Clinical trial design- the patient **standpoint**

workshop
London
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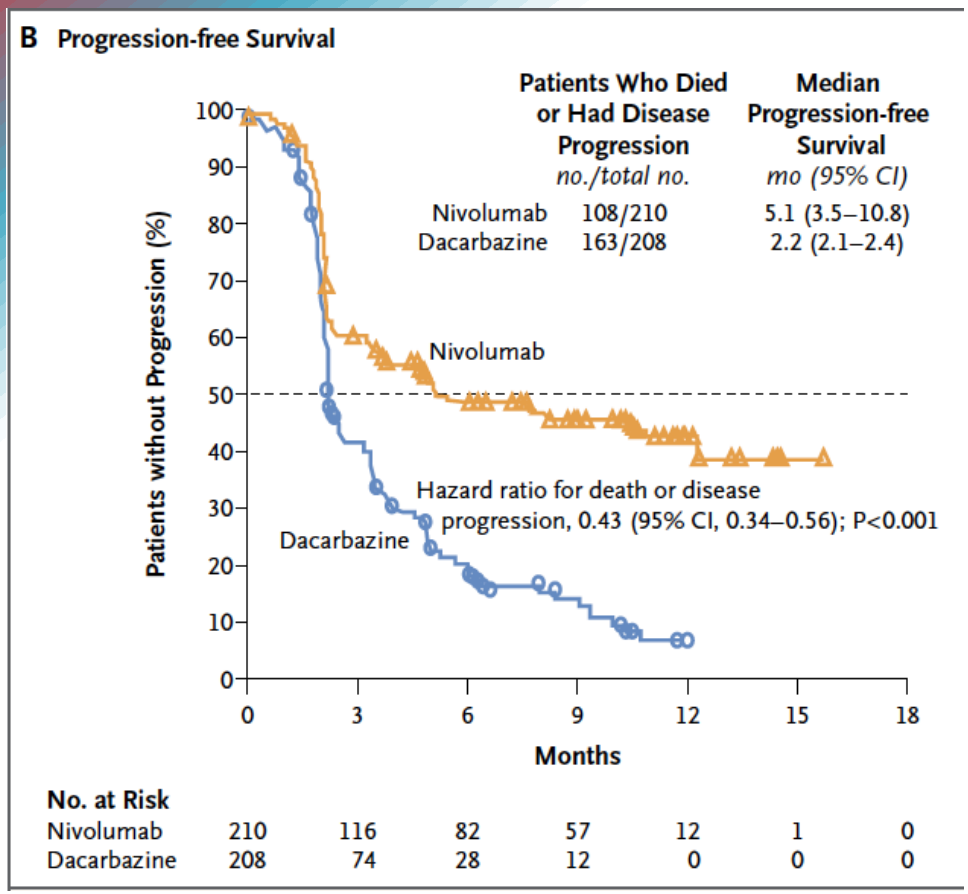
Melanoma Patient Network Europe

Clinical trials are personal and randomisation affects real people

capture clinical impact of response, not only (easily measureable) radiological response

