Speaking up for patients

Patient organisation involvement in Health Technology Assessment, with a focus on Patient and Clinical Engagement at the Scottish Medicines Consortium

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Foreword

For many people living with cancer, and the patient organisations and charities that support and represent them, rapid and easy access to new and innovative treatments as they come through the research, development and approval pipeline is vitally important for obvious reasons. For patients, those treatments may represent a cure, a life-extending treatment or a means to managing their condition in the longer term.

In the UK, patients who are treated by the NHS are generally dependent on technology appraisals of those new treatments as the means of deciding whether or not they are to be made available. Three bodies carry out that role across the four nations - the National Institute for Health and Care Excellence in England (the advice and recommendations of which are generally followed in Northern Ireland), the All Wales Medicines Strategy Group and the Scottish Medicines Consortium. In addition, in England, the Cancer Drugs Fund also plays a role in the access and management of new treatments. Each of those agencies takes a different approach to its role and how they involve patients and patient organisations in their work and as an organisation and alliance of over 80 cancer patient organisations throughout the UK, Cancer52 wanted to have a better of understanding of those variations and what difference they make to people affected by rare and less common cancers. As you'll see from this report, the answers are interesting and present a strong case for the proper evaluation of all the approaches, with then a move to consistent, patient-focused best practice across the UK.

Technology appraisal and rapid access to new and innovative treatments is particularly important for people with a rare or less common cancer - and those who represent and support them. There are literally hundreds and hundreds of rare and less common cancers, each with their own drug development pipelines - some with well-funded research programmes, many others less so. Almost all of them, however, will be relying on small pools of patients and patient data in clinical trials to provide the evidence for marketing authorisation, and then, NHS availability. While much of the appraisal process understandably relies on clinical and economic data, patient experience is rightly seen as a important plank of the system. This applies to all cancers, but it's particularly important for rare and less common cancer where trial data may not be as mature and where patient numbers are low. In such situations, strong and accessible patient involvement processes and protocols can help shine a light on real world data and intelligence. Through such processes, we can better understand the reality of the disease in question, what impact the new treatment has and what the side effects and quality of life issues mean in practice - that says so much more than clinical evidence and health economics can say on their own.

This important report speaks to those issues and asks everyone involved in the world of health technology appraisal to speak up for patients - whether they be clinicians, health economists, appraisers, health professionals, or commissioners. If we don't all take on this role - and demand that the patient voice is heard - then our cancer treatments and their availability will be the worse for it. And, it will be those with rare and less common cancer who will feel it the most.

Jonathan Pearce, Chair, Cancer52
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Executive Summary

Main findings

- There is no gold standard for patient and patient group involvement in HTA with agencies exploring with different approaches.
- Patient involvement is particularly important for rare and less common cancers where there may be gaps in the evidence base reflecting small patient numbers. Cancer52 believes that patients can contribute to a fuller understanding of the impact of new medicines.
- All parties – including patient organisations – need to share experience, learn from each other, evaluate the effectiveness of their approaches to patient involvement and jointly develop best practices.

Introduction and objective

Health Technology Assessment (HTA) is a key tool affecting access to new cancer medicines. Access to cancer medicines, in turn, can influence patient outcomes. The involvement of patients and the public in HTA is encouraged, with new approaches being developed and implemented across the UK. This is particularly the case with the new Patient and Clinical Engagement (PACE) process by the Scottish Medicines Consortium (SMC) for end of life and orphan medicines. Orphan medicines are medicines to treat diseases with a prevalence of less than 5 per 10,000 people in Europe (EMA Undated).

The PACE process aims to give patient groups and clinicians a stronger voice in SMC decision making, by describing the added benefits of a medicine, from both patient and clinician perspectives, which may not have been be fully captured within the conventional clinical and economic assessment process.

If an end of life or orphan medicine is not recommended following evaluation by the New Drugs Committee, the pharmaceutical company can choose to request a PACE meeting. The meeting will hear from representatives from patient groups and clinicians from the specialty. As a result of these discussions an agreed document is added to the evidence presented at the next SMC meeting. The SMC states that the output from the PACE meeting will be a major factor in the SMC decision.

Cancer52 wanted to explore how patient and patient organisations views are incorporated into HTA in the UK, focusing in particular on the new PACE model used in Scotland, and identifying recommendations for change.

Context

Public and patient involvement in HTA is being encouraged, though it is being approached cautiously with debate on who, when and how it is best achieved. There is an opportunity to make HTA more comprehensive and fairer, whilst also promoting patient empowerment. This will require sufficient resources.

Approach

Literature on patient involvement in HTA was reviewed, and supplemented with a review of HTA agencies websites. Ten Cancer52 members took part in a structured telephone interview between September and November 2015, of whom nine had experience of PACE.
Opportunities for involvement of patients and patient organisations in HTA in the UK

Table 1 below summarises the opportunities for patient and patient group organisation involvement across UK HTA agencies.

The comparison illustrates that there are now different approaches for evaluating some medicines and different opportunities for patient involvement.

Table 1: Summary of opportunities for patient and patient group organisation involvement across UK agencies

<table>
<thead>
<tr>
<th></th>
<th>All Wales Medicines Strategy Group (AWMSG)</th>
<th>National Institute for Health and care Excellence (NICE)</th>
<th>Scottish Medicine Consortium (SMC)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Strategy/Policy in place?</strong></td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td><strong>Support</strong></td>
<td>• Staff provide support</td>
<td>• PIP programme with dedicated staff providing outreach, training and support</td>
<td>• Public involvement team, with dedicated staff proving outreach, training and support</td>
</tr>
<tr>
<td></td>
<td>• Dedicated section on website</td>
<td></td>
<td>• Dedicated section on website</td>
</tr>
<tr>
<td></td>
<td>• FAQs</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
| **Opportunities for input**    | • Single (if not an orphan/ultra-orphan/medicine for rare disease, and only if likely negative recommendation and company requests CAPIG adding time to the process) | • Multiple opportunities from scoping through to consultation on guidance  
• No difference according to type of medicine  
• Individual patient experts can participate | • Single (if not an orphan/ultra-orphan/end of life medicine, and only if likely negative recommendation and company requests PACE adding time to the process) |
| **Submission process**         | • Template                                 | • Template                                               | • Template                       |
|                                | • CAPIG submission                         | • Patient expert statement                               | • PACE submission                |
|                                | • CAPIG template                           |                                                          | • PACE template                  |
|                                | • CAPIG statement                          |                                                          | • PACE statement                 |
| **Involvement in decision making** | • Indirectly, via the representation made by lay members | • Indirectly, via the representation made by lay members and representation made by patient experts | • Indirectly via the representation made by Public Partners and PACE Chairperson |
| **Opportunity to appeal**      | ✗                                         | ✓                                                       | ✗                                 |

Notes:
CAPIG = Clinician and Patient Involvement Group  
FAQs = Frequently Asked Questions  
PACE = Patient and Clinician Engagement  
PIP = Public Involvement Programme

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Involvement of patients and patient organisations in HTA in the UK: patient organisation perspectives

The results should be read with caution reflecting the small sample (9) and limited experience of taking part in PACE.

However, it seems that PACE:
• requires little extra preparation for patient organisations;
• is an open approach, although that may be daunting for patients;
• is likely to be a smoother process where patient organisations plan ahead and engage with others who are participating;
• is actively and helpfully supported by SMC;
• is swift, with meetings taking an hour or an hour and a half, this is perceived as long enough to allow patient organisations to raise relevant issues – at the moment. More time could be needed if there were differences of opinion on the merits of a medicine in the future.

