MANHÃS INFORMATIVAS

Utilização de dados em saúde no apoio à decisão

Modo Híbrido

10 de maio de 2022, Auditório INFARMED, I.P.

A utilização de dados em saúde: a experiência do IPO Porto

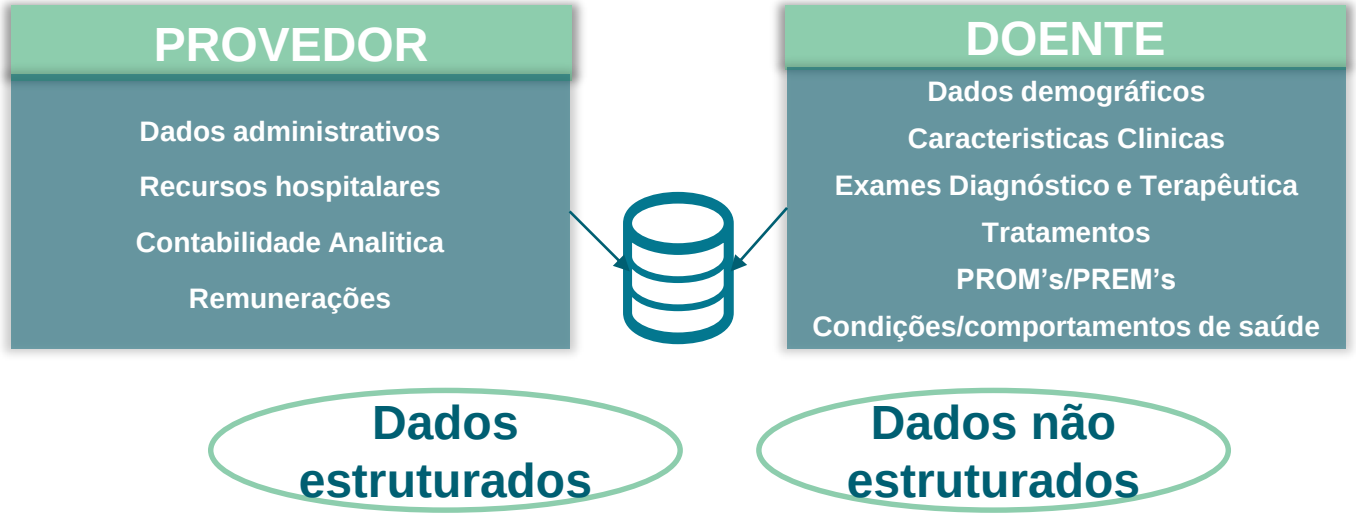
Maria José Bento

Serviço de Epidemiologia/Registo Oncológico

Experiência do IPO do Porto

- Estrutura orgânica e cultura da organização
- Registo Oncológico
- Marcos tecnológicos
- Serviços envolvidos
  - Serviço de Epidemiologia/Grupo de Investigação em Epidemiologia do Cancro do CI-IPOP
  - Serviço de Planeamento e Apoio à Gestão/Grupo de Investigação em Gestão, Resultados e Economia em Cuidados de Saúde do CI-IPOP
  - Outcomes Research Lab
  - Clínicas de Patologia – Investigador principal

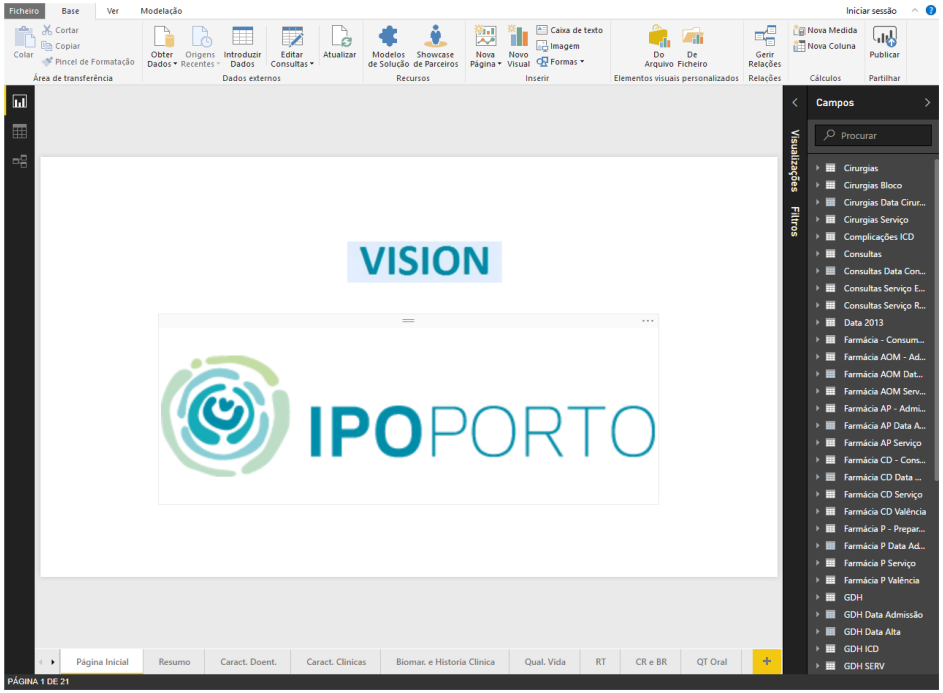
A utilização de dados em saúde: a experiência do IPO Porto

