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PROMs: Role in Clinical Research and Contributions to PAH Patient Perspectives

Announcer:
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Dr. Ford:
This is CME on ReachMD, and I'm Dr. Jimmy Ford. In this series, we've discussed the use of patient-reported outcome measures, or PROMs, in the routine clinical care of PAH, as well as how they would be quite beneficial to incorporate routinely to better understand the patient's situation, quality of life, and goals of care. Now let's talk about the use of PROMs in PAH clinical research.

The use of PROMs as clinical trial endpoints is well established in PAH, with more generic PROMs being used in earlier studies and more disease-specific—or PAH-specific—PROMs being utilized in more recent PAH clinical trials. PROMs are unique in clinical trials in capturing patients' perspectives of symptoms and their impact, health-related quality of life, and the effect of treatments. They have not been utilized as primary endpoints in PAH trials to date. Regulatory agencies have advocated for the inclusion of patient quality-of-life assessments as new therapeutics are being studied for ultimate potential approval. PROMs are integral in achieving these goals as well.

Pulmonary hypertension–specific PROMs commonly used in trials to date include CAMPHOR, EmPHasis-10, PAH-SYMPACT, and the Living With PAH tool. They differ from each other, as you may have learned already, in the domains covered, the statements which participants score, the recall period being assessed, the number of questions being asked, and the type of scale utilized that patients score symptoms and assessments on. These all influence which PROMs are chosen for a particular trial.

The choice of the optimal PROM for a trial needs to be based upon patient selection; international guidance on PROM selection for clinical trials in general; the aim of the specific trial and the therapy being investigated; the expected properties and side effects of the planned intervention; the PROM's acceptance by regulatory agencies; and other trial endpoints that are being studied in conjunction. PROMs must also attempt to achieve cultural equivalence of measures across different linguistic and cultural groups that may be recruited into the trial, as best as possible.

PROM results need to be presented in a clinically relevant format to understand the implications of the findings. For example, patients not taking part in a trial may use the results to judge the likely benefits of a treatment in their own case once the drug is ultimately approved and comes to market.

Beyond just the inclusion of pulmonary hypertension–specific PROMs in PAH trials, the 7th World Symposium on Pulmonary Hypertension patient perspectives task force also advocated for the inclusion of patients in clinical trial design and reporting of trial results to the lay public and other patients. They also advocated for patient input in PROM selection for clinical trials.

In addition, clinical trial steering committees should consider including an expert on PROM selection, analysis, interpretation, and communication to improve the utility and relevance of the PROM used in the trial and the results from the trial, as regards to the PROM

findings.

The key takeaway here is that patients should be involved in PAH clinical trials, particularly as it relates to PROMs and the inclusion of relevant endpoints, much in the same manner as the shared decision-making approach in routine clinical care.

Thank you for tuning in and thank you for your attention. I hope you found this information useful in your practice.

Announcer:
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