

Summarising current evidence for the PIONEER study-a-thon: Systematic Literature Review of prostate cancer patients managed with watchful waiting

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Introduction

- Prostate cancer (PCa) affects more than 2 million men in Europe.
- Clinical management of PCa is challenging and involves difficult trade-offs, especially when one should consider not to treat the disease.
- 'Watchful Waiting' (WW) is recommended when local treatment of prostate cancer would not increase the survival of the patient, with the aim of avoiding treatment-related side effects and is a management option for men with low-risk PCa who have a limited life expectancy and for whom curative treatment at the time of progression is not deemed to be beneficial.
- The need for long term outcomes in patients managed with WW makes this topic a good subject for the new Real World Evidence approaches pioneered by OHDSI and EHDEN.
- We therefore conducted a study-a-thon to look at the long term outcomes of prostate cancer managed with WW.
- A study-a-thon is a focused event in which a large-scale study, which traditionally takes many months to complete, is executed and completed in a few days.
- Here, we report on the systematic literature review which was needed to guide the study-a-thon.

Background



- PIONEER's goal is to ensure the optimal care for all European men diagnosed with PCa by unlocking the potential of big data
- The goal of EHDEN is to make the large-scale analysis of health data in Europe a reality. The project aims to do this by building a federated data network of allowing access to the data of 100 million EU citizens standardised to a common data model

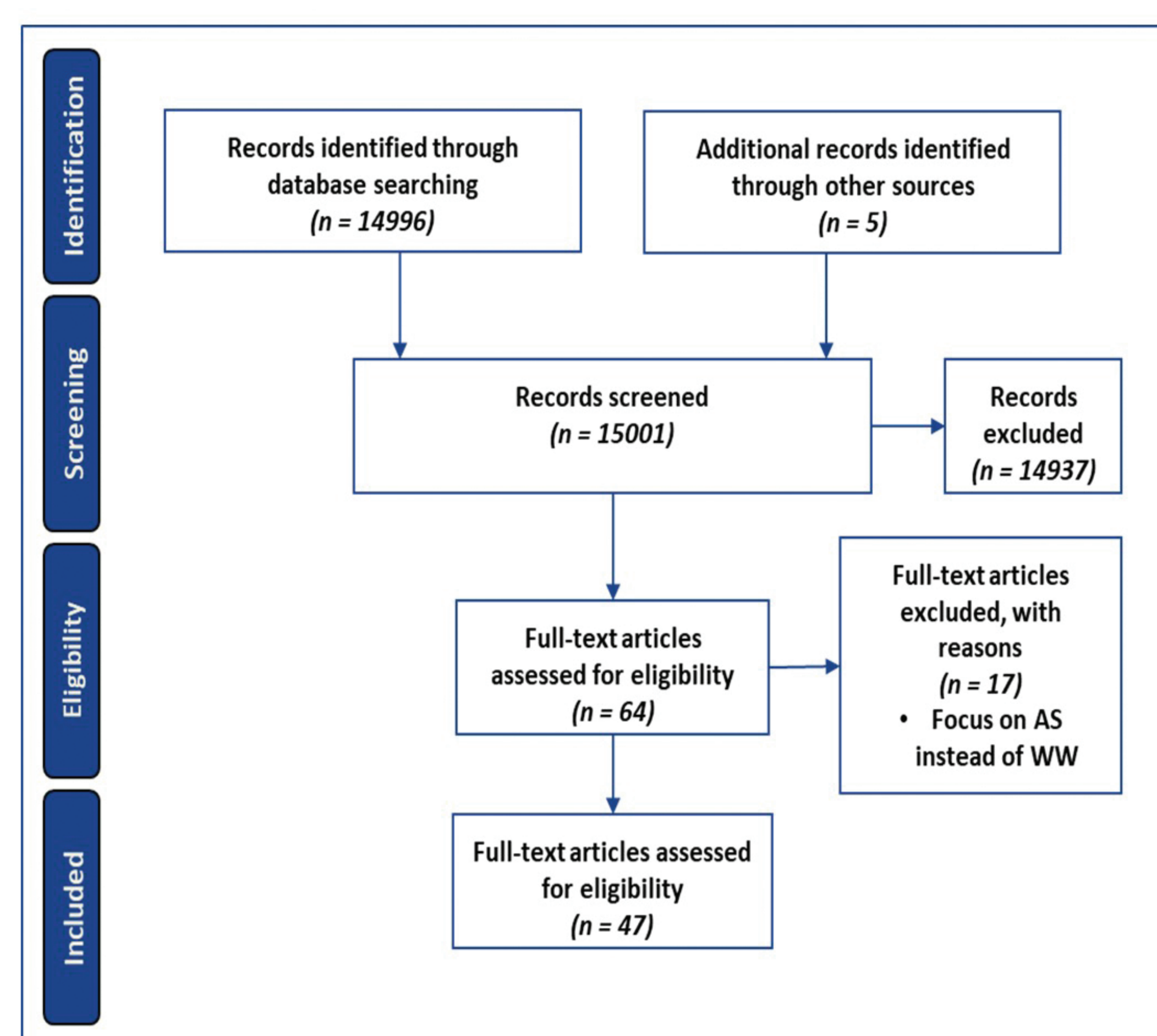
Methods

- We conducted a systematic review of all literature published between 1980 and 2021 reporting on adult men (≥ 18 years of age) who were managed with WW to provide the evidence for the study-a-thon question: "What are the long-term outcomes of prostate cancer patients undergoing non-interventional management (i.e. WW) and what is the impact of comorbidities and life expectancy?"
- We searched for RCT's, non-randomized comparative studies and case cohort studies. All type of reviews were scrutinised for potential papers on WW.
- We extracted: author, year, title, link, country, data source, study design, sample size, target cohort definition, intervention, outcomes, aims and gaps in the literature to ultimately guide the study-a-thon.

Results

- We systematically reviewed 14,996 articles during the months before the study-a-thon.
- After abstract and full text screening, 47 articles were included (see Figure 1).

Figure 1. PRISMA



Results (continued)

- 12 papers reported clinical trials.
- The age range of included patients was 40-88 years, most of the studies used <75 years as the cut off age.
- Research gaps in the literature were identified: e.g. cost effectiveness, better description of the differences in the sub-groups characteristics, better understanding of patient experience on WW, longer follow up.
- The main limitations in the studies identified were small sample sizes and unclear use of terminology (Active Surveillance vs Watchful Waiting).
- As part of the preparation for the study-a-thon, our multidisciplinary group (urologists, patients, epidemiologists and data scientists) translated the identified evidence into data requirements to develop the patient cohorts.

Conclusion

- A systematic review is the key to gather all known available information before starting a study-a-thon.
- The presented work supported research groups in the process of developing a protocol for this study-a-thon.
- A systematic review is key to collect data on information gaps in the available evidence for prostate cancer management.
- The systematic review outcomes can be used to develop cohort definitions, a selection can be used for study-a-thon outcomes and sets a standard for a multidisciplinary group to communicate in 'one language'.
- The outcomes of the systematic review enables the use of different skill sets in which Real World Evidence projects lead to meaningful conclusions.

Study-a-thon overview



5 days
245 participants
4 sub-teams



20 countries
5 time zones



5 patient cohorts
64 phenotypes
17 databases



R package built
4 federated analytic tasks
Shiny app available



Patient-level prediction module executed



2 scientific papers in preparation



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