

# Importance of Intermediate Clinical Endpoints in Prostate Cancer for Patients: a Cluster Analysis

Dotsikas K<sup>1</sup>, McCallion J<sup>2</sup>, Laurent J<sup>1</sup>, Lesbros C<sup>1</sup>, Radoszycki L<sup>1</sup>

<sup>1</sup> Carenity, Online Patient Community, Paris, France

<sup>2</sup> Johnson & Johnson, Raritan, NJ, USA

## Key Takeaway



Patients' knowledge and personal experience with prostate cancer appear to shape how strongly they value intermediate clinical endpoints like MFS, EFS, NED, and pCR, indicating these outcomes may resonate more with better-informed patients.

## Conclusions



MFS, EFS, NED & pCR were perceived as highly important for patients (mean importance score of at least 9/10 for each)



Clusters of perceived importance patterns are driven by the patients' knowledge and awareness of and their personal experience with PC



Findings underscore the need to incorporate patient understanding when defining meaningful endpoints for PC management and suggest that ICEs warrant consideration in payer decision-making and clinical investigations alongside OS considerations, as they have perceived value by patients

The abstract can be found [here](#)

## Introduction

- Overall survival (OS) is the standard endpoint in prostate cancer (PC)<sup>1-3</sup>
- Because localized PC develops slowly, intermediate clinical endpoints (ICEs) are used as potential predictors of overall survival.<sup>4,5</sup> **The endpoint definitions were simplified by a medical expert advisory board to improve patient understanding (patient-friendly definitions below).**
  - Metastasis-Free Survival (MFS): Time from treatment start until cancer spreads beyond the prostate and is seen on scans<sup>3</sup>
  - Event-Free Survival (EFS): Time from treatment start until any signs of PC are detected.
  - No Evidence of Disease (NED): No signs of PC 4 years after successful treatment, with hormone levels restored.<sup>4</sup>
  - Pathological Complete Response (pCR): No cancer detected in tested tissue after successful treatment.<sup>6</sup>

- Understanding the patient relevance of ICEs is crucial for patient-centered care and decision-making by Health Technology Assessment agencies
- Our objectives were to assess the patient relevance of ICEs, identify distinct patient profiles, and determine which factors best explain differences in perceptions of ICEs

## Methods

### Study design

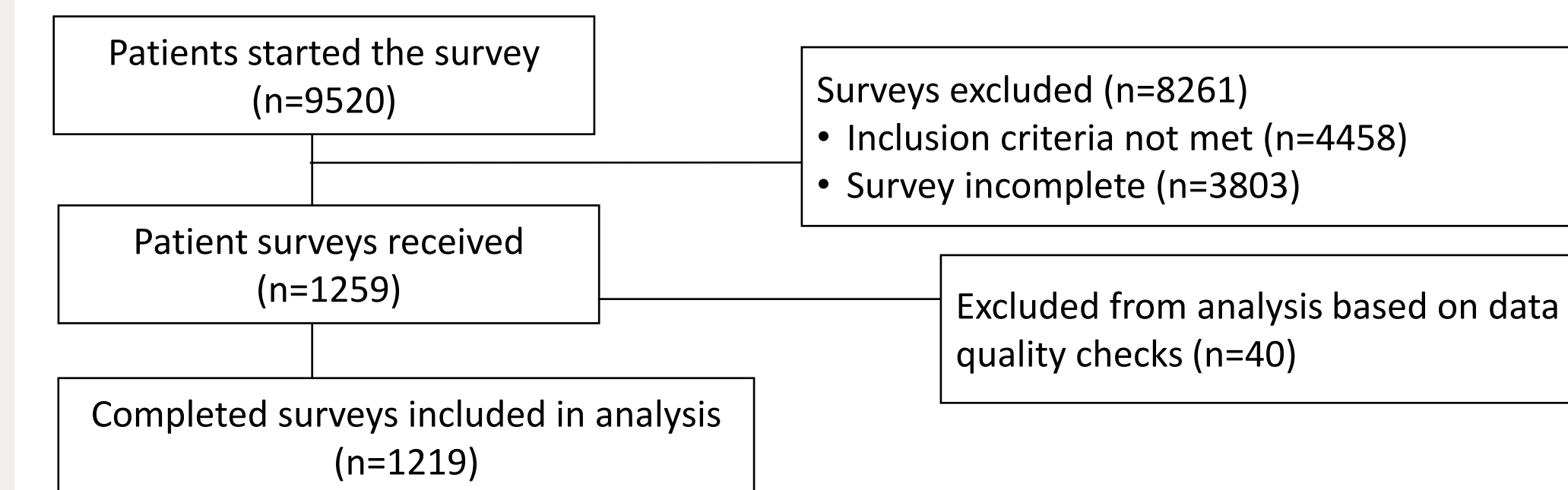
- Following institutional review board approval, patients were recruited via Carenity, an online international community of >500,000 patients and caregivers in Europe and the US, and through its local partner in Asia
- The self-administered online survey had 30 questions and was conducted on the Carenity platform from January to May 2025 among patients with PC in China, France, Germany, Italy, Japan, Spain, UK, and US

