New Data Models: Managing Uncertainty and Leveraging New Sources of Evidence

a patient perspective

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Melanoma Patient Network Europe

Melanoma- example for break-through innovation in a high unmet need situation

- ca 23 000 deaths/ year in the EU
- 'young' cancer
- historically 6-9 months median survival once metastatic
- once surgery impossible, very limited traditional treatment options: Melanoma considered resistant to chemotherapy and radiotherapy
- rapidly evolving treatment landscape with game-changing innovative therapies (targeted therapy, immune therapy)

- survival chances dependent on access to costly innovation
- a cure will depend on systematic and sustained innovation

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

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

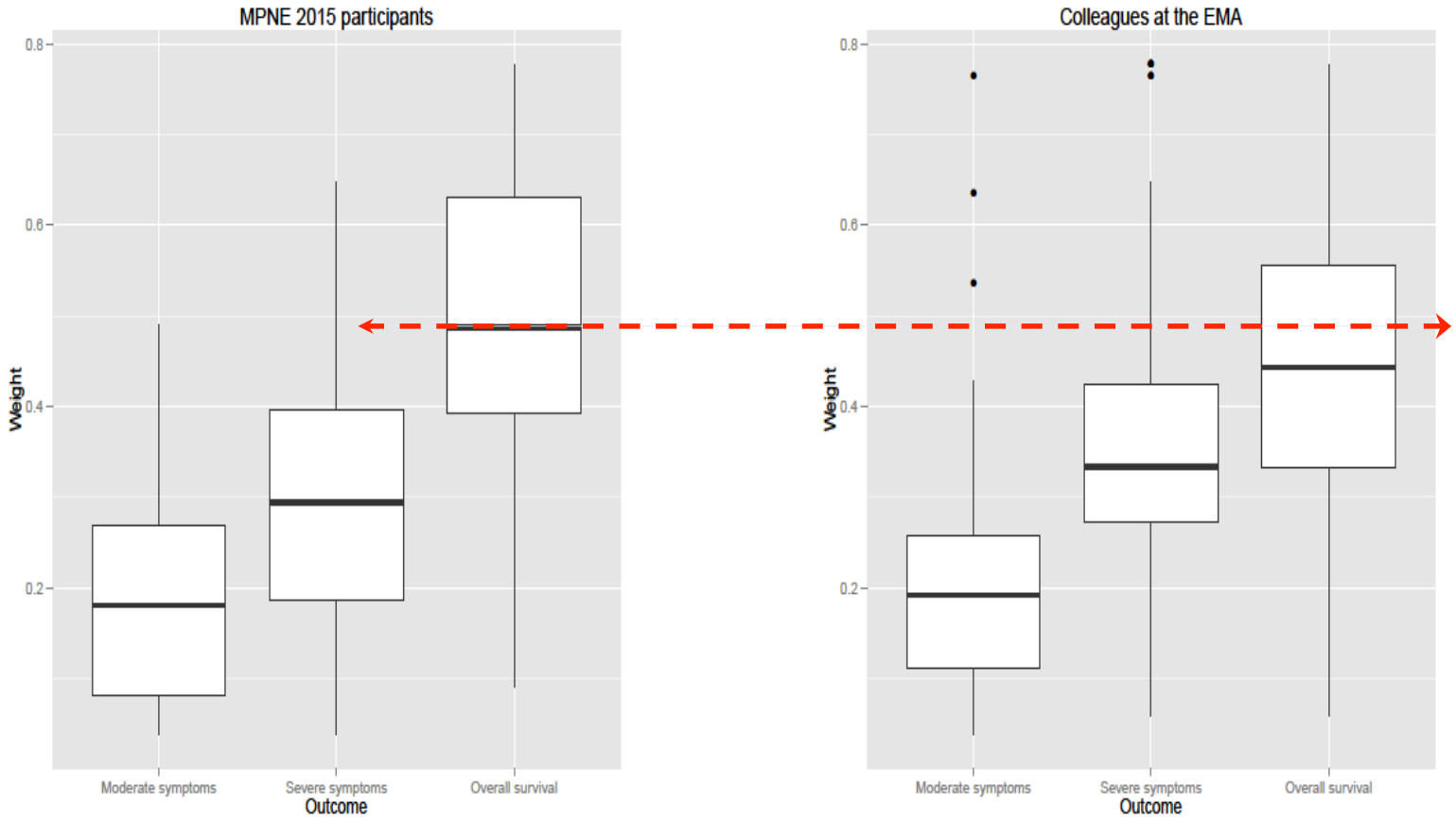
Patient values in benefit-risk assessment

