## Pathways approach

# Process statement (renal cell carcinoma)

### Project summary

1. The overall goal of this work is to use model-based approaches to inform the development of guidance. Time and resource efficiencies for NICE and external stakeholders are expected to be found by assessing multiple technologies in a disease pathway and building an evolving core economic model.

2. The phases of work are as follows:

    - Phase 1 – Scoping and preparatory work
    - Phase 2 – Academic synthesis and modelling work
    - Phase 3 – Evaluation and decision-making

3. The technology appraisal processes are detailed in the NICE health technology evaluations guidance development manual. Pathway appraisals correspond to the steps in the guidance development manual but are re-sequenced and with different timelines in order to allow the exploration of a new approach (in a test and learn environment) that will develop overall process efficiencies and improvements. The overview of each phase outlined below details how the guidance development steps in the manual are followed and timelines are available in tables 1 to 4.

### Phase 1 – Scoping and preparatory work

4. The activities in phase 1 broadly correspond to section 2 of the guidance development manual. See section 1.3.10 to section 1.3.19 of the manual for details on the nomination and selection of experts.

5. The scoping process aims to define what question the evaluation will answer and what will and will not be included. The scope provides the framework for the evaluation.

6. A scoping workshop will take place between NICE, External Assessment Groups (EAGs) and relevant stakeholders including company representatives, clinical experts, and patient representatives.

7. The scoping workshop will outline the renal cell carcinoma (RCC) care pathway. This will include any sequence of tests and treatments, any

subgroups of interest, patient characteristics and possible comorbidities for the relevant population.

8. To promote maximum engagement with the process the standard stakeholder list has been expanded to include companies with technologies already recommended for treating renal cell carcinoma, that are not expected to be comparators to the technologies being evaluated.

**Phase 2 – Academic synthesis and modelling work**

9. The activities in phase 2 broadly correspond to section 5.6 of the guidance development manual.

10. The EAG will develop an analysis plan outlining what the EAG will do during the evaluation and the information it will provide in the external assessment report. This will be based on the draft scope and consultation with clinical experts, and the scope that was updated after the scoping workshop and Phase 1 of the evaluation.

11. The EAG will then carry out an assessment of the publicly available clinical outcomes within the disease area, including accessing data on novel technologies (see section 5.6.15 of the guidance development manual). The assessment will include:

    - Systematic evidence reviews for technologies entering the disease pathway

    - Targeted evidence review of systemic treatments for existing technologies in the treatment pathway, prioritising searches based on volume of results

    - Targeted evidence review for data input parameters and natural history for the economic evaluation.

12. Construction of economic model(s) of the progression and outcomes of the disease.

13. Model validation will be completed during phase 2. Validation will be aligned with the guidance development manual. It is expected that model outputs will be compared to the data used as model inputs (including any real world evidence), to ensure accuracy of model structure and data derivation. The model will then be compared to the projections from other models previously used for NICE technology appraisals in the same decision node.

14. The EAG will develop a preliminary external assessment report, presenting an assessment of the publicly available clinical outcomes and costs throughout the disease pathway. The report will also provide a summary of the expected

model structure and transparently document and justify any expected assumptions.

15. The preliminary external assessment report will be produced and sent to stakeholders to provide comments. Stakeholders will have at least 21 working days to comment on the preliminary external assessment report.

16. NICE will invite stakeholders, including companies with new technologies being evaluated and comparator companies involved in the decision nodes, to submit evidence and comments. Stakeholders will have at least 21 working days to provide evidence.

17. NICE will be seeking evidence from companies that is not included in the preliminary external assessment report, it is expected that this data will primarily comprise clinical trial data.

18. The EAG will incorporate relevant stakeholder evidence into its model and provide responses to consultation comments on the preliminary external assessment report. Responses may include clarifications, rebuttals, or where appropriate summaries of adaptations to the model structure.

19. The EAG will provide a summary of its base case. The EAG will also provide scenarios with alternative assumptions that it did not consider suitable but which were preferred by stakeholder. The EAG will produce a final external assessment report which will present an assessment of the clinical outcomes and cost effectiveness of the technologies. The report should also provide a summary of the model structure, transparently document and justify any assumptions made. Key issues per decision node should also be documented and areas of data paucity should be highlighted. The EAG's assessment should highlight the uncertainties in the evidence.

20. A lay summary of the model for patient and clinical experts will also be developed by the EAG.

21. The final external assessment report will be sent to stakeholders to provide comments. Stakeholders will have at least 21 working days to comment on the final external assessment report.

22. During the consultation on the final external assessment report, NICE and the lead team committee members will also risk assess the external assessment report and model to highlight differences in approaches and additional analyses the EAG should model.

23. Information will be handled as outlined in section 3.2 of the interim proportionate approach methods and process guide. Information marked as confidential should be kept to an absolute minimum.

24. An executable version of the EAG's model will be provided online to stakeholders. It may use dummy data where data from the company whose technology is being appraised is marked as confidential.

25. Previous models submitted to NICE in the pathway will be used (with permission from the original submitting company) for validation purposes. Scenario analyses run by the EAG for validation of the model may encompass values from previous submissions within a range. The EAG will create a confidential appendix to its report where necessary. This will only be shared with NICE and the committee.

**Phase 3 – Evaluation and decision-making**

26. The committee's consideration of the evidence, draft guidance consultation (where needed) and development of draft final guidance will follow the steps outlined in sections 5 of the guidance development manual.

27. Committee recommendations on the specific technology will be as per section 6 of the guidance development manual.

28. Finalising and publishing the guidance will be as per section 7 of the guidance development manual.

29. Committee recommendations on the pathways assumptions will be summarised in a separate report (Pathways Guidance), and will include conclusions about the model structure, sources to estimates baseline event rates, utilities, resource costs and severity at different decision nodes. The Pathways Guidance will be sent to stakeholders to provide comments, aligning with the principles in section 7 of the guidance development manual.

**Table 1: Timelines for Phase 1 for the RCC pilot**

| | Phase 1 |
|---|---|
| December 2022 | External Assessment Group starts work |
| January 2023 | Scoping workshop |

**Table 2: Timelines for Phase 2 for the RCC pilot**

| | Phase 2 |
|---|---|
| 28 March 2023 | Stakeholder information meeting |
| April 2023 | Company evidence submission |
| April 2023 | Preliminary External Assessment Report |
| April 2023 | Consultation on Preliminary External Assessment Report |
| May 2023 | Non-company stakeholder evidence submission |

| | |
|---|---|
| July 2023 | Final External Assessment Report |
| July to August 2023 | Consultation on Final External Assessment Report and executable model |

**Table 3: Timelines for Phase 3 for the RCC pilot**

| | Phase 3 |
|---|---|
| 20 September 2023 | **First appraisal committee meeting** |
| October 2023 | Draft final guidance issued for appeal |
| Dec 2023/Jan 2024 | Final guidance published |

**Table 4: If draft final guidance cannot be produced following the first appraisal committee meeting, the subsequent indicative timelines are currently:**

| | Phase 3 |
|---|---|
| 20 September 2023 | **First appraisal committee meeting** |
| November 2023 | Draft guidance consultation |
| Q1 2024 | Second appraisal committee meeting |
| Q1 2024 | Draft final guidance issued for appeal |
| Q1 2024 | Final guidance published |