FDA and EMEA Guidances: Which Impact on the Industry?

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- Do we need Patient-Reported Outcomes?
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PRO = The patient's perspective

Key stakeholders:

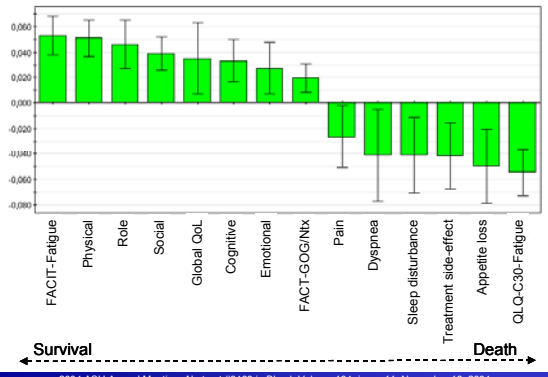
- Patients
- Clinicians
- Regulatory agencies
- Payers
- Industry

Decision-making criteria have changed:

- Need to demonstrate added therapeutic value
- Need to demonstrate value-for-money

- PRO's can serve as independent measures of:
  - Clinical benefit,
  - Tolerability,
  - And even mortality risk
- Complementing traditional physician assessments and test results

Prognostic value of PRO: Multiple Myeloma



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2. Do we need FDA and EMEA Guidances?

- PRO in 30% of FDA approved product labels
- The only type of endpoint used in the label for 23 products

Source: Richard J. Willke, Laurie B. Burke, Pennifer Erickson. Controlled Clinical Trials 25 (2004) 535–552

2. Do we need FDA and EMEA Guidances?

- Scientific quality standards: a MUST for all
- Goal of guidances: To provide explicit criteria for high quality standards

- An opportunity, rather than a threat
- Areas of concern remain
- Upside potential …

Areas of concern:

- Consistency between EMEA and FDA guidances
- EMEA reflection paper:
  - Insufficient emphasis on interpretation issues
  - Potentially counterproductive validation process requirements

Interpretation Issues:

- *"HRQL improvement" as a claim implies that the most important and clinically relevant health-related concepts (domains) that impact patient's quality of life are known and measured."*
- ERIQA proposes that a section on the interpretation of HRQL outcomes be added to the Reflection Paper.

Validation process in clinical trials:

"As a general rule, the same study should not be used to validate the HRQL instrument and for the testing of the HRQL change.

In all cases, the validation of HRQL instrument should have been completed before their use in therapeutic confirmatory trials."

3. Which impact on the Industry?

Validation process in clinical trials:

- ~~*"As a general rule, the same study should not be used to validate the HRQL instrument and for the testing of the HRQL change. In all cases, the validation of HRQL instrument should have been completed before their use in therapeutic confirmatory trials."*~~

3. Which impact on the Industry?

Impact on the Industry:

- If the science is good, then the data should be considered in the review process.

*"If the two-step procedure and all methodological prerequisites are fulfilled, an additional claim in the SmPC with the respect to HRQL (i.e. in section 5.1) ~~may~~ **will** be considered depending on the strength of the evidence …."*

Conclusions

- YES, we need Patient-Reported Outcomes
- YES, we need FDA and EMEA guidances
- YES, a potential opportunity for the Industry