

Scottish Medicines Consortium (SMC)

Process guide for submitting companies

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www.scottishmedicines.org.uk

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1 Role and remit

1.1 About the Scottish Medicines Consortium (SMC)

The Scottish Medicines Consortium (SMC) provides NHS boards and Area Drug and Therapeutics Committees (ADTCs) across NHSScotland with a single source of advice about the clinical and cost effectiveness of newly licensed prescription-only medicines, new indications and new formulations of established medicines.

1.2 SMC remit

Some medicines (described in Table 1) are considered outwith SMC remit and a submission is not required. Before making a submission to SMC, the Marketing Authorisation Holder (MAH) should consider whether any of the following exclusions criteria apply. If you are unsure whether a product falls within these exclusion criteria, please contact the SMC Secretariat for advice.

Table 1: Outwith remit criteria

1.	The medicine was initially licensed and made available to the United Kingdom (UK) market prior to 31 January 2002, for the indication in question, that is, prior to the inception of SMC.
2.	The medicine is not a Prescription Only Medicine (PoM). The SMC remit covers proprietary medicines categorised as PoMs only and excludes Pharmacy and General Sales List medicines.
3.	The medicine is used in immunisation and guidance on its use is issued by the Joint Committee on Vaccination and Immunisation.
4.	The product is a medical device and is not licensed as a medicine by the Medicines and Healthcare products Regulatory Agency (MHRA).
5.	The product is used in diagnosis not treatment. SMC does not consider medicines licensed for use only in a diagnostic setting.
6.	The product is classified as a blood product (excluding anti-bradykinin and C1 inhibitor therapies). SMC reserves the right to request a submission for a new blood-derived product if an assessment of clinical and cost effectiveness is required by NHS boards. Please consult the SMC Secretariat for further advice.
7.	The product is a medical gas.
8.	The product is a parenteral preparation for fluid and electrolyte imbalance or parenteral nutrition.

9.	The product is used as a supportive intervention in surgical procedures or wound management. Please consult the SMC Secretariat for further advice.
10.	The product is used for the acute treatment of poisoning.
11.	The product is a medicine used in tropical diseases.
12.	There has been a change to the MAH, trade name or manufacturer, with no increase in product cost.
13.	The Marketing Authorisation update is solely for a new strength or new presentation of an existing proprietary medicine(s) (accepted for use by SMC or which predates SMC), with no associated change to the licensed indication or route of administration and the new product costs the same per patient or less. Please note that if a Patient Access Scheme (PAS) is in place for the existing proprietary medicine then the MAH is asked to contact the Patient Access Scheme Assessment Group (PASAG) Secretariat at nss.np-pasag@nhs.scot to update the existing PAS application to include the new product.
14.	The product is a new formulation (or fixed-dose combination in a single pharmaceutical form) of an established medicine(s) which is either: - An oral formulation of an established generic medicine intended for patients unable to swallow tablets or capsules, or - An alternative formulation (or fixed-dose combination in a single pharmaceutical form) of an established medicine(s) (accepted for use by SMC or which predates SMC) which costs the same per patient or less.
15.	The product is a generic, branded generic or hybrid medicine. NHS boards may make decisions about the continued application of 'not recommended' advice for the reference product when generic, branded generic or hybrid medicines become available.
16.	The product is a biosimilar medicine. NHS boards may make decisions about the continued application of 'not recommended' advice for the reference product when biosimilar medicines become available.
17.	In certain circumstances, where there are no patients in Scotland who are eligible for treatment a submission may not be required. Please consult the SMC Secretariat before assuming that this criterion is satisfied.
18.	For some products where there is a large number of proprietary medicines with the same active substance(s) and similar costs, SMC may occasionally advise ADTCs that these are outwith remit. ADTCs should make local decisions on these

	products as required. These products are testosterone products for testosterone replacement therapy in male hypogonadism (from June 2019), ursodeoxycholic acid products (from February 2016), colecalciferol products for vitamin D deficiency (from February 2015), some combined oral contraceptives (from April 2012), and some preparations of mesalazine (from January 2011). Please consult the SMC Secretariat for further advice on medicines in these categories.
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1.3 Horizon scanning

SMC has a horizon scanning function that aims to improve financial and service planning within NHS boards through the provision of early intelligence on new medicines in clinical development. Information on horizon scanning can be found on the SMC website. To ensure that company products are considered in NHS board financial planning cycles, companies are requested to ensure that they enter all relevant data on medicines in phase III clinical development or those that are 3 years from being made available to prescribe in the UK onto UK PharmaScan. This will allow SMC to extract most of the required horizon scanning data from this source.

2 SMC organisation

2.1 Overarching structure

Several groups with distinct roles contribute to SMC's process: the New Drugs Committee (NDC) provides technical assessment; the SMC Committee issues advice on new medicines; and the SMC Executive provides strategic and process oversight. The committees and SMC Executive are supported by the SMC Secretariat and professional staff groups. SMC Terms of Reference are published on the [SMC website](#).

2.2 SMC Executive and committees

The SMC Executive's role is to guide the strategic direction of SMC and provide oversight of process and policy issues. It consists of the chair, the vice chairs of the NDC and SMC Committee, the SMC Clinical Adviser and members of the SMC senior staff.

The SMC Committee is the body that makes the decision as to whether a medicine is accepted for use in NHSScotland, or not. It is made up of around 25 members. All 14 NHS boards are represented via nominations from ADTCs and there is a wide mixture of backgrounds to ensure that decisions are made from an appropriately wide perspective, not simply from the viewpoint of clinical evidence. Healthcare managers, academic health economists, public partners and the pharmaceutical industry are all members of SMC and take part in its decision making. SMC meetings are held publicly either online or as hybrid meetings.

The NDC has around 20 members, comprising clinicians, pharmacists, nurses and pharmaceutical industry members. It has an academic, technical remit and its role is to complete the technical assessment of a company's submission and make a recommendation to the SMC Committee. NDC meetings are not held in public.

Membership of the SMC Executive and committees is listed on the [SMC website](#).

2.3 Professional support staff

NDC and SMC committees are supported by the following:

- the SMC Secretariat manages administration and communication;
- the Patient and Public Involvement Team supports patient groups to contribute evidence and perspectives that help SMC members understand the impact of a new medicine on patients and carers;
- an assessment team and statisticians experienced in critical appraisal, health technology assessment (HTA) and health economic modelling. For each full submission or resubmission being assessed, the assessment team comprises a pharmacist, health economist, health services researcher and an NDC committee member (known as the lead assessor) who are allocated to review the company's submission.

Underpinning the whole organisation is the SMC Secretariat which provides full administrative and logistical support to the committees and assessment teams. The SMC Secretariat also liaises with the pharmaceutical industry and other external agencies. The point of contact for a pharmaceutical company should be the SMC Secretariat. The secretariat will be aware of possible upcoming submissions via the horizon scanning process and, as the expected date of marketing authorisation comes closer, will contact the company asking for confirmation of the submission date. At this time the company will be asked to advise the date they intend to make their submission and whether the product could be considered as an [end-of-life medicine](#), [orphan medicine](#), or [orphan equivalent medicine](#). The submission requirements are different for [ultra-orphan medicines](#), see Section 5.3 for more detail. The SMC Secretariat can give advice on submission requirements and timings of submissions. The submission should then be made, as per the [Guidance to submitting companies for completion of New Product Assessment Form \(NPAF\)](#), via the SMC Secretariat.

2.4 Timelines

SMC aims to issue timely advice, ideally as soon as possible after the medicine becomes available for prescribing in the UK. If a submission may be delayed, companies should inform the secretariat as soon as possible stating the reasons for delay and their intended submission timeline. While the SMC professional support staff are happy to answer questions about a company's submission at other points of the process, the staff cannot comment or give opinions on specific aspects of the evaluation of a submission while the assessment process is underway.

Both SMC and NDC meet monthly, with a gap of usually 6 or 10 weeks between a medicine being assessed at NDC and then SMC, depending on whether, for example, the medicine is eligible for the Patient and Clinician Engagement (PACE) process (see Sections 2.5.3 and 3.2). The usual overall assessment timeline is 18 weeks from scheduling of a submission to issuing advice. A longer timeline (usually 26 weeks) is required for submissions for: end-of-life/orphan medicines that require PACE meetings; medicines with a complex PAS; and, occasionally for complex submissions such as those which include multiple clinical studies or analyses. Resubmissions made within 3 months of the original SMC decision may proceed via a fast-track process if the only change is a new or improved PAS (see Section 5.4.2). This will allow the resubmission to proceed directly to the SMC committee with an overall assessment timeline of up to 14 weeks. In periods where a high number of submissions are received SMC may need to prioritise submissions to meet the needs of patients, prescribers and the healthcare system. Please refer to the SMC website for the criteria used to prioritise submissions. The work programme and outputs from SMC assessment are available in the public domain on the SMC website. Appendix 1 presents an overview of SMC process and timelines.

The SMC Secretariat contacts the submitting company to confirm the assessment timeline and scheduling to an NDC meeting. Once an SMC submission has confirmed dates for assessment, the submitting company cannot withdraw it from the process (unless for regulatory/ licensing reasons). Any requests to pause the assessment process by the submitting company will be considered only in exceptional circumstances and will be assessed on a case-by-case basis by the SMC Executive Team.

The assessment timeline consists of the following stages.

2.4.1 NDC assessment

The submission is allocated to and reviewed by an assessment team. Companies should be aware that, following submission and before the medicine is considered by the NDC, they will often be contacted by the SMC Secretariat by email for further information, clarification on information already submitted or additional analyses in relation to the health economics component of the submission. In some cases, the company will be expected to provide significant amounts of information within tight timescales. Assessment teams complete economic, clinical and—when relevant— indirect treatment comparison checklists, that, together with the company submission and clinical expert comments, go to the NDC.

The NDC reviews the evidence and makes a preliminary recommendation which is sent in confidence to the submitting company on the Friday following the meeting. The company is given an opportunity to comment on the preliminary advice in the Detailed Advice Document (DAD) with a 10-working day deadline for response – see Section 7.2. At this stage there may also be requests for clarification of information submitted or further analysis of information already submitted. Companies may submit a new or revised PAS at company comments stage.

2.4.2 Review by SMC Committee or SMC Executive

Positive NDC recommendations are considered by the SMC Executive and negative NDC recommendations are considered by the SMC Committee. The company response to the preliminary advice is included in the paperwork for review by SMC Executive or SMC Committee members, alongside the submission and preliminary NDC recommendation.

The SMC Committee considers additional information, such as patient group submissions and evidence from PACE meetings, and takes a broader view than the strictly evidence-based approach of the NDC.

2.4.3 Communication of SMC decisions

Following review of the submission by the SMC Committee, the submitting company will be informed in confidence of the SMC decision, and sent the DAD, on the Friday following the meeting. SMC does not anticipate notification of any further changes after the Company Comments deadline following NDC. Positive SMC decisions from the SMC Executive are shared at the SMC Committee meeting.

NHS boards are also informed of the advice, in confidence, at this stage. SMC decisions will not be made public, via the SMC website, until 4 weeks after the SMC meeting. In the intervening period, NHS boards are expected to prepare for the consequences of the decision.

If relevant, competitor companies are sent a copy of the advice on the Monday of the week following the SMC meeting, to enable them to review statements on their product in the DAD. See Section 8.2 on consultation with competitor companies.

2.5 Meeting administration

2.5.1 NDC

NDC usually meets on the last Tuesday of every month. NDC meets virtually and these are private meetings; members of the public and representatives of the submitting companies are not able to attend.

2.5.2 SMC Committee

The SMC Committee usually meets on the first Tuesday of every month. SMC meetings are open to the public and are held virtually using Zoom (with approximately three hybrid meetings per year). In addition to the SMC Committee members, members of staff involved in managing the meeting and supporting the chair will also be in attendance. Invited observers may also be present.

