Exploring The Difference That Patient Involvement In HTA Makes In Cancer

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Introduction and objectives

Patient involvement is important and valuable in the context of health technology assessment (HTA)[1]. Interest in patient involvement in HTA has increased over time, however it has been recognised that the resources required to produce comments or to participate effectively on committees, are often beyond the reach of many patient organisations[2,3]. The importance of patient involvement in HTA has been reiterated by the Implementing Act of the EU Health Technology Assessment Regulation (HTAR) for Joint Clinical Assessments (JCA) of medicinal products, where it has been noted that patient organisations should be given the opportunity to provide their input on JCAs[4]. Opportunities and impact of patient involvement in HTA was reviewed in the Acute Leukemia Advocates Network's (ALAN) exploratory comparative report[5]. The current research further explored what difference patient involvement makes to HTA recommendations in cancer.

Methods

The number of cancer patient group submissions to HTA agencies was identified from IQVIA's Market Access Insights database[6]. HTA recommendations made with, and without patient group submissions, were compared. The data included 871 HTAs between January 2020 and March 2023 conducted by CDA-AMC (formerly CADTH), NICE, HAS, the G-BA and IQWiG and the SMC. A subjective assessment of the openness to patient involvement was conducted. Additionally, the websites of HTA agencies were reviewed to provide wider context on their approach to patient involvement in HTAs.

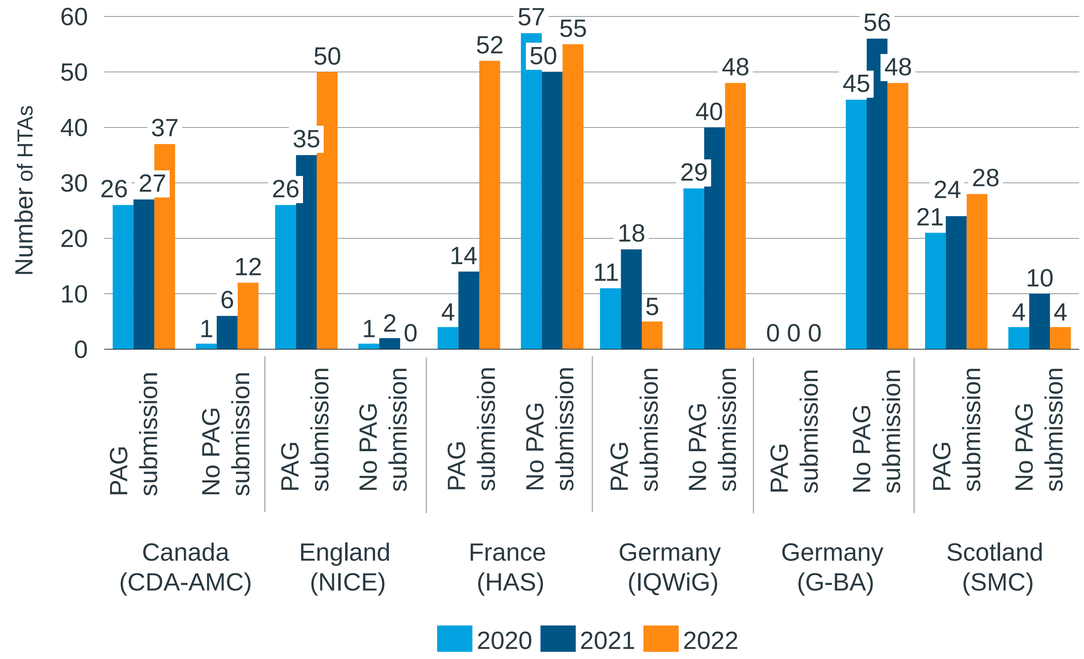
Results

- All HTA agencies in scope allow patient organisations to get involved in the HTA process, either by submitting written statements or by participating in the working groups or committees within an HTA body (see Table 1)
- The number of patient advocacy group (PAG) submissions has increased at CDA-AMC (from 26 in 2020 to 27 in 2021 and 37 in 2022), NICE (26, 35, 50), HAS (4, 14, 52) and the SMC (21, 24, 28), so workload for both patient groups and HTA agencies has increased. Patient group submissions fell at IQWiG (11, 18, 5) (see Graph 1)
- NICE received patient group submissions for 97% of cancer HTAs, CDA-AMC 84% and SMC 81% and those three agencies seem most open, while for HAS and IQWiG/G-BA, the percentage of cancer HTAs with patient group submissions was lower (see Graph 2)
- There was no clear pattern in the distribution of positive, restricted / conditional and negative HTA recommendations with and without PAG submissions
- Some cancer indications with a lower number of HTAs overall had a higher percentage of HTAs with patient group submissions. Percentage of HTAs with patient group submissions were lower for cancer types with the highest number of HTAs (lung cancer, breast cancer, lymphoma, leukaemia, myeloma) (see Graph 3)

