

Patient involvement in drug development

CML Horizons 2016
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ESMO PAWG chair

Overview

- why care about drug development?
- where and how to get involved?

Why should patient advocates care about drug development?

The ultimate purpose of medicines and healthcare is to benefit patients.

1. Patients take the ultimate risk in drug development: the risk to their lives by

- testing new drugs in clinical trials,
- inappropriate trial designs,
- delays in development,
- not having access to therapies because of cost.

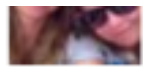
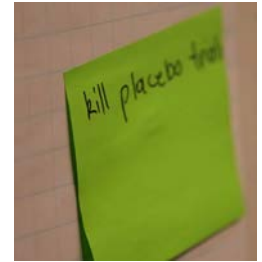
2. Patients know what it is like to be a patient. Patient networks therefore have access to the primary source of information- the patients themselves. Today greatly facilitated through social media.

Melanoma- case study for innovative break-through in a high unmet-need situation with resulting access problems due to cost.

- Melanoma: median survival 6-9 months prior to arrival of 2 classes of new therapies (targeted therapy, immune therapy); prior standard of care, DTIC, a form of chemotherapy, is ineffective
- New therapies with significant improvement on therapeutic efficacy based on scientific insights; speed of scientific development not mirrored by development in regulatory science (nor HTA)

Why I care

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



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>

Anti-PD1s are antibodies that blocks a 'brake' in the immune system so that it continues to attack the cancer. First tested and now approved in Melanoma, anti-PD1s now also show promise in many other cancers.

Now- **where** to get involved
in drug development?

Choose strategically
for maximum impact
for your patients.

Patient involvement in clinical trial design

clinical question/
problem ★

current patient involvement

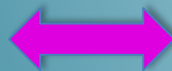
clinical trial
design ★

patient recruitment
e.g. providing information on
clinical trials, advertising trials

trial management,
e.g. consent, patient
information leaflets, trial
adherence

impact on patients

doing the right thing



doing things right

*And **how** to maximise our impact.*

Turning opinions
into DATA.

1. There is no such a thing as ONE patient preference or opinion.

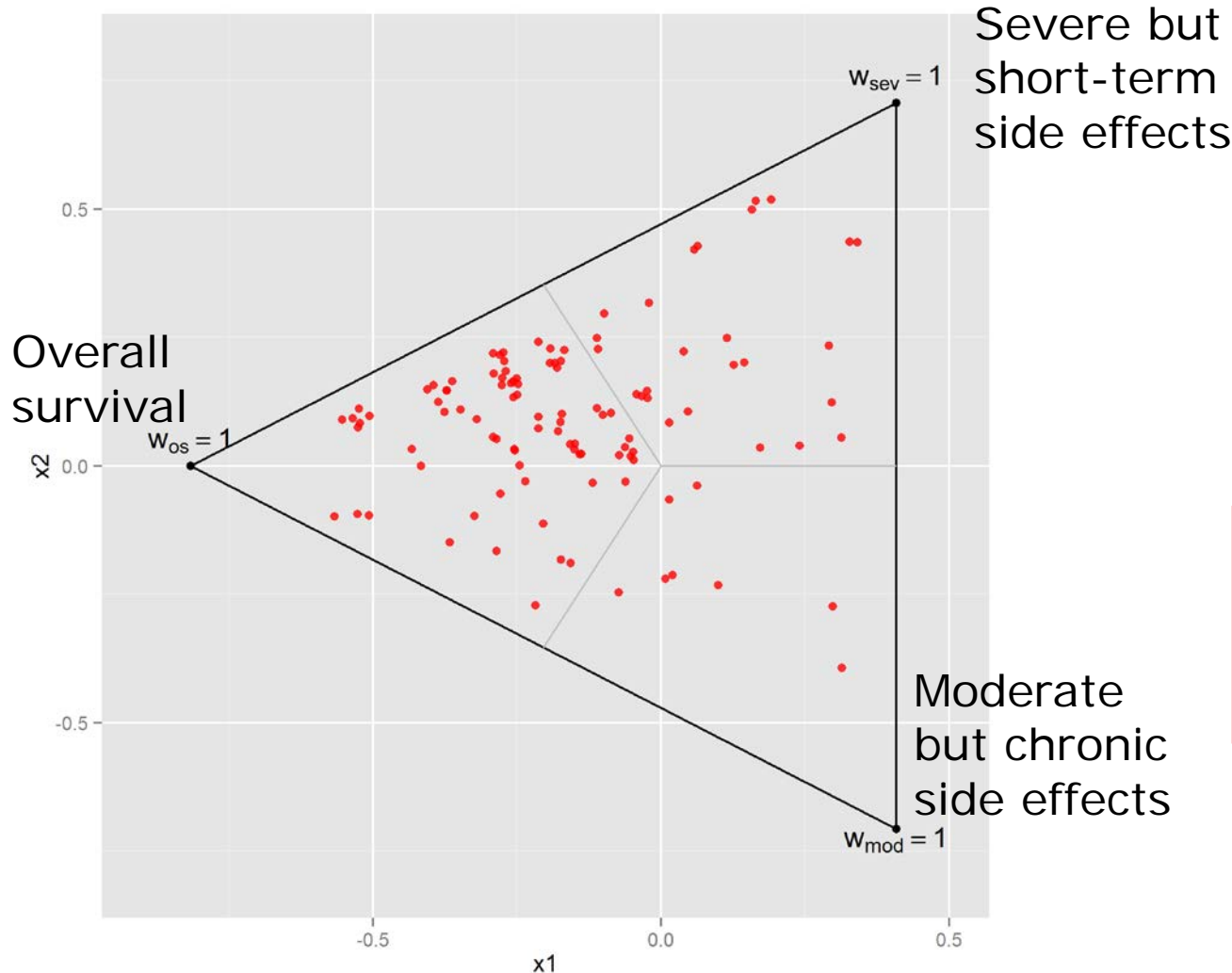
- Patient preferences have a natural spread- just like other preferences. Depending on the question, the spread might be narrower or wider.