The submitting patient group partner representatives are invited to participate in SMC Committee meetings to respond to specific queries relating to patient and carer issues and provide points of clarity relating to their patient group submission. SMC Committee meetings are open for members of the public to register to attend. Children under the age of 16 years cannot attend committee meetings.

Submitting company participants are also invited to participate in SMC Committee meetings and will be sent an agenda. SMC Committee meetings will use PowerPoint slides during the presentations. Further detail on company participation in SMC meetings is given in Section 7.3.

Each committee member is issued with extensive supporting paperwork for each submission via the SMC secure site. This includes a detailed analysis of the clinical and cost effectiveness of the medicine, along with all patient group submission forms, patient group partner registration forms, and feedback from expert clinical advisors. It also contains information the submitting pharmaceutical company has identified as confidential, and therefore, this is only provided to SMC Committee members.

The presentation and discussion of each medicine usually takes around 30 minutes, although this is variable. The chair will call regular breaks to avoid meeting fatigue.

If any participants are unsure of any aspect of their role or contribution to the meeting, they are encouraged to let a member of the team know and they will provide assistance. Appropriate arrangements for any accessibility requirements will be made; participants are asked to let a member of the SMC team know in advance of the meeting.

When assessing each medicine, the SMC Committee aims to hold all of the discussion in public (Part 1). However, on occasion part of the discussions for a specific medicine may require a closed session (Part 2). This is mainly because SMC has a legal obligation to maintain the commercial confidentiality of any information identified as such by the submitting company. This is standard practice in all HTA organisations in the UK. The chair may declare, or be asked by a committee member to declare, a closed session, allowing detailed discussion of this confidential material.

After the discussion for all submissions has finished, the meeting moves to the closed session (Part 2) and each voting member of SMC casts their vote for each medicine electronically. The outcome is based on a simple majority, and the number of votes is confidential. The decision on each medicine is then made known to the committee but not announced publicly at that time.

2.5.3 Patient and Clinician Engagement (PACE)

Medicines for orphan/orphan equivalent and end-of-life conditions are eligible for a PACE meeting. If the preliminary NDC advice for these medicines is not recommended, and the NPAF included a request for a PACE meeting, then this is convened by SMC and the submitting company is asked to submit a PACE statement alongside comments on the NDC draft advice. PACE meetings are held virtually using

Microsoft Teams. This process usually extends the assessment timelines to 26 weeks. The benefit of PACE is that clinicians and patients have a stronger voice in the assessment of medicines for orphan/orphan equivalent and end-of-life conditions and SMC Committee decision making is more flexible.

Each PACE meeting is tailored to the medicine under consideration. The meeting is chaired by an NDC vice-chair, SMC vice-chair or someone else with specific experience of the SMC process and supported by SMC staff. An SMC public partner attends the PACE meeting to provide public scrutiny of the process. Representatives are sought from patient groups and clinicians from the relevant specialty (identified by Managed Clinical Networks and regional Cancer Networks). The company can also submit a brief statement for consideration by the PACE group.

The paperwork provided in relation to the PACE meeting and any information discussed at the meeting is confidential and attendees are made aware of the importance of not breaching confidentiality. Additionally, the completed PACE statement is treated in confidence until presented at the SMC Committee meeting.

A PACE template is completed during the meeting and the content agreed by participants. The resulting PACE statement is included in the SMC meeting papers for the medicine alongside the NDC DAD, company comments, collated clinical expert responses, patient group submission(s) and any new or revised PAS. The output from the PACE meeting is a major factor in the SMC decision.

2.5.4 Declarations of interest

All meeting participants are required to adhere to the Healthcare Improvement Scotland Evidence Directorate policy on declarations of interest which can be found on the Healthcare Improvement Scotland website.

Committee members are required to make a full declaration of interest on appointment and annually thereafter, in accordance with the policy. Members are also required to declare relevant interests at meetings, regardless of whether those interests have previously been declared to SMC. Members who declare a personal specific interest must take no part in discussions for that agenda item and are not eligible to vote. Members who declare a personal non-specific interest must take no part in discussions for that agenda item, except at the chair's discretion to answer questions from other members and will not be permitted to vote.

PACE participants declare their interest, and this is included in the SMC paperwork. Having a conflict of interest does not preclude clinicians or patient group representatives from participating in a PACE meeting.

3 SMC decision making

In reaching its decision on a medicine SMC can exercise flexibility in some specific circumstances. These relate to when SMC decision modifiers are applicable and/or where a PACE meeting has taken place. These are outlined below.

3.1 Modifiers

In assessing the relative clinical and cost effectiveness of new medicines, the SMC requires a robust clinical and economic case to be made and for the submitting company to demonstrate the medicine's value for money. In some specific situations SMC may exercise greater flexibility in its decision making to allow consideration of additional factors. These additional factors, known as modifiers, may allow SMC to accept either more uncertainty in the health economic case or a higher cost per quality-adjusted life year (QALY).

The additional factors include (but are not limited to) the following:

3.1.1 Where more uncertainty in the economic case may be accepted

Orphan medicines

The SMC has adopted the definition of orphan medicines provided by the MHRA – the prevalence of the condition in the UK must not be more than 5 in 10,000 which equates to <2,500 per 5 million. This applies to the full licensed indication. SMC can apply this modifier to any UK designated orphan or a medicine to treat an equivalent size of population.

SMC requires that all submissions are comprehensive and that all sections of the NPAF are completed. This is required for orphan medicines, for which a meaningful attempt needs to be made to present robust clinical and economic data. SMC recognises that orphan medicines may have a smaller clinical trials programme and, therefore, less information may be available for some sections (for example, on efficacy and safety). Additional detail should be provided where required, for example to justify the relevance of surrogate markers and the theoretical basis for their selection, which should then be related to quality-of-life data.

Recognising the limited data on efficacy for orphan medicine submissions, SMC will accept a greater level of uncertainty in the economic case. Additional factors, such as whether the medicine: treats a life-threatening disease; substantially increases life expectancy and/or quality of life; can reverse, rather than stabilise, the condition; or bridges a gap to a 'definitive' therapy, will also be considered in assessing both the level of uncertainty and cost per QALY which is acceptable.

3.1.2 Where a higher cost per QALY may be accepted

SMC does not have a formal threshold cost per QALY below which cost effectiveness would be considered shown and above which cost effectiveness would be considered not to have been demonstrated. The cost per QALY is only part of a wider judgment of the value of a new medicine.

Where the cost per QALY is relatively high, other factors also play a role in SMC's assessment and may modify the final decision. These modifiers include (but are not limited to):

- evidence of a substantial improvement in life expectancy (with sufficient quality of life to make the extra survival desirable). Substantial improvement in life expectancy would normally be a median gain of 3 months, but SMC assesses the particular clinical context in reaching its decision;
- evidence of a substantial improvement in quality of life (with or without survival benefit);
- evidence that a subgroup of patients may derive specific or extra benefit and that the medicine in question can, in practice, be targeted at this subgroup;
- absence of other therapeutic options of proven benefit for the disease in question and provided by the NHS;
- possible bridging to another definitive therapy (for example bone marrow transplantation or curative surgery) in a defined proportion of patients; and
- emergence of a licensed medicine as an alternative to an unlicensed product that is established in clinical practice in NHSScotland as the only therapeutic option for a specific indication. Some possible examples include caffeine injection for the treatment of apnoea of prematurity and betaine anhydrous for the adjunctive treatment of homocystinuria.

SMC also looks at any other special issues which may have been highlighted by the manufacturer of the medicine, by clinical experts and/or by patient groups. These special issues are usually very specific to the medicine or disease under consideration and are thus not readily categorised.

The modifiers are only applied for a relatively high cost per QALY when the committee is satisfied that the clinical and economic case for the medicine is robust.

3.2 Patient and Clinician Engagement (PACE) meetings

For medicines used to treat end-of-life and/or orphan conditions, the SMC offers the submitting company the opportunity to request a PACE meeting if the preliminary NDC advice is 'not recommended'. The output from the PACE meeting, which includes a wider perspective from clinician and patient groups who are experts in the relevant disease area, is an important factor in the SMC decision making.

The aim of the PACE meeting is to describe the added benefits of the medicine, from patient, carer/family and clinician perspectives, that may not be fully captured within the conventional clinical and economic assessment process.

These may include, but are not limited to:

- Added value of the medicine for the patient

For example: impact on quality of life such as the ability to work or continue in education/function with any associated financial impact, symptoms such as fatigue, pain, psychological distress, also factors such as convenience of the treatment, whether it allows self-care or the ability to maintain independence and dignity.

- Added value of the medicine for the patient's family/carers

For example: time for accompanied visits for treatment, requirement for assisting the patient with personal care and support, impact on family life, and impact on the carer's ability to work and any associated financial impact.

- Clinical issues

For example: unmet need, severity of the condition, place in the treatment pathway, service/infrastructure changes/benefits because of using the medicine.

4 Patient Access Schemes (PAS)

PAS are schemes proposed by pharmaceutical companies to improve the cost effectiveness of medicines, thereby facilitating patient access. PAS will be considered by NHSScotland to facilitate access to medicines that are not, or might not be, in the first instance found to be cost effective by SMC.

PAS are examined by the Patient Access Scheme Assessment Group (PASAG). PASAG is a separate body from the SMC which undertakes an objective and independent assessment of PAS submitted by companies on a national basis. SMC will only consider the financial benefits of a proposed PAS if the scheme has been accepted for use in Scotland by PASAG.

Companies should submit any PAS at the same time as they are making their submission to SMC. Both submissions must be made via the SMC Secretariat. Submitting companies also have a second opportunity to submit a new or revised PAS to the SMC Secretariat within a 2-week period following the issue of preliminary NDC advice. A new application pack should be completed when revising the previous PAS application. It is important to note that a new or revised submission at this stage may extend timelines for the SMC review of the medicine. For this reason, pharmaceutical companies are strongly encouraged to submit any proposed PAS at the first opportunity with the initial SMC submission.

The submission of a PAS is a condition for ultra-orphan medicines.

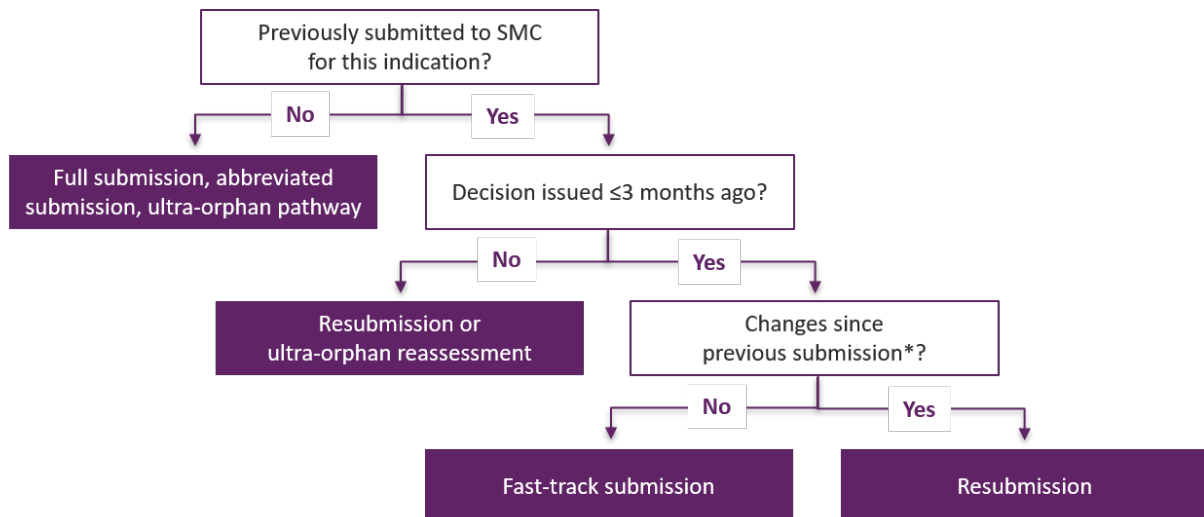
Full guidance covering submission of a PAS and links to the most up to date PAS application forms to be used with submissions is available on the SMC website. Companies should refer to the Guidance to submitting companies for completion of the New Product Assessment Form (NPAF) for information on how a PAS should be incorporated into the economic analysis and budget impact template.