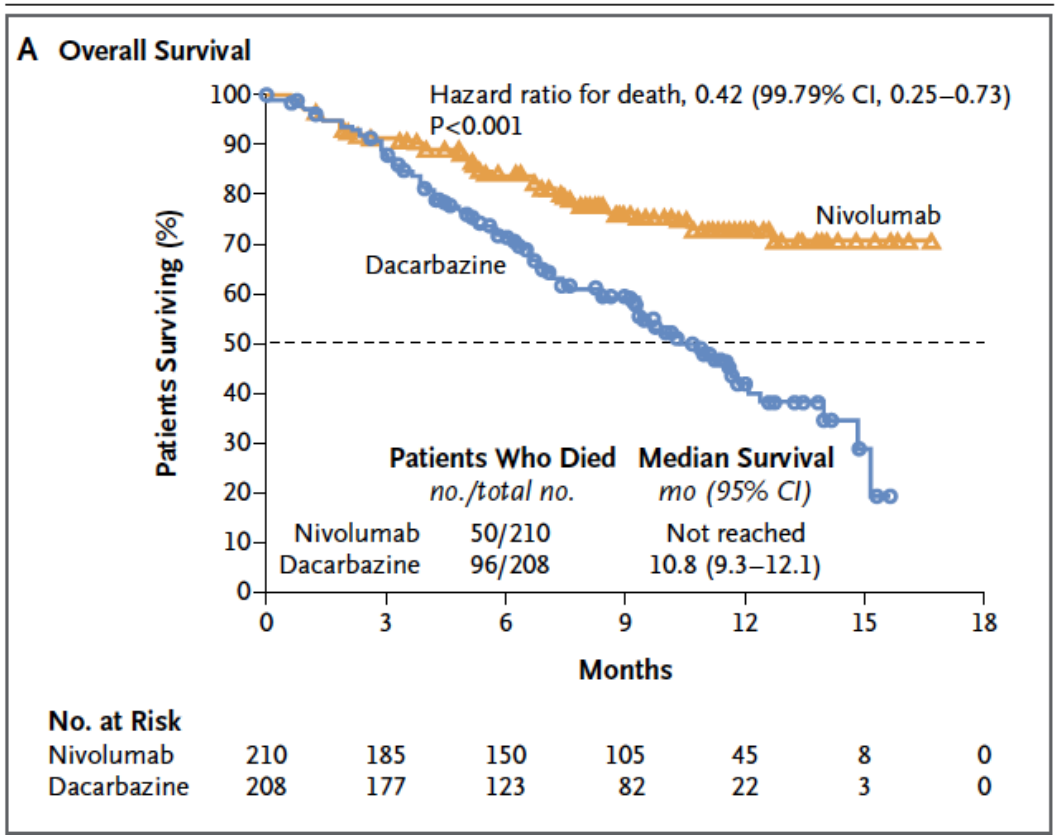


- 'plethora of effective therapies'- but they need to be used
- PFS is a patient-friendly end-point as clinically actionable
- BUT should lead to follow up to gain insight into the sequencing of treatments

Robert et al. 2015

Figure 1. Survival End Points.

Panel A shows the Kaplan–Meier curves for overall survival. The median follow-up for overall survival was 8.9 months in the nivolumab group and 6.8 months in the dacarbazine group. Panel B shows the Kaplan–Meier curves for progression-free survival.



- median OS NOT a good marker for immune therapies

Robert et al. 2015

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This is what one of our Melanoma patients said after being randomized to DTIC (ineffective prior standard of care) versus anti-PD1:

29 November 2013 · Granton, United Kingdom ·

This feels like some 21st century equivalent of medieval torture, not physical but psychological. First you are told your mole is nothing to worry about, then you are told its going to kill you, then, we have a drug that could help but you're not going to get it.

I thought I was strong but boy, if someone up there is testing me, let me tell you mate, you've won

Lori's experience on a clinical trial
https://www.youtube.com/watch?v=H03vz24JhgM

Early Access, the risk of NOT taking risks and who is right?

"Would you jump out of a plane if you knew that there was a 1 in 10 chance that your parachute would not open and you would die?"

"Well, if that plane was heading towards a cliff, then yes, I would".

MPNE2015 documentary
www.youtube.com/watch?v=VIreDdQG4kc

quote from a patient workshop, kindly provided by M. Longley, WIHSC

also- the misconception about therapeutic misconception

Hardly surprising....

