10.1 PROMS COMMONLY USED IN MYELOMA STUDIES

10.1.1 EORTC-QLQ-C30

The EORTC QLQ-C30 is a disease-specific questionnaire developed to capture more clinically meaningful differences and assess the QOL in cancer patients (Fragola, 2020, Proskorovsky et al., 2014). EORTC QLQ-C30 is a widely-used, validated, 30-item survey designed with multi-item modules and single-item measures. These include five functional scales (Physical, Role, Cognitive, Emotional and Social Functioning), three symptom scales (Fatigue, Pain and Nausea/Vomiting), a Global Health Status/ QoL scale and six single items (Constipation, Diarrhoea, Insomnia, Dyspnoea, Appetite Loss and Financial Difficulties) (Fragola, 2020, Proskorovsky et al., 2014). Single items are also assessed to evaluate other symptoms described by patients, including shortness of breath, loss of appetite changes, difficulty sleeping, changes in bowel patterns and the financial burden of being affected by illness (Fragola, 2020).

10.1.2 EORTC QLQ-MY24 AND QLQ-MY20

The EORTC QLQ-MY24 is an instrument recommended as a supplement to the EORTC QLQ-C30 instrument for patients with MM. The module consisted of 24 questions but was later redeveloped into a 20-question module (EORTC QLQ-MY20). The QLQ-MY20 addressed four important domains in MM: Disease Symptoms, Side Effects of Treatment, Body Image and Future Perspectives (Sonneveld et al., 2013). Three of the four domains are multi-item scales: Disease Symptoms (including bone aches or pain, back pain, hip pain, arm or shoulder pain, chest pain and pain increasing with activity); Side Effects of Treatment (including drowsiness, thirst, feeling ill, dry mouth, hair loss, upset by hair loss, tingling hands or feet, restlessness/agitation, acid indigestion/heartburn and burning or sore eyes); and Future Perspective (including worrying about death and health in the future, and thinking about illness). The Body Image scale is a single-item scale that addresses physical attractiveness (Proskorovsky et al., 2014).

10.1.3 FACT-G

The FACT-G (Functional Assessment of Cancer Therapy-General) is a questionnaire that is part of a series aimed at assessing QoL in cancer patients receiving treatment. It features 27 items composed of general questions organised into four core dimensions of QoL: physical well-being, social/family well-being, emotional well-being and functional well-being. The FACT series of tools are built around a core, plus another module that contains questions related to different malignancies (Yost et al., 2013). For example, FACT-Multiple Myeloma (FACT-MM), FACT-Lymphoma (FACT-Lym), FACT–anaemia (FACT-An) and FACT–bone marrow transplant questionnaire (FACT-BMT) (Osborne et al., 2012).

10.1.4 FACT-GOG – NTX

Chemotherapy-induced peripheral neuropathy (CIPN) is a common neurological symptom in individuals receiving chemotherapy. It frequently impairs everyday functioning and QoL, as well as causes psychological discomfort and limited socialising. The Functional Assessment of Cancer Therapy/ Gynaecologic Oncology Group—Neurotoxicity (FACT/GOG-Ntx) is a validated, 11-item PRO measure developed to assess CIPN symptoms. It was first created by the FACIT organisation in partnership with

the Gynaecologic Oncology group and is now integrated into the FACT-G questionnaire's core quality of life measure. Each item is rated on a five-point scale (0 = not at all, 4 = very much), with a higher score indicating a more severe case of CIPN. It is accessible in a number of different languages (Cheng et al., 2020).

10.1.5 MYPOS

The Myeloma Patient Outcome Scale (MyPOS) is a validated QoL PROM that was designed specifically for patients with MM. MyPOS was created by UK academics and has a component of the Integrated Palliative/Patient Care Outcome Scale (IPOS), a multifaceted questionnaire for assessing issues in palliative care patients. MyPOS contains the main components of the IPOS along with myeloma-specific disease questions. The MyPOS consists of 13 symptoms and 20 QoL measures, each rated on a 5-point Likert scale (Gerlach, 2019).