5 Assessment processes

5.1 Introduction

SMC has several submission routes and assessment processes which are outlined in Figure 1. Submitting companies should contact the SMC Secretariat about their submission plans in advance (see Section 2.3 for further information).

Figure 1: Which SMC submission route applies?



*Excluding a new/revised PAS or change to list price

5.2 Full submissions

SMC will issue advice on all new medicines and all new indications for established products that are within remit (see Section 1.2) through the full submission process, unless suitable for the abbreviated process (see Section 5.5.1). SMC can make one of three decisions on a medicine:

- accepted
- accepted with a restriction(s) (for example, the medicine can only be recommended in a particular group of patients with the condition. This typically occurs because the company has requested this explicitly in their submission)
- not recommended.

The committee also has the opportunity to accept some medicines on an interim basis subject to ongoing evaluation and reassessment (see Section 5.2.3).

SMC issues advice on new medicines to NHS boards based on an assessment of the clinical and cost effectiveness within the NHS in Scotland. It is therefore important that companies submit clinical and economic evidence according to the principles and standards outlined in the guidance documents.

The SMC assessment of the clinical and cost effectiveness of a medicine is based on the evidence in the company's submission. In their submission, the company needs to demonstrate the case for the clinical and cost effectiveness of the medicine through a clear, concise, unbiased and robust case to support the application. Each NDC/SMC meeting involves up to six submissions, therefore it is imperative that companies provide a concise case. Robustness will be judged on the basis of the methodological quality of the case submitted. The application needs to show that the medicine will:

- (i) provide additional health benefits that are valued by patients compared with current Scottish practice and that this is at a net cost to the NHS that offers acceptable value in relation to other uses of the same resources;
- or
- (ii) offer equivalent levels of health benefit to patients at an equivalent or lower net cost to the NHS.

5.2.1 Positioning within licence

SMC has a remit to consider the full indication covered by the marketing authorisation for a new medicine. In some circumstances, however, the company may wish to make a case (selective submission) for the clinical and cost effectiveness of the medicine for a narrower population than that covered by the full licensed indication(s), for example, where the company wishes to make the case for the medicine when positioned for use in a specific patient subgroup to maximise its cost effectiveness.

- (i) Where a submission covers only part of the marketing authorisation for a product the submitting company must detail all other aspects of the marketing authorisation that are within SMC remit but have not been covered in the submission.
- (ii) Where a submission positions a medicine for use in a subgroup of patients narrower than that covered by the marketing authorisation, the submitting company should ensure that the proposed population for treatment is appropriate and valid within the licensed indication under consideration in the submission.

The company must state explicitly on the registration page under 1b) of the NPAF that SMC is asked to consider the use of the medicine when positioned for use in a subgroup of the population covered by the marketing authorisation.

5.2.2 Submissions for medicines for end-of-life or orphan conditions

For medicines used to treat end-of-life and/or orphan conditions, SMC offers the submitting company the opportunity to request a PACE meeting if the preliminary NDC advice is 'not recommended'. For SMC to assess eligibility for a PACE meeting, this must be indicated in Section 1 of the NPAF and supporting evidence and rationale for end-of-life and orphan status must be included in Section 2b. Refer to Sections 2.5.3 and 3.2 of this guide for further information. The definitions of [orphan/orphan equivalent medicines](#) are based on the full population of the licensed indication irrespective of whether or not the company wishes SMC to consider the product when positioned for use in a subpopulation of the licensed indication. The definition of an end-of-life medicine may be based on a subpopulation of the licensed indication where the company provides adequate justification.

SMC will consider the submitting company's case and confirm eligibility for consideration as an end-of-life or orphan/orphan equivalent medicine. All supporting data should be fully referenced in the NPAF. The submitting company may be asked to provide further justification. If the proposed end-of-life and/or orphan/orphan equivalent status is not accepted after a validation process, the submitting company may appeal the validation decision. This should be done within 2 weeks of being notified of the validation decision by submitting a short statement explaining the basis of the disagreement with the validation decision. No new data can be submitted as part of the appeal.

5.2.3 Interim acceptance decision option

5.2.3.1 Overview

SMC has an interim accepted decision option for medicines meeting certain eligibility criteria relating to regulatory pathways. The MHRA supports the development of medicines that address unmet medical needs of patients. In the interest of public health, certain medicines are eligible for different regulatory pathways ([UK conditional marketing authorisation](#), [Innovative Licensing and Access Pathway \(ILAP\)](#), [Early Access to Medicines Scheme \[EAMS\]](#)) depending on the seriousness of the condition and/or lack of currently available treatment options.

All medicines with UK conditional marketing authorisation are eligible for this decision option. SMC may issue interim accepted/accepted restricted advice if the committee considers that the additional efficacy and/or safety data requirements outlined in the MHRA specific obligations are expected to address the key uncertainties in the evidence presented by the submitting company.

The interim accepted decision option is also available for medicines included in the ILAP and/or which have received a positive MHRA EAMS scientific opinion if it is likely that ongoing clinical studies could address key uncertainties in the evidence.

The company will be required to provide an updated submission to SMC when the conditional marketing authorisation is converted to a standard marketing authorisation, typically after 2 to 3 years. For medicines included in EAMS and ILAP, SMC will liaise with the company to agree on further regular points of contact about the clinical evidence, to ensure a practical and mutually acceptable date can be set with the company for submission for reassessment.

5.2.3.2 Process

Submitting companies should follow the standard submission process. The company should use the current NPAF available from the 'Making a submission' section on the SMC website, which requests details of ongoing studies that will potentially address the key uncertainties, where relevant. For each study, companies are asked to provide a brief description of:

- the study design, including details of blinding and randomisation;
- the main inclusion criteria, that define the patient population included in the study;
- the primary and/or other relevant outcome(s) measured in the study and likely timescale for reporting of these.

SMC recognises the challenges in providing robust clinical and economic evaluations for medicines where there are uncertainties in the evidence base due to immature clinical data, however, a full clinical and economic evaluation to demonstrate the value for money should be submitted.

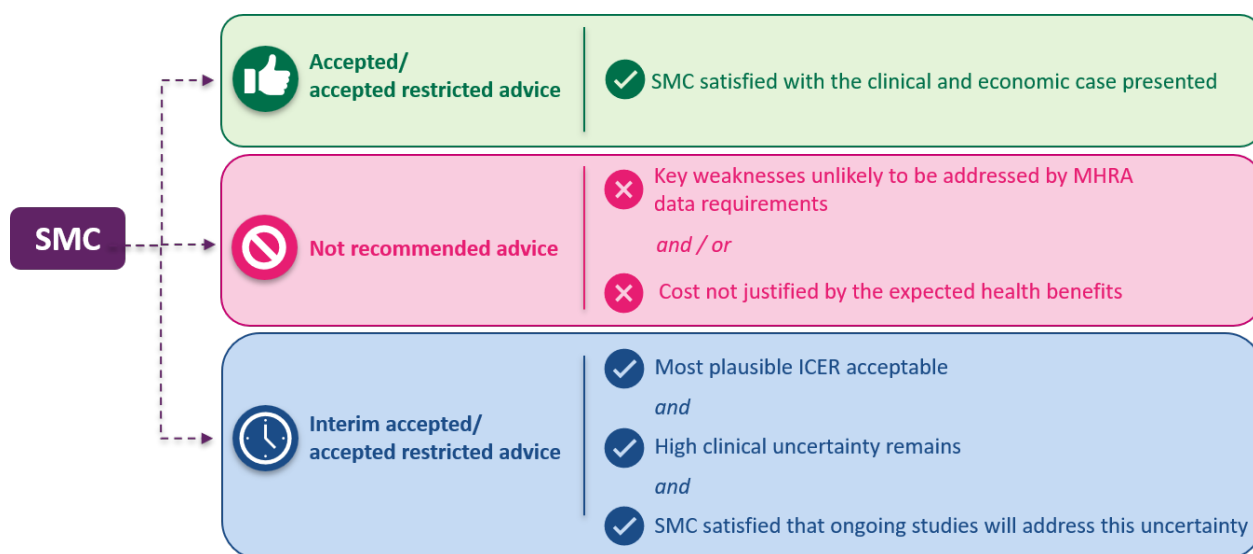
The NDC will review the ongoing studies presented by the submitting company. In its preliminary advice to SMC (the NDC DAD), NDC will advise SMC if the additional data could be expected to address the key uncertainties, for example results of an ongoing comparative study if uncontrolled data have been presented as the key evidence. If a key weakness of the evidence is that the plausible incremental cost-effectiveness ratio (ICER) is higher than the levels generally accepted by SMC, then the additional efficacy and/or safety data are unlikely to address this issue.

The submitting company will have the opportunity to comment on the NDC DAD and may, if appropriate, provide further clarity to SMC on how the ongoing studies could support the key areas of uncertainty.

If the NDC preliminary advice is 'not recommended', the submitting company can also submit a new or revised PAS and, if the medicine has been validated as meeting orphan/orphan equivalent and/or end-of-life criteria, request a PACE meeting.

As part of its review and decision-making process, SMC will assess the information provided by the company in addition to other sources of evidence including patient group submissions, clinical expert comments and, where relevant, the output from the PACE meeting, and the Cancer Medicines Outcome Programme-Public Health Scotland (CMOP-PHS) data where available. The SMC decision options at initial assessment are outlined in Figure 2.

Figure 2 SMC decision options at initial assessment



As SMC decisions are made by majority vote, a two-stage approach to voting for medicines eligible for an interim accepted decision will be adopted. The chair will ask members to vote in the usual way. If the first decision is to not recommend the medicine, then a second vote to consider interim acceptance will take place.

Vote 1: If the majority vote is to accept the medicine then a second vote will not be required. The medicine is then accepted for use in line with the indication or restricted population as proposed by the company.

If the majority vote is to not recommend the medicine then a second vote will be required.

Vote 2: Members will vote to accept the medicine on an interim basis or to not recommend the medicine for the indication or restricted population as proposed by the company.

Medicines with interim accepted advice from SMC will be considered for local formulary inclusion, in line with current practice for other medicines accepted by SMC for use in Scotland.

