Prior Authorization Update: Nusinersen

Purpose of Review:
The Oregon Health Authority (OHA) is seeking P&T support to participate in the Spinal Muscular Atrophy Research: The Effectiveness of Nusinersen (SMARTEN) project with the Center for Evidence-based Policy. The SMARTEN project is a collaboration of multiple state Medicaid agencies whose goal is to collect and analyze long-term, clinical outcomes data for nusinersen in type 1 and 2 spinal muscular atrophy. The purpose of this prior authorization update is to present changes to the Oregon Medicaid Fee-for-Service (FFS) prior authorization criteria which would be required for FFS participation in the project. These recommendations are not based on evidence. However, participating in the SMARTEN program would provide an opportunity to collect relevant clinical outcomes for an orphan drug for which there may not be additional clinical outcome data in the future from clinical trials.

If the OHA participates in SMARTEN, updates to FFS prior authorization criteria would be required to ensure that all outcomes of interest are collected at times specified in the SMARTEN protocol (at baseline, 6 months, then yearly until the 30 month follow-up period is complete). Motor skills are measured in SMARTEN by the Hammersmith Infant Neurological Examination Section 2 (HINE-2) for patients 2 years and younger, by the Hammersmith Functional Motor Scale-Expanded (HFMSE) for ambulatory patients 3 years and older, and by the revised Upper Limb module (RULM) for non-ambulatory patients 3 years and older. The SMARTEN project is approved through the Oregon Health and Science University Institutional Review Board and state Medicaid agencies are currently developing data use agreements for the SMARTEN project.

Recommendation:
- Consider updating the prior authorization criteria to document necessary outcomes data for OHA participation in the SMARTEN project.

Proposed Prior Authorization Criteria:

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Goal(s):
- Approve nusinersen for funded OHP conditions supported by evidence of benefit (e.g. Spinal Muscular Atrophy)

Length of Authorization:
- Up to 68 months for initial approval and up to 12 months for renewal.

Requires PA:
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November 2018
- Nusinersen (billed as a pharmacy or physician administered claim)

**Covered Alternatives:**
- Current PMPDP preferred drug list per OAR 410-121-0030 at [www.orpdl.org](http://www.orpdl.org)
- Searchable site for Oregon FFS Drug Class listed at [www.orpdl.org/drugs/](http://www.orpdl.org/drugs/)

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<td><strong>1.</strong> What diagnosis is being treated?</td>
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| **2.** Is this a request for continuation of therapy? | Yes: Go to **Renewal Criteria**  
No: Go to #3 |
| **3.** Have all of the following information been documented by the provider: |  
- Date of SMA diagnosis  
- Prior enrollment in clinical trials for nusinersen | Yes: Go to #4  
No: Pass to RPh. Request additional information. |
| 3-4. Does the patient have type 1, 2 or 3 Spinal Muscular Atrophy documented by genetic testing and at least 2 copies of the SMN2 gene? | Yes: Go to #54  
No: Pass to RPh. Deny; medical appropriateness. |
### Approval Criteria

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| **4.5.** Is a baseline motor assessment available such as one of the following functional assessment tools: | **Yes:** Go to #65  
**Document the following:**  
- Baseline motor assessment score:  
- Tool used:  
- Measurement date:  
- Provider type who administered the tool: | **No:** Pass to RPh. Deny; medical appropriateness. |
| **Type 3 SMA:**  
- Hammersmith Infant Neurological Examination (HINE-2)  
- Hammersmith Functional Motor Scale (HFSME)  
- Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND)  
- Upper Limb Module (ULM)  
- 6-Minute Walk Test | | |
| **Type 1 and 2 SMA:**  
- Patients <2 years old: Hammersmith Infant Neurological Examination (HINE-2)  
- Patients >3 years old: Hammersmith Functional Motor Scale (HFSME) (for ambulatory patients) or revised Upper Limb Module (RULM) (for non-ambulatory patients) | | |
| **5.6.** Is the patient ventilator dependent (using at least 16 hours per day on at least 21 of the last 30 days)?  
**Note:** This assessment does not apply to patients who require ventilator assistance | **Yes:** Pass to RPh. Deny; medical appropriateness. | **No:** Go to #76. |
| **6.7.** Is the drug being prescribed by a pediatric neurologist or a provider with experience treating spinal muscular atrophy? | **Yes:** For initial approval, approve 45 doses over 86 months. | **No:** Pass to RPh. Deny; medical appropriateness. |
## Renewal Criteria

1. **Has the following information been documented by the provider:**
   - **a.** Recent assessment of motor function with one of the following scales (preferably the same scale used at baseline):
     - i. For ≤2 years old: Hammersmith Infant Neurological Examination (HINE-2)
     - ii. For patients >3 years old: Hammersmith Functional Motor Scale (HFSME) (for ambulatory patients) or revised Upper Limb Module (RULM) (for non-ambulatory patients)
   - **b.** Need for permanent ventilation (i.e., 16 hours or greater in a day) and date of initiation if applicable

   **Yes:** Go to #2
   Document the following:
   - Motor assessment score: _______
   - Tool used: ___________________
   - Measurement date: ____________
   - Provider type who administered the tool: ________________________
   - Document date of permanent ventilator initiation if applicable: ______________

   **No:** Pass to RPh. Request additional information.

2. **Has the patient’s motor function improved as demonstrated by:**
   - Improvement from baseline motor function score documented within one month of renewal request
   - **AND**
   - More areas of motor function improved than worsened

   **Yes:** Approve for 12 months

   **No:** Pass to RPh; Deny; medical appropriateness.
### Renewal Criteria

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| 2. Has the patient experienced any serious adverse events related to nusinersen treatment? | **Yes:** Pass to RPH; Deny; medical appropriateness  
Document the following:
- Serious adverse event:  
- Date:              | **No:** Approve for 12 months |

**P&T Review:** 11/18 (JP); 7/17; 3/17  
**Implementation:** TBD; 9/1/17; 5/17