



Spinraza[®] and Zolgensma[®] for Spinal Muscular Atrophy: Effectiveness and Value

Draft Questions for Deliberation and Voting: March 7, 2019 Public Meeting

These questions are intended for the deliberation of the New England CEPAC voting body at the public meeting.

Clinical Evidence

Patient Population for questions 1-3: Patients with infantile-onset (Type I) spinal muscular atrophy (SMA).

1. Is the evidence adequate to demonstrate that the net health benefit of nusinersen (**Spinraza[®]**, Biogen Inc.) added to supportive care is superior to that provided by supportive care alone?

Yes No

2. Is the evidence adequate to demonstrate that the net health benefit of onasemnogene abeparvovec (**Zolgensma[®]**, AveXis/Novartis AG) added to supportive care is superior to that provided by supportive care alone?

Yes No

3. Is the evidence adequate to distinguish the net health benefit between **Spinraza** and **Zolgensma**?

Yes No

Patient Population for question 4: Patients with later-onset (Type II/III) SMA.

4. Is the evidence adequate to demonstrate the net health benefit of **Spinraza plus supportive care** is superior to that provided by **supportive care alone**?

Yes No

Patient Population for questions 5-6: Patients with presymptomatic SMA.

5. Is the evidence adequate to demonstrate the net health benefit of administering **Spinraza prior to development of symptoms** is superior to that of **waiting for symptoms to develop and then administering Spinraza**?

Yes No

6. Is the evidence adequate to demonstrate the net health benefit of administering **Zolgensma prior to development of symptoms** is superior to that of **waiting for symptoms to develop and then administering Zolgensma**?

Yes No

Potential Other Benefits and Contextual Considerations

7. Does treating patients with the following interventions offer one or more of the following potential “other benefits?” (select all that apply)
- a. Compared to **Spinraza, Zolgensma** intervention offers reduced complexity that will significantly improve patient outcomes.
 - b. **Spinraza and Zolgensma** will significantly reduce caregiver or broader family burden.
 - c. **Spinraza and Zolgensma** offer a novel mechanism of action or approach that will allow successful treatment of many patients for whom other available treatments have failed.
 - d. **Spinraza and Zolgensma** will have a significant impact on improving patients’/caregivers’ ability to return to work and/or their overall productivity.
 - e. **Spinraza and Zolgensma** will have a significant positive impact outside the family, including communities.
 - f. **Spinraza and Zolgensma** will have a significant impact on the entire “infrastructure” of care, including effects on screening for affected patients, on the sensitization of clinicians, and on the dissemination of understanding about the condition, that may revolutionize how patients are cared for in many ways that extend beyond the treatment itself.
 - g. There are other important benefits or disadvantages that should have an important role in judgments of the value of **Spinraza and Zolgensma**: _____
8. Are any of the following contextual considerations important in assessing **Spinraza’s** long-term value for money? (select all that apply)
- a. **Spinraza** is intended for the care of individuals with a condition of particularly high severity in terms of impact on length of life and/or quality of life.
 - b. **Spinraza** is intended for the care of individuals with a condition that represents a particularly high lifetime burden of illness.

12. Given the available evidence on comparative effectiveness and incremental cost-effectiveness, and considering other benefits, disadvantages, and contextual considerations, what is the long-term value for money of treatment with **Zolgensma** versus **Spinraza** in patients with infantile-onset (Type I) SMA?²

Low

Intermediate

High

13. Given the available evidence on comparative effectiveness and incremental cost-effectiveness, and considering other benefits, disadvantages, and contextual considerations, what is the long-term value for money of treatment with **Spinraza** versus **supportive care** in patients with later-onset (Type II/III) SMA?

Low

Intermediate

High

14. Given the available evidence on comparative effectiveness and incremental cost-effectiveness, and considering other benefits, disadvantages, and contextual considerations, what is the long-term value for money of treating patients with **Spinraza before symptoms develop** versus **best supportive care**?

Low

Intermediate

High

² Note: Zolgensma value votes will be taken only if the therapy's price has been announced by the time of the March 7 public meeting.