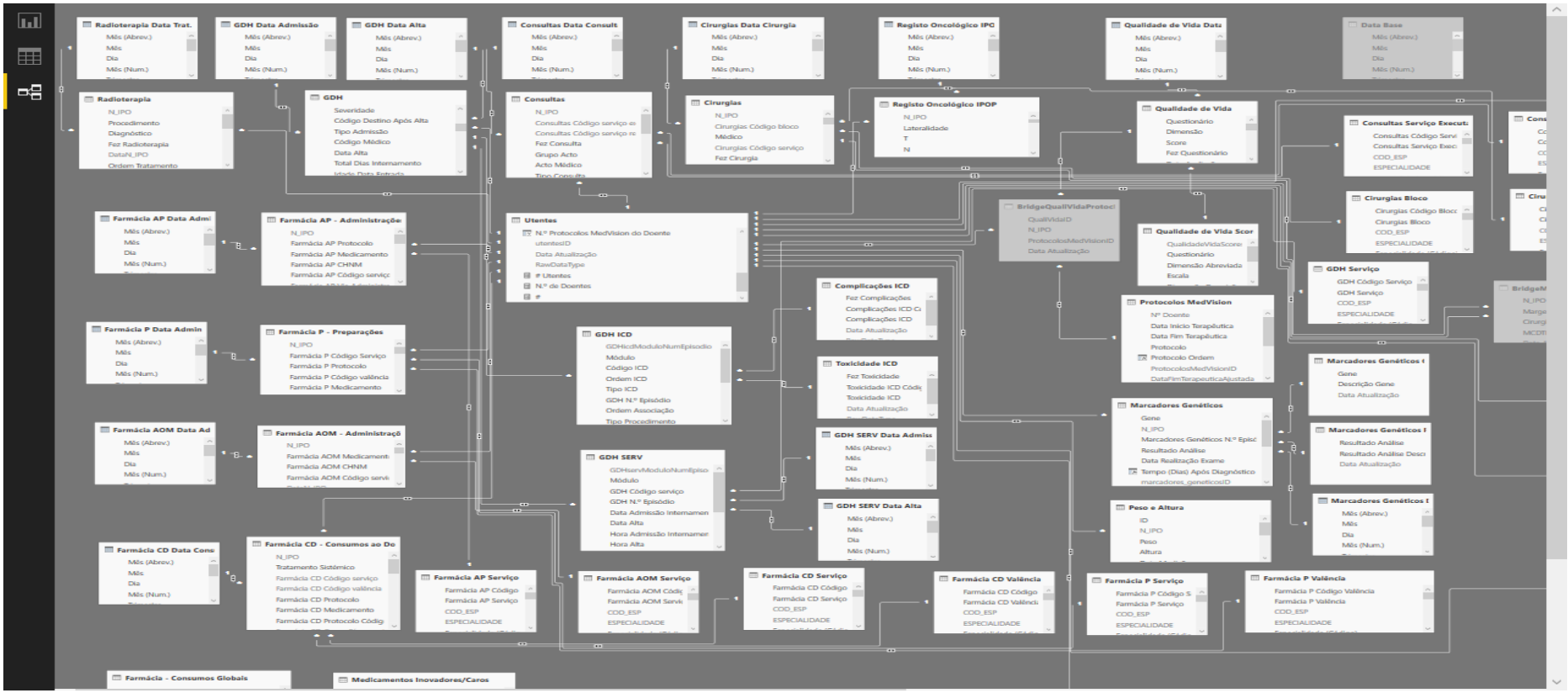


- Que dados é que a minha Instituição tem disponíveis?
- O que se está atualmente a recolher?
- Como integrar os dados?
- Como armazenar grande volume dados?
- Que análises vamos querer fazer?

Vision

- Base de dados que agrega informação clínica e administrativa proveniente dos diversos sistemas de informação existentes no IPO Porto;
- Visão integrada do percurso do doente (360º);
- Mais de 500 variáveis selecionadas
- Responder rapidamente a questões práticas da gestão e da tomada de decisão.




Radioterapia Data Trat.
GDH Data Admissão
GDH Data Alta
Consultas Data Consult
Cirurgias Data Cirurgia
Registo Oncológico IPO
Qualidade de Vida Data
Data Base
Radioterapia
GDH
Consultas
Cirurgias
Registo Oncológico IPOP
Qualidade de Vida
Consultas Serviço Execut
Cirurgias Bloco
Farmácia AP Data Admi
Farmácia AP - Administração
Utentes
BridgeQualiVidaProtocl
Qualidade de Vida Scor
GDH Serviço
Farmácia P Data Admin
Farmácia P - Preparações
Complicações ICD
Protocolos MedVision
Farmácia AOM Data Ad
Farmácia AOM - Administraçã
GDH ICD
Toxicidade ICD
Marcadores Genéticos
Marcadores Genéticos
GDH SERV
GDH SERV Data Admiss
GDH SERV Data Alta
Peso e Altura
Farmácia CD Data Cons
Farmácia CD - Consumos ao Do
Farmácia AP Serviço
Farmácia AOM Serviço
Farmácia CD Serviço
Farmácia CD Valência
Farmácia P Serviço
Farmácia P Valência
Farmácia - Consumos Globais
Medicamentos Inovadores/Caros

