“Measuring Effectiveness"
1. **Theoretical Component:**
   - What is the National Oncology Registry (RON)?
   - What is the information collected?
   - What is the purpose of measuring effectiveness?
   - Methodologies used?
   - Comparators
   - Indicators and Outcomes to be measured
   - Particularities and difficulties in effectiveness monitoring in oncology
   - Importance of the RON for measuring effectiveness.
Overview of the presentation

1. Theoretical Component:
   • What is the National Oncology Registry (RON)?
   • What is the information collected?
   • What is the purpose of measuring effectiveness?
   • Methodologies used?
   • Comparators
   • Indicators and Outcomes to be measured
   • Particularities and difficulties in effectiveness monitoring in oncology
   • Importance of the RON for measuring effectiveness.

2. Practical cases

3. Discussion
Types of registries

Hospital-based registries

- Quick and easy access to clinical data. Data used primarily for administrative purposes and for monitoring performance indicators. Limitations for epidemiological purposes;

- **Advantages**: Greater exhaustiveness, homogeneity, completeness and more frequent updates;

- Useful for foreseeing human and material resources.

Population-based registries

- Enables statistics on the cancer cases (incidence, prevalence, survival). Data primarily used for epidemiological and public health purposes;

- **Advantages**: Knowledge of the population covered;

- Useful for supporting health policy decisions.
Background

Population-based registries in Portugal

Main Goals

1. To describe the **nature and extension of oncologic disease** and to support the definition of priorities in public health;
2. To act as information source for various **observational studies**;
3. To monitor and evaluate the efficacy of activities related to the control of oncologic diseases

2) Portaria nº 35/88, de 18 de Janeiro.
Innovations in the current law: practical functioning

Artigo 3.º
Registo Oncológico Nacional

1. É obrigatório o registo na plataforma eletrónica o RON de todos os novos casos de diagnóstico de cancro, por parte de todos os estabelecimentos e serviços de saúde do sector público, social e privado, independentemente da sua natureza jurídica, localizados no Continente ou nas regiões autónomas, no prazo máximo de nove meses contado da data do conhecimento do diagnóstico, e a posterior atualização, no mínimo anual, do estádio da doença oncológica, das terapêuticas oncológicas usadas e do estado vital do doente.

2. Os dados existentes nos Registos Oncológicos Regionais (ROR) são integrados no RON.

3. Os dados do denominado registo oncológico diário português são integrados no RON.

4. Os dados dos registos das regiões autónomas são integrados no RON, sem prejuízo das competências próprias daquelas regiões na matéria.

Artigo 17.º
Financiamento e incentivos

1. No âmbito do processo de contratação de serviços de cuidados de saúde que se encontra implementado no Serviço Nacional de Saúde (SNS) e nos serviços regionais de saúde, são introduzidos mecanismos de incentivo e penalização associados a uma adequada prática de registo oncológico nos termos do disposto na presente lei.

2. Para efeitos do número anterior, no âmbito dos contratos-programa a celebrar pela Administração Central do Sistema de Saúde, I. P. (ACSS, I. P.), com os hospitais, os centros hospitalares e as unidades locais de saúde integradas no SNS e, nas regiões autónomas, entre os serviços regionais de saúde e as entidades públicas prestadoras de cuidados de saúde, as modalidades de pagamento às instituições contemplam o registo do RON na atividade realizada.

3. Os custos relacionados com a administração do RON, em matéria de prestação de serviços relativos a sistemas de informação e comunicação, são suportados pela ACSS, I. P., no âmbito do contrato-programa anual celebrado entre este instituto público e a SPMS — Serviços Partilhados do Ministério da Saúde, E. P. E.
Overview of the presentation

1. Theoretical Component:
   • What is the National Oncology Register (RON)?
   • What is the information collected?
   • What is the purpose of measuring effectiveness?
   • Methodologies used?
   • Comparators
   • Indicators and Outcomes to be measured
   • Particularities and difficulties in effectiveness monitoring in oncology
   • Importance of the RON for measuring effectiveness.

2. Practical cases

3. Discussion
Information sources linked to RON

- Electronic clinical records
- SICO (death certificates)
- RNU (patients registry)
- Other
How is our database organized?

- Institution registering the case
- Identification of the citizen
- Identification of the district of residence at diagnosis
How is our database organized?