In addition:
• All those who participated would take part in PACE again; time and priorities permitting. One respondent summed up the PACE experience as a “breath of fresh air.”
• The roles of both the Chair and the lay member are important for the smooth running of the PACE meeting, but to also ensure that all relevant points are identified and carried forward to the next SMC discussion;
• PACE is perceived as being different to the ‘usual’ approach to involvement by virtue of it being a meeting ‘for the patient organisations and clinicians’, and because it focuses on producing a consensus based statement;
• Following a PACE meeting the PACE template, a document that has been filled in during the meeting, is shared. There is a further opportunity for patient organisations to comment on the content, although the fast turnaround times may limit actual opportunities for this to happen;
• No major gaps were perceived in terms of who should be attending meetings, at the moment. There is an outstanding question of the merits, or otherwise, of including industry members;
• It is unclear what impact PACE has on resulting decisions.

There is concern that at NICE the scale and tone of appraisal committee meetings can be ‘intimidating’, patient perspectives feel like they are left to last and economics can dominate.

Recommendations

There is no gold standard for patient and patient group involvement in HTA with agencies using different approaches. Cancer52 believes that all parties – including patient organisations - need to share experience, learn from each other, evaluate the effectiveness of their approaches to patient involvement and jointly develop best practices. We recommend that:

1. AWMG and SMC publish their evaluations of CAPIG and PACE in a timely fashion, so that others can learn from their experience.
2. A conference is organised to bring together HTA agencies and patient organisations to reflect on their experience of patient organisation involvement and to learn lessons from across the UK.
3. NICE should re-order their agenda for Appraisal Committee meetings to start first with the patient perspective, with the lead lay member presenting an overview, followed by an opportunity for the patient groups in attendance to correct any factual mistakes.

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Introduction

Health Technology Assessment (HTA) is defined as “a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. Its aim is to inform the formulation of safe, effective, health policies that are patient focused and seek to achieve best value” (EUnetHTA Undated).

HTA has been identified as a key policy tool that affects access to new cancer medicines (Jonsson 2013). In turn, access to cancer medicines can be one of a number of influences on patient outcomes (Karanikolos, Ellis, Coleman et al 2013, Munro 2014).

Objectives

Involvement of the public and patients in HTA is now increasingly being encouraged (Gagnon, Candas, Desmartis et al 2014, Barham 2012, Hanson, Draborg and Kristensen 2011). In light of this, Cancer52 wished to carry out a comparative study and generate a report considering how the views of patients and patient organisations are currently incorporated into Health Technology Assessment (HTA) systems in the UK. The study’s focus was on new models and their advantages and disadvantages (e.g. the Patient and Clinical Engagement (PACE) model from the Scottish Medicines Consortium (SMC)).

This report explores two main areas:

• How patients and patient organisations can become involved in HTA with the UK’s three agencies that influence access to new cancer medicines (the All Wales Medicines Strategy Group (AWMSG), the National Institute for Health and Care Excellence (NICE) and the Scottish Medicines Consortium (SMC)).

• The perceptions of nine individuals who have taken part in the newest approach of involving patient groups: the Patient and Clinician Engagement (PACE) process at the SMC.

Finally, it provides recommendations for changes to continue the evolution of bringing patient voices into the heart of decision-making across the NHS.
Context for patient and patient group involvement in HTA

Increasing the involvement of the public and patients in decision making in health was promoted as early as 1978 by organisations such as the World Health Organization (Lopes, Carter and Street 2015). However, the involvement of patients in decision-making remains controversial (Lopes, Carter and Street 2015).

It has been recognised that much of the focus of HTA during its establishment has been on the medical and economic implications of decisions. More recently, the importance of social and ethical implications are being recognised (Bombard, Abelson, Simeonov et al 2011, Whitty and Littlejohns 2015). Now public and patient involvement in HTA is being encouraged (Gagnon, Candas, Desmartis et al 2014, Hanson, Draborg and Kristensen 2011), albeit approached cautiously (Gauvin, Abelson, Giacomini et al 2011) and without consensus over which members of the public should be involved, or the respective rationales and benefits of public engagement (Kreis and Schmidt 2013).

Debate has included how far health professionals can represent patient views (Paterson 2011, Wong-Rieger 2011), as well as the concern that patient views may be seen as anecdotal and biased (Facey, Boivin, Gracia, et al 2010).

The benefits of involving patients and the public

Actively engaging all key stakeholder groups is considered one of fifteen principles for the improved conduct of health technology assessment (Drummond, Sanford Schwartz and Jonsson et al 2008). Patients are arguably implicit in achieving this.

There is a general view that the involvement of patients and the public is desirable in HTA (Menon and Stafinski 2011) and patients are considered to be able to offer an important contribution (Coulter 2004). Involvement of patients in HTA could:

• add key dimensions to the evaluation of technologies that might otherwise be overlooked (Gagnon, Desmartis, Lepage-Savary et al 2011);
• create a fair deliberative process (Facey, Boivin, Gracia, et al 2010);
• promote patient empowerment (Bridges and Jones 2007).

Such involvement does however require dedicated staff time (Royle and Oliver 2004).

Achieving meaningful involvement

There is not yet consensus on how to achieve meaningful involvement (Messina and Grainger 2012). Earlier research (Messina and Grainger 2012) has identified three key areas of advancement including:

• Industry could help bring the patient perspective into the HTA process through incorporating patient experiences early in the drug development process, and by including qualitative research on patient experiences in HTA dossiers;
• Recognising and supporting the role of patient advocacy groups and making use of their access to the genuine patient perspective and experience of living with the condition in question;
• Continuous development of HTA systems and processes to better facilitate involvement, increasing transparency and feedback, exploring new options for reaching patients and focusing on creating an active and informed health consumer.

Public involvement, such as in England’s National Institute of Health Research’s HTA program, has been described as uneven across the stages of HTA (Moran and Davidson 2011).

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1 Note that there is a rich literature on the many ways that the patient perspective may be captured through tools such as Patient Reported Outcome Measures (PROMs) that is outside the scope of this work

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It has also been argued that there is a considerable opportunity for further research into pragmatic, robust and meaningful approach to public engagement to strengthen HTA policy and decision-making frameworks (Whitty 2013).

There have been recent efforts to improve patient involvement in HTA through the development of values and quality standards for patient involvement (HTAi 2014). The values are:

- relevance;
- fairness;
- equity;
- legitimacy;
- capacity building.

In the context of rare and less common cancers, there can be particular challenges to providing the evidence desired by HTA agencies. Recognition of this has led to calls for increased public and patient involvement to inform these decisions (Douglas, Wilcos, Burgess et al 2015). In the context of orphan drugs, patients can provide information on the clinical benefits, to contribute to discussion of any uncertainties about this (Menon, Stafinski, Dunn and Short 2015).

Evaluating patient and public involvement

When agencies evaluate their own approaches to involvement, these remain unpublished, which has caused some concern (Barham 2011). However, there is some evaluation of patient involvement in HTA. For example, NICE conducted a patient expert survey in 2012 of those who had participated in Technology Appraisals, following up on an earlier survey in 2006/7 (NICE 2014). The report, published in 2014, found that:

- Patient expert experiences vary widely;
- Patient experts felt supported by PIP in their preparation for the Appraisal Committee, but felt they would like more help understanding their role at the Committee;
- Patient experts want more help completing their personal statement;
- The Committee Chair was key to the patient experts’ level of participation;
- There was a lack of clarity about the role of the lay member;
- Committee meetings were seen as daunting, with clinical and cost effectiveness overshadowing patient issues;
- Three quarters of the patient experts said their overall experience of committee meetings was excellent or good, although some felt their presence was tokenistic.

NICE uses exit surveys with patient experts, but the findings that NICE has presented do not separately identify any findings for the technology appraisal programme.

The Department of Health has recommended that NICE increase its profile, work more flexibility and further develop relationships, including with patients and their families. They have also recommended that NICE should make its approach to supporting patients more transparent, as well as identifying where it can provide more support (Department of Health 2015).

