Oncology Case Studies

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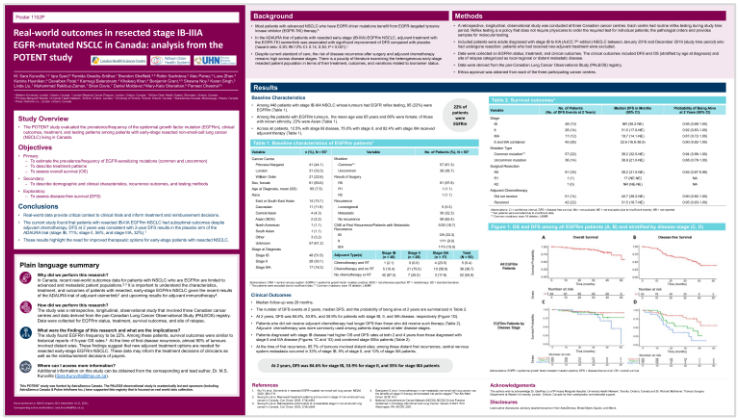
Case study:

Phase IV pricing & reimbursement support

ESMO GOOD SCIENCE BETTER MEDICINE BEST PRACTICE

Real world outcomes in resected stage IB-IIIA EGFR mutated NSCLC in Canada: Analysis from the POTENT study

M. Sara Kuruvilla1,2, Iqra Syed, Femida Gwadry-Sridhar, Brandon Sheffield, Robin Sachdeva, Alec Pencz, Luna Zhan, Katrina Hueniken, Devalben Patel, ..., Parneet Cheema


Real-world outcomes in resected stage IB-IIIA EGFR-mutated NSCLC in Canada: analysis from the POTENT study

Background

Methods

Results

Study Overview

Objectives

Conclusions

Plain language summary

Real world data provide critical context to clinical trials and inform treatment and reimbursement decisions where patients with resected IB-IIIA EGFRm NSCLC had suboptimal outcomes, despite adjuvant chemotherapy.

Pulse's RWD identified a considerable unmet need for a large patient population.

Large registries providing robust cohorts for sub-analysis (e.g., specific mutations)

Diverse populations including under-represented groups across multiple sites

Uniform data and disease models, and curation across sites and conditions

[Click here to read the poster]

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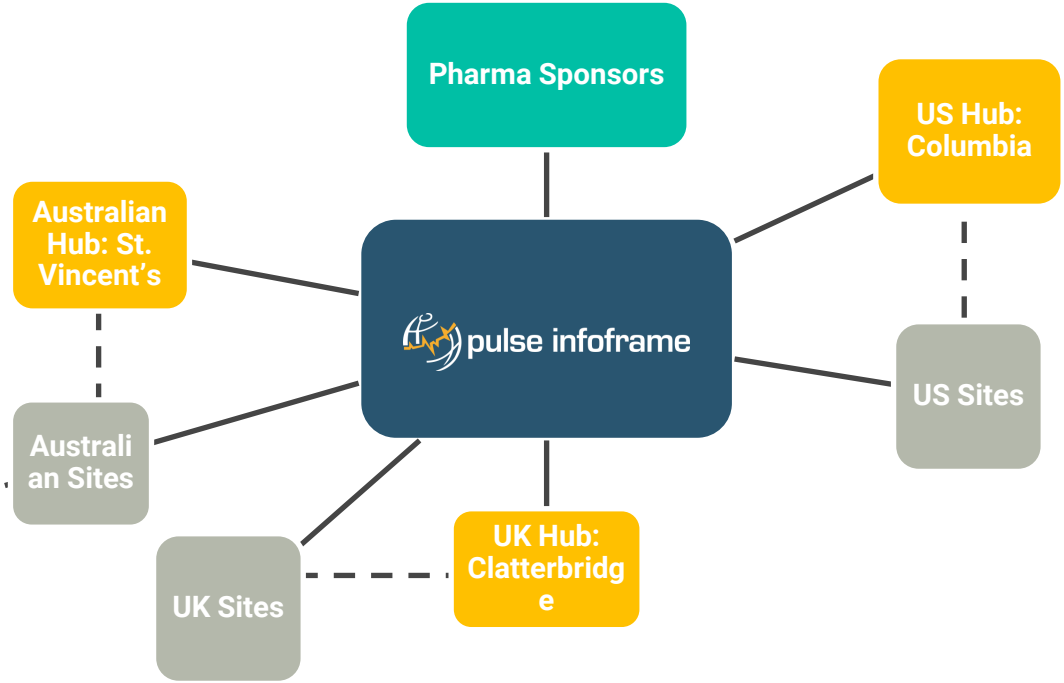
Case Study: The Uveal Melanoma registry

