Thinking like a Scientist

An introduction for patient advocates

MPE webinar

Bettina Ryll, MD/ PhD

ESMO PAWG chair

Melanoma Patient Network Europe founder

14th December 2017

Overview

- Why care about Science?
- Thinking like a Scientist and the power of evidence
- How to find and read a scientific medical paper
- A useful too– the ESMO MCBS (Magnitude of Clinical Benefit Scale) introduced
- Summary

Why advocates should care about Science

for individual patients

- understanding the latest scientific developments allows patients to make informed treatment choices- especially critical in the absence of established treatment paths
- evidence-, not eminence- based Science
- true patient empowerment impossible without a certain understanding of clinical Science
- protection from quakery/ false hopes

B. Ryll, MPNE, Dec 2017

Why advocates should care about Science

as advocates

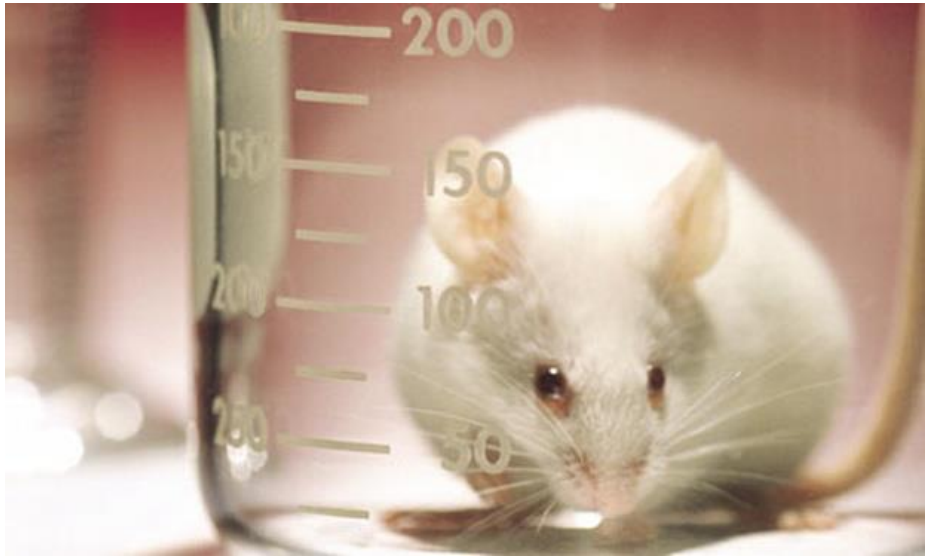
- Medical progress depends on progress in Science
- The way Science is conducted is not a law of Nature but based on common principles of understanding- and affects outcomes for patients
- one can only influence what one understands
- it is impossible to provide accurate healthcare information to patients without an accurate understanding of the underlying Science
- Supporting advocacy claims with evidence dramatically increases their chances of being heard- and are way harder to discard
- Our own advocacy work should be based on evidenced not suspected patient need

B. Ryll, MPNE, Dec 2017

Scientific thinking
is the basis
for evidence-based advocacy

The good news

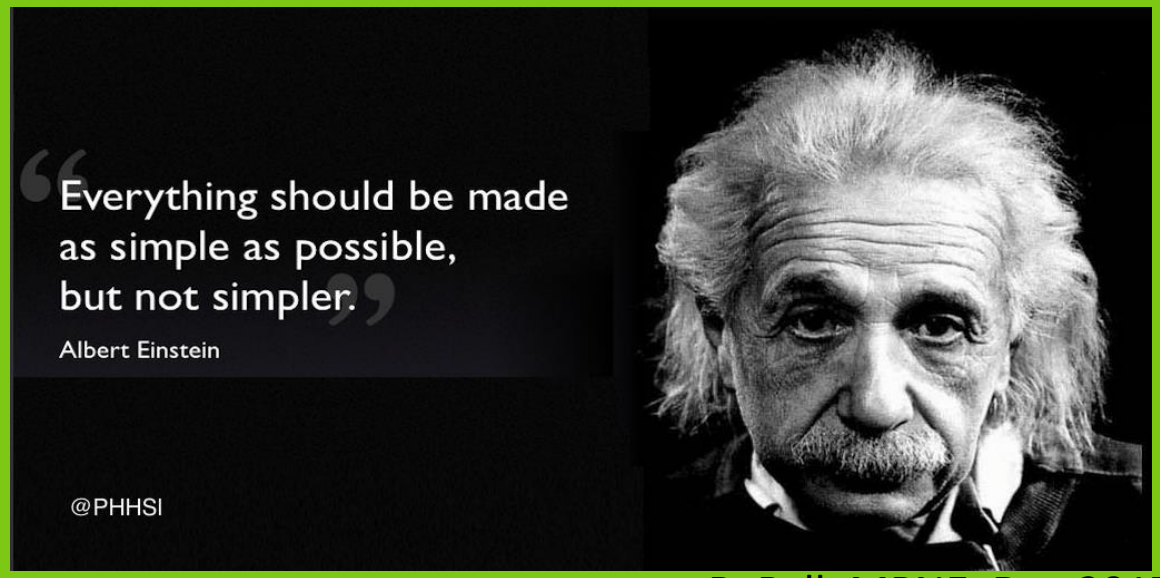
‘Science isn’t about authority, or white coats, it’s about following a method’
Ben Goldacre in The Guardian