Despite interest in patient involvement in HTA, research is not clear on its impact. Early work published in 2003 suggested that patient organisation submissions to NICE can increase the likelihood of a routine rather than restricted use recommendation (Quennell 2003). However more recent research suggests that patient group submissions at NICE have had no significant effect on NICE decisions (Dakin, Devlin, Feng et al 2015).

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2 A report from the 2006/7 evaluation is not yet available from NICE. NICE states in their report that they expect to compare the results from the 2006/7 evaluation to the 2012 evaluation.

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Approach

A mixed methods approach was adopted, which included:

- A literature review using Pubmed and Google Scholar conducted on the 17 August 2015. The search terms used were ‘public and patient involvement’, ‘Health Technology Assessment’, ‘HTA’, ‘All Wales Medicines Strategy Group’, ‘National Institute for Health and Care Excellence’ and ‘Scottish Medicines Consortium’;
- A review of AWMSG, NICE, and SMC websites in August 2015, and key documents published by these agencies;
- Structured telephone interviews with Cancer52 members, conducted from 25 September to 18 November 2015.

Cancer52 conducted the recruitment for the structured telephone interviews using two main routes:

- Using information supplied by the SMC on the products subject to PACE up to 2 June 2015, which identified the Cancer52 members who were most likely to have participated. Cancer52 followed up by inviting individuals from these organisations to participate in this research;
- A general call for participation via email to all Cancer52 members.

In total, ten patient organisations took part in telephone interviews, one of which had not participated in a PACE meeting.

Cancer52 invited their members to participate. Therefore this research should be seen as an early and exploratory study of the perceptions of patient organisations who represent patients with rare and less common cancers on PACE. This should not be taken to represent the views of any other patient groups who have experience of PACE, especially as PACE is open to groups representing patients with other conditions. Nor should it be taken to be definitive, given the very limited experience of PACE of those who took part in the structured telephone interviews.

This research also focused on the direct opportunities for patient and patient organisation involvement in HTA. It could be argued that patients and patient organisations participate indirectly in the HTA process because patients participate in clinical trials and other forms of research that provide key data inputs into the HTA process. The appropriateness or otherwise of these forms of indirect involvement are not discussed here. In addition, this research did not focus on patient and patient organisation involvement in policy on HTA, although this can be important in shaping the environment as highlighted by respondent 7 during the telephone interviews. These areas are likely to be fruitful areas for further research.
Opportunities for the involvement of patients and patient organisations in HTA in the UK

The UK’s main HTA agencies differ in the opportunities they provide for involving of patients and patient organisations in the appraisal process. The following sections describe their approaches and Table 1 provides a comparison.

Throughout, there is a focus on orphan drugs as these are particularly relevant for the treatment of rare and less common cancers. From the 84 drugs licensed with a current orphan drug designation from the European Medicines Agency, there are 36 licensed in the antineoplastic and immunomodulating agent disease classification (as at July 2015) (Orphanet 2015).

All Wales Medicines Strategy Group (AWMSG)

The AWMSG provides advice to the Welsh Government Minister for Health and Social Services on new medicines (AWMSG 2015a). AWMSG will consider those new medicines not on the NICE work programme, or where NICE guidance is expected to take over 12 months following licensing. In the latter case, AWMSG provides interim guidance to the NHS in Wales (AWMSG 2015b).

Recommendations from AWMSG consider how well new medicines work, how cost effective they are and which patients would benefit the most (AWMSG 2015a). There are different criteria for orphan and ultra-orphan medicines and medicines for rare diseases, which may not have an orphan drug designation, and separate provisions for end of life medicines (set out in appendix 2).

The AWMSG finalised a Patient and Public Engagement Strategy in November 2014. This Strategy includes four priorities to improve patient and public engagement (AWMSG 2014):

- To raise public awareness of the work of the All Wales Therapeutics and Toxicology Centre (AWTTC) and AWMSG in promoting effective use of medicines across NHS Wales;
- To develop a better understanding of the issues affecting patients/carers and members of the public, and to provide clear information in an appropriate way;
- To facilitate input and enable everyone to have a voice in optimising medicine use; and
- To establish and strengthen effective relationships to maximise engagement.

AWMSG has a Patient and Public Interest Group (PAPIG). PAPIG has considered barriers to engagement with AMMSG and contributed to the Strategy (AWMSG 2015c).

AWMSG has a section of their website to provide information to patients, carers and patient organisations. This includes Frequently Asked Questions and contact information for staff (AWMSG 2015d).

Figure 1 outlines the opportunities for patient and patient group input into the appraisal process. The appraisal includes the new Clinician and Patient Involvement Group (CAPIG) process, introduced in September 2015. This change was the result of a broader review of appraising ultra-orphan and orphan medicines in Wales conducted in 2013 (AWMSG Undated).

CAPIG is only open to medicines that are considered as orphan/ultra-orphan or medicines for rare diseases and at the manufacturer's request, if the initial decision from the New Medicines Group (NMG) is that the medicine is not to be recommended.

The AWMSG has described the main purpose of CAPIG is to identify and discuss in more detail any additional benefits of the medicine from both a clinician and patient perspective. This is used to produce a CAPIG statement that is then presented to AWMSG. At CAPIG meetings issues such as how the medicine affects a patient’s quality of life and how it impacts on a patient’s family or carers are discussed. Discussion may include:
• the severity of the condition;
• any unmet need;
• the added value of the medicine for the patient;
• the patient’s family or carers;
• where in the patient pathway the medicine could most appropriately be used;
• specific patient groups that may benefit more from the use of the medicine;
• any important considerations in relation to treatment delivery.

AWMSG states that CAPIG can discuss (AWMSG Undated):
• The ability to continue work or education;
• The management of symptoms such as: pain and extreme tiredness;
• Helping relieve psychological distress;
• Convenience of how and where the treatment is received; and
• The ability to self-care or maintain independence and dignity.

Attendees at the CAPIG meeting include:
• Chairman;
• AWMSG Patient and Public Interest Group (PAPIG) representative;
• Patient organisation/patient support group representatives;
• Clinical experts (maximum of 3 - normally nominated by the specialist advisory group);
• Public/Lay representative (normally nominated by Community Health Councils);
• AWMSG Lay member and/or deputy;
• Applicant company representative in non-voting capacity (they can present a short statement at the meeting and may input into discussions, but will leave the meeting before any voting or completion of the CAPIG report and final statement);
• AWTTTC Appraisal Lead (non-voting capacity);
• AWTTTC Administrator/Medical Writer (non-voting capacity); and
• AWTTTC Liaison Manager (non-voting capacity).

Everyone attending must declare any conflicts of interest and sign a confidentiality agreement, as details of the discussion at CAPIG meetings is kept confidential.

All of those involved in CAPIG have a month to prepare, with submissions from patient organisations and clinicians due to take part in CAPIG, due to AWMSG two weeks before the CAPIG meetings.

The AWMSG and New Medicines Group (NMG) include lay members whose role is to comment from a patient perspective (Bracchi Undated).
Figure 1: AWMG appraisal process and opportunities for patient and patient group input

Key points to note from the flow chart include:
- The single opportunity for patients and patient organisations to input into the process with the exception of CAPIG in the case of orphan, ultra-orphan and medicines for rare diseases;
- CAPIG involvement occurs at the company's request and can add a further 8 to 12 weeks to the process.

Key:
Dark grey box - opportunity for patient and patient organisation involvement
Light grey boxes - possible opportunity for patient organisation involvement in the case of orphan, ultra-orphan or medicines for rare diseases
Note: (not shown) AWMG provides a questionnaire for input six weeks before the NMG meeting or comments can be made online.

