10th edition
GLOBAL CONGRESS ON PROSTATE CANCER
JUBILEE EDITION

Do we need more real-world data for PCa and BCa?

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Conflicts of interest

Type of affiliation / financial interest	Name of commercial company
Receipt of grants/research supports	Ipsen
Receipt of honoraria or consultation fees	Bayer
Stock shareholder	NA
Other support (please specify):	NA

Real World Data?

Definition

Slides adapted from Dr Beth Russell

Real World Data

- Within the oncology community, growing interest exists in using data from the real world to address clinical and policy-relevant questions that cannot be answered with data from clinical trials.
- The FDA has defined RWD as data relating to patient health status and/or the delivery of health care that are collected from sources that include electronic health records (EHRs), billing claims and product and disease registries .


Real-world data: towards achieving the achievable in cancer care

Christopher M. Booth[1]*, Safiya Karim[2] and William J. Mackillop[1]

Types & Sources of Real-World Data

Clinical Data

- Electronic Health Records
- Case Report Forms (eCRFS)

Patient-Generated Data

- Health & treatment history
- Biometric data
- Patient-reported Outcomes (PROs)

Cost & Utilization Data

- Claims datasets
- Public datasets such as CMS and AHRA

Public Health Data

- Government data sources
- National networks and centers

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Real world evidence can be used to complement evidence from **controlled clinical trials**

THE NEED FOR RCTS AND RWE

- Patients in RCTs are highly selected and have a lower risk profile than real-world populations, with the frequent exclusion of elderly patients and patients with co-morbidities.
- Supplementing RCT evidence with data from observational settings can also improve the external validity of oncology drug trials, such that physicians treating patients in real-world setting have the appropriate evidence on which to base their clinical decisions.