10.1.6 EQ-5D (EQ-5D-3L) AND EQ-5D-5L

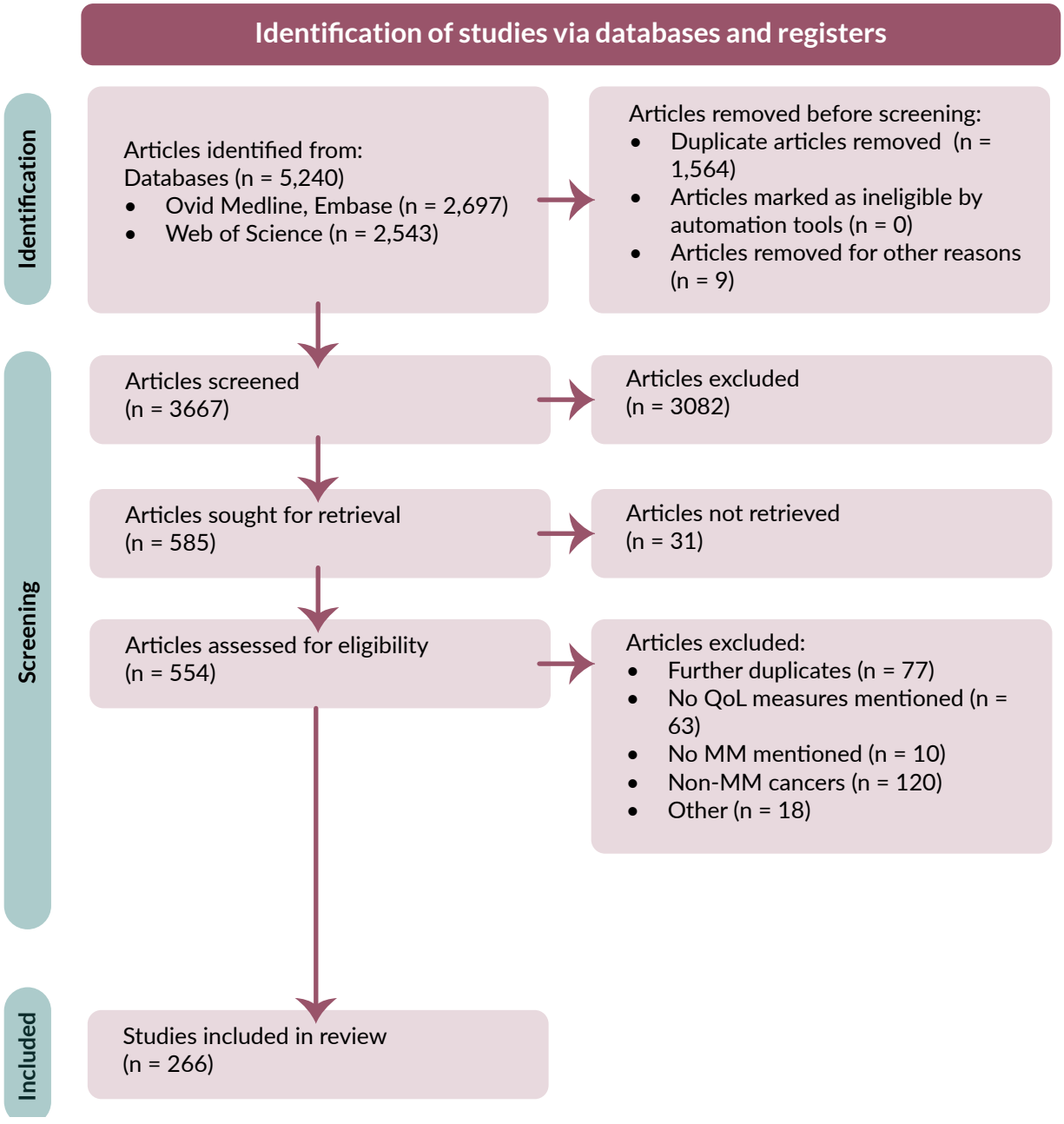
The most common generic preference-based instrument is the EuroQol-5 Dimensions (EQ-5D or EQ-5D-3L). The EuroQol Group initially introduced the 3-level EQ-5D (EQ-5D-3L) version and then the 5-level EQ-5D version (EQ-5D-5L) in the 1990s. Compared to the EQ-5D-3L, the EQ-5D-5L was designed to enhance the instrument's sensitivity and minimise ceiling effects. Both tools are simple, quick and can be applied to patients with any disease type and are accessible in over 150 languages, with a variety of management options (paper, digital, phone, through caregivers and face-to-face interviews) (EUROQOL).