MedVision

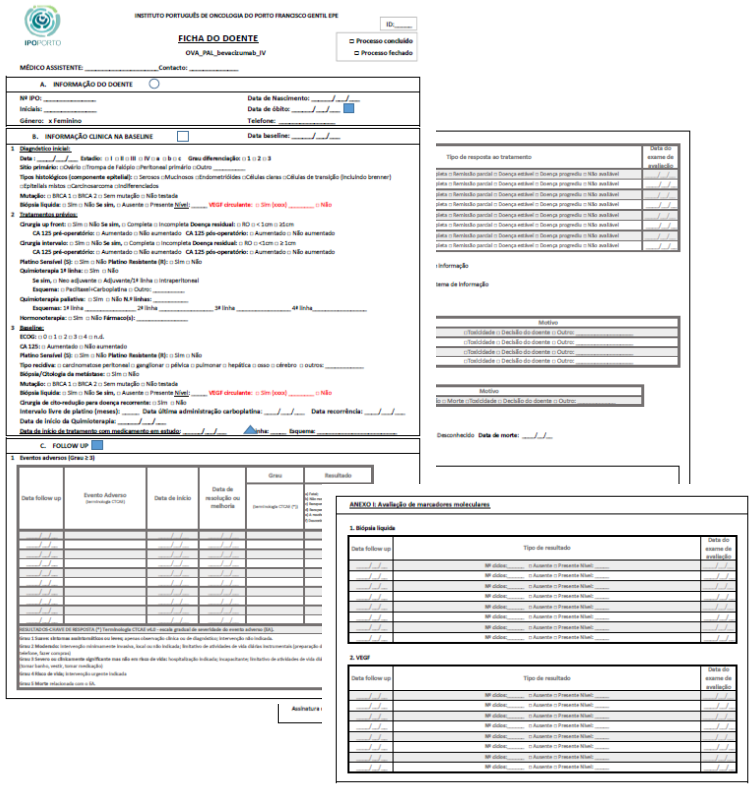
- Acompanhamento de estudos observacionais e gestão da despesa com **medicamentos inovadores**;
- Necessidade de sistematizar a informação relativa ao custo, segurança e efetividade das **tecnologias da saúde inovadoras** utilizadas no IPO Porto;
- Ferramenta informática agregadora de informação clinica e de farmácia hospitalar;
- Visão holística do tratamento prescrito ao doente;
- Geração de *outcomes* no âmbito de avaliação de tecnologias de saúde (sobrevivência, segurança, qualidade de vida e custo do tratamento).

Medicamentos inovadores

Ponto de Partida:

Protocolo de Investigação
Investigador Principal Médico

- **Ficha do doente**
  - Baseline (dados demográficos e informação clínica)
  - Eventos Adversos => Segurança
  - Resposta ao Tratamento => Efetividade
  - Qualidade de Vida (PROM) => Efetividade
  - Efetivação do Tratamento => Custo
  - Suspensão do Tratamento => Efetividade/Segurança
  - Óbito -> Efetividade/Segurança



PROM’s no IPO Porto – Gabinete QdV

- Avaliar a qualidade de vida (QdV) dos doentes durante o seu percurso de tratamento, de modo a poder intervir-se atempadamente e de forma eficiente na prestação de melhores cuidados de saúde, melhorando o bem-estar do doente.
- Implementação de um Gabinete em Out/20.
- 73% taxa de adesão.
- Gabinete informatizado em que os doentes são convidados a responder a um questionário QdV
- 1ªfase: apenas fármacos inovadores.
- Software amigável, adequado para recolha e armazenamento de dados em grande escala e disponibilização de relatórios.
- Questionários validados internacionalmente.
- Apoio de um profissional de enfermagem como monitor de qualidade.

Circuito de avaliação de propostas dos estudos observacionais:

- Proposta de estudo
  - Entidade externa
  - Investigador interno
- “Feasibility” e constituição da equipa
- Se houver acordo
  - Submissão do protocolo, (dispensa) consentimento informado, AIPD (previamente avaliado pelo EPD) à Comissão de Ética para a Saúde
  - Contrato – avaliado pelo EPD e Gabinete Jurídico

    – Conselho de Administração
  - Execução do estudo (teste à qualidade dos dados, transferência de dados)

IASLC 2021 World Conference on Lung Cancer
SEPTEMBER 8-14, 2021 | WORLDWIDE VIRTUAL EVENT

ELSEVIER

Journal of Thoracic Oncology
Volume 16, Issue 10, Supplement, October 2021, Page S1112