5.2.3.3 *Reassessment of the medicine*

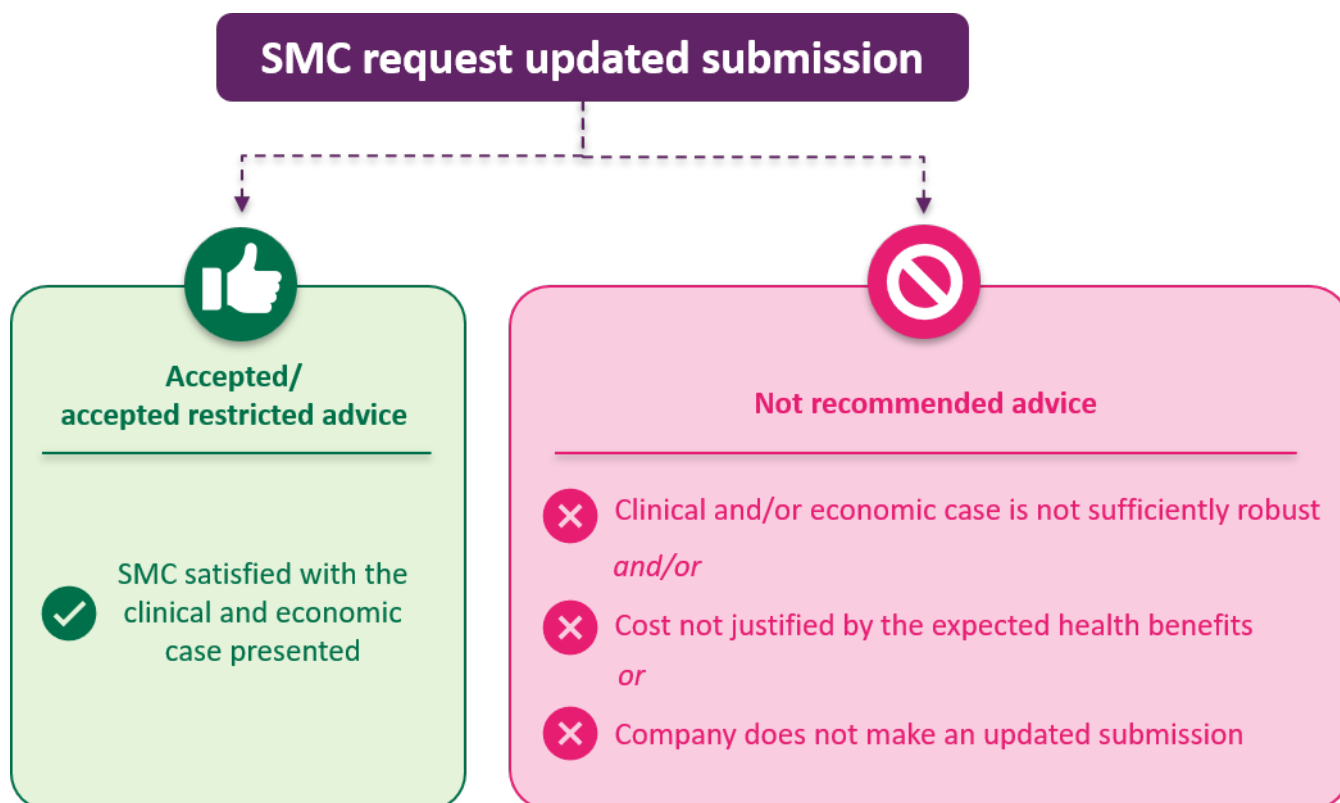
If SMC issues interim accepted advice the company will be required to provide an updated submission in agreement with SMC

- when the conditional marketing authorisation is converted to standard marketing authorisation (if relevant)
- for medicines that do not have a conditional marketing authorisation, SMC will liaise with the company to agree on further regular points of contact about the clinical evidence, to ensure a practical and mutually acceptable date can be set.

The updated submission must be provided in line with SMC guidance and within the context of the current treatment pathway in Scotland at the point of reassessment. The submitting company may also provide relevant data in addition to those from ongoing clinical studies to support their clinical and economic case such as observational or real-world data. The submitting company must provide a new PAS application with their updated submission in line with PAS guidance at the point of reassessment. If there are few changes to the original submission then it would be helpful to highlight new information in the NPAF.

The SMC decision options at reassessment are outlined in Figure 3.

Figure 3 SMC decision options at reassessment



The SMC advice following reassessment supersedes the interim accepted advice.

If the medicine is accepted for use at the point of reassessment the updated PAS will come into effect.

In the event that the medicine is not recommended for use at reassessment or due to non- submission, the previously established PAS would continue to be in effect for the minimum period specified in said PAS agreement. For patients already taking a medicine that was previously accepted on an interim basis and who continue to derive clinical benefit, it is expected that the patient would remain on the medicine until the patient and clinician consider it appropriate to stop treatment. Where SMC advises that a medicine is not recommended for use in NHSScotland but a clinician thinks it may be of benefit for a particular patient then the clinician should follow the relevant local NHS board procedures to seek access.

If the company does not provide an updated submission for a medicine accepted on an interim basis when requested, SMC will issue 'not recommended' advice. This will replace the previous interim accepted advice.

Medicines that are accepted for use on an interim basis can be considered as relevant comparators on a case-by-case basis but would need to be supported by appropriate data. In these circumstances, SMC may also require a comparison against an alternative medicine accepted by SMC or against a placebo/ best supportive care option.

5.3 Ultra-orphan medicines

5.3.1.1 Overview

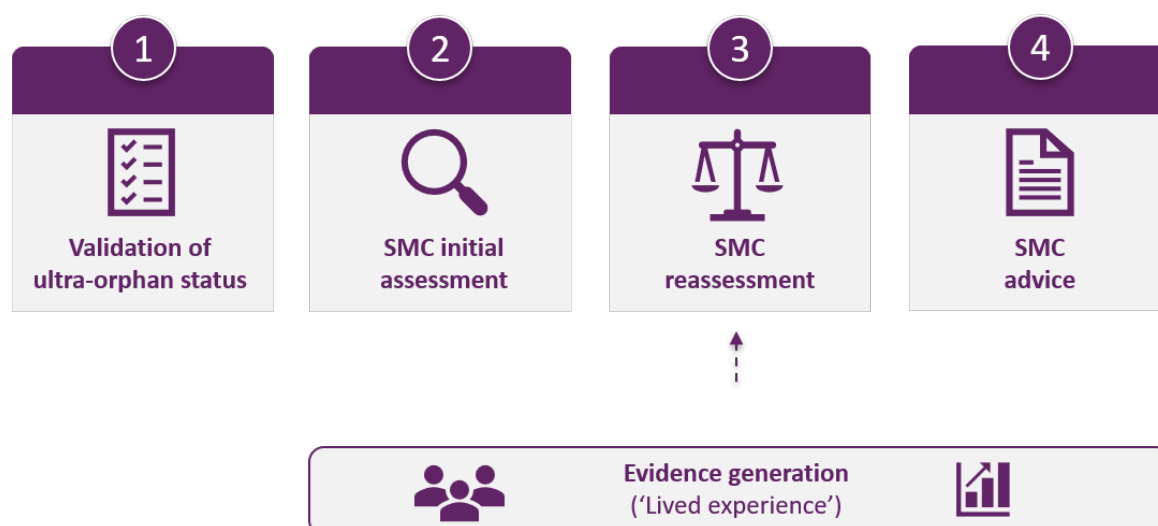
The Scottish Government published a [Review of Access to New Medicines](#) in December 2016. This recommended a new definition and pathway for medicines to treat for [ultra-orphan](#) conditions in NHSScotland.

A submission for a medicine that is validated as ultra-orphan according to the definition will be assessed by SMC and can then be prescribed within the ultra-orphan pathway while further evidence on its effectiveness is generated. The company should then provide an updated submission for reassessment allowing SMC to make a decision on routine use of the medicine in NHSScotland. If the company does not provide an updated submission for reassessment following the period of data collection, SMC will issue 'not recommended' advice.

The ultra-orphan pathway involves the following key stages:

- validation of ultra-orphan status
- SMC initial assessment
- medicine can be prescribed within the ultra-orphan pathway during evidence generation period
- SMC reassessment followed by issue of advice to NHSScotland.

Figure 4: Ultra-orphan pathway



5.3.1.2 Validation

Companies are encouraged to seek confirmation that a medicine meets the ultra-orphan definition at an early stage **before making a submission** by completing the ultra-orphan proforma available on the 'Making a submission' section of the SMC website. If preferred, a company can choose to submit under the orphan assessment process, with the opportunity for a PACE meeting.

The SMC Executive will review the proforma and will confirm whether the medicine has been validated as ultra-orphan within around 8 weeks. NHS boards are informed in confidence when a medicine is validated as meeting the ultra-orphan criteria. If the company submits the ultra-orphan proforma to SMC for validation in advance of the MHRA granting a UK orphan marketing authorisation, then the relevant European Medicines Agency (EMA) orphan designation can be referenced. The final decision will be contingent on the MHRA granting a UK orphan marketing authorisation.

If the company has new evidence to address evidence gaps identified in the original ultra-orphan validation, there is the opportunity to resubmit an updated proforma for review.

If the company disagrees with the outcome of the validation, there is the opportunity to appeal. The company should provide a short statement to explain the basis of the disagreement within 3 months of being notified of the validation outcome.

The appeal panel will consist of three members of the SMC Executive who did not participate in the initial validation, including the SMC Chair. The panel will review the proforma initially provided by the company to support ultra-orphan status, together with responses from clinical experts. No new data can be submitted as part of the appeal. Conflicts of interest will be handled in line with standard SMC arrangements.

The appeal panel will reach a consensus view on whether the medicine meets the ultra-orphan criteria. The company and NHS boards will be advised of the conclusion.

In addition to SMC ultra-orphan status, Scottish Government has outlined three further conditions which must be met to enable a medicine to enter the pathway. The company is required to:

- make a full submission to SMC to allow an initial assessment of clinical and cost effectiveness
- offer a PAS that complies with the standard terms and conditions considered acceptable by PASAG
- support data collection arrangements that meet evidence generation requirements for assessment under the ultra-orphan pathway.

Confirmation of ultra-orphan status is required before the company makes a submission for SMC initial assessment (using the New Product Assessment Form for ultra-orphan medicines).

Ultra-orphan validation decisions will expire after 2 years. Thereafter if a product has UK marketing authorisation from the MHRA and is launched in the UK; if there are eligible patients in NHSScotland; and if no submission is forthcoming, SMC will move to issue 'not recommended' advice.

Following validation, the company can request an early engagement meeting with SMC to discuss the challenges within their submission, the uncertainties that they foresee and their proposed submission timelines. Further details on early engagement with companies are given in Section 7.1.

5.3.1.3 SMC initial assessment of ultra-orphan medicines

An initial assessment of the clinical and cost effectiveness of the medicine will be conducted. This will highlight uncertainties within the available evidence base and will help to inform the data collection stage of the ultra-orphan pathway.

SMC will use a broad framework to assess ultra-orphan medicines which takes account of the following:

- nature of the condition
- impact of the medicine
- value for money
- impact of the technology beyond direct health benefits and on specialist services
- costs to the NHS and Personal Social Services.

Companies should complete the NPAF for ultra-orphan medicines available in the 'Making a submission' section on the SMC website. This is structured to mirror the framework described above. Supplementary guidance is available for the ultra-orphan NPAF on the additional information requirements for this group of medicines and should be used in conjunction with the general Guidance to submitting companies for completion of NPAF. These documents are available on the SMC website.

At the initial assessment, SMC will appraise the evidence presented prior to the medicine being made available for an initial period as part of the ultra-orphan pathway. The clinical and economic evidence to support the initial assessment of an ultra-orphan medicine will be assessed by NDC. An economic evaluation on the value for money of the medicine remains a requirement, but with the flexibility in approach described in the guidance for the ultra-orphan NPAF. An ultra-orphan medicine assessment report will be structured according to the ultra-orphan framework. The ultra-orphan medicine assessment report will provide a critique of the evidence base presented, including key limitations and

uncertainties in the clinical and economic evidence to help inform data collection during the period of initial availability of the medicine. As part of the review process, other sources of evidence will also be invited, for example from clinical experts and patient group submissions. This will ensure as complete a picture as possible of the condition and the potential impact of the medicine. A PACE meeting will not take place at the time of the initial assessment but will play an important role in decision making at reassessment.

Following NDC the company is able to submit a new or revised PAS aimed at improving the cost effectiveness of the medicine. Refer to the Guidance to submitting companies for completion of New Product Assessment Form (NPAF) for details of updated analyses required within the Company Comments document.

