AXIS
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Trends in patient organisation submissions in Ireland

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01. Introduction

In Ireland, the National Centre for Pharmacoeconomics (NCPE) calls for submissions from patient organisation (PO) groups upon receipt of a company health technology assessment (HTA).

Many PO's also function as patient advocacy groups (PAG) seeking improved access to treatment. Inclusion of the patient voice ensures that their perspective of the disease and treatment-pathways is incorporated into the decision-making process by the Irish authorities.

The effective communication of the lived experiences of patients, can play a critical role in influencing the outcome of HTA evaluations.

The key objectives of this research are to:

1. Build Highlight the current process for involving patient perspectives in Ireland.
2. Investigate the long-term trends in PO submissions submitted to the NCPE
3. To categorise PO submissions to understand the focus on rare disease and cancer-related conditions versus other medical conditions over time

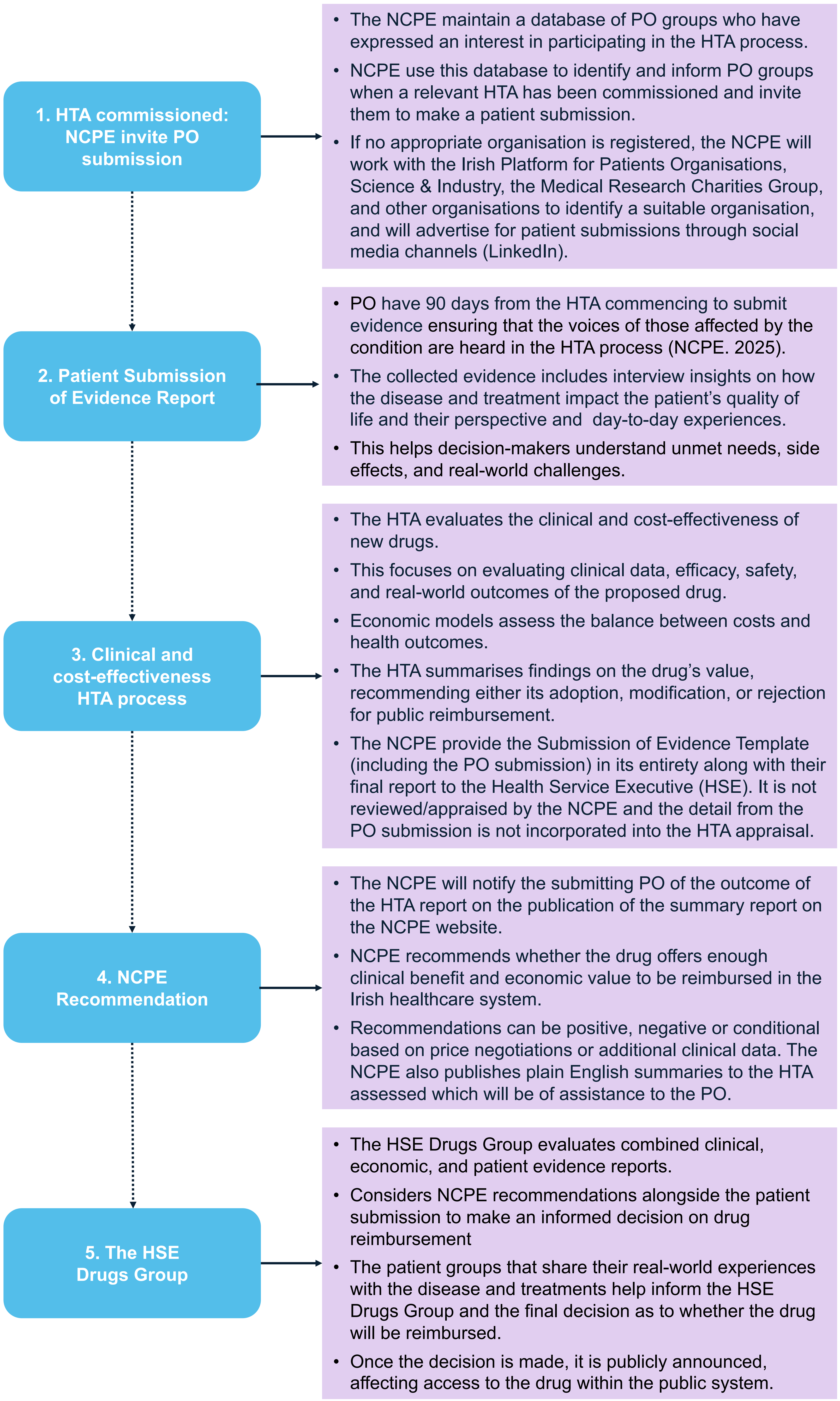
03. Involvement of POs in HTA process

The inclusion of patients' experiences and perspectives is an integral component of the reimbursement process (Figure 1). Patients provide valuable insights into the advantages and disadvantages of currently available therapies, which may not be reflected in the literature or well understood by experts in HTA and decision-makers.

Patients play a crucial role in describing the burden of disease, helping decision-makers fully understand the unmet needs that novel therapies should address. Addressing the burden of disease requires a holistic understanding that extends beyond just medical or clinical outcomes. It involves recognising the economic, social, and emotional impacts of diseases on patients and society.

By incorporating patient perspectives into decision-making, healthcare systems can better allocate resources and develop treatments that not only prolong life but also improve its quality. Addressing health inequalities and unmet needs is essential to reducing the overall burden of patients.

Figure 1: Inclusion of patient voice across the Irish reimbursement process



1. HTA commissioned: NCPE invite PO submission

- The NCPE maintain a database of PO groups who have expressed an interest in participating in the HTA process.
- NCPE use this database to identify and inform PO groups when a relevant HTA has been commissioned and invite them to make a patient submission.