## Results

### Patient demographic and clinical characteristics

- The analysis included 1219 patients who fully completed the survey (Figure 1)
- Among 1167 patients who knew their disease stage, 75% had non-metastatic (nm) PC and 25% had metastatic PC (mPC)
- There were 149 to 162 patients in each country.
- Patients were 31 to 94 years old, with a median age of 69 years.
- Most patients had disease duration of 2-5 years (38%),
- 42% currently had no signs of PC (no symptoms/pain, stable/undetectable prostate-specific antigen (PSA) levels, no signs of cancer on imaging)

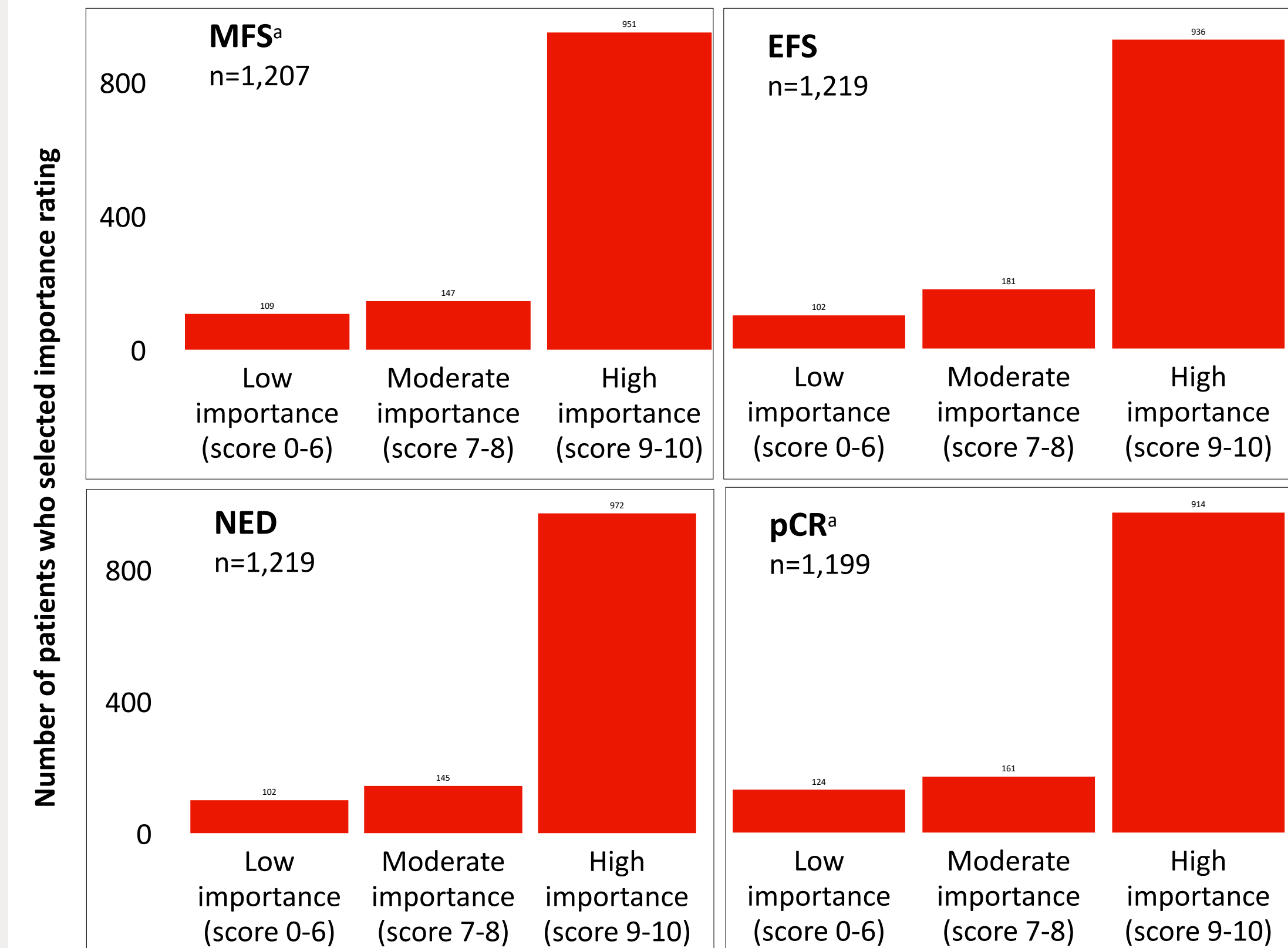
Figure 1: Patient survey population



### Importance of ICEs

- Among all patients, 79%, 77%, 80%, and 76% rated MFS, EFS, NED, and pCR as high importance, respectively (Figure 2)
- The mean (SD) importance scores were 9.1 (1.8) for MFS, 9.0 (1.7) for EFS, 9.1 (1.7) for NED, and 9.0 (1.8) for pCR

Figure 2: Patient ratings of ICE importance



<sup>3</sup> Patients with mPC could choose to skip questions related to MFS and pCR. A total of 12 and 20 patients preferred not to provide a rating for the MFS and pCR endpoints, respectively. Note: scores of 0 to 6, 7 or 8, and 9 or 10 were considered to reflect low, moderate, and high importance, respectively