No decision on routine availability in NHSScotland is made following the initial assessment by NDC. Following review of the medicine by the NDC, the draft ultra-orphan medicine assessment report will be shared with the submitting company and company comments invited. The final ultra-orphan medicines assessment report will be shared with SMC Executive and SMC Committee will be advised. There will be no requirement for SMC Committee discussion at this stage of the process.

SMC will publish the ultra-orphan medicine assessment report on its website within standard timelines.

5.3.1.4 Evidence generation

Following the initial assessment and publication of the report, pharmaceutical companies should develop a data collection plan detailing how relevant clinical and patient reported outcomes will be captured. The data collection methodology should aim to generate data that addresses (as much as possible) the uncertainties identified within the existing evidence base. This should increase the SMC's confidence in the clinical and cost effectiveness of the medicine at the time of reassessment.

In particular, pharmaceutical companies should:

- undertake to collect data on the ultra-orphan medicine for the duration of its availability through the pathway;
- ensure that the focus of the data collection activity attempts to address the uncertainties within the existing evidence base, including those highlighted in the initial SMC assessment report;
- draw on any existing data collection activities from other parts of the UK and beyond as part of the overall package of evidence generation;
- seek to collect Patient Reported Outcome Measures (PROMs), where appropriate, and data on the impact beyond direct health benefits and on specialist services; and
- cover all the costs associated with the data collection.

Whilst it is recognised that evidence generation activities and the data collection plan is unlikely to be fully developed until after the SMC's initial assessment report is issued, there will be aspects that can be established in advance. Companies are therefore encouraged to plan the data collection work as soon as possible and ideally following a positive validation from the SMC.

Companies will be responsible for determining whether the data being produced during the evidence generation period are adequately measuring and achieving the outcomes identified in their data generation and collection plan, and whether any additional action is required.

5.3.1.5 SMC reassessment of ultra-orphan medicines

The company will be required to provide a full updated submission within 3 months following the period of evidence generation. Companies should complete the NPAF for ultra-orphan medicines available in the 'Making a submission' section on the SMC website as outlined above. Results of further controlled studies, observational, registry or other real-world evidence gathered during initial availability of the medicine within the ultra-orphan pathway should be included in relevant sections of the NPAF to address uncertainties identified at the time of the initial assessment. The submission for reassessment must be provided in line with standard Guidance to submitting companies for completion of New Product Assessment Form (NPAF), including the relevant comparator(s) and within the context of the current treatment pathway in NHSScotland at the time of reassessment.

The clinical and economic evidence to support an ultra-orphan medicine will be assessed by NDC before consideration by the SMC Committee. The draft NDC DAD will be structured according to the ultra-orphan decision-making framework.

Following review of the medicine by the NDC, the draft NDC DAD will be shared with the submitting company and company comments invited before consideration by the SMC Committee.

If the preliminary NDC advice is not recommended, the submitting company will be offered the opportunity submit a new or revised PAS and a PACE meeting will be convened. Refer to Guidance to submitting companies for completion of New Product Assessment Form (NPAF) for further information.

SMC will review the information presented in the decision-making framework, examining the nature of the condition, impact of the medicine, value for money, costs to the NHS, and impact beyond direct health benefits using the criteria set out above. SMC will also consider the additional evidence generated during the period of initial availability of the medicine within the ultra-orphan pathway.

As part of its review, SMC will assess the information the company has provided within the ultra-orphan NPAF as well as other sources of evidence to supplement the framework within the NDC DAD, for example from clinical experts, Patient Group submissions and the output from a PACE meeting. This will ensure SMC is provided with as complete a picture as possible of the relevant aspects upon which the final decision will be based.

The SMC decision options at reassessment include:

- accepted for use
- accepted for restricted use
- not recommended.

For patients already taking a medicine that was previously available on the ultra-orphan pathway but not recommended on reassessment and who continue to derive clinical benefit, it is expected that the

patient would remain on the medicine until the patient and clinician consider it appropriate to stop treatment.

5.3.1.6 Patient Access Schemes (PAS)

Provision of a PAS to improve cost effectiveness of the medicine is a condition of the ultra-orphan pathway specified by Scottish Government. The company will offer the PAS at the time of initial SMC submission.

At the time of reassessment the submitting company must provide a new PAS application in line with PAS guidance (refer to NHSScotland PAS guidance available on the 'Making a submission' *section* of the SMC website). The company has the opportunity to propose a new or revised PAS post-NDC. If the medicine is accepted for use at the time of reassessment the updated PAS will come into effect.

In the event that the medicine is not recommended for use at reassessment, or due to non-submission, the previously established PAS at initial assessment would continue to be in effect until such time as the PAS Agreement was terminated or replaced.

5.4 Resubmissions

If 'not recommended' advice has been issued for a medicine, and the company wishes to consider a resubmission, they can request, via the SMC Secretariat, a meeting with SMC, with the aim of better understanding why their product was not recommended, thus allowing an appropriate focus for the resubmission (see Section 7.4).

A resubmission requires new clinical evidence or a new analysis of existing information about the medicine. In most cases the resubmission will follow the same process and timelines as all other submissions. If, however, the only change is a new or improved simple PAS or a list price reduction that has been confirmed with the Department of Health, the company may resubmit using a fast-track process (within 3 months) which allows the submission to proceed directly to SMC within a shorter assessment timeline (see Section 5.4.2).

5.4.1 Resubmissions for PACE eligible medicines

If the SMC committee is unable to accept a PACE eligible medicine for use in NHSScotland despite the added flexibility of PACE, then the company has the option of resubmitting to SMC. If the company resubmits within 6 months of the PACE meeting being held then the original PACE statement will be used for the resubmission. If the resubmission is received more than 6 months later then SMC staff will contact the original participants to establish if the previous PACE statement is still valid in terms of capturing the potential added value or if an updated PACE statement or a further PACE meeting is required.

5.4.2 Fast-track resubmissions

If a company wishes to make a resubmission (within 3 months) and the only change is a new or improved simple PAS or confirmed list price reduction, the company may submit using the fast-track process. This will allow a resubmission to proceed directly to the SMC committee with an overall assessment timeline of (usually) up to 14 weeks; there is no consideration by the NDC.

To be considered for the fast-track resubmission process, **all** criteria listed should be met.

- The only change to the original submission is a new or improved simple PAS or a list price reduction that has been confirmed with the Department of Health.
- The resubmission must be received within 3 months of the date the original SMC decision was issued to the company.
- Any changes to the list price of the medicine under review and/or to comparator medicines are reflected in the revised documents submitted.
- There is no change to any other aspect of the submission (including the proposed positioning of the medicine).
- There has been no previous fast-track resubmission for the medicine under review.

Resubmissions that do not meet these criteria may be considered through the standard SMC process.

Companies that wish to make a fast-track resubmission should provide the following documents to the SMC Secretariat.

- a) A completed fast-track resubmission proforma to confirm that the resubmission meets the criteria for the fast-track resubmission process. The fast-track resubmission proforma is available on the 'Making a submission' section of the SMC website.
- b) A new or improved concise PAS application or confirmation of Department of Health list price change.
- c) Updated cost-effectiveness results as follows:
 - (i) Submissions where comparators (and/or medicines used in combination with the medicine under review) are not associated with a PAS
 - A summary table showing updated cost-effectiveness results (base case and all sensitivity analyses presented in the SMC final advice) using the new/improved PAS price or list price reduction.
 - An appendix showing updated cost-effectiveness results using the new/improved PAS price or list price reduction corresponding to all sensitivity analyses presented in the original NPAF plus any additional scenario/sensitivity analysis requested during the original submission process.
 - (ii) Submissions where the comparator(s) (and/or medicines used in combination with the medicine under review) are associated with a confidential PAS

For each comparator with a PAS

- Companies should refer to SMC's standard process for dealing with comparator PAS as described in Section 6.10 of the Guidance to submitting companies for completion of New Product Assessment Form (NPAF) which can be found in the 'Making a submission/Companies/Patient Access Schemes' section of the SMC website.
- d) A budget impact template to reflect the new/improved PAS or list price reduction.
- e) A Word document showing the summary table from the budget impact template for the new/improved PAS/list price reduction scenario.

The SMC Committee will be provided with the original SMC paperwork, the company's fast-track proforma together with updated economic data, an updated DAD, and a 'Results for SMC decision-making document' (if a comparator medicine is also available under a confidential PAS). Presentation of the resubmission at the SMC meeting will recap on the clinical data and the rationale for the original decision and then focus on the impact of the change in PAS or list price reduction on the cost-effectiveness results.

5.4.3 Independent review panel

When a company disagrees with the SMC decision and there are no new data or analyses, the submitting company may request that SMC convenes an Independent Review Panel (IRP) to reassess the data, analysis and interpretation of the original submission. An IRP may be requested following completion of a full SMC assessment, up to 12 months after publication (provided a resubmission is not underway). If a medicine has been considered by SMC more than once (a submission and resubmission) then the IRP timescale will refer to the most recent submission.

The submitting company must provide a summary statement to justify and provide clarity on why an IRP is sought, to include confirmation that no new evidence is available and there is no scope for reanalysis of the existing evidence.

5.5 Abbreviated submissions

5.5.1 Overview

An abbreviated submission may be made for some new medicines when a full submission is not required due to a low net budget impact. Guidance should be sought from the SMC Secretariat by completing the Company Information Request Form, available on the 'Making a submission' section of the SMC website.

The abbreviated process requires companies to complete and submit an Abbreviated Submission Form. The Abbreviated Submission Form template is available on the 'Making a submission' section of the SMC website. In order that SMC can advise on new products as close as possible to the product being available for use in NHSScotland, Abbreviated Submission Forms should be sent to the SMC Secretariat ideally around the time that the product receives marketing authorisation from the MHRA.

Key considerations for deciding whether an abbreviated submission may be appropriate are:

- The medicine's likely impact on budgets and resource allocation across NHSScotland. If a medicine could potentially have a significant net budget impact, a full submission should be made. Guidance can be sought from the SMC Secretariat, by completing the Company Information Request Form.
- That similar clinical effectiveness can be demonstrated briefly, in simple terms.

Companies must provide the basis for the submission and justification for applying via the abbreviated route (see below). Guidance should be sought from the SMC Secretariat as outlined above in advance of making a submission.

Medicine marketing authorisation for:	Type of submission required:
1. New active substance – even if the number of potential patients is small and the expected net budget impact is low	Full
2. Additional indication/licence extension approved by the MHRA/EMA – even if the number of potential patients is small and the expected net budget impact is low	Full
3. New medicine where alternatives within the same therapeutic class have previously been accepted for use (or restricted use) by SMC and the new medicine costs the same or less or has a limited net budget impact	Please complete the Company Information Request Form to obtain advice from SMC on submission requirements once final details of product pricing are available. See section below on abbreviated submission process (therapeutic class).
4. New formulation (for example, slow-release presentation, liquid) at more than pro rata cost relative to the existing formulation, which has	Please complete the Company Information Request Form to obtain advice from SMC on submission

Medicine marketing authorisation for:	Type of submission required:
been accepted for use by SMC or predates SMC establishment (31 January 2002) for the same indication	requirements once final details of product pricing are available.
5. Fixed-dose combination product of established medicines in a single pharmaceutical form at more than pro rata cost relative to the existing medicines, which have been accepted for use by SMC or predate SMC establishment (31 January 2002) for the same indication	Please complete the Company Information Request Form to obtain advice from SMC on submission requirements once final details of product pricing are available.
6. Licensed medicine of an established unlicensed preparation	Please complete the Company Information Request Form to obtain advice from SMC on submission requirements once final details of product pricing are available.
7. Marketing authorisation for medicine has been extended for use in children or adolescents, where advice has previously been issued for the same indication in adults or the indication in adults predates SMC establishment (31 January 2002)	See section below on paediatric licence extensions.

