OMERACT II Conference - Outcome Measures in Rheumatoid Arthritis Clinical Trials: Conclusion

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TOXICITY

(1) The discussions confirmed that more work on the assessment of toxicity is urgently needed to bring it to the same level of validity and reliability as measurement of benefit.
(2) The assessment of toxicity has not received the same attention as the assessment of benefit. Both need similar levels of accuracy, because both are needed for patients and clinicians to make informed decisions.
(3) There have been important initiatives in developing standardized forms; however, these are not used uniformly. Investigators setting up and running both trials and post-marketing medical surveillance studies should be encouraged to use these.
(4) There was considerable interest in the suggestion that drug approval agencies might be prepared to release the data on toxicity (and presumably efficacy) within the submission dossiers for approval into the public domain, for those drugs that are approved.
(5) At the end of the conference, participants were asked to vote on the following question:

> *Would you be prepared to include a toxicity index or supplementary questionnaire in addition to the usual case report form in your next rheumatology drug trial?*

Response: 80% of the respondents indicated they would be prepared to include a toxicity index or supplementary questionnaire in their next drug trial; 20% indicated they would not.

Recommendation. A working group needs to be established that will systematically review the advantages and disadvantages of the available instruments and develop an assessment instrument that can be tested in upcoming studies.

GENERIC QUALITY OF LIFE AND UTILITY/ PATIENT PREFERENCE ASSESSMENTS

(1) There is remarkably little experience with these endpoints in rheumatology clinical trials or phase IV studies. Rheumatology is well behind some other disciplines in collecting data to ensure that the benefits of intervention are accurately reflected by these questionnaires, which is especially important since resource allocation within health and health care is increasingly likely to be based upon such data.
(2) A number of questions were raised around the scaling used in these questionnaires. The following questions were asked at the end of the conference:

> *In your next rheumatology drug trial, in addition to the usual endpoints, would you be prepared to include one of the generic/utility questionnaires?*

Response: 87.5% of the respondents voted yes and 12.5% voted no.

> *In your next rheumatology drug trial, in addition to the usual endpoints, would you be prepared to include a rating scale/feeling thermometer?*

Response: 71.9% of the respondents voted yes and 28.1% voted no.

> *In your next rheumatology drug trial, in addition to the usual endpoints, would you be prepared to include a standard gamble?*

Response: 17.2% of the respondents voted yes and 82.8% voted no.

> *In your next rheumatology drug trial, in addition to the usual endpoints, would you be prepared to include a time tradeoff?*

Response: 28.1% of the respondents voted yes and 71.9% voted no.

Recommendation: It was recommended that a working group be set up to develop a program for collecting the appropriate data on responsiveness to change and other aspects of validity, so those designing studies can be asked to include the appropriate questionnaire and the relevant data can be collected.

HEALTH ECONOMIC EVALUATION

(1) There was major interest in incorporating economic eval-

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uation components into future clinical trials in rheumatology.
(2) For the cost effectiveness studies, it is important that there be standardization of the endpoints and their measurement within each of the rheumatologic conditions to be studied. Conditions and specific issues that arose in the workshop are reviewed by condition in the proceedings.
(3) This question was asked at the end of the conference:

In your next rheumatology drug trial, would you be prepared to include a costing component so that a concurrent cost effectiveness substudy can be included?

Response: 87.5% of the respondents voted yes and 12.5% voted no.

Recommendation: A working group should be formed to develop a template on how these can be added to clinical trial design in a way that is methodologically rigorous while minimizing the expense and additional data collection requirements.

THE NEXT CONFERENCE - OMERACT III

Two summary questions were asked at the end of the conference:

How often should future OMERACT conferences be organized?

Response: Over 60% of the respondents voted for every 2 years.

What is the most important direction for the next OMERACT conference?

Response:(1) 18.6% of the respondents replied: Revalidate RA core set of endpoints and improvement criteria based on prospective data.
(2) 31.4% of the respondents replied: Design core endpoint sets for other musculoskeletal diseases.
(3) 50.0% of the respondents replied: Evaluate research on efficacy/cost tradeoffs on the basis of the research agenda proposed here.