



Deflazacort, Eteplirsen, and Golodirsen for Duchenne Muscular Dystrophy: Effectiveness and Value

Draft Questions for Deliberation and Voting: July 25, 2019 Public Meeting

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

Clinical Evidence

1. For patients with DMD, is the evidence adequate to demonstrate that the net health benefit of **deflazacort** (Emflaza® PTC Therapeutics) is superior to that provided by **prednisone**?

Yes No

2. For patients with DMD amenable to exon 51 skipping, is the evidence adequate to demonstrate that the net health benefit of **eteplirsen** (Exondys 51™, Sarepta Therapeutics) added to corticosteroids and supportive care is superior to that provided by **corticosteroids and supportive care alone**?

Yes No

3. For patients with DMD amenable to exon 53 skipping, is the evidence adequate to demonstrate that the net health benefit of **golodirsen** (SRP-4053, Sarepta Therapeutics) added to corticosteroids and supportive care is superior to that provided by **corticosteroids and supportive care alone** in patients with DMD?

Yes No

Potential Other Benefits and Contextual Considerations

Deflazacort

4. Is it likely that treatment with **deflazacort** offers one or more of the following potential “other benefits” that are not adequately captured in the base-case cost-effectiveness model? (select all that apply)¹
 - a. **Compared to prednisone, deflazacort** will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
 - b. There are other important benefits or disadvantages that should have an important role in judgments of the long-term value for money of **deflazacort**: _____

5. Are any of the following contextual considerations important in assessing **deflazacort**’s long-term value for money? (select all that apply)¹
 - a. **Deflazacort** 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. **Deflazacort** is intended for the care of individuals with a condition that represents a particularly high lifetime burden of illness.
 - c. There is significant uncertainty about the long-term risk of serious side effects of **deflazacort**.
 - d. There is significant uncertainty about the magnitude or durability of the long-term benefits of **deflazacort**.
 - e. There are additional contextual considerations that should have an important role in judgments of the value of **deflazacort**: _____

Eteplirsen and Golodirsen

6. Is it likely that treatment with **eteplirsen or golodirsen** offers one or more of the following potential “other benefits” that are not adequately captured in the base-case cost-effectiveness model? (select all that apply)¹
 - a. **Eteplirsen and golodirsen** will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
 - b. **Eteplirsen and golodirsen** will significantly reduce caregiver or broader family burden.
 - c. **Eteplirsen and golodirsen** will have a significant impact on improving patients’ ability to return to work and/or their overall productivity.
There are other important benefits or disadvantages that should have an important role in judgments of the value of **eteplirsen and golodirsen**: _____

¹Votes will be taken on an abbreviated list of potential other benefits and contextual considerations. Although ICER’s value framework identifies a broader list, the omitted options were determined not to apply to the treatment in question.

7. Are any of the following contextual considerations important in assessing **eteplirsen's and golodirsen's** long-term value for money? (select all that apply)¹
- a. **Eteplirsen and golodirsen** are 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. **Eteplirsen and golodirsen** are intended for the care of individuals with a condition that represents a particularly high lifetime burden of illness.
 - c. **Eteplirsen and golodirsen** are the first to offer any improvement for patients with this condition.
 - d. There is significant uncertainty about the long-term risk of serious side effects of **eteplirsen and golodirsen**.
 - e. There is significant uncertainty about the magnitude or durability of the long-term benefits of this **eteplirsen and golodirsen**.
 - f. There are additional contextual considerations that should have an important role in judgments of the value of this eteplirsen and golodirsen: _____

Long-term Value for Money²

8. 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 **deflazacort** versus **prednisone**?
- a. Low long-term value for money
 - b. Intermediate long-term value for money
 - c. High long-term value for money
9. 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 **eteplirsen** versus **supportive care and corticosteroids alone**?
- a. Low long-term value for money
 - b. Intermediate long-term value for money
 - c. High long-term value for money

² No value votes will be taken for golodirsen (SRP-4053, Sarepta Therapeutics) as its price is currently unknown since the FDA has yet to issue a decision.