5.5.2 Abbreviated submission process (therapeutic class)

Under this process, new medicines where alternatives within the same therapeutic class have previously been accepted for use (or restricted use) by SMC may be eligible for the abbreviated submission process. Before making a submission through this route, the company should complete a Company Information Request Form to obtain advice from SMC on submission requirements once final details of product pricing are available. The Company Information Request Form is available on the 'Making a submission' section of the SMC website.

When making an abbreviated submission (therapeutic class) to SMC, the company should consider the following.

- Similar clinical effectiveness to other medicines within the same therapeutic class must be demonstrated briefly and in simple terms. An indirect treatment comparison (published or in-house analysis) may be provided to demonstrate similar clinical effectiveness within class where required.
- The new medicine must cost the same or less or have a limited net budget impact (with PAS or contract pricing where relevant) compared with comparators within the same therapeutic class.
- Any restriction that is applied to comparators within the same therapeutic class is usually applied to the new medicine.

If the medicine is not eligible for the abbreviated submission process, the company has the option to either apply again by providing a new or improved PAS or provide a full submission. If the company is unable to make a submission in an acceptable timeframe, SMC will consider the need to issue advice to

NHSScotland indicating that the medicine is not recommended for use. This does not preclude a submission at a later date.

5.5.3 Scheduling and publication of advice for abbreviated submissions

Companies will be informed of scheduling once the SMC assessment team has confirmed acceptability of the submission for the abbreviated submission route, which will be within 3 weeks from receipt of the abbreviated submission assuming marketing authorisation has been granted.

SMC reserves the right to request a full submission in relation to any medicine in the event that it is anticipated to have an impact on NHSScotland resources which has not been fully taken into account by the submitting company or if similar clinical effectiveness has not been demonstrated briefly, in simple terms. The decision of the SMC in this respect is final and binding.

Advice for abbreviated submissions will be issued following SMC Executive review rather than full committee consideration and will be published on the SMC website in the form of a short advice document. Advice for medicines which have been routed through the abbreviated submission process (therapeutic class) will only be published on the SMC website if the medicine is accepted through this route (see Section 5.5.2 for further detail if the medicine is not eligible for the abbreviated submission process).

5.5.4 Patient Access Schemes (PAS)

If a PAS is available for the existing formulation (previously accepted for use by SMC) or for the parent medicine (a product whose marketing authorisation has been extended to include new age groups within an existing indication) then the PAS should be extended for the medicine under review. This requires companies to submit a new PAS application, see the 'Patient access schemes (PAS)' page via the 'Making a submission' section of the SMC website for further information.

5.5.5 Paediatric licence extensions

SMC no longer requests abbreviated submissions for paediatric licence extensions where advice has already been issued for the corresponding indication in a different age group. ADTCs in Scotland have been advised that they may make formulary decisions on paediatric licence extensions for medicines that SMC has previously accepted for use (or restricted use) in a different age group. Where relevant, the company should liaise with PASAG to ensure that any PAS is extended to include the new age group. Medicines that are not recommended by SMC for use in adults or a different age group are also not recommended when the licence is extended to include other age groups.

Note that SMC reserves the right to request a submission if an assessment of clinical and cost effectiveness is later required by NHS boards.

If the company wishes to make a submission for a paediatric licence extension where the product is not recommended for use in a different age group, then a full submission is likely to be required. Please seek advice from the SMC Secretariat.

6 Submission documents

6.1 The New Product Assessment Form (NPAF)

The NPAF provides a template for the evidence required by SMC to make recommendations to NHS Boards and ADTCs. This form should be used for all full submissions. In some circumstances an abbreviated submission may be appropriate – see Section 5.5.

The Guidance to submitting companies for completion of New Product Assessment Form (NPAF), available on the SMC website, gives detailed advice on what should be provided. This includes a description of the type of information expected to be submitted within each section of the form; an indication, where appropriate, of the expected source of the information; and how the information should be presented.

There is a separate NPAF for ultra-orphan medicines and additional guidance notes for the completion of the NPAF.

The deadline dates for submitting NPAFs to SMC can be found in a timetable on the website. Incomplete submissions cannot be scheduled for assessment. As noted in Section 4, PAS documentation must be provided at the point of submission.

Submissions should be concise but also include all relevant data. The required information is stated for each section of the document and applicants should focus on these requirements and not include information that is not directly relevant to the indication under review. As far as possible, companies should limit the electronic size of the document since it may have to be distributed across servers with varying limits to file size. For guidance, most submissions have a file size of around 5 megabytes (excluding the economic model).

The submission should be a standalone document. It should focus on information related to the indication for which approval is sought, rather than all available data for the medicine. Appendices may be used for information that exceeds the level of detail requested in the guidance but only when considered essential; they should not present core information. For example, it is not acceptable to attach a key study as an appendix and to complete the efficacy section with 'see Appendix X'. In some cases, it will be more appropriate to include data as a supporting document, referenced in the text, than as an appendix. SMC also requires completion of an Excel budget impact template for all full submissions. A blank template can be found on the SMC website.

6.2 SMC review checklists

The SMC/NDC use critical appraisal checklists as part of their review process, as these improve consistency and aid in supporting an efficient review process. Blank copies of the economic checklist, clinical checklist and indirect treatment comparison (ITC) checklist are made available to industry on the 'Making a submission' section of the SMC website. It should be noted, however, that this is provided to aid understanding of the SMC review process. It is not a tick-list for submitting companies; providing all the information on the checklist does not mean that no other information is required. The

full Guidance to submitting companies for completion of New Product Assessment Form (NPAF) must be followed.

6.3 Key points on references

- All evidence included in the submission should be referenced throughout the NPAF and references should be numbered in the order in which they first appear in the text. Each reference should have only one number that is repeated if it is cited more than once.
- At the end of the submission a list of all references should be provided in the Vancouver style, numbered and ordered strictly in accordance with their numbering in the text. Author/date styles of referencing should not be used. Submissions have been received where listed references did not correspond to the citation in the text, including some with mixed referencing styles. These are unacceptable. Referencing using word processing devices such as footnotes/endnotes is also not acceptable.
- Electronic copies of all references cited in the submission should be sent to the secretariat in a zipped file or uploaded to the Egress Secure Workspace to be received no later than the monthly deadline for receipt of company submissions. Should the file size exceed 20 megabytes, please condense into separate files and send each file separately. Each reference should be provided as an individual electronic file and should be in an electronic format that can be searched, such as Word or Portable Document Format (PDF). Scanned documents are not acceptable other than in exceptional cases where it is impossible to provide a document in a searchable format.
- Full versions of in-house clinical study reports and/or drafts for publication should be provided where these have been used as data sources. These are required for assessors to make factual checks and to gain a comprehensive understanding of relevant study methodology, conduct and results. Synopses and selective extracts are not sufficient.
- On request from the submitting company, SMC will treat data from these sources (that are not otherwise in the public domain) as confidential and the information will not be disclosed in any form to persons or organisations outwith the SMC Committee and NDC, SMC clinical and economic assessors and secretarial staff. Academic in confidence (AiC) information will also be available to attendees of the PACE meeting within the NDC preliminary advice.
- These data will be annotated (by highlighting and underlining) to indicate that they are confidential in paperwork provided to the SMC Committee and NDC and will be removed from the SMC DADs that are issued to the NHS and posted on the SMC website.
- While SMC encourages full referencing of the evidence presented, please avoid the inclusion of unnecessary references such as those that duplicate evidence provided by more robust sources.

Further guidance on referencing, including the use of abstracts and posters as sources of information, is provided in the Guidance to submitting companies for completion of New Product Assessment Form (NPAF).

6.4 Confidentiality of information

SMC is committed to transparency in its decision making. For this reason, confidential information should be kept to a minimum and only data that are genuinely confidential, such as actual numbers should be marked by the company as 'in confidence'. If the medicine is subject to a confidential PAS,

SMC preference is to quote the 'with PAS' ICER used for decision making at the SMC meeting and to publish this figure in the final DAD.

Information provided in company submissions will only be available to members of SMC and NDC, SMC clinical and economic assessors, statisticians and secretariat staff.

Information that is commercial in confidence (CiC) or AiC will be annotated by underlining and highlighting in the DAD made available to committee members and will be removed before making the DAD available to the public. During SMC meetings in public, confidential information will be redacted from PowerPoint slide presentations, AiC data may be verbalised unless the company notifies SMC that the data are not theirs and therefore, they cannot grant permission. Should CiC information need to be discussed, a closed Part 2 meeting will be held.

Companies are required to state why the data are confidential and the timescale within which they will remain confidential should be detailed within the NPAF. SMC will respect confidentiality but reserves the right to include data that are already in the public domain, for example as a published abstract or conference poster. In such cases, SMC will not exceed the level of detail in the published source and the submitting company will have an opportunity to review the DAD as part of the routine consultation process, as outlined below. It should be noted that SMC's critique of the clinical and economic evidence as summarised in the DAD is owned by SMC and may not be marked as confidential (excluding information that is genuinely confidential as described above). SMC is committed to adhering to the guidelines agreed with the Association of the British Pharmaceutical Industry (ABPI) that are published on the SMC website.

7 Engagement between SMC and submitting companies

7.1 Early engagement with companies

If a company is unsure about whether the medicine is within SMC remit or the type of submission required, the pharmaceutical company may complete and return a Company Information Request Form to the secretariat. For other company enquiries, please use the 'Contact us' form on the SMC website.

SMC staff do not routinely meet with submitting companies in advance of making a submission; there is no option for a scoping meeting. However, the opportunity for pharmaceutical companies to meet with SMC staff prior to submission for a newly licensed medicine may be provided in specific circumstances.

The main purpose of these early engagement meetings is to support pharmaceutical companies in understanding SMC processes in order that they may determine the nature and focus of a submission and to help NHSScotland by reducing the number of non-submissions to SMC. Pharmaceutical companies may then be able to produce a high-quality submission for review by SMC where they previously felt unable to submit.

As SMC is unable to facilitate meetings for all potential submissions, early engagement meetings with an individual pharmaceutical company will take place only in specific circumstances, as detailed below.

Early engagement meetings will be considered where:

- intelligence suggests a submission may not be forthcoming and where there is exceptionally high patient need, for example a medicine used for a condition where no other treatment is available
- a company has had limited previous experience of engagement with SMC, and/or aspects of the submission suggest there would be mutual benefit from a meeting, to discuss concerns or issues relating to SMC process or policy at an early stage.

These criteria are not exhaustive and SMC will retain the flexibility to hold a meeting when it is considered this would be mutually beneficial to both the company and SMC. Meetings will be focused on a single submission.

Some examples of medicines that may be discussed include those in EAMS, validated ultra-orphan medicines and Advanced Therapy Medicinal Products.

To request an early engagement meeting, companies should complete and submit the early engagement information request form available from the SMC website. The completed form should contain enough information to enable SMC to determine whether the submission meets the above criteria. Please note that a maximum of 3 company representatives are welcome to attend an early engagement meeting.

The meetings are held virtually, using the MS Teams platform, on a mutually agreeable date and time and are scheduled to last 1 hour.

SMC aims to respond within 15 working days to requests for these meetings and will then endeavour to arrange a meeting as soon as convenient for both parties.

All communication queries and requests relating to early engagement should be directed to his.smcsubmissionportal@nhs.scot.

7.2 Responding to New Drugs Committee (NDC) preliminary advice

After the NDC meeting has taken place, the submitting company is asked to comment on the NDC DAD for their medicine. This stage of the process is generally referred to as 'Company Comments' by SMC and the submitting company. The following is a general guide on issues which should be considered when compiling a response to the NDC DAD and what information submitting companies should provide within their Company Comments.