Source: Author drawing upon the process outlined in AWMG Appraisal Process: Overview and AWMG A flow chart outlining the appraisal principles and process and AWMG Process for appraising orphan and ultra-orphan medicines and medicines developed specifically for rare diseases Effective from September 2015

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National Institute for Health and Care Excellence (NICE)

NICE undertakes assessment and appraisal of medicines referred to them by Ministers (Department of Health 2015). In future, NICE may become more influential on access to cancer medicines as it could become the single expert body for appraisals, taking on appraisals in the Cancer Drugs Fund (CDF) from April 2016 (Department of Health 2015). NHS England and NICE began a consultation on a reformed CDF on 19 November 2015, with proposals for a joint process and ‘managed access’ (NHS England 2015).

Recommendations from NICE consider (Diaby, Goeree, Hoch et al 2015):
• severity of illness;
• end of life (criteria are set out in appendix 3);
• stakeholder persuasion;
• significant innovation;
• disadvantaged populations;
• children;
• cost effectiveness.

NICE Appraisal Committees include members drawn from patient and carer organisations (NICE 2015a). The Appraisal Committee member lists identify lay people, however, it is not always clear which of these are from patient and carer organisations. NICE stress that all committee members are recruited as individuals, and not as representatives of organisations (NICE, personal communication 2015).

NICE’s approach to public and patient involvement is underpinned by their Public Involvement Policy (NICE 2013a). The policy includes two key principles:
• “that lay people, and organizations representing their interests, have opportunities to contribute to developing NICE guidance, advice and quality standards, and support their implementation; and
• that, because of this contribution, our guidance and other products have a greater focus and relevance for the people most directly affected by our recommendations.”

NICE also has a Public Involvement Programme (PIP). The PIP team develops and supports patient, carer and public involvement (NICE 2015b). PIP also works with a collaboration of patient groups, Patients Involved in NICE (PIN). NICE published their first Public Involvement Programme Annual Report in 2015 (NICE 2015c). This outlines the work of the team to support lay involvement across the work of NICE, including outreach, training and support.

NICE’s Method Guide encourages submissions that provide patient and carer perspectives on (NICE 2013b):
1. The experience of having the condition/caring for someone with the condition;
2. The experience of receiving care;
3. The experience of having specific treatments;
4. The outcomes of treatment that are important to patients and carers. This recognizes that these may be different to those measured in clinical studies and those in generic measures of health related quality of life;
5. The acceptability of different treatments and modes of treatment;
6. Preferences for different treatments and modes of treatment; and
7. Expectations about the risks and benefit of the technology.

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3 The Cancer Drugs Fund is a separate fund for cancer medications that are rejected by NICE, are awaiting a NICE recommendation or will not be appraised by NICE. The CDF only exists in England.
4 Based on review of membership of Committee A which identifies 3 lay members none of which appear to be from patient organisations either currently or in the past based on information hosted on the NICE website, review of membership of Committee B which identifies 2 lay members, 1 of which used to work for a patient organisation, review of membership of Committee C which identifies 2 lay members, 1 of which currently works for a patient organisation and 1 of which used to work for a patient organisation, and review of membership of Committee D which identifies 2 lay members, 1 of which used to work for a patient organisation.
The guide states that: “the information is best taken directly from people with the condition (or their family or carers) in the form of written accounts of their experiences and points of view. Narrative summaries, preferably with illustrative quotes, addressing the issues listed…..are acceptable.” The guide also suggests that qualitative research techniques can facilitate the synthesis of this type of evidence, but that this is not required for submissions. This appears to be a marked change to the previous guide, which stated that “Patient evidence is most useful when presented as a synthesis of information, balancing positive and negative views, rather than a series of individual testimonials”.

NICE has three types of appraisal: Single Technology Appraisals (STAs) which consider a single technology, Multiple Technology Appraisals (MTAs) which consider a number of technologies and Highly Specialised Technology (HST) appraisals. This report focuses on the STA, as this is the most common approach from NICE, and is closest to the approach of the AWMSG and the SMC. The following figure illustrates the STA process with annotations highlighting the points at which patient organisations can contribute to the appraisal.
Figure 2: NICE STA appraisal process and opportunities for patient and patient group input

Key points to note from the flow chart include:
- The multiple opportunities for patients and patient organisations to participate in the process; and
- The same opportunities for patients and patient organisations to participate in the process irrespective of the type of medicine under consideration; and
- There is an opportunity for consultees (which include patient organisations) to appeal NICE recommendations.

Key:
Dark grey boxes - opportunity for patient and patient organisation involvement

Notes: (not shown)
- NICE provides opportunities for involvement in the scoping process before the appraisal starts and provides a template for input. Consultees include commissioners, health professional groups, manufacturer and national patient and carer organisations.
- Commentators can comment but unlike consultees, cannot appeal the NICE FAD.
- Upheld appeals go back to an AC meeting as presented in NICE’s own flowchart, however they can also go to any point in the process to be looked at again (NICE, personal communication 2015).
- NICE also includes a scoping phase which precedes the appraisal process. This stage is seen by both respondent 6 and 7 in the telephone interviews as particularly important, as scoping can either permit or exclude key issues from the patient perspective.
- NICE appraisal takes around 9 months to complete (NICE 2013b).

Source: Author adapted from NICE guide to the processes of technology appraisal 2013

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Scottish Medicines Consortium (SMC)

The SMC assesses all new medicines in Scotland. It considers the effectiveness of the medicine, which patients would benefit, whether the medicine is as good, or better, than others that the NHS already uses to treat the particular condition, the cost and the value for money (NHS Scotland 2012). SMC may also apply ‘modifiers’ which allow greater flexibility in decision-making. Modifiers are set out in appendix 4.

SMC has a public involvement team who proactively engage with patient groups for every medicine being assessed by the SMC. They help gather patient group submissions and provide a dedicated section on the SMC website (SMC Undateda). Their work sits within the broader strategy for public involvement set out for Health Improvement Scotland, of which SMC is a part (SMC, personal communication 2015). The SMC is committed to meeting the principles laid out by the HTAi subgroup for patient involvement in HTA (SMC Undatedb).

Included in the SMC appraisal process is the relatively new Patient and Clinical Engagement (PACE) process. This was introduced in 2014 as part of a wider review of access to new medicines in Scotland, undertaken in 2013 (SMC Undatedc).

The PACE process is open only for medicines that are considered as end of life or orphan/ultra-orphan medicines and at the manufacturers request, if the initial decision from the New Drugs Committee (NDQ) is that the medicine should not be recommend.

The SMC has described the main purpose of PACE as gathering detailed information that will allow a discussion on a medicine, including how it can impact on a patient’s quality of life. Companies, clinical experts and patient groups can provide a PACE submission on their perspectives on the new medicine.

The main part of PACE is a meeting to discuss the benefits of a medicine, including how it can impact on quality of life, as this may not always be fully captured within the conventional assessment process using a standard set of questions. PACE can explore issues such as (SMC Undated):

- The ability to continue work or education;
- The management of symptoms such as: pain and extreme tiredness;
- Helping relieve psychological distress;
- Convenience of how and where the treatment is received; and
- The ability to self-care or maintain independence and dignity.

Attendees at the PACE meeting include:
- Expert advisers representing the patient and carer voice (nominated by Scottish Cancer Coalition, Rare Diseases UK or Genetic Alliance – up to three representatives per meeting)5;
- Clinical expert advisors (nominated by clinical networks - up to three representatives per meeting);
- SMC Public Partner;
- SMC Public Involvement Team member; and
- SMC New Drugs Committee member.

Everyone attending must declare any conflicts of interest and sign a confidentiality agreement, as details of the discussion at PACE meetings is kept confidential.

Companies do not attend meetings, but are able to send a short statement to be considered at the meeting.

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5 Note that this is the formal description of the process from SMC, although those who took part in interviews suggest that this requirement for nomination is no longer part of the PACE process.
All those involved in the PACE process have a month to prepare, with submissions to provide the patient organisation and clinician perspectives, due to SMC two weeks before the PACE meetings.

