

# We are living in the real world

REAL WORLD EVIDENCE in oncology

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# Why it matters

Controlled setting



Real world



Real patient populations differ from the patient populations in clinical trials- **this limits external validity**

# Real world data- it's a *negative* definition

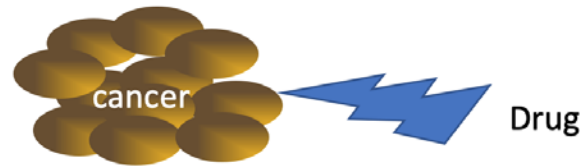
- Data collected in the real-world setting without intervention  
*or*
- Data collected outside a classic randomised controlled trial

# Why we as MPNE patient advocates were interested in Real World Evidence

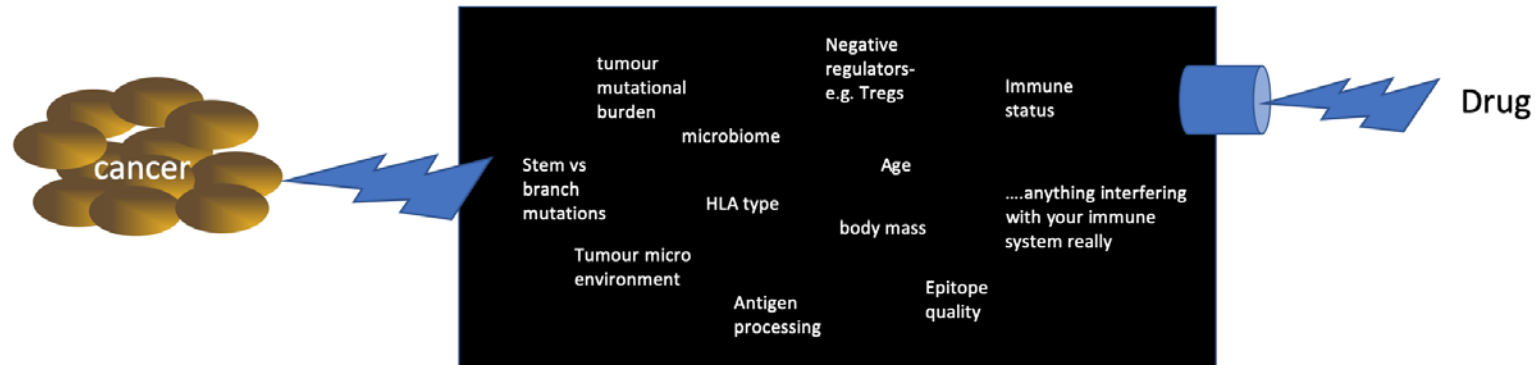
- **Patient interest taking precedence:** potential to avoid randomisation, RWE already used for regulatory approval by FDA (Pembro in MSI-H cancers, Palbociclib in male breast cancer) and in health technology assessments.
- **Lean learning about what is REALLY going on:** Systematic, fast and efficient learning- e.g. from Early Access Programs and registries
- **Dealing with complexity:** Potential to apply epidemiological thinking to increasingly sophisticated clinical and integrated data sets: the more personalised the medicine, the larger the datasets we need

# Immune therapy- treating through a big black box

**Classic cancer treatment- e.g. chemotherapy targets the cancer directly.**



**Immune therapy targets the cancer indirectly- via the immune system, a 'big black box'.**



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**MPNE**

# Further reading

- Use of RWE by HTA bodies: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5834594/>
- FDA approval of Palbociclib for male breast cancer - <https://www.pharmaceutical-technology.com/news/male-breast-cancer-ibrance-label-expansion/>
- Interesting reasoning on approval of immune therapy for MSI-high cancers in specific but also histology-agnostic approvals in general  
[https://www.ema.europa.eu/en/documents/presentation/presentation-session-5-approval-pembrolizumab-msi-h/dmmr-considerations-site-agnostic-development-drugs-oncology-steven-lemery\\_en.pdf](https://www.ema.europa.eu/en/documents/presentation/presentation-session-5-approval-pembrolizumab-msi-h/dmmr-considerations-site-agnostic-development-drugs-oncology-steven-lemery_en.pdf)
- IMI GetReal project: use of real-world data by HTA bodies- useful tools and educational resources: <https://www.imi-getreal.eu/>

# Thank you for listening

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