What matters most? An exploration of decision criteria considered by patients with GEP-NET and physicians using holistic Multi-Criteria Decision Analysis

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ABSTRACT

BACKGROUND: Patient-centered care implies identifying what matters most to patients and physicians through shared decisionmaking on disease management. EVIDEM provides a generic holistic MCDA platform to explore decision criteria and trade-offs. The study aimed to develop a comprehensive decision framework and identify preferences of patients and physicians in the management of unresectable, well- or moderately differentiated non-functioning GEP-NET.

METHODS: A decision support framework was designed based on EVIDEM structure, literature review and insights from a Chatham-house panel of US physicians and patients, representative of different management approaches for GEP-NET. During a second extended panel session (5 patients, 6 physicians), participants provided criteria weights using Hierarchical Point Allocation and Direct Rating Scale (DRS, sensitivity analyses). Insights were collected in writing and through discussions.

RESULTS: The decision support framework included 6 domains pertaining to Outcomes of the intervention (Effectiveness, Patient-Reported Outcomes, Safety); Type of benefit; Need (Disease severity; Unmet needs; Population size); Costs & constraints (Intervention; Medical and Non-medical [to patients or the healthcare system]); Knowledge (Quality of evidence, Expert consensus) and Feasibility (System capacity). Of the 30 criteria and subcriteria, 26 were considered by more than 90% of participants. Criteria weights were widely distributed reflecting variability in individual perspectives on what matters most. At the group level, highest weights were attributed to Effectiveness (0.18 ± SD 0.12 on a total of 1) and Disease severity (0 .12 ± 0.08), followed by Safety (0.10 ± 0.09), Type of therapeutic benefit (0.10 ± 0.08) and Quality of evidence (0.09 ± 0.06). Most important Effectiveness subcriteria were Overall survival (33% of effectiveness criteria), followed by Progression-free survival (30%). DRS showed similar overall results.

CONCLUSIONS: Many aspects are considered by patients and physicians in their decisionmaking processes. Holistic MCDA reveals and structures the complexity and variability of what matters most to patients.

OBJECTIVES

- To explore the preferences and underlying criteria that patients and clinicians use when making their decisions on the treatment options of unresectable, well- or moderately differentiated nonfunctioning GEP-NET, by elucidating which criteria are considered and how they are considered using an MCDA approach. The study aims to develop a comprehensive decision framework and identify preferences of patients and clinicians.

BACKGROUND

- Patient-centered care implies identifying what matters most to patients and physicians through shared decisionmaking on disease management.
- Multi-criteria decision analysis (MCDA) provides a structured and transparent approach to holistically elicit preferences and tackle the ethical trade-offs between conflicting demands.[1-8]
- The open-source EVIDEM MCDA framework was designed to stimulate structured reflection and pragmatic collection of insights on the true value of interventions from all stakeholders, through a broad set of quantitative and qualitative criteria, each explicitly rooted in ethical aspects inherent to fair and accountable decisionmaking.[4,9-11]
- EVIDEM presents a number of assets:
  - Provides a platform to organize very complex data and systematically consider scientific evidence on different treatment options.
  - Takes into account the values and preferences of patients and healthcare professionals when deciding on the most appropriate treatment path.
  - Provides a powerful analytical tool to collect qualitative and quantitative data and insights in a structured format.
  - Facilitates communication of the benefits, risks and preferences and concerns pertaining to different treatment options among patients, caregivers and clinicians.
  - Flexible design allows the inclusion of scientific and colloquial evidence, contextual specificities and individual insights.

METHODS

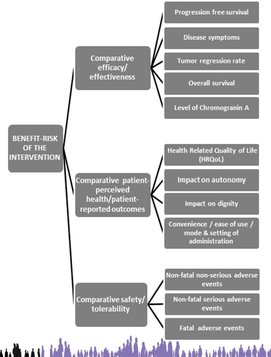
Design of the workshops

- Invited physician panelists were identified by the research team, based on the physicians' area of expertise and interest. Patients were invited by reaching out to local patient associations (the Carcinoid Cancer Foundation and the Neuroendocrine Cancer Awareness Network (NCANN)) and patient support groups in the Greater Boston area and New York.
- Two panel sessions were organized: The Criteria workshop and the Decision Support workshop. Both sessions were held under the Chatham House Rule.
- During the Criteria workshop, participants were introduced to the MCDA methodology and prompted to validate and identify relevant criteria when deciding what matters with regards to treatment of unresectable, well- or moderately differentiated non-functioning GEP-NET.
- During the Decision Support workshop, participants explored what matters and how it matters by providing qualitative and quantitative feedback on how each criterion is considered for different decision scenarios.
- Criteria being explored were core Benefit-Risk criteria (effectiveness, patient-reported outcomes, safety) and Modulating criteria, identified from the EVIDEM framework and the literature review, and from participants' insights collected during the Criteria workshop.

MCDA mechanics - weighting

- Participants indicated the relative importance of criteria for decisionmaking by assigning weights using the Hierarchical Point Allocation (HPA) method.[12] Participants were instructed to divide 100 points across the criteria, and divide 100 points across the subcriteria within each criterion.
- An alternative weighting technique (Direct Rating Scale) was used for sensitivity analyses.[4]
- Weights were normalized at the level of the individual participant by dividing each weight by the sum of weights across all criteria assigned by the participant. Thus, the sum of normalized weights for all criteria for each panelist was 1.

