PED/PROs IN THE DESIGN OF CLINICAL TRIALS

ESC
Cardiovascular Round Table

Group 2. How to overcome the obstacles and limitations in using PED/PROs as measures in CVD Clinical Trials.

1. Use PRO and PED in all of your clinical trials (academia and industry).
2. PRO / PED are very valuable as secondary outcomes.
3. We are behind. So far only one CV PRO proposal for qualification and one for submission (in PAH)
4. Early phase: PRO is feasible as primary outcome.
5. Have a PRO specialist on your trial committee.
6. EMA invites you to get advice from regulators early.
7. Predefine your PRO / PED analysis in an analysis plan prior to unblinding / data base lock.
8. Combining PRO / PED with biomarkers will improve their interpretation
9. Standardised environments are important for validated PROs
10. In some diseases (e.g. paroxysmal AF, angina), symptom severity changes over short periods of time. In other diseases (e.g. chronic heart failure, aortic stenosis), the effects of a cardiovascular condition are more stable. Repeated PRO measurements can capture short-term effects.
11. Multimorbidity is on the rise. This impacts PROs, e.g. masking of a disease-specific effect by effects due to other diseases. Repeated measurements combined with physiological measurements can potentially overcome this.
12. Non-validated PROs and settings (apps) can be validated in your trial. Placebo control will help.
13. Can we share the effort of validation of PROs.
14. Short questionnaires and intelligent, digital questionnaires will increase return rates.
15. Involving patients early may further increase return rates.
16. Rules on how to count missing values are important. This remains an important area.