- If no appropriate organisation is registered, the NCPE will work with the Irish Platform for Patients Organisations, Science & Industry, the Medical Research Charities Group, and other organisations to identify a suitable organisation, and will advertise for patient submissions through social media channels (LinkedIn).

2. Patient Submission of Evidence Report

- PO have 90 days from the HTA commencing to submit evidence ensuring that the voices of those affected by the condition are heard in the HTA process (NCPE. 2025).
- The collected evidence includes interview insights on how the disease and treatment impact the patient's quality of life and their perspective and day-to-day experiences.
- This helps decision-makers understand unmet needs, side effects, and real-world challenges.

3. Clinical and cost-effectiveness HTA process

- The HTA evaluates the clinical and cost-effectiveness of new drugs.
- This focuses on evaluating clinical data, efficacy, safety, and real-world outcomes of the proposed drug.
- Economic models assess the balance between costs and health outcomes.
- The HTA summarises findings on the drug's value, recommending either its adoption, modification, or rejection for public reimbursement.
- The NCPE provide the Submission of Evidence Template (including the PO submission) in its entirety along with their final report to the Health Service Executive (HSE). It is not reviewed/appraised by the NCPE and the detail from the PO submission is not incorporated into the HTA appraisal.

4. NCPE Recommendation

- The NCPE will notify the submitting PO of the outcome of the HTA report on the publication of the summary report on the NCPE website.
- NCPE recommends whether the drug offers enough clinical benefit and economic value to be reimbursed in the Irish healthcare system.
- Recommendations can be positive, negative or conditional based on price negotiations or additional clinical data. The NCPE also publishes plain English summaries to the HTA assessed which will be of assistance to the PO.

5. The HSE Drugs Group

- The HSE Drugs Group evaluates combined clinical, economic, and patient evidence reports.
- Considers NCPE recommendations alongside the patient submission to make an informed decision on drug reimbursement
- The patient groups that share their real-world experiences with the disease and treatments help inform the HSE Drugs Group and the final decision as to whether the drug will be reimbursed.
- Once the decision is made, it is publicly announced, affecting access to the drug within the public system.

