Amplifying the patient voice: characterising the impact of Patient and Clinician Engagement (PACE) meetings on SMC decision making

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Background

- Patient involvement can offer benefits throughout the medicine development cycle, from industry-led research to regulation and licensing to health technology assessments (HTAs).[1]
- In 2014, the Scottish Medicines Consortium (SMC) introduced Patient and Clinician Engagement (PACE) meetings for therapies used to treat end-of-life (EoL) and/or rare conditions following an initial 'not recommended' decision by the New Drugs Committee (NDC).[2]
- PACE meetings give patient groups and clinicians a stronger voice in decision making and provide additional perspectives that may not be captured within conventional clinical and economic assessment processes. These may include understanding clinical issues (e.g. unmet need, severity or pathway positioning), and the potential added value of a medicine for patients and their family or carers.[2]

SMC qualification criteria for a PACE meeting[1]

EoL: a medicine used to treat a condition at a stage that usually leads to death within 3 years with currently available treatments (can be based on a sub-population of the licensed indication).

Orphan: a medicine with MHRA orphan marketing authorisation (i.e., affecting <2,500 people per 5 million) or a medicine to treat an equivalent size of population irrespective of designated orphan status (only based on the full population of the licensed indication relevant to the submission).

Ultra-orphan [all criteria must be met]: (1) prevalence ≤1 in 50,000 in Scotland, (2) has MHRA orphan marketing authorisation, (3) the condition is chronic and severely disabling, and (4) the condition requires highly specialised management.

Objective

- To characterise the impact of PACE meetings conducted since their introduction in 2014, and to explore considerations around qualification criteria and discussion topics.

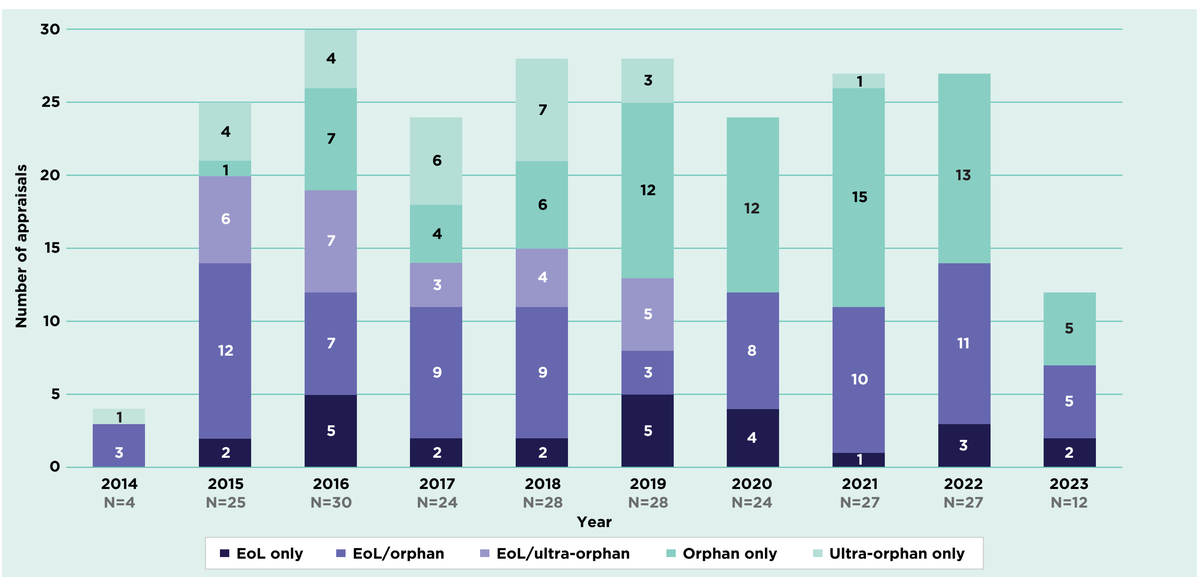
Methods

- SMC assessments published between 1 January 2014 and 20 June 2023 that included a PACE meeting were identified via the SMC website using the search terms "patient and clinician engagement" and "PACE".
- Advice publication date, indication, assessment outcome (accepted, restricted, not recommended), pathway (EoL, orphan, ultra-orphan or a combination), and inclusion of a patient access scheme (PAS) were captured.
- Key discussion details related to burden of disease (patient daily living impairment, patient QoL, caregiver impact, family impact), unmet need and positioning (defined unmet need, pathway positioning) and added value (Rx convenience, improved patient QoL, improved caregiver/family impact, improved ability to work (patient), improved ability to work (caregiver/family) were identified; each individual category was scored 'yes' or 'no' depending on whether it was mentioned in the 'Summary of patient and clinician engagement' section of the Detailed Advice Document.