The Uveal Melanoma registry was launched in March 2020 with the support from Immunocore, after a development phase that began in 2018.

During its development, the scope and size of the registry changed significantly, although it was still possible for Pulse to deliver a platform that collects 360+ data elements that are based on PI's guidance and it is inclusive of retrospective and prospective data

The expanded variables within the platform include tumor characteristics, systemic therapies, biomarkers, biobanking, distant metastasis and treatment, ECOG status, surveillance, comorbidity and others.

Initially launched in 11 sites across the US, UK, Canada and Australia, this has now increased. The registry utilizes a hub and spoke model where the Pulse platform is set up to ingest data from multiple sites in multiple countries. The platform has been developed to support current plans to expand the registry into new countries and to establish new sites in the original countries through additional sponsorship.



Capturing uveal melanoma (UM) global practice patterns and clinical outcomes in the collaborative ocular melanoma natural history (OMNi) study (NCT04588662)

Joseph J. Sacco, Marlana M. Orloff, Sapna Pradyuman Patel, Max Conway, Li-Anne Lim, Lotte S. Fog, David Sia, John McKenzie, Daniel McKay, Roderick O'day, Timothy Isaacs, Alexander Noor Shoushtari, Ryan J. Sullivan, Sarah Kin, Femida Hussein Gwadry-Sridhar, Anthony M. Joshua, Richard D. Carvajal

The OMNi dataset can serve and aid in interpretation of clinical trial outcomes in the real-world, facilitate cutting-edge research, and accelerate the development of diagnostics and therapeutics.

Summary:

- An ambispective database developed to provide contemporary real-world data of UM, capturing its natural history and serving as a virtual biospecimen repository
- Objective to characterize regional/international UM management practice patterns and associated clinical outcomes to inform best practice recommendations.
- Will facilitate new insights, hypothesis testing, as well as clinical trial development and conduct
- Governance structure to make accessible for research

Pulse manages the OMNi registry – co-designed program, hosts on Healthie® platform, manages sites, data curation, and is responsible for commercial relationships with biopharma

- Large registries providing robust cohorts for sub-analysis (e.g., specific mutations)
- Diverse populations including under-represented groups across multiple sites
- Uniform data and disease models, and curation across sites and conditions

Case Study:
Trial Support

Data curation & PROs to support interventional trial

Adjuvant Crizotinib in High-Risk Uveal Melanoma Following Definitive Therapy

Shaheer Khan, Jose Lutzky, Alexander Noor Shoushtari, Joanne M. Jeter, Cody Chiuzan, Naomi Sender, Lauren Esther Blumberg, Alexandra Nesson, Shahnaz V. Singh-Kandah, Susana Hernandez, Grazia Ambrosini, Oliver Surriga, Gary K. Schwartz, Richard D. Carvajal

The use of adjuvant crizotinib in patients with high-risk UM did not reduce rates of relapse in this multicenter, single arm trial. 9/32 (28%) pts required dose modification or discontinuation due to AE which may have limited efficacy.