Knowledge is infinite

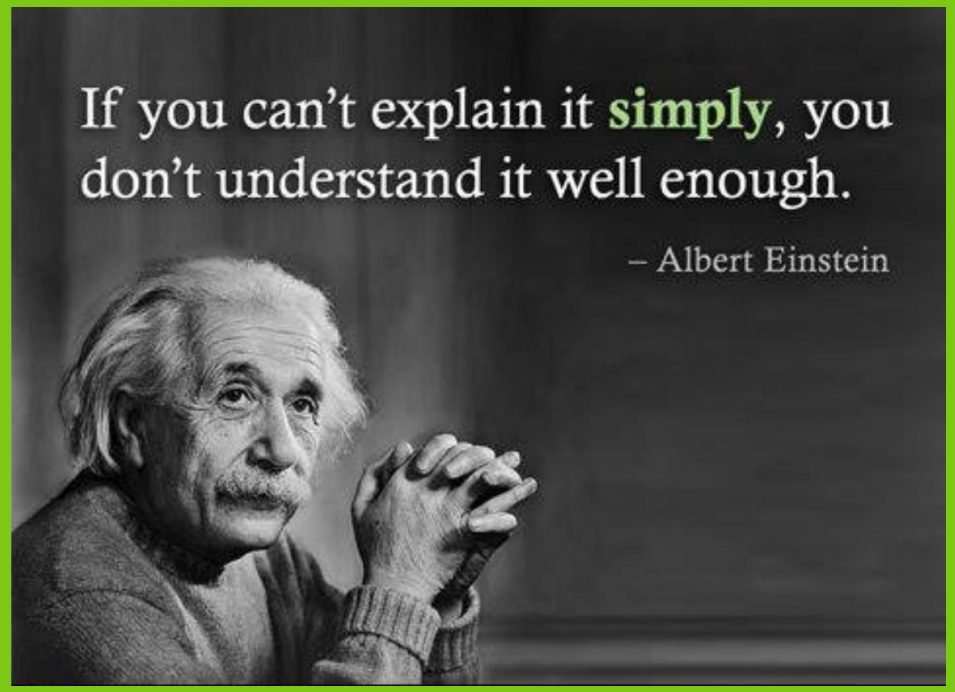
- No one needs permission to learn
- The learner defines how much knowledge is ‘enough’
- simple is hard

how we think about knowledge



People can learn

It the motivation and effort that make the difference and...



how we think about learning

The bad news

it DOES require effort
as it is a skill to master.