The EQ-5D-3L and the EQ-5D-5L consist of the EQ-5D descriptive system and the EQ visual analogue scale (EQ-VAS). The descriptive system is composed of five dimensions (mobility, self-care, usual activities, pain/discomfort and anxiety/depression). In the EQ-5D-3L version, each dimension has three levels (no problems, some problems and extreme problems). In the EQ-5D-5L version, each dimension has five levels (no problems, slight problems, moderate problems, severe problems and extreme problems). The dimension helps to describe the patient's health state. The EQ VAS collects data on the patient's self-rated health using a vertical visual analogue scale with endpoints labelled 'Best imaginable health state' and 'Worst imaginable health state'. The VAS can be used as a quantitative outcome measure representing the patient's subjective judgement (EUROQOL).

10.1.7 MAPPING

Preference-based instruments are essential from an economic standpoint since they can be easily implemented at population level for economic analysis. Algorithms for mapping QoL values from the core and disease-specific modules to generic utility values are an alternate method. Numerous research (Wu et al., 2007, Kontodimopoulos et al., 2009, McKenzie and Van der Pol, 2009,, Crott and Briggs, 2010, Versteegh et al., 2012, Gray et al.,2021) has established the viability of mapping EORTC QoL data to EQ-5D scores in cancer patients.

10.1.8 FIGURE 1: QOL MYELOMA LITERATURE SEARCH PRISMA FLOW DIAGRAM



10.1.9 TABLE 1: SUMMARY OF INSTRUMENTS IDENTIFIED IN LITERATURE RESEARCH

Setting used	Abbreviation	Definition
Clinical Practice	AIS	The American Spinal Injury Association impairment scale
Research and Clinical Practice	BDI	The Beck Depression Inventory
	BFI	Brief Fatigue Inventory
Research	BFI	Brief Fatigue Inventory
Research and Clinical Practice	BPI	Brief Pain Inventory
Research and Clinical Practice	BPI-SF	Brief Pain Inventory-Short Form
Clinical Practice	BQ-13	Barriers Questionnaire - 13 items
Research and Clinical Practice	Brief-COPE	The Brief-Coping Orientation to Problems Experienced
Research	CarerQoL 7D	CarerQol subjective burden
Research	CarGOQoL	CareGiver Oncology Quality of Life Questionnaire
Research and Clinical Practice	CES-D (CES-D-10)	Center for Epidemiologic Studies Depression Scale
	CQOLC	The Caregiver Quality of Life Index-Cancer
Clinical Practice	CPR	Clinical Pain Response
Research and Clinical Practice	CQOLC	The Caregiver Quality of Life Index-Cancer
Research	CTC-AE (CTCAE, CTC or NCI-CTC, PRO-CTCAE)	The Common Terminology Criteria - Adverse Events
Clinical Practice	DASS-21	The Depression, Anxiety and Stress Scale - 21 Items
	EORTC QLQ-BM22	The European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire - Bone Metastases Module
Clinical Practice	DN-4	The Douleur Neuropathique 4 Questions
Research	EORTC QLQ-30	The European Organisation for Research and Treatment of Cancer Quality of Life questionnaire
Research	EORTC QLQ-BM22	The European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire - Bone Metastases Module
	EORTC QLQ-ELD15	The European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire - Elderly Module
Research	EORTC QLQ-BR23	The European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire - Breast-specific Module
Research	EORTC QLQ-C15-PAL	The European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire Core 15 Palliative Care
Research	EORTC QLQ-CIPN20	The European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire - Chemotherapy-Induced Peripheral Neuropathy Module
Research	EORTC QLQ-ELD15	The European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire - Elderly Module
Research	EORTC QLQ-MY20	The European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire - Multiple Myeloma Module
Research	EORTC QLQ-MY24	The European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire - Multiple Myeloma Module
Research	EQ-5D-3L	European Quality of Life Five Dimension - Three Levels
Research	EQ-5D-5L	European Quality of Life Five Dimension - Five Levels
Clinical Practice	ESAS	Edmonton Symptom Assessment Scale (palliative care)
Research and Clinical Practice	ESS	Epworth Sleepiness Scale

