Professor Dr James N'Dow explains how the PIONEER project will unlock the power of big data and big data analytics to transform the diagnosis and treatment of prostate cancer

PIONEER: Prostate Cancer DIagnOsis and TreatmeNt Enhancement through the Power of Big Data in EuRope

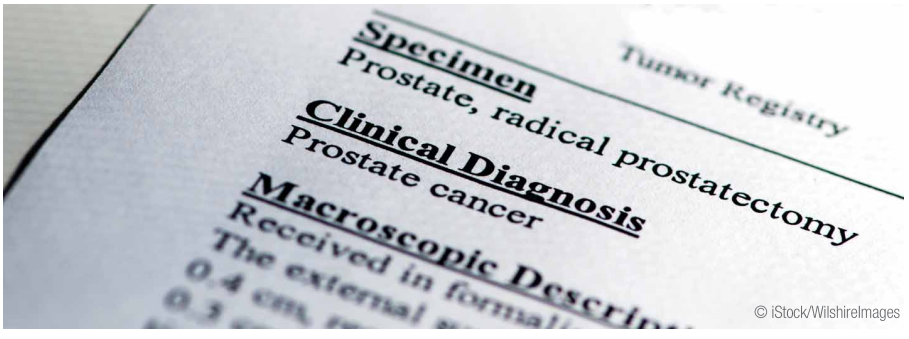
PIONEER is a European Network of Excellence for Big Data in Prostate Cancer, consisting of 32 private and public stakeholders in prostate cancer research and clinical care from across nine countries. Our goal is to ensure the optimal care for all European men living with prostate cancer by unlocking the potential of big data and big data analytics. BD4BO is an umbrella programme of the Innovative Medicines Initiative 2 (IMI2), which is a collaboration between the European Union and European Federation of Pharmaceutical Industries and Associations (EFPIA), and is the world's largest public private partnership in the life and biomedical sciences. EFPIA companies and associated partners do not receive any EU funding but contribute to the projects 'in kind', for example by contributing their researchers' time and expertise or providing access to research facilities or resources. The aim of the BD4BO programme is to improve health outcomes and healthcare systems in Europe by maximising the potential of big data.

Why PIONEER?

It is estimated that there will be approximately 1.3 million new cases of prostate cancer and 359,000 associated deaths worldwide in 2018, making prostate cancer the second most frequent cancer and the fifth leading cause of cancer death in men.[1] Prostate cancer healthcare costs were estimated at €8.43bn per year in the EU in 2009 and accounted for 7% of all cancer costs in Europe.[2] The socioeconomic burden associated with prostate cancer is predicted to dramatically increase in the coming years due to Europe's ageing population. Overall, prostate cancer affects nearly as many people and is as deadly as breast cancer (incidence 7.1% versus 11.6%; mortality 3.8% versus 6.6%, respectively).[1] However, up to now it has received comparatively minimum research funding, and progress made in this field has been limited when compared to other major cancer types.

At present, there are a number of critical knowledge gaps in relation to the screening, diagnosis and treatment of prostate cancer patients, including:

- Lack of standardisation of prostate cancer definitions across all stages of the disease: localised, locally advanced and metastatic prostate cancer
- Insufficient knowledge of the risk factors for developing prostate cancer
- Insufficient knowledge of patient characteristics, including genetic profiles, for optimal stratification of patients at time of diagnosis