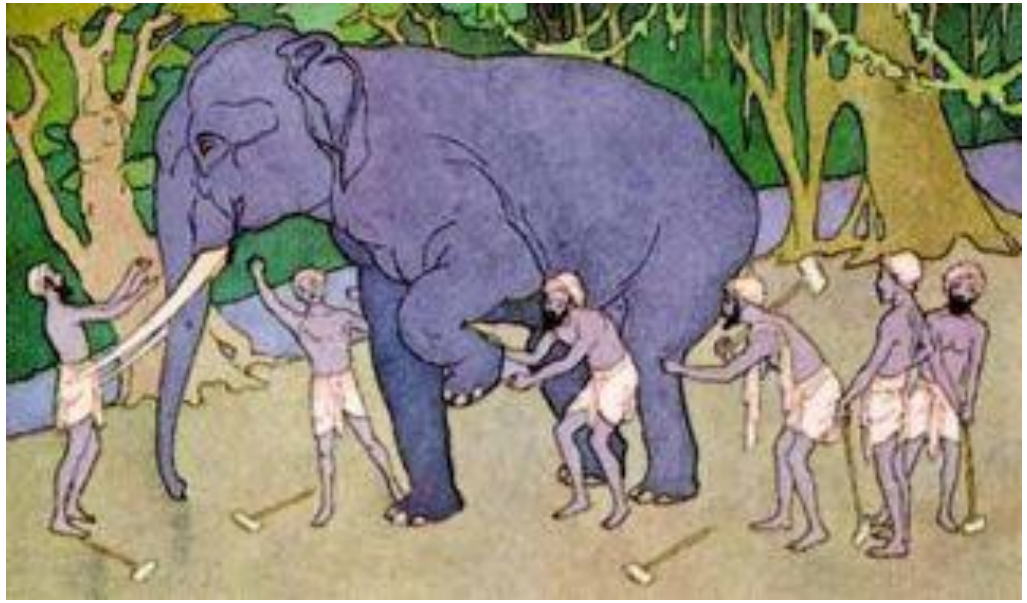
So thank you for being here!

The essence of modern Science.

Falsification- or the art of nit-picking.

B. Ryll, MPNE, Dec 2017

We only ever see part of the truth



http://rhinestonearmadillo.typepad.com/the_rhinestone_bookmark/2013/04/the-blind-men-and-the-elephant-fairy-tale-art.html

Verification versus **Falsification.**
The difference between **being right** and **not being wrong.**

Modern Science assumes that we always miss a part of the total truth- there is always something we don't know.

This means that we can never really prove we're right about- or VERIFY- our claims.

We can only prove we are not wrong by checking for all potential mistakes- and once we've shown endless ways of not being wrong, people will generally accept we're (kind of) right. FOR NOW.

So reading papers is about looking for potential mistakes that could mean that the authors' interpretation is wrong.

Quantitative versus qualitative research

Not everything that matters can be measured and not everything that can be measured matters.

Anything that can be measured- numbers

- Surveys
- Interviews
- Focus groups

Quantitative versus qualitative research

Not everything that matters can be measured and not everything that can be measured matters.

Many fields of research relevant to patient advocacy

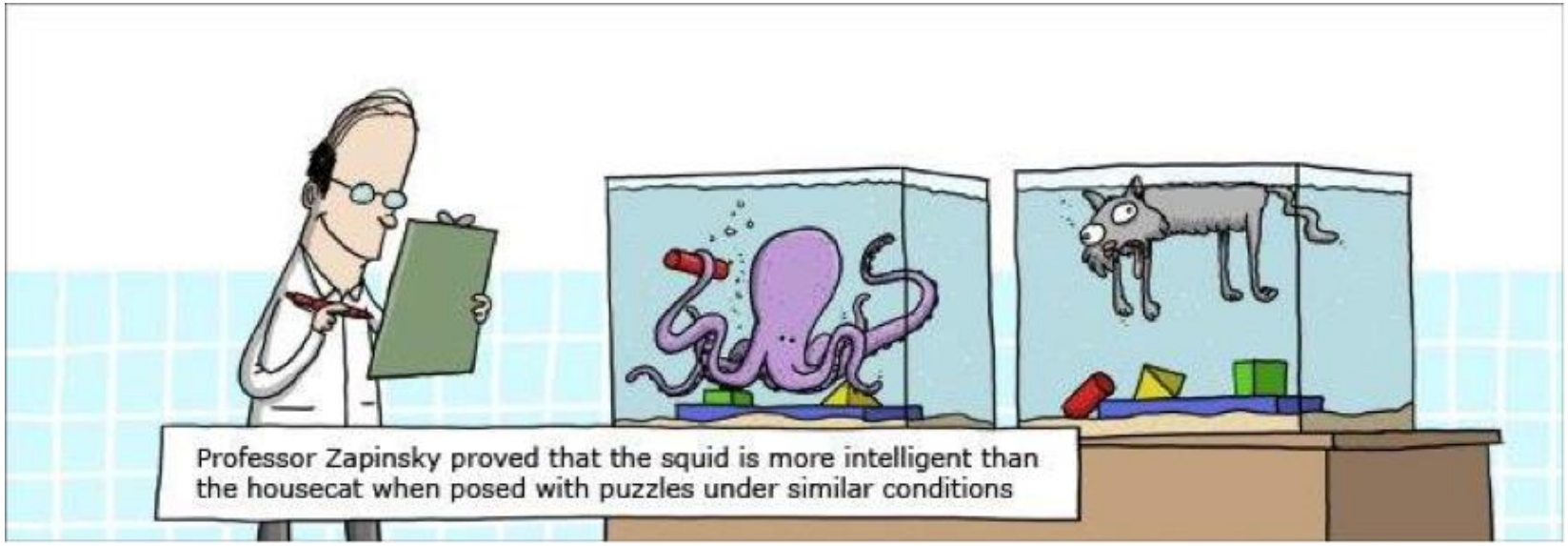
- Fundamental biological research
- Clinical Medical research (clinical studies)
- Epidemiological research (population-based research)
- Health Systems research (how to healthcare systems perform, e.g. waiting times for patients)
- Health-economic research
- Sociological research
- Political research

