



Why do we need to use evidence-based advocacy? And HOW do we do it?

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MPE Advocate Development Programme @ European Cancer Summit 17/11/2020

Topics of this session

Part 1

- **Why use evidence in advocacy?**

Why "patient knows best" is not always the most impactful approach...

- **Evidence-based advocacy** – what is this?

- **Examples for evidence-based advocacy** in research, regulatory, health policy – and some learnings

- **Methodology** – why it counts

Part 2:

- **European CanCer Organisation Summit 2020 Agenda**
and how would evidence help us argue our case?

Why are we advocates?

We want impact for the good of patients

- **Support patients** and their families affected by a challenging disease to make the right choices
- **Help clinicians** to give the best service possible to their patients, and prevent bad and outdated practice
- **Influence regulators and payors** to make sure they are basing their decisions on patient preferences and needs
- **Tell politicians to do policy *for* patients**, not just about patients

Patient advocacy operates on three levels



Patient Support

- Inform, support, navigate



Health Policy

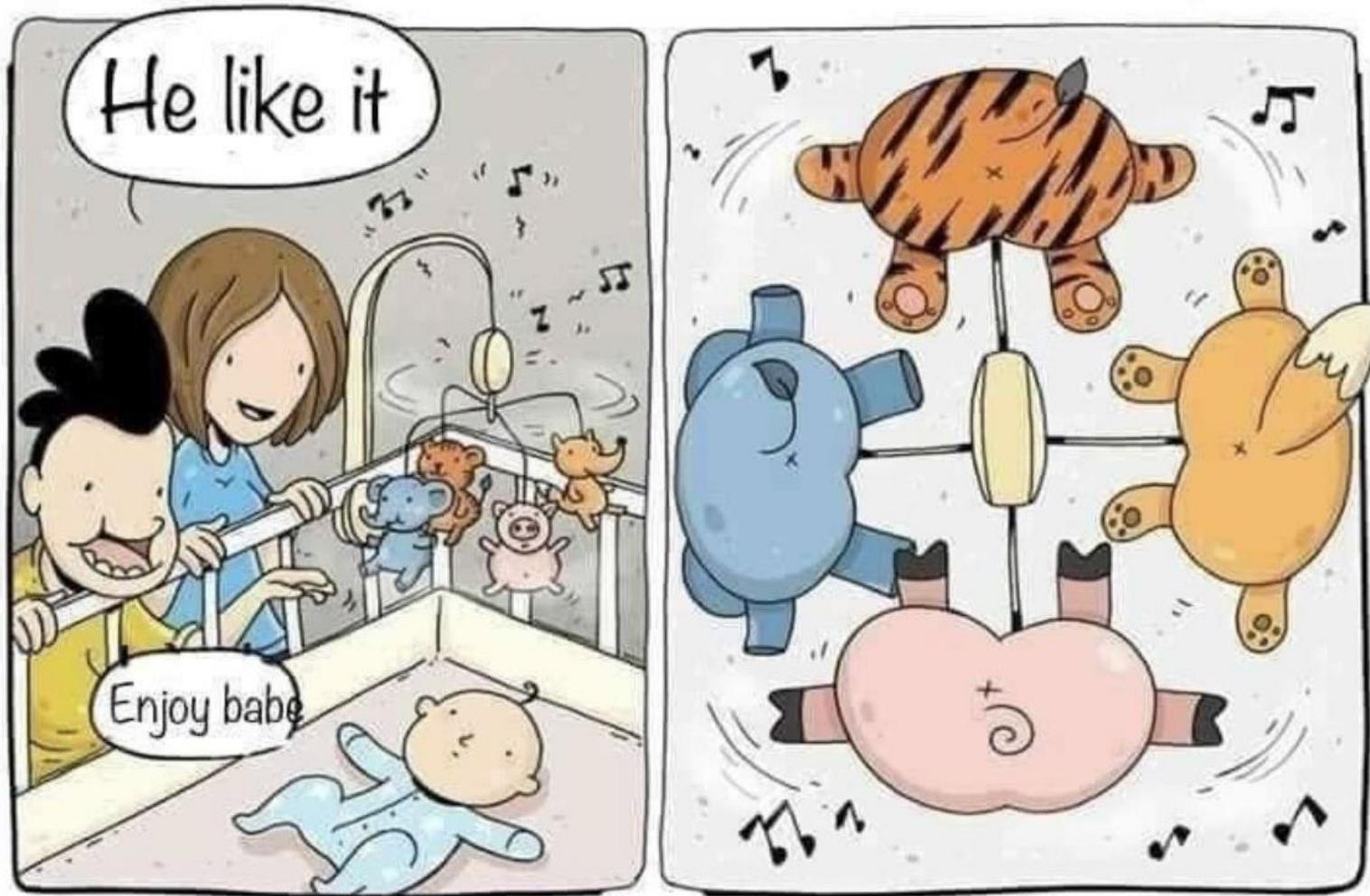
- Influence health policy, patient care



Research

- Contribute in partnership with clinicians, networks and industry

It's often a matter of perspective...



Courtesy to Zack Pemberton-Whiteley @ZPWLC

Is „patient knows best“ any better than „doctor knows best“? Base it on data!



Unattractive choices/trade-offs are often the norm for patients, unfortunately



The unknown



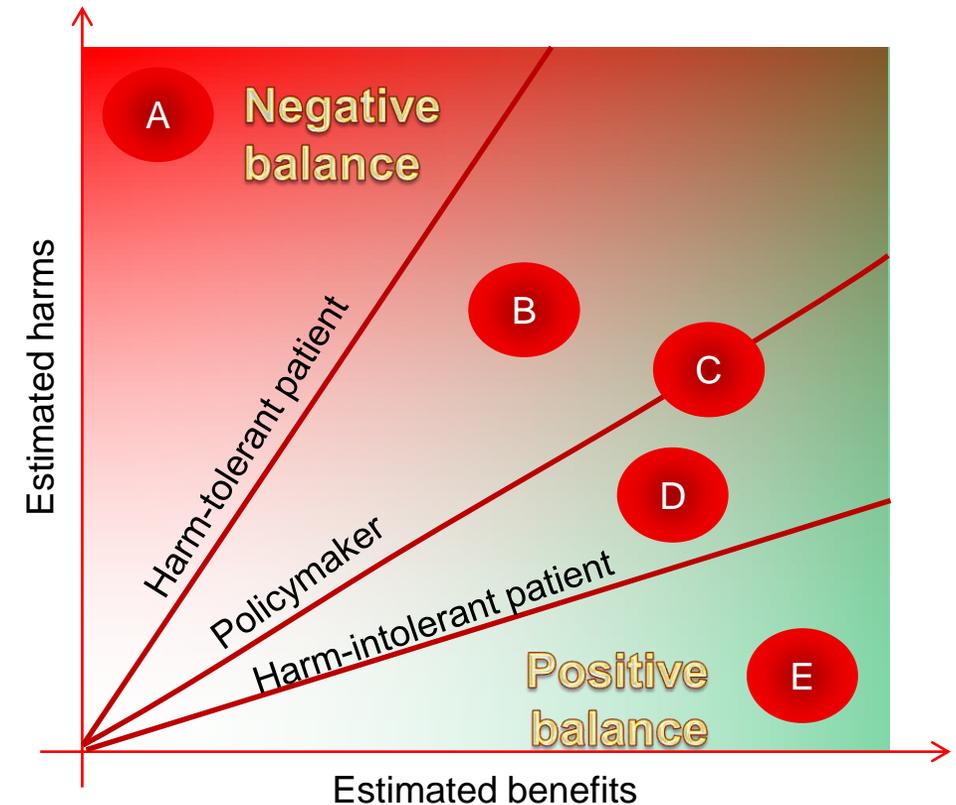
The bad



The ugly

Same disease, same stage, same age. Different preferences. Patients are not the same.