Research	FACT-An	The Functional Assessment of Cancer Therapy - Anaemia
Research	FACT-BMT	The Functional Assessment of Cancer Therapy – Bone Marrow Transplant Module
Research	FACIT-F	The Functional Assessment of Chronic Illness Therapy – Fatigue Module
Research	FACT-G (FACIT)	Functional Assessment of Cancer Therapy - General
Research	FACT-GOG - Ntx	Functional Assessment of Cancer Therapy - Gynaecologic Oncology chemotherapy-induced peripheral neuropathy (Ntx) module
Research	FACT-MM	The Functional Assessment of Chronic Illness Therapy – Multiple Myeloma Module
Clinical Practice	FAS	Fatigue Assessment Scale
Research and Clinical Practice	FSFI	The Female Sexual Function Index
Clinical Practice	FSI	Fatigue Symptom Inventory
Clinical Practice	FSS	Fatigue Severity Scale
Clinical Practice	GAD-7	Generalised Anxiety Disorder Questionnaire
Clinical Practice	GHQ	General Health Questionnaire
	HAP	Human Activity Profile
Clinical Practice	GDS	Geriatric Depression Scale
Research	GLTEQ	Godin Leisure-Time Exercise Questionnaire
Clinical Practice	HADS	Hospital Anxiety and Depression Scale
Research and Clinical Practice	HAP	Human Activity Profile
Research and Clinical Practice	HRQoL-Q	Health Related Quality of Life Questionnaire
Clinical Practice	IADL	Instrumental Activities of Daily Living
Clinical Practice	ICPN	Indication Common Toxicity Criteria Peripheral Neuropathy Questionnaire
Clinical Practice	IES	Impact of Event Scale
Research and Clinical Practice	IIEF	The International Index of Erectile Function
Clinical Practice	LANSS	The Leeds Assessment of Neuropathic Symptoms and Signs
Research and Clinical Practice	MAC	Mental Adjustment Scale to the Cancer Scale Partner
Clinical Practice	MARS-5	The Medication Adherence Report Scale - 5-item version
Research	MCS	Mental Health Composite Scale
Research and Clinical Practice	MCSQ	The Multilingual Corpus of Survey Questionnaires
Research and Clinical Practice	MDASI	M. D. Anderson Symptom Inventory
Research and Clinical Practice	MDASI-MM	The MD Anderson Symptom Inventory for Multiple Myeloma
Clinical Practice	MGT	Morisky-Green Test
Clinical Practice	MMSE	Mini-Mental State Examination
Clinical Practice	MoCA	The Montreal Cognitive Assessment
Clinical Practice	MOS-SSS	Medical Outcomes Study-Social Support Survey
Clinical Practice	M-QoL8	8-question Quality of Life tool to assess mental health in myeloma patients
Research and Clinical Practice	MSAS-SF	The Memorial Symptom Assessment Scale – Short Form (MSAS-SF)
Clinical Practice	MyPOS	Myeloma Patient Outcome Scale
Clinical Practice	MySIm-Q	The Multiple Myeloma Symptom and Impact Questionnaire
Clinical Practice	NCCN- DT	National Comprehensive Cancer Network - Distress Thermometer Score
Clinical Practice	NPS	Neuropathy Pain Scale
Clinical Practice	NRS	Numerical Rating Scale