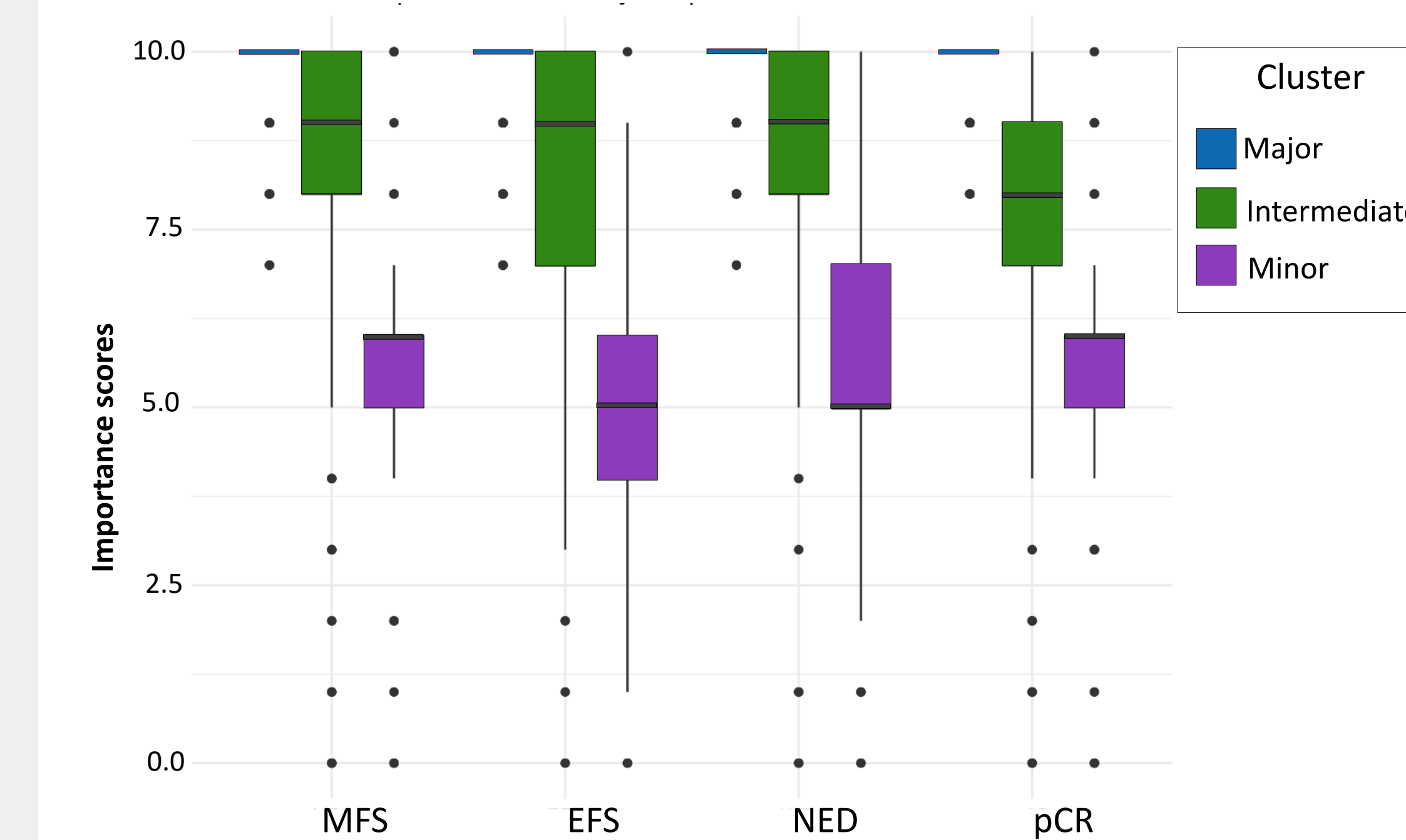
## References

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### Clusters of patients with similar patterns of ICE importance scores

- The HCA yielded three distinct clusters (Figure 3):
  - “Major Importance” (Blue) cluster → participants who systematically rated all endpoints as highly important, reflecting a uniform prioritization of ICEs
  - “Intermediate Importance” (Green) cluster → individuals who assigned intermediate scores to the endpoints, heterogeneity driven by MFS and pCR
  - “Minor Importance” (Purple) cluster → substantial internal variability, suggesting diverse perspectives, although participants generally assigned lower importance scores to the endpoints

Figure 3: Distribution of importance scores by endpoint and cluster



The boxplots display the distribution of importance scores assigned to the four endpoints (MFS, EFS, NED, pCR) across the three patient clusters identified through HCA.