- Same disease, same stage, same side effects, same age.
- But: Different preferences
- We need solid evidence on what patients want and need..

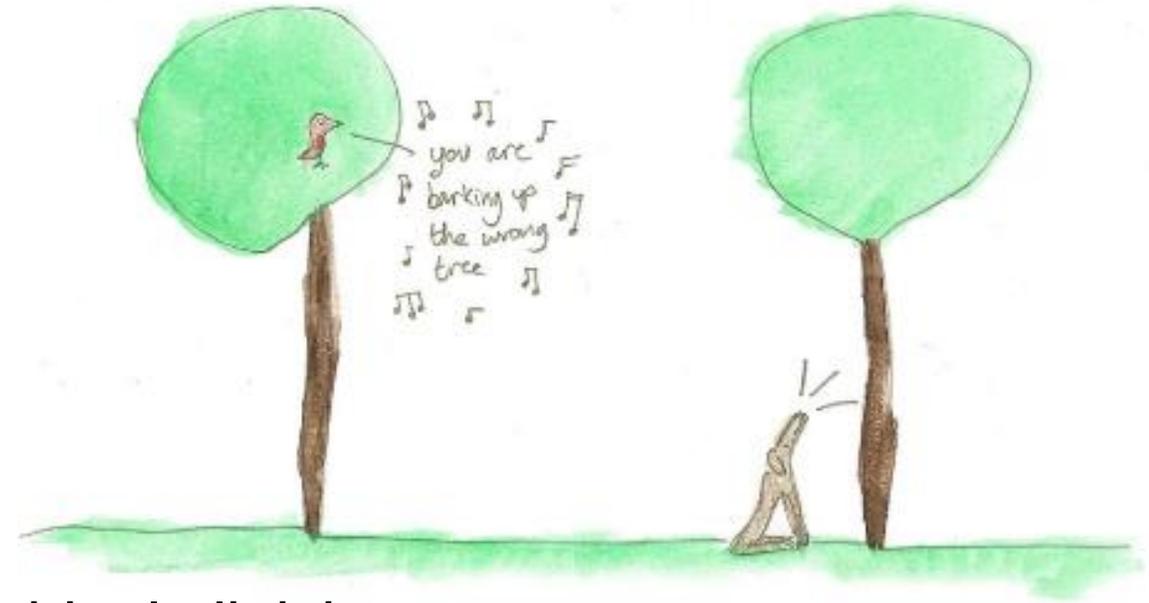


Slide adapted from Francesco Pignatti (EMA)

Know your trees – and bark up the right one

Think about who you'd like to influence:

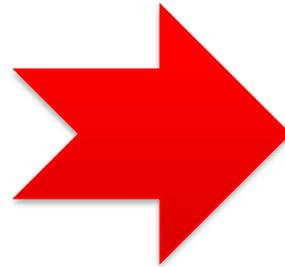
- EU level vs. national level
(e.g. EMA, HTA/reimbursement)
- Medical societies vs. study groups vs. individual clinicians
- Companies (→ drugs) vs. industry associations (→ systems)
- Disease-specific action (→ Osteoporosis)
vs. cross-disease joint action (→ healthcare system)
- Know what you can do, and what your umbrella organisations can do best,
within the limits we all have



Scientific and policy work go hand in hand



**Know what
you want**



**Know how to
achieve it**

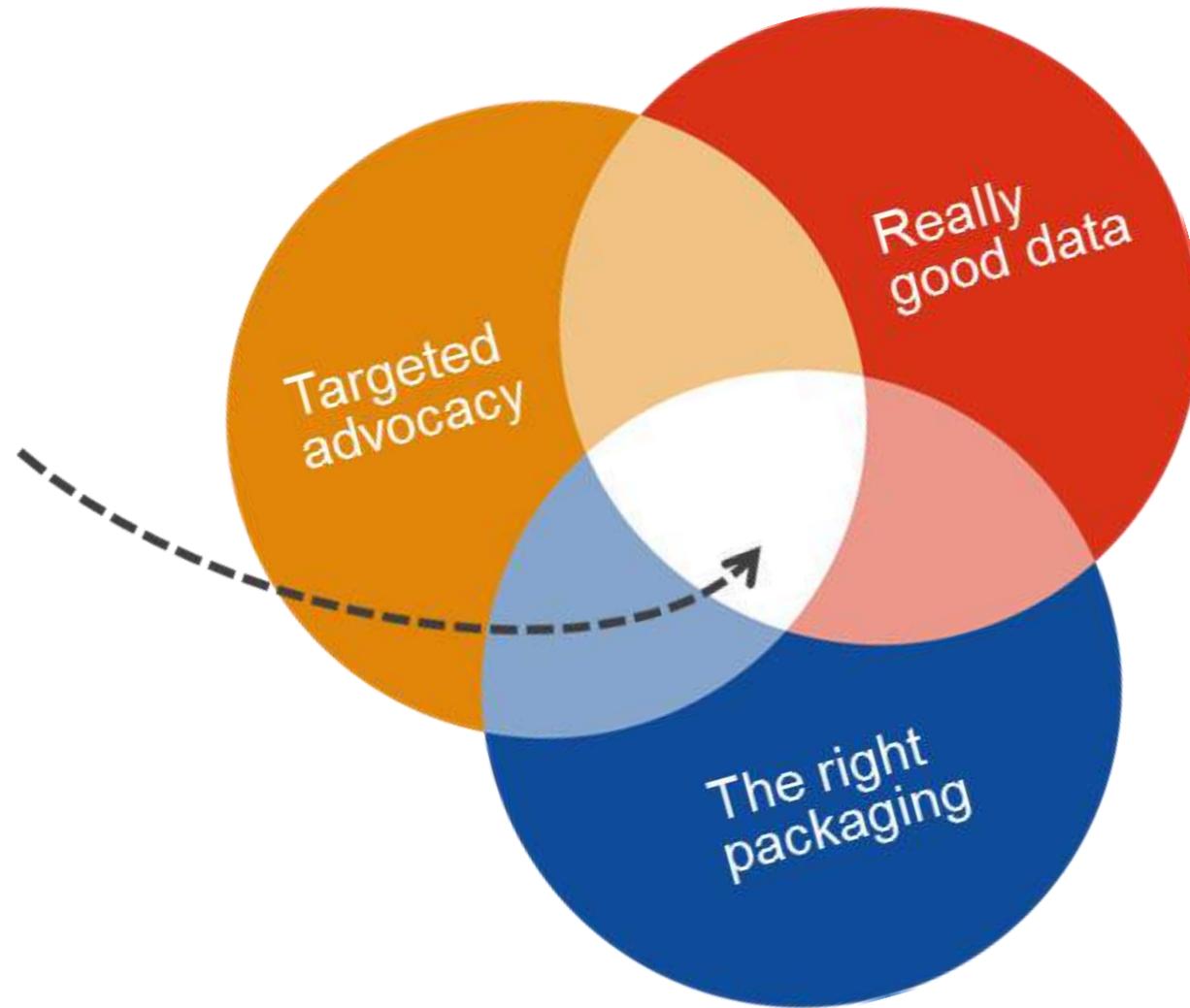
Negotiation tactics and building your case