As of 2 June 2015, SMC has held 25 PACE meetings. Of those, the final SMC finding has been (SMC, personal communication 2015):

- recommended for 9 (36%),
- restricted recommended for 8 (32%),
- not recommended for 7 (28%) and
- one final decision pending product availability.

Just under two thirds (16/25) of PACE meetings held to June 2015 have been for medicines to treat rare cancers. Fifty-three patient organisations had participated in PACE meetings up to June 2015 (SMC, personal communication 2015).

The SMC includes Public Partners whose role is to help ensure the views of patients, carers and members of the public are taken into account during SMC decision-making (SMC Undatedc). They are also members of the Public Involvement Network Advisory Group (PINAG) who help SMC continuously improve how SMC involves patients, carers and members of the public in their work (SMC Undatedb).

Figure 3 sets out the appraisal process used by SMC and the opportunities for patient and patient group input.
Figure 3: SMC appraisal process and opportunities for patient and patient group input

Key points to note from the flow chart include:
- The single opportunity for patients and patient organisations to input into the process, with the exception of PACE in the case of end of life or orphan and ultra-orphan drugs;
- PACE occurs at the company's request;
- PACE can add a further 4 to 12 weeks to the process.

Key
Dark grey box - opportunity for patient and patient organisation involvement
Light grey boxes - possible opportunity for patient organisation involvement in the case of end of life, orphan or ultra-orphan medicines
Note: (not shown) SMC provides a template for input and guidance for submissions.

Source: Author adapted from SMC Assessment Process Flowchart and SMC PACE Overview Document
# Comparison of opportunities for patient and patient organisation involvement across UK HTA agencies

The table below compares and contrasts the opportunities for patient and patient group organisation involvement in the three HTA agencies in the UK.

## Table 1: Summary of opportunities for patient and patient group organisation involvement across UK agencies

<table>
<thead>
<tr>
<th>Agency</th>
<th>AWMSG</th>
<th>NICE (STA)</th>
<th>SMC</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Strategy/Policy in place?</strong></td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td><strong>Support</strong></td>
<td>AWMSG staff available to support Dedicated section on website FAQs</td>
<td>PIP programme with dedicated staff providing outreach, training and support</td>
<td>Public involvement team with dedicated staff proving outreach, training and support Dedicated section on website</td>
</tr>
<tr>
<td><strong>Opportunities for input</strong></td>
<td>Single if not an orphan/ultra-orphan/medicine for rare disease and only if likely negative recommendation and company requests CAPIG adding time to the process</td>
<td>Multiple opportunities from scoping through to consultation on guidance No difference according to type of medicine Individual patient experts can participate</td>
<td>Single if not an orphan/ultra-orphan/end of life medicine and only if likely negative recommendation and company requests PACE adding time to the process</td>
</tr>
<tr>
<td><strong>Submission process</strong></td>
<td>Template CAPIG submission CAPIG submission CAPIG statement</td>
<td>Template Patient expert statement</td>
<td>Template PACE submission PACE template PACE statement</td>
</tr>
<tr>
<td><strong>Involvement in decision making</strong></td>
<td>Indirectly via the representation made by lay members</td>
<td>Indirectly via the representation made by lay members and representation made by patient experts</td>
<td>Indirectly via the representation made by Public Partners and PACE Chairperson</td>
</tr>
<tr>
<td><strong>Opportunity to appeal</strong></td>
<td>✗</td>
<td>✓</td>
<td>✗</td>
</tr>
</tbody>
</table>

Notes: AWMSG = All Wales Medicines Strategy Group CAPIG = Clinician and Patient Involvement Group FAQs = Frequently Asked Questions PACE = Patient and Clinician Engagement PIP = Public Involvement Programme

The table illustrates the similarities and differences between agencies in the way that they approach opportunities for patient and patient group involvement in appraisals.

Key similarities include:
- Providing opportunities for participation – no agency excludes the involvement of patients and patient organisations;
- Providing support to encourage patient and patient group participation; and
- Providing opportunities for written submissions.

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Key differences include:
- The opportunity for individual patient experts to take part in the appraisal process at NICE and at SMC (subject to support from a patient organisation), this does not appear to be permitted at AWMSG;
- A differential approach to patient organisation involvement, according to the type of medicine as seen with CAPIG and PACE at SMC. No such differentiation in approach is available at NICE;
- The opportunity to develop a consensus on the benefits to patients from selected new medicines, through a deliberative discussion with patient organisations and clinicians (CAPIG and PACE respectively for AWMSG and SMC). This results in an additional written input to later committee discussions at both AWMSG and SMC. No such opportunity is available at NICE. However, the precise approach differs between AWMSG and SMC, with this option available for different types of medicines (orphan/ultra-orphan and medicines for rare diseases at AWMSG versus orphan and end of life medicines at SMC). AWMSG also permits limited company involvement in CAPIG meetings, whereas SMC does not; and
- There is no opportunity for patients or patient organisations to appeal recommendations made at AWMSG or SMC, whereas this is an option at NICE.
Involvement of patients and patient organisations in HTA in the UK: the views of patient organisations

Extent of involvement in the PACE process

From the nine individuals who had been involved in PACE meetings with the SMC, four had attended two PACE meetings and five had attended one PACE meeting.

Preparation for PACE

Preparation for the PACE process, over and above that for the ‘usual’ patient group submission, included the preparation and submission of the patient organisation PACE template. The template is very open; it consists of a first page asking for the name of the patient group/organisation, the name of the person providing the statement, the name of the medicine, SMC reference number and the date of the scheduled PACE meeting. The first page also includes guidance for what to include. The second page is a text box with the instruction not to exceed two pages. Respondents often undertook outreach work to gather patient views about the new drug.

Patient group views on preparation

The openness of the template, and the meeting itself, is seen both as an advantage and disadvantage. For example, respondent one highlighted that, “the very informality of [PACE] and the lack of structure within it, was a shock. I really didn’t know what they wanted to hear.” At the same time, this allows patient organisations to participate in an “open, flexible, deliberative process.” Respondent three supported the openness of the PACE template, noting that it “feels much more holistic.”

Respondent five suggested that the “quite small request form with free form answers didn’t steer you or focus you”. This was however, “quite concerning, and made us suspicious.” This respondent was particularly concerned that it was a tick box exercise, yet having participated in the meeting he was “pleasantly surprised.” He also noted that whilst the template, “allows you to select what you think is important [to include],” that “some organisations won’t necessarily have the skills, knowledge and resources [to fill the template in]” and that this could then mean it would be “daunting.”

The approach though does have appeal to some, as “it’s very straightforward” according to respondent eight. Respondent ten said “I really like the form. It’s the only place in any HTA where there is a blank box”, which he describes as allowing “[patient organisations] to tell a story, - you can put the information you feel is relevant rather than what [HTA agencies] tell you we need to know from you.”

Some respondents pointed out that there were similarities between their PACE submission and their original submission to the SMC process. Respondent two said, “[our submission] was pretty much the same as before. We did add more quotes and a couple of stats.” However the flexibility of the PACE template did allow his organisation to, “lauy our case as we wanted to, rather than be constrained by the [original submission] template. That was really useful.” Respondent five said that the “submission ahead of PACE, involved us being able to re-emphasise key points and strengthen our [original] submission.” Respondent nine pointed out that the form for PACE is not compulsory, so organisations do not need to fill it in if they feel that their original submission to SMC captures all the relevant points.

Respondent two suggested that the patient group PACE template had, by virtue of the guidance on the first page, prompted identification of “issues we hadn’t thought of for our NICE submission.”
Preparation for the PACE process may also include interaction with others who will be at the meeting outside of the SMC. For example, respondent two said that their organisation had prepared a joint PACE submission with another patient organisation. They had also held discussions with the patient organisation and clinicians in advance to discuss how best to approach the meeting. Respondent five, eight and nine noted that their patient organisations had agreed in advance with the other patient organisation how to approach the meeting. They had agreed who would go first and how best to complement each other’s points.