Research and Clinical Practice	ODI	The Oswestry Disability Index (also known as the Oswestry Low Back Pain Disability Questionnaire)
Clinical Practice	OMDQ	Oral Mucositis Daily Questionnaire
Clinical Practice	PCL-C	Post-Traumatic Stress Disorder Checklist - Civilian version
Clinical Practice	PGIC/S	Patient Global Impression of Change/Severity
Research and Clinical Practice	PHQ-8	Patient Health Questionnaire depression scale – 8 items
Research	PQAT	Patient's Qualitative Assessment of Treatment
Research	PQA-RWR	Patient's Qualitative Assessment of Treatment- Real World
Research and Clinical Practice	PREMs	Patient-Reported Experience Measures
Clinical Practice	PRI	Patient-Reported Information
Research and Clinical Practice	PROMIS	The Patient-Reported Outcomes Measurement Information System
Research and Clinical Practice	PROMIS-SD	Patient-Reported Outcomes Measurement Information System - Sleep Disturbance
Clinical Practice	PROs	Patient-Reported Outcomes
Clinical Practice	PSQI	Pittsburgh Sleep Quality Index
Clinical Practice	RMQ	Roland-Morris Disability Questionnaire
Clinical Practice	RPER	(Borg) Rating of Perceived
Clinical Practice	SAGE	The Self-Administered Gerocognitive Exam
Research and Clinical Practice	SEIQoL-DW	The Schedule for the Evaluation of the Individual Quality of Life - Direct Weighting
Clinical Practice	SF-12	Short Form 12 Health Survey Questionnaire
Clinical Practice	SF-36	Short Form 36 Health Survey Questionnaire
Clinical Practice	SF-SUNS	Short Form Survivor Unmet Needs
Clinical Practice	SPARC	Sheffield Profile for Assessment and Referral for Care
Clinical Practice	SPPB	Short Physical Performance Battery
Research and Clinical Practice	SSSS	Social Support Satisfaction Scale
Research	TSQM-9	Treatment Satisfaction Questionnaire for Medication
Clinical Practice	TUGT	Timed Up and Go Test
Research	VAS	Visual Analogue Scale
Research	VRS	Verbal Rating Scale

10.1.9 TABLE 2: SUMMARY OF QOL DATA SUBMITTED IN MM NICE TA

TA	Company Name	Year	Process	Clinical Trial(s) used in TA	Clinical trial/Literature QoL Data Collected	QoL Instruments	Mapping	QoL data caused uncertainty	Quality of QoL Data
TA228	Janssen Celgene	2011	MTA	VISTA IFM99/06 IFM 01/01 GIMEMA (MRC) Myeloma IX HOVON	VISTA	EORTC QLQ C-30 EQ-5D	Y	Y	0
TA311	Janssen	2014	STA	GIMEMA IFM 2005-01 PETHEMA GEM05MENOS65	van Agthoven et al. (2004)	EQ-5D	N	N	1
TA380	Novartis	2016	STA	PANORAMA-1 Acaster et al. 2013	PANORAMA-1 Acaster et al.2013	EORTC QLQ-C30 EORTC-MY20 EQ-5D	Y	N	1
TA427 (TA338)	Celgene	2015	STA	MM-003	MM-003	EORTC QLQ-C30 EORTC QLQ-MY20 EQ-5D	N	N	1
TA505	Takeda	2018	STA	TMM1	TMM1	EQ-5D	N	N	1
TA510	Janssen	2018	STA	MMY2002 GEN501	Palumbo et al. (2013)	EQ-5D EQ-5D-5L	N	N	1
TA573	Janssen	2018	STA	CASTOR ENDEAVOR	CASTOR ENDEAVOR	EQ-5D-5L EQ-5D-3L	Y	Y	1
TA586 (TA171)	Celgene	2019	STA	MM-009 MM-010	van Agthoven et al. (2004)	EQ-5D	N	Y	0
TA587	Celgene	2019	STA	FIRST MM-020	FIRST MM-020	EQ-5D EORTC QLQ-C30	Y	Y	0
TA657 (TA457)	Amgen	2020	STA	ENDEAVOR ASPIRE	ENDEAVOR	EORTC QLQ-C30	Y	N	1
TA658	Sanofi	2020	STA	ICARIA-MM	ICARIA-MM	EQ-5D-5L EQ-5D-3L	Y	N	1