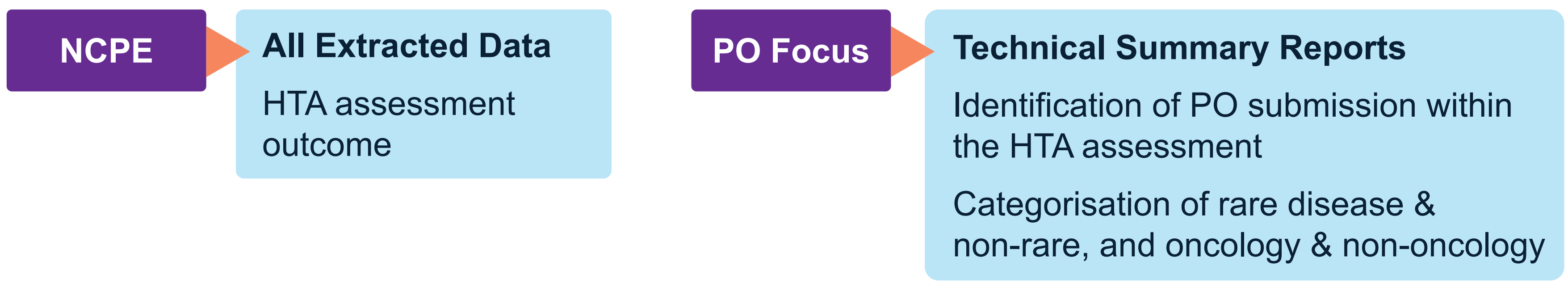
Abbreviations *HSE: Health Service Executive; HTA: health technology assessment; NCPE: National Centre for Pharmacoeconomics; PAG: patient advocacy groups; PO: patient organisation.*

02. Methods

A comprehensive analysis was undertaken to compile HTA evaluations completed between 2018 and 2024. This process involved gathering data for the NCPE into a unified database.

NCPE HTA assessments completed between 2018 and 2024 were compiled into a Microsoft Excel® database using published data from the NCPE website. All relevant details on reimbursement submissions were extracted including the technical summary reports.

Descriptive statistics were used to quantify the number of PO submissions received overall, within oncology-specific, non-oncology, and rare-disease indications.



NCPE → **All Extracted Data**
HTA assessment outcome

PO Focus → **Technical Summary Reports**
Identification of PO submission within the HTA assessment
Categorisation of rare disease & non-rare, and oncology & non-oncology