Respondent three noted that ahead of the PACE meeting all participants were able to see the submissions from others.

Patient group views on support for participation

Those who had participated in PACE suggested that the support offered by SMC was helpful. For example, respondent one said that, “I’d been in contact with the SMC liaison who helpfully explained what they were looking for [from a patient organisation] in a PACE meeting”. Respondent two echoed the same theme, stating that “SMC were very helpful.” He went on to say that, “SMC were aware that it was a new procedure, and they were clear that they would be on call to help us with any questions or queries.” Respondent three, five, six and seven echoed the same themes.

Respondent five also noted that, “[SMC] ensure that you understand the process and from day one emphasise that they are not looking for scientific or clinical input.” Perhaps most strikingly, he also said that, “there were no barriers, we felt wanted.”

Participants noted that support had included a pack on the PACE process.

Patient group views on the PACE meeting

Respondent one highlighted the important role of the Chair in the PACE meeting. He described how the “second PACE meeting was very different to the first, and this was due to the Chair. The Chair sets the tone and character of what happens within the [framework of the] PACE meeting”. Similarly respondent six said that, “the second [PACE] meeting was much better because of a different Chair.”

Respondent three said that his experience was of a “well chaired” meeting, with “sensitive chairing covering a list of questions that they wanted to cover.” Respondent four also noted that, “the meetings are well run.” However, respondent four also noted that there had been one PACE meeting where, “one consultant basically took over the call”, which then made it difficult for “others to have their say.” “This could have been resolved via the Chair”.

Respondent six cited her experience that in one PACE meeting the Chair did not start the meeting for 20 minutes because an attendee was late joining, and this effectively resulted in the loss of time for discussion, as the meeting still ended at the appointed time. She felt the Chair could have taken the decision to start, despite the absence of a patient organisation participant.

Respondents described the flow of the meeting as starting first with introductions from the Chair, followed by the Chair inviting verbal statements first from the patient organisations, then the clinicians, before opening up for a wider conversation which allowed questions of clarification to be tabled from the SMC side and a discussion of the issues which were seen to be particularly important. All of this is structured around the PACE template which needs to be completed during the meeting and finalised afterwards.
Respondent two suggested that the PACE meeting itself was, “informal” and “conversational.” The meeting allowed him to, “highlight issues that we wanted to. We could also go back to particular points.” He pointed out that, “it’s a positive process, [the SMC] want to get as much information as possible, and they help to draw this out.” He went on to say that, “there was no interrogation, no challenge on points, so all of the time was given to patient groups and clinicians and as we had talked to the clinician in advance, all went well. That meeting was given to us to use as we wanted.”

Respondent three picks up on this theme and elaborates that, “it [PACE meeting], seemed genuinely collaborative.” Respondent five elaborates on the theme of collaboration. He said that, “what made PACE totally unique was at the head of the meeting there is a blank template that has to be completed and summarised together. Rather than individual submissions, we needed to work together with the SMC to agree the content to go into the PACE template.” Further he says that, “this was the first time as a patient group that we’ve been actively involved in the process.” Respondent eight noted that the meeting “was not SMC dominated.” Respondent nine described the meeting as “a listening meeting at the start”, where “you can say whatever you want, no-one interrupts you, and then you have the ability to comment on what doctors say and participate in discussion. You do feel included.”

Respondent four highlighted that the PACE meeting allowed the patient organisation to, “bring in case studies.” For this to be possible and effective, the patient group representative at the meeting, must have genuine insight into the experience of patients, for example by being a patient themselves or by working regularly with patients in clinic, or both.

Different viewpoints were offered on the time available for PACE meetings. Respondent one said that, “it’s literally an hour and you’re out before the hour is up. Yet it feels like you’ve had a free hand to say what you want, to ask questions, and there’s no feeling at all that you can’t intervene when you wish too.” He described the PACE meeting as, “a breath of fresh air.” Respondent three also said that the meeting was “relatively short, not more than an hour and a half”, but that “it didn’t feel rushed.”

Respondent two felt that there was sufficient time given to patient groups in the meeting. However, he also said that he could envision a scenario, particularly where patient groups and clinicians had not discussed issues ahead of the meeting and where there may be less of a consensus, where the meeting might not be long enough. Respondent six suggested that, “it is really hard to keep [PACE meetings] to an hour”, implying that it may need to longer. Respondent ten felt that “[an hour] probably wasn’t long enough,” noting that “the first 45 minutes were very good, we were talking productively,” but that towards the end of the meeting they were asked “to get firm ideas on paper.” He suggested that perhaps two hours might be better, although acknowledged that it can be hard for clinicians to find time to participate. This could be more challenging if meetings were longer.

Patient group views on PACE participants

Different views were expressed on whether all relevant parties were able to participate in PACE. For respondent one, it was seen as a strength that PACE is just for clinicians and patients. He said, “Constituents such as the pharmaceutical company have had their go. [PACE] gives clinicians and patients our turn.” This is particularly because, “[PACE] is going beyond the clinical trials and cost effectiveness and into real world [use].”

Respondent nine noted that “industry do want to be in there, because it a discussion about their drug”, however, “if industry were in there, it would lead to a different type of discussion,” although the respondent did not imply any judgment as to whether this would be good, or bad. Respondent ten thought that industry participation in meetings was a “question that needs answering”, but that they had not “come to a firm conclusion.”
Respondent five believed that including clinicians helped as they can be “allies in the appraisal process.” Respondent three suggested that in respect of having all relevant parties at the PACE meeting, “it was probably about right.” However, both Respondent two, three and six suggested that there might be an opportunity to involve patients directly as well. Respondent nine noted however that “SMC doesn’t have much capacity to support involvement of individual patients.” Individual patients can participate if they are supported by patient organisations (SMC, personal communication 2015).

Respondent four believed that clinical nurse specialists (CNS) could add insights, reflecting their experience of “seeing patients every day.” Respondent eight, nine and ten agreed that a CNS might add a useful perspective. Clinical nurse specialists can participate if they wish to, as clinical experts (SMC, personal communication 2015).

Respondent one described his opportunity, as a patient organisation representative in PACE to, “try to describe what it is like to live with the drug for long period over time. For example, what appear to be minor side effects, can, over time become significant. Fatigue experienced for a decade results in a major impact.”

Participants also noted the use of technology to enable participation for those who cannot attend a meeting in Scotland in person. For example, respondent one said, “[SMC] take very seriously video conferencing”. The majority of respondents had participated by phone, rather than in person.

**Patient group views on the output of the PACE meeting: The consensus PACE statement**

Of those participants who had been able to review the filled in PACE template, they were content with the output of the PACE process itself. For example, respondent one said, “I was impressed by the returned template, I thought [the SMC] had got the essentials of what the patient groups and the clinicians were saying, and the weave was good between patient groups and clinicians”. Further, he suggested that this implied that, “[we’d been] listened to seriously.”

Respondent three noted that he didn’t have comments on the statement, because it was “as discussed”. Respondent two echoed this theme, saying that he had been concerned that perhaps the nuances of the discussion at the meeting would not necessarily come through in the later statement. However, he said that, “we were lucky that the SMC staff member, who summarized, was fantastic. I got the feeling that he saw it as his duty to faithfully represent what was said at the PACE meeting at the subsequent SMC meeting.” His concern was that this might not be the case for every SMC staff member. Respondent eight noted that “[I didn’t need to add anything in” because the statement picked up on what was discussed at the meeting.

The PACE statement can also be refined as a result of sharing a draft after the meeting itself. Respondent one noted that, “changes were made as a result [of changes asked for by the patient groups]”.