…

How we started in Melanoma….

MPNE November 2015 workshop

Reading Scientific Papers for Melanoma Advocates



Krusenberg Herrgård, 27th- 29th November 2015

http://www.melanomapatientnetworkeu.org/mpne-workshop-2015.html

1. Why reading original scientific medical literature?

Time

- scientific publications appear before the knowledge diffuses through other media
- not all physicians are up to date on the latest progress in your specific disease

Correctness

- frequent misleading reporting of scientific findings in the general media
- lay versions often omit important details (like presenting a 'cure for cancer' while in reality, the research was only performed in cells in a dish)

Empowerment

- 'great power comes with great responsibility'- being informed is one of them

Read

Ben Goldacare on Misreporting in Science

2. Where to find scientific papers?

The GOOGLE of medical literature

Pubmed

http://www.ncbi.nlm.nih.gov/pubmed/

publicly available database with biomedical literature based on Medline, another data repository.

Has a good ‘how-to’ introduction

but only as good as
you know what to look for.

For a successful search, you need to know for **whom, what** and **where** to look for.

Whom?

Look for the established authorities in your disease area.

Not sure?

Look at the LARGE medical meetings and see who is showing up everywhere- usually, the community is limited and you quickly find out who works on what.

ASCO

ESMO

ECCO

your specific disease area

What?

The latest medical meetings- often with very well-done reports- podcasts- articles- will give you an idea about what the current problems in your specific disease area are-

the **big unanswered questions**.

Note- for established treatments, society guidelines will be a better source of information.

Where?

Although Pubmed only lists medical literature, the number of medical journals is continuously growing and it neither possible nor advisable to read all of them.

There are 2 very **highly regarded** medical journals- the Lancet and the New England Journal of Medicine.

Basic research you are most likely to find in Nature or Science.

Plus usually 1 or 2 **highly regarded** disease-specific journal.

It is worth spending some time on these websites as they offer additional blogs, podcasts and comments, often designed for the non-scientist- these can be extremely valuable.

Highly regarded-
the impact factor debate.

- Regard for a Scientific Journal in terms of the quality of the Science published there is measured by the impact factor (the higher, the better).
- There is an ongoing debate whether impact factors are a good way to measure the quality of science (probably not) but for the time being, it is the only proxy people seem to agree upon[1,2].

1- 'OHHHH, we shouldn't be placing so much emphasis on impact factors!! PAUSE. Have you seen THAT last publication in Nature?!!!'

2- Research funding is largely distributed based on publication record- how many publications in high-impact journals.

3. How to access an article

Once you found an interesting article on Pubmed, you will unfortunately often find out that it is **pay-walled,** so only accessible against payment.

Open access to medical information is a formidable advocacy topic in itself, so here some ideas:

- the Open Access button initiative
- write to the first or corresponding author on the paper and ask for a pdf version- this tends to work. You can check on here whether the journal allows the author to share the article- most do, albeit often with strings attached.
- register via your organisation on ResearchGate for free under 'other'- it allows you to upload your own publications and easily contact authors to send you an author's copy of their paper.
- some journals offer free access for patients, e.g. PatientACCESS
- ask your oncologist to send you the pdf- they often have institutional subscriptions and advocate that patients without whom none of this research would exist, should get free access to all scientific publications.

we are working on this in MPNE- hopefully more to come soon!

4. How to read a scientific paper

The critical point is to find out whether you trust the results or not- and why.

So the most important part of the paper are the **RESULTS**, usually presented in **Figures**.