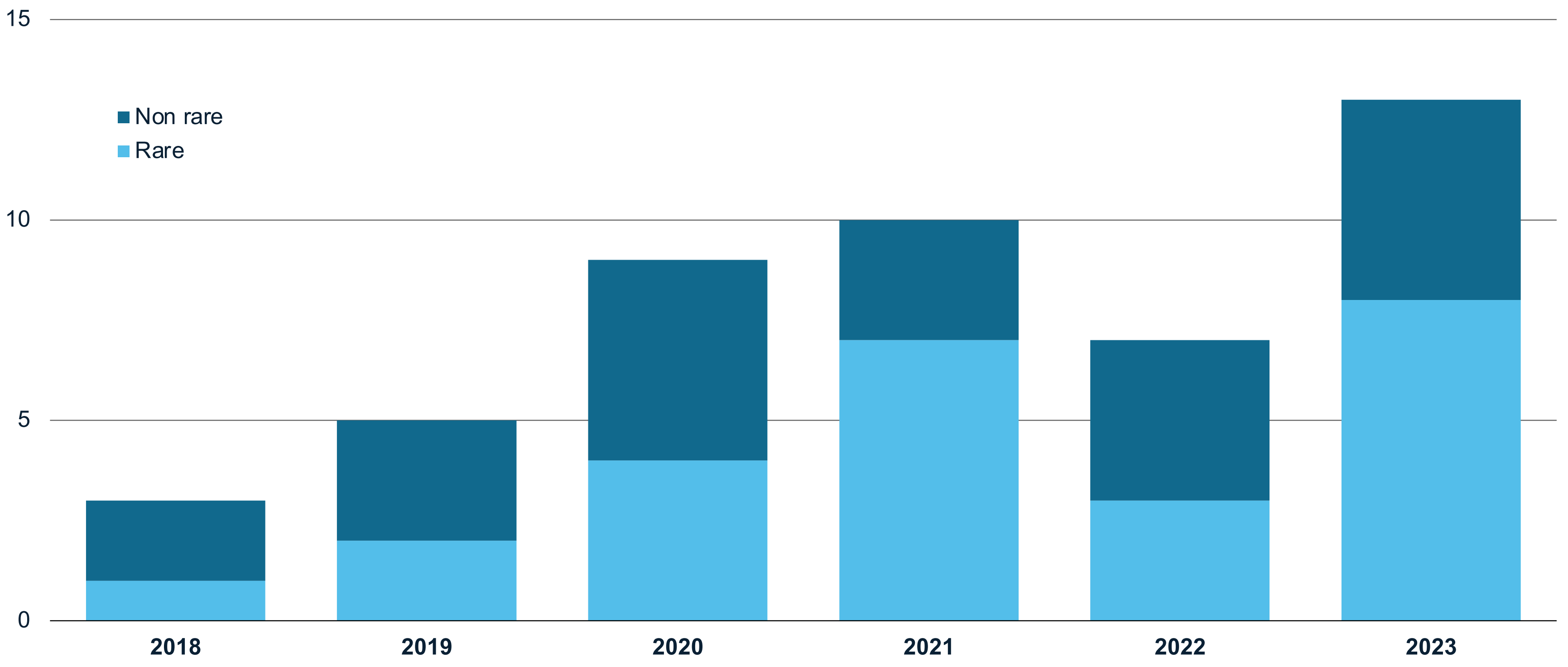
05. Results

Long-term six-year analysis of HTA submissions (N=139) showed a marked increase in the proportion of PO submissions from 2.16% (2018) to 10.8 % (2023). Although some 2024 HTAs are still in progress and thus don't have published summaries, 44.4% of the available technical reports referenced receipt of a PO submissions suggesting a continuing long-term trend of PO's making submissions upon request from NCPE.

The HTA submissions (N=139) from 2018-2024 were mostly oncology-specific (58%) versus non-oncology (42%), while the non-rare (63%) category outnumbered rare (37%). Rare cancer POs were included in both oncology-specific and rare analysis. Of all PO submissions identified (51/139): most were non-oncology (61%) as opposed to oncology-specific (39%), with rare-diseases accounting for 55% (non-rare, 45%).

The yearly PO submission analysis for treatments for rare or non-rare diseases is provided in Figure 2. From 2018 to 2023, submissions related to rare diseases increased.