- 1 • Define the outcome and if possible, a fall-back position (alternative outcome)
- 2 • Consider the other party's position, possible resistance
- 3 • Be clear with your reasoning, the benefits, the risks
- 4 • Present evidence and proof to reassure
- 5 • Make your delivery compelling
- 6 • Explore barriers and resistance with empathy
- 7 • Represent your case, ask for commitment

Evidence-based advocacy

Advocating in a targeted, evidence-based, well-educated and professional manner, and measure impact and outcomes of what we do





Generating evidence in patient advocacy

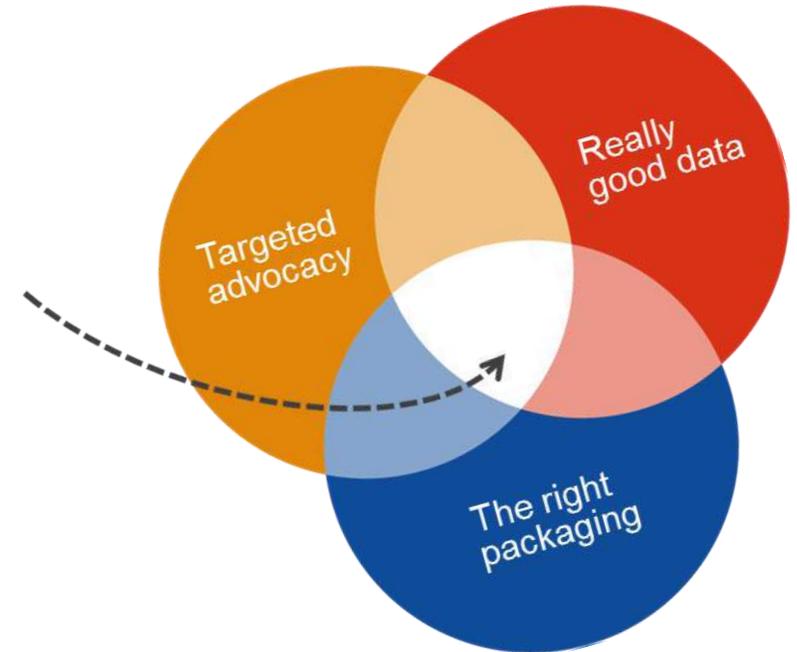
Challenges of patient organisations to generate data for advocacy

- Numerous presentations (e.g. IKCC, MPE, MPNE, MPE, WECAN Academy) about evidence-based advocacy have strongly increased awareness and importance of evidence-based advocacy in our community
- However, only a small number of patient organisations have
 - the **knowledge on how to generate data** in a methodologically sound way
 - the capacity to **implement** an evidence generation project
 - the knowledge on how to **use the data** in advocacy
 - knowledge, capability and resources to **publish the data**
 - a **strategy** to ensure the generation of data not only when invited/encouraged by industry



Meaningful data that patient groups can generate

- **Adherence** to therapies
- **Inequalities in real-world access** to diagnostics or therapies
- **Current care patterns**
- **Quality of Life, burden of disease, daily lived experience** in real world
- **Impact of illness** on society
- **Disease-related outcomes**
- **Patient preferences on benefit/risk**



How to use evidence..

1. Listen to the evidence
2. Act on the evidence

DO NOT use evidence just to back up your existing point of view



I see here that I'm right about everything.

Examples for evidence generation & use in patient advocacy

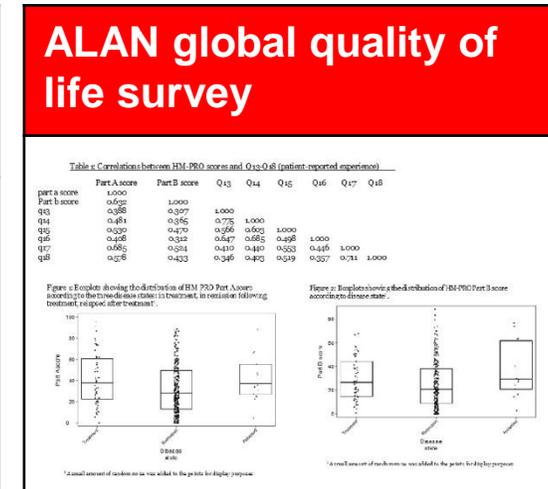
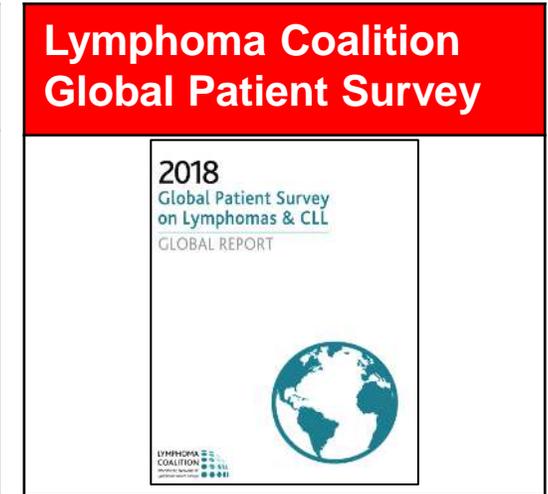
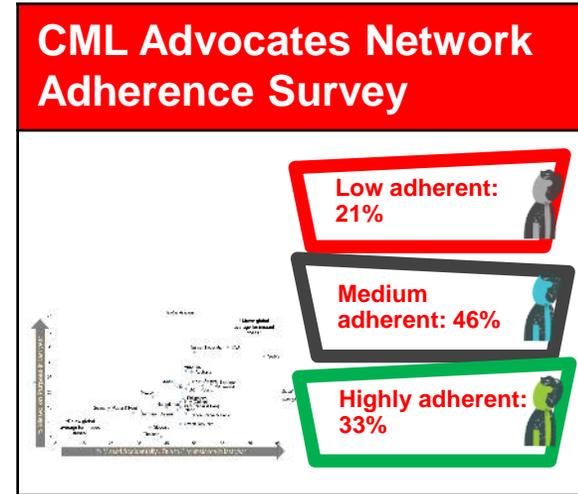
1. Disease mapping
incl. patient preferences
2. Inequalities
3. Health policy

