OPTIMA

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- Education

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No Financial COI to declare

OPTIMA - €21.3M EU IMI funded kicked-off October 2021

OPTIMA

OUR VISION

That every patient should have access to the most up-to-date individualised treatments and to innovative therapies.

By strengthening shared decision-making based on dynamic computer-interpretable guidelines (CIGs), innovative broad data access and AI-driven technology and tools, we envision revolutionizing oncology care in Europe.

Prostate, Breast, and Lung cancer

OUR MISSION

To design, develop and deliver an interoperable and GDPR-compliant European real-world oncology data and evidence generation platform from the onset based on the needs of the clinicians and patients, in an inclusive and sustainable way

imi | innovative medicines initiative

EAU Guidelines — eau European Association of Urology

Aberdeen Urology CHaRT Trials Portfolio

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Scandal of poor medical research

"We need less research, better research, and research done for the right reasons. Abandoning using the number of publications as a measure of ability would be a start."

Professor Douglas Altman
1948 - 2018

Richard Smith: Medical research—still a scandal

January 31, 2014

Twenty years ago this week the statistician Doug Altman published an editorial in the *BMJ* arguing that much medical research was of poor quality and misleading. In his editorial entitled, "The Scandal of Poor Medical Research," Altman wrote that much research was "seriously flawed through the use of inappropriate designs, unrepresentative samples, small samples, incorrect methods of analysis, and faulty interpretation." Twenty years later I fear that things are not better but worse.

Challenges of patient care needing global solutions including targeted EDUCATION

- 90% of what is published in the scientific literature is unreliable and unfit to trigger a change of Clinical Practice Guideline Recommendations

- In some EU member states, up to 4 out of 5 patients **DO NOT** receive evidence based care for certain conditions
  - Risks poorer outcomes
  - Risks unnecessary complications
  - Risks unnecessary costs
  - Risks potentially harmful care (25%)

Our PURPOSE

1972 - **Improvement of patient care at the forefront of everything the association does**

2024 - **Improvement of patient care must be evidence-based, patient-centric, cost-effective and data-driven towards value-based healthcare provision** *(Equitable, sustainable and transparent use of the available resources to achieve better outcomes and experiences of care for every person)*

Why a focus on Real World Data (RWD) now?

What we know today:

- Clinical trial populations not generalisable
- Key subgroups typically not represented, eg.
  - Elderly / Multiple comorbidities / Polypharmacy / Obesity / Ethnicity / LMICs
- Longterm outcomes and adverse events often missing in RCTs

Complementarity between clinical trials & RWD

Mistake to think published outcomes from Centres of Excellence representative of practice in general (need RWD)

The European Network of Excellence for Big Data in Prostate Cancer

Who we are

IMI2 funded pan-EU public private partnership (2018-2023) - **€12 million**

Our goal

To ensure the optimal care for all European men diagnosed with prostate cancer by unlocking the potential of big data and big data analytics.