<table>
<thead>
<tr>
<th>Identificação</th>
<th>Diagnóstico</th>
<th>Tumor</th>
<th>Tratamento</th>
<th>Estado</th>
</tr>
</thead>
<tbody>
<tr>
<td>Data Início Sintomas</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Data 1ª Observação</td>
<td>25 3 2014</td>
<td></td>
<td></td>
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</tr>
<tr>
<td>Data 1ª Consulta</td>
<td>2 4 2014</td>
<td></td>
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<tr>
<td>Data C.D.T.</td>
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<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Data Pat. Morf.</td>
<td>8 4 2014</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Data Outros Exames</td>
<td>3 4 2014</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Distrito</td>
<td>08</td>
<td>FARO</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Concelho</td>
<td>11</td>
<td>PORTIMÃO</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Freguesia</td>
<td>03</td>
<td>PORTIMÃO</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Diagnóstico na instituição:</td>
<td>Sim</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

- Date when symptoms started
- Date of first appointment at the institution
- Date of therapeutic decision
- Date of anatomical-pathological evaluation

<table>
<thead>
<tr>
<th>Tipo Exame</th>
<th>Data</th>
<th>Instituição</th>
<th>Tipo Registo</th>
<th>Editar</th>
<th>Remover</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hemoglobina (g/dl)</td>
<td>3-4-2014</td>
<td>HOSPITAL DE FARO</td>
<td>Processo</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Proteína C Reactiva _ PCR</td>
<td>3-4-2014</td>
<td>HOSPITAL DE FARO</td>
<td>Processo</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Albumina (g/dl)</td>
<td>3-4-2014</td>
<td>HOSPITAL DE FARO</td>
<td>Processo</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Creatinina</td>
<td>3-4-2014</td>
<td>HOSPITAL DE FARO</td>
<td>Processo</td>
<td></td>
<td></td>
</tr>
<tr>
<td>T. Bioquímica/T. Imunologia</td>
<td>3-4-2014</td>
<td>HOSPITAL DE FARO</td>
<td>Processo</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Cálcio (Normal/Elevada)</td>
<td>3-4-2014</td>
<td>HOSPITAL DE FARO</td>
<td>Processo</td>
<td></td>
<td></td>
</tr>
<tr>
<td>82 Microglobulina (Normal/Elevada)</td>
<td>3-4-2014</td>
<td>HOSPITAL DE FARO</td>
<td>Processo</td>
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<td></td>
</tr>
<tr>
<td>TAC</td>
<td>7-4-2014</td>
<td>HOSPITAL DE FARO</td>
<td>Processo</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Histologia Primário (NP)</td>
<td>8-4-2014</td>
<td>HOSPITAL DE FARO</td>
<td>Processo</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

- Imagiological and anatomo-pathological evaluation
- Analytic parameters at diagnosis
How is our database organized?

- Site/location of the neoplasm
- Specific histologic description of the neoplasm
- Parameters for staging and prognosis

International Codification of Diseases for Oncology (ICD-O) 3rd edition 1st revision
How is our database organized?

<table>
<thead>
<tr>
<th>Identificação</th>
<th>Diagnóstico</th>
<th>Tumor</th>
<th>Tratamento</th>
<th>Estado</th>
</tr>
</thead>
<tbody>
<tr>
<td>Tratamento Inicial</td>
<td>Tratamento Com Actividade Antitumoral</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Vigilância**
- Sim
- Não

**Tipo Tratamento** | **Data** | **Instituição** | **Tipo Registo** | **Editar Remover**
--- | --- | --- | --- | ---
- Radioterapia | 21-05-2014 | QUADRANTES | Processo |
- Bifosfonatos | 27-05-2014 | Hospital de Faro | Processo |
- Tratamento Sistémico | 27-05-2014 | Hospital de Faro | Processo |
- Transplante | 31-12-2014 | Hospital de Faro | Processo |

**Data de Início** | **26 05 2014**
**Data de Fim** | **30 11 2014**

**Regime de acesso** | **Convencional**
**Linha Terapêutica** | **Primeira Linha**

**Regime / DCI** | **MPT (Melfalan, Talidomida, Prednisolona)**
**Fármaco 1** | **Prednisolona**
**Dose 1** | **30 mg**
**Nº Ciclos/Admin.** | **6**
**Descontinuação** |  
**Instituição** | **117020 CENTRO HOSPITALAR DE LISBOA CENTRAL, EPE**

**Verificar resposta**
- Tratamento na instituição
- Estado Após Tratamento
- Data de Remissão

**Therapeutic response**
How is our database organized?

Identificação | Diagnóstico | Tumor | Tratamento | Estado
---|---|---|---|---
Tratamento Inicial | Tratamento Com Actividade Antitumoral

