The end point discussion

patient-relevant endpoints

ISPOR, 9th November 2022

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Declaration of interests

- MPNE (Melanoma Patient Network Europe) is a volunteer-based network whose activities are or were in the past (the last 2 yrs were unrepresentative due to COVID) funded by balanced support by the following companies: Amgen, BMS, Delcath, Idera, Incyte, Immunocore, MSD, Merck Serono, Novartis, Pierre Fabre, Roche. Support never includes editorial rights, influence on MPNE's program nor activities. In addition, MPNE has received funding through 3 Horizon2020 grants (UMCURE, Share4Rare and iTobos).

- **Unfortunately, we have been unsuccessful in diversifying our funding sources by support from regulatory, HTA and payer bodies or medical societies.**

- In the last 3 years, BR received personal consultancy fees for work in patient affairs from- AstraZeneca, BMS, Celgene, Clinigen, IQVIA, MSD, Novartis, Pfizer, Roche.
- BR's work for MPNE outside the Horizon2020 projects is non-remunerated
- Between 2019 and 2021, BR was member of the first EU Cancer Mission Board
- BR currently works as subject matter expert for Precision Medicine for Vision Zero Cancer, Sweden

To find out which end points are relevant to patients….

what about asking patients themselves?

Too many leading questions and biased analyses….

so here some observations from our work in MPNE instead.

what a patient wants depends on

- specific context like stage of disease, line of treatment, available options
- personal preferences

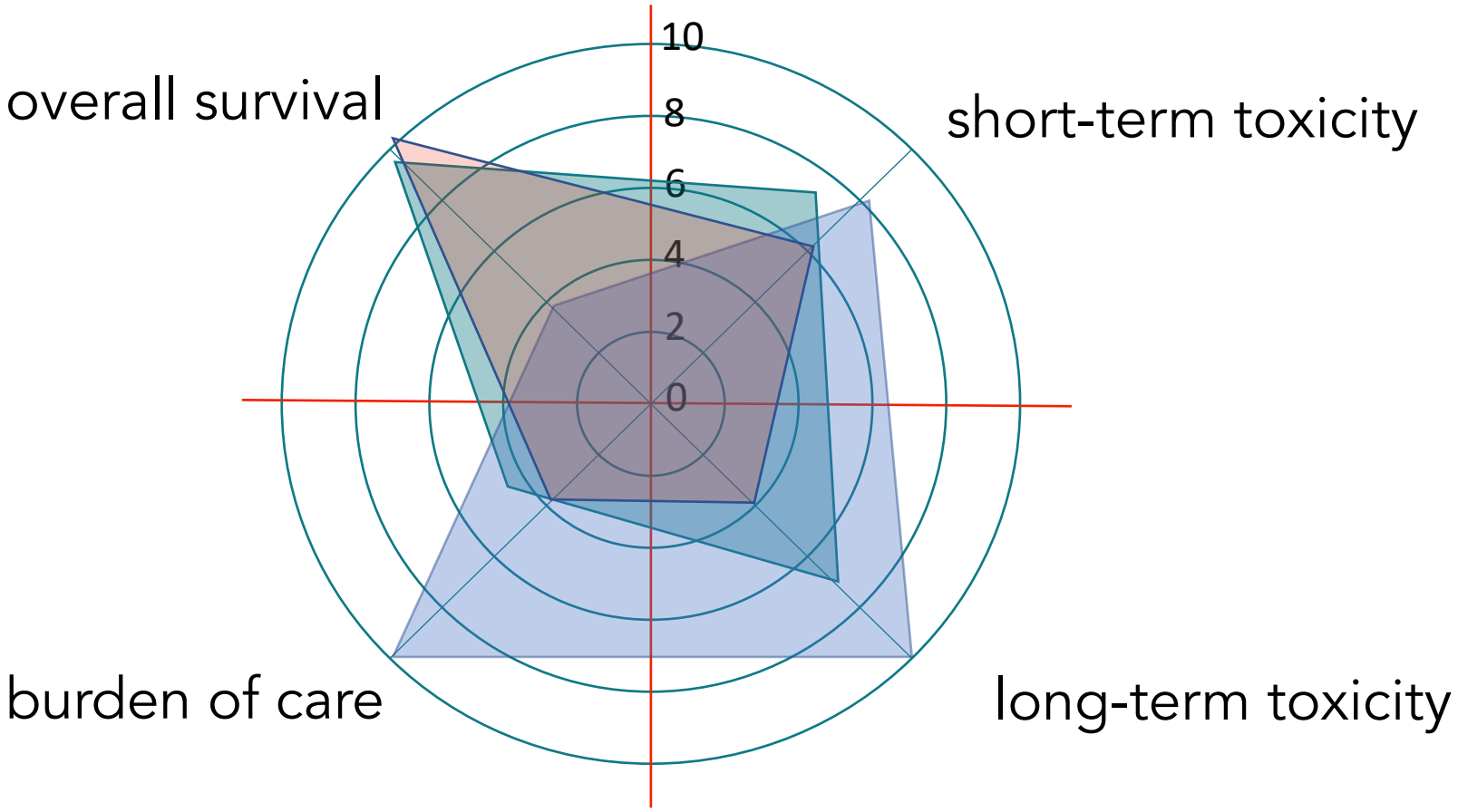
so there is no single answer.