MPNE 2015 Annual conference
24TH- 26TH APRIL 2015

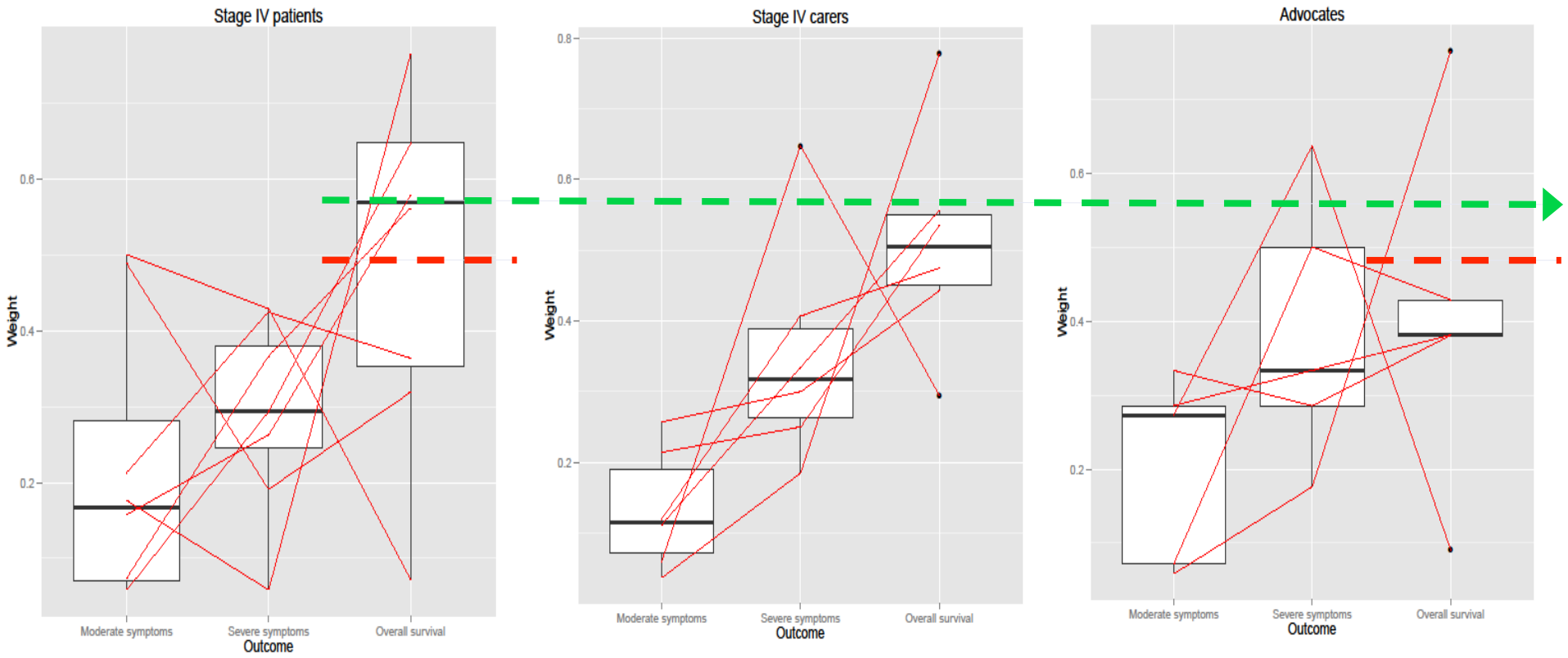
Presented by Francesco Pignatti

Head of Oncology, Haematology and Diagnostics; European Medicines Agency

How does this compare which the survey conducted at the EMA (n=73)?



Comparison across subgroups



next control group: Melanoma oncologists

We are living in the real world- the problem of limited external validity

RCTs- the (not so golden) Standard

- 'RCTs tell us whether a drug works'- but with **limited external validity**: the difference between efficacy and effectiveness
- 'RCTs tell us whether a drug is safe'- a large effect size leads to much smaller RCTS- which limits the number of adverse events that can be effectively be observed- so **limited safety data**
- high effect size of a drug also leads to **unethical, equipoise-violating trials** as the control arm will be *known* to be inferior from the start
- costly

In particular in the case of drugs with high effect size (the real break-throughs we are all hoping for!!) RCTs as the 'gold standard' of clinical evidence generation actually deliver data of limited external validity, insufficient safety data and there is a high risk for unethical trials.