P48 NOVEL THERAPEUTICS AND TARGETED THERAPIES - EGFR

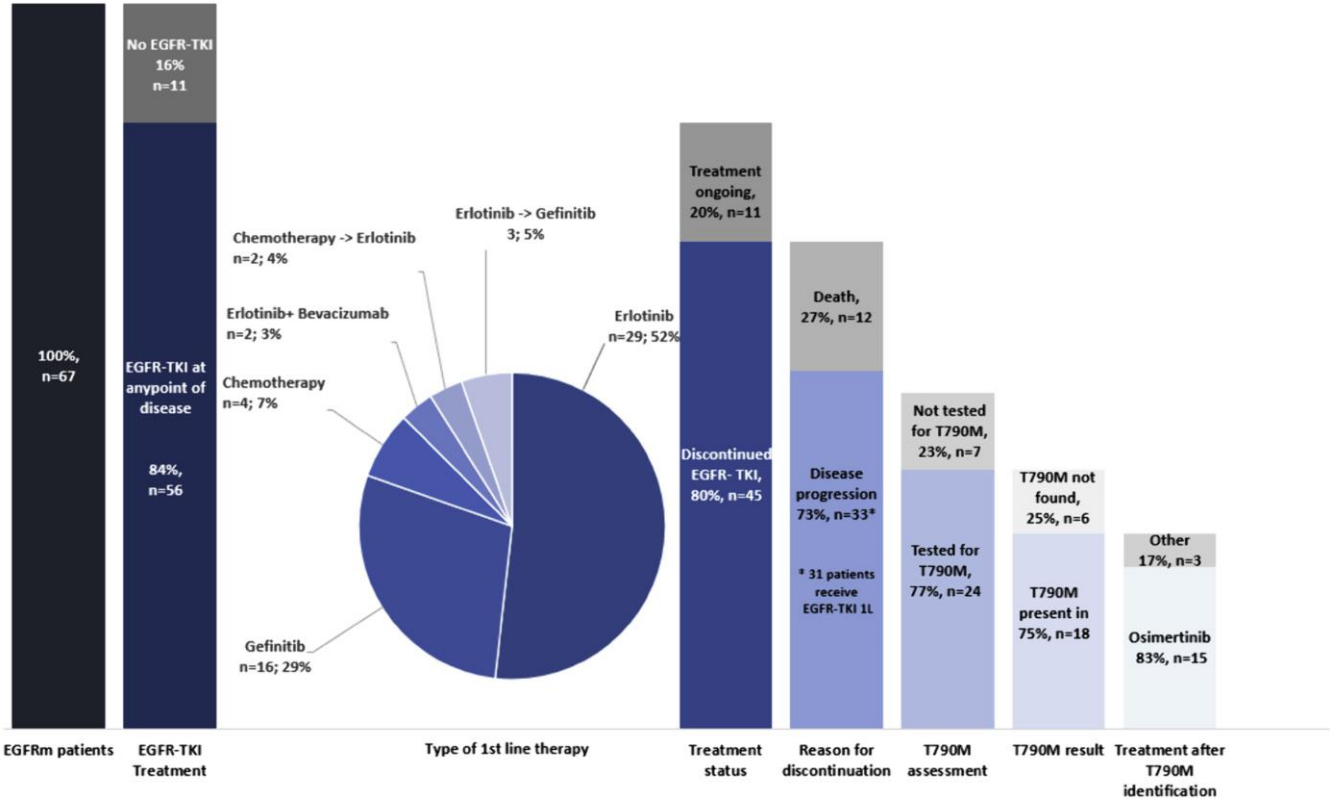
P48.15 EGFR Mutated Non-Small Cell Lung Cancer Treatment Pathway – What Is the Best Way?

M. Soares [1], S. Gonçalves-Monteiro [2], L. Antunes [2], F. Bernardo [3], S. Figueiredo [4], M. Borges [2], M.J. Bento [5], P. Redondo [6]

DOI: 10.1016/j.jtho.2021.08.526

P48.15 EGFR Mutated Non-Small Cell Lung Cancer Treatment Pathway – What Is the Best Way?

Figure 1 - Summary of EGFRm patient journey



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M. Soares, S. Gonçalves-Monteiro, L. Antunes, F. Bernardo, S. Figueiredo, M. Borges, M.J. Bento, P. Redondo

Journal of Thoracic Oncology , Volume 16 Issue 10 (October 2021) DOI: 10.1016/j.jtho.2021.08.526

Protocol Title:	Real-World Response/Survival and Treatment Patterns among Patients with Relapsed/Refractory Indolent Non-Hodgkin Lymphoma in USA, UK, France, Spain, and Portugal Oncology Practices
Protocol Number:	KTE-iNHL-RW2020

Parecer da Comissão de Ética para a Saúde: 23/07/2020

A Comparison of Clinical Outcomes from Updated ZUMA-5 (Axicabtagene Ciloleucel) and the International SCHOLAR-5 External Control Cohort in Relapsed/Refractory Follicular Lymphoma (r/r FL)

M Lia Palomba[1]; Paola Ghione[1,2]; Anik R Patel[3]; Kevin Deighton[4]; Caron A Jacobson[5]; Myrna Nahas[3]; A Scott Jung[3]; Anthony J Hatswell[4]; Steve Kanters[6]; Eve Limbrick-Oldfield[6]; Sally W Wade[7]; Julia Thornton Snider[3]; Sattva S Neelapu[8]; Maria Teresa Ribeiro[9]; John Gribben[10]; John Radford[11]; Sabela Bobillo[12]; Herve Ghesquieres[13]

[1]Memorial Sloan Kettering Cancer Center, New York, NY; [2]Roswell Park Comprehensive Cancer Center, Buffalo, NY; [3]Kite, A Gilead Company, Santa Monica, CA; [4]Delta Hat, Nottingham, UK; [5]Dana-Farber Cancer Institute, Boston, MA, USA; [6]RainCity Analytics, Vancouver, BC, Canada; [7]Wade Outcomes Research and Consulting, Salt Lake City, UT; [8]The University of Texas MD Anderson Cancer Center, Houston, TX, USA; [9]Portuguese Oncology Institute of Porto, Porto, Portugal; [10]Cancer Research UK Barts Centre, London, UK. [11]The Christie NHS Foundation Trust and University of Manchester, Manchester, UK; [12]Vall D'Hebron Institute of Oncology, Barcelona, Spain; [13]Centre Hospitalier Lyon Sud, Lyon, France