Results

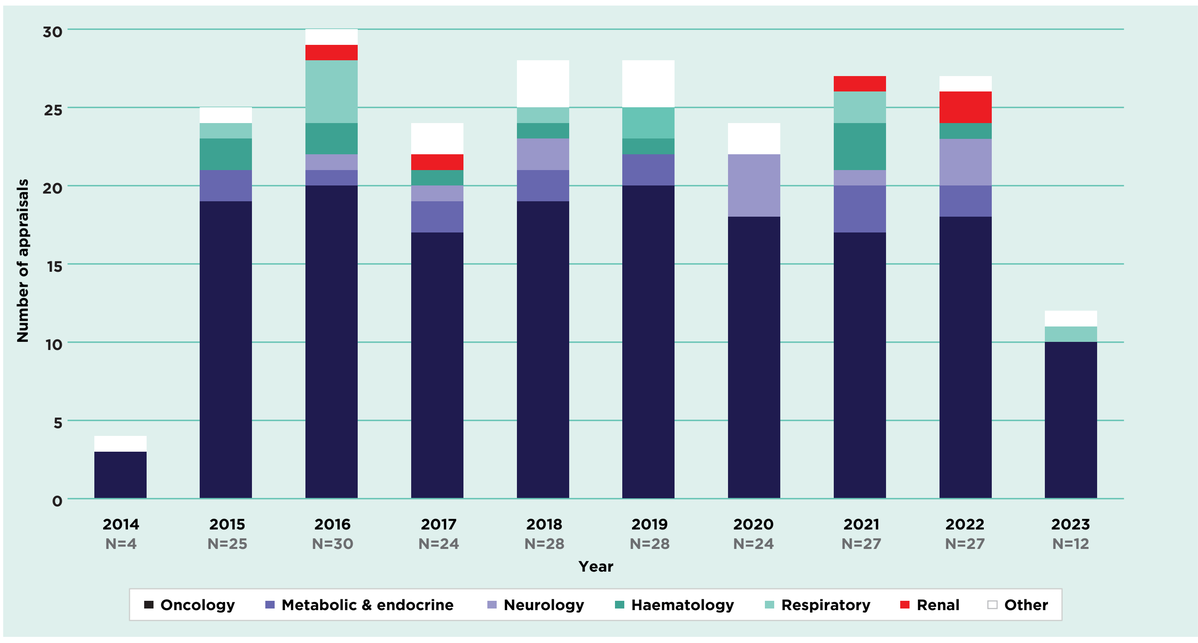
- Of the 229 appraisals identified, 128 (55.9%) PACE meetings were for EoL appraisals (26 (11.4%) were categorised as 'EoL' only, 77 (33.6%) were 'EoL/orphan' and 25 (10.9%) were 'EoL/ultra-orphan'); 75 (32.8%) were for orphan only medicines and 26 (11.4%) were ultra-orphan only medicines. Of note, only one ultra-orphan therapy has been granted a PACE meeting since 2020 (**Figure 1**).

Figure 1: SMC appraisal pathway by year (N=229)



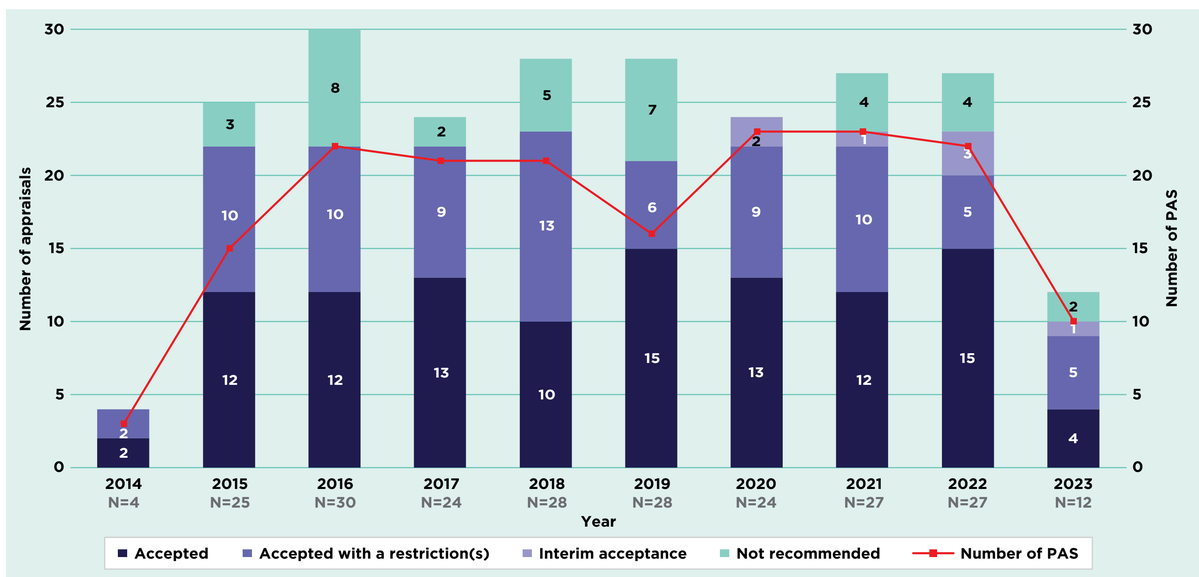
- From 2015 onwards, the number of PACE meetings the SMC conducts each year has remained fairly constant, at between 24 and 30 per year (not including 2023).
- The majority of appraisals that utilised a PACE meeting were for oncology indications (161; 70.3%), followed by metabolic and endocrine (14; 6.1%), neurology (12; 5.2%), haematology (11; 4.8%) and respiratory (11; 4.8%) indications (**Figure 2**).