Examples for evidence generation & use in patient advocacy

- 1. Disease mapping
incl. patient preferences**
2. Inequalities
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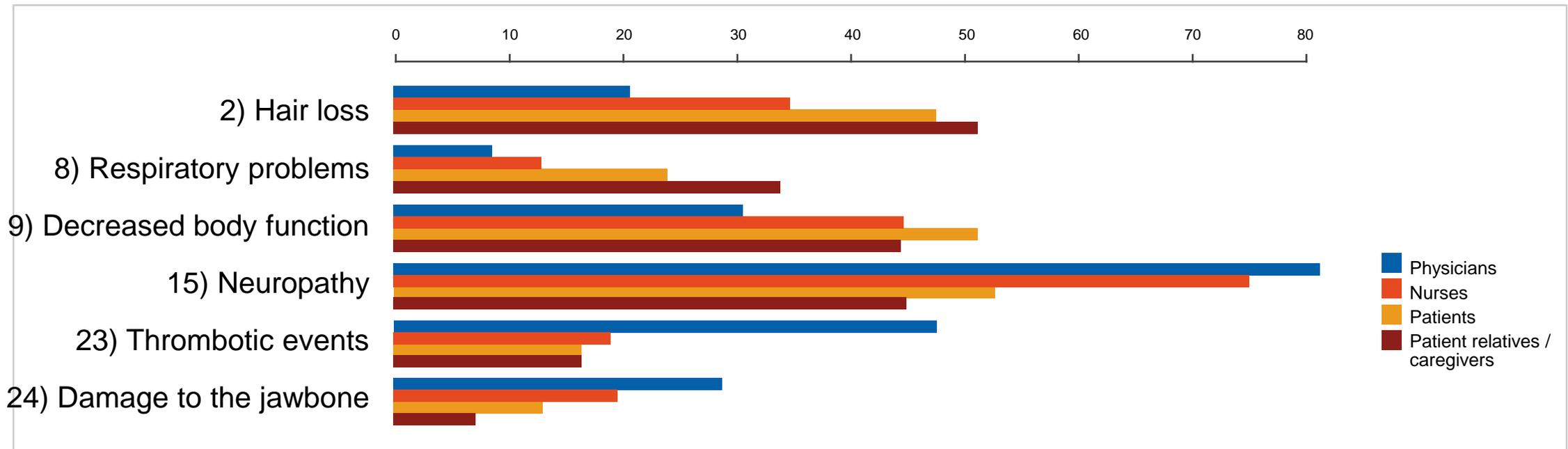
Disease Mapping

- Most types of evidence generation done by patient advocacy groups involves disease mapping (in different forms)
- Often used to look at areas such as:
 - Patient experience
 - Quality of life
 - Burden of disease and unmet needs
 - Patient preferences
 - A specific issue – e.g. adherence
- The simplest category of evidence generation we'll look at



Patients have a unique perspective on side effects. “KOL” (HCP) doesn’t always know best

Treatment side-effects with the most negative impact on overall well-being (%)



Detecting Myeloma, ways to shortening an often painful and tedious patient odyssey: Results from an international survey. Myeloma Euronet (2009).

314 physicians & nurses, 260 patients & carers, 43 countries

ALAN Quality of Life Survey



ALAN
Acute Leukemia Advocates Network

 **Global Quality of Life Survey (10+ Languages)**

 **Aim: identify factors associated with QoL differences in acute leukemia**

 **Hypotheses Questions: Test against HM-PRO to assess how they impact on quality of life**

 **Follow Up Questions: To gather further information on patient experience of each hypotheses**

Identifying differences in the quality of life of patients with acute leukemia: a global survey
Zack Pemberton-Whiteley¹, Jan Gieseler², Sophie Wittmich³, Esther Oliv...
¹- Acute Leukemia Advocates Network; ²- HM-PRO; ³- Quality Hea...

Acute leukemia patients more likely to have a reduced quality of life if they:

- are undergoing treatment
- are female
- report a worse patient experience.

GLOBAL QUALITY OF LIFE SURVEY

A survey of patients to understand their experiences, key issues, and unmet needs throughout their journey with acute leukemia.

Abstract

Introduction: The aim of this study was to investigate the quality of life (QoL) of patients with acute leukemia (AL) and to identify factors associated with QoL differences in acute leukemia.

Methods: The study included 100 patients with AL who completed the HM-PRO questionnaire. The study included 100 patients with AL who completed the HM-PRO questionnaire. The study included 100 patients with AL who completed the HM-PRO questionnaire.

Characteristic	Number
Male	50
Female	50
Undergoing treatment	30
Not undergoing treatment	20
Reported worse patient experience	15
Reported better patient experience	15

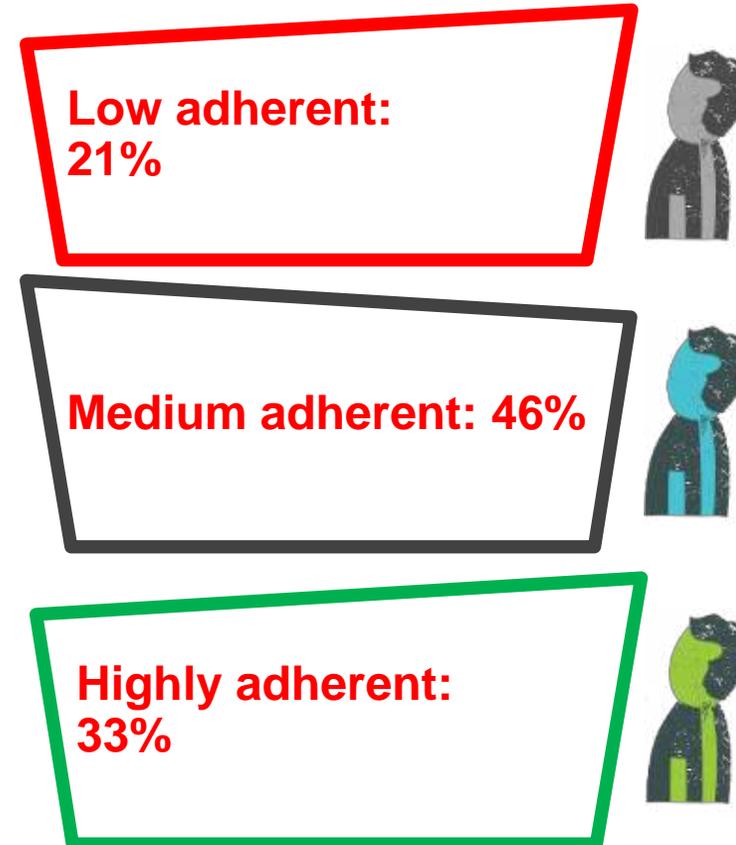
Results: The study included 100 patients with AL who completed the HM-PRO questionnaire. The study included 100 patients with AL who completed the HM-PRO questionnaire.