Figure 1. MCDA Benefit-Risk tree



METHODS *(cont.)*

Figure 2. Modulated MCDA Benefit-Risk tree

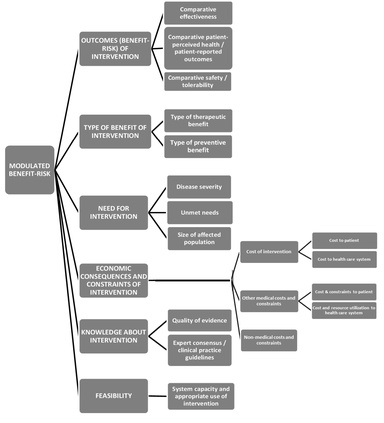
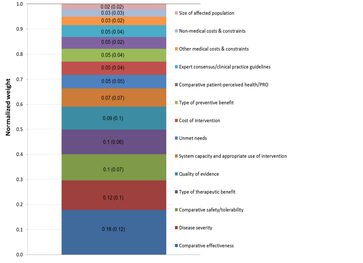


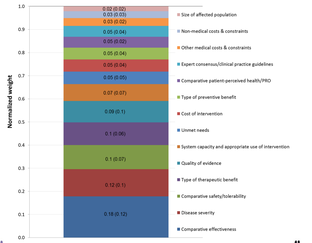
Figure 3. Mean (SD) normalized weights assigned to each Benefit-Risk criterion by participants using the hierarchical point allocation (HPA) weight elicitation technique



RESULTS

- Three patients and three clinicians practicing in different settings and with different approaches to treatment participated in the Criteria workshop.
- The decision support framework included 6 domains of quantitative criteria including one pertaining to the benefit-risk criteria: Outcomes of the intervention (criteria & [subcriteria]: Effectiveness [*Progression-free survival*, *Disease symptoms*, *Tumour regression*, *Overall survival*, *Level of chromogranin A*]; PRO [*QoL, autonomy, dignity & convenience*]; Safety [*Non-fatal non-serious adverse events*, *Non-fatal serious adverse events*, *Fatal adverse events*]) – see Figure 1) and five encompassing modulating criteria: Type of benefit (Therapeutic benefit, Preventive benefit); Need (Disease severity; Unmet needs; Population size); Economic consequences & constraints (Intervention cost [*To patients, To healthcare system*]; Medical cost [*To patients, To healthcare system*]; Non-medical cost); Knowledge (Quality of evidence, Expert consensus) and Feasibility (System capacity) (see Figure 2).
- Of the 30 criteria and subcriteria, 26 were considered as important by more than 90% of participants.
- Among Benefit-Risk criteria, *Overall survival* had the highest normalized weight (mean 0.16), followed by *Progression-free survival* (mean 0.15), *Fatal adverse events* (mean 0.14), and *Disease symptoms* (mean 0.11). The least weight was assigned to *Level of chromogranin A* (mean 0.01) and *Convenience and ease of use* (mean 0.04) (Figure 3).
- Among Modulated Criteria, *Comparative effectiveness* received the highest normalized weight (mean 0.18), followed by *Disease severity* (mean 0.12 ± 0.08), and *Comparative safety/tolerability* (mean 0.1 ± 0.09) and *Type of therapeutic benefit* (mean 0.1 ± 0.08). The least weight was assigned to *Size of affected population* (mean 0.02 ± 0.02) and *Non-medical costs & constraints* (mean 0.03 ± 0.03) (Figure 4).
- An exploratory subgroup analysis (descriptive statistics only, due to small sample size) was performed to compare patients' versus clinicians' responses. At the subgroup level, patients assigned a higher weight to *Comparative effectiveness* (mean 0.23 ± 0.13), followed by *Comparative safety/tolerability* (mean 0.15 ± 0.07) and Type *of therapeutic benefit* and *Type of preventive benefit* (both mean 0.08 ± 0.05). By contrast, clinicians assigned a higher weight to *Disease severity* (mean 0.16 ± 0.12), followed by *Comparative effectiveness* (mean 0.13 ± 0.09) and *Quality of evidence* (mean 0.12 ± 0.13).
- Weights for the criteria *Comparative effectiveness*, *Comparative safety/tolerability*, and *Type of preventive benefit* were approximately two-fold higher for patients compared to clinicians. Weights for the criteria *Disease severity*, *System capacity* and *Quality of evidence* were two-fold higher for clinicians.

Figure 4. Mean (SD) normalized weights assigned to each Modulated Benefit-Risk criterion by participants using the hierarchical point allocation (HPA) weight elicitation technique



RESULTS *(cont.)*

- When comparing criteria weight elicitation techniques, mean normalized weights largely followed similar patterns with *Comparative effectiveness, Comparative safety/ tolerability, Type of therapeutic benefit* and *Disease severity* having the highest weights (mean 0.09 each). The least weight was assigned to *Size of affected population* (mean 0.03).

CONCLUSIONS

- Explorations of criteria allowed identification of what matters to patients and physicians for the management of GEP-NET.
- The majority of participants deemed that all criteria of the EVIDEM MCDA framework are relevant to engage in a discussion around treatment initiation
- Participants found the process innovative and helpful since it allowed them to make their thinking more explicit while providing a different perspective when assessing data. It fostered an environment where both patients and clinicians had the opportunity to discuss sensitive elements with each other.
- This study illustrates how holistic MCDA reveals and structures the complexity and variability of what matters most to patients.

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