### Patient characteristics by importance cluster

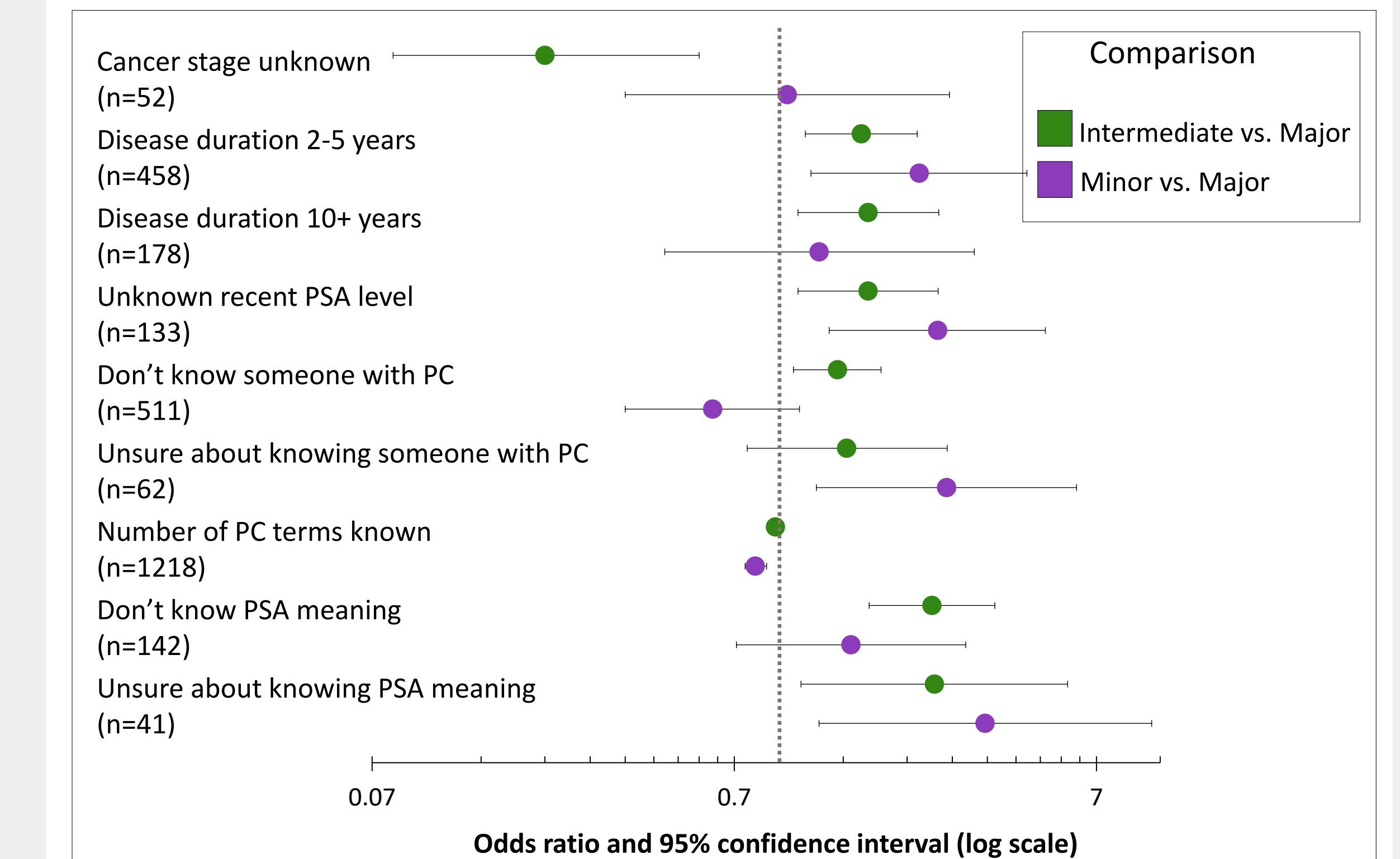
- To investigate how importance clusters relate to patients' characteristics and personal experiences with PC, the following characteristics were considered:
  - Prostate cancer knowledge and awareness:** understanding of PSA level meaning, knowledge of their most recent PSA level, knowledge of their Gleason score, and familiarity with a list of PC terminology
  - Clinical characteristics:** PC stage and duration
  - Personal experience and engagement with PC:** knowing a relative, friend or acquaintance affected by PC, and prior clinical trial participation
- Descriptive analyses showed a gradient across clusters:
  - Major importance cluster → greater PC knowledge and awareness, stronger personal engagement
  - Intermediate/Minor importance clusters → lower disease awareness, longer disease duration, fewer personal connections to PC

