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How to increase the credibility of patient-reported outcomes in the drug development process?

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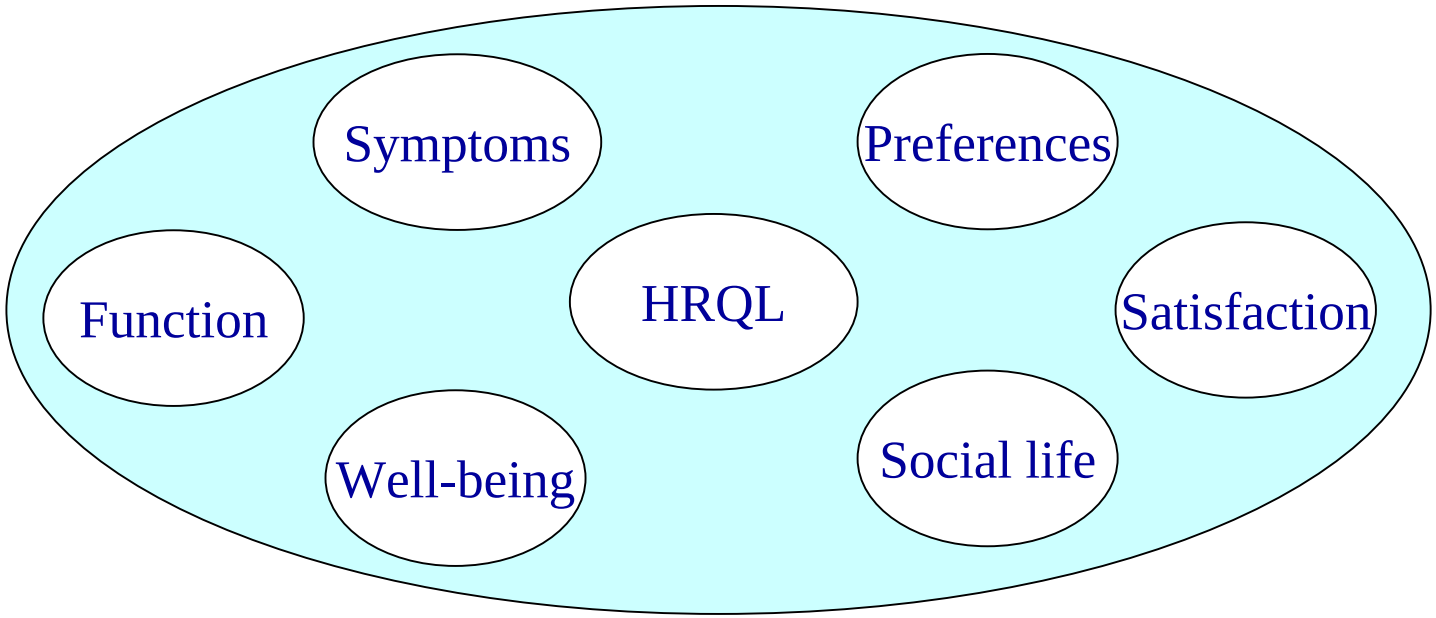
Credibility for PRO measures: few criteria

- Be based on solid concepts
- Have an explicit rationale
- Use tools that are adequate (selection) and have good measurement properties (validity)
- Collect data in appropriate experimental or quasi-experimental settings, using appropriate methods
- Meet usual scientific standards for data analysis and reporting
- Be interpretable per se, and relative to other medical parameters

What does that mean in practice?