Figure 2: Therapy area by year (N=229)



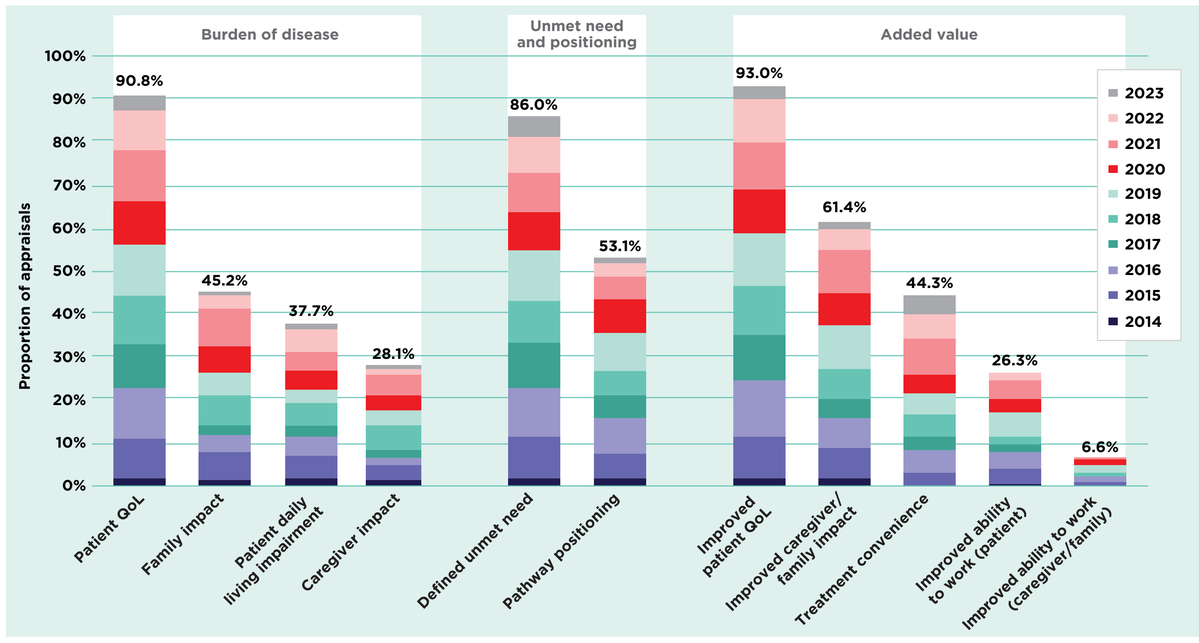
- After PACE meetings, 108 assessments were accepted for the full target population (47.2%), 79 were accepted with a restriction(s) (34.5%), 7 received interim acceptance (3.1%), and 35 were not recommended (15.3%). A PAS was agreed for 176 of the appraisals (76.9%) (**Figure 3**).

Figure 3: SMC decision and number of appraisals that incorporated a PAS by year (N=229)



- Patient quality of life (burden of disease, and potential improvements with the medication under assessment) was discussed in over 90% of PACE meetings, and a similar proportion of meetings also explored unmet medical need (86.0% of meetings). Meetings also provided an opportunity to explore improved family and/or caregiver impact (61.4% of meetings), and more than half discussed where the new therapy would be positioned in the treatment pathway (53.1% of meetings) (**Figure 4**). Conversely, only 26.3% and 6.6% of PACE meetings involved discussion of improved ability to work for patients and family/caregivers, respectively.

Figure 4: Percentage of appraisals that included key discussion categories at PACE meetings each year (N=228*)



* One appraisal did not include PACE meeting details within publicly available documentation.
Abbreviations: QoL, quality of life

Conclusions

- While PACE meetings extend the appraisal process, over 80% of appraisals that included one resulted in a positive recommendation (for either the full population or a restricted population), demonstrating the benefit of incorporating the patient perspective into SMC decision making.
- Interestingly, 23% of appraisals did not incorporate a PAS, indicating that PACE meetings have had a positive impact on approvals even in the absence of a net price discount. Moreover, while very few assessments resulted in an interim acceptance, this decision has only been an option for the SMC since its introduction in 2018.
- Given the high approval rate, these data suggest that PACE meetings, whilst not the deciding factor, positively contribute to SMC decision making. Manufacturers should be aware of the circumstances under which the SMC is likely to include a PACE meeting during its assessment, and the patient, caregiver and family impacts that are likely to be addressed during the process.

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Disclosures
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References

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