www.prostate-pioneer.eu

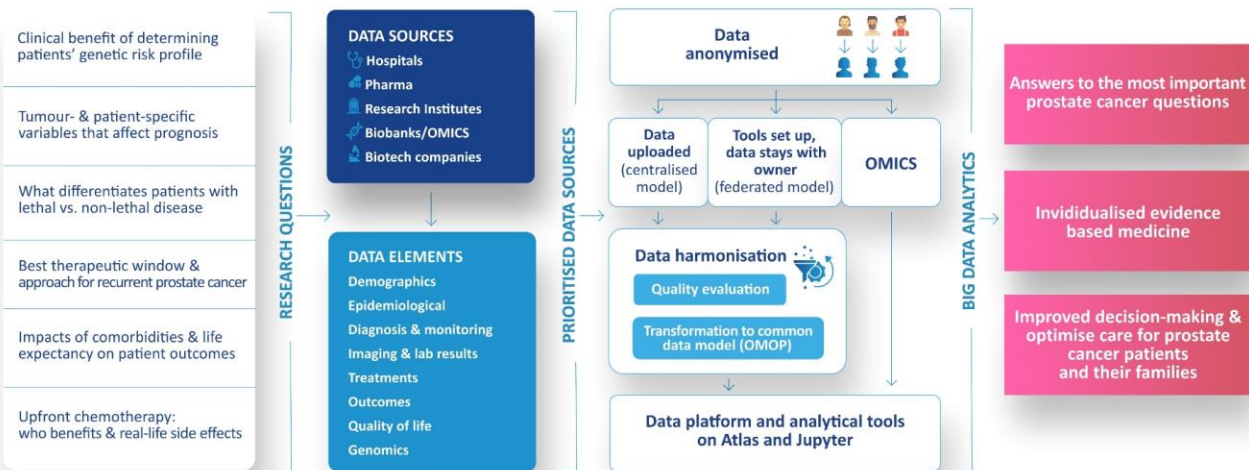@ProstatePioneer

Neutral Platform

PIONEER (€12M - 2018) showed us the Path (& limitations) of Secondary Data Sources

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.

RESEARCH QUESTIONS

- Clinical benefit of determining patients' genetic risk profile
- Tumour- & patient-specific variables that affect prognosis
- What differentiates patients with lethal vs. non-lethal disease
- Best therapeutic window & approach for recurrent prostate cancer
- Impacts of comorbidities & life expectancy on patient outcomes
- Upfront chemotherapy: who benefits & real-life side effects

DATA SOURCES
- Hospitals
- Pharma
- Research Institutes
- Biobanks/OMICS
- Biotech companies

DATA ELEMENTS
- Demographics
- Epidemiological
- Diagnosis & monitoring
- Imaging & lab results
- Treatments
- Outcomes
- Quality of life
- Genomics

PRIORITISED DATA SOURCES

Data anonymised

Data uploaded (centralised model)

Tools set up, data stays with owner (federated model)

OMICS

Data harmonisation
- Quality evaluation
- Transformation to common data model (OMOP)

Data platform and analytical tools on Atlas and Jupyter

BIG DATA ANALYTICS

- Answers to the most important prostate cancer questions
- Invidividualised evidence based medicine
- Improved decision-making & optimise care for prostate cancer patients and their families

KEY LESSONS

Every dataset has limitations including:
- *Missing clinical concepts*
- *Incompleteness*
- *Variation in coding practices*
- *Poor quality control*
- *Spotty patient histories*

Translating clinical questions & concepts into computer ontologies is imperfect at best

Widespread variation in clinical practice limits ability to pool across data sources

It takes time to learn and understand the nuisances of each dataset

Best results are obtained when multi-disciplinary teams are given time to interact with the data and each other in refining research protocols

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KNOWLEDGE GAPS

56 research questions identified
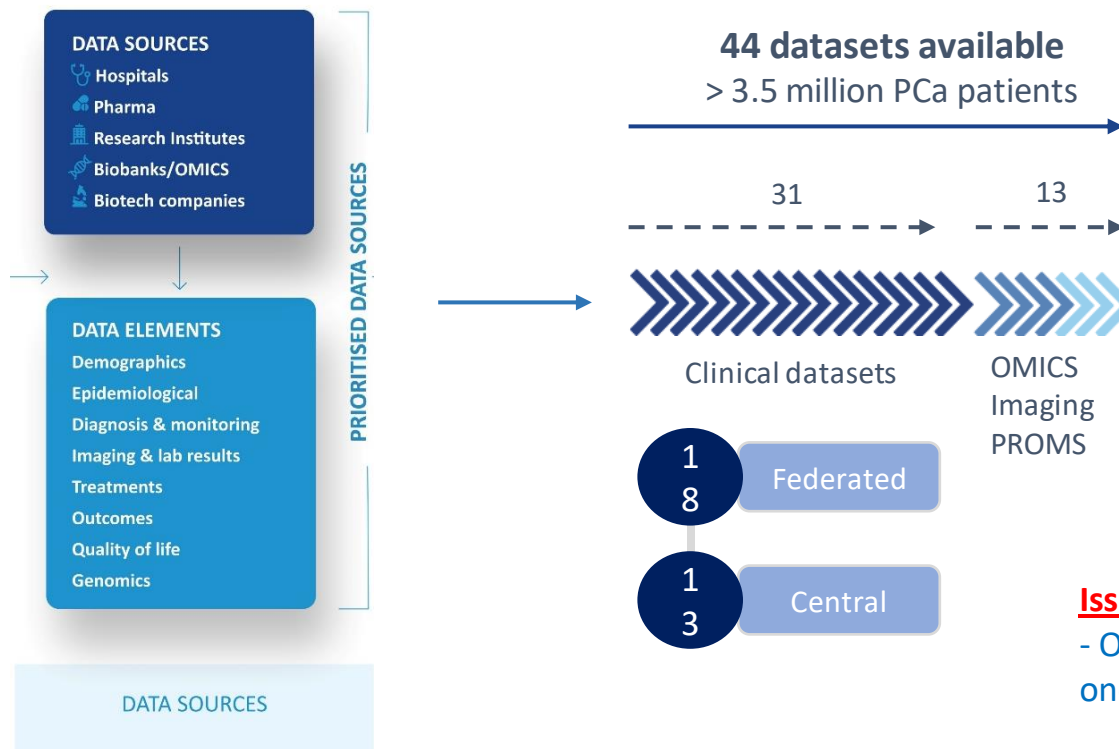14 active study teams formed