Data de Início | 01 01 2014
Data de Fim | 05 05 2014
Regime de acesso | Convencional
Linha Terapêutica | Segunda Linha
Regime / DCI | Pembrolizumab
Dose 1 | 200 mg
Trat. Manutenção |
Nº Cíclos/Admin. | 10
Descontinuação |
Instituição | 117020 CENTRO HOSPITALAR DE LISBOA CENTRAL, EPE

Progressão da Doença
Morte
Recusa
Outra
Reação Adversa ao Medicamento
Low Level Terms, The Medical Dictionary for Regulatory Activities (MedDRA);
National Cancer Institute Common Terminology Criteria for Adverse Events (CTCAE)

How is our database organized?
How is our database organized?

Epidemiological and pharmacoepidemiological studies
Overview of the presentation

1. Theoretical Component:
   - What is the National Oncology Register (RON)?
   - What is the information collected?
   - **What is the purpose of measuring effectiveness?**
   - Methodologies used?
   - Comparators
   - Indicators and Outcomes to be measured
   - Particularities and difficulties in effectiveness monitoring in oncology
   - Importance of the RON for measuring effectiveness.

2. Practical cases

3. Discussion
The efficacy-effectiveness gap


Artigo 4.º
Recolha de dados

1 — Os dados recolhidos para tratamento no RON são os seguintes:

a) A identificação do nome, do sexo, da data de nascimento, da morada, do número de utente, da identificação da instituição, do número de processo clínico, da profissão e da naturalidade do doente;

b) A data e os resultados dos exames efetuados, para diagnóstico e estadiamento, que sejam relevantes para a história clínica;

c) A identificação do código da Classificação Internacional da Doença (CID), na versão em vigor à data do registo no RON, correspondente à neoplasia diagnosticada;

d) A caracterização da neoplasia, não limitada à localização primária, morfologia, estadiamento, recetores, marcadores moleculares e marcadores tumorais, os dados relativos ao diagnóstico e ao estudo genético da neoplasia, quando aplicável;

f) A data do diagnóstico e do início do tratamento, bem como das várias modalidades de tratamento, como cirurgia, radioterapia e quimioterapia;

g) A caracterização de cada linha de tratamento;

h) O registo anual do estado geral do doente, estado da neoplasia e as suas modificações, incluindo as dependentes dos tratamentos, e a melhor resposta obtida da neoplasia no fim de cada linha de tratamento;

i) A data de óbito e a causa de morte.

Artigo 5.º
Monitorização da efetividade terapêutica

1 — Para os efeitos previstos no n.º 2 do artigo 4.º do Decreto-Lei n.º 97/2015, de 1 de junho, no que se refere à recolha de dados necessários à monitorização de efetividade da utilização de medicamentos e dispositivos médicos, podem ser ainda recolhidos dados para quantificação dos diferentes parâmetros de avaliação de resultados da utilização na prática clínica não experimental.

2 — Os registos de dados de monitorização da efetividade terapêutica devem ser efetuados no prazo indicado pelo INFARMED, I. P., para cada tipo de situação.
1. Theoretical Component:
   - What is the National Oncology Register (RON)?
   - What is the information collected?
   - What is the purpose of measuring effectiveness?
   - Methodologies used?
   - Comparators
   - Indicators and Outcomes to be measured
   - Particularities and difficulties in effectiveness monitoring in oncology
   - Importance of the RON for measuring effectiveness.

2. Practical cases

3. Discussion
Methods for measuring therapeutic effectiveness

**Option 1**

**Abstract**

The goal of the present study was to compare the outcomes of new generation tyrosine kinase inhibitors (NG-TKIs) versus imatinib in patients with newly diagnosed chronic phase chronic myeloid leukemia and to assess the effect of the risk scores on the treatment response. NG-TKIs resulted in a greater major molecular response, and the degree of benefit from NG-TKIs on the complete cytogenetic response and major molecular response was equivalent across the risk groups.