INTRODUCTION

- In the pivotal ZUMA-5 single-arm trial,[1] axicabtagene ciloleucel (axi-cel; an autologous anti-CD19 chimeric antigen receptor T-cell therapy) demonstrated high rates of durable response in r/r FL patients, including those with high-risk disease.
- The international SCHOLAR-5 external cohort was constructed to allow the

RESULTS

- 143 patients were identified in SCHOLAR-5, reducing to a weighted sum of 85 after applying propensity score weights, versus 86 patients in ZUMA-5 (**Table 1**).
- Median follow-up time for ZUMA-5 and SCHOLAR-5

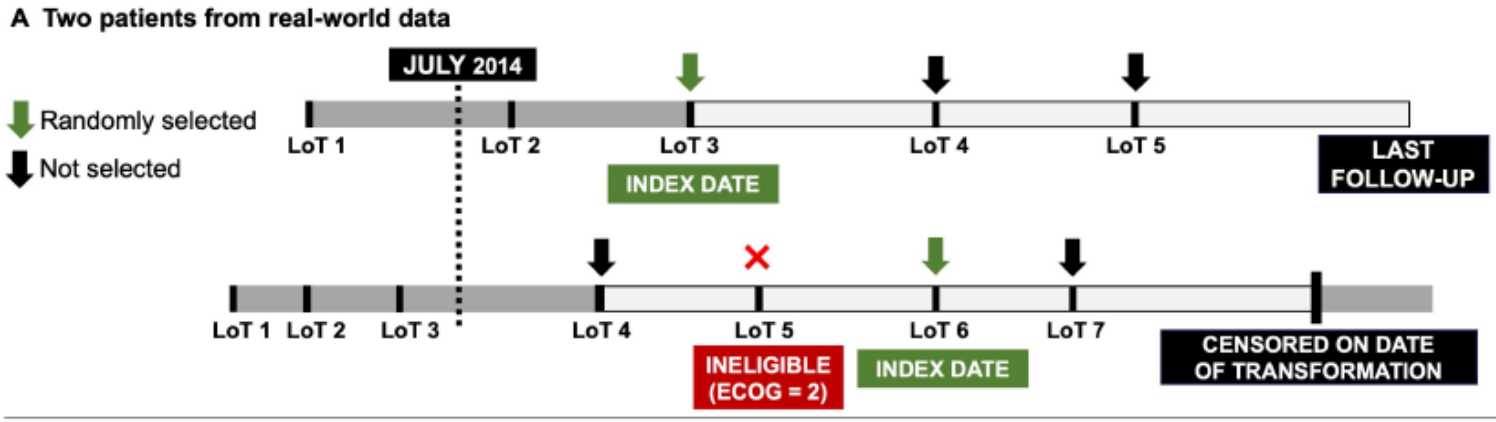
Table 1. Patient characteristics before and after propensity weighting

	SCHOLAR-5 before weighting (n = 143)	ZUMA-5 (n = 86)	SCHOLAR-5 after weighting (n = 85)	SMD (p-value)
Median age (range), years	64 (36 – 89)	62 (34 – 79)	61 (36 – 89)	0.036 (.85)

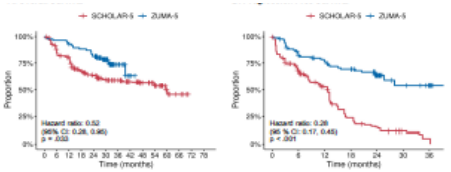
CONCLUSIONS

- Compared to currently available therapies in r/r FL patients, axi-cel demonstrated a clinically and statistically significant improvement in

Figure 2. LoT selection for real-world data



- The SCHOLAR-5 and ZUMA-5 cohorts were balanced (standardized mean difference [SMD] <0.1) for patient characteristics through propensity scoring on prespecified prognostic factors and standardized mortality ratio weighting.[3]
- ORR was compared using odds ratio. OS, PFS and next treatment-free survival (NTFS; time to next treatment or death) were evaluated using Kaplan-Meier analysis.
- Subgroup analyses were conducted on patients who initiated ≥4th LoT.



Kaplan-Meier curves showing survival outcomes in ZUMA-5 (blue), compared to SCHOLAR-5 (red).

	% (95% CI)	66.3% (55.5, 76.0)	53.1% (38.3, 67.5)	37.4% (22.1, 55.7)
CR	N responders	38/89	16/49	6/35
	% (95% CI)	42.7% (32.3, 53.6)	32.7% (19.9, 47.5)	17.1% (7.9, 33.3)
Time-to-event outcomes				
		N = 98	N = 52	N = 27
OS	Median months (95% CI)	NR (53.2 – NE)	30.4 (22.3 – NE)	13.1 (12.0 – NE)
	24 months % (95% CI)	79.6 (71.5 – 88.5)	57.3 (44.4 – 73.8)	36.1 (21.7, 60.1)
PFS	Median months (95% CI)	11.0 (8.6, 17.1)	7.4 (5.3, 15.1)	4.0 (3.1, 11.4)
	24 months % (95% CI)	20.4 (11.9 – 35.2)	11.5 (4.6 – 28.5)	3.5 (0.6, 22.6)
NTFS	Median months (95% CI)	21.2 (16.3 – 41.9)	22.9 (9.1 – NE)	8.7 (4.3 – 16.7)
	24 months % (95% CI)	48.3 (38.7 – 60.3)	46.2 (33.7 – 63.3)	22.38 (12.6 – 39.8)