DATA SOURCES

104 Data sets Catalogued

BIG DATA PROCESSING

44 Data sets available
18 Federated / 13 Central
3 Study-a-thons Conducted

PIONEER OUTCOMES

12 Manuscripts Published
20+ Presentations & Posters
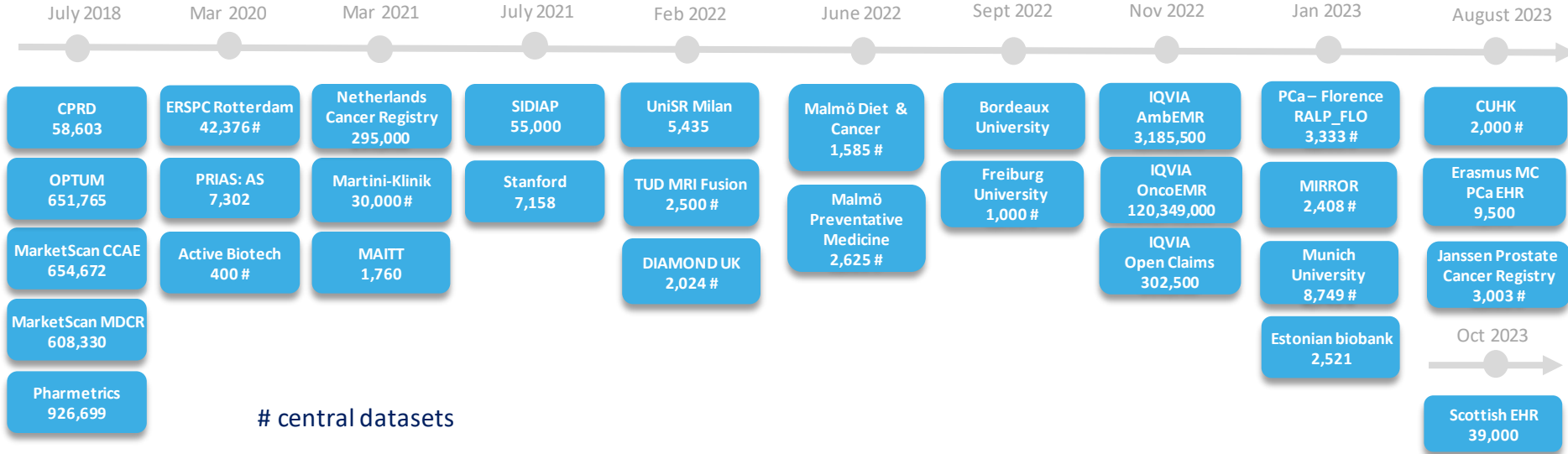15 Manuscripts in development

PIONEER Clinical Data Sources Overview

UNIVERSITY OF ABERDEEN

eau European Association of Urology

95 Data sources identified

31 Datasets ready in platform

Dataset onboarding to the PIONEER Platform
(number of prostate cancer patients per dataset shown)

July 2018	Mar 2020	Mar 2021	July 2021	Feb 2022	June 2022	Sept 2022	Nov 2022	Jan 2023	August 2023