Deploying evidence in science advocacy: CML Advocates Network Survey: Other than HCPs and industry expected, adherence to CML therapy is poor



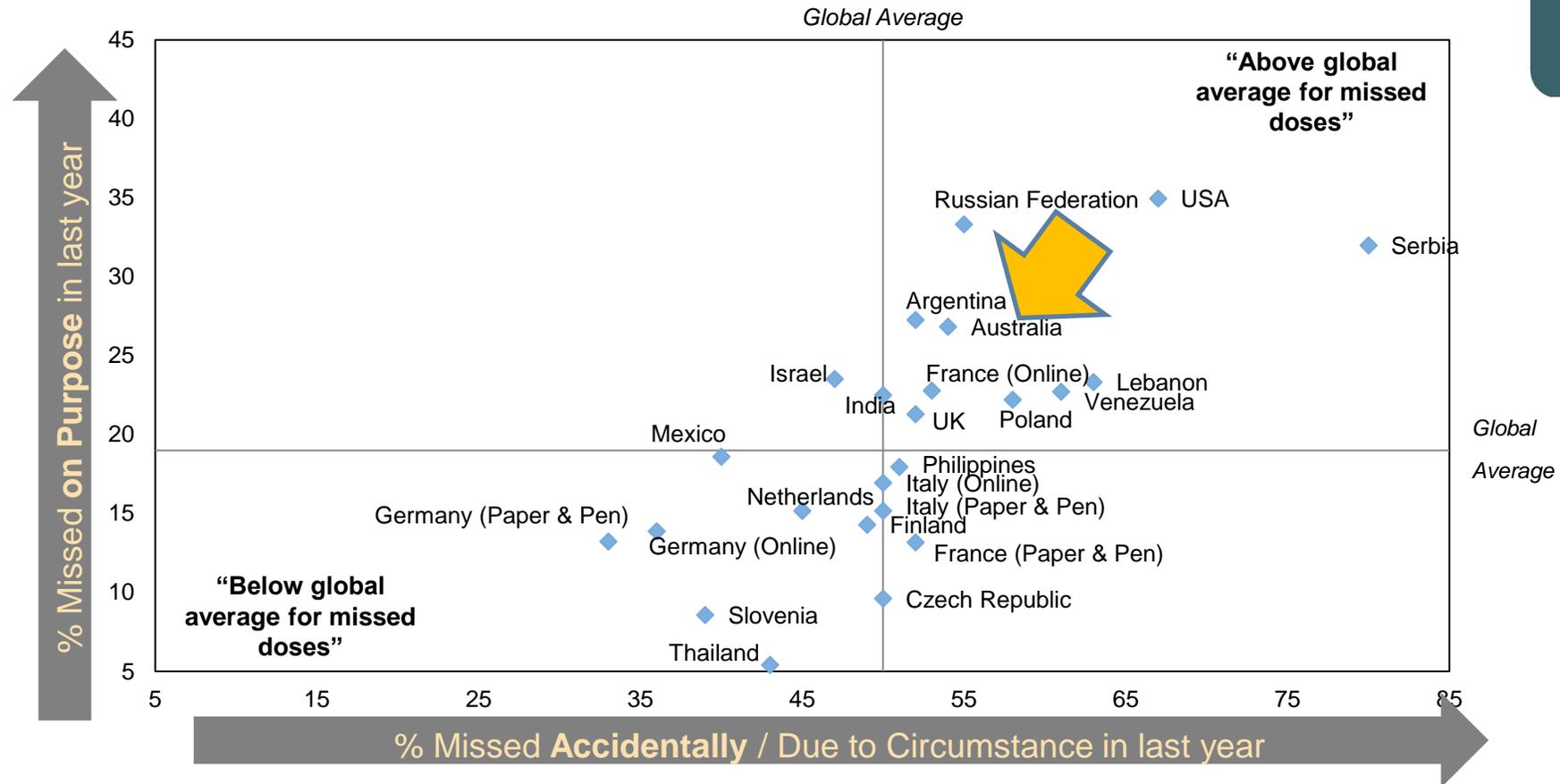
Patient-led adherence research in CML:

- 12 languages, 2546 patients from 63 countries recruited in 3 months
- Use of validated adherence instrument
- Changes in dialogue between patients and clinicians
- Changes in treatment paradigm



www.cmladvocates.net/adherence Factors influencing adherence in CML and ways to improvement: Results of a patient-driven survey of 2546 patients in 79 countries. Geissler et al, Journal of Cancer Research and Clinical Oncology (2017), DOI: 10.1007/s00432-017-2372-z

CML Adherence Survey: Rock-solid, multinational data to convince the „KOLs“



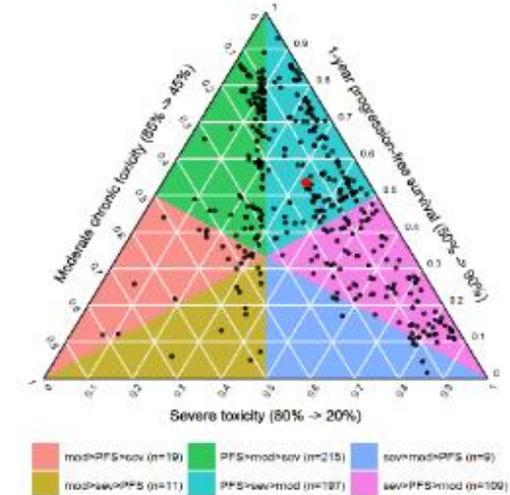
C2a / base=all respondents (n=2546) - Patients sometimes are not able to take their medication as prescribed. In the last month, have you missed a dose accidentally or due to circumstances that were outside of your control? C2c / base=all respondents (n=2546) - In the last year, have you missed a dose accidentally or due to circumstances that were outside of your control? C4a / base=all respondents (n=2546) - Patients sometimes make a conscious decision to miss a dose of medication. In the last month, have you decided to miss a dose? C4b / n=2258 - In the last year, have you decided to miss a dose?

Patient preference studies

“Patient preferences are concerned with **measuring the patient's value** for a specific component, or attribute, either in absolute terms or in relation to another attribute. The **relative importance** is identified by choices that inevitably require **trading off** one or more desirable outcomes (including price/co-payment) in a given area (or domain) in order to obtain a more desirable composite outcome.”

