

# The value of progression-free survival from the perspectives of patients with multiple myeloma and treating physicians: qualitative research findings from eight countries

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## Introduction

- Advances in treatment options for patients with multiple myeloma (MM) have led to improved outcomes, and 5-year overall survival (OS) is now approximately 50%<sup>1</sup>
  - This long period of survival makes it challenging to rely on OS data for regulatory approvals and reimbursement decisions
- Due to these improved outcomes, progression-free survival (PFS) is being increasingly considered as an alternative endpoint in clinical trials investigating treatments for regulatory approval in patients with MM<sup>2</sup>; however, some countries do not routinely consider alternative endpoints such as PFS in assessments of early treatment benefit

## Objective

- This research assessed the value of efficacy endpoints, particularly PFS, in MM and evaluated how progression impacts patients’ quality of life (QoL) from a patient and physician perspective

## Methods

- One-on-one qualitative interviews were conducted with patients diagnosed with MM and treating oncologists/hematologists in the USA, the UK, Germany, Spain, France, Italy, Brazil, and Japan
- To qualify for study inclusion:
  - Patients must be ≥ 18 years of age and have been diagnosed with MM ≤ 10 years prior to study start
  - Physicians must have practiced for 3-30 years in their primary specialty of hematology oncology, medical oncology/hematology oncology, or medical oncology, and treated ≥ 10 patients with MM in the previous 6 months
- Participants were recruited via online panels
- From October 2023 to March 2024, 30-minute telephone interviews were conducted in the participants' native language
- Transcripts were coded using the qualitative analysis software MAXQDA (VERBI GmbH), and content and thematic analyses were performed