- Patients rated the importance of each ICE using a scale from 0 (not important) to 10 (very important)

### Cluster analysis

- Clustering methods were used to group patients into “profiles” based on how they valued each ICE
- Principal Component Analysis (PCA) was applied to reduce data complexity
- Patients with similar importance score patterns were grouped using Hierarchical Clustering Analysis (HCA) on principal components
- Associations between patient characteristics and importance profiles were examined in bivariate analyses and multinomial logistic regression (MNL)
- The final model was obtained using a backward elimination procedure: for each retained predictor, odds ratios (ORs) were calculated and reported along with their 95% confidence intervals (CIs) and corresponding p-values

Figure 4: Association between patient characteristics and importance score clusters



Forest plot of ORs and 95% CIs for characteristics presenting significant associations with cluster membership in the reduced MNL model (backward selection). The “Major importance” cluster is the reference group. ORs >1 indicate higher odds of membership in the Intermediate/Minor clusters, whereas ORs <1 indicate lower odds. ORs are displayed on a log scale; CIs crossing the dotted vertical line at 1 indicate no significant association.

### Interpretation of MNL results

- PC knowledge and awareness → lower odds of belonging to the Intermediate or Minor importance clusters relative to the Major importance cluster (Figure 4)
- Longer disease duration → increased odds of membership in the Intermediate/Minor clusters
- No personal connections to PC → Intermediate/Minor cluster membership
- Clinical factors → weaker associations overall

### Major importance (n=805)

- Shorter disease duration (<2 years)
- Greater PC knowledge (familiar with terminology, PSA meaning, recent PSA level known)
- Personal connections to prostate cancer

### Minor importance (n=80)

- Cancer stage unknown
- Longer disease history (2-10 yrs)
- Very limited disease awareness (few PC terms known, don't know about PSA meaning)
- No knowledge of recent PSA level
- Unsure about PC personal connections

### Figure 5: Characteristics of importance clusters

#### Intermediate importance (n=334)

- Intermediate disease history (2-5 yrs)
- Limited PC knowledge (fewer terms known, unsure or don't know PSA meaning)
- No knowledge of recent PSA level
- No personal connections to PC

Knowledge and experience drive cluster membership