10.1.9 TABLE 2: SUMMARY OF QOL DATA SUBMITTED IN MM NICE TA

TA	Company Name	Year	Process	Clinical Trial(s) used in TA	Clinical trial/Literature QoL Data Collected	QoL Instruments	Mapping	QoL data caused uncertainty	Quality of QoL Data
TA680	Celgene	2021	STA	Myeloma XI CALGB GIMEMA	Acaster et al. (2013)	EQ-5D-3L	N	Y	0
TA695	Amgen UK	2021	STA	ASPIRE	ASPIRE	EORTC QLQ-C30 EORTC QLQMY20 EQ-5D-3L	Y	Y	0
TA763	Janssen-Cilag	13-Jul-05	STA	CASSIOPEIA IFM 2005-01 GMMG-MM5	CASSIOPEIA van Agthoven et al. (2004)	EORTC) QLQ-C30 EQ-5D- 5L EQ-5D-3L	Y	N	1

Abbreviation: MTA, Multiple Technology Appraisal; STA, Single Technology Appraisal
Y= Yes; N= No
Quality of QoL 0=Poor; 1= Acceptable; 2 =Good

10.1.10 TABLE 3: SUMMARY OF CLINICAL TRIALS IDENTIFIED IN MM NICE TAS

Study (Trial Number)	Study Design	Line of Treatment	Quality of the Study (as graded by ERG/AG)	Location	Number of Participants Enrolled in Study (completed)	Mean age in years	Primary outcome	Secondary Outcome
IFM 99/06 (NCT00367185)	Phase 3 Open label RCT	Comparing Melphalan-Prednisone(MP) with MP-THALIDOMIDE and Autologous Stem Cell Transplantation in the treatment of newly-diagnosed elderly patients with MM	Poor	France	447	≥ 70	OS	RR PFS SaP AE
IFM 01/01 (NCT00644306)	Phase 3 Double blinded RCT	Comparing Melphalan-Prednisone (MP) with MP Plus Thalidomide in the treatment of newly-diagnosed very elderly patients (> 75 Years) with MM	Poor	France	232	≥ 80	OS	RR PFS AE
GIMEMA (NCT00232934)	Phase 3, Open-label RCT	Comparing Melphalan, Prednisone (MP) and Thalidomide vs MP in elderly Myeloma patients	Good	Italy	400	65-72	RR PFS EFS	OS AE
VISTA (NCT00111319)	Phase 3, Open-label RCT	Comparing Bortezomib, Melphalam and Prednisone vs Melphalan and Prednisone for previously untreated multiple Myeloma patients who were ineligible for high-dose therapy.	Moderate	22 countries in Europe, North and South America, and Asia	682	71	TTP	OS PFS ORR AE HRQol (EORTC QLQ-C30)
The Medical Research Council (MRC) Myeloma IX (ISRCTN68454111)	Phase 3, Double blinded RCT	Comparing the efficacy of Thalidomide, Cyclosphosphamide and Attenuated Dexamethasone (CTDa) vs Melphalan–Prednisone (MP) in newly-diagnosed symptomatic Myeloma patients or non-secretory MM	Good	UK	1930	59	OS PFS RR	HRQoL(EORTC QLQ- C30/.QLQ-MY24 and the EQ-5D) AE
GIMEMA (NCT01134484)	Phase 3, Open-label RCT	Comparing the efficacy and safety of 3 cycles of Bortezomib, Thalidomide and Dexamethasone with 3 cycles of Thalidomide and Dexamethasone as induction treatment before autologous stem cell transplantation. Also evaluating subsequent consolidation treatment consisting of 2 cycles of either Bortezomib, Thalidomide and Dexamethasone, or Thalidomide and Dexamethasone. Maintenance treatment with Dexamethasone was continued until disease progression or relapse	Poor	Italy	480	18 - 65	CCR	CRR TTP PFS OS AE