You want to make up your own mind and not be influenced by what the authors think- they might be wrong after all and will definitely try to present their data in the most favorable light.

Looking at the results before reading their interpretation is therefore smart!

The anatomy of a scientific paper

- Abstract
- Introduction
- Materials and Methods
- **Results**
- Discussion
- Conclusion

Note- the Journal prescribes the order of the sections, so Materials and Methods are often at the end, and some have a combined Discussion/ Conclusion.

‘Don’t trust statistics you haven’t faked yourself’

Why the devil is in the detail and having a best friend statistician is worth a lot.

B. Ryll, MPNE, Dec 2017

My personal recipe

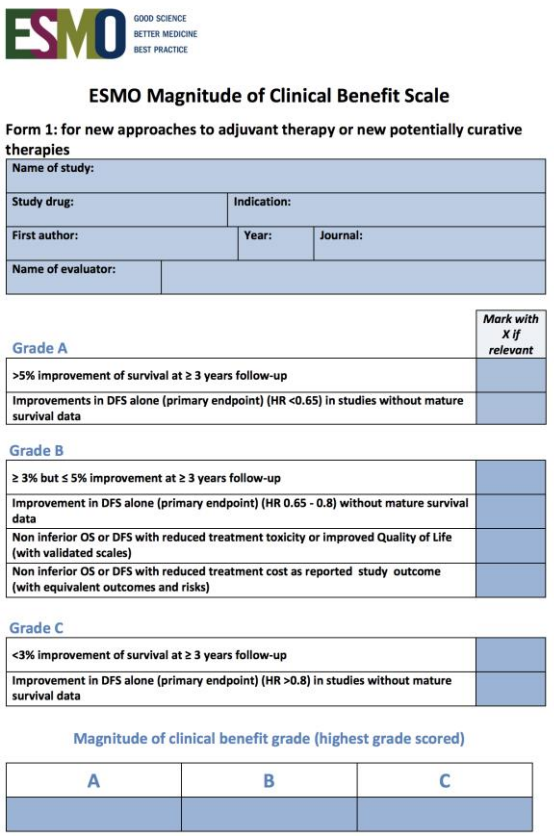
- read the abstract- what is this about?
- go to the figures- what does the data look like?
- read the results section: have I seen this in the figures?
- if anything unclear, check with materials and methods: how was this data generated?
- read introduction (if I managed to resist until now)
- go back to results in case I've missed something
- read discussion and conclusion- do I agree?
- sigh about the last sentence 'and now we are going to save the world/ cure cancer/ other highly hypothetical sentences sure to be picked up by mass media'

B. Ryll, MPNE, Dec 2017

ESMO- MCBS

- Helps to think systematically and to compare different therapies- great for journal clubs
- Original focus on ‘hard’ endpoints overall survival, progression-free survival, severe toxicity
- Limited ability to deal with immature data sets

