INFORMATION FOR THE COMPLETION OF A SUBMISSION PACKAGE FOR THE WHO COORDINATED SCIENTIFIC ADVICE PROCEDURE FOR HEALTH PRODUCT R&D MEDICINES

The WHO Coordinated Scientific Advice (CSA) Submission Package should provide a ***comprehensive scientific overview of the product and its development programme***. The level of detail provided within each section will depend on the questions raised by the applicant and subject to CSA.

Applications should be uploaded in electronic format through a link provided by WHO.

1. STRUCTURE OF THE SUBMISSION PACKAGE

Applicants are requested to include a Table of Contents following the outline of this document and following instructions as provided in each section.

2. APPLICANT INFORMATION

Name of Organization		
Organization Address	Street Name and No.:	
	City:	
	Province/State:	
	Postcode:	Country:
Contact person	Name	
	Title	
	Email	
	Telephone number(s)	

3. SUMMARY OF PRODUCT CHARACTERISTICS

Product Name	
Product type	
Active Ingredient(s)	

WHO/SCI/RFH/2021.02

Concentration of Active Ingredient(s)	
INN	
Pharmacotherapeutic group	
Disease(s) or condition(s) addressed by the product	

4. BACKGROUND INFORMATION

4.1. Disease or condition addressed by the product

Applicants are asked to describe the disease or condition being addressed by the product under development including its symptoms and outcomes if not treated, the global disease burden and information on current standard of care. Please provide a list of references to support your statements.

4.2. Product information:

Applicants are requested to provide a detailed description of the product including:

- mode of action, chemical structure and pharmacological classification;
- intended indication: information should be included on the role of the proposed product in the treatment pathway (first, second, third line treatment);
- target population;
- whether it is intended as a combination treatment or monotherapy;
- information on the type of treatment (e.g., curative, palliative, preventive, disease-modifying etc.);
- whether a companion medical device, in vitro diagnostic or medical intervention is required for any treatment decision or administration;
- method of administration: including administration route, dose, frequency of administration and the duration of use based on available data at the time of submission;
- likely product stability and storage requirements;
- Drug-drug interactions.

5. SPECIFIC QUESTIONS FOR CSA

This section should include a rationale for seeking advice, with a clear, concise and unambiguous description of the question(s). Questions should be specific and clearly labelled according to the

expertise required for the assessment and numbered sequentially. Applicants should also provide a position on each of the questions with an accompanying justification for the chosen methodology.

Questions should focus on data needs for **policy development and PQ processes**, e.g. review of proposed choice and number of endpoints, target population and intended use setting, stability.

5.1. Questions on PQ process

This section will include all questions pertaining to prequalification assessment requirements.

5.2. Questions on policy development

This section will include all questions pertaining to requirements for WHO policy development and recommendations.

Note: Questions regarding issues that are relevant to both policy development and prequalification assessment will be clearly marked and labelled as such.

6. BACKGROUND

6.1. Quality background information

Active substance/ Finished product, including Good Manufacturing Practice (GMP).

6.2. Non-clinical background (pharmacology, pharmacokinetics, pharmacodynamics, and toxicology)

Applicants are requested to include a list of completed, ongoing and planned studies. For each study, the following information should be provided:

- Study description
- Study identifier
- Study objectives and design features
- Study participants
- GLP status
- Date of initiation
- Date of completion/estimated completion
- A summary of study findings or expected outcomes.

6.3. Clinical background

In this section, applicants are requested to include a list of completed, ongoing and planned studies. For each study, the following information should be provided:

- Study description
- Study identifier (including clinical trial registry number)
- Study objectives
- Trial design features including randomization, statistical methods, time point frequency of data collection

- Doses
- Treatment duration
- Comparator
- Patient population, patient sub-groups, patient numbers
- Centres that will be participating in the pivotal clinical studies
- GCP status
- Date of initiation
- Date of completion/estimated completion
- A summary of study findings or expected outcomes, including primary and secondary endpoints, patient-reported outcomes (PROs), Adverse Events (AEs)

For completed studies, information can be provided as a study report in the form of an annex.

6.3.1. Clinical efficacy

The application should include a detailed overview of the clinical development programme based on a comprehensive presentation of the main clinical results so far and the planned pivotal clinical studies with full methodology. This section should describe the most important findings accumulated so far and identify any current challenges in the clinical development programme.

6.3.2. Clinical safety

The application should include a general overview of the safety profile of the product based on a comprehensive presentation of the safety database, adverse events observed so far, serious adverse events and deaths, laboratory findings, safety-related discontinuations, specific safety findings, immunological events, and safety in specific populations (e.g. pregnancy).

6.4. Relative effectiveness

Applicants are requested to describe any plans or guidance for evidence generation on relative effectiveness based on clinical trial efficacy.

6.5. Economic analysis

Applicants should describe any plans for economic analysis and describe the methodology selected to carry out the studies.

7. PREVIOUS SCIENTIFIC ADVICE RECEIVED

Applicants are requested to briefly describe any previous scientific advice received from other organizations (e.g. European Medicines Agency (EMA)/Committee for Medicinal Products for Human Use (CHMP) Scientific Advice, FDA, other national regulatory authorities).

8. LIST OF REFERENCES

List of references: any relevant publications included in the list of references should be annexed (in .pdf format).

9. LIST OF ANNEXES

Annexes should include any information potentially relevant to the questions subject to CSA, e.g.:

- Investigators' brochure
- Study protocols (final, draft or outline/synopsis)
- Study reports (final/draft/synopsis)
- Any published data

Note: press releases are not considered acceptable as a source of information.