10.1.10 TABLE 3: SUMMARY OF CLINICAL TRIALS IDENTIFIED IN MM NICE TAS

Study (Trial Number)	Study Design	Line of Treatment	Quality of the Study (as graded by ERG/AG)	Location	Number of Participants Enrolled in Study (completed)	Mean age in years	Primary outcome	Secondary Outcome
PETHEMA (GEM05MENOS65) (NCT00461747)	Phase 3, Open-label RCT	Comparing the efficacy and safety of Bortezomib in combination with Thalidomide and Dexamethasone against Thalidomide and Dexamethasone in people with newly-diagnosed symptomatic MM and measurable disease (serum and/or urine M protein), who were eligible for autologous stem cell transplantation	Poor	Spain	390	<65	safety and efficacy of induction treatment	safety and efficacy of the maintenance treatments
IFM 2005-01 (NCT00200681)	Phase 3, Open label RCT	Comparing 4 cycles of the efficacy and safety of Bortezomib + Dexa (with or without consolidation treatment with Dexamethasone, Cyclophosphamide, Etoposide and Cis-platinum) with Vincristine, Doxorubicin and Dexa (with or without intensification)	Moderate	France	493	55.6	CR nCR	ORR CR nCR AE
PANORAMA- 1 (NCT01023308)	Phase 3, placebo-controlled, double-blind RCT	Comparing Panobinostat, Bortezomib and Dexamethasone with placebo, plus Bortezomib and Dexamethasone in patients with relapsed or relapsed and refractory multiple myeloma, and who have had 1-3 previous treatments	Good	34 countries in Europe, North and South America, Middle East, North Africa and Asia	767	62.1	PFS	OS ORR TTP TTR DoR AE HRQoL
MM- 003 (NCT01311687)	Phase 3, Open-label RCT	Comparing efficacy and safety of Pomalidomide in combination with low-dose Dexamethasone vs high-dose Dexamethasone in subjects with refractory multiple Myeloma or relapsed and refractory multiple Myeloma	Good	15 countries in Europe and North America and Australia	455	63.6	PFS	OS RR TTP TTR DoR TTF HRQoL (EORTC QLQ-C30)

10.1.10 TABLE 3: SUMMARY OF CLINICAL TRIALS IDENTIFIED IN MM NICE TAS

Study (Trial Number)	Study Design	Line of Treatment	Quality of the Study (as graded by ERG/AG)	Location	Number of Participants Enrolled in Study (completed)	Mean age in years	Primary outcome	Secondary Outcome
TOURMALINE-MM1 (TMM1) (NCT01850524)	Phase 3, double blinded RCT	Comparing Ixazomib (plus Lenalidomide and Dexamethasone) with Lenalidomide plus Dexamethasone.	Good	7 countries, including 2 North America. 3 European countries, Korea and New Zealand	705	73.6	PFS	OS TTP RR HRQoL (EQ-5D) AE
MMY2002 (NCT01985126)	Phase 3, Open-label randomised (dose) trial	2-part study investigating different doses of Daratumumab (Part 1: Daratumumab 16mg/kg licence dose weekly for weeks 1-8, Daratumumab 8mg/kg every 4 weeks; Part 2: Daratumumab 16mg/kg)	Poor	North America and Spain	124	63.5	ORR	OS PFS DoR
GEN501 (NCT00574288)	phase I and 2 open-label, non-randomised trial	2-part study investigating different doses of Daratumumab (Part 1: dose escalation of Daratumumab from 0.0005mg/kg to 24 mg/kg; Part 2: Daratumumab 8mg/kg, Daratumumab 16mg/kg)	Poor	Denmark, Netherlands, Sweden, United States	104	61.4	AE	OS PFS
CASTOR (NCT02136134)	Phase 3, Open-label RCT	Comparing Daratumumab plus Bortezomib plus Dexamethasone with Bortezomib plus Dexamethasone	Moderate	16 countries in Europe, North and South America and Asia, including Australia	500	63.3	PFS	OS TTD AE HRQoL (EQ-5D-5L)
MM-009 (NCT00056160)	Phase 3 trial Quadruple blinded, RCT	Comparing treatment with Lenalidomide plus Dexamethasone with placebo plus Dexamethasone in people with multiple Myeloma who had received at least 1 prior therapy	Good	North America	353	62.9	TTP	OS PFS RR AE
MM-010 (NCT00424047)	Phase 3 trial, Quadruple blinded, RCT	Comparing treatment with Lenalidomide plus Dexamethasone with placebo plus Dexamethasone in people with multiple Myeloma who had received at least 1 prior therapy	Good	Australia, Europe and Israel	351	62.6	TTP	OS PFS RR AE