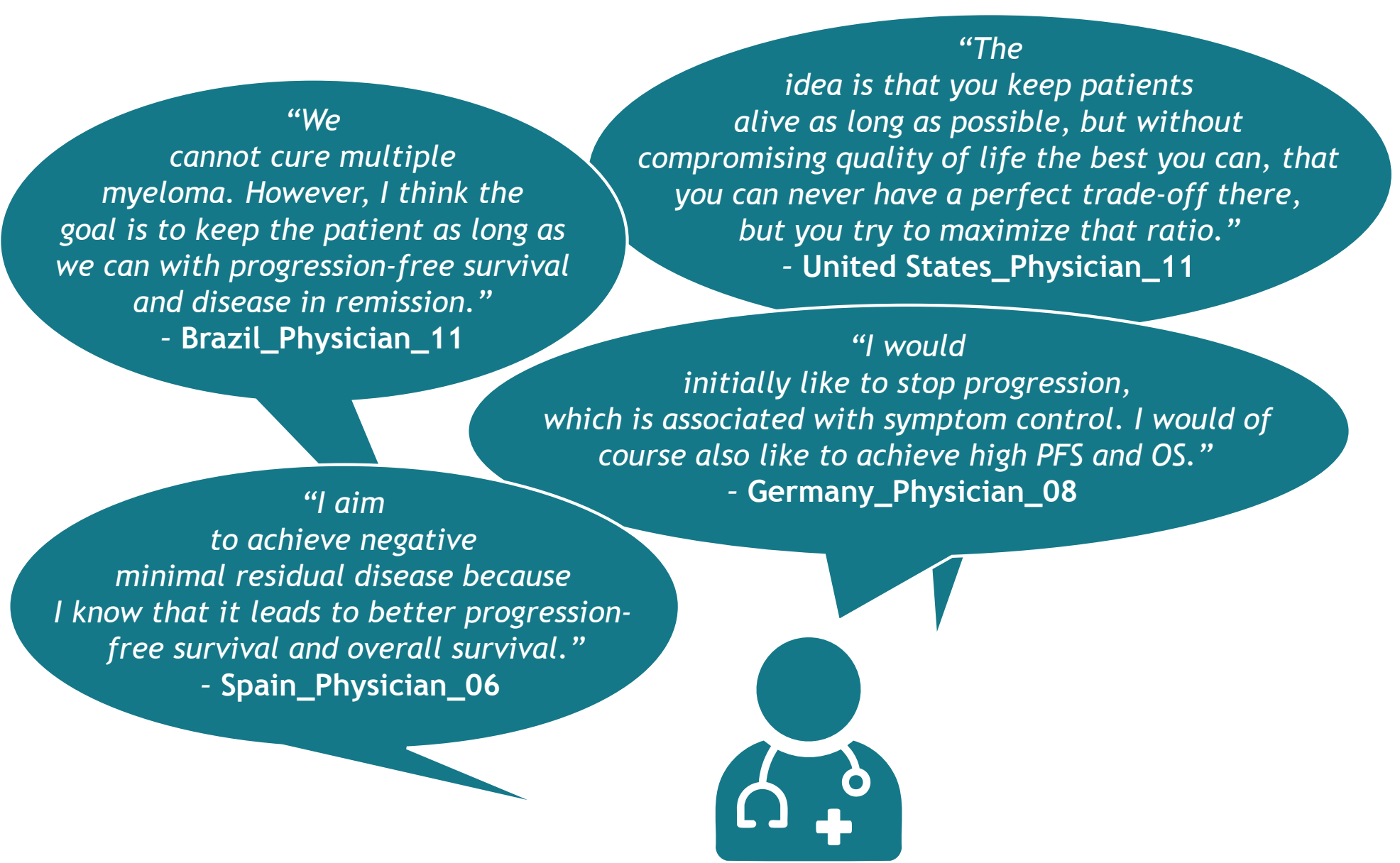
## Results

### Sample description

- Overall, 68 physicians (USA, n = 12; UK, n = 8; Germany, n = 8; Spain, n = 8; France, n = 8; Italy, n = 8; Brazil, n = 8; Japan, n = 8) and 68 patients (USA, n = 12; UK, n = 8; Germany, n = 8; Spain, n = 8; France, n = 8; Italy, n = 8; Brazil, n = 8; Japan, n = 8) participated
- Physicians had an average time in practice ranging from 11.8 (USA and UK) to 24.7 (Japan) years and saw an average of 39 (Japan) to 99 (UK) patients with MM in the previous 6 months
- The mean age of patients ranged from 47 (Brazil) to 63 (USA) years, with female patients ranging from 13% (Brazil) to 75% (France); the mean time since diagnosis ranged from 2 (Italy) to 7 (Germany and Spain) years

