GENERATING DATA DIRECTLY ADDRESSING THE UNMET NEED OF THE PATIENT

PATIENT, THE NEXT KOL

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The aim → A satisfied patient ☺

The stakeholders

- The patient
- The physician
- The pharmaceutical industry
- The regulatory agencies

Their needs

Clinical studies

- …
- To achieve clinical improvement
- To show the benefits of their product
- To easily assess risk-benefit ratios

Adapted from McKenna SP. Measuring patient-reported outcomes: moving beyond misplaced common sense to hard science. *BMC Med*. 2011;9:86. Published 2011 Jul 14. doi:10.1186/1741-7015-9-86

The background: The patient coming to the center

- No longer a passive receiver of the results of clinical research

Clinical research

Patient-centricity

The challenge: Reflection of the patient perspective

- ➢ ***PRO and QoL*** *measures are increasingly becoming incorporated in clinical study designs, however* **they do not necessarily address issues that matter to the patient**

- ➢ *Common tools used to capture patient satisfaction may fail to address directly the patients needs and worries*
  - ▪ *Example: QoL measures cannot address adaptations in the lives of patients living with chronic diseases and thus may fail to identify improvements with otherwise effective interventions*

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Clinical research

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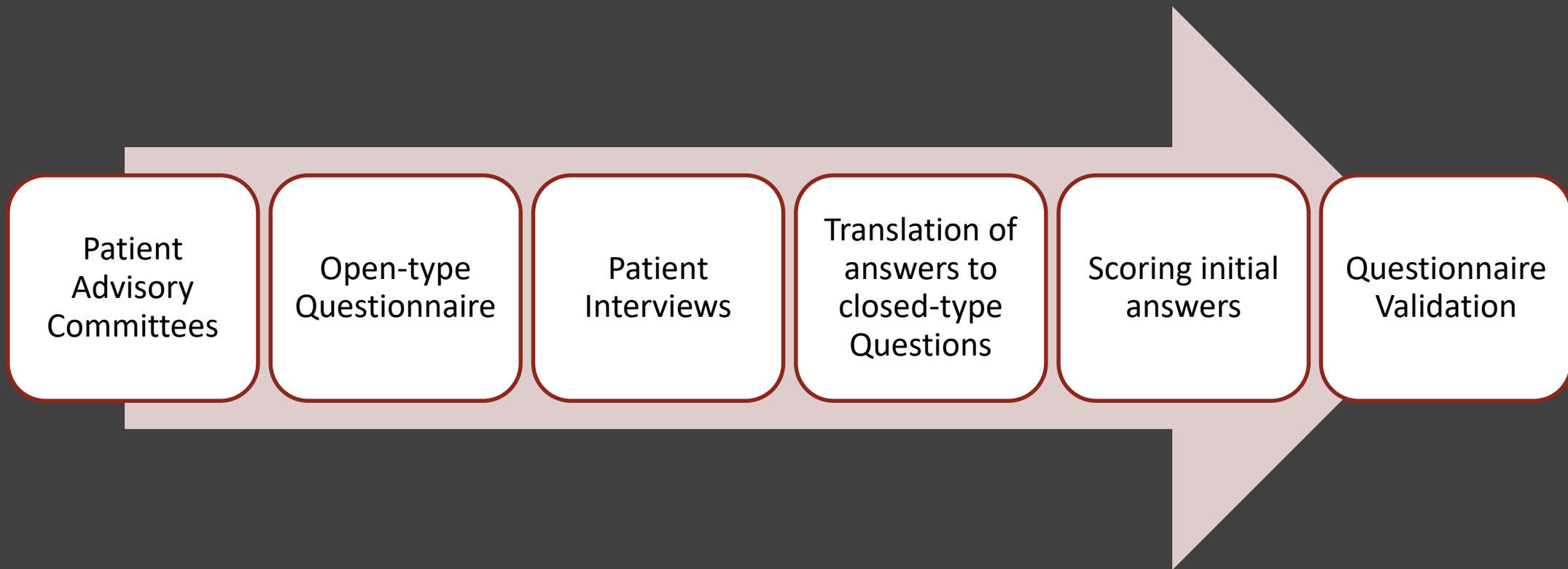
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The strategy: A tool developed by the patients themselves

Patient Advisory Committees → Open-type Questionnaire → Patient Interviews → Translation of answers to closed-type Questions → Scoring initial answers → Questionnaire Validation

To be used alongside standard PRO and QoL data in post-marketing studies

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Ask the patient. Step 1: Record the unguided patient opinion

What questions should you ask?

- Patient Advisory Committees
  - ✓ Engagement of patient organizations for the development of the pilot, disease-specific, open-type questionnaire

- How should you ask?
  - ✓ Written or oral (interviews)

Ask the patient. Step 2&3: Translation and Validation

How can you translate-interpret the answers?

- Patient Advisory Committees
  - ✓ Interpret the answers
  - ✓ Redevelop-Translate the questions into closed-type, scorable format

Make sure you are not lost in translation.

- Back translation-Validation
  - ✓ Score the remastered questionnaire based on the initial answers
  - ✓ Give the close-type questionnaires back to the initial patients to answer it
  - ✓ Compare the scores-Validate
  - ✓ Iteration as needed

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A truly patient-centric endpoint

Incorporation in RWE studies

- ✓ Comparison between different treatment options
- ✓ Identification of patient characteristics that predict satisfaction by a specific treatment

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Use in interventional studies?

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Use in interventional studies?
- ✓ Anchoring to clinical endpoints
- ✓ MCID identification

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Thank you!