CPRD 58,603

ERSPC Rotterdam 42,376 #

Netherlands Cancer Registry 295,000

SIDIAP 55,000

UniSR Milan 5,435

Malmö Diet & Cancer 1,585 #

Bordeaux University

IQVIA AmbEMR 3,185,500

PCa – Florence RALP_FLO 3,333 #

CUHK 2,000 #

OPTUM 651,765

PRIAS: AS 7,302

Martini-Klinik 30,000 #

Stanford 7,158

TUD MRI Fusion 2,500 #

Malmö Preventative Medicine 2,625 #

Freiburg University 1,000 #

IQVIA OncoEMR 120,349,000

MIRROR 2,408 #

Erasmus MC PCa EHR 9,500

MarketScan CCAE 654,672

Active Biotech 400 #

MAITT 1,760

DIAMOND UK 2,024 #

IQVIA Open Claims 302,500

Munich University 8,749 #

Janssen Prostate Cancer Registry 3,003 #

MarketScan MDCR 608,330

Estonian biobank 2,521

Oct 2023

Pharmetrics 926,699

 # central datasets

Scottish EHR 39,000

PIONEER Platform imaging, omics and PROM data
(number of prostate cancer patients per dataset shown)
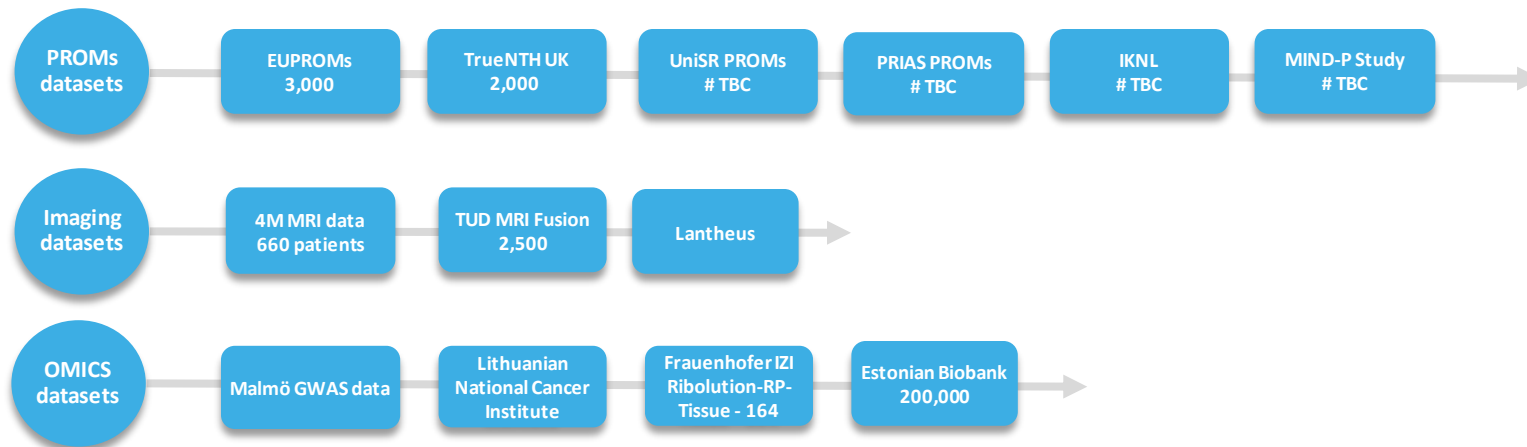
PROMs datasets
- EUPROMs 3,000
- TrueNTH UK 2,000
- UniSR PROMs # TBC
- PRIAS PROMs # TBC
- IKNL # TBC
- MIND-P Study # TBC

Imaging datasets
- 4M MRI data 660 patients
- TUD MRI Fusion 2,500
- Lantheus

OMICS datasets
- Malmö GWAS data
- Lithuanian National Cancer Institute
- Frauenhofer IZI Ribolution-RP-Tissue - 164
- Estonian Biobank 200,000

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"Neutral Platform"

innovative medicines initiative

eau Guidelines

eau European Association of Urology

A strong alliance of public & private partners

38 Partners across 13 countries in the following fields:

- IMI experienced researchers / SMEs
- Professional medical societies
- Cancer key opinion leaders
- Leading guideline developers
- Patient organisations
- Committed industry partners
- EMA steering group members
- AI in healthcare experts
- Implementation scientists

Access to the right data

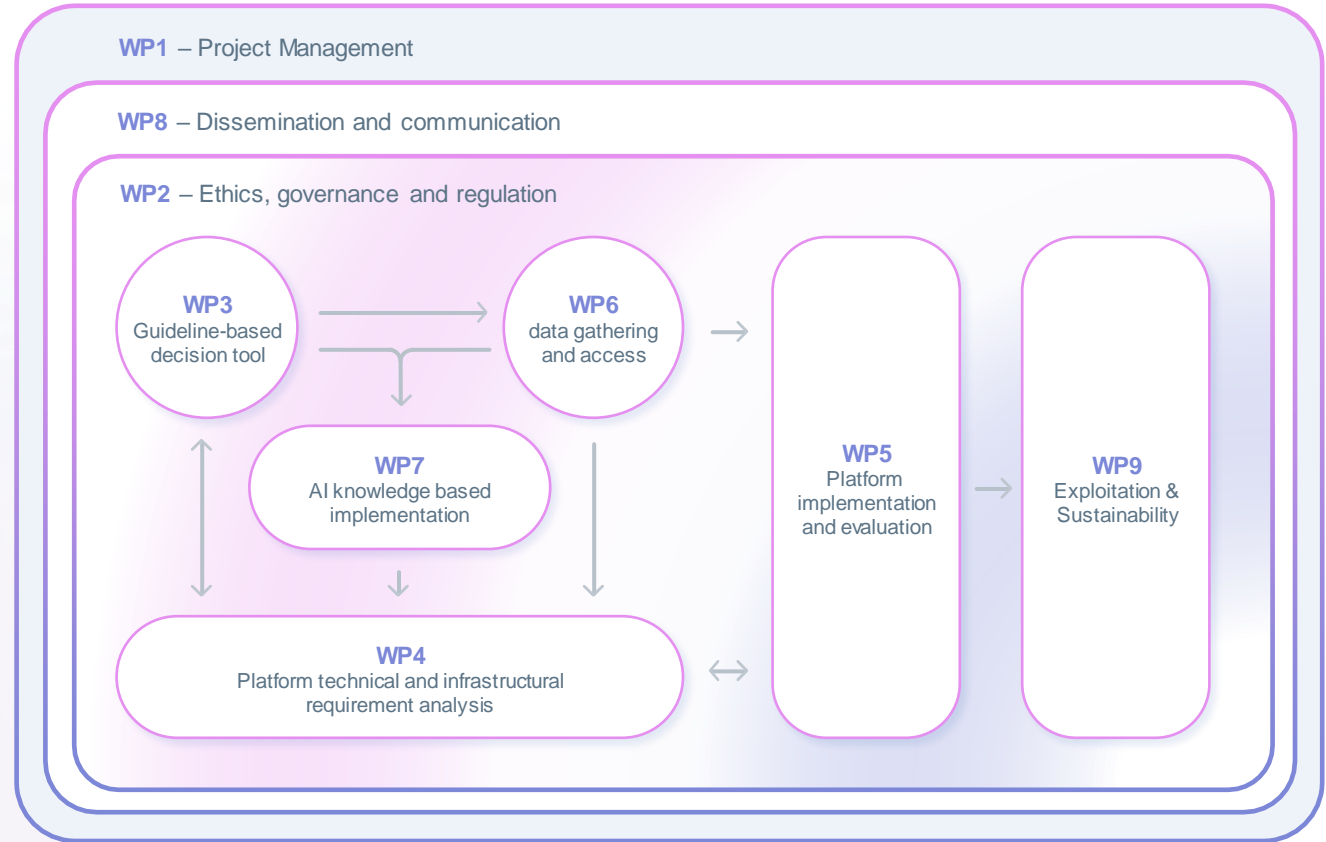**OPTIMA has secured access to data covering >200 million people**

- EHR data of **>138m** people in Europe and **>56m** people in the US

- Biobanks, cancer registries and cohort data of **>7m** patients in Europe

- Trial or prospective cohort data of **>6,000** participants
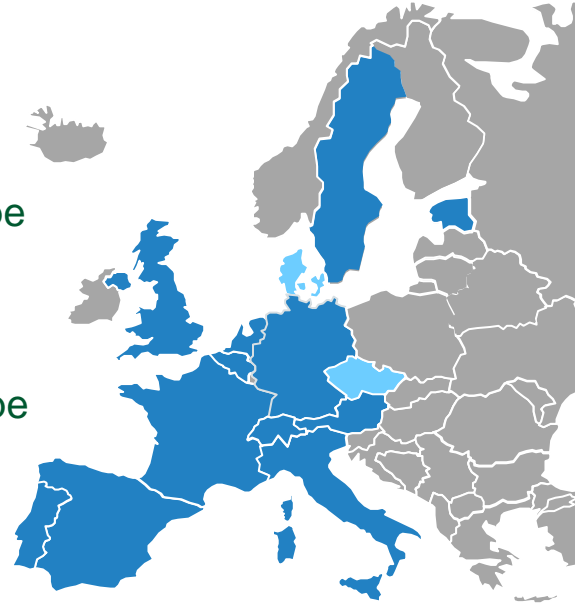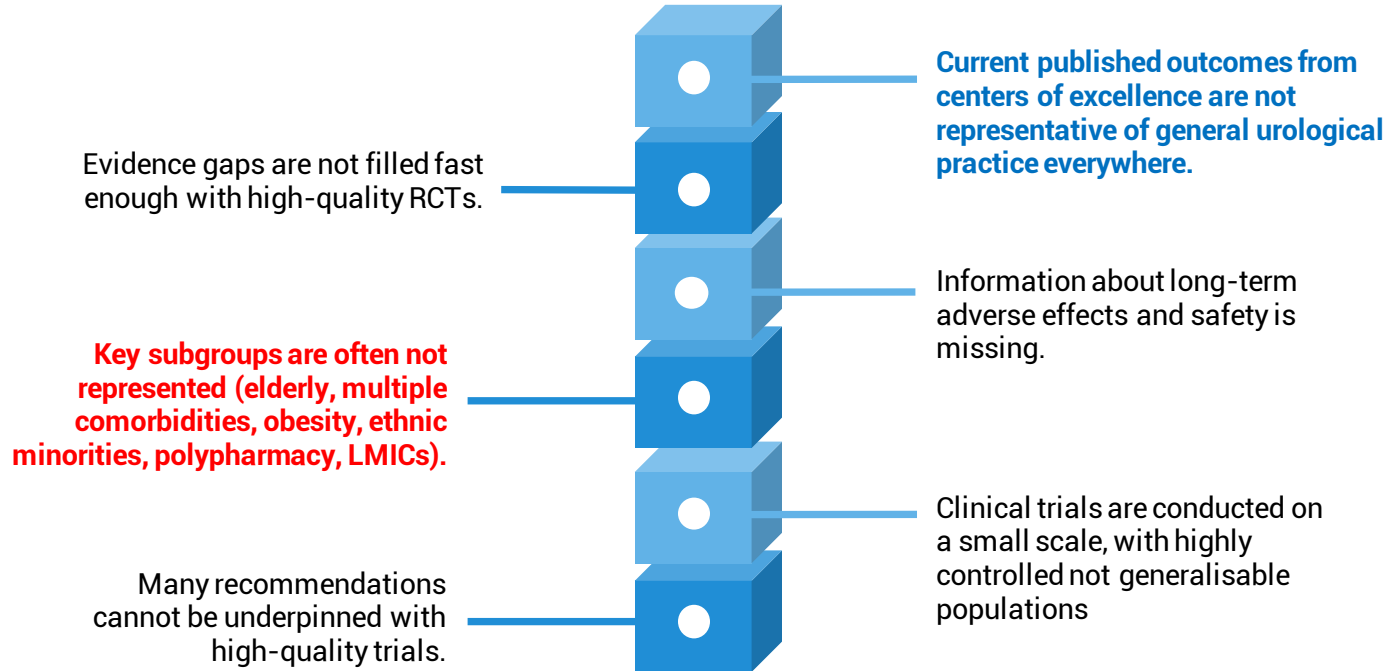
From generic guideline recommendations to truly individualising patient care driven by data

Work packages

Lay the early requirements for long-term sustainability – first deliverables.

WP1 – Project Management

WP8 – Dissemination and communication

WP2 – Ethics, governance and regulation

WP3
Guideline-based decision tool

WP6
data gathering and access

WP7
AI knowledge based implementation

WP5
Platform implementation and evaluation

WP9
Exploitation & Sustainability
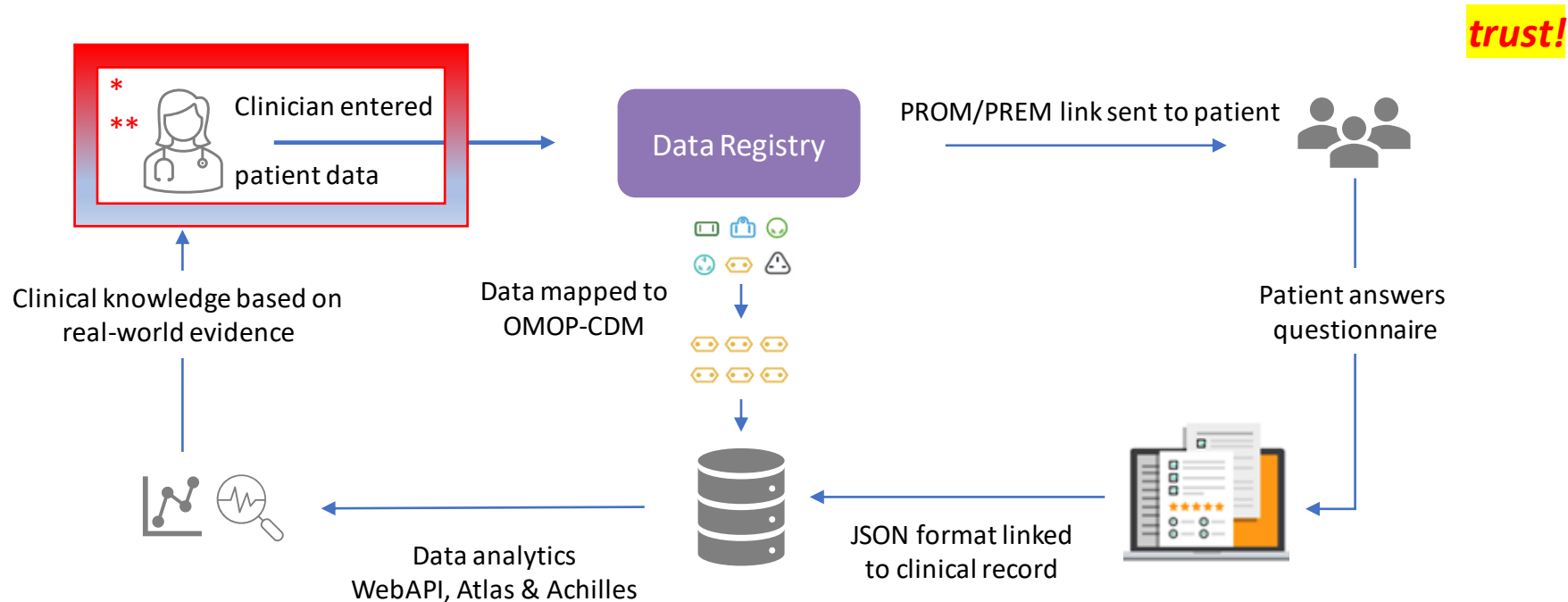
WP4
Platform technical and infrastructural requirement analysis

EXPANDING Real-World Evidence has high potential to boost urology research and address current practice challenges <u>Globally</u>

Current published outcomes from centers of excellence are not representative of general urological practice everywhere.

Evidence gaps are not filled fast enough with high-quality RCTs.

Information about long-term adverse effects and safety is missing.

Key subgroups are often not represented (elderly, multiple comorbidities, obesity, ethnic minorities, polypharmacy, LMICs).

Clinical trials are conducted on a small scale, with highly controlled not generalisable populations

Many recommendations cannot be underpinned with high-quality trials.

A new approach in urology patient care is urgently needed.

Data and Real World Evidence are the game changers!

eau
uroevidence
hub

Data Haven - prospective registries (COS)

trust!

Clinician entered patient data
*
**

Data Registry

PROM/PREM link sent to patient

Clinical knowledge based on real-world evidence

Data mapped to OMOP-CDM

Patient answers questionnaire

Data analytics
WebAPI, Atlas & Achilles

JSON format linked to clinical record

* Diagnosis – patient and disease characteristics; ** Change of treatment/Recurrence/missing data

Living Guidelines incorporating RWE

Results of continuous real-world updating and accurate translation to the individual patient in their specific treatment setting will create next generation "living" guidelines and a new way of interaction between clinicians, patients and care-givers.

- improved quality of care

- strengthening the role of shared treatment decisions

- empower individualised treatment recommendations and care plans that consider physical, emotional and psychological patient needs and preferences.

- Context-sensitive (including LMIC context)

OPTIMA
Thank you for your attention!

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