**Mod. 5.3.5 - Summary of main efficacy results**

*A tabulated summary of the most relevant information to describe the efficacy data generated in the main trial(s) should be presented. This summary should be tailored to the data set which was used by the CHMP for its conclusion on efficacy. Therefore, it will be important to reflect the results from the analysis that was deemed most relevant (preferably (m)ITT and PP, but maybe also clinically defined sub-group [pre-specified or post-hoc], etc.). The pre-specified primary analysis should be presented in any case.*

*The following template table should be used to display the data for the specific studies. The level of detail should be adjusted to the data later needed for the discussion and conclusion on benefits, as well as the benefit-risk assessment. Treatment groups should be presented in separate cells, and so should be information on different analysis sets (e.g. ITT and PP).
Reasons for drop-outs should be summarised.*

*Different main trials should be presented in separate tables. No additional text is foreseen in this section apart from these tables. A detailed description of these trials with for instance information on design and power calculation is presented in other sections. The safety data is subject to the section “Clinical safety”.*

The following tables summarise the efficacy results from the main studies supporting the present application. These summaries should be read in conjunction with the discussion on clinical efficacy as well as the benefit risk assessment (see later sections).

**Table XXX. Summary of efficacy for trial <trial>**

|  |
| --- |
| **Title:** <title> *{as indicated on the study report}* |
| Study identifier | <code>*{list all codes starting with the protocol number followed by – as available - EudraCT number, ISRCT number, other codes that allow cross-referencing to publications}* |
| Design | <free text>*{describe key elements of the design (cross-over, parallel, factorial, dose- escalation, fixed-dose response) including randomization, blinding, allocation concealment, mono-/multi-centre, etc.}* |
| Duration of main phase:Duration of Run-in phase: Duration of Extension phase: | <time><time> <not applicable><time> <not applicable> |
| Hypothesis | <Superiority> < Equivalence> <Non-inferiority> <Exploratory: specify> |
| Treatments groups*{add as many rows as needed to describe the treatment groups}* | <group descriptor>*{provide abbreviation for use later in the table of the results section}* | <treatment>. <duration>, <number randomized> |
| <group descriptor> | <treatment>. <duration>, <number randomized> |
| <group descriptor> | <treatment>. <duration>, <number randomized> |
| Endpoints and definitions*{add as many rows as needed to describe the endpoints; for the secondary endpoints select the ones considered most relevant and reported in the results section}* | <Co->Primary endpoint | <label>*{generate abbreviation for use later in the table of the results section}* | <free text> *{provide brief description}* |
| <Secondary><other: specify> endpoint | <label> | <free text> *{provide brief description}* |
| <Secondary><other: specify> endpoint | <label> | <free text> *{provide brief description}* |
| Database lock | <date> |
| **Results and Analysis***{present the result separate for each analysis that is considered relevant for the conclusion on the trial; in any case the pre-specified primary analysis should be presented}* |
| **Analysis description** | **Primary Analysis** |
| Analysis population and time point description | <Intent to treat> <Per protocol> <other: specify>*{consider adding a brief description of the definition of the population}*<time point> |
| Descriptive statistics and estimate variability | Treatment group | <group descriptor>*{as per above terminology}* | <group descriptor>*{as per above terminology}* | <group descriptor>*{as per above terminology}* |
| Number of subject | <n> | <n> | <n> |
| <endpoint>*{label as above}*(<statistic>)*{e.g. mean, median, etc}* | <point estimate> | <point estimate> | <point estimate> |
| <variability statistic>*{e.g. standard deviation, confidence interval, etc}* | <variability> | <variability> | <variability> |
| <endpoint> (<statistic>) | <point estimate> | <point estimate> | <point estimate> |
| <variability statistic> | <variability> | <variability> | <variability> |
| <endpoint> (<statistic>)<variability statistic> | <point estimate><variability> | <point estimate><variability> | <point estimate><variability> |
| Effect estimate per comparison*{add as many rows as needed to describe the relevant statistical testing performed}* | <Co->Primary endpoint | Comparison groups | <group descriptors>*{as per above terminology}* |
| <test statistic> *{e.g. difference between groups}* | <point estimate> |
| <variability statistic>*{e.g. confidence interval, etc}* | <variability> |
| P-value *{indicate statistical test used, e.g. ANOVA}* | <P-value> |
| <<Co->Primary ><Secondary><other: specify> endpoint*{indicate endpoint using terminology as per section “Endpoint and definitions}* | Comparison groups | <group descriptors> |
| <test statistic> | <point estimate> |
| <variability statistic> | <variability> |
| P-value | <P-value> |
| <<Co->Primary ><Secondary><other: specify> endpoint | Comparison groups | <group descriptors> |
| <test statistic> | <point estimate> |
| <variability statistic> | <variability> |
| P-value | <P-value> |
| Notes | <free text>*{consider amongst others the following information:**reasons for drop-outs**critical findings with regard to the analysis}* |
| **Analysis description** | **<Secondary analysis> <Co-primary Analysis> <Other, specify: >***{also indicate if the conduct of the analysis was pre-specified}* |
| *{repeat all the above sections for analysis that is each considered relevant}* |  |