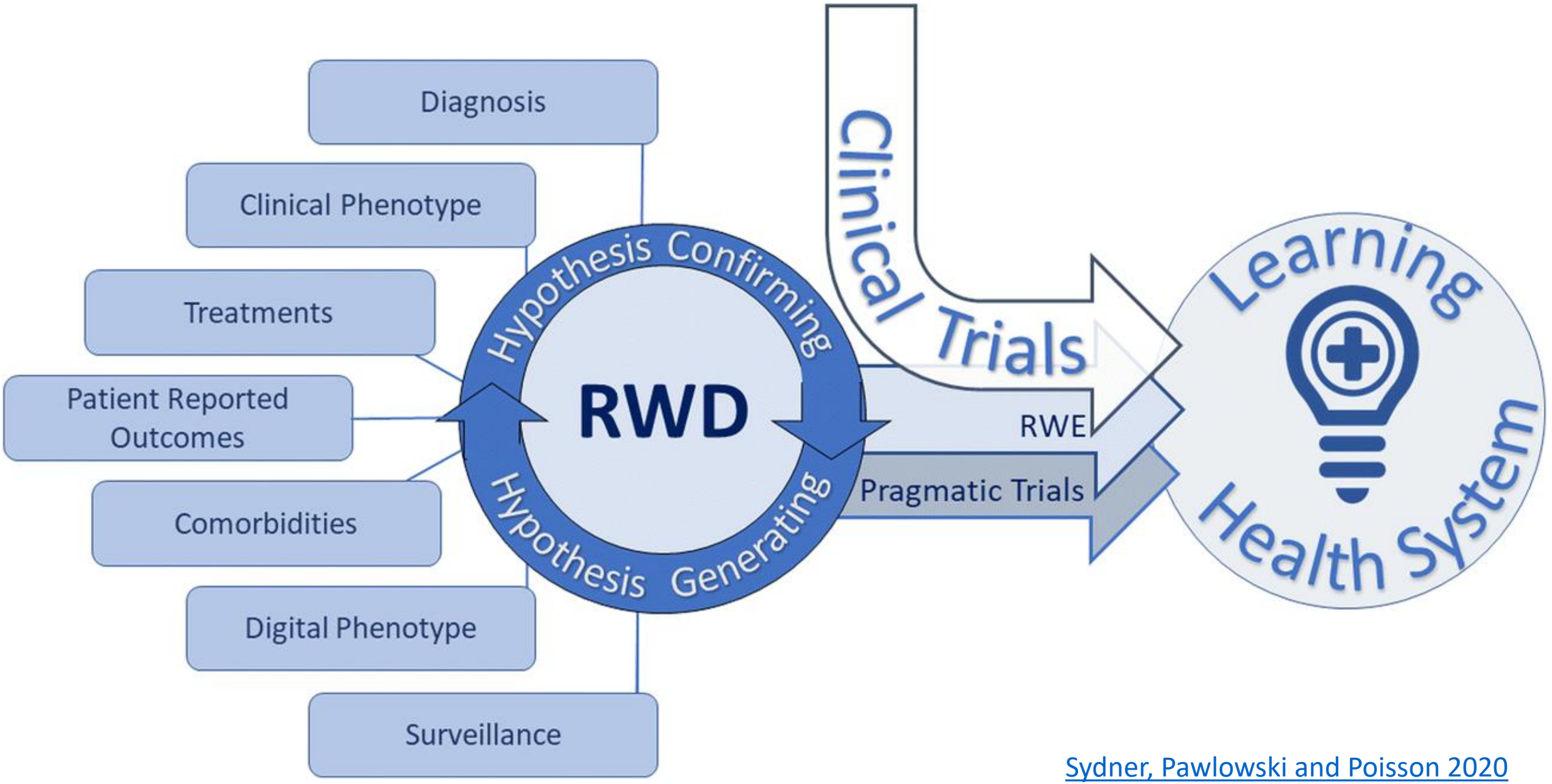
NICE DEFINITION OF RWE

Real-world data can be used to contextualise randomised trials, to estimate effects of interventions in the absence of trials, or to complement trials to answer a broader range of questions about the impacts of interventions in routine settings.

RWE in practise

- NICE Real World Evidence Framework published June 2022
- “Real-world data can improve our understanding of health and social care delivery, patient health and experiences, and the effects of interventions on patient and system outcomes in routine settings”
- “The framework aims to improve the quality of real-world evidence informing our guidance”

NICE
National Institute for Health and Care Excellence



Sydner, Pawlowski and Poisson 2020

Real World Data

Prostate Cancer

PCBase Sweden

FDA Drug Safety Communication: Update to Ongoing Safety Review of GnRH Agonists and Notification to Manufacturers of GnRH Agonists to Add New Safety Information to Labeling Regarding Increased Risk of Diabetes and Certain Cardiovascular Diseases

Absolute and Relative Risk of Cardiovascular Disease in Men With Prostate Cancer: Results From the Population-Based PCBaSe Sweden

Mieke Van Hemelrijck, Hans Garmo, Lars Holmberg, Erik Ingelsson, Ola Bratt, Anna Bill-Axelson, Mats Lambe, Pär Stattin, and Jan Adolfsson

A B S T R A C T

Purpose
Cardiovascular disease (CVD) is a potential adverse effect of endocrine treatment (ET) for prostate cancer (PC). We investigated absolute and relative CVD risk in 76,600 patients with PC undergoing ET, curative treatment, or surveillance.

Methods
PCBaSe Sweden is based on the National Prostate Cancer Register, which covers more than 96% of PC cases. Standardized incidence ratios (SIRs) and standardized mortality ratios (SMRs) of ischemic heart disease (IHD), acute myocardial infarction (MI), arrhythmia, heart failure, and stroke were calculated to compare observed and expected (using total Swedish population) numbers of CVD, taking into account age, calendar time, and previous CVD.