The speed at which the filled in template is provided to participants of the PACE process, and a limited time to respond (identified by respondents as a maximum of 48 hours) elicited mixed views. Respondent one suggested that, “speed is of essence, [we’ve] got to try to keep to being timely.” However, respondent six said that she simply had not had the opportunity to open and read them because of her part-time working pattern and the speed at which SMC request feedback. Respondent ten noted that the “timelines for the statement were tight. A week for feedback might strike a balance between opportunity for review, yet still trying to keep the process moving” SMC has clarified that as PACE meetings are held on Tuesdays, draft statements are typically sent out at close of business on Wednesday, or first thing Thursday, with a request for response by the following Monday. This is four calendar days, or two business days. This timeline is to ensure that the statement can be included for the subsequent SMC meeting (SMC, personal communication 2015).

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Post PACE meeting

Following the PACE meeting, respondents described being advised on the date of the next SMC meeting where the drug will be considered as well as the sharing of the PACE template for comment (as described above).

Patient group views on the SMC meeting, post PACE process

Most respondents did not attend the subsequent SMC meeting where the drug was considered, alongside the PACE consensus statement. For some, this is a reflection of the practicalities of attending a meeting in Scotland, as well as a competing priorities for their time (as described by respondents one, two, six and eight). Respondent four’s response suggested that they did not know that they could attend the meeting.

Respondent five’s colleagues did attend the subsequent SMC meeting. He suggested that, in light of feedback he received, “the evidence from the PACE process was properly presented to the Committee.” He highlighted that this was in large part because of the SMC member of staff, and how they approached feeding back from PACE.

Patient group views on the influence of PACE on the resulting SMC decision

Respondents held mixed views on the impact of PACE on the resulting SMC decision (where final recommendations were available). Respondent eight said he had the “perception that if [the patient organisation and clinicians] make a good case, [the drug] will get through”. Respondent two said that, “I was confident, because of the SMC staff member, that [the PACE statement] would be given due weight”. His one experience of the PACE process also resulted in a positive recommendation. He noted that this in part, reflects wider changes. He said that, “having read the policy documents from the Scottish Parliament and the Ministerial response, it is clear that Ministers are saying [to SMC], go ahead and spend more money.”

Respondent five said that, “the outcome [of PACE and the subsequent SMC recommendation] doesn’t shape our view of PACE. I believe that our inputs were properly collected, in a non-threatening and inclusive way, taken seriously and we could see how evidence was used appropriately.”

Respondent four believed that, “the decision is already made before PACE.” This reflects the focus on cost in the wider SMC decision-making process. Respondent six said that she could not identify the influence of PACE, because “I can’t see any case where we can counter the cost and economic decision”, however she recognised that, “where it is borderline [on cost effectiveness] PACE could maybe make a difference.”

Patient group willingness to participate in SMC in the future

All participants said that they would take part in the PACE process again in the future, should the situation arise. However, it is clear that patient organisations need to consider capacity and priorities. For example, respondent three noted that his patient organisation had been invited to a PACE meeting in August but no-one was able to attend.

Respondent six said she questioned whether the PACE meeting is adding anything to the PACE submission, but acknowledged that longer meetings could help to tease out the added value of patient organisation inputs to the SMC process. Respondent nine found PACE “useful” but noted that it is an “add on to the SMC process”, which reflects the broader political desire for reform that required SMC to change. Respondent nine went on to ask, “why isn’t this done for all diseases?”
Patient group views on the future of the PACE process

Respondents highlighted different issues that may affect the future success of the PACE process. One of these was the trend towards small patient populations as research identifies patient subgroups within specific cancers. Respondent one said, “as the patient population gets smaller and smaller, it is harder to find patients who have taken the drug. Much of patient involvement rests on the assumption that there are many patients whose experience can be drawn upon.”

Respondent two suggested that there could be a problem in future if there are too many requests for PACE. He could see that SMC might struggle to accommodate all of these.

Patient group views on involvement with the different HTA agencies across the UK

Although limited time prevented a full discussion with participants on how patient group involvement compares across agencies, respondents did highlight a number of differences between NICE and the SMC. Respondents generally had much less experience with AWMSG compared to both NICE and SMC.

Respondent two highlighted that support from NICE to support patient organisation involvement is “helpful”, but that “the problem [at NICE] comes at the committee stage.” Respondent five elaborated, suggesting that, “there is too much technical information”. He also suggested that the scale and tone of NICE meeting is “intimidating.” Respondent six used this same phrase, saying that, “at the NICE [Appraisal Committee] meeting there can be 20 or more people around the table.”

Respondent nine described NICE meetings as having “a different atmosphere” and that both SMC and AWMSG feel more “flexible in their approach.” Respondent eight contrasted SMC’s PACE process with other agencies, pointing out that at SMC, the best feature is “simplicity”, this avoids “falling down a rabbit hole, with complicated clinical data, patient pathways and combinations of therapies.” NICE he said, needed to “be simpler, less time consuming.”

There was a perception amongst some that patient perspectives may be left to last with NICE. Respondent one suggested that PACE at SMC, “as a meeting versus a written submission, gives [patient organisations] more of a chance of saying things and getting our points across”. In contrast, he describes the process at NICE as “taking so long, and the patient group bit is last, so is when the Appraisal Committee is running out of time.”

There is a perception amongst some respondents of the dominance of the clinical and economic issues at NICE. Respondent two suggested that, “NICE base decisions on cost and clinical effectiveness. The third rail is patient engagement, but the train doesn’t touch the third rail. There’s very little we could give [NICE] that would change their decision.”

Respondent one explained that watching NICE Appraisal Committee meetings felt to him like, “arguments between economists”. Respondent six suggested that at NICE, it can “feel like you have to assert yourself in [Appraisal Committee] meetings.” She went on to say, “my experience is that the discussion is dominated by economics and the data.” Respondent 10 said that, “NICE committee meetings are very focused on cost effectiveness,” and went on to say that, “it’s hard to comment on economics when you don’t have expertise in health economics.”

The experience of patient groups can also affect their intentions for involvement in the future. Respondent two said that his organisation would, “not go to such extent [to engage with NICE] in the future.” He cites the example of identifying and encouraging a patient expert to participate at NICE, where one patient died, one patient was too ill to go, but even when there is a patient willing and able to participate, “why put a patient through that [the Appraisal Committee meeting] if they won’t be listened to?”

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Chairs were identified as an important difference between NICE and SMC. Respondent five said that chairing at NICE is variable, with one Chair in particular being perceived as dictatorial. Engagement with patient groups and clinicians can also feel cursory. That contrasts to SMC where, the Chair was “extremely engaging and friendly.” Respondent six suggests that, “the Chair can make or break how the patient organisation representative, or the patient expert themselves, can participate in the process.” Respondent seven cited an example where the Chair had insulted a patient expert.

Lay representatives were identified as an important difference between NICE and SMC. Respondent two said that, “the lay member [at the first SMC meeting] was incredibly good. He went out of his way to draw out all the points [made by patient organisations].” This contrasts to his NICE experience where NICE “presents three slides [on the patient perspective].” He suggests that the lay member at NICE, “didn’t go to the same extent to summarise the patient group submission [as the lay member at SMC].” Further, the lay member “misquoted [the patient organisation]” and there is “no right to reply.” He also acknowledged that his positive experience at SMC may reflect the particular lay member and that there may be variation between lay members. Respondent six said that in her experience with NICE, she has, “never been able to identify who that [lay member] was at the meeting. I have never seen them actively participate.”

The SMC process was seen by some as more flexible than NICE. For example, respondent two said that, “at NICE, the algorithm says no”. Using PACE at SMC allowed his patient organisation to “explain why the condition is exceptional”. Respondent eight pointed out that, “a barrier to [patient group] involvement in HTA is because it’s hugely technical, time consuming and involves health care economics and clinical information…PACE is the easiest way [for patient organisations] to get involved [in HTA].”

