Spinraza (nusinersen)
Effective 08/2017

Plan	☒ MassHealth ☐ Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☐ Pharmacy Benefit ☒ Medical Benefit (NLX)		
Specialty Limitations	N/A		
Contact Information	**Specialty Medications**		
	All Plans	Phone: 866-814-5506	Fax: 866-249-6155
	Non-Specialty Medications		
	MassHealth	Phone: 877-433-7643	Fax: 866-255-7569
	Commercial	Phone: 800-294-5979	Fax: 888-836-0730
	Exchange	Phone: 855-582-2022	Fax: 855-245-2134
	Medical Specialty Medications (NLX)		
	All Plans	Phone: 844-345-2803	Fax: 844-851-0882
Exceptions	N/A		

Overview

Spinraza (nusinersen) is a survival motor neuron-2 (SMN2)-directed antisense oligonucleotide indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.

Coverage Guidelines

1. Patient Population
   AllWays Health Partners may authorize coverage of Spinraza (nusinersen) for members, when ALL of the following criteria are met:
   - Members have a documented diagnosis of spinal muscular atrophy (SMA) type 1, 2, or 3 confirmed by molecular genetic testing
   - Clinical documentation of baseline (pre-treatment) motor function skills has been submitted
   - Members have already established care with a SMA multidisciplinary care center
   - Members have none of the following: hospitalization for surgery or pulmonary event within past 2 months, active infection, brain or spinal cord disease, meningitis, implanted CSF shunt, treatment with another investigational drug <1 month of evaluation

2. Prescribing
   - Prescribed by neurologist with expertise in the management of SMA

3. Dosing and Administration
   - 4 loading doses: First 3 loading doses at 14 day intervals, 4th loading dose 30 days after 3rd dose
   - Maintenance dose every 4 months after the 4th loading dose

- Dose: 12 mg (5 mL) given intrathecally as bolus injection over 1-3 minutes using a spinal anesthesia needle
- Prior to administration, remove 5 mL of cerebral spinal fluid (CSF)
- Administered by attending neurologist experienced in administering intrathecal injections

4. Monitoring
    - At baseline and prior to each dose, obtain a platelet count, coagulation test (i.e., prothrombin time, activated partial thromboplastin time) and quantitative spot urine protein test
    - At each visit, assessment for improvement in clinical outcomes via motor function using HINE, CHOP-INTEND, HFMSE or other age-appropriate motor function scales

5. Duration of Therapy
    - May be continued until disease progression or unacceptable toxicity (may require several months to a year for improvement in motor function to be seen)
    - Discontinuation of drug to be determined based on age-appropriate performance on motor function and patient reported outcome scales using standardized instrument(s)

Continuation of Therapy

Reauthorization requires physician documentation of assessment of improvement in clinical outcomes via motor function using HINE, CHOP-INTEND, HFMSE or other age- appropriate motor function scale.

Limitations

1. Approvals will be granted for 12 months.

References

1. A study to assess the efficacy and safety of IONIS-SMN Rx in patients with later-onset spinal muscular atrophy. https://clinicaltrials.gov/ct2/show/NCT02292537 (Accessed on May 15, 2017).
2. Chiriboga CA, Swoboda KJ, Darras BT, et al. Results from a phase I study of nusinersen (ISIS-SMN$_{Rx}$) in children with spinal muscular atrophy. *Neurol* 2016;86(10):890-897.
3. Clinicaltrials.gov. Available at: https://www.clinicaltrials.gov/ct2/show/NCT02462759?term=nusinersen&rank=3. (Accessed January 25, 2017)
4. D'Amico A, Mercuri E, Tiziano FD, et al. Spinal muscular atrophy. *Orphanet J of Rare Dis* 2011, 6:71. http://www.ojrd.com/content/6/1/71.
5. EMA: nusinersen. Available at : http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/pips/EMEA-001448-PIP01-13-M02/pip_001167.jsp&mid=WC0b01ac058001d129
6. FDA summary review. http://www.accessdata.fda.gov/drugsatfda_docs/nda/2016/209531Orig1s000SumR.pdf (Accessed February 6, 2017).
7. Iannaccone ST, Hynan LS, Morton A, et al. The PedsQL in pediatric patients with spinal muscular atrophy: feasibility, reliability, and validity of the Pediatric Quality of Life inventory generic score scales and neuromuscular module. *Neuromuscul Disord* 2009 December; 19(12): 805–812. doi:10.1016/j.nmd.2009.09.009.
8. Nusinersen prescribing information. Biogen®. December 2016.

9. Prior TW. Carrier screening for spinal muscular atrophy 2008; *Genet Med*. Nov; 10(11):840-2. doi: 10.1097/GIM.0b013e318188d069.
10. Spinal muscular atrophy. ACOG Committee opinion No. 432. American College of Obstetricians and Gynecologists. *Obstet Gynecol* 2009;113: 1194-6.
11. Varni J. The PedsQL Measurement model for pediatric quality of life inventory. Available at: http://www.pedsql.org/about_pedsql.html.
12. Wang CH, Finkel RS, Bertini ES, et al. Consensus statement for standard of care in spinal muscular atrophy. *J Child Neurol* 2007;22(8):1027-1049.

Review History

02/2017 – Reviewed by Clinical Experts
08/2017 – Revised (P&T approval)
11/2018 – Reviewed
03/18/2020 – Reviewed P&T Mtg

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