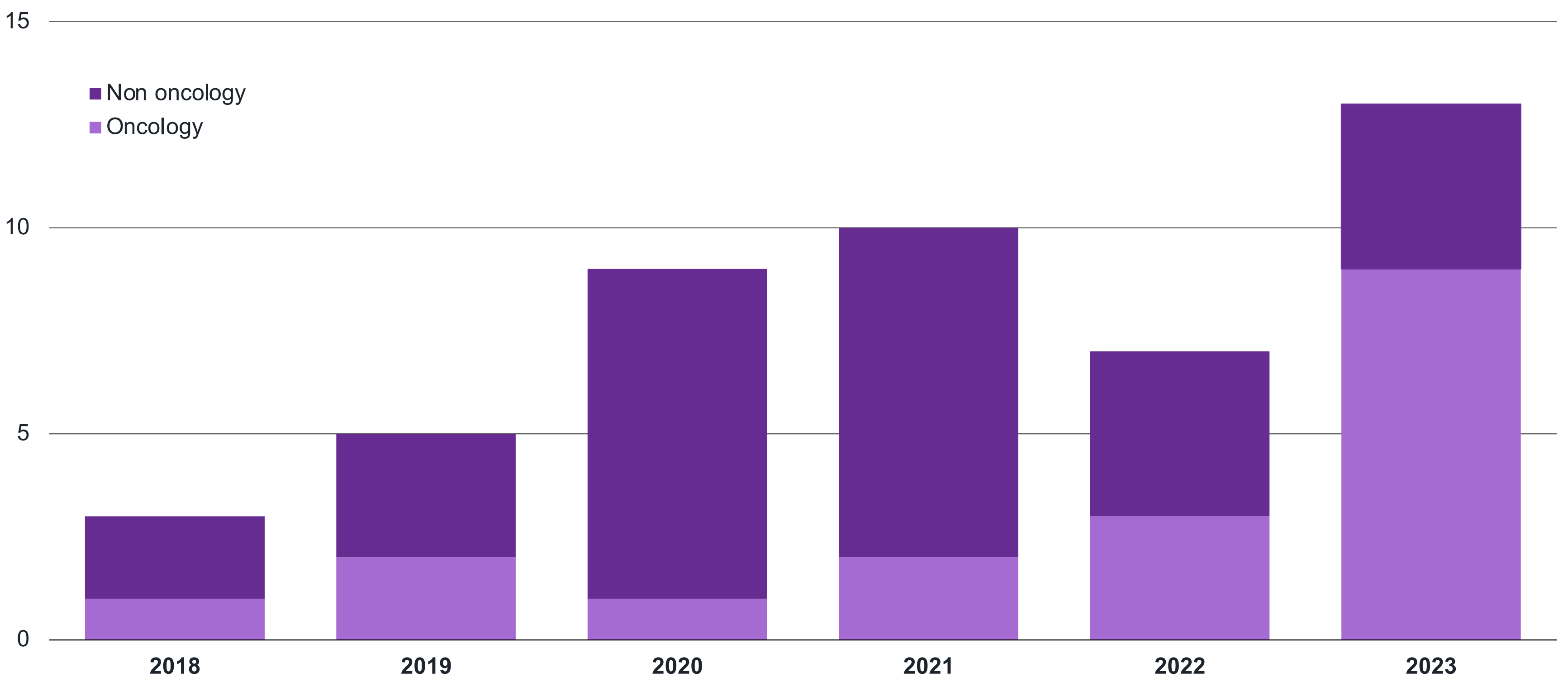
Figure 2: Patient organisation submissions in Non-rare disease and Rare disease appraisals



PO submissions for non-oncology submissions (Figure 3) represented a significant portion of the total submissions.

Between 2018 and 2022, PO submission for non-oncology appraisals consistently outnumbered oncology appraisals except for 2023. There was an increase in oncology-related PO submissions from years 2020 to 2023.

Figure 3: Patient organisation submissions in Oncology and Non-oncology appraisals



06. Conclusions

There is an increasing long-term trend in PO submissions to the NCPE since 2018. This highlights the work of the NCPE to increase involvement of patient advocacy in the HTA process in Ireland. The shift in HTA towards more patient-centricity, reflects the ethical principles of respect for autonomy and justice by considering the interests and rights of patients in decision-making.

The high levels of rare disease advocacy may reflect the unique challenges faced by patients with these conditions, the importance of their representation in the HTA process, and available resourcing dedicated to their advocacy. Such advocacy prevents rare disease patients from being deprioritised.

Submissions for non-oncology conditions continue to dominate in yearly PO submissions, highlighting the broad range of patient needs across many therapeutic areas. However recent trends show that POs are increasingly support to new cancer treatments.

Future linear regression analysis will focus on examining the long-trends following the NCPE public consultation in 2017 to implement strategies to refine the PO submission process.

Given the fact that not all medicines undergo a full HTA evaluation, consideration should be afforded to how POs might ensure the patient voice is considered during the preliminary evaluation stage by the NCPE with the Rapid Review.

Involvement of patient voices in every step of the HTA process will enhance the quality and transparency of evidence and patient centred recommendations.

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