7.2.1 Focusing the response

In order to provide a robust response to the NDC DAD for consideration by the SMC, the Company Comments document should be focused and concise. The NDC DAD will have outlined the main issues or weaknesses in the clinical and economic evidence within the 'Clinical Effectiveness' and 'Health Economics' sections of the document respectively. The health economics section will generally present the weaknesses in order of priority, but it should be noted that on occasion the multiple issues outlined could be equally important, or have a cumulative impact, on the decision uncertainty and thus each should be addressed. In addition, where an indirect comparison has informed the economic case, then weaknesses with the indirect comparison may be described in the clinical effectiveness section of the NDC DAD.

It is important that these issues are addressed in the response. When responding to a particular weakness in the NDC DAD, be it a clinical effectiveness or health economics issue, it is advisable to quote or make clear which weakness or text is being addressed, as well as referencing the relevant page number of the NDC DAD.

SMC welcomes additional information that can reduce the uncertainty or limitations within the analysis. However, provision of a completely new indirect comparison at the Company Comments stage may constitute a large amount of information which may not be practicable for the SMC to assess.

7.2.2 Providing clarification

The Company Comments document may also be used to provide further clarification regarding analyses or data reported in the NDC DAD. For example, further clarification and context may be provided as to why a particular ICER is less relevant to the appraisal process or represents a less plausible scenario. This is an opportunity to carefully review the NDC DAD and provide notification of any factual inaccuracies as well as suggest amendments. The document should also clarify what data presented in the NDC DAD are confidential (either AIC or CIC) and cannot be presented in the final published SMC DAD. If the Company Comments quotes or presents information which are considered

confidential please ensure that appropriate AIC and CIC markings are also applied to these data in the Company Comments document. If necessary, an Updated Checklist of Confidential Information – Company Comments should be submitted with the Company Comments. This is available in the ‘Making a submission’ section of the SMC website. Note that SMC would not anticipate notification of any further changes requested to the DAD after the Company Comments deadline.

7.2.3 Presentation style

When compiling the Company Comments document, it may be useful to consider the presentation of the information from the perspective of an SMC Committee member, as the Company Comments are included in full in members’ paperwork. A bulleted summary of the key points and main issues covered in the response included at the start of the document can be useful where several issues are addressed. In addition, please include a short, bulleted summary outlining why SMC should accept the medicine for use in NHSScotland. Please ensure that the key points regarding a company response are made succinctly in the main body of the Company Comments document. The preference is that appendices are not used routinely but may be included if necessary.

To ensure that the Company Comments document is succinct and well presented, please avoid comments which do not address inaccuracies or deficiencies in the evidence such as restating points already presented in the submission, or reiterating positive statements made in the NDC DAD. It is also helpful to indicate how SMC modifiers may apply to the medicine under review, and if applicable, highlight how the wider perspectives of the ultra-orphan framework are relevant.

Finally, the Company Comments should be submitted as a standalone document for consideration by the SMC. It is not appropriate to submit an edited version of the NDC DAD as the Company Comments document; however, an edited NDC DAD may be submitted as supplementary information alongside the Company Comments where a company wishes to suggest revisions to the text for consideration by SMC.

7.2.4 Provision of additional analyses or data

A submitting company may have been asked to provide additional analyses to address weaknesses specified in the DAD. The response to these questions and the related analyses may have been submitted to SMC as a separate document, however it can be helpful to summarise such responses and their impact on any key uncertainties within the Company Comments. On occasion, more recent data regarding the efficacy of a medicine may become available between the time of submission to the SMC and the Company Comments stage of the process. These data should be provided alongside references, and copies of references, when submitting Company Comments.

Due to the tight timelines in SMC process, large amounts of new data cannot be accepted at the Company Comments stage since significant amounts of additional data may only be considered through the resubmission process.

7.3 Attending a virtual SMC committee meeting

Company representatives will be invited to participate in a meeting where a full submission or resubmission for one of their products is being considered. There is no obligation for the company to attend and non-attendance will not prejudice the review.

The submitting company will receive an invitation to attend the SMC meeting which will include the deadline for company representatives to register for the meeting. Registration is mandatory and must be completed in advance of the meeting, through the SMC website. SMC Committee meetings are also open to members of the public to observe. As noted in Section 2.5.2, approximately three meetings per year are hybrid; on these occasions company representatives will have the option to attend in person or virtually.

Requests for a company representative to join a meeting will be confirmed the day prior to the meeting and the confirmation email containing joining instructions should be retained. The joining instructions are unique to each attendee and should not be shared with anyone as only one person can join a meeting per unique URL.

The meetings run from 12.30pm to 4.30pm (approximately). Although the meeting does not commence until 12.30pm, it is essential to join promptly at 12 noon. There are a series of security checks to run whilst attendees are held in a virtual meeting room followed by a number of briefings. To leave before the end of the meeting, please use the chat function to notify the co-host before leaving.

If a meeting is cancelled or the time and/or location of a meeting changes, details of this will be published on the SMC website and attendees will be informed as quickly as possible.

SMC allows a maximum of two company representatives for each medicine under consideration. The role of the company representatives is to respond to specific enquiries and/or comment on any matters of factual accuracy as directed by the SMC chair and provide clarification on any outstanding issues which they believe SMC should be aware of prior to reaching its decision on advice to NHSScotland.

The company representative should:

1. Be an employee of, or be acting on behalf of, the submitting company and ideally have been directly involved in the development of the company's submission
2. Have detailed knowledge of the product under consideration and be able to engage effectively with the SMC committee
3. Have a comprehensive understanding of SMC submission and decision-making processes
4. Be able to comment accurately on the product's
 - a. Comparative efficacy
 - b. Comparative safety
 - c. Clinical effectiveness
 - d. Cost effectiveness
5. Agree to be bound by the terms and conditions of SMC's existing confidentiality policy regarding medicines under assessment.

SMC meetings routinely consider up to six full submissions. As it is not possible to specify timings for individual product discussions in advance, the company representatives will be invited to attend from the start of the committee meeting. Company representatives have the option to leave the meeting after their agenda item has been discussed. They will receive a briefing prior to the meeting and may be asked for feedback following their attendance.

Company representatives will be invited to participate in the meeting at the point where their product is under discussion. The chair will invite them to introduce themselves to the committee. Once introductions have been carried out, company representatives will be placed on mute and the discussion will commence. They should keep their camera on throughout the discussion of the medicine.

If a committee member has a question that they would like to ask of a company representative, they will use the 'hand up' facility to ask permission from the chair for the question to be asked. If the question is appropriate, the chair will invite the company representative to answer.

Company representatives are asked not to speak during discussions, unless invited by the chair. This is to ensure that business is conducted efficiently.

When consideration of the medicine is coming to an end, the chair will ask the company representatives if they are content with the accuracy of discussions and if they would like to provide any additional points of clarity relating to the medicine. **It is not permitted to add any new information at this point, unless it is to provide clarity on a particular point or to answer a specific question.** When all discussions about the medicine are finished, the chair will thank the company representatives and they will then be placed back onto mute and their camera will be turned off again.

In the event of a closed session being required within the meeting, company representatives will normally **be asked to leave** the virtual meeting room although at the discretion of the chair they may be asked to remain in the room for this part of the discussion to specifically respond to questions from committee members about confidential information in the company's submission.

At the time of the SMC meeting, the NDC has considered in detail the evidence submitted by the pharmaceutical company who has had the opportunity to respond with clarification, further analysis and comments on any weaknesses identified. SMC will consider NDC's preliminary advice together with clinical expert opinion, patient group contributions and, where relevant, the output of a PACE meeting and any applicable decision modifiers. There is no requirement for members to repeat the detailed discussion held at NDC. The company response to the NDC preliminary advice is included in the meeting papers and reflected in the presentation given by the SMC team to committee members before the medicine is discussed.

The presence of representatives from the submitting company provides a further opportunity for members to raise any outstanding points about the clinical and cost-effectiveness evidence that they feel the company has not adequately addressed in their submission or in the Company Comments after NDC. New evidence from the company is not admissible at this stage in the process. Company representatives should be aware that there may be no need for questions, if the submission is straightforward and there are no outstanding issues.

Company representatives will not be participants in the discussion on the submission. All questions for company representatives should be directed through the chair and the company will be invited to respond at the chair's discretion.

Members should not ask the company to answer questions that directly refer to commercial in confidence information, such as the value of a PAS, but they may ask the company about outstanding

issues in relation to the clinical, economic or budget impact information within the submission or supporting documentation.

The submitting company delegates will be invited to respond and provide clarification of any outstanding issues. After all submissions have been discussed, a closed session will take place where members will vote. The decision for each medicine will be announced to the committee after the vote.

SMC meeting minutes will contain the names of the companies in attendance, but not the names of the individuals.

Please note that medicines which are being assessed for the first time using the ultra-orphan pathway follow a different process. Since SMC does not make a decision at this stage in the ultra-orphan process there will be no requirement for SMC Committee discussion on the initial assessments of ultra-orphan medicines.

7.4 Meetings with pharmaceutical companies post-SMC

If a medicine is not recommended for use, and the company wishes to consider making a resubmission, a meeting with SMC representatives is recommended. The aim of the meeting is to help the company understand why their medicine has not been accepted and determine the nature and focus of any resubmission or independent review panel (see Section 5.4.3).

These company meetings are arranged via the SMC Secretariat and are held following publication of the DAD on the SMC website; they normally involve a member of the SMC Executive (usually the vice chairs of NDC and SMC), and a member of the SMC Secretariat. It should be noted that individual members of the assessment team are not involved in these meetings. Meetings will be submission-specific, led by a representative of the SMC Executive and will be scheduled for 1 hour. A minute of the meeting will not be provided.

A maximum of two company representatives is permitted.

The standard format for meetings will be as follows:

- a. Introductions.
- b. A disclaimer: Advice or guidance provided is the personal opinion of the SMC Executive member leading the meeting and does not guarantee that NDC/SMC would support a resubmission that subsequently encompasses the advice or guidance given.
- c. Agreement on the purpose of the meeting: The purpose of the meeting is to support manufacturers in understanding the SMC decision and process in order that they may determine the nature and focus of a resubmission or IRP. Any specific technical questions which arise may need to be managed outwith the meeting.
- d. Discussion of agenda items.
- e. Discussion of next steps.

An evaluation form will be provided to enable the company to provide feedback.

If a company advises prior to the publication of SMC advice that they wish to make a resubmission, the advice box in the DAD will note that the submitting company has indicated their intention to resubmit.

7.5 'Not recommended' advice following non-submission by a pharmaceutical company

Pharmaceutical companies are encouraged to keep the SMC Secretariat informed of their proposed timelines for introducing a medicine to the UK market, as this will help ensure that the timing of SMC advice meets the needs of NHSScotland.

SMC aims to issue advice to NHSScotland on newly licensed medicines as soon as practical after medicine availability. To achieve this, taking into account the NDC/SMC assessment timescale, pharmaceutical company submissions should be made before the medicine becomes available for use. Ideally, a company should make a submission as soon as practical after a positive opinion has been issued by the EMA, Committee for Medicinal Products for Human use (CHMP) or no more than 2 months before final approval by the MHRA.

If the pharmaceutical company is unable to make a submission for a medicine that is within remit in an acceptable timeframe, SMC will issue advice to NHSScotland indicating that the medicine is not recommended for use. The pharmaceutical company has the opportunity to engage with the SMC Secretariat about what might be an acceptable submission timeline before the decision to issue 'not recommended' advice is taken.

