologist is required at least annually. **On review of a continuing authorisation period** Patients demonstrates clinical improvement or stability in opsoclonus symptoms. AND There has been no further deterioration in the degree of disability as measured by the Cerebellar Functional System Score. Regular review by a neurologist is required; frequency as determined by the clinical status of the patient. For stable patients on maintenance treatment, review by a neurologist is required at least annually.	

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		Second-line treatment for OMA in adults following the use of corticosteroids. Regular review by a neurologist is required; frequency as determined by the clinical status of the patient. For stable patients on maintenance treatment, review by a neurologist is required at least annually. Efficacy of Ig treatment is demonstrated by improvement in symptoms of OMA and improvement in, or no deterioration of, disability. **On review of the initial authorisation period** Patient demonstrates clinical improvement in opsoclonus symptoms. AND There has been no further deterioration or some improvement in the degree of disability as	

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		measured by the Cerebellar Functional System Score. Regular review by a neurologist is required; frequency as determined by the clinical status of the patient. For stable patients on maintenance treatment, review by a neurologist is required at least annually. **On review of a continuing authorisation period** Patient demonstrates stable or improved opsoclonus symptoms. AND There has been no further deterioration in the degree of disability as measured by the Cerebellar Functional System Score. For stable patients on maintenance treatment, review by a neurologist is required at least annually.	

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Dose	**Induction:** 1–2 g/kg in 2 to 5 divided doses. **Maintenance:** 0.4–1 g/kg, 4 to 6 weekly. Aim for the minimum dose to maintain optimal functional status. **Refer to the current product information sheet for further information.** **The aim should be to use the lowest dose possible that achieves the appropriate clinical outcome for each patient**.	**Child with OMA** **Induction:** 1–2 g/kg in 2 to 5 divided doses. **Maintenance:** 0.4–1 g/kg, 4 to 6 weekly. The aim should be to use the lowest dose possible that achieves the appropriate clinical outcome for each patient. **Refer to the current product information sheet for further information.** **Second-line treatment for OMA in adults following the use of corticosteroids.** **Induction:** 1–2 g/kg in 2 to 5 divided doses. **Maintenance:** 0.4–1 g/kg, 4 to 6 weekly. The aim should be to use the lowest dose possible that achieves the appropriate clinical outcome for each patient. **Refer to the current product information sheet for further information.**	No change to dosing.

POTENTIAL OPERATIONAL IMPACT			
There is not expected to be any significant impact operationally as the definition of the Cerebellar functional system scoring and rating for change in opsoclonus symptoms will be fully documented in the Ig system.			
POTENTIAL IMPACT ON DEMAND			
Patient Numbers **2013-14** **Usage 2013/14**	26 patients (<1%) 2013/14 Usage: < 1%	Given the very few patients with this condition it is unlikely that there will be any significant impact on demand.	
POTENTIAL IMPACT ON COST			
Current cost		**Anticipated reduction in cost, if any** **Marginal** = borderline or unchanged from current cost **Minor** = decrease by $500K - $1.99M from current cost **Major** = decrease $2M+ from current cost	**Marginal**
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Glatz, K, Meinck, HM & Wildemann, B 2003, 'Para-infectious opsoclonus-myoclonus syndrome: high dose intravenous immunoglobulins are effective', *Journal of Neurology, Neurosurgery and Psychiatry*, vol. 74, no. 2, pp. 279–80. Kornberg, AJ, for the Asia–Pacific IVIg Advisory Board 2004, *Bringing consensus to the use of IVIg in neurology. Expert consensus statements on the use of IVIg in neurology*, 1st edn, Asia–Pacific IVIg Advisory Board, Melbourne, pp. 80–82. Kurtzke, JF 1983, 'Rating neurologic impairment in multiple sclerosis: an expanded disability status scale (EDSS)', *Neurology*, vol. 33, no. 11, pp. 1444–1452. National Institute of Neurological Disorders 2006, 'NINDS opsoclonus myoclonus information page', January. Available from: www.ninds.nih.gov/disorders/opsoclonus_myoclonus/opsoclonus_myoclonus.htm [cited 7 Dec 2007]			
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