

# Patient-Reported Outcomes strategy in oncology drug development trials: Any consistencies in the approach to evaluate patient's quality of life between Head and Neck Cancer and Multiple Myeloma?

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## Background & objectives

- The objective of this study was to identify consistencies and differences in Health-Related Quality of Life (HRQoL) Patient Reported Outcomes (PRO) endpoint strategy across clinical trials from two oncology indications. Using COA Accelerator to gather our dataset, we decided to include two indications with numerous endpoints available for analysis. This selection led us to compare the following indications:
- Multiple Myeloma (MM)
- Head and Neck cancer (H&N)

## Methods

- IQVIA's COA Accelerator platform was used to retrieve HRQoL PRO endpoints from two indications: Multiple Myeloma and Head and Neck Cancer
- The search was performed on Clinical trials conducted between 2006 to 2023
- HRQoL-related endpoints measured using PROs were then identified and extracted out of the total pool of endpoints for both indications
- An endpoint was considered HRQoL-related when a PRO score (Total, domain(s), item(s), or customized) evaluating QoL was used to measure the endpoint
- Then, we defined a set of variables of interest for this study based on COA Accelerator's classifications: PRO type (generic, oncology – specific, or disease-specific), endpoint position (primary, secondary, or exploratory), and endpoint type (change from baseline, descriptive statistic, time to event)

**Figure 1. Screenshot from COA Accelerator Clinical Study search**

## Results

- COA Accelerator yielded a total pool of 141 clinical trials from its database. Out of those, 82 trials were found for MM, and 59 trials for H&N
- Out of those 141 clinical trials, a total 237 HRQoL PRO endpoints were identified out of which, 152 were from MM, and 85 from H&N
- Endpoint positions (primary, secondary, and exploratory) distribution was found as follow: 2; 142; 7 for MM and 0; 68; 17 for H&N respectively. One endpoint position was not available for MM
- Regarding endpoint type (change from baseline, descriptive statistic, time to event), the distribution was found as follow: 116; 14; 13 for MM and 52; 23; 9 for H&N respectively. Also, 9 endpoints types were not available for MM, and 1 for H&N

### PROs used in MM

- PROs identified in HRQoL endpoints were described for Multiple Myeloma in **Table 1a**, and for Head and Neck Cancer in **Table 1b**

**Table 1a. HRQoL PRO Endpoints in Multiple Myeloma**

PROs	Generic/specific	Number of endpoints
EQ-5D Total	Generic	38 (25%)
EQ-5D	Generic	2 (1.3%)
EQ-5D-3L	Generic	3 (2%)
EQ-5D-5L	Generic	33 (21.7%)
EORTC QLQ-C30	Oncology-specific	74 (48.3%)
FACT-G	Oncology-specific	1 (0.7%)
EORTC QLQ-MY20	MM-specific	36 (24%)
FACT-MM	MM-specific	3 (2%)
<b>Total (Percentage)</b>		<b>152 (100%)</b>

EQ-5D: Euroqol EQ-5D; EQ-5D-3L: EuroQol-5 dimensions-3 levels – Self-complete version on paper; EQ-5D-5L: EuroQol-5 dimensions-5 levels – Self-complete version on paper; EORTC QLQ-C30: EORTC Quality of Life Questionnaire – Core Questionnaire; FACT-G: Functional Assessment of Cancer Therapy – General; EORTC QLQ-MY20: EORTC Quality of Life Questionnaire – Multiple Myeloma Module; FACT-MM: Functional Assessment of Cancer Therapy – Multiple Myeloma

- Out of the 152 HRQoL PRO endpoints, the EORTC QLQ-C30 represented nearly 50% of the PROs used to measure quality of life
- Only 26% of the HRQoL endpoints identified used a MM-specific PRO to assess quality of life in multiple myeloma. The remaining 74% of the endpoints used either generic, or oncology-specific PROs

### PROs used in H&N

**Table 1b. HRQoL PRO Endpoints in Head & Neck Cancer**

PROs	Generic/Specific	Number of endpoints
EQ-5D Total	Generic	12 (14%)
EQ-5D	Generic	2 (2.3%)
EQ-5D-3L	Generic	3 (3.5%)
EQ-5D-5L	Generic	7 (8.2%)
EORTC QLQ-C30	Oncology-specific	38 (45%)
FACT-G	Oncology-specific	1 (1.2%)
EORTC QLQ-H&N35	H&N-specific	24 (28%)
FACT-HN	H&N-specific	10 (11.8%)
<b>Total (Percentage)</b>		<b>85 (100%)</b>

EQ-5D: Euroqol EQ-5D; EQ-5D-3L: EuroQol-5 dimensions-3 levels Self complete version on paper; EQ-5D-5L: EuroQol-5 dimensions-5 levels Self complete version on paper; EORTC QLQ-C30: EORTC Quality of Life Questionnaire – Core Questionnaire; FACT-G: Functional Assessment of Cancer Therapy – General; EORTC QLQ-MY20: EORTC Quality of Life Questionnaire – Multiple Myeloma Module; FACT-MM: Functional Assessment of Cancer Therapy – Multiple Myeloma

