20th Expert Committee on Selection and Use of Essential Medicines

Peer Review Report #1

Bedaquiline

(1) Does the application adequately address the issue of the public health need for the medicine?

Yes ☑ No ☐

The evidence submitted and approved so far is adequate with regard to the public health need for the medicine.

(2) Have all important studies that you are aware of been included in the application?

Yes ☑ No ☐

It needs to be said that the FDA approval was based on a phase 2b clinical trial, with recommendation to develop phase 3 studies and safety ones, after started clinical use.

(3) Does the application provide adequate evidence of efficacy/effectiveness of the medicine for the proposed use?

Yes ☑ No ☐

The problem with the drug is related to cardiac toxicity (QT prolongation) and the higher number of deaths occurred in the group that used BDQ in the double blind studies. Phase 3 further studies as well as “real life use” following current recommendations and limitations should demonstrate efficacy and confirm effectiveness.

(4) Is there evidence of efficacy in diverse settings and/or populations?

Yes ☑ No ☐

No, this is the reason why the WHO Task Force group for new treatments for tuberculosis recommended the clinical use of the drug in the following conditions: failed MDR, pre-XDR and XDR cases, with exceptions of pregnant women, HIV patients, persons over 65 years old, and children.
(5) Has the application adequately considered the safety and adverse effects of the medicine? Are there any adverse effects of concern, or that may require special monitoring?

Yes ☐ No ☐

Cardiac toxicity. Hepatic impairment.

ADDITIONAL CONSIDERATIONS:

(6) Are there special requirements or training needed for the safe, effective and/or appropriate use of the medicine?

Yes ☐ No ☐

Observe comments given for question 4

(7) Are there any issues regarding the registration of the medicine by regulatory authorities? (e.g., recent registration, new indications, off-label use)

Yes ☐ No ☐

The drug has been approved by FDA in December 2013, for MDRTB, pre-XDR and XDR cases.

(8) Is the medicine recommended for use in a current WHO GRC-approved Guideline (i.e., post 2008)?

Yes ☐ No ☐

(9) Please comment briefly on issues regarding cost and affordability of this medicine.

1. Although there are defined three different levels of countries for pricing the drug, it is still too expensive and not affordable by a large number of countries.
2. Countries can consider supply of this product for individual patient basis (compassionate use).
3. Special deployment as donation from the producer, following countries priorities and WHO recommendations should provide access to it.

(10) Any additional comments?

No

(11) Please summarise the action you propose the Expert Committee takes.

To include in the WHO Model EML

1. The need for new drugs for MDR forms of tuberculosis is urgent
2. Bedaquiline permits to be used with other TB compounds (making optimal basic therapy) in multiresistant cases.
3. Proper Diagnostic and treatment reference facilities although not available in many countries should be defined to use the drug
4. Safety issues must be stringently observed.
5. No sufficient evidence on efficacy / effectiveness or risk in HIV aids patients.