http://www.esmo.org/Policy/Magnitude-of-Clinical-Benefit-Scale



ESMO GOOD SCIENCE BETTER MEDICINE BEST PRACTICE

ESMO Magnitude of Clinical Benefit Scale

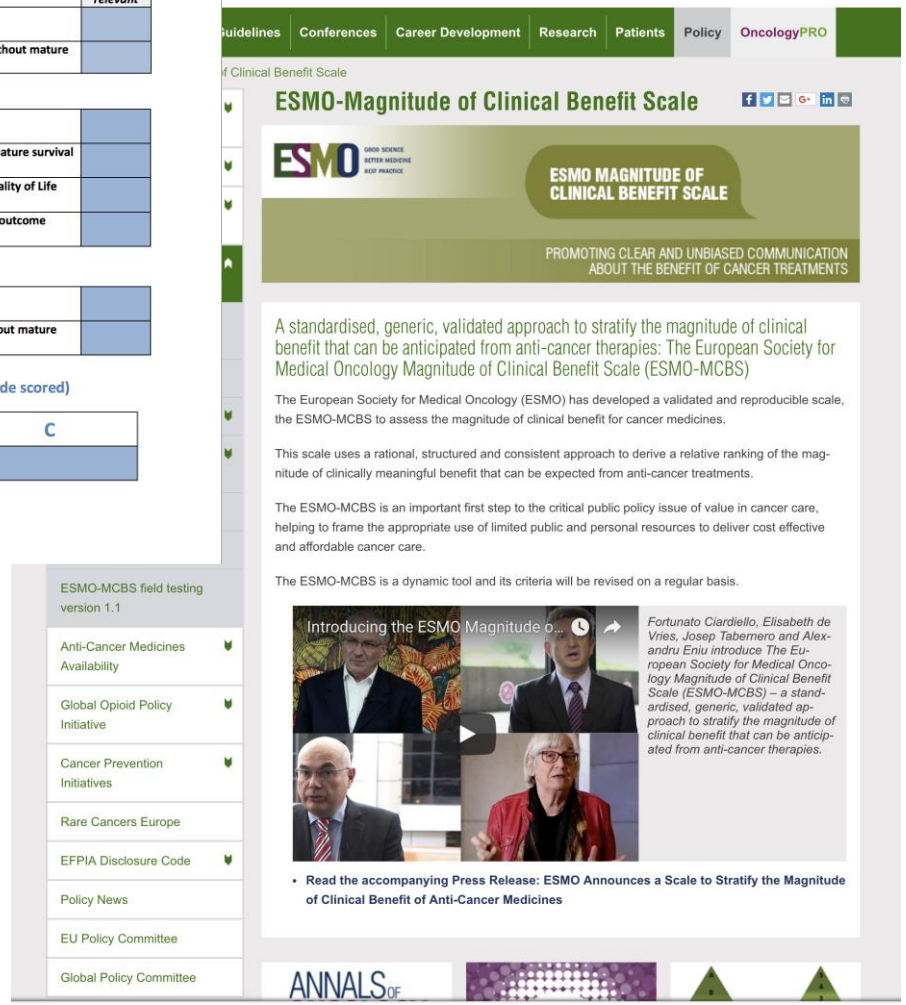
Form 1: for new approaches to adjuvant therapy or new potentially curative therapies

Name of study:		
Study drug:	**Indication:**	
First author:	**Year:**	**Journal:**
Name of evaluator:		

	Mark with X if relevant
Grade A	
>5% improvement of survival at ≥ 3 years follow-up	
Improvements in DFS alone (primary endpoint) (HR <0.65) in studies without mature survival data	
Grade B	
≥ 3% but ≤ 5% improvement at ≥ 3 years follow-up	
Improvement in DFS alone (primary endpoint) (HR 0.65 - 0.8) without mature survival data	
Non inferior OS or DFS with reduced treatment toxicity or improved Quality of Life (with validated scales)	
Non inferior OS or DFS with reduced treatment cost as reported study outcome (with equivalent outcomes and risks)	
Grade C	
<3% improvement of survival at ≥ 3 years follow-up	
Improvement in DFS alone (primary endpoint) (HR >0.8) in studies without mature survival data	

Magnitude of clinical benefit grade (highest grade scored)

A	B	C



Guidelines | Conferences | Career Development | Research | Patients | Policy | OncologyPRO

f Clinical Benefit Scale

ESMO-Magnitude of Clinical Benefit Scale

ESMO MAGNITUDE OF CLINICAL BENEFIT SCALE

PROMOTING CLEAR AND UNBIASED COMMUNICATION ABOUT THE BENEFIT OF CANCER TREATMENTS

A standardised, generic, validated approach to stratify the magnitude of clinical benefit that can be anticipated from anti-cancer therapies: The European Society for Medical Oncology Magnitude of Clinical Benefit Scale (ESMO-MCBS)

The European Society for Medical Oncology (ESMO) has developed a validated and reproducible scale, the ESMO-MCBS to assess the magnitude of clinical benefit for cancer medicines.

This scale uses a rational, structured and consistent approach to derive a relative ranking of the magnitude of clinically meaningful benefit that can be expected from anti-cancer treatments.

The ESMO-MCBS is an important first step to the critical public policy issue of value in cancer care, helping to frame the appropriate use of limited public and personal resources to deliver cost effective and affordable cancer care.

The ESMO-MCBS is a dynamic tool and its criteria will be revised on a regular basis.

Fortunato Ciardiello, Elisabeth de Vries, Josep Tabernero and Alexandru Eniu introduce The European Society for Medical Oncology Magnitude of Clinical Benefit Scale (ESMO-MCBS) – a standardised, generic, validated approach to stratify the magnitude of clinical benefit that can be anticipated from anti-cancer therapies.