10.1.10 TABLE 3: SUMMARY OF CLINICAL TRIALS IDENTIFIED IN MM NICE TAS

Study (Trial Number)	Study Design	Line of Treatment	Quality of the Study (as graded by ERG/AG)	Location	Number of Participants Enrolled in Study (completed)	Mean age in years	Primary outcome	Secondary Outcome
FIRST (MM-020) (NCT00689936)	Phase 3, Open-label RCT	Comparing Lenalidomide plus Dexamethasone with Thalidomide plus Melphalan plus Prednisone (MPT)	Moderate	21 countries in Europe, North and South America, Middle East, North Africa and Asia	1623	73.1	PFS	OS CR TTF AE TTNT RR HRQoL(EORTCQLQ C30)
ENDEAVOR (NCT01568866)	Phase 3, Open-label RCT	Comparing Carfilzomib plus Dexamethasone with Bortezomib plus Dexamethasone at second line	Good	26 countries in Europe, North and South America, Middle East, North Africa and Asia	929	65.0	PFS	OS OR DoR AE
ASPIRE (NCT01080391)	Phase 3 open-label RCT	Comparing Carfilzomib plus Lenalidomide and Dexamethasone with Lenalidomide plus Dexamethasone at third line	Good	20 countries in Europe, North America and Israel	792	63.9	PFS	OS ORR DCR DoR DDC HRQoL (QLQ-C30)
ICARIA-MM (NCT02990338)	Phase 3, Open-label RCT	Comparing Isatuximab, Pomalidomide, and Dexamethasone to Pomalidomide and Dexamethasone in refractory or relapsed and refractory multiple Myeloma patients	Moderate	24 countries in Europe, North and South America, Middle East, North Africa and Asia	307	65.9	PFS	ORR OS TTP DoR PRO with HRQoL (EQ-5D-5L)

10.1.10 TABLE 3: SUMMARY OF CLINICAL TRIALS IDENTIFIED IN MM NICE TAS

Study (Trial Number)	Study Design	Line of Treatment	Quality of the Study (as graded by ERG/AG)	Location	Number of Participants Enrolled in Study (completed)	Mean age in years	Primary outcome	Secondary Outcome
Myeloma XI (NCT01554852)	Phase 3, Open-label RCT	Lenalidomide maintenance treatment of multiple Myeloma after autologous stem cell transplantation	Good	UK	4420	61	PFS OS	RR AE
CALGB trial (NCT00114101)	Phase 3, double-blind, placebo-controlled, randomised trial	Compare Lenalidomide with placebo in treating patients with multiple Myeloma, who are undergoing autologous stem cell transplant	Good	US	460	59	TTP	OS RR
GIMEMA trial (NCT00551928)	phase 3, open label, RCT	Comparing the efficacy of the combination of Lenalidomide with low-dose Melphalan vs high-dose Melphalan in newly-diagnosed, symptomatic MM patients	Good	Italy	402	65>	PFS RR DoR	OS
CASSIOPEIA (NCT02541383)	Phase 3, Open-label RCT	Evaluating Daratumumab in transplant eligible participants with previously untreated multiple Myeloma	Good	France, Belgium, Netherlands	1,085	< 65	sCR PFS	PFS 2 TTP OS
GMMG-MM5 (EudraCT Number: 2010-019173-16)	Phase 3 randomised trial	Comparing Bortezomib, Cyclophosphamide and Dexamethasone with Doxorubicin plus Dexamethasone.	Good	Germany	540	>=65	PFS	OS RR AE
HOVON 49	Randomized Phase 3 study	Comparing myelo-ablative chemo-/ radiotherapy and autologous stem cell transplantation with only chemotherapy in patients with multiple Myeloma	Not graded	Holland	344	>65	EFS	RR OS PFS

Abbreviations: AE, Adverse Event: CRR, Complete Remission Rate; sCR, stringent Complete Response; DCR, Disease Control Rate; DoR, Duration of Response; EFS, Event-Free Survival; HRQoL, Health-Related Quality of Life; rrMM, relapsed refractory Multiple Myeloma; OS, Overall Survival; ORR, Overall Response Rate; PFS, Progression-Free Survival; PRO, Patient-Reported Outcome measured; RR, Response Rate; TTP, Time To Progression; TTR, Time To Response; TTF, Time to Treatment Failure; TTD, Time to Treatment Discontinuation; TTNT, Time To Next Treatment.

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