# RWE – Patient Viewpoint

## Issues and Solutions ?

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# Clinical research is not an end in itself

We are looking for how we can deliver the over-riding objectives:

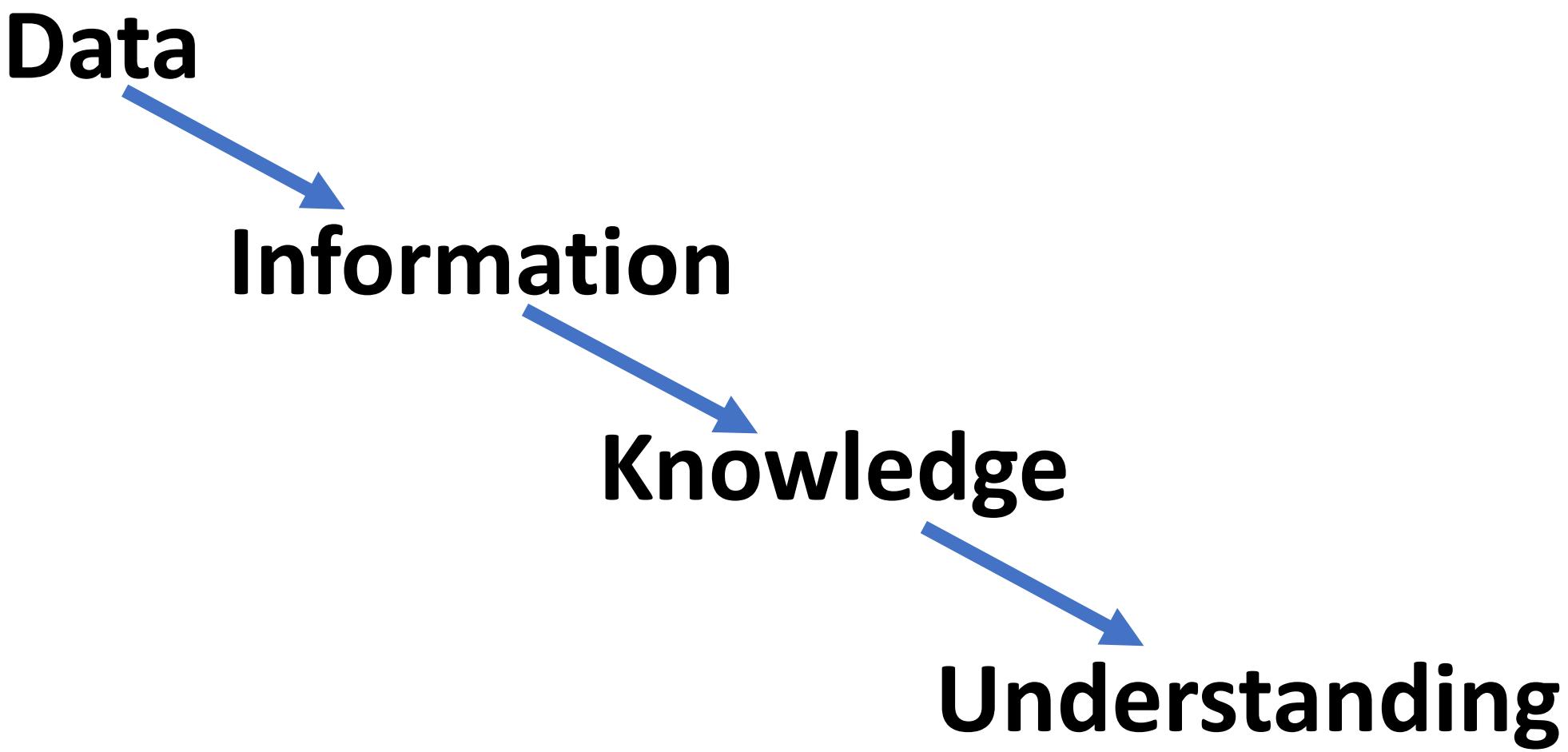
- Better treatments
- Better information to support patients
- Better, more meaningful, outcomes