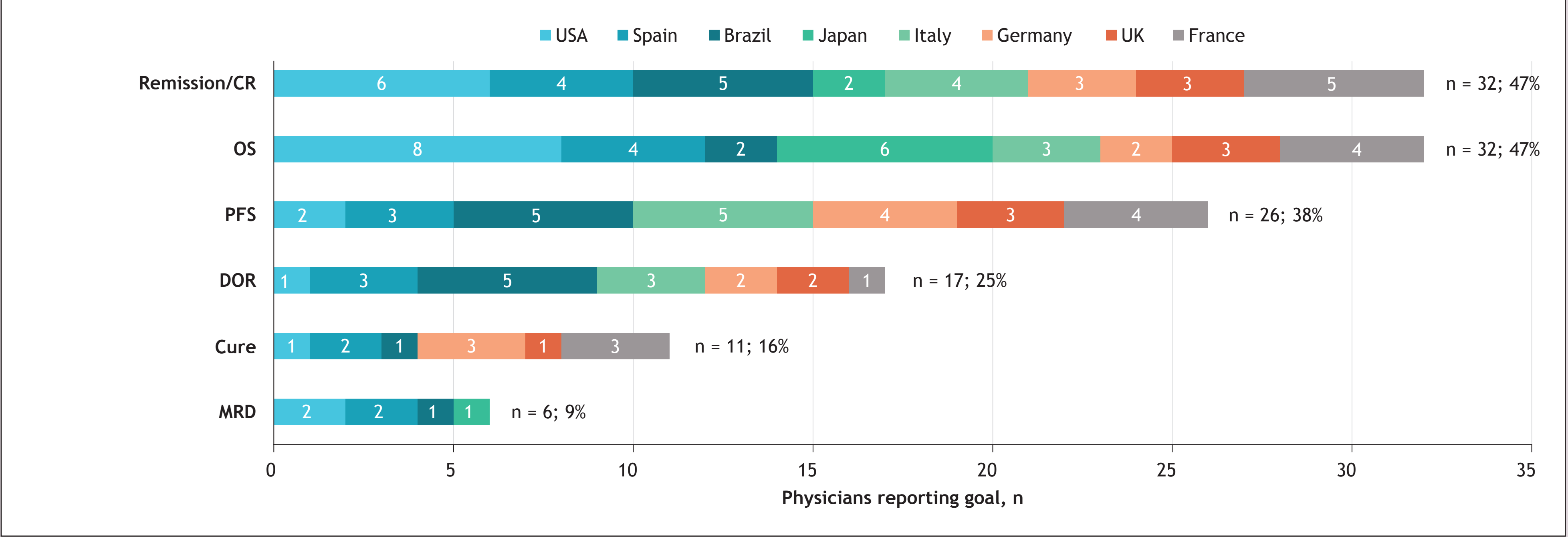
### Physicians’ treatment goals

- Physicians frequently identified multiple efficacy endpoints when asked about their treatment goals in MM:
  - Improve symptoms/maintain QoL (n = 39; 57%); achieve remission/complete response (CR) (n = 32; 47%); extend survival (n = 32; 47%); achieve long progression-free period (n = 26; 38%); minimize treatment burden/toxicity (n = 16; 24%)
