Expert peer review on application for adding Nifurtimox-Eflornithine (NECT) FDC

1. Assessment of efficacy
   a. Have all relevant studies on efficacy been included
      Yes √  No (if no, please provide reference and information)
   b. Summarize the data on efficacy, in comparison to what is listed in EML where applicable (limit to 2 to 3 sentences)
      From the pharmacovigilance study by Jose R. Framo et al 2010-2011 NECT seems to have adequate efficacy.
   c. Please provide any additional relevant information with reference

2. Assessment of safety
   a. Have all relevant studies on safety been included
      Yes √  No (if no, please provide reference and information)
   b. Summarize the data on safety, in comparison to what is listed in EML where applicable (limit to 2 to 3 sentences)
      From the pharmacovigilance study by Jose R Framo et al in 2010-2011
      - 60% had adverse event;
      - 1.1% had serious adverse events 0.5% fatality rate
      - The best option for treatment of second stage gambiense Human African Trypanosomiasis
   c. Please provide any additional relevant information with reference
      No safety data available in children but it has been used in children (data from the same study)

3. Assessment of cost and availability
   a. Have all relevant data on cost and availability provided
      Yes  No (if no, please provide reference and information)
b. Summarize the data on cost and cost effectiveness, in comparison to what is listed in EML where applicable (limit to 2 to 3 sentences)

From the application there is no adequate data that gives specific cost/cost effectiveness.

c. Please provide any additional relevant information with reference

d. Is the product available in several low and middle income countries?

4. Assessment of public health need
   a. Please provide the public health need for this product (1-2 sentences)

It has been found to be effective for sleeping sickness, multicentre clinical trials have confirmed there is a need for it.

b. Do guidelines (especially WHO guidelines) recommend this product? If yes, which ones? List 1 or 2 international preferable

   Yes ✓ (At present it is not on the WHO Model Essential Medicines List)
   - Centre for Diseases Control and Prevention (CDC)
   - WHO Collaborating Centre for African Trypanosomiasis at CDC

5. Are there special requirements for use or training needed for safe/effective use?
   If yes, please provide details in 1-2 sentences

6. Is the proposed product registered by a stringent regulatory authority?

   Yes ✓ No

7. Any other comments

   No

8. What is your recommendation to the committee (please provide the rationale)

There are few medicines available for Trypanosomiasis. Despite the one case fatality in the study, the benefits outweigh risks and NECT should be included in the essential list.