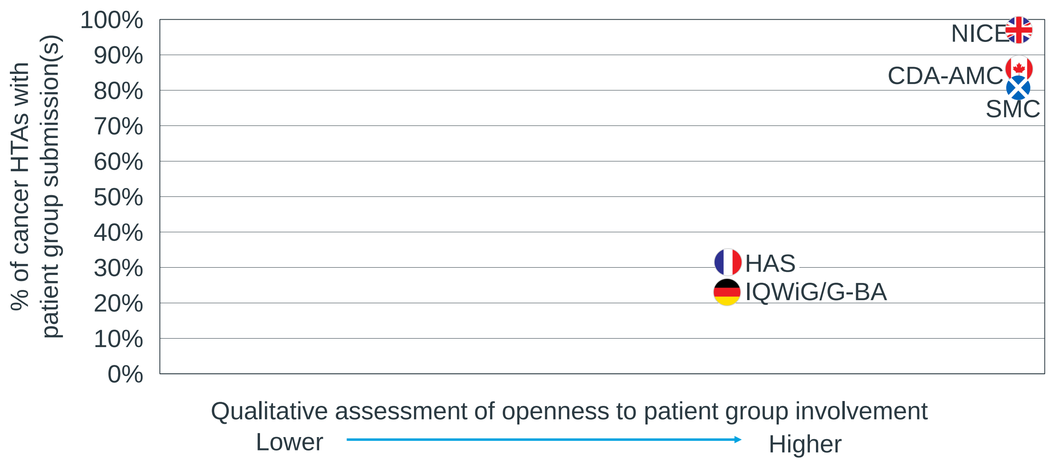
Further research

- Individual case studies could shed further light on the difference patient input makes; there may be lessons from NICE's HTA of venetoclax in combination with obinutuzumab for chronic lymphocytic leukaemia, where NICE initially restricted the recommended population in line with the clinical trial population[7]. Patient expert input was provided on the importance of having access to venetoclax in the initially excluded subpopulation. NICE placed the initially excluded subpopulation in the Cancer Drugs Fund. In NICE's HTA of gliteritinib in acute myeloid leukaemia, patient experts noted the advantages of oral administration, preferred over the current intravenous chemotherapy[8]. NICE added disutilities for chemotherapy in the economic model, leading to improved cost-effectiveness of gliteritinib.

Graph 1: Number of cancer patient group submissions by agency, 2020 to 2022



Graph 2: HTA openness to patient group involvement and proportion of cancer HTAs where a patient group submission was received, 2020-2023



Graph 3: Number of cancer HTAs and proportion with a patient group submission by cancer type, 2020-2023