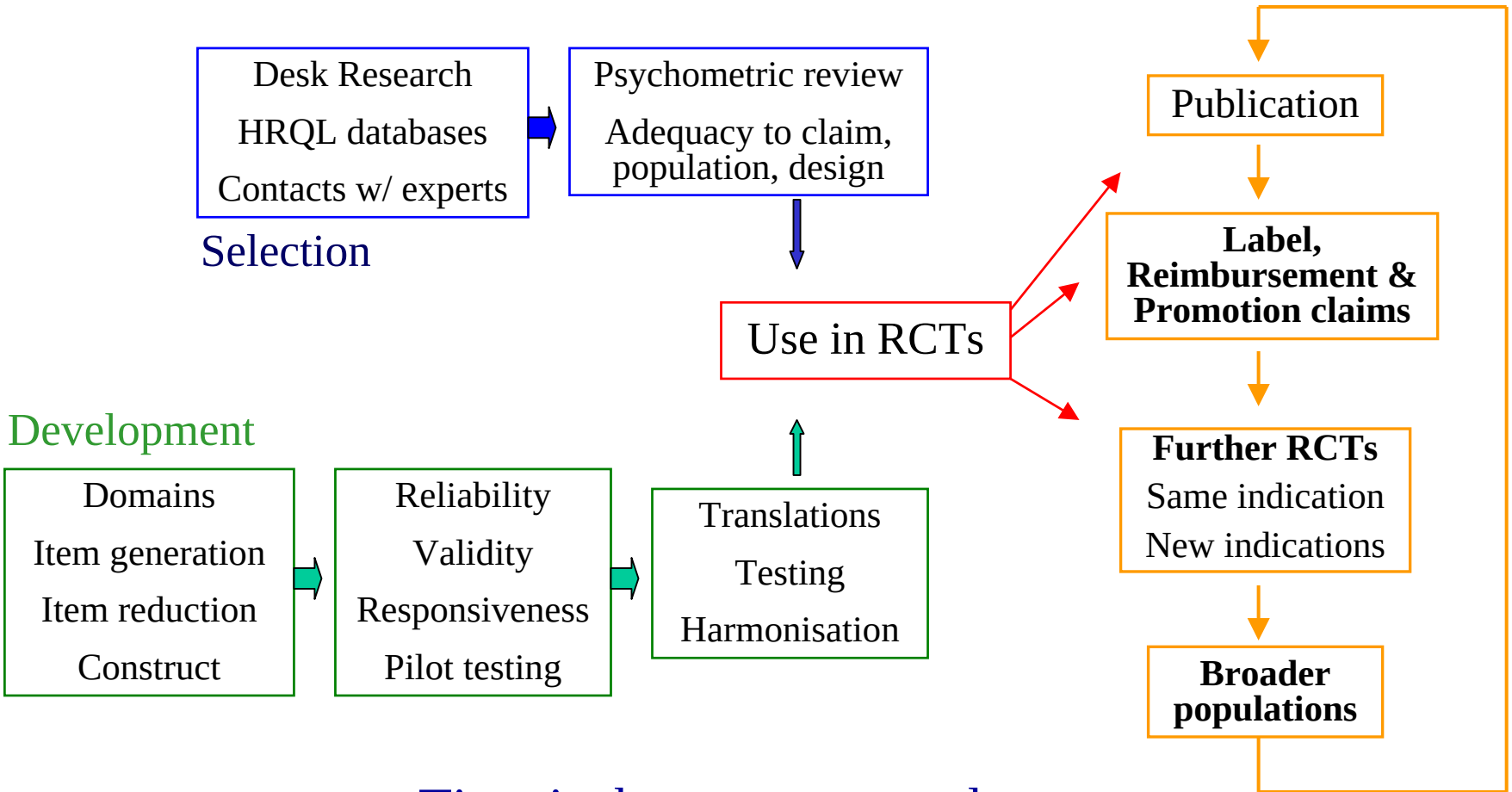
Patient-related outcomes (PRO): a multi-dimensional matrix