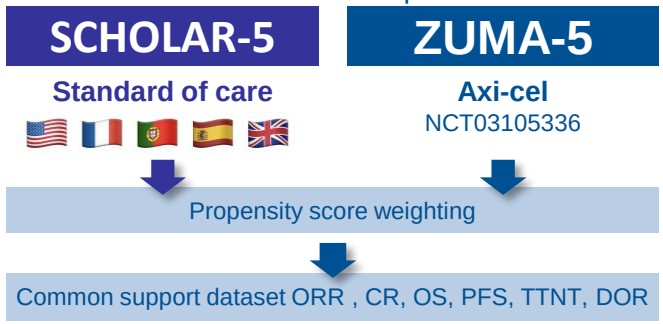
CR, complete response; LoT, line of treatment; NTFS, next treatment-free survival; ORR, overall response rate; OS, overall survival; PFS, progression-free survival.

63rd American Society of Hematology (ASH) Annual Meeting and Exposition, December 11-14, 2021

External control comparison of ZUMA-5 and SCHOLAR-5 in R/R FL

Objective: To compare clinical outcomes from the ZUMA-5 trial cohort to the international SCHOLAR-5 external control cohort

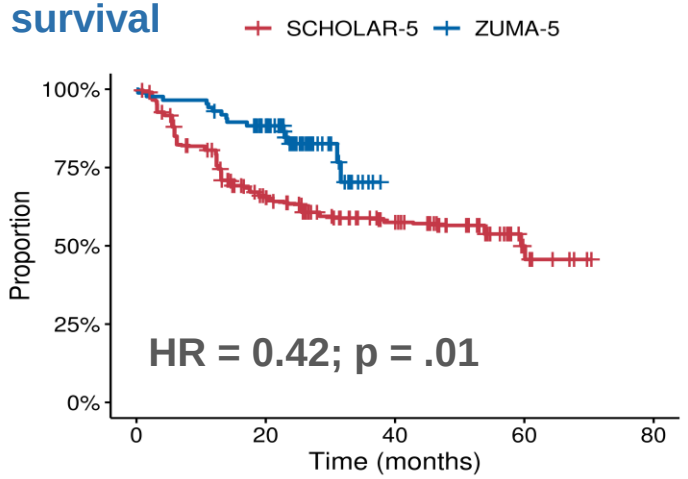
Methods: Propensity score methods were used to compare available treatments for r/r FL patients with axi-cel



- Axi-cel demonstrated a substantial improvement in all clinical endpoints including ORR, CR, OS, PFS and TTNT
- Findings support that axi-cel represents a significant improvement in treatment options for patients with r/r FL

		SCHOLAR-5	ZUMA-5	Odds Ratio (95% CI)	p-value
Overall response rate	Yes	42 (49.9%)	81 (94.2%)	16.24 (5.63, 46.85)	<0.0001
	No	43 (50.1%)	5 (5.8%)		

Overall survival



Outros estudos de RWD como comparador externo

- CATERPILLAR-RWE study: Real-world contemporary systemic anti-cancer therapy effectiveness in patients with advanced/metastatic non-small cell lung cancer (NSCLC) harbouring epidermal growth factor receptor Ex20ins (i.e. external control cohort); adjusted comparison between real-world outcomes in the external control cohort and amivantamab outcomes in the CHRYSALIS trial
  - CES – 14-04-2021

- Comparison of MVX-ONCO-1 efficacy with standard-of-care effectiveness in the treatment of Advanced Head and Neck Squamous Cell Carcinoma using SAKK 11/16 Trial (NCT02999646) data and two hospital databases in Europe
  - CES – 07-04-2022

- A Multicenter Observational Study of Patients Receiving Systemic Treatment for Advanced Synovial Sarcoma and Myxoid/Round Cell Liposarcoma
  - CES – 05-05-2022

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

DOI: 10.1111/ecc.13026

ORIGINAL ARTICLE

WILEY European Journal of Cancer Care

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

Luís Antunes[1] | Francisco Rocha-Gonçalves[1] | Sérgio Chacim[1] | Cinira Lefèvre[2] | Marta Pereira[3] | Sónia Pereira[4] | Aleksandra Zagorska[2] | Maria José Bento[1]

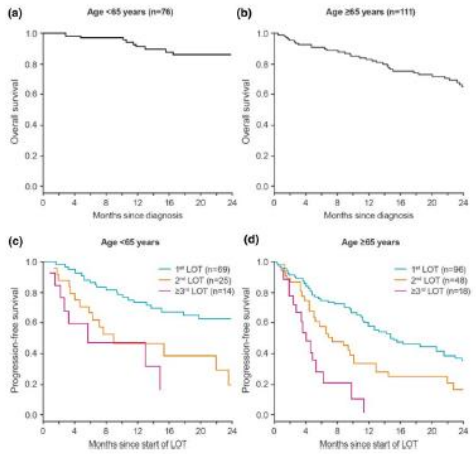


FIGURE 1 Overall survival probabilities by age and progression-free survival probabilities by LOT. (a) Overall survival probabilities for patients <65 years of age from the overall cohort and (b) for patients ≥65 years of age from the overall cohort; (c) Progression-free survival probabilities by LOT for patients <65 years of age and (d) for ≥65 years of age. LOT: line of therapy