MPNE patient trade-offs (work in progress, unpublished)

- with regards to treatment, patients consider 4 domains
- due to Melanoma's specific presentation (very high survival for early stage, considerably lower survival for advanced stage), we expected differences between early and late stage domains- this turned out to be incorrect
- independent of stage, all patients seemed to consider the same 4 domains, albeit with stage-specific weightings

- work to be continued!

MPNE patient trade-offs (work in progress, unpublished, shapes are hypothetical)



Pitching today's patients against future patients

Which end point you consider the most relevant depends on whether you are on the trial yourself- or whether you want someone else to go on that trial.

If you are a trial participant, you want end points that optimise your chances to survive, independent of a single product. These are clinically actionable endpoints: remain on therapy, alter therapy. **NOTE- Stable disease means you are not dead**.

As a non-trial participant, you want a (ideally, a single) product that you know will cure you- even if it means that people died to prove it.

Mark Lewis
@marklewismd

As a doctor who treats cancer and as a human being I think that stability is massively underrated as an outcome

full thread: shorturl.at/aJLP1

12:14 AM · Sep 28, 2022 · Twitter Web App

Some accounts you follow often like this Tweeter

Josh Mailman @Globeseek · Sep 29

Replying to @marklewismd

For what it is worth we are dealing with decades of media, medical groups and patient awareness groups telling us to fight cancer or eliminate cancer. Some of us just want to string a couple of decades of stability with high quality of life.

1 6

Followed by some Tweeters you've liked

M'Fezi @LilMas_ · Sep 28

Replying to @marklewismd

As a stable patient w MBC, totally agree. I can live like this just fine for another 30 years if medicine will make it happen! I don't need to be cured, I just don't want to die yet. I've had chronic health issues for 20 years,I'd love to have my onc tell me this is just another!

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You and this Tweeter share some mutual follows

Myriam Chalabi @MyriamChalabi · Sep 28

Replying to @marklewismd

Agreed! I say this to my patients when we start a new treatment so they're not disappointed when I share the news of stability as good news: we're happy with stable disease, and everything that comes after is of course bonus...

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Some accounts you follow often like this Tweeter

Steven Vogl @VoglNY · Sep 29

Replying to @marklewismd

Stable without symptoms or significant toxicity is fine. Stable disease and miserable from either disease caused symptoms or toxicity is a situation to rectify aggressively, especially the latter, where dose reduction or drug-free intervals are easy.

1

Some accounts you follow often like this Tweeter

Denver Veggie @Denverveggie · Sep 28

Replying to @marklewismd

As a patient, I refer to stability as the worst good news. 😂 Of course, I'd love for the lesions to shrink, but stability is the next best thing.

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Concluding

- There is no simple definition of ‘patient-relevant endpoint’
- Multiple stakeholders claim their endpoints to be ‘patient-relevant’, these claims are typically entirely unsupported (‘my patient said’) or based on biased selection and undue generalisation (prostate CA is the classic example for overtreatment)
- For patients, Overall Survival, OS, is an important but in the absence of curative treatment not the clinically actionable endpoint

patient-relevant endpoints need to address the needs of today’s (optimise survival, QoL) as well as future patients (OS): multiple end points collected systematically

Thank you

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