Results
Between 1997 and 2007, 30,642 patients with PC received primary ET, 26,432 curative treatment, and 19,527 surveillance. SIRs for CVD were elevated in all men with the highest for those undergoing ET, independent of circulatory disease history (SIR MI for men without circulatory disease history: 1.40 [95% CI, 1.31 to 1.49], 1.15 [95% CI, 1.01 to 1.31], and 1.20 [95% CI, 1.11 to 1.30] for men undergoing ET, curative treatment, and surveillance, respectively). Absolute risk differences (ARD) showed that two (arrhythmia) to eight (IHD) extra cases of CVD would occur per 1,000 person-years. SMRs showed similar patterns, with ARD of zero (arrhythmia) to three (IHD) per 1,000 person-years.

Conclusion
Increased relative risks of nonfatal and fatal CVD were found among all men with PC, especially those treated with ET. Because ET is currently the only effective treatment for metastatic disease and the ARDs were rather small, our findings indicate that CVD risk should be considered when prescribing ET but should not constitute a contraindication when the expected gain is tangible.

J Clin Oncol 28:3448-3456. © 2010 by American Society of Clinical Oncology

Research Priorities

Introducing PIONEER: a project to harness big data in prostate cancer research

Muhammad Imran Omar, Monique J. Roobol, Maria J. Ribal, Thomas Abbott, Paul-Michael Agapow, Sonia Araujo, Alex Asiimwe, Charles Auffray, Irina Balaur, Katharina Beyer, Chiara Bernini, Anders Bjartell, Alberto Briganti, John-Edward Butler-Ransohoff, Riccardo Campi, Marinel Cavelaars, Bertrand De Meulder, Zsuzsanna Devecseri, Marc Dietrich Voss, Konstantinos Dimitropoulos, Susan Evans-Axelsson, Billy Franks, Louise Fullwood, Denis Horgan, Emma Jane Smith, Amit Kiran, Kati Kivinummi, Mark Lambrecht, Doron Lancet, Peter Lindgren, Sara MacLennan, Steven MacLennan, Maria Manuela Nogueira, Fredrik Moen, Maxim Moinat, Kishore Papineni, Christian Reich, Kristin Reiche, Stijn Rogiers, Claudio Sartini, Kees van Bochove, Femke van Diggelen, Mieke Van Hemelrijck, Hein Van Poppel, Jihong Zong, James N'Dow and The PIONEER Consortium

PIONEER

PIONEER

- PIONEER is part of the Innovative Medicine Initiative's (IMI's) "Big Data for Better Outcomes" (BD4BO) umbrella programme. The BD4BO mission is to improve health outcomes and healthcare systems in Europe by maximising the potential of Big Data
- PIONEER aims to transform the field of prostate cancer care with particular focus on improving prostate-cancer related outcomes, health system efficiency and the quality of health and social care across Europe.