Data

Output

Outcome

Understanding


Data
Information
Knowledge
Understanding

Short-term small Phase 2 studies – deliver poor quality data

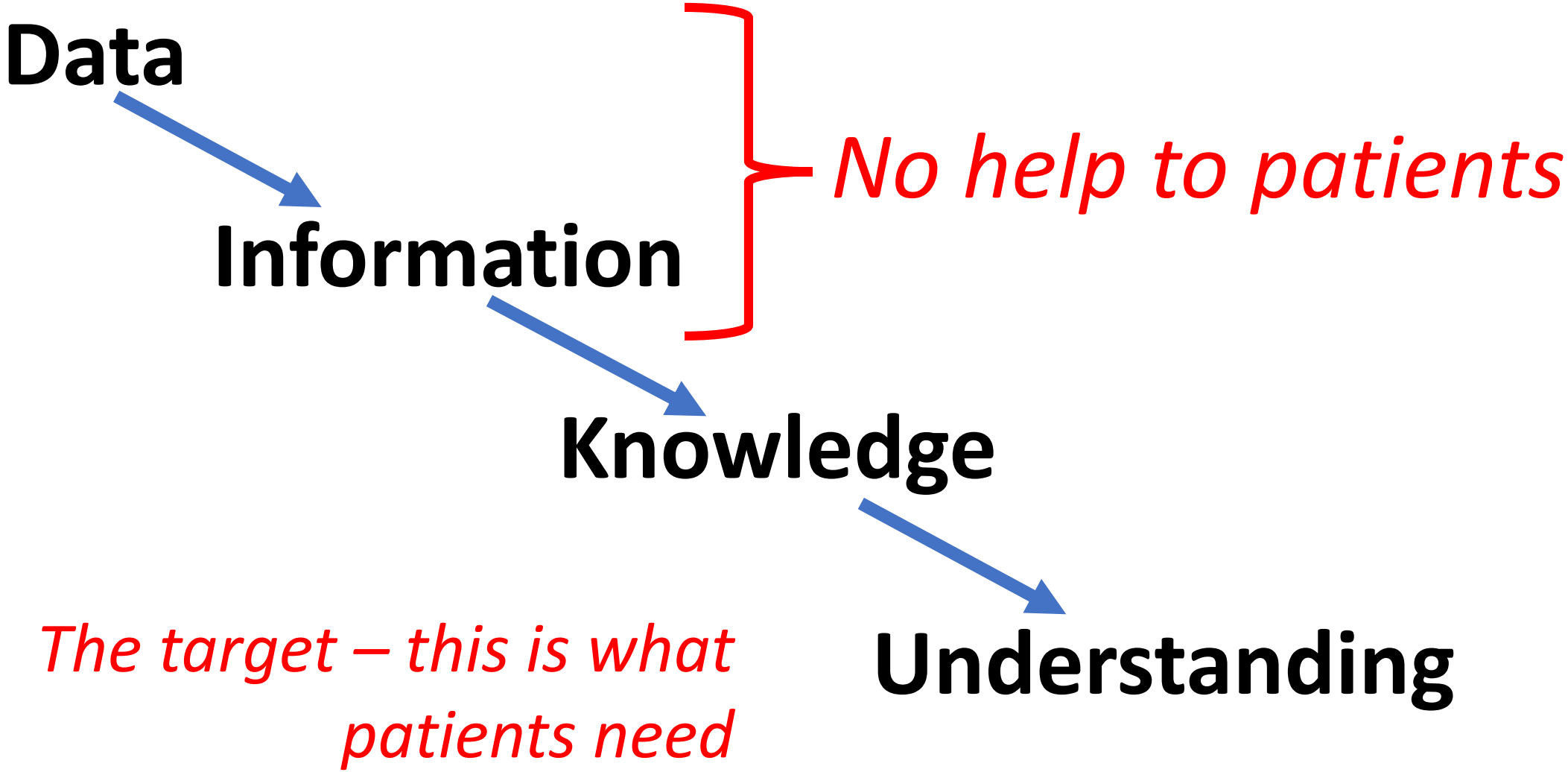
Surrogate endpoints deceive patients and support risk taking. Bias is also an issue – poor quality information

**This is drug-centred research**

Many patients want the knowledge which comes from rigorous science

Are we clear about the endpoints which really matter?  They unlock understanding

**We need patient-centred research**


Data
Information
Knowledge
Understanding
No help to patients
The target – this is what patients need

*"In this large, umbrella analysis of surrogate validation studies, we found most surrogates in oncology had low or modest correlation with OS."*[1]

*"Confirmatory trials for one-fifth (19/93) of cancer drug indications approved via the FDA's accelerated approval pathway demonstrated improvements in overall patient survival."* [2]

*Eighty-five indications for 59 cancer drugs were identified*
*38% received regular approval, and 62% were granted accelerated approval*
*55% of accelerated approvals were later converted to regular approval*
*Of these, 6 drugs showed overall survival benefit – 10%* *(Edited)* [3]

*"The current clinical research enterprise has insufficient safeguards against uninformative clinical trials that do not fulfill the contract between researchers and research participants."* [4]

*"… although the regulatory system respects the wishes of those willing to take on more risk, it fails patients preferring more information."* [5]

*"The existing literature evaluating patients' understanding, preferences, and values toward the end point of PFS was severely limited by the heterogeneity of methods, attribute selection, and descriptions used to define PFS to patients."* [6]

*A majority of patients 51/90 had 'no idea' or were 'unclear' what PFS meant …"* [7]

*"Vague, meaningless phrases used to describe the toxic effects of drugs are not helpful to patients or their physicians."* [8]

We need accelerated access to treat, and long term data to inform, patients – Real World Evidence

Real World Data must cover the endpoints which deliver understanding - Overall Survival and Quality of Life

Patients must be routinely involved in developing RWE research studies

## The solutions we are looking for:

- Overcoming the tyranny of small Phase 2 studies
- Change drug-centred to patient-centred
- Rigorous science with meaningful endpoints
- Accelerated access and provision of robust data
- Involve patients in developing studies
- Quality of life and PROs – longitudinal studies
- Do we need (some) new study methods?
- Funding/oversight/accountability

# References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