- Similar key efficacy outcomes were identified by physicians in all countries, except for Japan, where PFS, duration of response (DOR), and achieving cure were not identified (Figure 1)



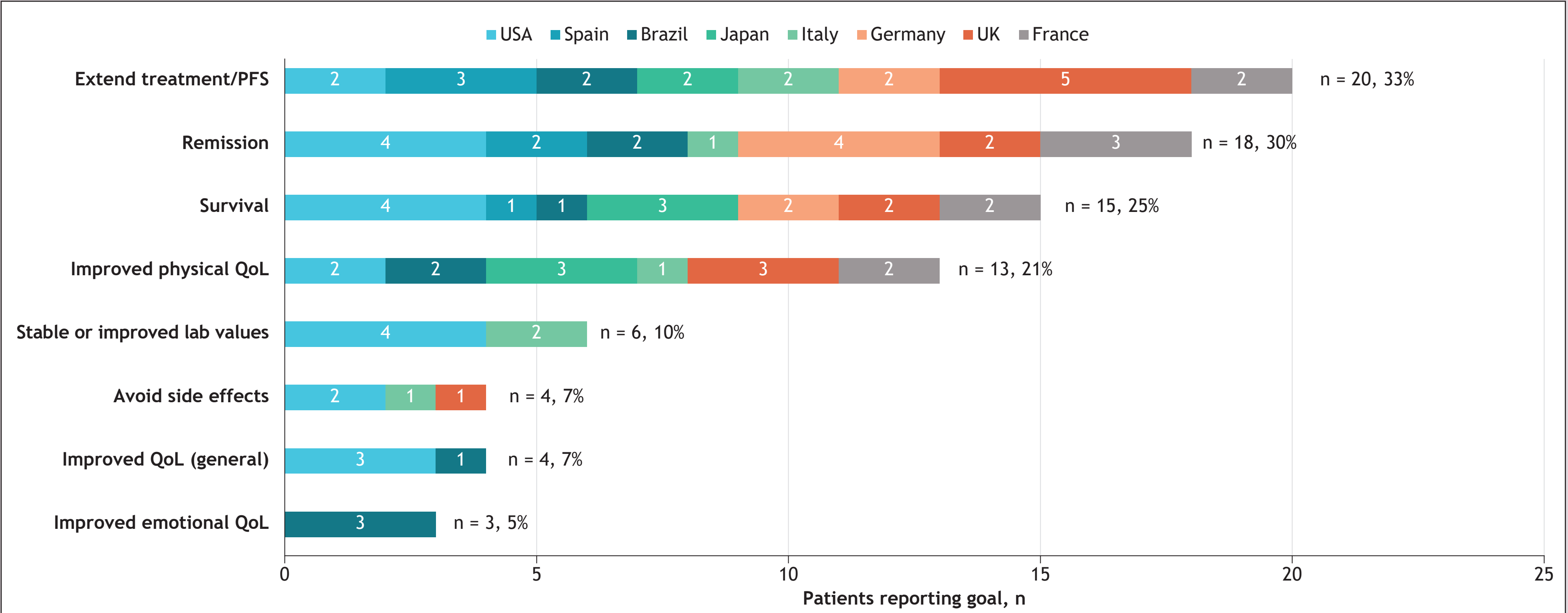
PFS is highly valued by both physicians and patients as a key treatment goal and is considered equally or more important than OS

