Patient-reported Outcomes (PROs): An Approach to Evaluate Treatment Efficacy of Chinese Medicine or Integrative Medicine

ZHAO Li (赵利), and CHAN Kelvin (陈金泉)

Chinese medicine (CM) has been an experience based practice over the past three thousand years. The safety and efficacy of CM has been practised through experience, rather than nowadays’ modern scientific measurements. In this way, the development of CM has not been keeping pace with the advance in science and technology and the progress in orthodox medicine. In order to meet the requirements of modern science and technology, an evidence based approach must be adopted. Reassurance is needed to convince people for safety and efficacy of CM and give confidence to the evidence based demands — “Tradition is confirmed by Modern Science” (1).

Long historical use and practices of CM based on experience passed from generation to generation has demonstrated the safety and efficacy of treatment in CM, but a major challenge that the CM researchers and physicians have to confront is how to evaluate treatment efficacy on CM or integrative medicine (IM) using modern scientific approaches. This paper proposes an approach in establishing an evaluation system of the clinical measurements based on patient-reported outcomes (PROs), in conjunctions, if applicable, with other objective clinical laboratory outcomes during the whole course of treatment on CM alone or integrated orthodox medicine and CM.

Patient-reported outcomes refer to patients’ self-reported outcomes in the context of health care, including any reports generated directly from the person or persons affected about their living environment, health condition(s) and treatment. PROs include not only health status and quality of life but also reports on satisfaction with treatment and care, adherence to prescribed regimens when directly related to end-result, and any other treatment or outcome evaluation obtained directly from patients through interviews, self-completed questionnaires, diaries or other data collection tools such as hand-held devices and web-based forms (2). Health-related quality of life is one of several types of PROs data that may be collected in the context of a clinical trial. Other PROs include, but are not limited to, symptoms, patient satisfaction with treatment, functional status, psychological well-being, and treatment adherence (3).

In fact, the patient’s reports of symptoms, adverse reactions, and other health-related data gathered during treatment history taking are important entries in the multifactorial database that is the foundation for medical diagnosis and treatment. Especially in CM, the CM practitioner relies almost entirely on patient reports in diagnosis and evaluation. Questioning, one of the four methods of diagnosis, is to collect the patient-reported information including objective symptoms, such as diet, sleep, pain and subjective feeling and emotion. Such information not only provides evidence of diagno-

Research and Development Division, School of Chinese Medicine, Hong Kong Baptist University, Hong Kong, China Correspondence to: Prof. CHAN Kelvin, Tel: 852—34115308, Fax: 852—34115317 E-mail: prochan@hkbu.edu.hk
sis but also gives indices of evaluating treatment efficacy for subsequent treatment regime and follow-up within the practice of CM. It is understood that to cure an illness is to eliminate all signs and symptoms, providing efficacy means to have less symptoms and signs, and inefficacy refers to no improvement of symptoms and signs. Such follow-up by the patients during CM treatment will emphasize the importance of patient’s self-reporting. But this is not PROs because this depends on the subjective conclusion of CM physicians. This belongs to clinician-reported outcomes.

On the other hand, there is no perfect system of evaluating treatment efficacy in CM or IM although some efforts have been made to explain the efficacy and science of treatment in research and practice of CM in China for a long time. For example, guidelines for clinical research on new Chinese medicinal products were published by the Ministry of Health in China\(^4\). Every symptom is divided into five levels as the criteria in the guidelines and assessed by CM physician. We consider that this also belongs to clinician-reported outcomes. Laboratory indices are added to act as criteria of evaluating the efficacy e.g. in “Pi Qi Deficiency syndrome” and “Blood Stasis syndrome”. Sometimes, the current indices and criteria in Western (orthodox) medicine are directly used as assessment in the research and clinical practice of CM. These should be considered as physiological outcomes. These outcomes are useful to evaluate treatment efficacy in CM or IM, but it is debatable if these outcomes are appropriate inclusion as objective treatment outcomes for CM treatment as they are not really characteristic of practising CM. From the viewpoint of the patient, it may not matter which kind of treatment (CM, orthodox medicine or IM) is selected provided the treatment reflects efficacy. CM focuses on patient’s self-feeling. Thus, PROs may be an approach to evaluate treatment efficacy of CM or IM.

Presently, some CM practitioners have adopted the method of questionnaires into clinical research for assessment of efficacy in CM treatment. For example, to measure and assess the quality of life of patients with pulmonary tuberculosis, the quality of life of 228 patients with pulmonary tuberculosis and 228 healthy subjects as control were assessed by administering SF-36 (The Short Form-36 Health Survey Questionnaire), QLI (Spitzer’s Quality of Life Index) and KPS (Karnofsky Performance Status) scales; and single and multiple stepwise regression analysis was used to evaluate factors affecting quality of life. The results demonstrated that the quality of life of patients with pulmonary tuberculosis declined indicating the SF-36 scale was suitable for measurement of patients with pulmonary tuberculosis\(^5\). With measuring scales of fatigue, anxiety, depression and life event to observe the improvement by Xiaopi Yishen oral liquid（消疲怡神口服液, XPYS）on symptoms of 43 patients with chronic fatigue syndrome (CFS), the results revealed that patients compared with normal people were in obvious depressive and anxious state, the accumulative grades of the life event measuring scale was markedly higher as well. XPYS could improve the depressive and anxious state of CFS patients markedly\(^6\). Using the SF-36 in 421 postmenopausal women in community to survey and evaluate the quality of life in women with postmenopausal osteoporosis (PMOP), the result showed that the quality of life of the PMOP women was lower than that of the non-PMOP women. The authors consider that further study of quality of life may contribute to the diagnosis and evaluation of the treatment efficacy in PMOP\(^7\). Liu FB and co-workers\(^8\) set up clinically used version of the Syndrome Differentiation scale of the Pi-Wei (Spleen-stomach) diseases derived from World Health Organization Quality of Life Assessment Instrument (WHOQOL-100) and the characteristics of gastrointestinal diseases and CM.