Example

Risk/ benefit pilot project with the EMA and MPE and MPNE



Distribution of individual preferences



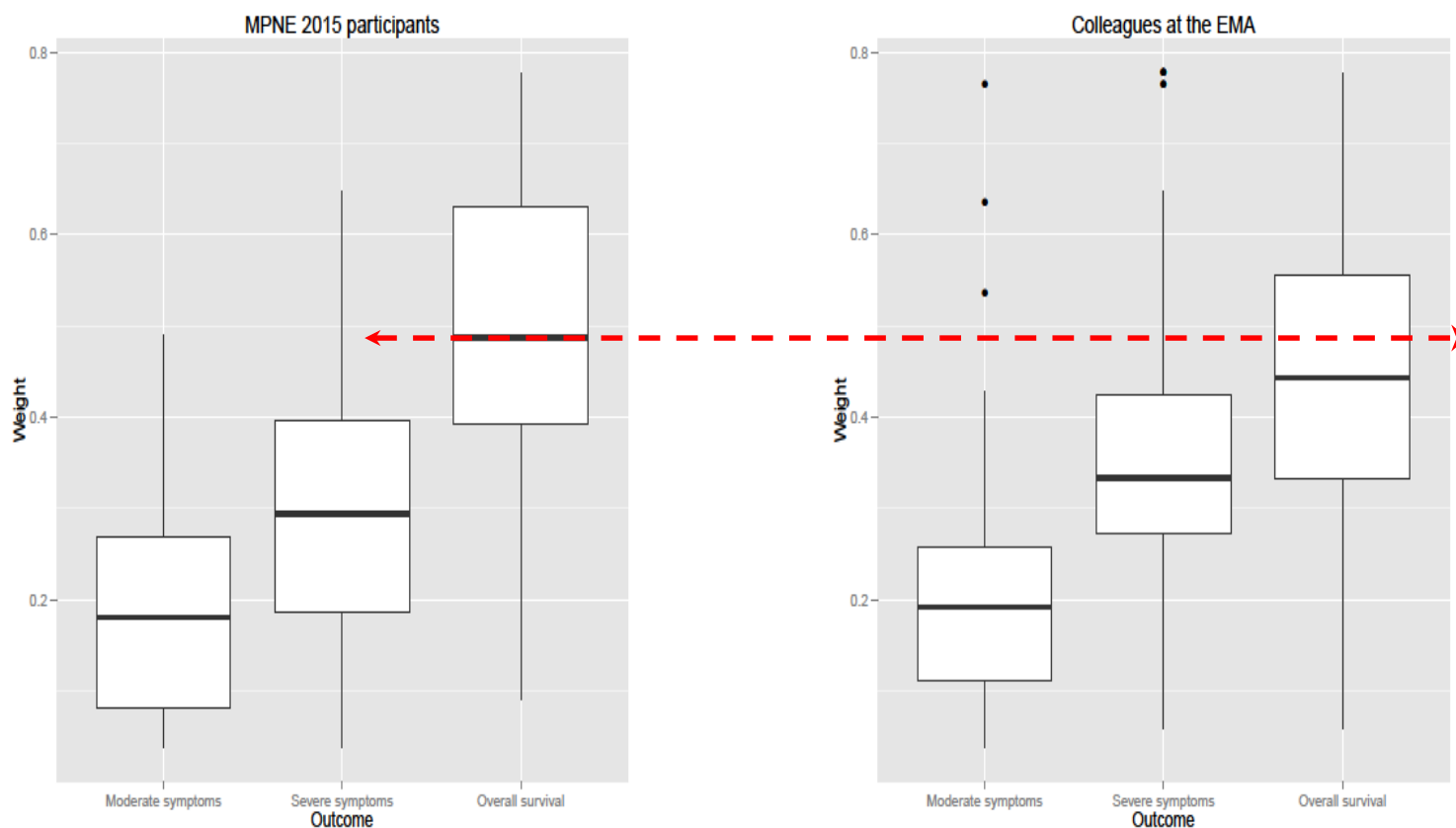
Comment Bettina:
This is a mix of
Myeloma and
Melanoma patients
and advocates
- lack of granularity

Note
This is a way out of the
representativeness
dilemma

2. Understand the patient population you are studying.

- Averages across diverse groups can hide important information.
- The common desire to have a ‘one-size-fits-all’ approach risks missing important subgroup information.