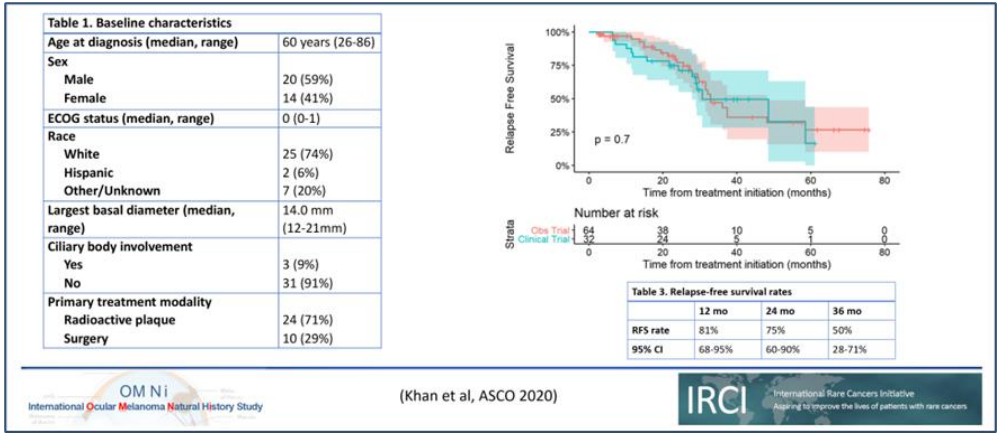


Table 1. Baseline characteristics	
Age at diagnosis (median, range)	60 years (26-86)
Sex	
Male	20 (59%)
Female	14 (41%)
ECOG status (median, range)	0 (0-1)
Race	
White	25 (74%)
Hispanic	2 (6%)
Other/Unknown	7 (20%)
Largest basal diameter (median, range)	14.0 mm (12-21mm)
Ciliary body involvement	
Yes	3 (9%)
No	31 (91%)
Primary treatment modality	
Radioactive plaque	24 (71%)
Surgery	10 (29%)

Table 3. Relapse-free survival rates			
	12 mo	24 mo	36 mo
RFS rate	81%	75%	50%
95% CI	68-95%	60-90%	28-71%

OMNi International Ocular Melanoma Natural History Study

(Khan et al, ASCO 2020)

IRCI International Rare Cancers Initiative

Pulse provided the platform, site and data management, and support for this interventional trial within our broader Uveal Melanoma registry

- Existing, established relationships with sites and investigators to facilitate programs
- All data curation and management, investigator dashboards, patient consents and PROs on Pulse Healthie® platform
- Uniform data and disease models, and curation across sites and conditions

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Case study:
Capturing clinical outcomes

ASCO®

Capturing uveal melanoma (UM) global practice patterns and clinical outcomes in the collaborative ocular melanoma natural history (OMNi) study (NCT04588662)

Joseph J. Sacco, Marlana M. Orloff, Sapna Pradyuman Patel, Max Conway, Li-Anne Lim, Lotte S. Fog, David Sia, John McKenzie, Daniel McKay, Roderick O'day, Timothy Isaacs, Alexander Noor Shoushtari, Ryan J. Sullivan, Sarah Kin, Femida Hussein Gwadry-Sridhar, Anthony M. Joshua, Richard D. Carvajal

MELANOMA/SKIN CANCERS

Check for updates

TPS9610 Poster Session

Capturing uveal melanoma (UM) global practice patterns and clinical outcomes in the collaborative ocular melanoma natural history (OMNi) study (NCT04588662).

Joseph J. Sacco, Marlana M. Orloff, Sapna Pradyuman Patel, Max Conway, Li-Anne Lim, Lotte S. Fog, David Sia, John McKenzie, Daniel McKay, Roderick O'day, Timothy Isaacs, Alexander Noor Shoushtari, Ryan J. Sullivan, Sarah Kin, Femida Hussein Gwadry-Sridhar, Anthony M. Joshua, Richard D. Carvajal; Clatterbridge Cancer Centre, Wirral, United Kingdom; Sidney Kimmel Medical College of Thomas Jefferson University, Philadelphia, PA; The University of Texas MD Anderson Cancer Center, Houston, TX; University of Sydney, Sydney, Australia; Royal Victorian Eye and Ear Hospital, Melbourne, Australia; Royal Adelaide Hospital, Adelaide, Australia; The Royal Children's Hospital Melbourne, Melbourne, Australia; University of Western Australia, Perth, Australia; Memorial Sloan Kettering Cancer Center, New York, NY; Massachusetts General Hospital, Boston, MA; Pulse InfoFrame, London, ON, Canada; Pulse Infoframe Inc., London, ON, Canada; Princess Margaret Cancer Center, University Health Network, University of Toronto, Toronto, ON, Canada; Columbia University Irving Medical Center, New York, NY