The evaluation of PRO is a valid and operational concept

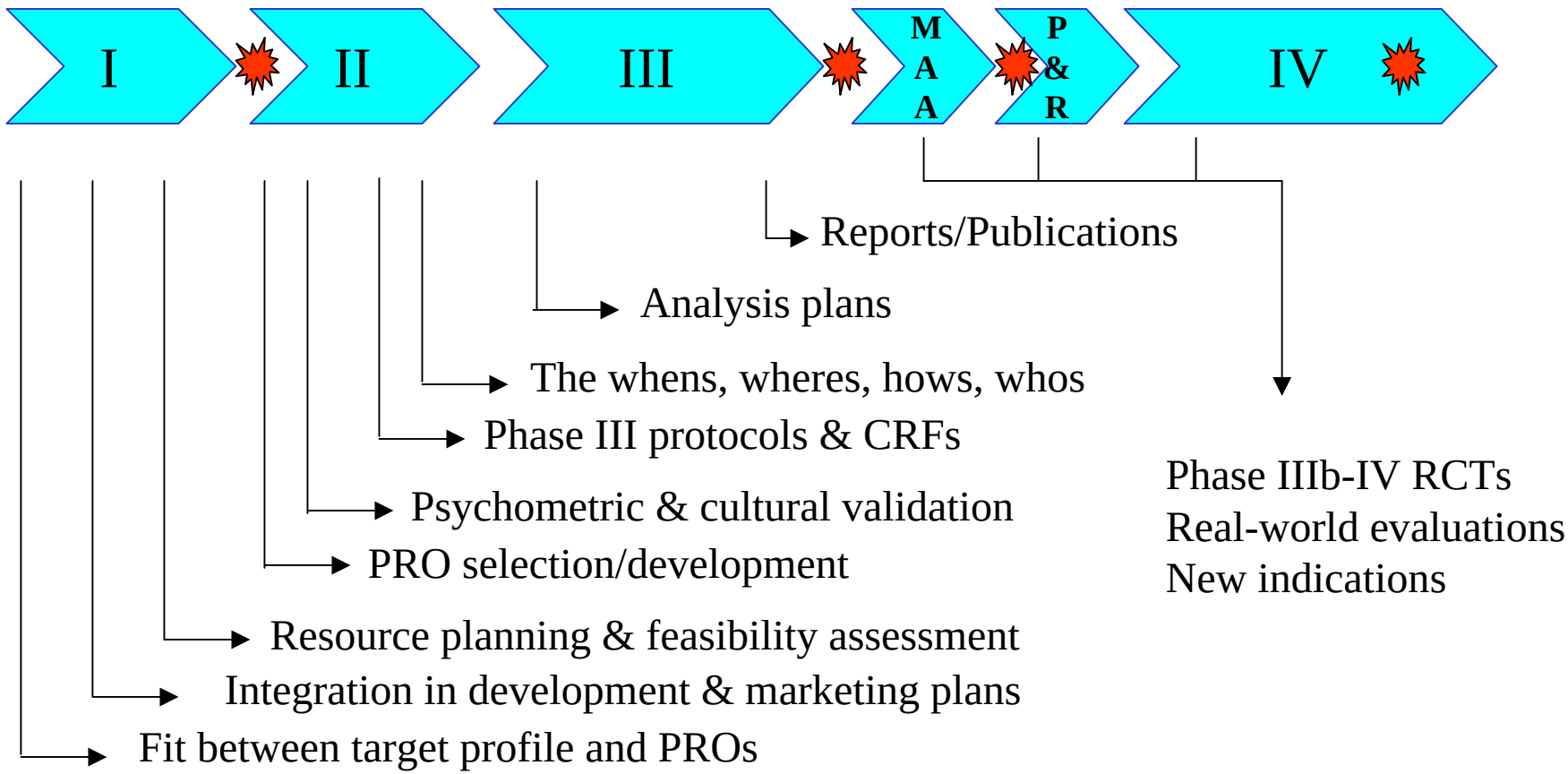
- allows a flexible integration of the patient's view into the evaluation
- is in tune with psychometric validation and clinical research processes
- is transparent in the analysis and interpretation of data

Processes for the selection, development and validation of PRO measures are well standardised