If the pharmaceutical company indicates that it does not intend to make a submission, SMC will issue 'not recommended' advice to NHSScotland soon after the medicine has become available for use. The company will be provided with the draft 'not recommended' Statement of Advice before it is considered by SMC. If the pharmaceutical company has advised that they will make a submission in the future (but not within an acceptable timeframe) the SMC Statement of Advice will include the following sentence: *"The holder of the marketing authorisation has indicated that they plan to make a submission to SMC in the future."*

When 'not recommended' advice has been issued due to non-submission, this does not prevent the pharmaceutical company from making a submission at any point in the future. SMC advice based on a subsequent submission will supersede the initial 'not recommended' advice.

8 Handling of data

8.1 Release of company data

Please refer to the following document which is published on the SMC website: *'Agreement between the Association of the British Pharmaceutical Industry (ABPI) and the Scottish Medicines Consortium (SMC), which is part of Healthcare Improvement Scotland (HIS), on guidelines for the release of company data into the public domain during SMC assessment'*.

8.2 Consultation with competitor companies

Companies marketing competitor products referred to within certain sections of the SMC DAD have the opportunity to comment on the factual accuracy of what is said about their product.

A competitor product may be any on-patent medicine or non-medicinal treatment available in the UK, which may be a licensed or off-label treatment option for the indication under review in the DAD (irrespective of whether the competitor medicine has previously been accepted for use by SMC).

The MAH of the competitor product will be consulted, in confidence, after the SMC decision has been communicated to NHSScotland, but prior to publication on the SMC website. They will be sent the full SMC DAD and invited to comment on the factual accuracy of information in relation to their product only, contained in the Clinical Context, Summaries of Clinical Evidence, Safety Evidence and Clinical Effectiveness Evidence Considerations sections of the DAD. Comments that do not relate to factual accuracy will not be considered. Comments will be reviewed by the SMC Executive and both the submitting company and competitor company notified of any resultant change(s) to the DAD.

Where a competitor product is specifically referred to in the Summary of Comparative Health Economic Evidence of the DAD (or Value for Money section of the ultra-orphan medicine assessment report), the competitor company will be forwarded a copy of the DAD, for information only.

Data that are CIC or AIC will be removed prior to consulting with competitor companies.

8.3 Release of clinical, economic and indirect comparison checklists

Clinical, economic and ITC checklists are provided to NDC members and SMC Committee members before their monthly meetings.

SMC assessment teams use the clinical and economic checklists to summarise the main points of the company's submission and highlight the key strengths and weaknesses of the case being presented. Considering information recorded on the checklist allows reviewers to come to a judgement on whether the case for the medicine has been demonstrated.

The ITC checklist, designed to assess all ITC methodologies, is used to evaluate the quality and validity of any indirect evidence, confirming the type of analysis, reviewing study selection and methodology and identifying the areas where statistical expertise is required.

Copies of the checklists are provided to the submitting company in confidence and for information only, on request, via the SMC Secretariat.

In accordance with data protection legislation, all personal information is removed from the checklist before it is released to the submitting company.

8.4 Freedom of Information

SMC is part of the Evidence and Digital Directorate in Healthcare Improvement Scotland, a public authority covered by the Freedom of Information (Scotland) Act 2002. Information on how to make a Freedom of Information request, and on your information rights, can be found on the [Healthcare Improvement Scotland website](#).

9 Glossary

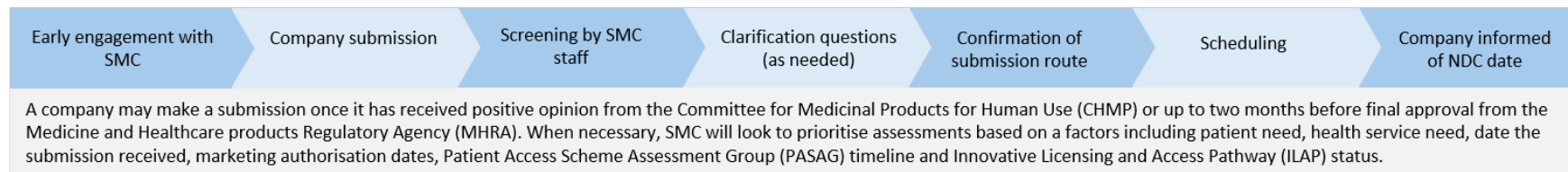
Term	Description/definition
Branded generic medicine	Branded generic medicines are generic medicines that are marketed under a brand name.
Early Access to Medicines Scheme (EAMS)	EAMS is a UK regulatory programme led by the MHRA. It aims to give patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorisation when there is a clear unmet medical need.
SMC end-of-life medicine definition	A medicine used to treat a condition at a stage that usually leads to death within 3 years with currently available treatments. The definition of end-of-life medicine may be based on a subpopulation of the licensed indication where the submission is positioned for use in this subgroup and the submitting company provides adequate justification.
United Kingdom conditional marketing authorisation	A medicine may be granted a conditional marketing authorisation by the MHRA where the benefit of immediate availability outweighs the risk of having less comprehensive data than normally required. The marketing authorisation holder will be required to complete specific obligations (ongoing or new studies, or collection of pharmacovigilance data) with a view to providing comprehensive data confirming that the benefit-risk balance is positive. Once comprehensive data on the product have been obtained, the marketing authorisation may be converted into a standard marketing authorisation (not subject to specific obligations).
Generic medicine	Generic medicines contain the same active substance(s) as the previously authorised reference medicine, and is used for the same indication(s). Generics are authorised to the same standards of safety, quality and efficacy as the reference medicines, and have to demonstrate in clinical studies that they are bioequivalent to the original product: that is, they deliver equal medical benefits to the patient. Generics can only be marketed

	once the marketing protection has expired, usually from 10 or 11 years from the date of first authorisation of the reference product.
Hybrid medicine	Hybrid medicines are medicines whose marketing authorisation depends partly on the results of tests on the reference medicine and partly on new data from clinical trials of the generic medicine. This happens when a manufacturer develops a generic medicine that is based on a reference medicine, but has a different strength, a different route of administration or a slightly different indication from the reference medicine.
The MHRA Innovative Licensing and Access Pathway (ILAP)	The aim of ILAP is to allow safe, timely and efficient development of innovative new medicines for patients. These medicines will be used to treat serious conditions and/or for conditions where there is a lack of treatment options. SMC is a partner in ILAP, alongside the MHRA, the National Institute for Health and Care Excellence (NICE) and the All Wales Therapeutics and Toxicology Centre.
Orphan medicine	A medicine with a UK orphan designation at the time of marketing authorisation from the MHRA (conditions affecting fewer than 2,500 people in a population of 5 million). The definition of orphan medicine is based on the full population of the licensed indication relevant to the submission, irrespective of whether or not the company wishes SMC to consider the product when positioned for use in a subpopulation of the licensed indication.
Orphan equivalent	A medicine to treat conditions affecting fewer than 2,500 people in a population of 5 million irrespective of whether it has designated orphan status. The definition of orphan equivalent is based on the full population of the licensed indication relevant to the submission, irrespective of whether or not the company wishes SMC to consider the product when positioned for use in a subpopulation of the licensed indication.
Patient Access Scheme (PAS)	Scheme proposed by pharmaceutical companies to improve the cost effectiveness of medicines, thereby

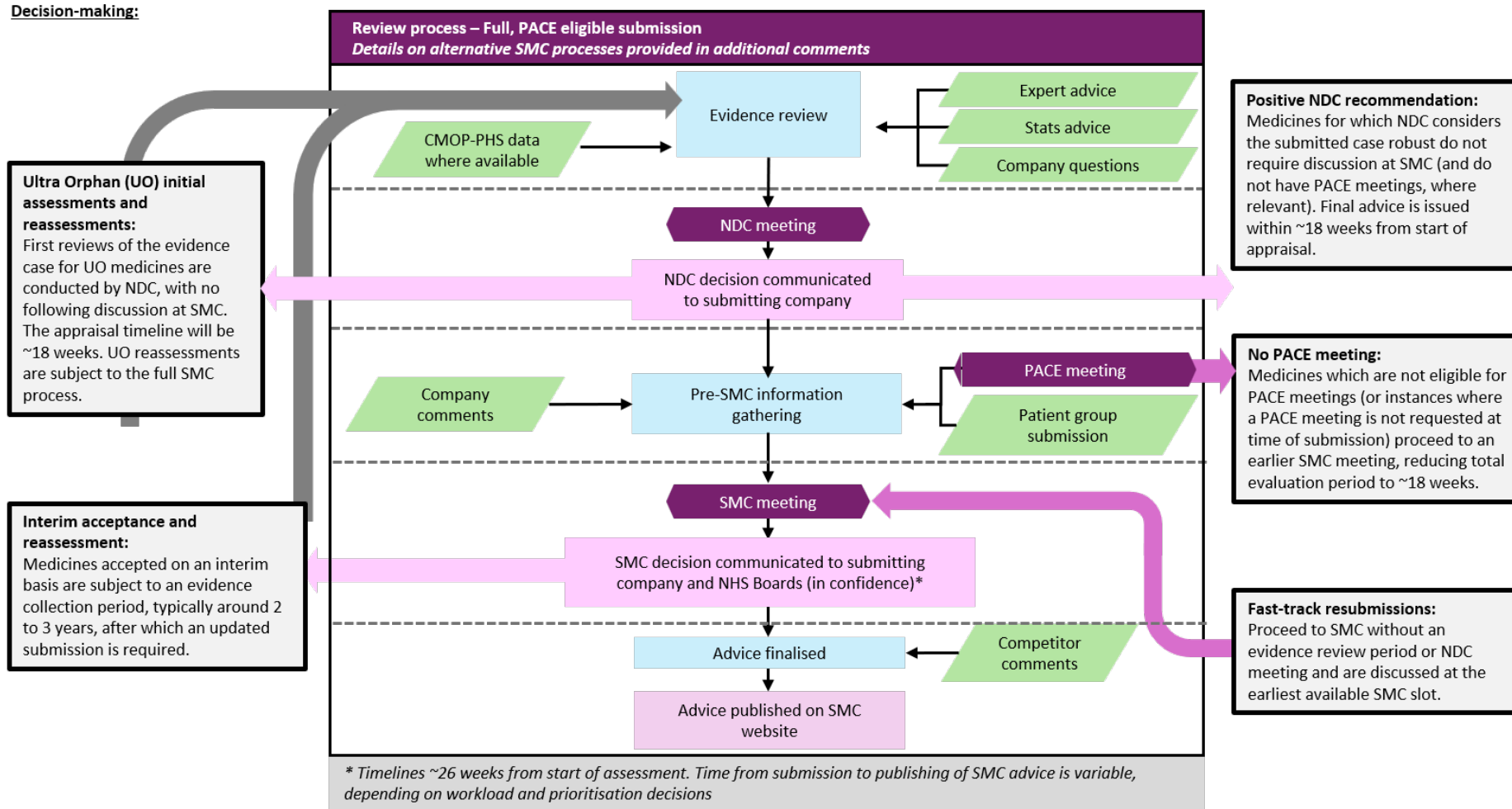
	facilitating patient access. For example, a confidential discount on the list price of the medicine.
Presentation	Presentation may relate to factors such as pack size, packaging, administration or delivery device.
SMC ultra-orphan medicine definition	To be considered as an ultra-orphan medicine all criteria listed should be met: 1. the condition* has a prevalence of 1 in 50,000 or less in Scotland 2. the medicine has a UK orphan marketing authorisation from the MHRA 3. the condition is chronic and severely disabling 4. the condition requires highly specialised management (for example within the context of a nationally funded service). *typically a recognised distinct disease or syndrome. SMC uses the description of the orphan condition within the MHRA's Orphan Register.

Appendix 1 SMC's assessment process

Scheduling:



Decision-making:



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You can read and download this document from our website.

We are happy to consider requests for other languages or formats.

Please contact our Equality and Diversity Advisor on 0141 225 6999
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