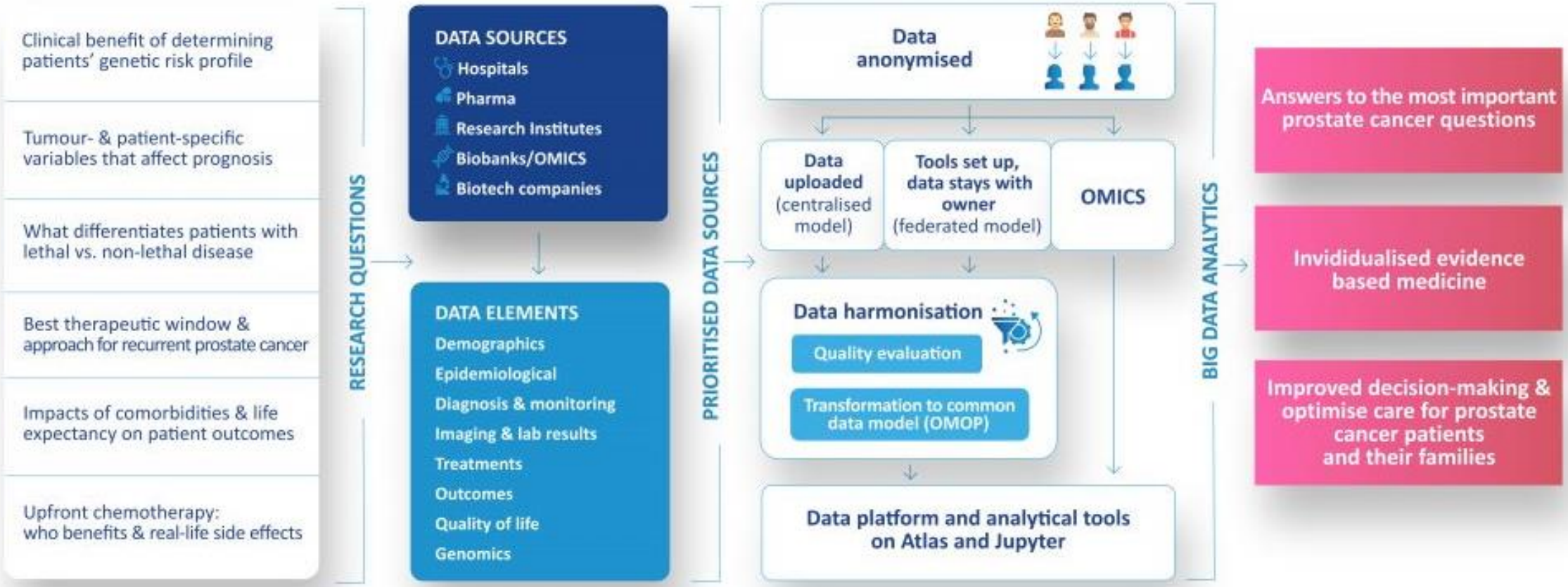
www.prostate-pioneer.eu

PIONEER

BIG DATA PLATFORM

THE EUROPEAN NETWORK OF EXCELLENCE FOR BIG DATA IN PROSTATE CANCER

Together we can ensure each individual patient receives the right treatment for them at the right time.



KNOWLEDGE GAPS | DATA SOURCES | BIG DATA PROCESSING | PIONEER OUTCOMES

Reducing heterogeneity in data collection

EUROPEAN UROLOGY 81 (2022) 503–514

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journal homepage: www.europeanurology.com

European Association of Urology

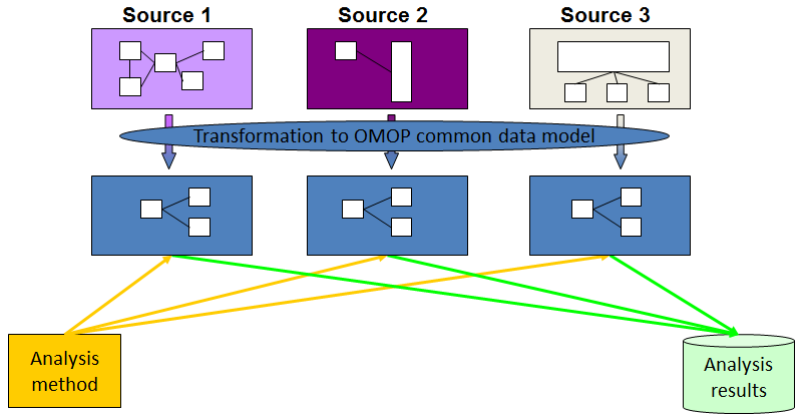
Prostate Cancer

Updating and Integrating Core Outcome Sets for Localised, Locally Advanced, Metastatic, and Nonmetastatic Castration-resistant Prostate Cancer: An Update from the PIONEER Consortium

