## African Vaccine Regulatory Forum (AVAREF)

# STATISTICAL ASSESSMENT

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|---|---|
| **Study's full title** | |
| **Short title** | |
| Protocol No. | |
| Version No. | |
| Investigational medical product | |
| Date of the review | |
| Reviewer's name | |

| **Version** | **Date** | **Comments** |
|---|---|---|
| Version 1 | May 2019 | Piloted at the domestication workshop with the SADC and EAC in Johannesburg, South Africa |
| Version 2 | October 2019 | To be tabled for adoption at the Avaref Assembly in Victoria Falls, Zimbabwe |

General information for reviewers:

- Text provided in blue and in the footnotes is indicative and aims to highlight aspects that need to be taken into account during the assessment. It should be deleted prior to sending the final assessment to the sponsor
- The not applicable (NA) box should be checked off when the information is not required. A justification from the sponsor is expected in this case. The assessor is to comment on the acceptability of the information

## Statistical/methodological assessment

### Study plan and design

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|---|---|
| **Type of design:** | |
| **Controlled/non controlled?** | Controlled ☐ Non controlled ☐ |
| **Randomized?** | Yes ☐ No ☐ |
| **Blinding (masking)?** | Open-label ☐ Blinded evaluator ☐<br>Single-blind ☐ Double-blind ☐ |
| **Brief** description of the study plan and design: | |
| Is the proposed study design acceptable? | Yes ☐ No ☐ |
| **Workspace:** | |
| **Comments:** | |

### Randomization and blinding

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| **Brief** description of the randomization and blinding procedures: |
| **Workspace:** |
| **Comments:** |

### Sample size, trial power, and level of significance used

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| Planned number of participants to be enrolled: | |
| Are the sample size calculation and justification acceptable? | Yes ☐ No ☐ |
| Are the trial power and level of significance acceptable? | Yes ☐ No ☐ NA ☐ |
| **Brief** description of the sample size, trial power, and level of significance: | |
| **Workspace:** | |

| **Comments:** |
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## Planned analysis

| **Brief** description of the planned analyses: | | | |
|---|---|---|---|
| Do the analyses reflect the study objectives? | Yes ☐ | No ☐ | Other, comment ☐ |
| Are the methods appropriate? | Yes ☐ | No ☐ | Other, comment ☐ |
| Are the considerations regarding missing values, unused, and spurious data acceptable? | Yes ☐ | No ☐ | Other, comment ☐ |
| Are the considerations regarding multiplicity acceptable? | Yes ☐ | No ☐ | Other, comment ☐ |
| Is a sensitivity analysis planned? | Yes ☐ | No ☐ | Other, comment ☐ |
| Are the planned analyses appropriate? | Yes ☐ | No ☐ | |
| **Workspace:** | | | |
| **Comments:** | | | |

## Analysis sets

**Efficacy sets (trial-dependent):**

1. Full analysis set[1]
2. Per protocol set[2]

Brief description of the efficacy sets

[1]This is a set of patients that is as close as possible to the ideal implied by the intention-to-treat principle. It is derived from the set of all randomised patients. The intention-to-treat principle asserts that the effect of a treatment policy can be best assessed on the basis of the intention to treat a patient, ie the planned treatment regimen, rather than the actual treatment given. It has the consequence that patients allocated to a treatment group should be followed up, assessed and analysed as members of that group irrespective of their compliance to the planned course of treatment

[2]This is the set of data generated by the subset of patients who complied with the protocol sufficiently to ensure that these data would be likely to exhibit the effects of treatment, according to the underlying scientific model. Compliance covers exposure to treatment, availability of measurements, and absence of major protocol violations

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| **Safety analysis set**[3]<br><br>Brief description of the safety set |
| **Workspace:**<br><br>Do the analysis sets match the trial's objectives and endpoints? Elaborate |
| **Comments:** |

**Interim analysis**

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|---|---|
| Does the trial have a data safety monitoring committee? | Yes ☐ No ☐ |
| Is there an interim analysis planned for this trial? | Yes ☐ No ☐ |
| **Brief** description of the interim analysis(es) (if applicable): | |
| **Workspace:** | |
| **Comments:** | |

**Assessor's overall conclusion on the statistical part**

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|---|---|
| The statistical aspects of the application are acceptable | Yes ☐ No ☐ |
| Supplementary information needs to be provided (refer to the list of requests for additional information) | Yes ☐ No ☐ |
| **Workspace:** | |
| **Comments:** | |

**Requests for additional information on biostatistics**

[3] This set usually includes those patients who received at least one dose of the IMP