- Patient preference studies are highly complex and the most difficult type we'll look at

MPNE & MPE & EMA Patient Preferences on Benefit-Risk

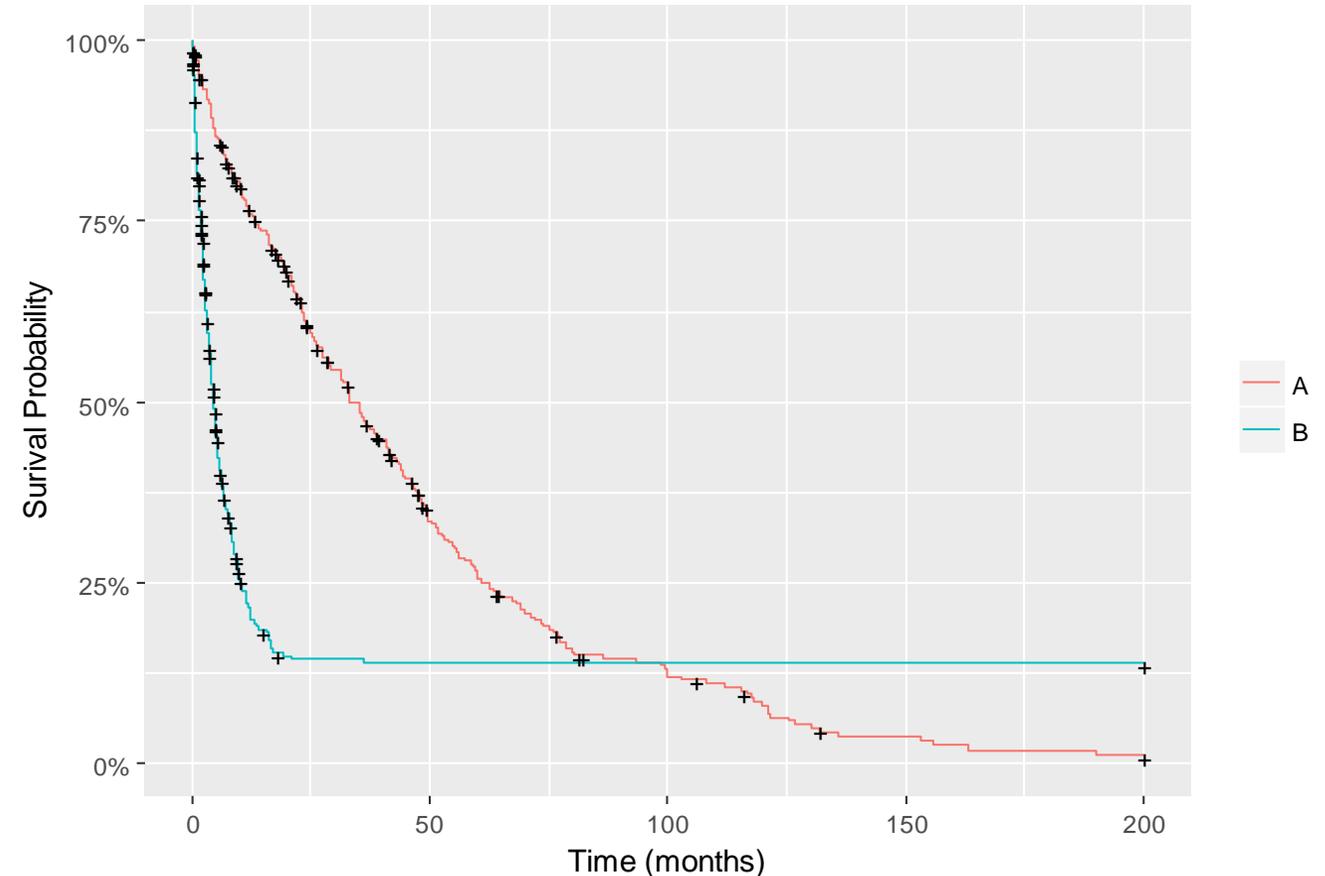


<https://www.ispor.org/docs/default-source/sig-documents/Patient-Preference-Methods.pdf>

Preferences vary a lot depending on who expresses them

In the following example:

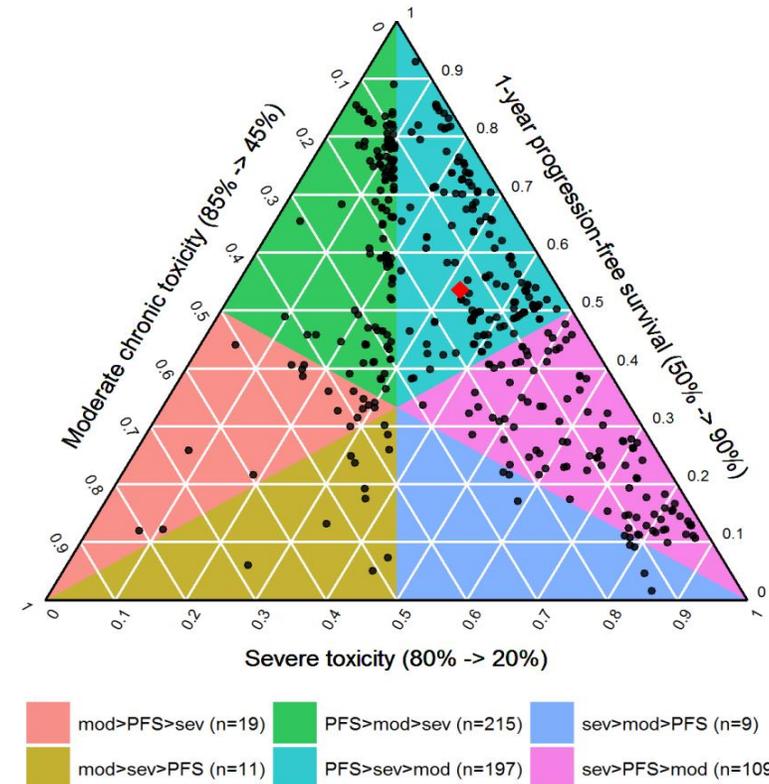
- Drug A:
 - 50% of patients will be alive in 3 years
 - all patients will be dead in 8 years
- Drug B:
 - 85% of patients will be dead in 2 years
 - 15% patients with long-term survival
- From a regulatory perspective, drug A might be better because more patients respond longer
- However, some patients may prefer treatment B because of the rare chance of surviving



Slide adapted from Francesco Pignatti

Preferences vary from one stakeholder to another, from one disease to another, and even within one single disease

- Needs and preferences have historically been captured in general as “the patient voice”
- However, there are clear subgroups within single diseases with very different preferences and risk attitudes
- In this example:
 - Considerable heterogeneity
 - Severe toxicity ranked higher among younger, working, and looking after dependent family members and who had more frequently experienced severe toxicity



Survey with 560 myeloma patients from the Myeloma UK, replicating the pilot of MPE, MPNE and EMA. D. Postmus *et al.* (2017) *The Oncologist*

