The drug below is being considered for possible coverage under the B.C. PharmaCare program. PharmaCare is a government-funded drug plan that helps British Columbians with the cost of eligible prescription drugs and specific medical supplies. For more information on PharmaCare, visit Ministry of Health - PharmaCare.

PharmaCare reviews each drug for treating a specific illness or medical condition (known as an “indication”). If a decision is made to cover the drug, it will be only for that illness or condition.

In some cases, PharmaCare may cover a drug only for people who have the illness or condition and have not responded to other drugs used to treat that illness or condition.

For more information on PharmaCare’s drug coverage review process, see the last page of this information sheet.

### Information about the drug

<table>
<thead>
<tr>
<th>Generic name (scientific name)</th>
<th>nusinersen</th>
</tr>
</thead>
<tbody>
<tr>
<td>Brand name</td>
<td>Spinraza™</td>
</tr>
<tr>
<td>Manufacturer</td>
<td>Biogen Canada Inc.</td>
</tr>
<tr>
<td>Indication</td>
<td>For the treatment of Spinal Muscular Atrophy</td>
</tr>
<tr>
<td>Has the drug been reviewed by the Common Drug Review (CDR)? (see the note below this table.)</td>
<td>Yes; For more information about the CDR’s review of nusinersen (Spinraza™), you can Search the CDR Reports.</td>
</tr>
</tbody>
</table>

**Public input start date** Wednesday October 18, 2017

**Public input closing date** Wednesday November 15, 2017 AT MIDNIGHT

**How is the drug taken?** Spinraza is administered by intrathecal injection (injection into the spinal theca, the loose sheath enclosing the spinal cord).

**How often is the drug taken?** Spinraza is administered based on the following schedule:
- the first 4 doses of Spinraza are administered on Day 1, Day 15, Day 30 and Day 60; and
- successive doses are administered every 4 months thereafter.

For more information about the CDR’s review of nusinersen (Spinraza™), you can Search the CDR Reports.
Information about the drug

General drug and/or drug study information

Spinraza is used to treat a genetic disease called Spinal Muscular Atrophy (SMA). SMA is caused by a problem with the 5q chromosome. SMA is characterized by degeneration of the anterior horn cells in the spinal cord and motor nuclei in the lower brainstem which lead to progressive muscle weakness and atrophy.

Spinraza is one of a group of medicines called antisense oligonucleotides (ASO). Spinraza works by helping the body to produce more of the protein that is reduced in SMA.

Studies looked at the following to determine if Spinraza is safe and effective for the treatment of SMA:

- Hammersmith Infant Neurological Examination (HINE) Section 2 motor milestone responders
- Time to death or permanent ventilation
- Overall survival
- Proportion of patients requiring permanent ventilation
- Improvement in the Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND) scale
- Bad reactions
- Serious bad reactions
- Patients leaving the trial due to bad reactions
- Mortality
- Bad reactions of special interest (vomiting and renal/urinary disorders)

Other considerations

None

Note:
The Common Drug Review (CDR) is a national organization that reviews drugs on behalf of Canadian public sector plans when manufacturers want to have the jurisdictions provide coverage for the drugs. For detailed information on B.C. PharmaCare’s drug review process, including the role of the CDR in that process, see The Drug Review Process in B.C. - Overview.

Cost of the drug under review compared to other drugs used to treat the same indication

<table>
<thead>
<tr>
<th>generic name (Brand Name) of Drug Comparator</th>
<th>PharmaCare Status (if and how the drug is already covered)</th>
<th>Usual Dose</th>
<th>Annual Cost of Therapy¹</th>
</tr>
</thead>
<tbody>
<tr>
<td>nusinersen (Spinraza)</td>
<td>Under Review</td>
<td>Intrathecal injections on days 1, 15, 30 and 60; then every 4 months</td>
<td>Year 1: $708,000 Subsequent Years: $354,000</td>
</tr>
</tbody>
</table>

Notes:
¹ Price based on manufacturer’s submission
The Drug Review Process in B.C.

A manufacturer submits a request to the Ministry of Health (Ministry).

An independent group called the Drug Benefit Council (DBC) gives advice to the Ministry. The DBC looks at:

- whether the drug is safe and effective
- advice from a national group called the Common Drug Review (CDR)
- what the drug costs and whether it is a good value for the people of B.C.
- ethical considerations involved with covering or not covering the drug
- input from physicians, patients, caregivers, patient groups and drug submission sponsors

The Ministry makes PharmaCare coverage decisions by taking into account:

- the existing PharmaCare policies, programs and resources
- the evidence-informed advice of the DBC
- the drugs already covered by PharmaCare that are used to treat similar medical conditions
- the overall cost of covering the drug


This document is intended for information only.
It does not take the place of advice from a physician or other qualified health care provider.