Figure 1. Identified goals related to treatment effectiveness by country (physicians [n = 68])



MRD, minimal residual disease.

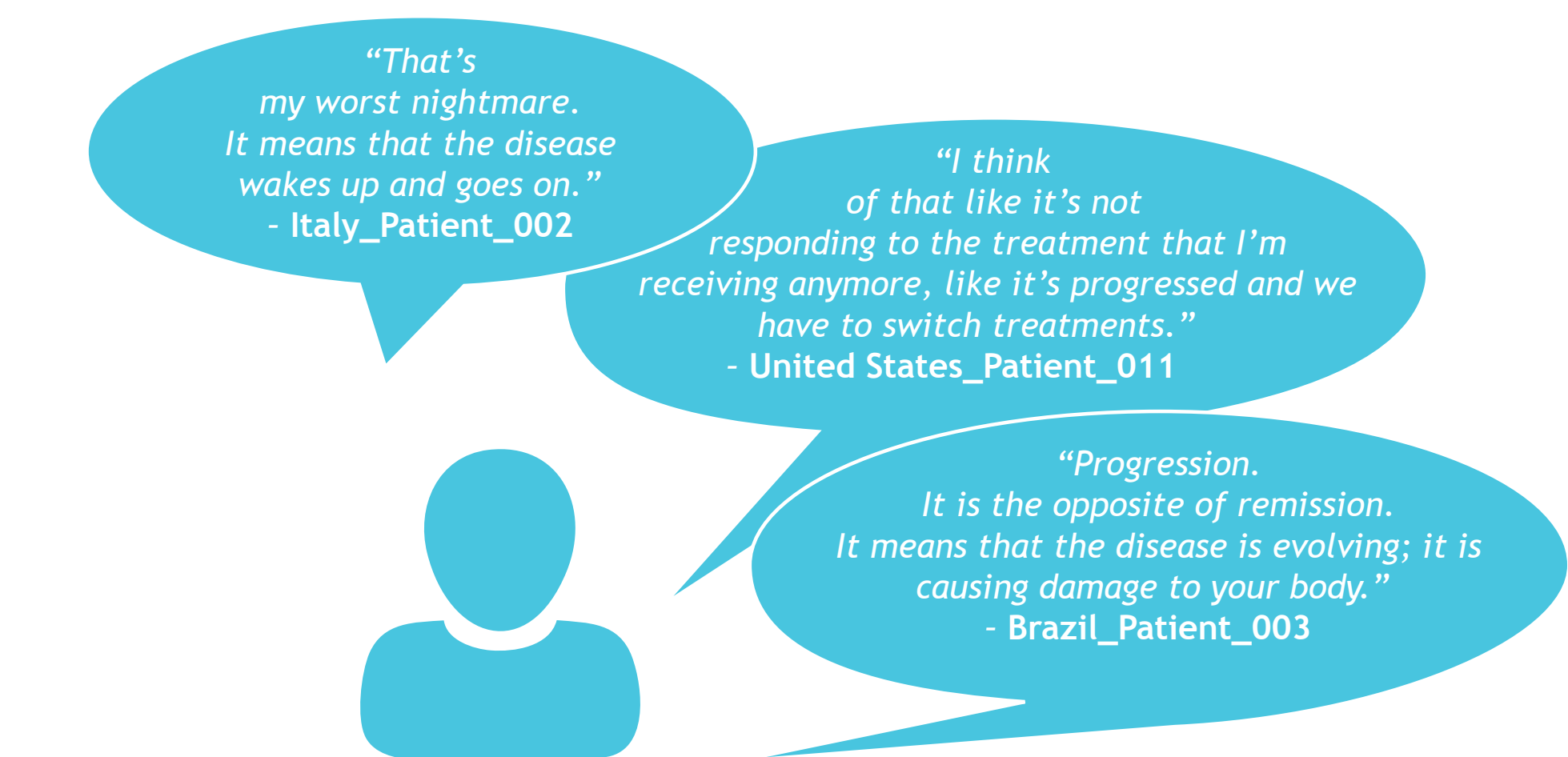
Figure 2. Identified goals related to treatment effectiveness by country (patients [n = 61])