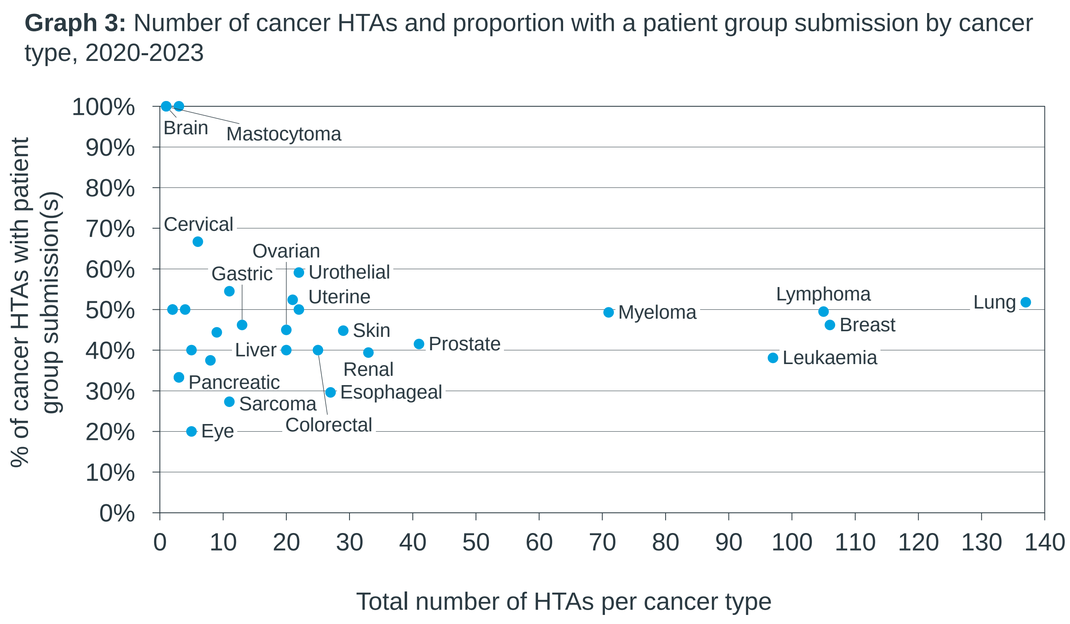


Table 1: Patient involvement opportunities across HTA agencies

HTA agency	Patient involvement opportunities in HTA
CDA-AMC	A call for patient input is issued for every HTA. Additionally, patient groups are invited to review and comment on the draft recommendations.
NICE	Patient groups may get involved before development of the HTA, by providing input on the proposed scope of the evaluation, during development, by submitting comments on a draft version of the guidance, and after publication by providing input on whether guidance should be updated.
SMC	Patient groups can engage in the assessments for medicines with scope for additional input for end of life or orphan or ultra-orphan medicines through the PACE meeting, which include patient group representatives and clinical experts to discuss the severity of the condition and how it impacts on a patient's quality of life and on family and carers.
HAS	Patients can participate in two ways: individual patients may provide their expertise, e.g. in a working group or during the review phase; patient association representatives can participate in institutional meetings or be heard on behalf of the association, as a stakeholder.
IQWiG	Patient groups can submit a written statement to comment on preliminary reports of the benefit assessments of the medicinal products and procedures.
G-BA	Patient involvement is limited to the patient representation in the decision-making committees (with no vote), who are only allowed if they are registered with 1 of 4 predefined organisations.

Conclusions

- **Patient involvement in HTA represents an increasing workload. HTA agency openness to patient group involvement may be encouraging patient group submissions. Patient groups may have capacity constraints, limiting the patient groups' ability to respond to all HTAs, especially in cancer types with multiple products.**
- **The difference patient group involvement makes to HTA recommendations requires more research. Although all HTA agencies encourage patient groups to input into HTAs, it is unclear what difference patient group submissions made to HTA recommendations. Patient groups may be optimising their involvement when they believe that their submission will have the biggest impact. Further research could explore whether it is possible to identify a priori the HTAs where patient input is most likely to have an influence to help patient groups plan for future involvement**

References: 1) INAHTA. (June 2021). INAHTA Position Statement: Patient Involvement. 2) Werkö SS, Staniszewska S (2021). Patient and public involvement in Health Technology Assessment: a new dawn? International Journal of Technology Assessment in Health Care 37, e54, 1–2. 3) Drummond, M., Torbica, A. and Tarricone, R. (2020) Should health technology assessment be more patient centric? If so, how? Eur J Health Econ 21, 1117–1120. 4) The Implementing Act of the EU Health Technology Assessment Regulation (HTAR) for Joint Clinical Assessments (JCA) of medicinal products. 5) ALAN: Opportunities and impact of patient involvement in health technology assessment of medicines for the purpose of reimbursement: An exploratory comparative report (2024); 6) IQVIA's Market Access Insights; 7) NICE: Venetoclax with obinutuzumab for untreated chronic lymphocytic leukaemia; Technology appraisal guidance; Reference number: TA663; Published: 09 December 2020; 8) NICE: Gilteritinib for treating relapsed or refractory acute myeloid leukaemia; Technology appraisal guidance; Reference number:TA642; Published: 12 August 2020; **Abbreviations:** ALAN: Acute Leukemia Advocates Network; CDA-AMC: Canada's Drug Agency / L'Agence des medicaments du Canada; G-BA: The Federal Joint Committee (Gemeinsamer Bundesausschuss); HAS: Haute Autorité de Santé; HTA: Health technology assessment; HTAR: Health Technology Assessment Regulation; IQWiG: Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen; JCA: Joint Clinical Assessment; NICE: The National Institute for Health and Care Excellence; PACE: Patient and Clinician Engagement; PAG: Patient advocacy group; SMC: Scottish Medicines Consortium

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