- As for Head and Neck, the most represented PRO in HRQoL endpoints was the EORTC QLQ-C30, representing 45% of the HRQoL PRO endpoints
- Out of the 85 endpoints, 34 were measured using a H&N-specific PRO, which represents almost 40% of the endpoint pool
- Nevertheless, generic and oncology-specific PROs still represented over 60% of HRQoL endpoints in Head and Neck Cancer

### HRQoL PRO Endpoints in Multiple Myeloma

- As per our methods, we then classified HRQoL PRO Endpoints for MM according to endpoint type and endpoint position (**Table 2a**; **Table 2b**)

**Table 2a. HRQoL PRO Endpoints Position in Multiple Myeloma**

Endpoint position	PROs	Primary	Secondary	Exploratory	Not Available
EQ-5D Total		0	37	1	0
EQ-5D		0	2	0	0
EQ-5D-3L		0	3	0	0
EQ-5D-5L		0	32	1	0
EORTC QLQ-C30		1	69	3	1
FACT-G		0	1	0	0
EORTC QLQ-MY20		1	33	2	0
FACT-MM		0	2	1	0
<b>Total (Percentage)</b>		<b>2 (1.3%)</b>	<b>142 (93.4%)</b>	<b>7 (4.6%)</b>	<b>1 (0.7%)</b>

EQ-5D: Euroqol EQ-5D; EQ-5D-3L: EuroQol-5 dimensions-3 levels – Self-complete version on paper; EQ-5D-5L: EuroQol-5 dimensions-5 levels – Self-complete version on paper; EORTC QLQ-C30: EORTC Quality of Life Questionnaire – Core Questionnaire; FACT-G: Functional Assessment of Cancer Therapy – General; EORTC QLQ-MY20: EORTC Quality of Life Questionnaire – Multiple Myeloma Module; FACT-MM: Functional Assessment of Cancer Therapy – Multiple Myeloma

**Table 2b. HRQoL PRO Endpoints Type in Multiple Myeloma**

Endpoint type	PROs	Change from baseline	Descriptive statistic	Time to event	Not available
EQ-5D Total		28	5	4	1
EQ-5D		2	0	0	0
EQ-5D-3L		1	1	0	1
EQ-5D-5L		25	4	4	0
EORTC QLQ-C30		52	9	9	4
FACT-G		1	0	0	0
EORTC QLQ-MY20		32	0	0	4
FACT-MM		3	0	0	0
<b>Total (Percentage)</b>		<b>116 (76.3%)</b>	<b>14 (9.2%)</b>	<b>13 (8.6%)</b>	<b>9 (5.9%)</b>

EQ-5D: Euroqol EQ-5D; EQ-5D-3L: EuroQol-5 dimensions-3 levels – Self-complete version on paper; EQ-5D-5L: EuroQol-5 dimensions-5 levels – Self-complete version on paper; EORTC QLQ-C30: EORTC Quality of Life Questionnaire – Core Questionnaire; FACT-G: Functional Assessment of Cancer Therapy – General; EORTC QLQ-MY20: EORTC Quality of Life Questionnaire – Multiple Myeloma Module; FACT-MM: Functional Assessment of Cancer Therapy – Multiple Myeloma

### HRQoL PRO endpoints in head and neck cancer

- As for Multiple Myeloma, the 85 HRQoL PRO endpoints for Head and Neck Cancer were classified according to endpoint position and endpoint type (**Table 3a**; **3b**)

**Table 3a. HRQoL PRO Endpoints Position in Head & Neck Cancer**

Endpoint position	PROs	Primary	Secondary	Exploratory	Not Available
EQ-5D Total		0	9	3	0
EQ-5D		0	2	0	0
EQ-5D-3L		0	1	2	0
EQ-5D-5L		0	6	1	0
EORTC QLQ-C30		0	34	4	0
FACT-G		0	0	1	0
EORTC QLQ-H&N35		0	21	3	0
FACT-HN		0	4	6	0
<b>Total (Percentage)</b>		<b>0 (0%)</b>	<b>68 (80%)</b>	<b>17 (20%)</b>	<b>0 (0%)</b>

EQ-5D: Euroqol EQ-5D; EQ-5D-3L: EuroQol-5 dimensions-3 levels Self complete version on paper; EQ-5D-5L: EuroQol-5 dimensions-5 levels Self complete version on paper; EORTC QLQ-C30: EORTC Quality of Life Questionnaire Core Questionnaire; FACT-G: Functional Assessment of Cancer Therapy – General; EORTC QLQ-MY20: EORTC Quality of Life Questionnaire Multiple Myeloma Module; FACT-MM: Functional Assessment of Cancer Therapy – Multiple Myeloma

