ROLE OF HRQL EVALUATION IN THE DRUG REGULATORY PROCESS : RESULTS FROM A EUROPEAN REGULATORY SURVEY

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BACKGROUND

The ERIQA Group was created with the following mission statement : *"establishing principles and practices for the integration of Health-related Quality of Life (HRQL) outcomes in the drug regulatory process".*

Objectives of the ERIQA Project are:

- to convince European Regulators that HRQL is a relevant key outcome, i.e. a credible criterion of evaluation of medicines;
- to have them confident in the quality of HRQL outcomes;
- to provide European regulatory authorities with guidance on:
  - how to assess the quality of HRQL studies in clinical trials,
  - how to evaluate the validity of HRQL claims

As part of its activities of year 1999, the ERIQA Group surveyed European Regulatory Authorities : EMEA and country-specific agencies.

OBJECTIVES

☛ To clarify in more details the role of HRQL in the European regulatory process of pharmaceutical products : 1. Registration, 2. Reimbursement, 3. Promotion.

☛ To evaluate the potential needs of regulators with regards to HRQL issues

METHODS

Three questionnaires were developed, one for each regulatory area (Q1, Q2 & Q3), and sent to 60 representatives of regulatory agencies in the 15 European countries represented at the EMEA, among them the 30 CPMP members. They covered the first 6 months of year 1999. Questionnaires were divided in three informative parts : Generic /ID; Specific; Perspectives for the future.

Part II of the questionnaire :

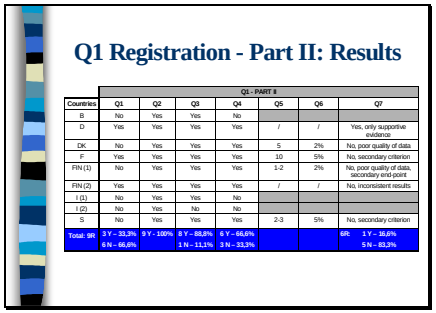
1. Do you follow any criteria to review the HRQL evidence included in registration files ?
2. Would you welcome guidance in form of recommendations or a checklist to review HRQL evidence ?
3. Would you be interested in training in HRQL evaluation ?
4. Are you aware of any registration files submitted in the first half of 1999 where HRQL was included as an evaluation criterion ? (if no, go to Part III)
5. Could you estimate how many files ?
6. What is the estimated percentage that these "HRQL" files represent with the respect to the total number of registration files submitted in the first half of 1999 ?
7. Among the number of files cited in Q5, are there any files where HRQL claims have been included in the approved product labeling ?

Part III of the questionnaire

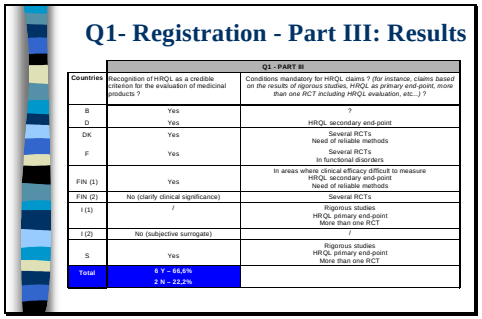
- In the future, do you think that HRQL will be recognized as a credible criterion for the evaluation of medicinal products ?
- In your opinion, which conditions would be mandatory for HRQL claims to be included in the product labeling ?

RESULTS

Results from questionnaires concerning Registration issues are presented :
Information was retrieved from 7 countries.
9 / 60 questionnaires were sent back.



Countries	Q1 - PART II Q1	Q2	Q3	Q4	Q5	Q6	Q7
B	No	Yes	Yes	No			
D	Yes	Yes	Yes	Yes	/	/	Yes, only supportive evidence
DK	No	Yes	Yes	Yes	5	2%	No, poor quality of data
F	Yes	Yes	Yes	Yes	10	5%	No, secondary criterion
FIN (1)	No	Yes	Yes	Yes	1-2	2%	No, poor quality of data, secondary end-point
FIN (2)	Yes	Yes	Yes	Yes	/	/	No, inconsistent results
I (1)	No	Yes	Yes	No			
I (2)	No	Yes	No	No			
S	No	Yes	Yes	Yes	2-3	5%	No, secondary criterion
Total: 9R:	5 Y – 33,3% 4 N – 66,6%	9 Y - 100%	8 Y – 88,8% 1 N – 11,1%	6 Y – 66,6% 3 N – 33,3%			6R: 1 Y – 16,6% 5 N – 83,3%



Countries	Q1 - PART III Recognition of HRQL as a credible criterion for the evaluation of medicinal products ?	Conditions mandatory for HRQL claims ? (for instance, claims based on the results of rigorous studies, HRQL as primary end-point, more than one RCT including HRQL evaluation, etc...) ?
B	Yes	?
D	Yes	HRQL secondary end-point
DK	Yes	Several RCTs Need of reliable methods
F	Yes	Several RCTs In functional disorders
FIN (1)	Yes	In areas where clinical efficacy difficult to measure HRQL secondary end-point Need of reliable methods
FIN (2)	No (clarify clinical significance)	Several RCTs
I (1)	/	Rigorous studies HRQL primary end-point More than one RCT
I (2)	No (subjective surrogate)	/
S	Yes	Rigorous studies HRQL primary end-point More than one RCT
Total	6 Y – 66,6% 2 N – 22,2%	

- Most of the respondents deplored the poor quality of HRQL data provided, leading them to discard HRQL evaluation in the regulatory process.
- Nevertheless, 100% expressed their need for guidance in HRQL issues, and 90% their need for training.

CONCLUSION

HRQL evaluation currently plays no determining role in the European drug approval. More efforts should be developed to improve the quality of HRQL studies, and therefore convince regulators that HRQL is a credible criterion of evaluation of medicines.