**Background:** BCR-ABL1 tyrosine kinase inhibitors (TKIs) have significantly improved the survival outcomes for patients with chronic myeloid leukemia (CML). In addition to imatinib, 3 newer generation TKIs (NG-TKIs) have been approved as first-line treatment of chronic phase (CP)-CML. These have been preferably used in patients with CP-CML with a high Sokal or Hasford risk score. We performed a systematic review and meta-analysis to compare the outcomes with NG-TKIs as a category versus imatinib in patients with newly diagnosed CP-CML and to indirectly compare the efficacy of NG-TKIs among each other. Furthermore, we assessed the effect of the risk scores on the complete cytogenetic response (CCyR) and major molecular response (MMR). **Materials and Methods:** The eligible studies were limited to randomized controlled trials comparing the efficacy of first-line treatment using NG-TKIs versus imatinib in adult patients (aged ≥ 18 years) with CP-CML. **Results:** The differences in the CCyR, progression-free survival, and overall survival between the NG-TKIs and imatinib were not statistically significant. NG-TKI-treated patients showed a significantly greater likelihood of MMR (relative risk [RR], 0.76; 95% confidence interval, 0.63-0.91; P = .003) and lower likelihood of progression to an accelerated phase/blast crisis (RR, 0.37; 95% confidence interval, 0.20-0.67; P = .001) than did imatinib-treated patients. Nilotinib, dasatinib, and radotinib showed significantly greater CCyR rates compared with bosutinib and ponatinib. All risk groups showed statistically equivalent benefits from NG-TKIs for the CCyR and MMR. **Conclusion:** In first-line treatment, the NG-TKIs as a category showed greater effectiveness in MMR and prevention of accelerated phase/blast crisis progression. Risk stratification was not found to affect the RR of CCyR and MMR.
Efficacy is the extent to which an intervention does more good than harm under ideal circumstances.

Effectiveness assesses whether an intervention does more good than harm when provided under usual circumstances of healthcare practice.

Objective: The optimal sequencing of targeted therapies for metastatic renal cell carcinoma (mRCC) is unknown. Observational studies with a variety of designs have reported differing results. The objective of this study is to systematically summarize and interpret the published real-world evidence comparing sequential treatment for mRCC.

Methods: A search was conducted in Medline and Embase (2009–2013), and conference proceedings from American Society of Clinical Oncology (ASCO), ASCO Genitourinary Cancers Symposium (ASCO-GU), and European Society for Medical Oncology (ESMO) (2011–2013). We systematically reviewed observational studies comparing second-line mRCC treatment with mammalian target of rapamycin inhibitors (mTORi) versus vascular endothelial growth factor (VEGF) tyrosine kinase inhibitors (TKI). Studies were evaluated for 1) use of a retrospective cohort design after initiation of second-line therapy, 2) adjustment for patient characteristics, and 3) use of data from multiple centers. Meta-analyses were conducted for comparisons of overall survival (OS) and progression-free survival (PFS).

Results: Ten studies reported OS and exhibited significant heterogeneity in estimated second-line treatment effects ($I^2=68\%$; $P=0.001$). Four of these were adjusted, multicenter, retrospective cohort studies, and these showed no evidence...
Ex post evaluations are used throughout the European Commission to assess whether a specific intervention was justified and whether it worked (or is working) as expected in achieving its objectives and why.
Overview of the presentation

1. Theoretical Component:
   - What is the National Oncology Register (RON)?
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2. Practical cases

3. Discussion
1. Theoretical Component:
   • What is the National Oncology Register (RON)?
   • What is the information collected?
   • What is the purpose of measuring effectiveness?
   • Methodologies used?
   • Comparators
   • **Indicators and Outcomes to be measured**
     • Particularities and difficulties in effectiveness monitoring in oncology
     • Importance of the RON for measuring effectiveness.

2. Practical cases

3. Discussion
## Outcomes

<table>
<thead>
<tr>
<th>Outcome</th>
<th>Abr.</th>
<th>Definition</th>
<th>Variables needed</th>
</tr>
</thead>
<tbody>
<tr>
<td>Global survival</td>
<td>OS</td>
<td>Time from diagnosis until death from any cause</td>
<td>Date of diagnosis</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Date of death</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Date of last known contact</td>
</tr>
<tr>
<td>Overall survival</td>
<td>OS</td>
<td>Time from treatment initiation until death from any cause</td>
<td>Date of treatment initiation</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Date of death</td>
</tr>
<tr>
<td></td>
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<td></td>
<td>Date of last known contact</td>
</tr>
<tr>
<td>Progression-free survival</td>
<td>PFS</td>
<td>Time from treatment initiation until disease progression or death</td>
<td>Date of treatment initiation</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Date of disease progression</td>
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<td>Date of death</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Date of last known contact</td>
</tr>
<tr>
<td>Objective Response Rate</td>
<td>ORR</td>
<td>Proportion of patients with reduction in tumor burden of a predefined amount</td>
<td>Therapeutic response</td>
</tr>
</tbody>
</table>
Overview of the presentation

1. Theoretical Component:
   • What is the National Oncology Register (RON)?
   • What is the information collected?
   • What is the purpose of measuring effectiveness?
   • Methodologies used?
   • Comparators
   • Indicators and Outcomes to be measured
   • **Particularities and difficulties in effectiveness monitoring in oncology**
   • Importance of the RON for measuring effectiveness.