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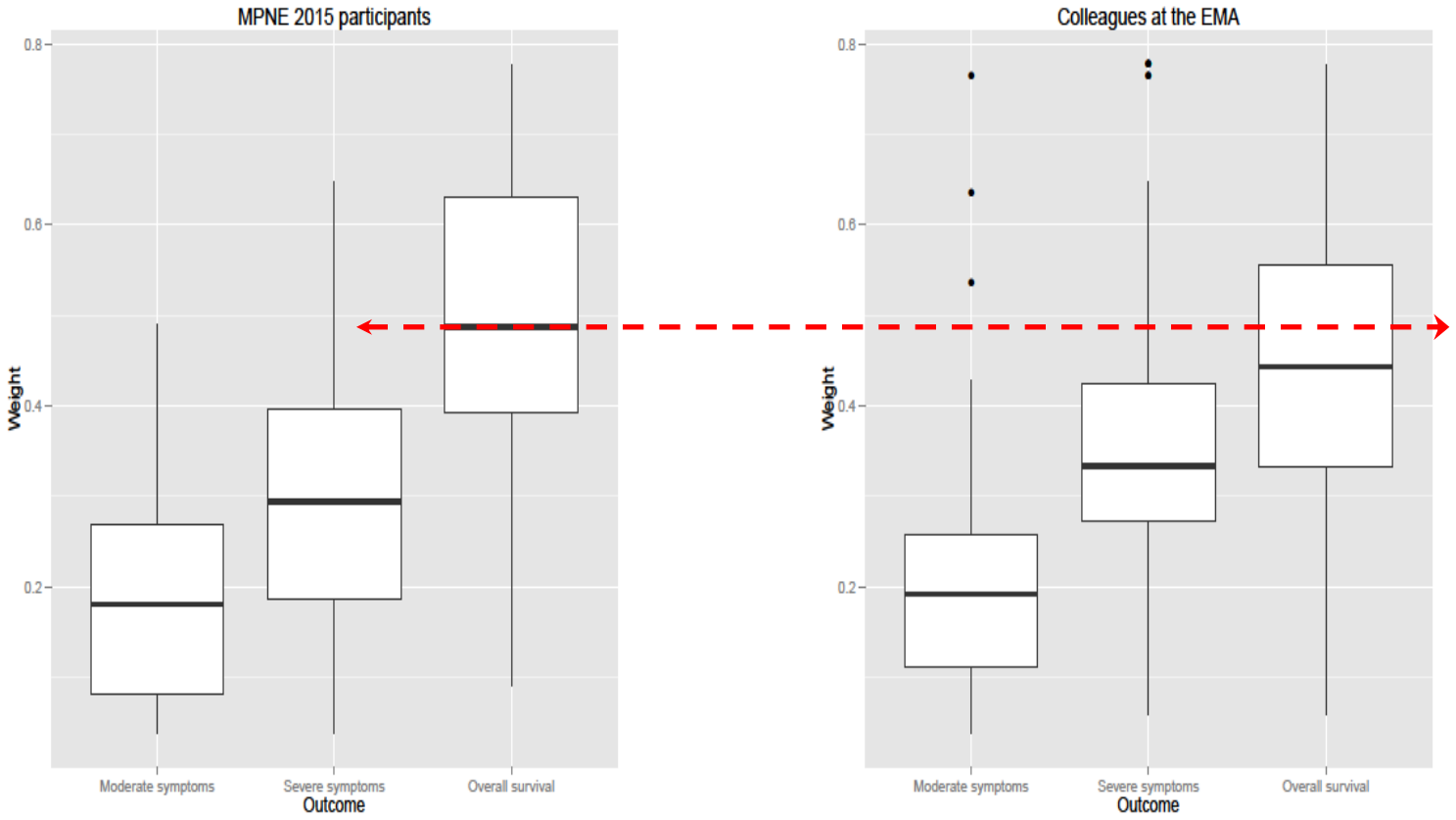
Comparing MPNE2015 conference participants and EMA (n=73) regulators-



EMA/MPNE pilot study on eliciting patient values - work in progress

But a closer look shows....