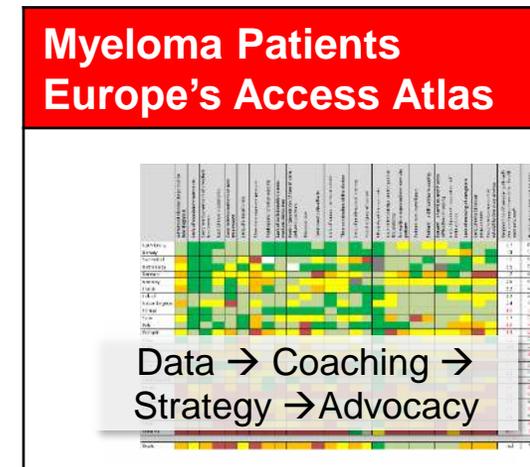
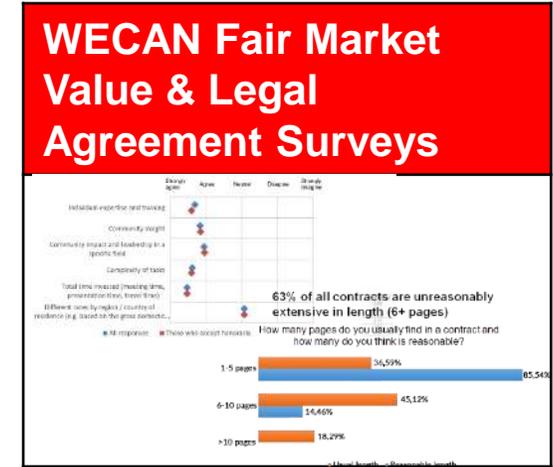
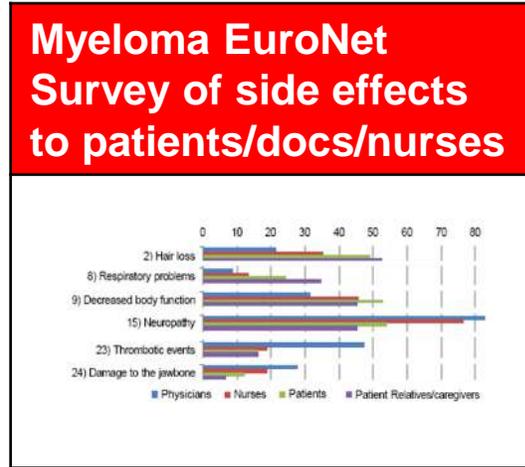
Slide adapted from Francesco Pignatti

Examples for evidence generation & use in patient advocacy

1. Disease mapping
incl. patient preferences
- 2. Inequalities**
3. Health policy

Generating data on inequalities

- Evolution of disease mapping into the more complicated ‘inequality mapping’ – looking for subgroups with differences (e.g. worst outcomes, lowest access)
- Often looked at by demographic (e.g. country, age, gender, or by a specific category)



Evidence-based advocacy on access to therapies and diagnostics

	Advanced disease stage due to late diagnosis	Lack of standard treatments	Cost/reimbursement of standard treatments	Lack of new treatments	Cost/reimbursement of new treatments	Lack of clinical trials	Slow drug approval process	Inadequate referral systems	Lack of collaboration across medical disciplines	Poor organisation of health care delivery systems	Bureaucracy	Treatment side-effects	Lack of supportive treatments	Time constraints of the doctor	Lack of professional training	Social stigma of cancer	Ethnicity of the patients	Lack of knowledge on the part of the patients	Unrealistic expectations from the patient	Patient non-compliance	Patient's difficulties in coping	Patient's families and friends difficulties in coping	Lack of patient support or self-help groups	Lack of training of caregivers	Lack of rehabilitation programmes	Cost/reimbursement of rehabilitation programmes	Proportion of people with self-declared unmet needs for health care services ¹⁶	Health expenditure in US\$ PPP per inhabitant (2010)		
Luxembourg																											0.7	6,743		
Norway																												1.3	5,426	
Switzerland																												1.0	5,394	
Netherlands																												0.5	5,038	
Denmark																												1.2	4,537	
Germany																												1.6	4,332	
France																												2.2	4,021	
Ireland																												2.2	3,794	
United Kingdom																														
Finland																														
Spain																														
Italy																														
Portugal																														
Malta																												1.1	2,261	
Israel																												n.d	2,186	
Turkey																												12.7	1,029	
Slovenia																												0.1	2,552	
Slovak Rep.																												2.2	2,060	
Czech Republic																												1.0	2,051	
Croatia																												3.6	1,514	
Poland																												9.0	1,476	
Hungary																												2.8	1,469	
Estonia																												8.3	1,226	
Serbia																												n.d	1,169	
Latvia																												12.3	1,093	
Romania																												10.7	811	
Russia																												n.d	998	

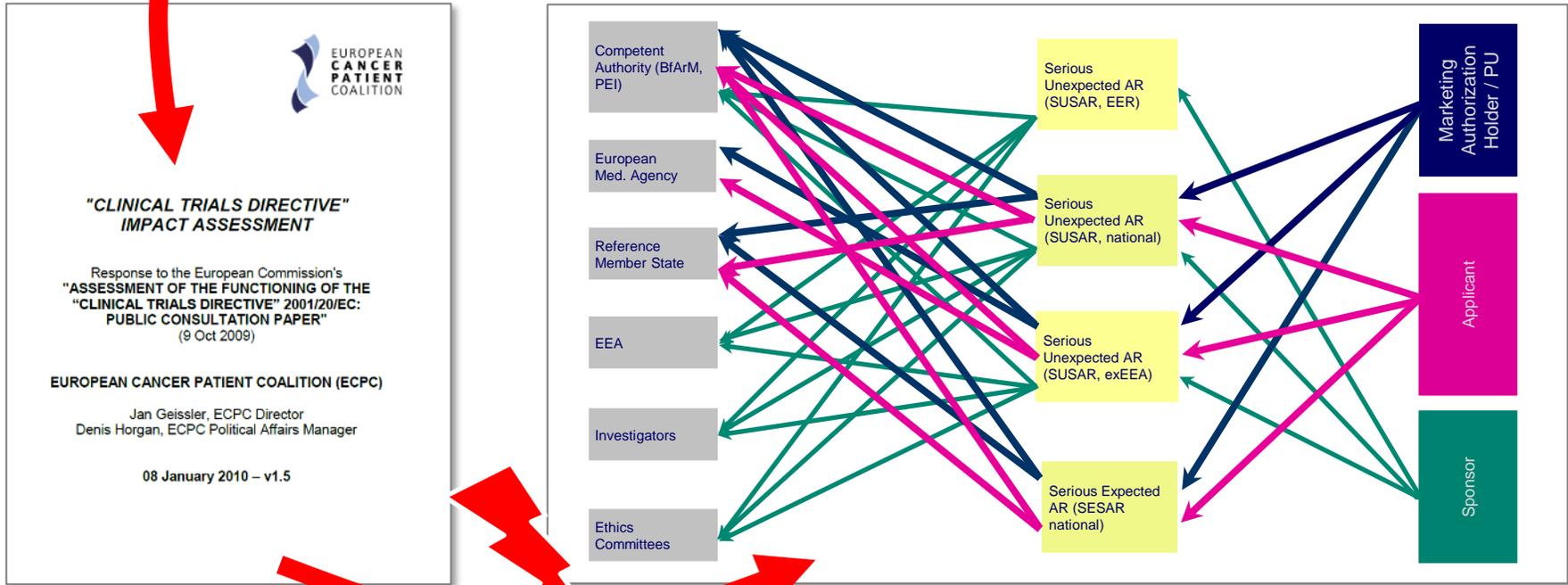
Data → Coaching → Strategy → Advocacy

Examples for evidence generation & use in patient advocacy

1. Disease mapping incl patient preferences
2. Inequalities
- 3. Health policy**

Deploying evidence in policy advocacy: Revision of the EU Clinical Trials Directive

„[...] The **German Hodgkin Study group** was required to provide 100.000 copied pages of documents to Ethics reviews and authorities for a single study with 280 participating clinics and 65 ethics committees. Furthermore, the **GMALL study group** had to provide 35 folders and 12.000 pages for a study conducted in 13 centres. [...]”