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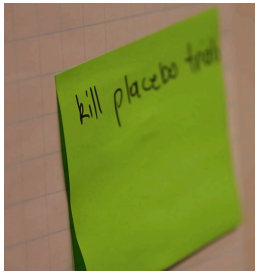
This is what one of our Melanoma patients said after being randomized to DTIC versus PD1:



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

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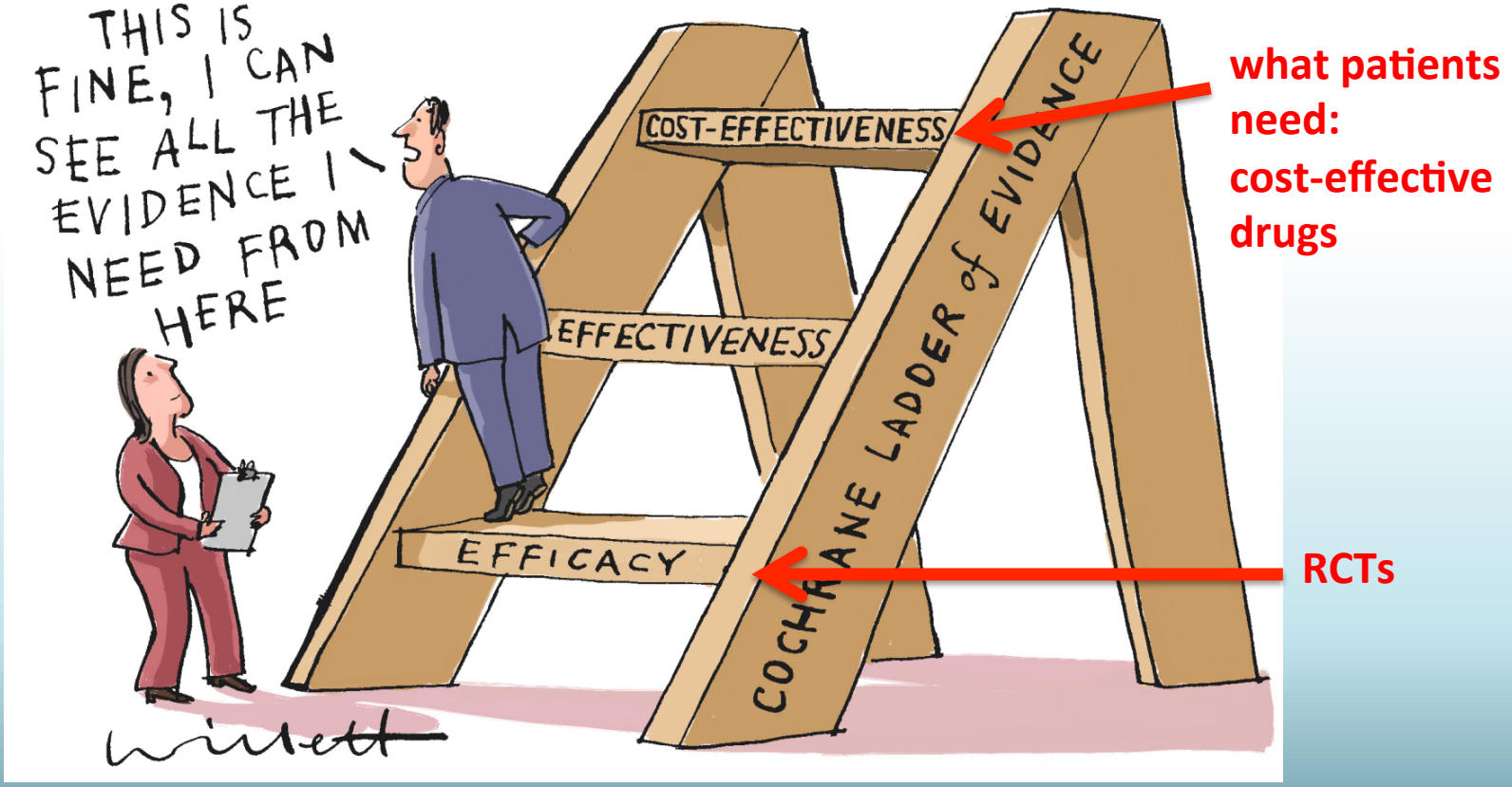
Patients need access to effective drugs- cost-effectiveness



The observed discrepancy between effects of a health intervention in routine clinical practice as compared with the effects demonstrated in randomised controlled clinical trials. (Adapted from Eichler et al., 2011)

“Of all the forms of inequality, injustice in healthcare is the most shocking and inhuman(e).”
Martin Luther King, Jr

Thank you for your attention

http://www.melanomapatientnetworkeu.org