2. Practical cases

3. Discussion
1. Theoretical Component:
   • What is the National Oncology Register (RON)?
   • What is the information collected?
   • What is the purpose of measuring effectiveness?
   • Methodologies used?
   • Comparators
   • Indicators and Outcomes to be measured
   • Particularities and difficulties in effectiveness monitoring in oncology
   • Importance of the RON for measuring effectiveness

2. Practical cases

3. Discussion
Examples of studies undertaken

Median **Overall survival** among exposed patients was **11.4 months**, slightly inferior to that reported in published clinical trials (**12.2 months**). However, the characteristics of patients in our sample indicate they had a **worse prognosis**.

Examples of studies undertaken

- NSCLC
  - Male: 85.5%
  - Female: 60.0%
  - Stage IV: 63.6%
  - Stage III: 36.4%

- Melanoma
  - Male: 40.0%
  - Female: 60.0%
  - Stage IV: 40.0%
  - Stage III: 89.3%

- Urothelial
  - Male: 12.5%
  - Female: 12.5%
  - Stage IV: 12.5%
  - Stage III: 87.5%

- Off label
  - Male: 66.7%
  - Female: 33.3%
  - Stage IV: 7.3%
  - Stage III: 87.5%
Overview of the presentation

1. Theoretical Component:
   • What is the National Oncology Register (RON)?
   • What is the information collected?
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   • Methodologies used?
   • Comparators
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   • Particularities and difficulties in effectiveness monitoring in oncology
   • Importance of the RON for measuring effectiveness

2. Practical cases

3. Discussion
SITUATION BEFORE RON PORTAL

- DATA

  - Poor Quality (accuracy and completeness)
  - Not Updated (low accessibility and sharing between agents)

Not enough contribute to clinical practice and knowledge progress
NEW RON PORTAL – Main Goals

TO INNOVATE

- Innovate Methodology on Registry Philosophy
- Innovate Technologically the existing Regional Computerized Registry Platform (dated from the 1990’s)

IMPROVE

- To create an Information Support System to Cancer Health Care Management, to Every Day Clinical Practice and to Cancer Epidemiological Research
  > Improve Information Sharing
  > Improve Interaction and Communication between agents
Practical cases

NEW RON PORTAL – Innovating Registry Methodology

On The Old Registry Method: Data Source oriented

- IPO Case A
- Portimão Case A

Database
Duplicated Registries
Several Registries to a same case
Practical cases

NEW RON PORTAL – Innovating Registry Methodology

On The New RON Portal: Tumor and Patient oriented

- IPO Case A
- Portimão Updates Case A

Database
No Duplicated Registries
One single record being updated
NEW RON PORTAL –

Improving Information Sharing

Cancer Registry Data Base built by integration of multiple local Data Bases

Better Data Quality

More Updated Information

Bigger Functionality and Use

Quicker Information Access

Optimized Communication between Agents
NEW RON PORTAL –

Improving Information Sharing

Cancer Registry Data Base built by integration of multiple local Data Bases

Better Data Quality

More Updated Information

Bigger Functionality and Use

Quicker Information Access

Optimized Communication between Agents
Practical cases

PORTAL RON - http://ron.min-saude.pt

First Step – Create an Account

Second Step - Log In

Click on Registry

You can:

- Search and consult on RON and on RNU Data Base, by patient, by case or by institution;

- Edit, change, update and delete cases on RON Data Base
Practical cases

Data “manager” cancer registry profile:

- Medical doctors
- Biologists
- Pharmaceuticals
- Psychologists
- Social workers
- Administrative staff

- According to different data access
- Signed confidentiality document
Practical cases

Indicators- developed in conjunction with different actors

- New cases - Incidence
- Time between diagnosis / treatment
- Stage
- Global Survival
- Survival till progression

- Hospital/Population based
Data availability

- National Health Administration
- National Statistics Institute
- Regional Health Administration
- Hospital Administration
- Mutidisciplinary teams and Units
- Specific Population/Hospital Based Studies Researchers
Thank You for the invitation and for Your attention