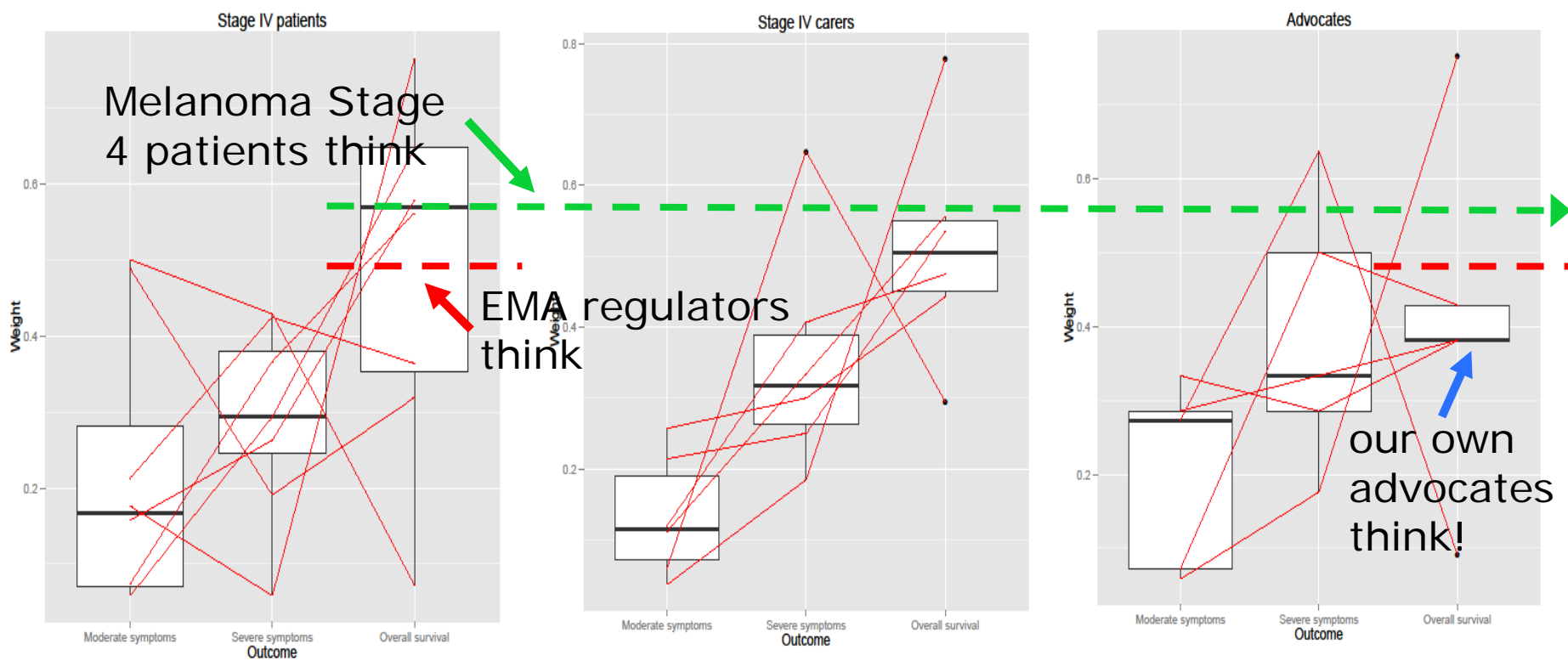
Comparing MPNE2015 conference participants and EMA (n=73) regulators-



But a closer look shows....



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.

Conclusion

- Patients carry the ultimate risk to their lives in drug development
- We need to be strategic in our involvement for maximum impact for our patients
- Social media give patient networks direct access to patients and the ability to capture grass-root level data.
- Appropriately measuring and documenting patients' interests and preferences turn opinions into data = evidence-based advocacy.

Thank you

www.melanomapatientnetworkEU.org

Bettina

MPNE 2014

The Melanoma trial of the future- the trials we want!

<https://www.youtube.com/watch?v=wilTXvFN2NU>

MPNE 2015

The risk of NOT taking risks in Melanoma

MPNE 2016

Power to the patient. With great power comes great responsibility.

17th- 20th March, Leuven

Projects

1. GetReal project- patient-centric trial design
2. EMA- Risk/ benefit assessment in Melanoma Stage IV
3. ongoing working relationship with UMC (Uppsala Monitoring Center) on Pharmacovigilance