

Patient Voice in SMC Technology Appraisals for Orphan and Ultra-Orphan Diseases

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INTRODUCTION

- Multistakeholder involvement is very beneficial in the appraisal of medications, especially for orphan (or ultra-orphan) diseases where data on clinical experience are extremely limited¹
- The Scottish Medicines Consortium (SMC) introduced a patient and clinician engagement (PACE) process in April 2014 as a way to capture patient and clinician perspectives for appraisals of orphan and ultra-orphan medicines²⁻⁵
- Adding PACE to the SMC assessment processes has resulted in an increased acceptance rate of appraisals to approximately 50%⁶
- The PACE group not only describes the impact of a condition on the quality of life (QoL) of patients and their caregivers, but also discusses the improvements that the new medicine could bring⁷⁻⁸

OBJECTIVES

- This review aims to assess criteria emphasized by PACE groups in the most recent SMC appraisals for orphan and ultra-orphan diseases in Scotland

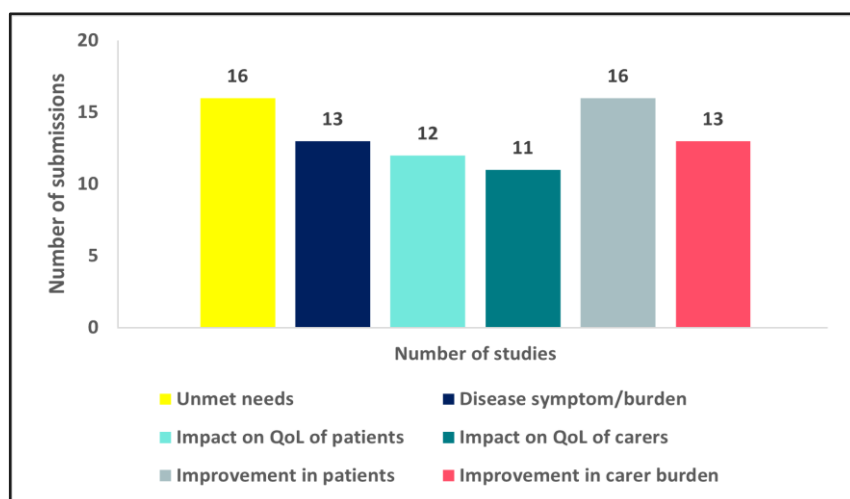
METHODS

- We assessed SMC technology appraisals between January 2022 and March 2023 that used the PACE process for orphan or ultra-orphan diseases
- Screening of the SMC submissions was performed by two independent reviewers, and discrepancies were checked by a third reviewer to ensure that the process was quality checked
- Data were extracted from the included submissions by one reviewer and quality checked against the original source by a second independent reviewer
- Key influencers identified during the PACE process were:
 - Unmet needs
 - Disease symptoms and QoL among patients and caregivers
 - QoL improvements while accessing new treatments

RESULTS

- Among all the screened SMC appraisals, 16 were relevant to the review criteria. Out of these 16 appraisals, nine were on genetic disorders, five were on tumours, one on systemic light-chain amyloidosis and one in kidney transplant patients
- Key influencer criteria identified during the PACE process are listed in Figure 1

Figure 1. Key influencers emphasized by PACE groups in SMC appraisals for orphan and ultra-orphan diseases



- All 16 appraisals discussed unmet needs. need for effective therapy predominated in 14 (87.5%), followed by impractical dosing/compliance issues and high disease burden due to frequent hospital visits in two (12.5% each) appraisals (Table 1)
- The impact on patient and caregiver QoL was discussed in 12 and 11 appraisals, respectively. Effects on relationships, personal life, and emotional and mental well-being were concerns raised in eight (50%) and five (31.3%) appraisals by patients and caregivers, respectively (Table 1)
- Patients also reported increased dependency on caregivers and medical services in five (31.3%), and distress due to disease symptoms in three (18.6%) appraisals as troublesome (Table 1)
- Indirect costs and productivity losses were also a concern among patients in five (31.3%) and caregivers in three (18.6%) appraisals (Table 1)
- Improvements in QoL for patients in all 16 (100%) and caregivers in 13 (81.3%) appraisals with access to new treatments were also reported (Table 1)