TABLE 3 Treatment patterns in MM by LOT

	1st LOT		2nd LOT		≥3rd LOT	
n (%)	<65 years (*n* = 69)	≥65 years (*n* = 96)	<65 years (*n* = 25)	≥65 years (*n* = 48)	<65 years (*n* = 14)	≥65 years (*n* = 18)
Any novel therapy	69 (100.0)	83 (86.5)	23 (92.0)	37 (77.1)	13 (92.9)	13 (72.2)
Combination IMiD+PI	57 (82.6)	15 (15.6)	3 (12.0)	3 (6.3)	1 (7.1)	0
IMiD-based (excluding combination IMiD+PI)						
Lenalidomide	0	0	3 (12.0)	1 (2.1)	7 (50.0)	8 (44.4)
Thalidomide	8 (11.6)	55 (57.3)	14 (56.0)	26 (54.2)	4 (28.6)	1 (5.6)
PI-based (excluding combination IMiD+PI)						
Ixazomib[†]	0	0	3 (12.0)	0	0	0
Bortezomib	4 (5.8)	13 (13.5)	0	7 (14.6)	1 (7.1)	4 (22.2)
Other, non-novel (any combination)[‡]	0	13 (13.5)	2 (8.0)	11 (22.9)	1 (7.1)	5 (27.8)
Reason for change in LOT						
Disease progression	–	–	20 (80.0)	41 (85.4)	12 (85.7)	18 (100.0)
Maximum clinical benefit	–	–	4 (16.0)	1 (2.1)	0	0
Intolerance	–	–	0	4 (8.3)	2 (14.3)	0
Completed planned treatment	–	–	1 (4.0)	0	0	0
Other	–	–	0	2 (4.2)	0	0

Note. IMiD: immunomodulatory drug; LOT: line of therapy; MM: multiple myeloma; PI: proteasome inhibitor.
[†]Ixazomib was used only as part of a separate clinical trial these patients were enrolled in; [‡]Includes chemotherapy, corticosteroids, melphalan and excluding any combinations with novel therapies (IMiDs and/or PIs).

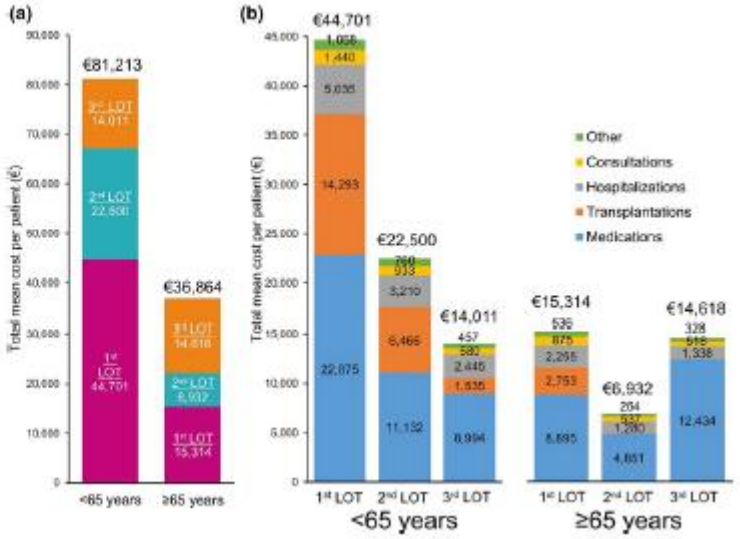


FIGURE 2 Total mean healthcare and resource costs of MM treatment to 24 months' follow-up per patient by LOT and age. (a) Total mean healthcare and resource costs; (b) Breakdown by category of healthcare resource. Other includes radiotherapy, emergency consultations, imaging tests and cytogenetics. LOT: line of therapy; MM: multiple myeloma

Antunes L, Rocha-Gonçalves F, Chacim S, et al. Real-world treatment patterns, resource use and cost burden of multiple myeloma in Portugal. *Eur J Cancer Care*. 2019;e13026.

PCN204
ISPOR EUR
2019

Cost-estimate of adding neoadjuvant pertuzumab for the treatment of HER2+ breast cancer in an institution in Portugal

BACKGROUND

RESULTS

OBJECTIVES

METHODS

mics Review

Open Access

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Table 5 Incremental cost-effectiveness ratio (ICER) by regimen and sensitivity analysis

Regimen	Effectiveness	Total direct cost per patient	ICER	Cost per pathological complete response	No. of patients who needed to be treated to have a pathological complete response	Additional cost for pathological complete response
AC-DH	33%	40,393 €		121,180 €	3.0	
AC-DHP	45%	56,375 €	1370 €	125,277 €	2.2	4097 €
Sensitivity analysis						
Scenario 1. Clinical staging II and negative HR						

Conclusions:

- The results obtained in the AC-DHP cohort, which included Pertuzumab as a NeoT, suggest better clinical outcomes compared to the AC-DH group, a confirmation based on real-world data.
- The AC-DHP average total cost/patient was 56,375€, with pertuzumab accounting for 13,978€ (24.79%) and increasing in 15,982€ the average cost/patient ($p < 0.001$). Clinical staging and hormone receptors (HR) were significantly associated with pCR. ICER was 1.370€ per percentage point of pCR.
- Future research using pertuzumab as comparator for newly treatments in the HTA and reimbursement assessment based on patient's outcomes in Portugal will surely consider these results