At the same time, there is recognition of trade-offs in the approaches taken by the different agencies. As respondent one noted, “NICE is thorough, but the process takes a long time, versus the SMC whose process is faster, but perhaps perceived as less thorough”.

Respondent ten arguably sums it up best by noting that, “there are different bits which are good [in how patients and patient organisations are involved across agencies], if you took the best bits from each one, you could get quite a good system.”
Conclusions and Recommendations

It is clear that patient organisation involvement is being taken seriously by all HTA agencies that we’ve looked at in this report.

There is evidence of the support offered and increasingly there is information in the public domain from agencies like NICE that relates to public and patient involvement, for example, the first annual report in 2015 and their evaluation of patient expert experience of technology appraisals in 2012. However, the literature review and telephone interviews have highlighted that there is no current consensus on the best way to involve patients and patient organisations in HTA. Agencies are exploring new approaches, as illustrated by PACE and CAPIG in the case of orphan and other medicines.

There are plans to evaluate experience of PACE and CAPIG. We hope that this report can make an early – if tentative – contribution to the evaluations that SMC and AWMSG undertake in the future. We recommend that AWMSG and SMC publish these evaluations in a timely fashion so that others can learn from their experience.

We believe that there will be useful opportunities to learn by comparing and contrasting approaches. Therefore, a conference to bring together HTA agencies and patient organisations, to reflect on their experience of patient organisation involvement and to learn lessons from across the UK, would be one way to contribute towards this.

Given the limitations of this work and no other evaluation available to date, it’s too early to advocate wholesale change to NICE’s approach. However, as an interim step NICE should re-order their agenda for Appraisal Committee meetings to start first with the patient perspective, with the lead lay member presenting an overview followed by an opportunity for the attending patient groups to correct any factual mistakes.

Cancer52 has previously called for NICE to commission a rapid review style, independent evaluation to explore the representativeness, role and performance of lay representatives. We repeat that call here.

Finally, there is a need for much wider research. This should explore:

- The potential to draw on HTAi standards and values to formulate an evaluation framework for patient and patient organisation involvement in HTA;
- The development of Key Performance Indicators for patient and patient group involvement in HTA, to be incorporated into annual reports by the HTA agencies on patient and patient involvement;
- The acceptable trade-offs between patient and patient organisation involvement and timeliness of decision making.

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About Cancer52

Cancer52 is a coalition of more than 80 cancer charities representing rare and less common cancers, which account for more than half of all cancer deaths in the UK. We campaign and work on issues and policies that impact on the rare and less common cancer community, including improving diagnosis, treatment and support.

Current data shows that 46% of cancers diagnosed are rare and less common cancers, yet they account for 54% of cancer deaths.
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- Sharon Tate, Head of Primary Care Development, Target Ovarian Cancer
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- Roger Wilson CBE, Cancer Patient Advocate
- Nick York, Trustee, CLL Support Association

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Leela Barham, an independent health economist and policy expert, drafted the report drawing on research she conducted between August and November 2015. The final report is however a Cancer52 report and reflects the views of Cancer52 and not necessarily the views of Leela Barham.

We would also like to thank staff at NICE and SMC for their comments on an early draft. Their participation does not necessarily imply agreement with the content of this report. AWMSG were sent an early draft, but did not comment.
Appendix 1: Bibliography

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Appendix 2: Approach to orphan/ultra-orphans and medicines for rare diseases and end of life criteria at AWMSG

Orphan/ultra-orphans and medicines for rare diseases

A new approach has been adopted by AWMSG since September 2015 for orphan/ultra orphan medicines and medicines for rare diseases. AWMSG will also consider:

- The degree of severity of the disease as presently managed, in terms of survival and quality of life impacts on patients and their carers;
- Whether the medicine addresses an unmet need (e.g. no other licensed medicines);
- Whether the medicine can reverse or cure, rather than stabilize the condition;
- Whether the medicine may bridge a gap to a “definitive” therapy (e.g. gene therapy) and that this “definitive” therapy is currently in development;
- The innovative nature of the medicine;
- Added value to the patient which may not be adequately captured in the QALY (e.g. impact on quality of life such as ability to work or continue in education/function, symptoms such as fatigue, pain, psychological distress, convenience of treatment, ability to maintain independence and dignity);
- Added value to the patient’s family (e.g. impact on a carer or family life).

End of life

AWMSG will consider additional criteria when appraising medicines which may extend life for patients with short life expectancy and are licensed for indications that affect small numbers of patients with incurable illnesses. AWMSG will apply end of life criteria where the cost per Quality Adjusted Life Year (QALY) is over £30,000 and the conditions below are met:

- The medicine is indicated for patients with a short life expectancy, normally less than 24 months (e.g. estimated from the median survival of patients in the control group of the pivotal study) and;
- There is sufficient evidence to indicate that the medicine offers an extension to life, normally of at least an additional three months, compared to current NHS treatment. The estimates of the extension to life should be robust and shown (or reasonably inferred) from either progression free survival or overall survival, and;
- NMG/AWMSG considers the cumulative population of each licensed indication of the medicine to be small.

If these conditions are satisfied then consideration is given to:

- The impact of giving greater weight to QALYs achieved in the later stages of terminal diseases, using the assumption that the extended survival period is experienced at the full quality of life anticipated for a healthy individual of the same age, and;
- The magnitude of the additional weight that would need to be assigned to the QALY benefits in this patient group for the cost-effectiveness of the medicine to fall within the current threshold range.

In addition, estimates of extension to life must be robust and assumptions used in the economic modeling must be plausible, objective and robust.

Sources:
AWMSG Process for appraising orphan and ultra-orphan medicines and medicines developed specifically for rare diseases Effective from September 2015
AWMSG Policy on appraising life-extending, end of life medicines July 2015

www.cancer52.org.uk
Appendix 3: End of life criteria at NICE

Supplementary advice was given to NICE Appraisal Committees in the case of drugs that are used at the end of life. Criteria for applying the advice to NICE guidance include:

- The treatment is indicated for patients with a short life expectancy, normally less than 24 months and;
- There is sufficient evidence to indicate that the treatment offers an extension to life, normally of at least an additional 3 months, compared to current NHS treatment, and;
- The treatment is licensed or otherwise indicated, for small patient populations.

If these conditions are met, then the Appraisal Committee will consider:

- The impact of giving greater weight to QALYs achieved in the later stages of terminal diseases, using the assumption that the extended survival period is experienced at the full quality of life anticipated for a healthy individual of the same age, and;
- The magnitude of the additional weight that would need to be assigned to the QALY benefits in this patient group for the cost-effectiveness of the technology to fall within the current threshold range.

Appraisal Committees will also need to be satisfied that the estimates of life extension are robust and assumptions in the economic modelling are plausible, objective and robust.

Source: NICE Appraising life-extending, end of life treatments 2009
Appendix 4: Modifiers used by SMC

SMC will accept greater uncertainty reflecting more limited data on efficacy for orphan drugs (treating diseases that affect fewer than five people in 10,000 in the EU).

SMC will also consider 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 definitive therapy.

SMC may also accept a higher cost per QALY when there is:

- 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 the 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 sub-group of patients may derive specific or extra benefit and that the medicine in question can, in practice, be targeted at this sub-group;
- Absence of other therapeutic options of proven benefit for the disease in question and provided by the NHS; and
- Possible bridging to another definitive therapy (e.g. bone marrow transplantation or curative surgery) in a defined proportion of patients;
- Emergence of a licensed medicine as an alternative to an unlicensed product that is established in clinical practice in NHS Scotland 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.

Sources: SMC modifiers used in appraising new medicines 2012
If we work together we can make as much noise as the big four.