- **Read the accompanying Press Release: ESMO Announces a Scale to Stratify the Magnitude of Clinical Benefit of Anti-Cancer Medicines**

ESMO-MCBS field testing version 1.1

Anti-Cancer Medicines Availability

Global Opioid Policy Initiative

Cancer Prevention Initiatives

Rare Cancers Europe

EFPIA Disclosure Code

Policy News

EU Policy Committee

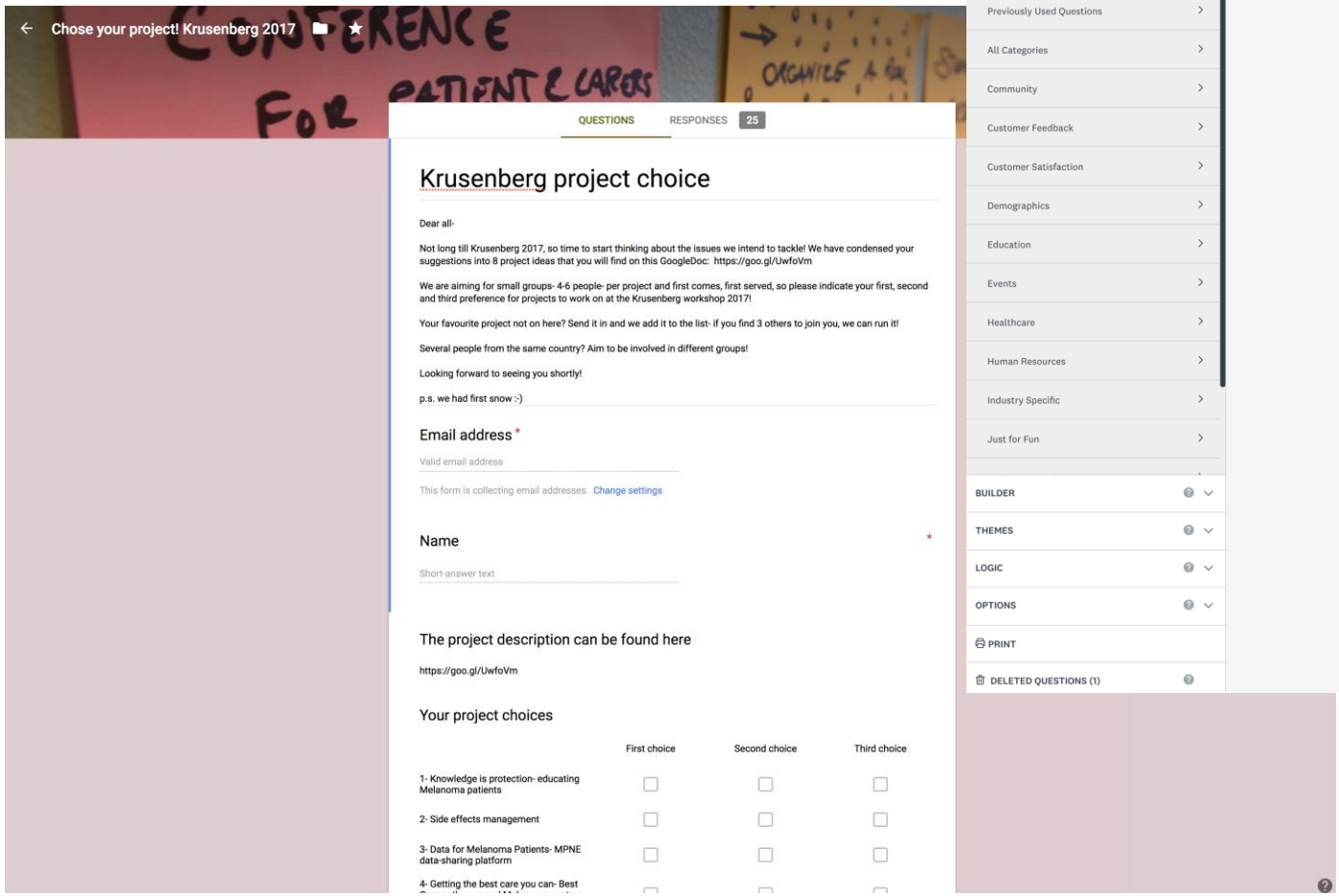
Global Policy Committee

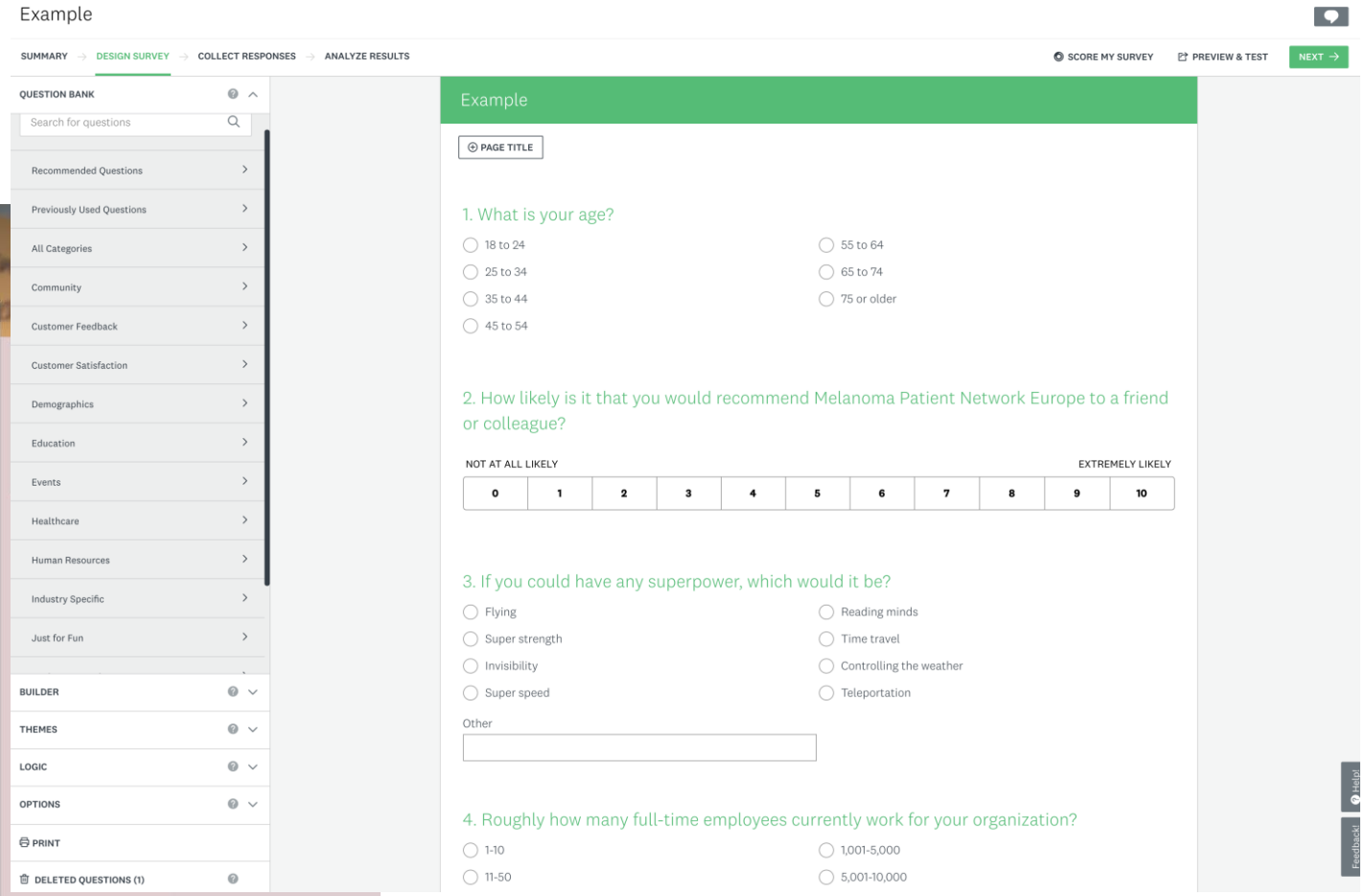
ANNALS OF

How to get started

- Start reading original publications in your field of disease
- Find like-minded people for discussions- form a Journal Club
- Find a filing system for your papers- it can get really messy fast
- Establish clear rules on your patient forums: any claim, especially regarding health, needs to be supported by evidence- the source where it comes from
- Start underpinning your advocacy claims with data, however basic
- Try to understand what GOOD research in any given field looks like

Useful tools





Survey monkey: pay for relevant features, less stylish, powerful functions like reminders and good analyses

Google forms : free, pretty, limited data analysis

Summary

- Argumentation based on evidence is powerful
- Scientific thinking is learned and requires training
- The ESMO- MCBS is a useful tool for a first judgement of clinical evidence- but be aware of its limitations

Thank you

bettina.ryll@mpneurope.org