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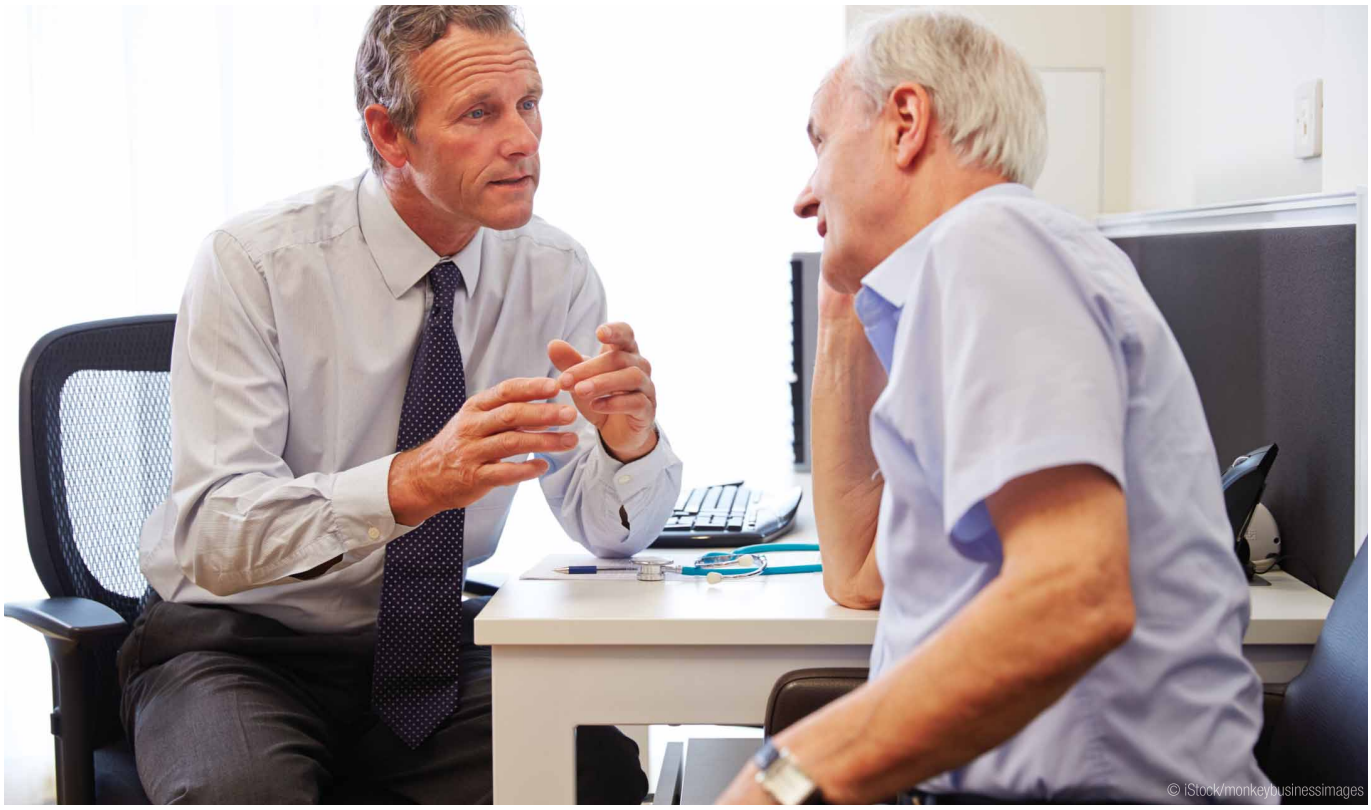
- Lack of meaningful engagement of all key stakeholders, including patients and policymakers, when defining core disease outcome sets
- Lack of effective implementation of knowledge gained into clinical practice, including knowledge informed by real-world data.

This lack of knowledge means that predicting which patients will have the best outcomes with specific treatments is suboptimal, whilst prediction of which patients may be managed safely without treatment remains poor.

Big data is becoming a pivotal part of healthcare and healthcare systems, effectively optimising clinical pathways, directing clinical research, guiding drug development, and shaping shareholders' opinions. However, big data in prostate cancer is currently associated with big challenges compounded by a lack of understanding of the disease, heterogeneity in prostate cancer-related outcomes, lack of data to support clinical practice, and lack of consensus



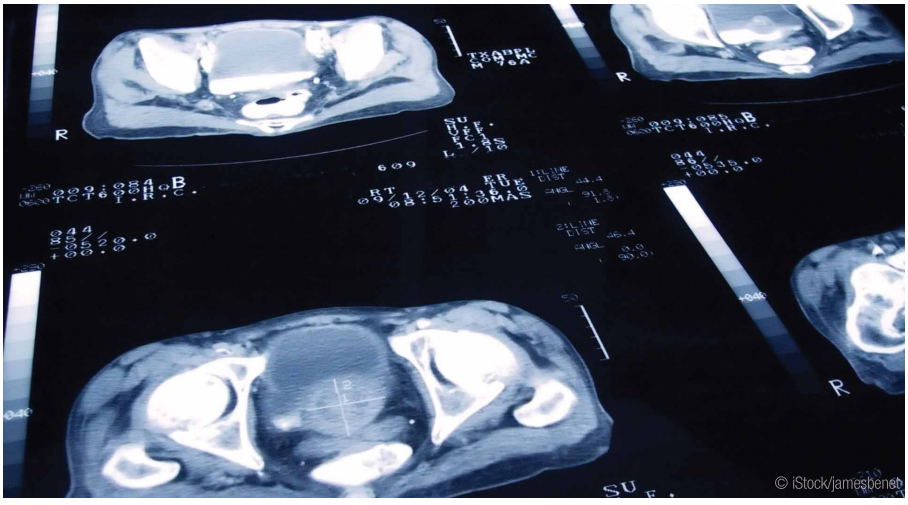
between the different stakeholders. These challenges, in combination with the lack of a suitable data infrastructure, data analytics and data harmonisation models, specifically designed for prostate cancer, severely limit the huge potential of big data in this field.