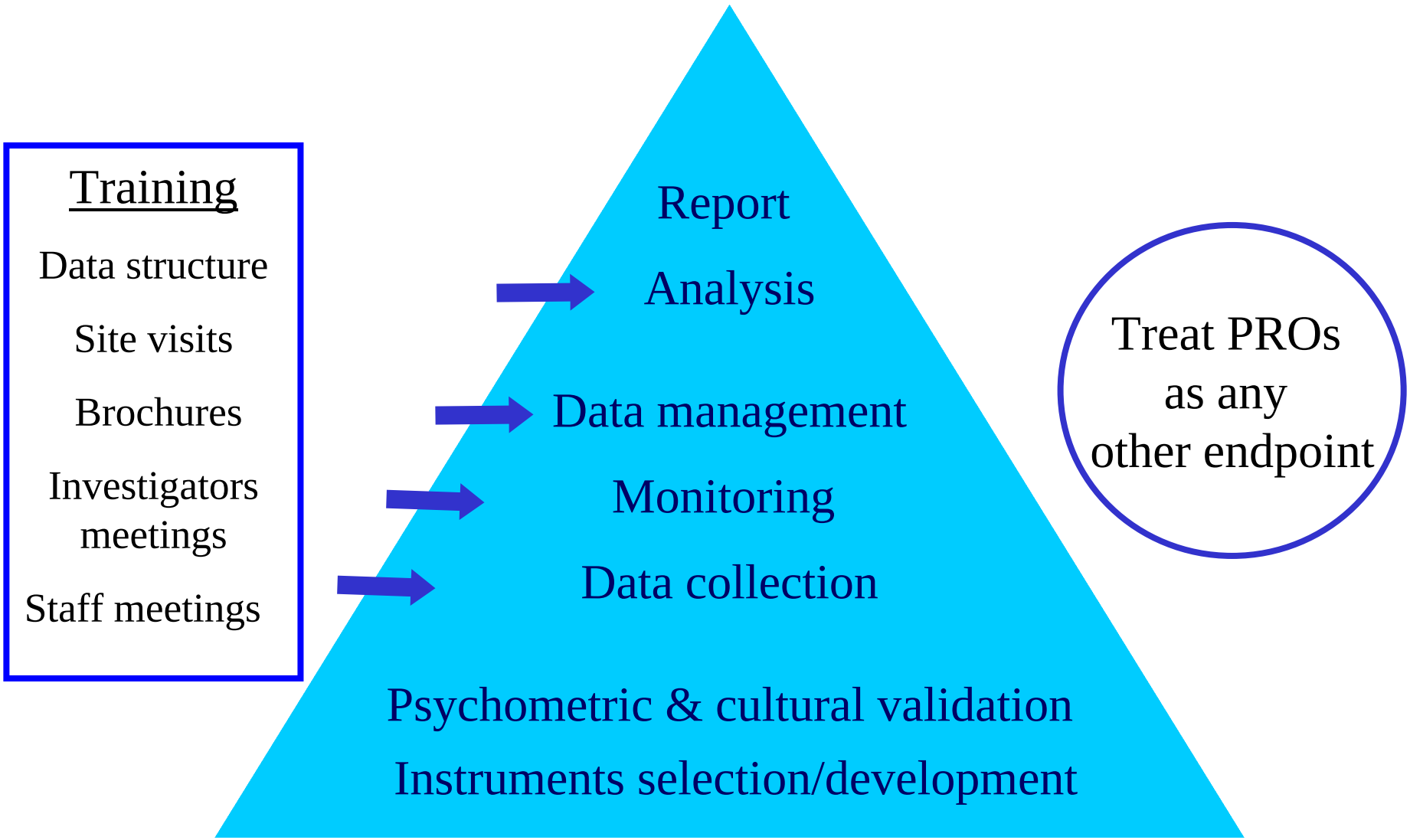
Time is the essence: need to plan backwards