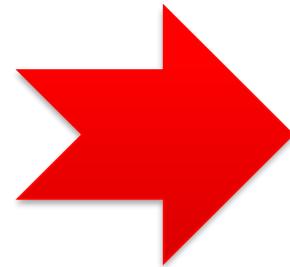
Sound methodology often counts

Scientific and policy work go hand in hand

**Scientific involvement
and policy work must
go together**

**If you could learn it, I
can learn it, too**

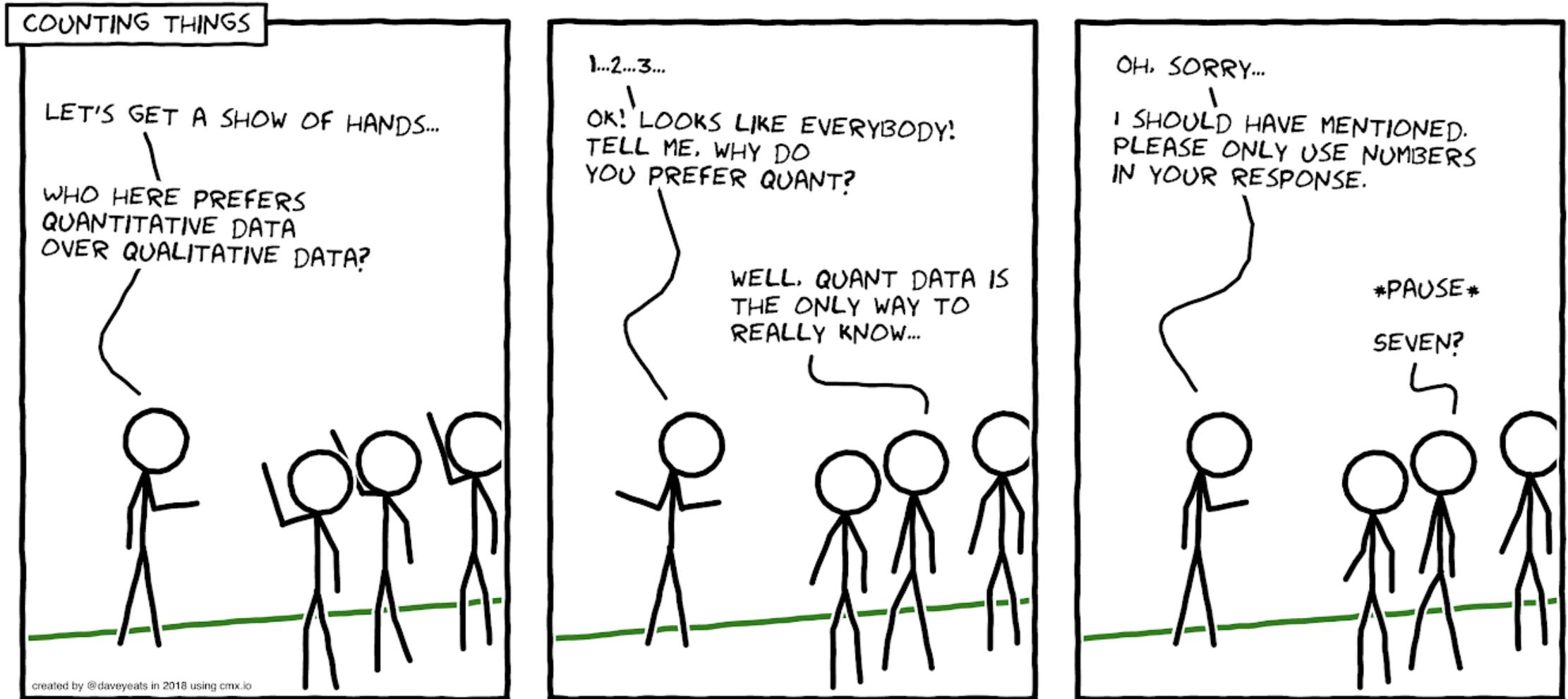
**Know what
you want**



**Know how to
achieve it**

What to use?

Different evidence is needed for different situations



STOP: Does your evidence show what you think it does?



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Is your methodology correct?

- Did you ask the **right question**?
- Did you ask the **right people**?
- Did you ask **enough people**?
- Is your conclusion **what the data actually shows**?
- Is it **statistically valid**?

Case study CML Advocates Network Adherence Survey

Where we should have been more strategic...

What we did well

- **Ran a pilot** to test our hypothesis and basic survey/research design (150 patients, 10 countries)
- **Used a professional market research agency** for survey tool, conduct, first data analysis
- **Used a validated instrument for adherence measurement** (license \$\$!) to generate trustable data
- **Used our global network** of patient organisations to recruit 2546 CML patients, supported by local patient organisations in recruitment, ethics approval, translations. Best recruiters ≠ largest country but active patient org!
- **Cooperated with 4 clinical networks** to remove „online bias“ with paper surveys distributed via hematology centers, with stamped return envelope

Where we should have been more strategic

- **No biostatistician involved:** Data sits on a hard disk, but no process in place to do analysis after the end of the agency contract
- **Pre-publication pitfall:** Discrepancy between interim analysis data presented at conferences, and “proper analysis” for journal article: Rejection by journal
- **No publication strategy,** no professional writer from the start, too much time lost for high-impact journal: Took us three years to get the data published
- **PRO license threat:** Legal threat as we were more successful than anticipated and didn't monitor licenses
- **Non-use of quantitative data:** We collected a lot, but we barely used it later on
- **No resources for follow-up, so what?:** Beyond videos, we did not do much on educational tools and presentations. Great impact in the scientific community, but did we have patient impact?

Publish or perish: Publications and Reporting

- We're advocates not academics, but for some stakeholders **'only published stuff counts'**
- Need to plan on this basis... not just decide to publish at the end of a project!
- WECAN & Envision soon to publish 4 training modules on patient involvement in publications



Steps

**Publications
& reporting**

Tasks

Generate
Presentations

Selection of media
and writing
publication

Identify
professional writer

Write publication



Questions?