The current knowledge gaps in the diagnostic and treatment care pathways of prostate cancer make clinical practice decision making difficult and inconsistent. These gaps are dispersed throughout the patient's journey (from diagnosis to treatment) and they compromise the quality of care provided to patients, as well as affecting their quality of life. For example, healthcare professionals do not always have enough information to support patients in their decisions (such as timing and need for prostate-specific antigen testing, the associated risks and possible treatment options). In addition, healthcare professionals do not always have access to all the available treatment options (i.e. not all the drugs or treatment modalities are available in every European country). This means that patients do not always receive the most appropriate treatment for them. Moreover, different treatment options have different side effects, and patients are not always correctly informed to make the right decision for them personally. This lack of understanding of the disease across all stages, coupled with a limited consensus on the most important outcomes and diagnostic and prognostic factors for prostate cancer, makes clinical decision making a complex task and creates unacceptable inequalities among European prostate cancer patients.

PIONEER's approach – how will PIONEER achieve its goals?

A better understanding of the disease and identification of the most relevant prostate cancer-related outcomes can only be achieved by bringing together and analysing real-world data. PIONEER will provide valuable insights into prostate cancer definitions and will deliver a core set of prostate cancer-related outcomes and diagnostic and prognostic factors that all relevant stakeholders agree on. These standardised outcomes and diagnostic and prognostic factors will be used to inform the different disease stages of the patient care pathway. Building on this, PIONEER will also generate robust supporting data (e.g. white papers, guidelines, consensus definitions) that will allow not only standardisation of the different care pathways across different European geographies, but also the implementation of these pathways into clinical practice. It is PIONEER's ultimate ambition to minimise inequalities between all European prostate cancer patients. Standardisation of care pathways across European borders is vital for achieving this.

PIONEER will standardise and integrate existing big data from quality multidisciplinary data sources into a single innovative systems medicine data and knowledge platform, which will leverage two existing data platforms, tranSMART and OHDSI, developed in previous IMI projects. This will result in a unique and comprehensive dataset that consists of the most relevant prostate cancer clinical trials and registries, large epidemiological



cohorts, electronic health records, and real-world data from different European and non-European patient populations. PIONEER's large and harmonised repository of prostate cancer data will be used to improve evidence-based decision making for all prostate cancer patients. The use of these datasets during and beyond PIONEER will be Findable, Accessible, Interoperable and Reusable (FAIR) both for human and machine-driven activities. This will increase their interoperability with current and future multifactorial prostate cancer datasets, allowing researchers to formulate and answer clinically relevant research questions beyond the project's current scope. PIONEER's harmonised data and knowledge platform will enable PIONEER to directly answer questions regarding the natural history, cost-effectiveness and clinical utility of new and innovative interventions during the prostate cancer diagnostic and treatment pathway for all stages of the disease.

PIONEER further aims to identify and address evidence gaps which can delay decision making by regulatory agencies, HTA bodies and payers, thus compromising timely patient access to innovative treatments for prostate cancer. The key to efficient assessment and adoption of new medicines is the successful and effective integration of stakeholder needs in the generation of relevant evidence, which addresses key uncertainties and supports decision making in relation to regulatory approval, HTA recommendation, reimbursement approval and patient access. Evidence from randomised controlled trials (RCTs) has traditionally been the gold standard for decision making regarding access decisions for new cancer drugs. However, prostate cancer is characterised by remarkable heterogeneity and patient sub-populations are often not adequately characterised in RCTs. In recent years, considerable amounts of data generated from an increasingly diverse number of sources (e.g. electronic health records) have offered new opportunities for evidence collection. PIONEER is in a unique position to provide high-quality datasets to address some of the gaps in the evidence used to support decision making regarding access to new drugs for prostate cancer patients, and for treatment guideline development and clinical adoption, thus providing a solid evidence base to inform increasingly personalised therapeutic approaches.

To capitalise on this unique position PIONEER will create an Expert Committee Policy Stakeholder Feedback Forum. The expert committee will include the PIONEER partners and associate members, external experts at the EU and Member States level ensuring broad representation of the diverse healthcare systems in Europe, patients and clinicians, regulators, HTA bodies and payers, as well as stakeholders involved in evidence generation. This forum will be essential to provide input and advice to PIONEER to ensure the generalisability and applicability of PIONEER's outputs to effectively influence decision making processes at the EU level.

PIONEER's ultimate objective

Through the development of a single advanced integrated systems medicine data and knowledge platform for prostate cancer and the application of novel methodologies and advanced analytical methods for 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. PIONEER will make a meaningful contribution towards improved patient stratification and improved identification of low- and high-risk patients, including which patients are more likely to respond to a specific treatment. Ultimately, PIONEER's results will positively affect the way clinical trials are performed (based on improved patient stratification), thereby driving drug development.

References

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