The PRO agenda is front-loaded: early planning is key

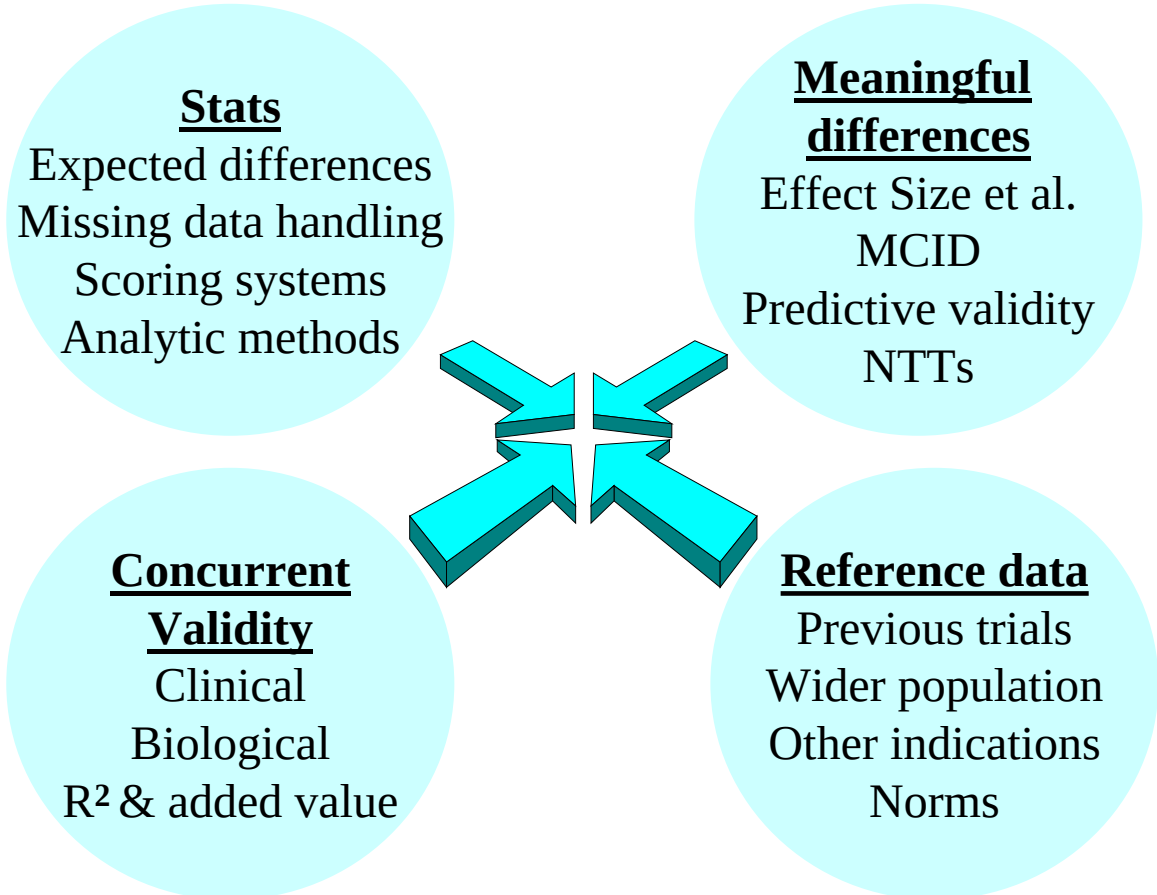


Goal: No compromise on quality, nor on development timelines

Quality of processes - Quality of data



Interpretation: the operational view



Cast fair balance between known instruments (easy interpretation) and new kids on the block (possible higher responsiveness)

Regulatory situation

- Despite wide differences across diseases, PRO claims for label and/or promotion are gaining momentum.
- For pricing & reimbursement claims, PRO measures have long been included in assessment of added value by pricing-reimbursement authorities.
- Support from multi-disciplinary groups (PRO Harmonisation, ERIQA, PhRMA-HOC) and Societies (ISPOR, IOQOL) has proven important in bringing stakeholders together. Should be pursued and extended.
- Creation of the SEALD Office at the FDA, and increased interest at EMEA and other regulatory agencies mark a turning point in the development of PRO measures.

Recent & ongoing regulatory developments
will boost the acceptability of PRO endpoints

4 Ts for PROs

- Thoughtfulness
  - Timing
    - Training
      - Transparency

Lead to a 5th one:

Trust