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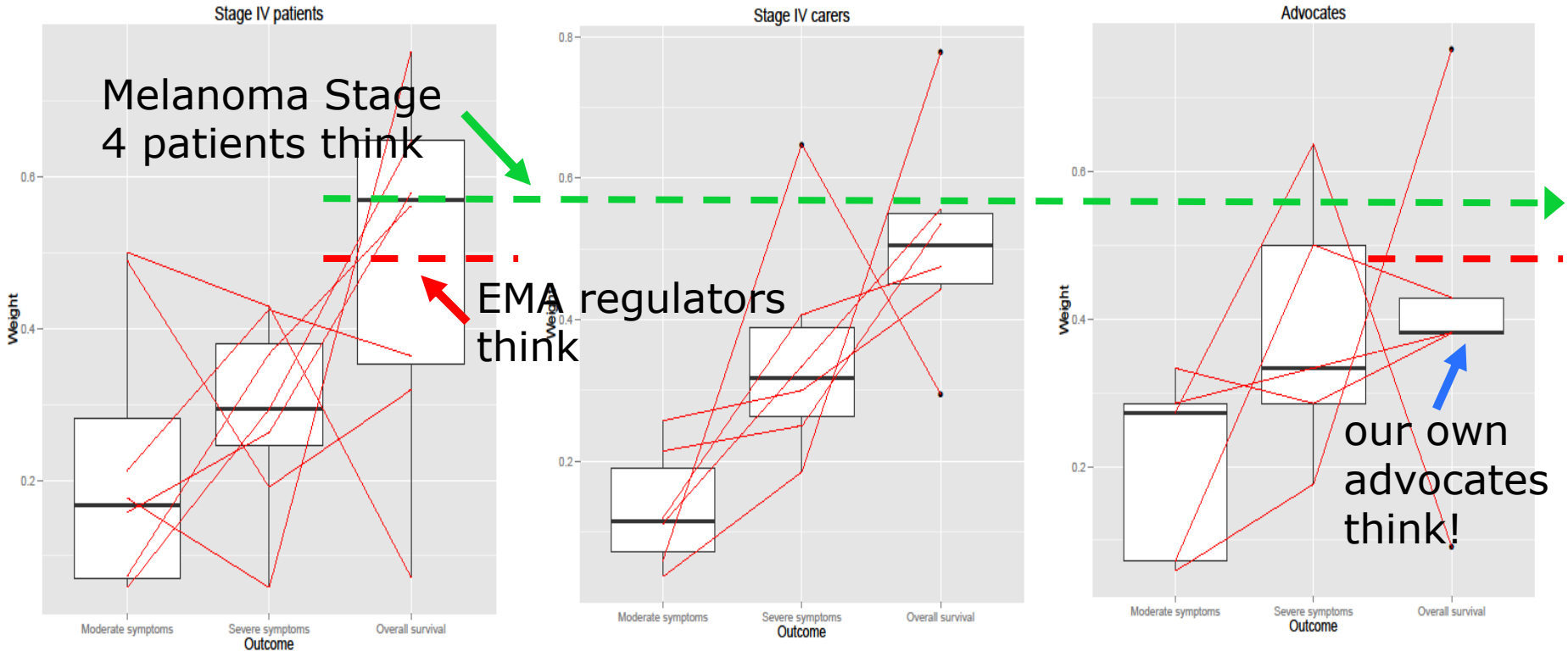
Melanoma Stage 4 patients don't think like their carers who don't think like advocates who are neither Stage 4 patients or carers themselves. And some are more risk-adverse than regulators!



EMA/MPNE pilot study on eliciting patient values - work in progress- commented slide.

Summary

- clinical trials remain a way to access treatment, in particular in the current economic climate
- randomisation effectively removes free choice
- large effect sizes by default lead to equipoise-violating randomisation
- a differences in risk acceptance not to be confounded with therapeutic misconception

'no patient in the right mind enters a trial because all the spots at the golf course were taken' MPNE quote

Thank you

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