

## Aim of the study

While the effectiveness of health interventions is widely studied in clinical trials (usually randomised controlled trials, RCTs), patients preferences in regard to their therapies are considered to a lesser extent. This is in contrast to the often postulated **inclusion of patients** in therapy evaluations and decisions (shared decision making etc.). Aim of this study was to explore the patients perceptions of an „ideal therapy“ for multiple myeloma (MM) in order to include **patients preferences** in decisions upon actual and future treatments.

## Methods

Examination of the state of the art as reported in the literature was followed by a qualitative study with three **focus groups** consisting of 6-8 MM-patients each. Goal was the collection of all relevant factors for an ideal MM-treatment to MM-patients, in order to achieve content validity. In a subsequent quantitative study phase, data was collected in an online or paper-pencil self-fill-in questionnaire. It included sociodemographic data, self rated health (SF12v2 variation) and patients preferences of therapy characteristics using **direct measurement** (16 items on a 5-point Likert-scale) as well as a **discrete-choice-experiment** (DCE, 8 pairs with 8 characteristics). Statistical procedures applied were analysis of variance, regression and factor analysis and random effect probit and logit models for the DCE.

## Results

N=282 patients answered the questionnaire; 46% female, age: mean 62 yrs (SD 10 yrs), duration of MM: 5 yrs (SD=3.8). Self rated health (SF12v2, SOEP-version) was worse compared to general population exp. for physical health (**fig. 1**). Direct measurement (**fig. 2**) showed „prolonged life expectancy“, „effectiveness“ and „further therapies possible“ in the first places, followed by „less side effects“ and „flexibility in application“.

In the DCE, alternatives with „longer life expectancy“, „combination with further therapies“, „flexibility“ and „better mental and physical quality of life“ were more likely to be chosen (**fig. 3**), giving thus **similar results**.

## Discussion + Conclusion

Besides the importance of **prolonged life expectancy** and effectiveness, MM-patients seem to highly value that therapies applied in prior lines do not affect the **possibility of following treatments**. Of further importance **breaks in therapies** (treatment free intervals) and **not always think on disease** (emotional quality of life (QoL)) and of minor importance is the possibility of **self application**.

Methodologically it seems important to **combine direct measurement** of patients' preferences with **choice-based measurements** like the DCE. The first gives the possibility to measure a longer list of potentially important aspects (content validity), the latter has the advantage to combine positive and negative therapy characteristics and to avoid the problem of ceiling effects and „all is very important“ results.

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 The patient advocacy groups for MM (APMM and DLH) supported us in the organisation of the qualitative and quantitative survey.*

fig. 1

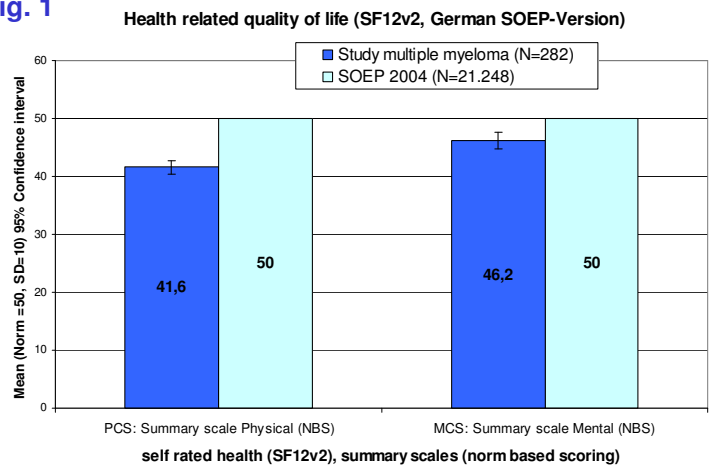


fig. 2

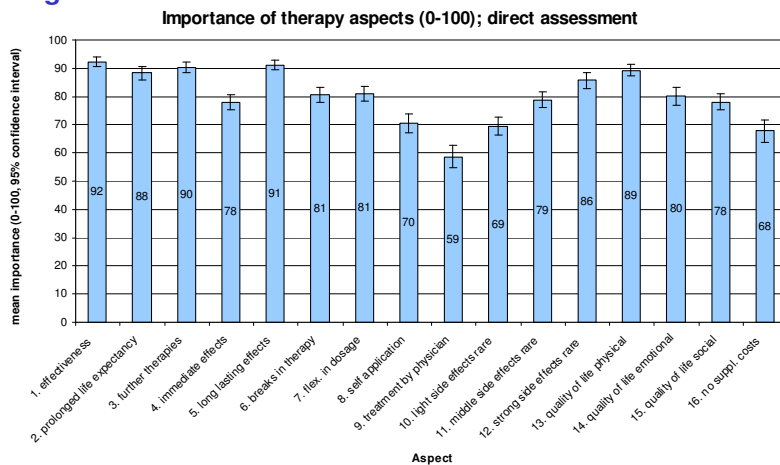


fig. 3

Importance of therapy aspects DCE; relative importance for decision

