NICE National Institute for Health and Care Excellence

CDA-AMC Canada's Drug Agency
L'Agence des médicaments du Canada
Drugs. Health Technologies and Systems. Médicaments, technologies de la santé et systèmes.

Briefing Book Guidance: NICE and Canada's Drug Agency (CDA-AMC) Parallel Scientific Advice

Preparing a Briefing Book

- Use the provided template to submit a Briefing Book for NICE and CDA-AMC Parallel Scientific Advice in Microsoft Word format.
- The company may insert its logo on the title page.
- Enter relevant information about your product or company into each section.
- The length of the briefing book should not exceed 50 pages, excluding list of references, or 10Mb in size.
- Any annexes or essential self-standing documents such as study protocols, reports, should be submitted as separate documents in Word or PDF format. Please do not embed these documents directly into your briefing book.
- The template should be used as a guide and judgement exercised as to which sections are relevant to the product for which advice is being sought.
- Additional sections may be inserted into the briefing book when required. Where relevant data are missing, this should be explained and an indication given as to when they may become available.
- Questions directed to CDA-AMC and NICE scientific advice should be followed by the company's explanation of its position. The wording of the questions should be clear and concise.
- As the focus is on parallel engagement with NICE and CDA-AMC, please limit questions directed to CDA-AMC only or to NICE only to no more than 2 questions each.
- It is not necessary to reference all statements in the briefing book; however, references should be provided if they relate to the methodology being proposed or the questions asked.
- Do not include preclinical data, pharmacodynamics or pharmacokinetic data unless specifically relevant to questions for scientific advice
- Results of phase 2 trials are not required if these are not available by the time of briefing book submission.

Selected section-specific points

3.1 Lay Summary

This should be a summary of your proposals written with a non-technical, general audience in mind. It will be used most frequently in projects where we identify a suitable patient expert and, ideally, should not exceed 800 words. Please try to use short, clear sentences and everyday English words in place of complex words, where possible. The text should provide brief answers to the key questions: Who, What, Where, When, Why and How?

3.3 Treatment Options and Relevant Guidelines

- Current clinical care pathway and variations across the NHS and Canada
- Relevant guidance
- Current clinical outcomes

- Include any products in established use regardless of the licence status
- Include non-drug treatment/procedure options if appropriate
- Include new treatments on the horizon in advanced stages of development if known

3.5 Regulatory Scientific Advice

Indicate if regulatory scientific advice has been or will be obtained on the product. While the minutes of regulatory advice might be of interest, the company is not required to submit these as part of this briefing book.

3.6 Scientific Advice from Health Technology Agencies

Indicate if scientific advice from other health technology agencies has been or will be sought on the product.

4.3 Indication and Target Population

- Specify clearly the intended indication(s).
- Specify the position of the product in the treatment pathway (for example, first line, second line, third line, screening pre-treatment, monitoring during treatment, et cetera).
- State if it is combination therapy or monotherapy.
- Aim of treatment (preventative, curative, palliative, symptomatic, disease modifying).
- Target population.

4.5 Summary of Patient Engagement Information

Briefly describe if you have engaged with patients or patient organisations as part of your product development programme, and the nature of that engagement. If so, what issues have you explored with patients or patient organisations? For example, real world applicability, limitations of the trials, outcomes of importance to patients, mode of administration, clarity of definitions etc.

4.6 Clinical Data Available to Date

Describe the clinical trials performed to date and provide results if available.

If the administration of the product is associated with the use of a diagnostic test, a medical device or a medical procedure, provide relevant information, for example, describe if:

- additional monitoring is required for the product
- additional resources and training are required
- there are adverse effects and describe the management of these adverse effects.

5. Product Value Proposition

This section of the briefing book is mandatory. Describe value propositions for the product and how the trial evidence will be used to support these.

6. Proposed Clinical Development Programme

For each trial, describe the objective, design (randomisation, blinding, etc.), location(s), doses and duration of treatment, comparator(s), number of subjects and description of studied population and end point(s). Provide a trial diagram if available. Specifically describe:

- Patient population (inclusion and exclusion criteria, patient characteristics). Discuss any differences between the licensed population and the population for the analysis.
- Subgroups identified (provide justification).
- Selected comparators (provide justification).
- End points (primary, secondary, other). All scales and scores that will be used for end point measurement should be presented and their validity should be reported.
- Study duration and follow-up.
- Crossover design (if applicable).
- Relevant methodologies and analyses of trial data.
- Data gaps expected in the evidence at the time of the initial appraisal.
- Provide plans to address these data gaps at the current time and following licensing.

7. Proposed Economic Analysis

This section is optional if no questions on economic evaluation are submitted to NICE and CDA-AMC Scientific Advice. If plans for the economic evaluation are provided, these should include to the extent possible:

- Description of the proposed model (diagram, modelling approach, time horizon, perspective).
- Data collection plans to inform the model:
  - Evidence synthesis/meta-analysis – sources of evidence
  - Comparators – mixed treatment comparisons and indirect comparisons and evidence available
  - Trial end points used to derive health outcomes in the model
  - Quality of life – source and methods, tools used to measure quality of life
  - Incorporation of adverse effects
  - Resource use – sources and methods, tools used to measure resource utilisation
- Methodological Approaches:
  - Extrapolation – assumptions and data sources
  - Continuation rules
  - Use of surrogate outcomes
  - Planned sensitivity analyses

Evidence gaps and model assumptions should be described.

If you have further questions about the content of the briefing book, please contact NICE Scientific Advice and/or CDA-AMC Scientific Advice for an informal telephone discussion prior to your briefing book submission.