Experiência do IPO - Porto com células CAR T

abril de 2019 a abril de 2022

Doentes	N
Leucaféreses para CAR T	**47**
Tisagenlecleucel	**25**
Axicabtagene	**21**
Brexucabtagene	**1**
Ordens de compra para produção de CAR T	**45**
Número de CAR T infundidos	**39**
Linfomas não Hodgkin	**37**
Leucemia linfoblástica	**2**

Experiência do IPO - Porto com células CAR T

abril de 2019 a abril de 2022

Doentes	N
Doentes não infundidos	**6**
Morte antes da infusão	**4**
Resposta completa após “bridging”	**1**
Avaliação de Resposta	**32 doentes LNH DGCB**
Tisagenlecleucel	**46,1% RC**
Axicabtagene	**68,75% RC**
Ensaios clínicos	**(Juliet RC de 39%) (Zuma 1 RC de 54%)**

Lancet Oncol 2019; 20: 31–42

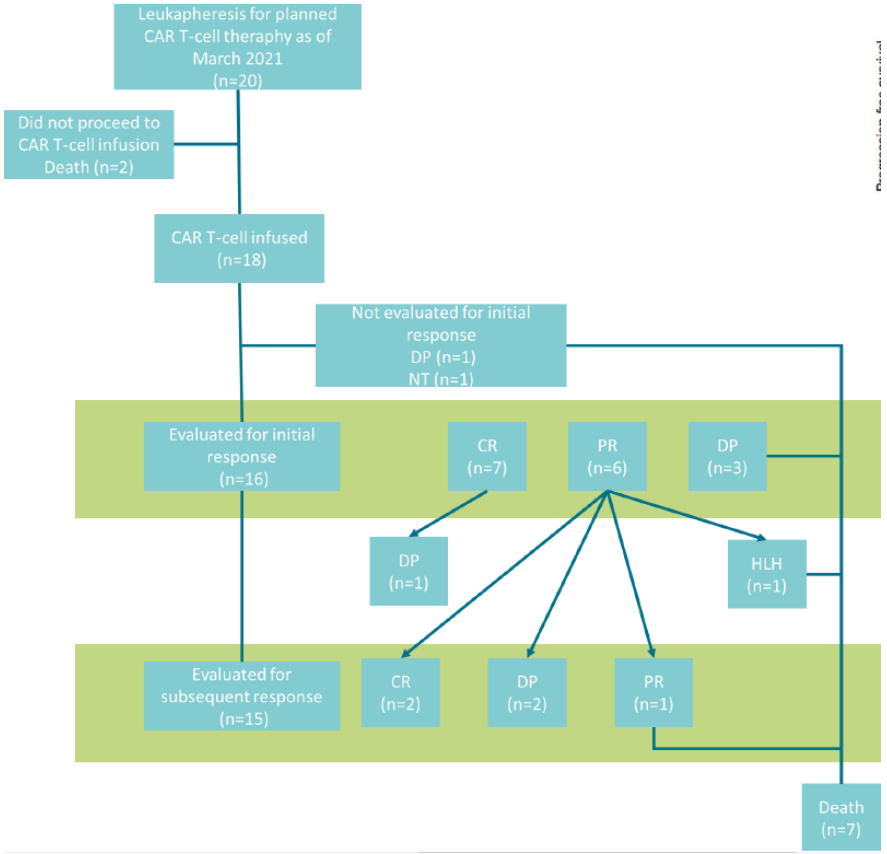
***Lancet Oncol* 2021; 22: 1403–15**

Costs, effectiveness, and safety associated with Chimeric Antigen Receptor (CAR)-T cell therapy: results from a Portuguese Comprehensive Cancer Center*

Authors and affiliations: Sérgio Chacim*, Teresa Monjardino* , José Luís Cunha, Pedro Medeiros ,
Patrícia Redondo, Maria José Bento, José Mário Mariz

Characteristics	Total (n=20)
Overall survival (OS)	
Alive with no evidence of disease	8 (40.0)
Alive with evidence of disease	3 (15.0)
Fatal events	9 (45.0)
Disease progression	7 (35.0)
Neurotoxicity	1 (5.0)
Hemophagocytic lymphohistiocytosis	1 (5.0)
Best Overall response rate (n=16*)	13 [81.3 (95%CI: 54.4 - 96.0]
Complete response (CR)	9 [56.3 (95%CI: 29.9 – 80.2]
Partial response (PR)	4 [25.0 (95%CI: 7.3 – 52.4]
Disease progression (DP)	3 [18.8 (95%CI: 4.0 – 45.6]

*Two patients died before infusion and 2 patients died before response assessment



cohort march 2021: 20 patients evaluated

*in press

Em Conclusão

- Facilitadores da implementação no IPO Porto
  - Estrutura orgânica e cultura da organização
  - Cooperação e multidisciplinariedade
  - Marcos tecnológicos (agendamento do Hospital de Dia, processo clínico eletrónico, prescrição eletrónica de medicamentos, registo de cancro, software da QoL, processo de acreditação…)

- Estudos são importantes para apoio à decisão
  - Profissionais avaliam os resultados obtidos na prática clínica
  - Controlo de qualidade dos dados
  - Incremento da investigação clínica

MUITO OBRIGADA

A utilização de dados em saúde: a experiência do IPO Porto

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