REFERENCES

1. Mikami and Sturdy. *Res Involv Engagem.* 2017; 3:1-14.
2. Nicod et al. *Value Health.* 2019; 22:S855.
3. Stafinski et al. *Orphanet J Rare Dis.* 2022; 17(1):1-14.
4. Hems et al. *Int J Technol Assess Health Care.* 2023; 39(1):e19.
5. Macaulay. *Value Health.* 2015; 18(7):A479.
6. Macaulay. *Value Health.* 2018; 21:S53.
7. Scottish Medicines Consortium. Patient and clinician engagement (PACE) process. 2021. Available from: <https://www.scottishmedicines.org.uk/how-we-decide/pace/>. Accessed: 24 May 2023.
8. Nicod et al. *Orphanet J Rare Dis.* 2020; 15:1-14.

Table 1. Key influencer criteria expressed by PACE groups in the SMC submissions and their common concerns for the orphan/ultra-orphan diseases

Key influencer criteria expressed by PACE group	Detailed description	N (%)
Unmet needs n = 16 (100%)	Need for more effective and efficacious therapy	14 (88%)
	Limited or no licensed treatments available	7 (44%)
	Palliative care/best supportive care being the only treatment option	2 (13%)
	Impractical dosing schedules and compliance issues	2 (13%)
	Challenging and time-intensive care	2 (13%)
	Off-label treatments with varying degrees of success	2 (13%)
	Frequent hospital visits and significant time off work	2 (13%)
Disease symptom/burden n = 13 (81%)	Weakness and fatigue	6 (38%)
	Severe muscular complications; bone, joint and eye pain	4 (25%)
	Functional neurological and motor disorders	4 (25%)
	Breathlessness, chest pain, cough, fatigue and weight loss	3 (19%)
	Hearing loss, infections, and growth problems	2 (13%)
	Lack of sex drive and infertility	2 (13%)
Impact on quality of life in patients n = 12 (75%)	Impact on psychological, social, and personal life along with disturbed relationships, emotional and mental health	8 (50%)
	Increased dependence on family, carers, and medical services	5 (31%)
	Indirect costs and productivity losses due to frequent hospital visits	5 (31%)
	Distress because of disease symptoms	3 (19%)
	Compliance issues due to impractical dosing regimens and frequency	2 (13%)
Impact on quality of life in carers n = 11 (69%)	Impact on psychological, social, and personal life along with disturbed relationships, emotional and mental health	5 (31%)
	Indirect costs and productivity losses due to frequent hospital visits	3 (19%)
	Distress because of disease symptoms in patients	3 (19%)
	Financial burden including giving-up of work	2 (13%)
	Increased caring responsibilities	2 (13%)
Improvement in patients n = 16 (100%)	Better disease control	8 (50%)
	Improved functioning and independence	7 (44%)
	Fewer hospital visits and reduced healthcare resource usage	6 (38%)
	Convenient dosing schedules and administrations	6 (38%)
	Reduced need for mobility aids, emergency care and hospital visits	5 (31%)
	Positive impact on relationships within personal and social life	4 (25%)
	Improved mental, physical and emotional wellbeing as the burden of treatment diminishes	3 (19%)
	Relieved anxiety, stress and improved energy	3 (19%)
Improvement in carer burden n = 13 (81%)	Fewer hospital visits and reduced healthcare resource usage	5 (31%)
	Improvements in mental health, relationships, and their social and working lives	4 (25%)
	Reduced treatment burden on healthcare services	4 (25%)
	Better sleep, reduced anxiety and stress as the caring responsibilities decrease	2 (13%)
	Improvements in emotional wellbeing	2 (13%)
	Convenient dosing schedules and administrations	2 (13%)
	Reduced disruption to education and work	2 (13%)
	Relieved stress as disease/symptom burden decreases	2 (13%)

CONCLUSIONS

- Our review of appraisals via PACE process demonstrates that there should be increased transparency on how patient group involvement impacts the SMC's decision making
- The SMC has structured templates for patient feedback and accepts inputs from the PACE group
- The PACE process empowers patients and clinicians to have a voice in SMC decision-making. It is not just limited to unmet needs, but also applies to the clinical and humanistic burden of the disease
- Patients' experiences are essential in understanding the impact of disease on them and their caregivers and, in turn, the improvements new medicines could offer



An electronic version of the poster can be viewed by scanning the QR code.