\*7 patients did not respond to this question.

### Use and understanding of PFS (patients)

- To patients, progression means their disease is advancing, causing damage, and will require new treatment, which increases anxiety
  - Progression impacts daily life, including having to quit work, having extreme fatigue, and increasing medical appointments
  - Patients remain on high alert for any signs of progression, actively monitoring themselves for symptoms and tracking lab results with their physician



### PFS versus OS

- Physicians and patients viewed PFS as being equal to or more important than OS (Table 1)
  - For both patients and physicians, PFS reflects disease control and maintaining QoL
  - Approximately half of the physicians surveyed viewed PFS as more important in early treatment, while the remaining physicians either viewed PFS as more important in later treatment lines or did not change treatment goals based on the line of therapy
  - Some physicians regarded PFS as a surrogate for OS

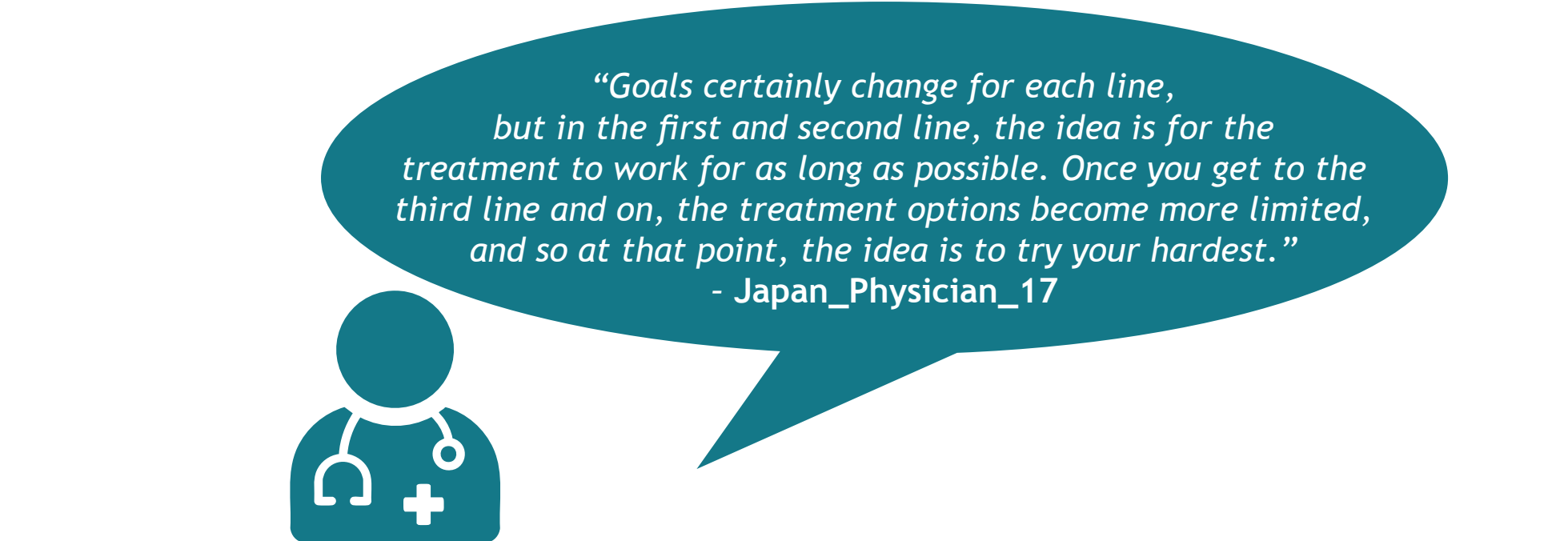


Table 1. Importance of PFS compared with OS for physicians and patients

	Physicians, %	Patients, %
PFS more important than OS	49 “I think PFS is probably the gold standard in MM treatments. Since patients survive for so long, our usual gold standard of overall survival is not always practical and can be heavily influenced by subsequent treatments.” - United States_Physician_06	51 “Holding out 10 years for the sake of 10 years is... irrelevant because if your quality of life is crap, if the disease is going to progress for those 10 years, I’d really rather not be around for the 10 years. I’d rather be around for the 5 with no progression.” - United Kingdom_Patient_003
No difference between PFS and OS	5 “They’re both considered... I always consider both data and I need to see what the duration is like too and how much it increases.” - Italy_Physician_02	29 “I would like to know all important data.” - Spain_Patient_006
OS more important than PFS	46 “When this patient comes to receive treatment, when it is a treatment-naïve patient, there is a chance for total remission. So, for this patient, in other words, although we do not like the word ‘cure’ that much, with a chance of cure, I need to aim at overall survival benefit.” - Brazil_Physician_02	20 “Overall survival is of course even more important, because if you don’t survive, you no longer need to worry about being progression-free or non-progression-free and you can certainly live with myeloma today, even if you are confined to bed; you still have opportunities to maintain social contacts today.” - Germany_Patient_009

### Patients’ treatment goals

- Avoiding disease progression was identified by patients in all countries as a key treatment goal (Figure 2)

### Use and understanding of PFS (physicians)

- Physicians use alternative descriptors of PFS with their patients. Frequently used descriptors include:
  - Duration of disease control; how long patients can stay on the treatment/time until next treatment; DOR/responding to treatment; time until tumor grows again/gets worse



### PFS in treatment decisions

- Almost all physicians (98%; 57 of 58) believe that PFS should factor into access and insurance coverage decisions
- Other opinions were:
  - PFS is a primary effectiveness measure (58%; 33 of 57)
  - PFS is one of many factors, including cost and toxicity, that should be considered as a whole (9%; 5 of 57)
  - Evaluating OS alone is too limiting in the treatment of MM due to new therapies (5%; 3 of 57)



## Conclusions

- Disease progression reduces patients’ QoL by resulting in more disease symptoms, interruptions to work and daily activities, as well as additional treatments and medical appointments
- Avoiding progression is a key treatment goal for patients; it is a tangible measure that patients can monitor over time, which may help them feel more in control of their health
- For most countries, PFS is largely considered an essential endpoint in MM by physicians, and it is a key factor influencing treatment selection
- Although physicians generally do not use the term “progression-free survival” with their patients, PFS is central to their treatment goals and selection
- Staying in remission/keeping the disease under control is a key goal of treatment among patients and physicians and is viewed as being equal to or more important than OS
- The majority of physicians believe that PFS should factor into access and insurance coverage decisions, and that evaluating OS alone is too shortsighted, especially in MM, where there are emerging treatment options

## References

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