**Table 3b. HRQoL PRO Endpoints Type in Head & Neck Cancer**

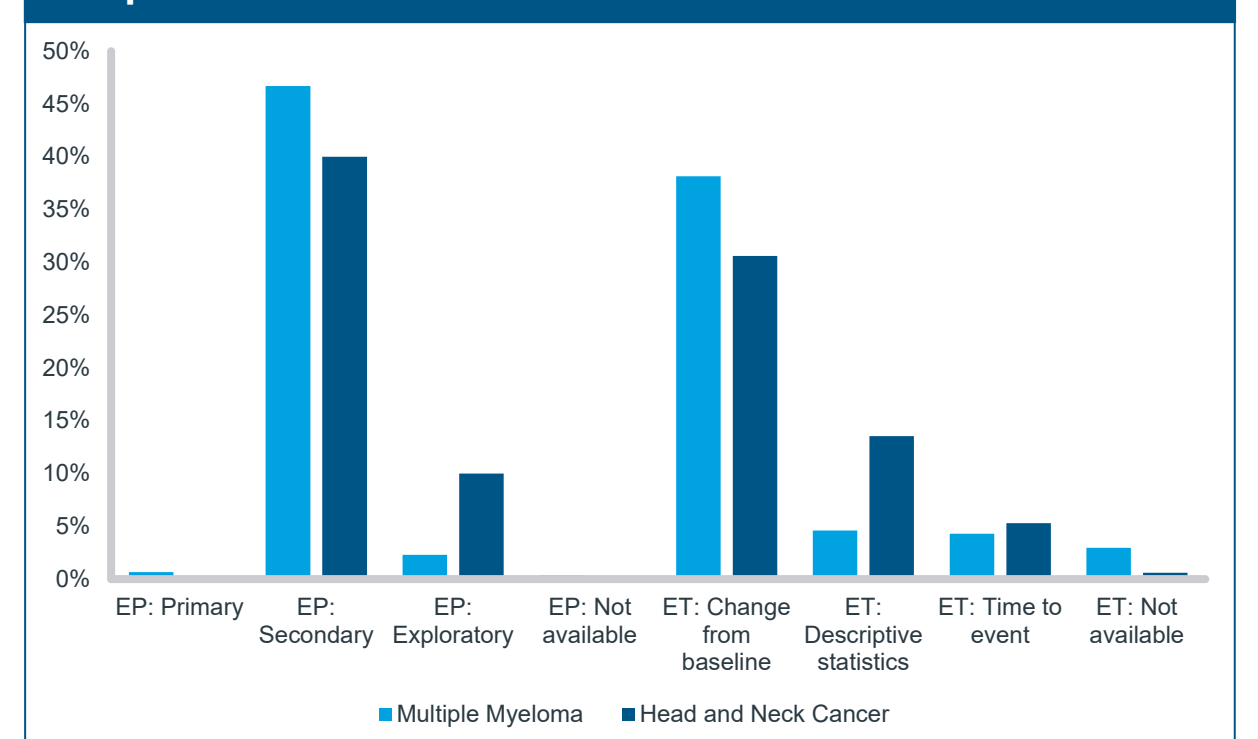
Endpoint type	PROs	Change from baseline	Descriptive statistic	Time to event	Not available
EQ-5D Total		5	6	1	0
EQ-5D		2	0	1	0
EQ-5D-3L		1	1	0	0
EQ-5D-5L		2	5	0	0
EORTC QLQ-C30		21	10	7	0
FACT-G		0	1	0	0
EORTC QLQ-H&N35		22	0	1	1
FACT-HN		4	6	0	0
<b>Total (Percentage)</b>		<b>52 (61.2%)</b>	<b>23 (27.1%)</b>	<b>9 (10.6%)</b>	<b>1 (1.2%)</b>

EQ-5D: Euroqol EQ-5D; EQ-5D-3L: EuroQol-5 dimensions-3 levels – Self-complete version on paper; EQ-5D-5L: EuroQol-5 dimensions-5 levels – Self-complete version on paper; EORTC QLQ-C30: EORTC Quality of Life Questionnaire – Core Questionnaire; FACT-G: Functional Assessment of Cancer Therapy – General; EORTC QLQ-MY20: EORTC Quality of Life Questionnaire – Multiple Myeloma Module; FACT-MM: Functional Assessment of Cancer Therapy – Multiple Myeloma

### HRQoL PRO Endpoints comparison between MM and HNC

- Similarities can be observed for HRQoL PRO endpoints between the two indications (**Figure 1**)
- Endpoint positions between the two indications were consistent, with 87% of the 237 endpoints being measured as secondary endpoints. Only roughly 1% of endpoints were measured as a primary endpoint
- Endpoint types between the two indications presented also similarities, with 69% of the 237 endpoints being measured as a change from baseline

**Figure 2. Evaluation of consistencies in PRO HRQoL Endpoints between MM and H&N**



EP: Endpoint Position; ET: Endpoint Type

## Conclusions

- Clear consistencies were observed regarding how HRQoL PRO endpoints are being measured between the two oncology indications
- Secondary endpoint was the most represented endpoint position, covering nearly 87% of the total endpoint sample
- Change from baseline was the most represented endpoint type, covering over 68% of the total endpoint sample
- Oncology-specific & generic PROs represented 67% of the total endpoint sample
- Further research should be conducted to understand the consistent use of generic HRQoL PROs despite the existence of validated disease specific questionnaires

### Acknowledgments

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