Katharina Beyer[a,*,†], *Lisa Moris*[b,†], *Michael Lardas*[c], *Muhammad Imran Omar*[d], *Jemma Healey*[d], *Sheela Tripathee*[d], *Giorgio Gandaglia*[e], *Lionne D.F. Venderbos*[f], *Eleni Vradi*[g], *Thomas van den Broeck*[b], *Peter-Paul Willemse*[h], *Tiago Antunes-Lopes*[i,j], *Luis Pacheco-Figueiredo*[i,j], *Serenella Monagas*[k], *Francesco Esperto*[l], *Stephen Flaherty*[m], *Zsuzsanna Devecseri*[n], *Thomas B.L. Lam*[d], *Paula R. Williamson*[o], *Rakesh Heer*[p], *Emma J. Smith*[q], *Alex Asiimwe*[g], *Johannes Huber*[r], *Monique J. Roobol*[f], *Jihong Zong*[s], *Malcolm Mason*[t], *Philip Cornford*[u], *Nicolas Mottet*[v], *Sara J. MacLennan*[d], *James N'Dow*[d], *Alberto Briganti*[e], *Steven MacLennan*[d,‡], *Mieke Van Hemelrijck*[a,‡], *on behalf of the PIONEER Consortium*

OHDSI
OBSERVATIONAL HEALTH DATA SCIENCES AND INFORMATICS

OMOP Common Data Model



Real World Data

Bladder Cancer

Research Priorities

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journal homepage: www.europeanurology.com

European Association of Urology

Research Letters

Consensus in Bladder Cancer Research Priorities Between Patients and Healthcare Professionals Using a Four-stage Modified Delphi Method

Agustina Bessa[a,*], *Steven Maclennan*[b], *Deborah Enting*[a,c], *Richard Bryan*[d], *Debra Josephs*[a,c], *Simon Hughes*[a,e], *Suzanne Amery*[f], *Muhammad Shamim Khan*[f], *Sachin Malde*[f], *Rajesh Nair*[f], *Fidelma Cahill*[a], *Harriet Wylie*[a], *Ramesh Thurairaja*[f], *Kathryn Chatterton*[f], *Netty Kinsella*[a,g], *Christel Häggström*[h,i], *Mieke Van Hemelrijck*[a]

Table 1 – Top 10 unanswered research questions for BC

Ranking	Research question
1	Could molecular profiling of MIBC help select and stratify patients for treatments in respect to risk of relapse and/or prognosis?
2	Which prognostic biomarkers are useful in patients with high-risk NMIBC and MIBC to select effective radical treatments?
3	Can new genomic markers help us risk stratify patients and predict response to therapies?
4	Which biomarkers would allow us to better select high-risk NMIBC patients for early cystectomy?
5	Can new urinary and/or serum-based markers replace invasive techniques in the diagnosis of BC?
6	Can new validated technologies improve the detection of metastatic disease in regional lymphatics and distant sites?
7	Can new urinary and/or serum-based markers improve the detection of BC recurrence after treatment and/or detection of metastatic BC?
8	Which PET imaging agent/tracer would increase the sensitivity and specificity of metastatic BC detection?
9	Following definitive treatment for MIBC, what is the optimal strategy for surveillance?
10	What is the consequence of delay between TURBT and definitive treatment?

BC = bladder cancer; MIBC = muscle-invasive bladder cancer; NMIBC = non-muscle-invasive bladder cancer; PET = positron emission tomography; TURBT = transurethral resection of the bladder cancer.

Bladder cancer data science partnerships?

- 9th most common malignancy worldwide.
- One of the most expensive malignancies to manage on a per patient basis from diagnosis to death.
- Receives <2% of cancer research funding - this has stifled innovation
- Outcomes have remained substantially unchanged for >30 years.
- Greater fundamental biological understanding could lead to rapid translation of diagnostic, prognostic, predictive and surveillance biomarkers, and novel treatment approaches, across all patient groups.

INTERNATIONAL BLADDER CANCER GROUP

NIH NATIONAL CANCER INSTITUTE
International Consortium of Bladder Cancer

BCAN Bladder Cancer Advocacy Network

NCRI National Cancer Research Institute

Conclusion

Real World Data …

- can help address research priorities.
- can complement RCTs.
- needs guidelines for data homogeneity.

- is further developed in the prostate cancer field.
- needs more support and development in the bladder cancer field.

- requires multidisciplinary COLLABORATIONS!

Thank you!

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