PIONEER's update and integration of a localised prostate cancer core outcome set for effectiveness trials and a standard set for clinical practice.

Authors: Katharina Beyer[1]; Steven Maclennan[2]; Michael Lardas[3]; Lisa Moris[4]; Muhammad Imran Omar[2]; Stephen Flaherty[5]; Tiago Antunes-Lopes[6]; Luis Pacheco-Figueiredo [6,7]; Serenella Monagas[8]; Francesco Esperto[9]; Alberto Briganti [10]; Mieke Van Hemelrijck [1*]

Affiliations:
[1]King's College London, Faculty of Life Sciences and Medicine, Translational and Oncology Research (TOUR), London, UK; [2]Academic Urology Unit, Health Services Research Unit, University of Aberdeen, Aberdeen, UK; [3]Department of Urology, Leto Hospital, Athens, Greece; [4]Department of Urology, University Hospitals Leuven, Leuven, Belgium; [5]International Consortium for Health Outcomes Measurement, Cambridge, USA; [6]Department of Urology, Hospital de São João, Porto, Portugal; [7]Life and Health Sciences Research Institute (ICVS), School of Medicine, Minho University, Portugal; [8]Urology Department, Leon University Hospital, Spain; [9]Department of Urology, Humanitas Gavazzeni, Bergamo, Italy; [10]Department of Urology, University Vita e Salute-San Raffaele, Milan, Italy.

Introduction & objectives: Harmonising assessment of men with prostate cancer (PCa) is key for both clinical research and practice. A core outcome set (COS) for localised PCa effectiveness trials, developed using, the Core Outcome Measures in Effectiveness Trials (COMET) methodology, and a standard set of outcomes for clinical audit developed by the International Consortium for Health Outcomes Measurement's (ICHOM) have been published. Although these two COS broadly overlap, their purpose and methodology differed. We updated and integrated both approaches as part of the PIONEER project, a consortium using Big Data for answering key questions in patients with PCa, funded through the IMI2 Joint Undertaking under grant agreement No. 777492-. By collaborating with the original development groups, we aimed at updating the systematic reviews to ascertain whether any new outcomes have been suggested and to recategorize the outcomes to a common taxonomy.

Material and methods: We conducted a systematic review of randomised controlled trials (RCTs) of any interventions for men with localised PCa (T1a-T2c N0 M0) published since 1st January 2013 and 23rd of October 2018. Abstract and full text screening were performed in duplicate. Outcome names, definitions, measurements and reported data were coded and categorised following the previous two classifications. The Williamson and Clarke taxonomy (2018) was used to integrate the outcome sets.

Results: Searches identified 1,198 references. Thirty-five systematic reviews were included from which 10 RCTs met the inclusion criteria. Compared to the original outcome prioritisation processes, no new outcomes were identified. Following the categorisation of the Williamson Clarke taxonomy (2018), the terminology across both COSs was integrated and standardised.

Conclusions: The Williamson and Clarke taxonomy helped standardise terminology across the two COS. This integrated COS will be used by the PIONEER Consortium as a basis for data harmonisation across

PIONEER is funded through the IMI2 Joint Undertaking and is listed under grant agreement No. 777492. This joint undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA.

diverse big data resources. It can also be recommended for effectiveness trials, systematic reviews, guidelines and clinical practice. Interim work will focus on reaching consensus on the most appropriate definitions of clinician observed outcomes and the psychometric properties and feasibility of the outcomes identified.

References:

Dodd, S., et al. (2018). "A taxonomy has been developed for outcomes in medical research to help improve knowledge discovery." J Clin Epidemiol 96: 84-92.

MacLennan, S., et al. (2017). "A core outcome set for localised prostate cancer effectiveness trials." BJU International 120(5B): E64-E79.

Martin, N. E., et al. (2015). "Defining a Standard Set of Patient-centered Outcomes for Men with Localized Prostate Cancer." Eur Urol 67(3): 460-467.

PIONEER is funded through the IMI2 Joint Undertaking and is listed under grant agreement No. 777492. This joint undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA.