



Autoridade Nacional do Medicamento
e Produtos de Saúde I.P.

CONFERÊNCIA INFARMED: UTILIZAÇÃO DE DADOS EM SAÚDE

30 ANOS A PROTEGER A SUA SAÚDE

COMEMORAÇÃO DOS 30 ANOS
DO INFARMED, I.P.

Com o Alto Patrocínio
de Sua Excelência
Eisden the High Patronage of the
President of the Portuguese Republic



O Presidente da República

25 SETEMBRO 2023

NOVA SCHOOL OF BUSINESS & ECONOMICS
CARCAVELOS

PRESTAÇÃO DE CUIDADOS DE SAÚDE BASEADO EM DADOS

Júlio Oliveira

Médico especialista em Oncologia Médica e Farmacologia Clínica
Presidente do Conselho de Administração do IPO Porto



IPOPORTO
INSTITUTO PORTUGUÊS DE ONCOLOGIA DO PORTO FG, EPE



INTEGRAÇÃO DE DADOS NOUTRAS INDÚSTRIAS (...E NA SAÚDE?)



Utilização de controlos eletrónicos » análise em tempo real »
saber como manter o avião seguro no ar e contribuir para a próxima geração de produtos

E porque
não
aprender
com outras
indústrias
e setores
da
sociedade?

Bottlenecks

Tecnologia

Propriedade dos dados

Política (desalinhamento/falta estratégia)

Assumir que “a saúde é diferente!”

Consentimento informado

Proteção de dados dos doentes/cidadãos

Conscienciarizar
para a
importância dos
dados em saúde

Apoio a iniciativas de *data-sharing*

Incremento da proteção de dados pessoais

Potenciar investimento e colaboração a
escala europeia (e global)

How is RWE driving impact in drug development and patient access to innovation?



Early discovery

- Oncology development is increasingly personalized and precise, with narrower and more nuanced indications characterized by **genomic alterations and signatures**.

Trial design and feasibility

- Can supports the design and optimization of clinical trials - **to design a protocol that is generalizable to standard of care, assess the impact of eligibility criteria on trial feasibility, and inform the selection of trial sites.**

Trial execution

- Adoption of external control arms which may reduce trial size (i.e., required number of patients), duration, and cost.

How is RWE driving impact in drug development and patient access to innovation?



Post-marketing studies

- As oncology therapies are increasingly approved through the FDA's Breakthrough Therapy Designation Pathway, the number and scope of resource intensive post-marketing studies is growing.

Indication expansion

- leveraging the series of natural experiments that occur as part of off-label use to clarify – and where possible, broaden – indications or guidelines for previously approved therapies.

Market access and reimbursement

- In the face of increasing therapy costs, payors are increasingly asking for evidence of clinical value before providing coverage.

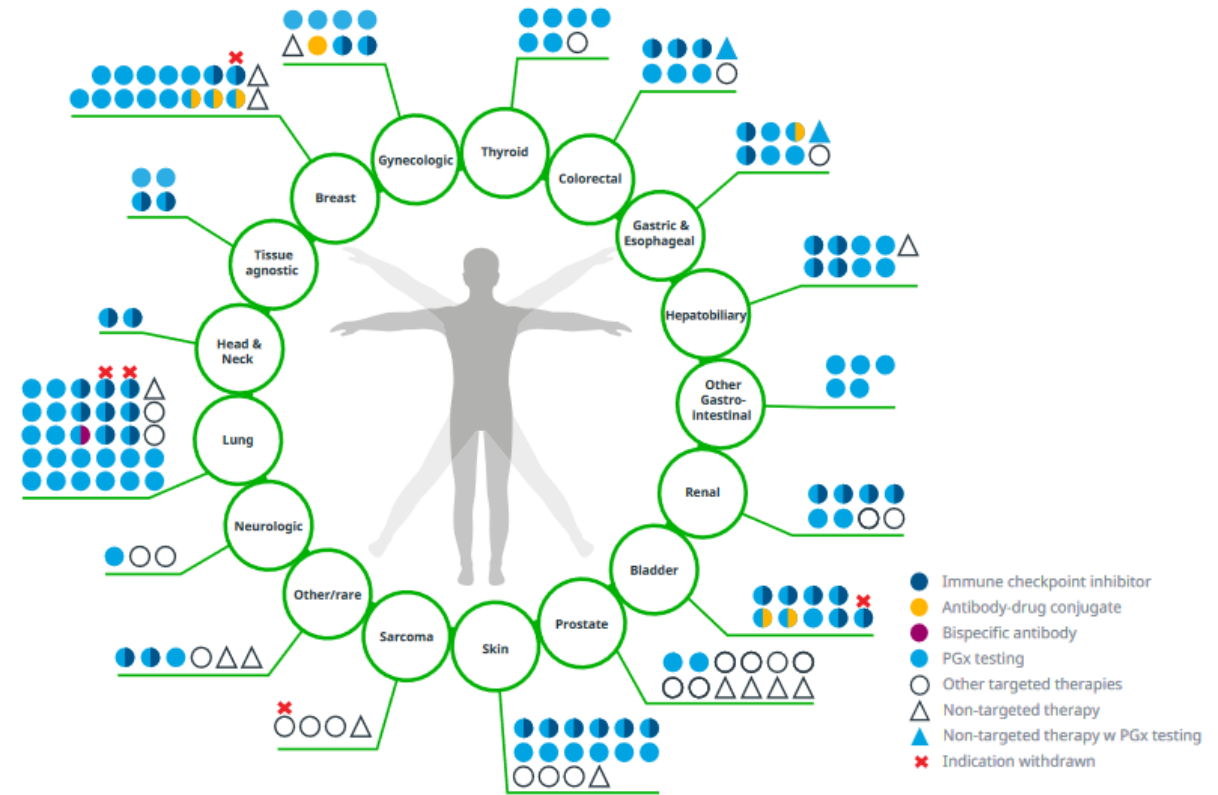
Cancer care

Incidence/Prevalence

Cost/Burden

Survival

Complexity



Cancer drugs expenditure (PT NHS HH; 2021)²: 498.7 MM € (32.0% drug expenditure); Δ 20/21 + 11.7%

A experiência do IPO Porto



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Estrutura orgânica e cultura da organização

- *Serviço de Epidemiologia*
- *Serviço de Planeamento e Apoio à Gestão*
- *Grupo de Investigação em Epidemiologia, Resultados economia e Gestão em Oncologia do CI-IPOP*
- *Outcomes Research Lab*

Infraestrutura

- **Registo Oncológico** e integração de dados de diferentes plataformas eletrónicas

Projetos e exemplo de resultados

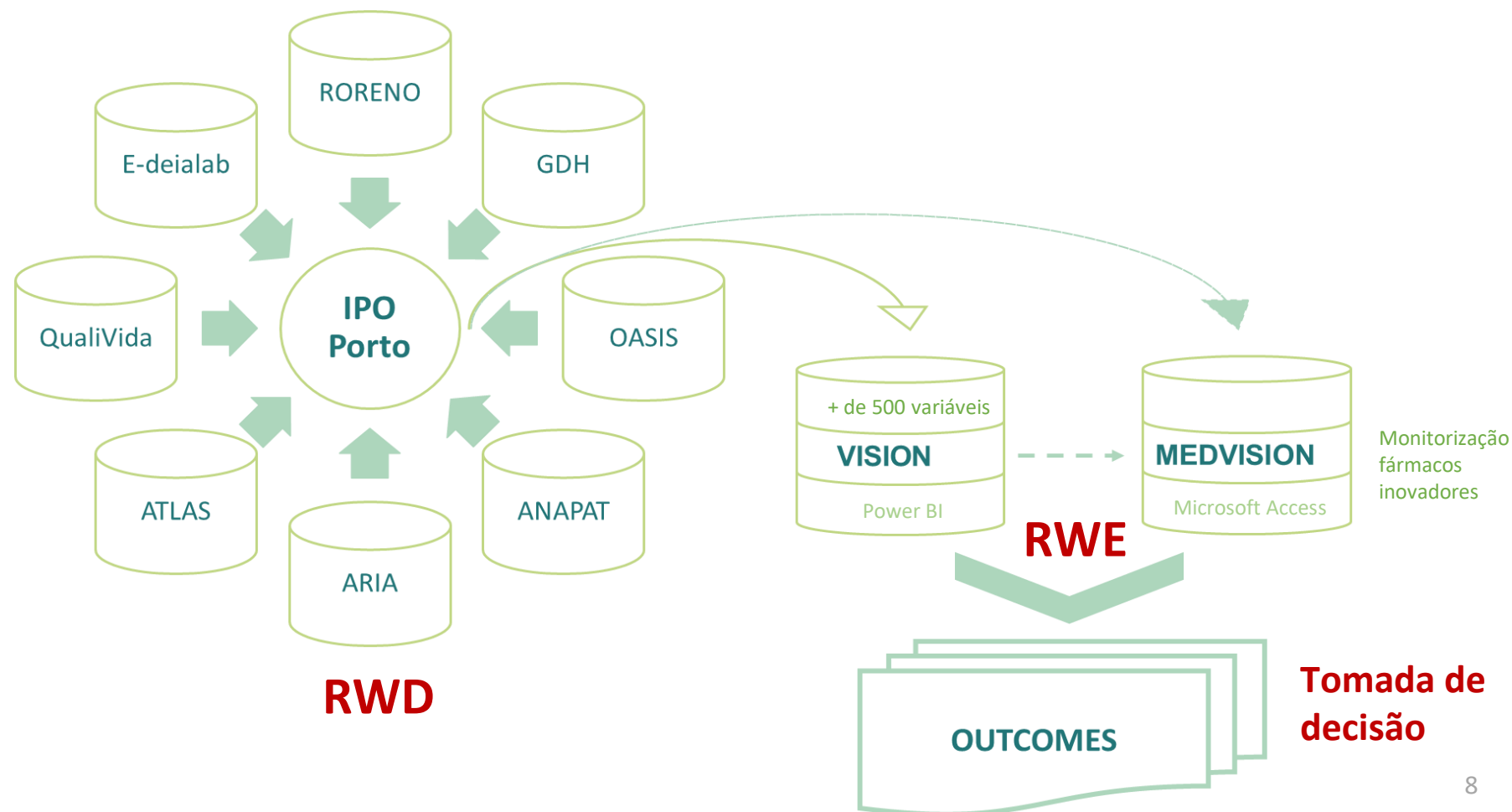
Estudos de “*Real World Evidence*”, comparador sintético, Inteligência Artificial (ex “*text-mining*”)...

Big Data

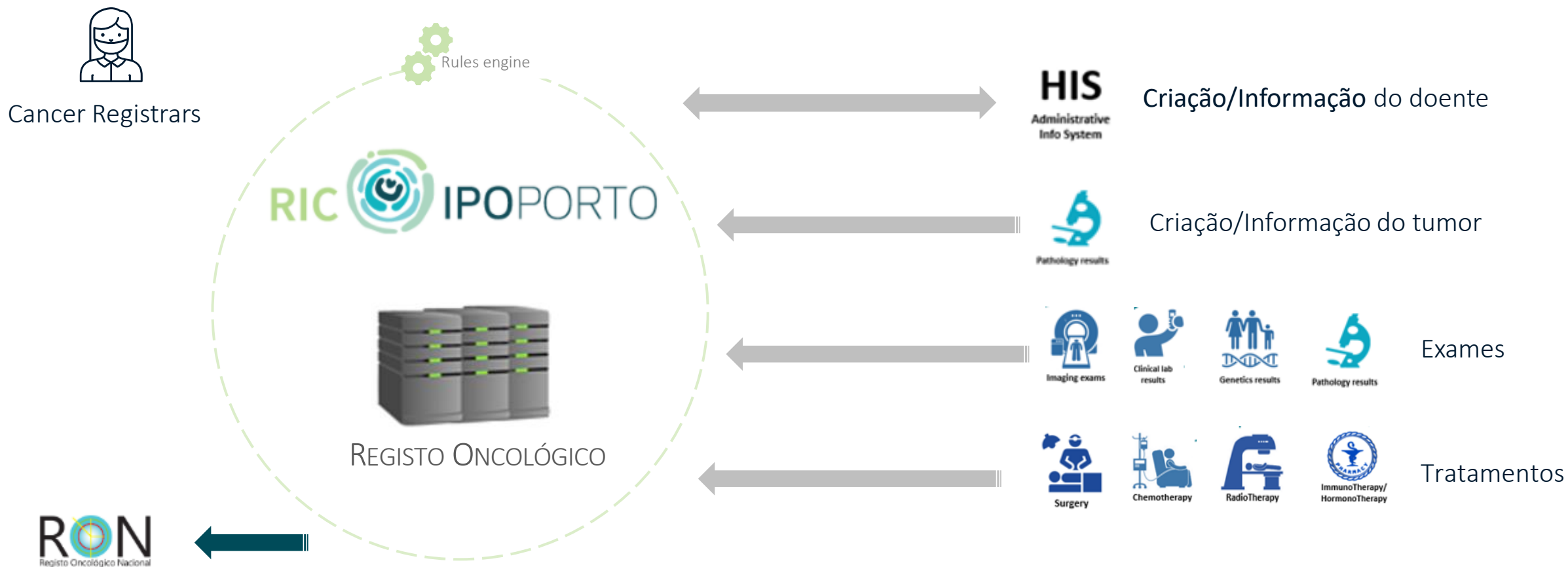
Ferramentas informáticas

RWD | RWE | Tomada de decisão

Porque é que os dados da vida real são uma mais valia e importantes para as decisões de saúde?



REPOSITÓRIO INTEGRADO DO CONHECIMENTO



RIC EM NÚMEROS



JUNE 2022

DECEMBER 2022

333,459 patients

337,619 patients



370,204 cancer cases

374,934 cancer cases



332,928 treatments

349,144 treatments



1,351,118 exams

1,439,891 exams

Áreas de atividade



MULTIDISCIPLINARIEDADE E COOPERAÇÃO

Serviço com equipa multidisciplinar (gestão, eng.biomédica, biotecnologia, ciências da saúde...)

Gabinete de QdV

Abertura
em
Out/2020

Apenas fármacos inovadores



IPOPORTO

Início

Planeamento

Resultados

Relatórios

Gestão de Questionários

Sair



Qualidade de Vida

Esta aplicação pretende acompanhar e perceber a Qualidade de Vida dos pacientes com cancro.

Para esse efeito, serão usados os formulários que têm por base os da EORTC - Organização Europeia para Pesquisa e Tratamento do Cancro, da qual o IPO Porto é membro, e são questionários desenvolvidos para avaliar a qualidade de vida de pacientes com cancro.

73 % de
adesão

RIC – GERAÇÃO DE CONHECIMENTO BASEADO EM DADOS

POPULATION-BASED STUDIES

Cancer incidence predictions in the North of Portugal: keeping population-based cancer registration up to date

Clara Castro^{a,b,c}, Luís Antunes^a, Nuno Lunet^{b,c} and Maria José Bento^a

Trends in gastric and esophageal cancer incidence in northern Portugal (1994-2009) by subsite and histology, and predictions for 2015

Clara Castro, Bárbara Peleteiro, Maria José Bento, Nuno Lunet

Letters to the Editor / Journal of Clinical Epidemiology 98 (2018) 152–161

Cumulative incidence estimates in the presence of competing risks

Samantha Morais, Luis Antunes, Maria Jose Bento, Nuno Lunet

Survival from cancer in the north region of Portugal: results from the first decade of the millennium

Luís Antunes^a, Lúcio L. Santos^b and Maria José Bento^{a,c}

RESEARCH ARTICLE

Open Access

No inequalities in survival from colorectal cancer by education and socioeconomic deprivation - a population-based study in the North Region of Portugal, 2000-2002

Luís Antunes^{1,2,3}, Denisa Mendonça^{4,5}, Maria José Bento^{1,2,6} and Bernard Rachet^{7*}



ELSEVIER

Contents lists available at ScienceDirect

Digestive and Liver Disease

journal homepage: www.elsevier.com/locate/dld



Oncology

Risk and survival of third primary cancers in a population-based cohort of gastric cancer patients

Samantha Morais^a, Luís Antunes^b, Maria José Bento^b, Nuno Lunet^{a,c,*}

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OBSERVATIONAL STUDIES

► PLoS One. 2022 Dec 9;17(12):e0278950. doi: 10.1371/journal.pone.0278950. eCollection 2022.

Costs, effectiveness, and safety associated with Chimeric Antigen Receptor (CAR) T-cell therapy: Results from a comprehensive cancer center

Sérgio Chacim^{1,2}, Teresa Monjardino³, José Luís Cunha^{4,5}, Pedro Medeiros^{4,5,6},
Patrícia Redondo^{4,5}, Maria José Bento^{3,7,8}, José Mário Mariz¹



Radiation doses in mammography exams: Effects of oncological treatments

Ana Ribeiro^a, Jéssica Rodrigues^b, Luís Antunes^b, Sandra Sarmiento^{c,*}

^a Physics and Astronomy Department, Faculty of Sciences, University of Porto, Portugal

^b Cancer Epidemiology Group, IPO Porto Research Center (CI-IPOP), Portuguese Oncology Institute of Porto (IPO Porto), Porto, Portugal

^c Management, Outcomes Research and Economics in Healthcare Group, IPO Porto Research Center (CI-IPOP), and Medical Physics Service, Portuguese Oncology Institute of Porto (IPO Porto), Porto, Portugal

RESEARCH ARTICLE

Open Access

Deprivation-specific life tables using multivariable flexible modelling – trends from 2000–2002 to 2010–2012, Portugal

Luís Antunes^{1,2,3*}, Denisa Mendonça^{3,4}, Ana Isabel Ribeiro^{3,5}, Camille Maringe⁶ and Bernard Rachet⁶

Received: 3 December 2020 | Revised: 13 February 2021 | Accepted: 18 February 2021

DOI: 10.1002/ijc.33532

CANCER EPIDEMIOLOGY



The impact of the COVID-19 pandemic on the short-term survival of patients with cancer in Northern Portugal

Samantha Morais^{1,2} | Luís Antunes³ | Jéssica Rodrigues³ | Filipa Fontes^{1,2,4} |
Maria José Bento^{3,5} | Nuno Lunet^{1,2}

Article

Deregulation of N6-Methyladenosine RNA Modification and Its Erasers FTO/ALKBH5 among the Main Renal Cell Tumor Subtypes

Catarina Guimarães-Teixeira^{1,2}, Daniela Barros-Silva^{1,2,†}, João Lobo^{1,3,4,†}, Diana Soares-Fernandes¹,
Vera Constâncio¹, Pedro Leite-Silva⁵, Rui Silva-Santos³, Isaac Braga⁶, Rui Henrique^{1,3,4},
Vera Miranda-Gonçalves^{1,4,†} and Carmen Jerónimo^{1,4,*}

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COST BURDEN STUDIES


Received: 23 April 2018 | Revised: 28 January 2019 | Accepted: 4 February 2019

DOI: 10.1111/icc.13026

ORIGINAL ARTICLE

WILEY European Journal of Cancer Care

Real-world treatment patterns, resource use and cost burden of multiple myeloma in Portugal

Luís Antunes¹  | Francisco Rocha-Gonçalves¹ | Sérgio Chacim¹ | Cinira Lefèvre² | Marta Pereira³ | Sónia Pereira⁴ | Aleksandra Zagorska² | Maria José Bento¹

Healthcare Resource Utilization in Treatment of Patients with Localized/Locally Advanced Prostate Cancer in a Portuguese Comprehensive Cancer Center

Isaac Braga¹, Salomé Gonçalves-Monteiro^{2,3}, Rita Calisto^{4,5}, Marta Rangel², Eduardo Medeiros², José Luís Cunha^{2,3}, Alina Rosinha¹, Ângelo Oliveira⁷, Maria José Bento^{4,5,8}, Ricardo Lopes³, Susana Santos³, Patrícia Redondo^{2, 8}

¹Urology Clinic, Portuguese Oncology Institute of Porto (IPO Porto) / Porto Comprehensive Cancer Centre (Porto.CCC) & RISC&CHPOP (Health Research Network), Porto, Portugal; ²Oncology Research Lab, Portuguese Oncology Institute of Porto (IPO Porto), Porto, Portugal; ³Management, Outcomes Research and Economics in Healthcare Group, IPO Porto Research Center (CHPOP), Portuguese Oncology Institute of Porto (IPO Porto) / Porto Comprehensive Cancer Centre (Porto.CCC) & RISC&CHPOP (Health Research Network), Porto, Portugal; ⁴Department of Epidemiology, Portuguese Oncology Institute of Porto (IPO Porto) / Porto Comprehensive Cancer Centre (Porto.CCC) & RISC&CHPOP (Health Research Network), Porto, Portugal; ⁵Cancer Epidemiology Group-Research Center, Portuguese Oncology Institute of Porto (IPO Porto) / Porto Comprehensive Cancer Centre (Porto.CCC) & RISC&CHPOP (Health Research Network), Porto, Portugal; ⁶Department of Clinical Epidemiology, Portuguese Oncology Institute of Porto (IPO Porto) / Porto Comprehensive Cancer Centre (Porto.CCC) & RISC&CHPOP (Health Research Network), Porto, Portugal; ⁷Tradition Oncology Department, Portuguese Oncology Institute of Porto (IPO Porto) / Porto Comprehensive Cancer Centre (Porto.CCC) & RISC&CHPOP (Health Research Network), Porto, Portugal; ⁸Univasa Portugal, Porto Salvo, Portugal

ISPOR Europe 2022 | 6-9 November – Poster HSD35

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METHODOLOGY STUDIES

[OEN: Multi-center, international, real-world evidence studies performed using health records without data pooling—The use of a common data model and shared analytical methods.](#)

Bethany Levick, Sue Cheeseman, Eun Ji Nam, Haewon Doh, Subin Lim, DongKyu Kim, Francois Bocquet, Elodie Martin, Paul Kubelac, Patriciu Achimaş-Cadariu, Rita Calisto, Marta Magalhães, Sven Becker, Andrea Wolf, Nicolas Niklas, Mariana Guergova-Kuras, and Geoff Hall

Journal of Clinical Oncology 2021 39:15_suppl, e13554-e13554

RIC – GERAÇÃO DE CONHECIMENTO BASEADO EM DADOS

EXTERNAL COMPARATOR STUDIES

The image shows the front cover of the journal 'Blood'. The top half of the cover is a solid red color. On the left side of the red band, there is a small graphic of red blood cells. In the center of the red band, the word 'blood' is written in a large, white, serif font. To the right of 'blood', the words 'Regular Article' are written in a smaller, white, sans-serif font. In the top right corner of the red band, there is a small white button with a magnifying glass icon and the text 'Check for updates'. Below the red band, the cover is white. The title 'Comparative effectiveness of ZUMA-5 (axi-cel) vs SCHOLAR-5 external control in relapsed/refractory follicular lymphoma' is written in a red, serif font. Below the title, the authors' names are listed in a small, black, sans-serif font. On the right side of the white area, there is a small, vertical, grey button with the text 'Download'.

GENE THERAPY

Comparative effectiveness of ZUMA-5 (axi-cel) vs SCHOLAR-5 external control in relapsed/refractory follicular lymphoma

Paola Ghione,^{1,2,*} M. Lia Palomba,^{2,*} Anik R. Patel,³ Sabela Bobillo,⁴ Kevin Deighton,⁵ Caron A. Jacobson,⁶ Myrna Nahas,³ Anthony J. Hatswell,⁵ A. Scott Jung,³ Steve Kanter,⁷ Julia Thornton Snider,³ Sattva S. Neelapu,⁸ Maria Teresa Ribeiro,⁹ M. Alan Brookhart,^{10,11} Herve Ghesquieres,¹² John Radford,¹³ and John G. Gribben¹⁴

Download

► PLoS One. 2022 Dec 9;17(12):e0278950. doi: 10.1371/journal.pone.0278950. eCollection 2022.

Costs, effectiveness, and safety associated with Chimeric Antigen Receptor (CAR) T-cell therapy: Results from a comprehensive cancer center

Sérgio Chacim ^{1 2}, Teresa Monjardino ³, José Luís Cunha ^{4 5}, Pedro Medeiros ^{4 5 6},
Patrícia Redondo ^{4 5}, Maria José Bento ^{3 7 8}, José Mário Mariz ¹

- Median total cost was 355.165€/patient
- CAR T cell drug expenses accounted for 97% of the overall cost
- Excluding CAR T- cell acquisition cost (344.498€), inpatient-care, diagnostic and therapeutic procedures and out patient-care contributed to 57%, 38% and 5% of the overall cost/patient, respectively.



FINANCIAMENTO | Fórmula para o Futuro

SAÚDE | uma Prioridade



FAROL

Institutions involved



Implementation of a funding model based on outcomes measurement and disease management for the lung cancer patients in IPO Porto.

VBHC

$$\text{Value} = \frac{\text{Outcomes}}{\text{Costs}}$$

3

Move to Bundled Payments for conditions

How we did it?

Roadmap for VBHC implementation

Define patient pathway

Pathway cost assessment

Measure treatment outcomes



Selection of
lung cancer as
a pathology to
analyze



Define the **care delivery value chain**



Develop **process maps** that
include each
activity in patient
care delivery



Obtain **time estimates** for each
procedure
(activity and
resource)



Estimate the **cost of supplying**
patient care
resources (direct
and indirect
resource)



Estimate each
resource's capacity –
to calculate **capacity cost rate**



Calculate total
cost of patient
care



Standard set
definition,
implementation
and analysis



Data collection,
compilation and
analysis



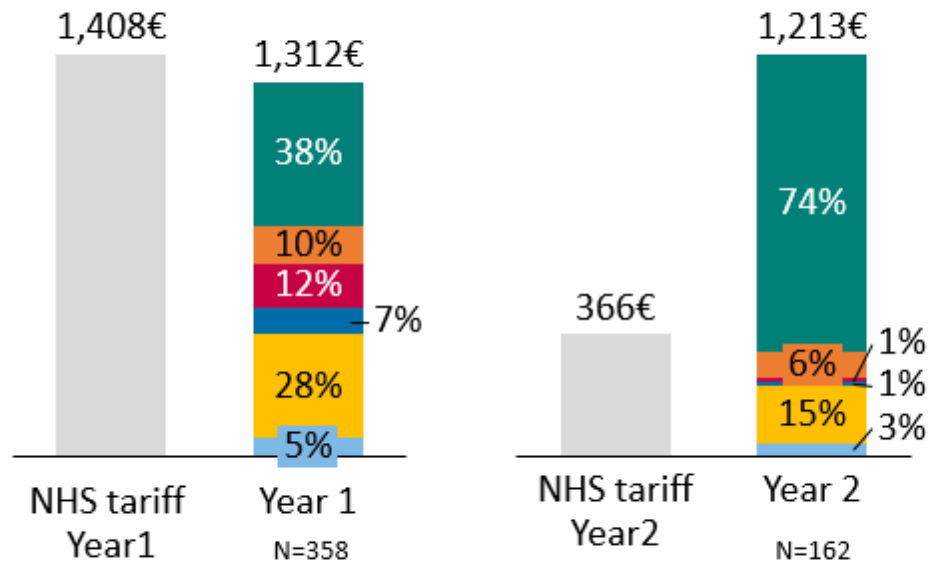
FAROL

Results

2. Pathway cost assessment

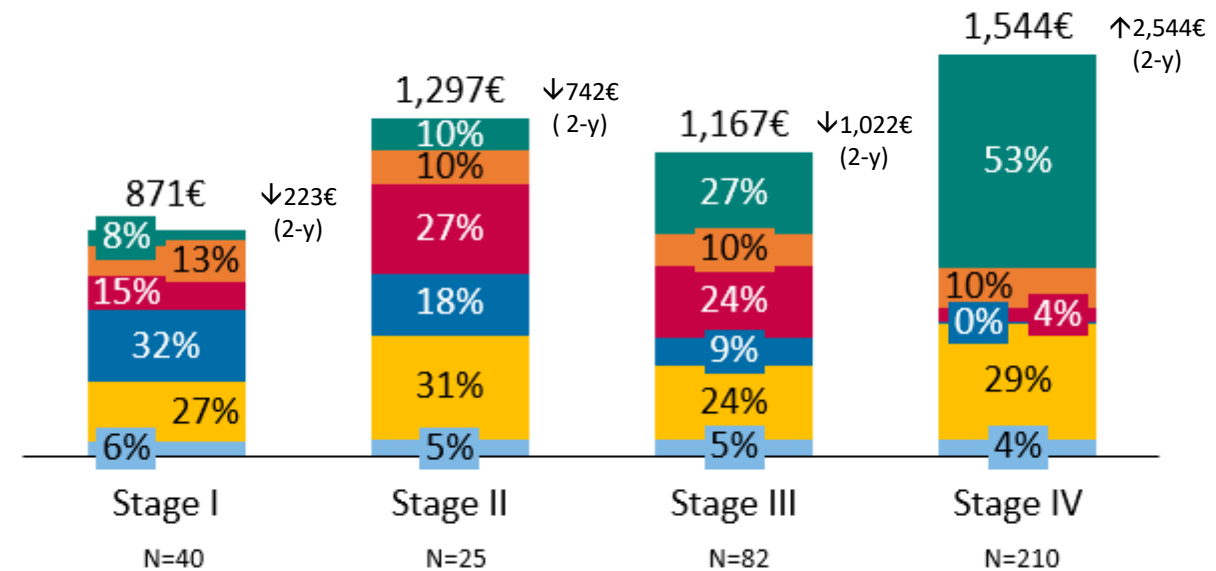
Monthly average cost and line distribution

(lung cancer, € per month, year 1 and 2)



Monthly average cost and line distribution, per disease stage

(lung cancer, € per month, year 1)



Pharmacological treatment Diagnostic tests Radiotherapy Surgery Hospitalizations Medical appointments



FAROL



ONCOVALUE

Implementing value-based oncology care at European cancer hospitals: An AI-based framework for assessing real-life effectiveness of novel cancer therapies in real-time

Grupo de Epidemiologia, Resultados, Economia e Gestão em Oncologia

<https://cordis.europa.eu/project/id/101095245>
<https://oncovalue.org>



The ONCOVALUE project at-a-glance

Next-generation tools and methods for the evaluation of real-life benefits of cancer therapies



Standardised **data collection and processing capabilities** for European cancer hospitals.



Next generation **AI-tools** for **real-time RWD delivery and evidence creation** based on structured and unstructured data.



Capacity building and training of health regulatory and HTA bodies for **RWE integration**.

Unique strengths

High-quality and comprehensive data pool of cancer patients



By bringing together the **clinical repositories of leading European cancer (research) centres** we create a high-quality clinical, quality of life, adverse events, and treatment costs data pool.

Collaboration between leading cancer institutes



The consortium is a unique collaboration between **several of the leading cancer institutes across Europe**, leading in the OEIC Accreditation and Designation Programme.

Contribution to the European Health data space



We contribute to the **European Health data space** (e.g., **DARWIN project**) by facilitating systematic structuring, extraction, and processing of RWD across European cancer centres.

Integration of the vision of HTA and regulatory bodies



We aim to actively integrate the vision of **European health regulatory and HTA bodies throughout the project** to support the implementation of a new EU legal framework on HTA

ONCOVALUE: The consortium



Early Phase Clinical Trials Unit

Mission & Objectives (start-up JAN2019)



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To promote **patients access to innovation** through clinical research

*To implement and develop a **molecular screening program** to expand the opportunities of biomarker driven clinical trial access*

To promote **scientific collaboration with academic/non-commercial partners, biotech and pharma companies**, focusing on translational research and early drug development

Clinical Trial Unit Coordinator:

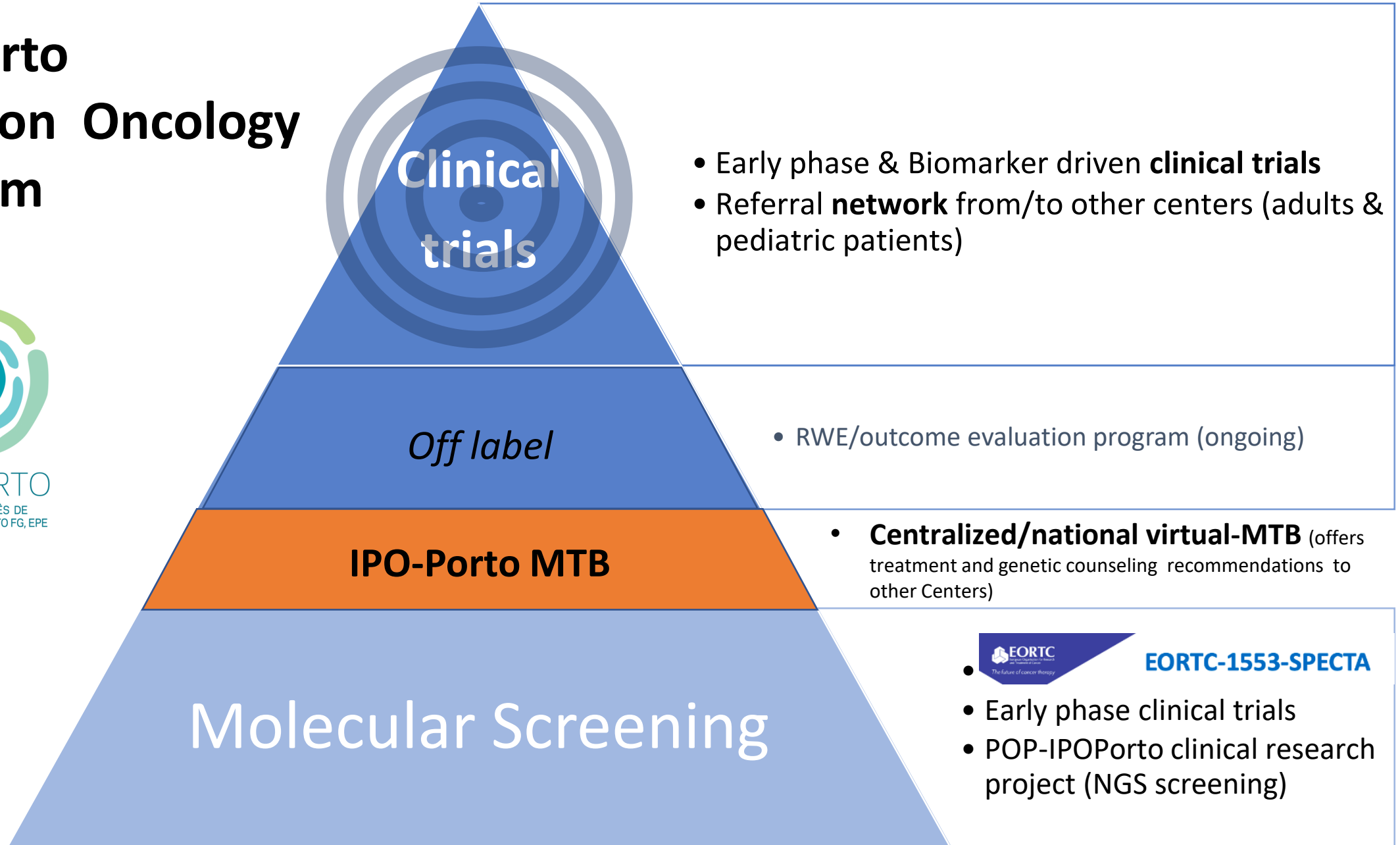
José Dinis, MD

Early Phase Clinical Trials Unit Coordinator: Júlio Oliveira, MD

IPO Porto Precision Oncology Program



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DLCTs- DRUP-Like Clinical Trials

- Family of independent, investigator-initiated, **pragmatic** clinical trials in precision medicine inspired by the design of the original DRUP protocol.

Phase II, multicentric, non-randomized, open-label clinical trials to assess the efficacy and safety of **off-label use of commercially available targeted anti-cancer drugs**



Letter | [Published: 30 September 2019](#)

The Drug Rediscovery protocol facilitates the expanded use of existing anticancer drugs

[D. L. van der Velden](#), [L. R. Hoes](#), [H. van der Wijngaart](#), [J. M. van Berge Henegouwen](#), [E. van Werkhoven](#), [P. Roepman](#), [R. L. Schilsky](#), [W. W. J. de Leng](#), [A. D. R. Huitema](#), [B. Nuijen](#), [P. M. Nederlof](#), [C. M. L. van Herpen](#), [D. J. A. de Groot](#), [L. A. Devriese](#), [A. Hoeben](#), [M. J. A. de Jonge](#), [M. Chalabi](#), [E. F. Smit](#), [A. J. de Langen](#), [N. Mehra](#), [M. Labots](#), [E. Kapiteijn](#), [S. Sleijfer](#), [E. Cuppen](#), ... [E. E. Voest](#)  [+ Show authors](#)

[Nature](#) 574, 127–131 (2019) | [Cite this article](#)



Test

Decide

Match



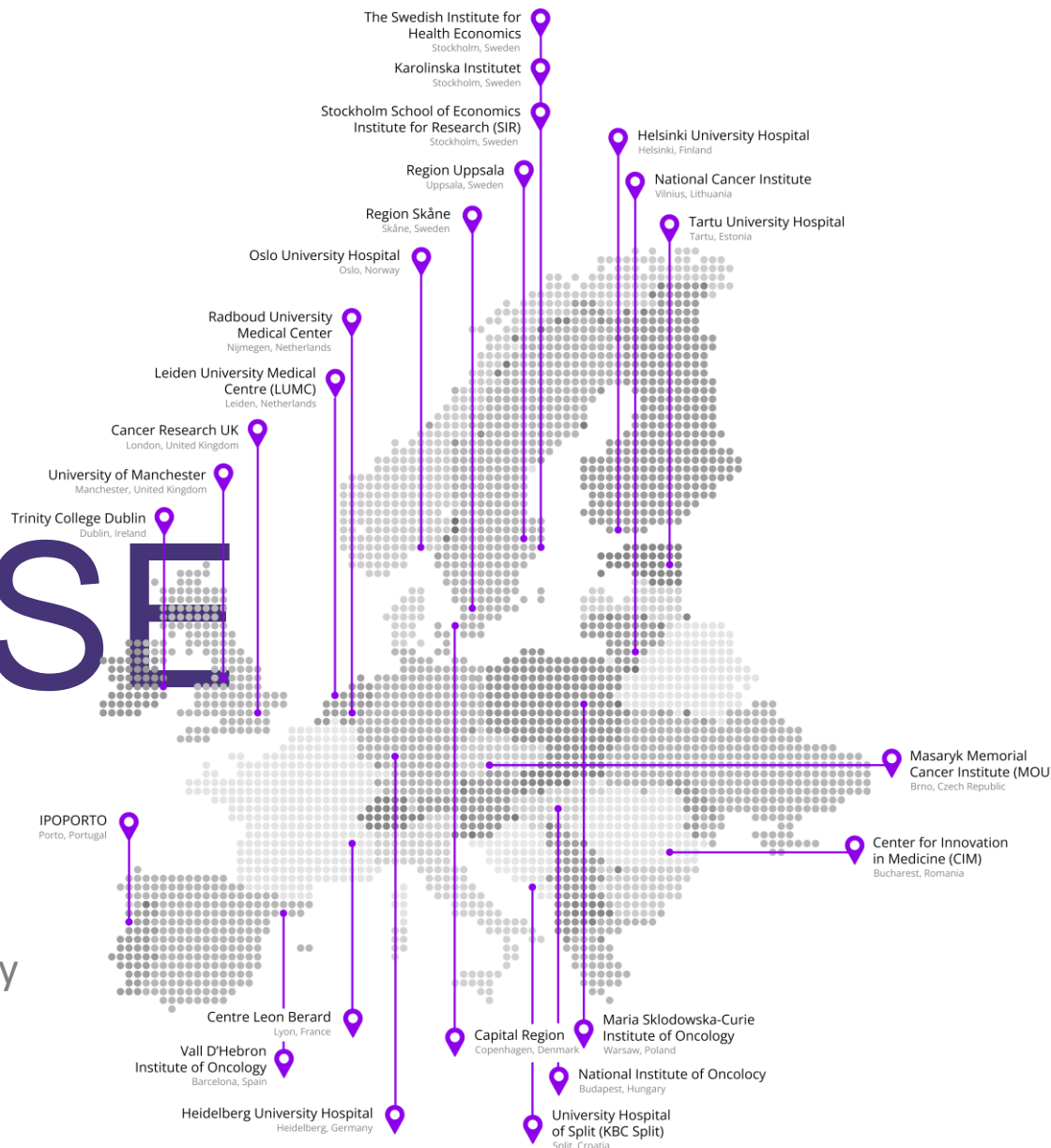
PRIME-ROSE

Precision Cancer Medicine Repurposing
System Using Pragmatic Clinical Trials

PRIME-ROSE

Precision Medicine Repurposing System
using pragmatic Clinical Trials

Kjetil Taskén, project coordinator, Oslo University Hospital, Norway



Funded by
the European Union

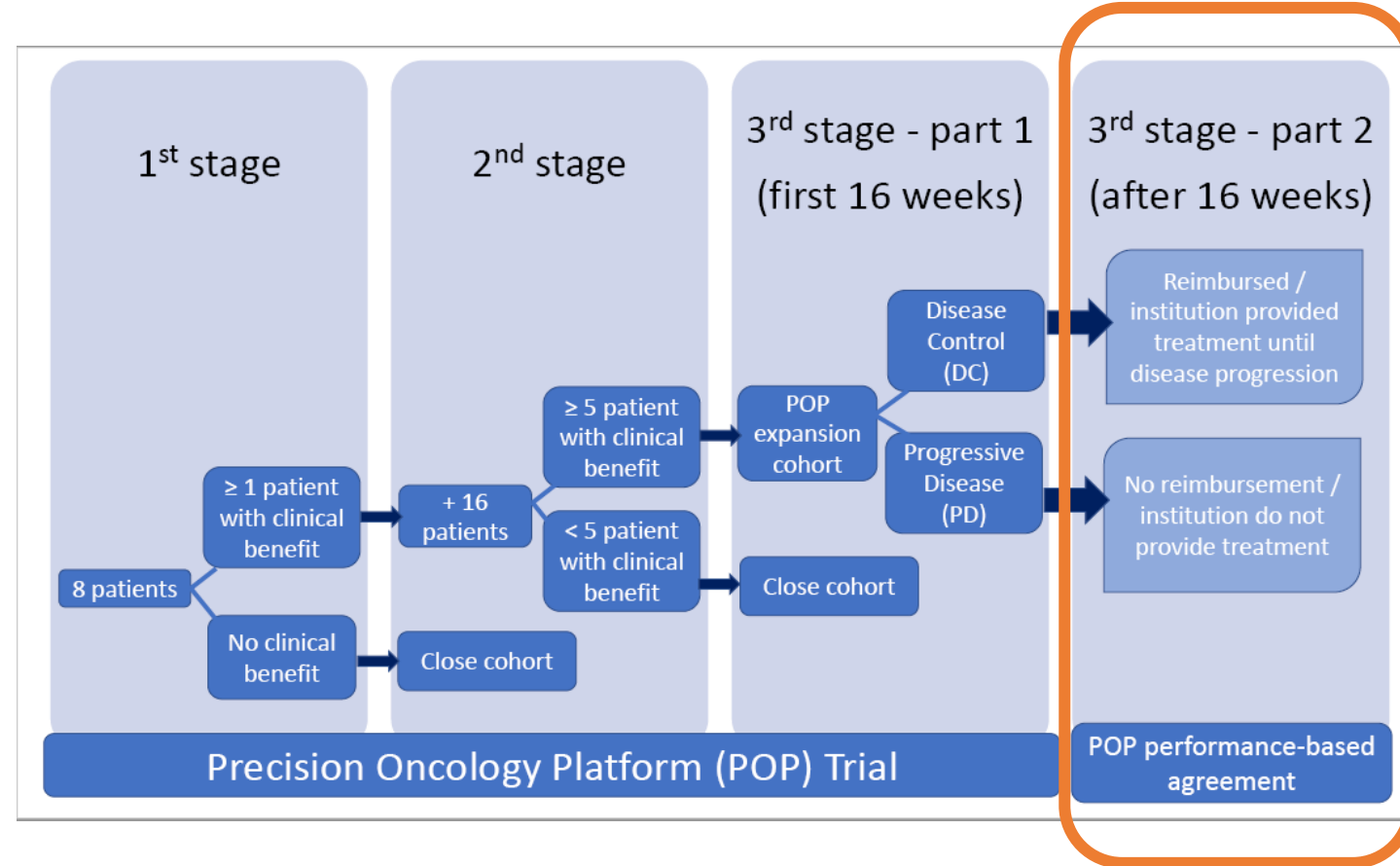
• HORIZON-MISS-2022-CANCER-01-03 Grant 101104269

Study concept POP- Precision Oncology Platform Trial (PT)

a “DRUP-like Protocol”



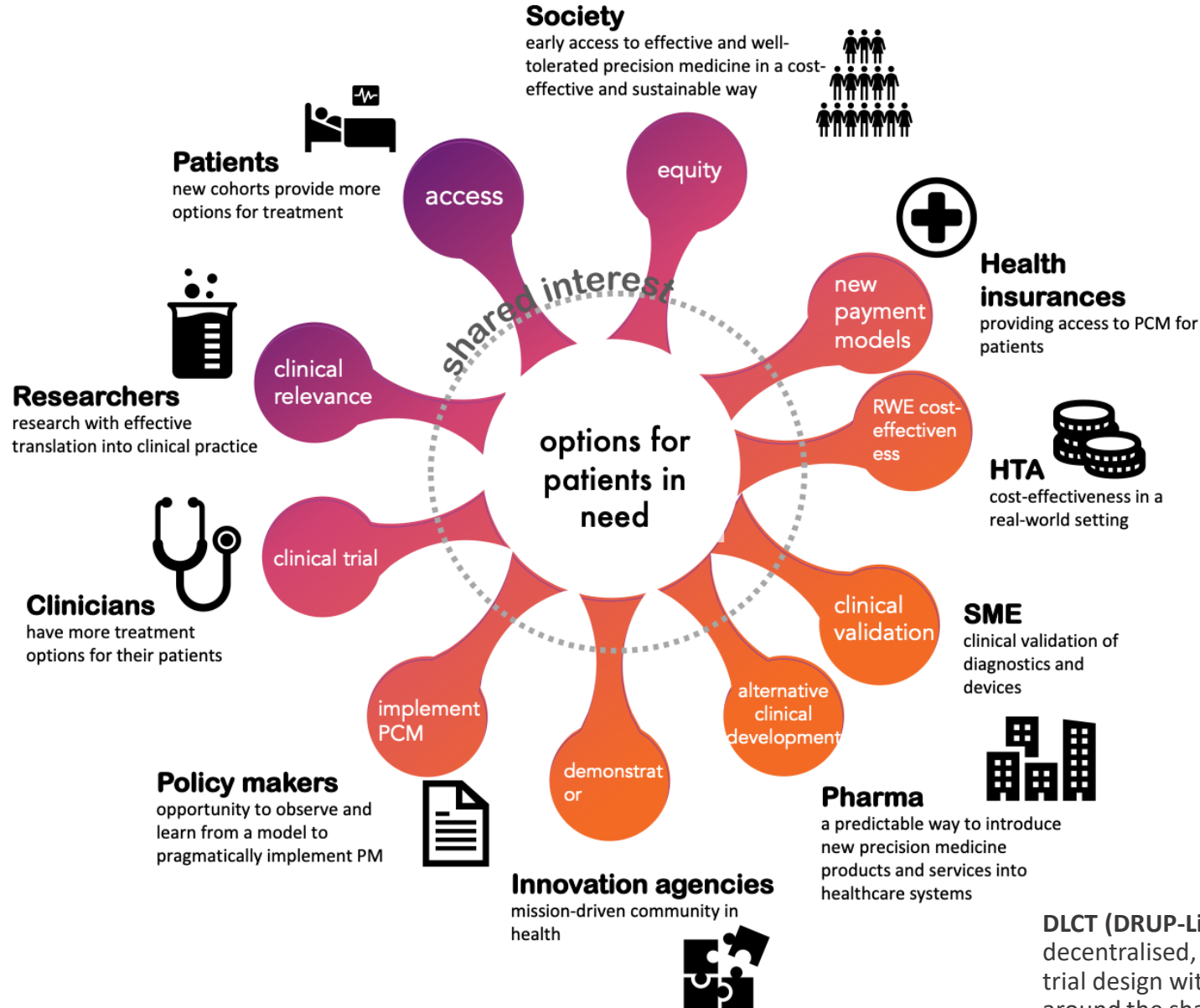
- Open-label, non-randomized clinical trial.
- Multi-center, single-country trial
- Investigator initiated-trial
- Study design is composed of **sequential stages**
- Patients will be enrolled in **multiple parallel cohorts**, each defined by one tumour type, one tumour profile and one study drug.
 - Each cohort will be monitored using a Simon-like two-stage ‘admissible’ monitoring plan to identify tumour/variant/drug cohorts with evidence of activity
 - Stage III is an **expansion cohort**, in which additional patients will be enrolled to confirm the results obtained in the first 24 patients.



Sponsor: IPO Porto

Funding: pharma and other private/public entities

A self-organising open innovation ecosystem





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O Presidente da República

25 SETEMBRO 2023

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