Background: Geographical differences in the management of primary UM, surveillance for recurrence, and care of metastatic disease have emerged based upon local expertise, treatment availability and insurance coverage. We have initiated accrual to OMNi (NCT04588662), an ambispective database developed to provide contemporary real-world data of UM, capturing its natural history and serving as a virtual biospecimen repository. The overall objectives of OMNi are to characterize regional/international UM management practice patterns and associated clinical outcomes in an effort to inform best practice recommendations. **Methods:** OMNi utilizes the Pulse Infoframe Healthie platform, a globally compliant platform which enables the structured collection of data mapped to Observational Medical Outcomes Partnership. The data fields created permit longitudinal capture of data including baseline patient and tumor characteristics, treatment of primary lesion and outcomes, surveillance patterns, time to disease recurrence, treatment of recurrent disease with outcomes, and survival. Inclusion criteria include a diagnosis of uveal melanoma and the ability to provide written informed consent for participation in the prospective registry or an institutional waiver by the IRB/ethics committee for retrospective data collection without written informed consent. We have initiated data collection at 3 US and 3 Australian centers, with 184 patients enrolled to date. Based upon feasibility assessment, we anticipate retrospective data entry for ~2,000 patients and annual recruitment of ~700 patients once all centers are active. Data collected in this OMNi collaboration, which will include additional US, UK and Australian sites, will facilitate new insights, hypothesis testing, as well as clinical trial development and conduct, and through a governance structure, will be made accessible for research. The OMNi dataset can serve and aid in interpretation of clinical trial outcomes in the real-world, facilitate cutting-edge research, and accelerate the development of diagnostics and therapeutics. Clinical trial information: NCT04588662. Research Sponsor: Immunocore and BMS.

The OMNi dataset can serve and aid in interpretation of clinical trial outcomes in the real-world, facilitate cutting-edge research, and accelerate the development of diagnostics and therapeutics.

- An ambispective database developed to provide contemporary real-world data of UM, capturing its natural history and serving as a virtual biospecimen repository.
- Objective to characterize regional/international UM management practice patterns and associated clinical outcomes to inform best practice recommendations.
- Will facilitate new insights, hypothesis testing, as well as clinical trial development and conduct.
- Governance structure to make data accessible for research in uveal melanoma.

Global Melanoma Research Network

WHAT WAS THE ISSUE?

The original purpose for the GMRN registry when it was developed 10 years ago was to understand how patients are doing after they've received treatments for melanoma. It would address the following:

- What are the benefits?
- What are the outcomes?
- Are there differences based on practice, region, and/or province?

WHY WAS IT NEEDED?

Patients are data generators: they're research partners. Those participating in the GMRN registry want to know what the impacts and/or results of the treatments they're undergoing were.

HOW WAS IT DEVELOPED?

The GMRN registry makes use of the healthie™ platform which provides a collaborative ecosystem made up of researchers, patients, and industry for drug development increases the chances for developing treatments that truly benefit patients. A platform that supports the voice of the patient while collecting rigorous, regulatory grade data is the solution that can propel research that truly benefits patients.

WHAT ARE THE RESULTS?

Treatments have evolved over the last 10 years, and the GMRN registry has provided both the research and medical communities opportunities to ask detailed questions to not only advance treatments but also to improve treatment outcomes for and impact on patients. As the registry expanded, it's been possible to extend the platform to support subtypes of skin cancers, e.g., Merkle cell, squamous cell, and basal cell carcinoma. Researchers can leverage one platform for exploring multiple other malignant diseases.

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GMRN registry

IMPACT OF THE REGISTRY

"Over the past 10 years, the Global Melanoma Research Network team has been able to track the impact on patients and the efficacy of treatment: Is the treatment having a positive impact? How does the RWD compare to the favorable results reported from the clinical trials?"

Dr. Scott Ernst, PI for Global Melanoma Research Network

4,900+ PATIENTS ENROLLED

Since its launch, the GMRN registry was enrolled over 4,900 patients and this number continues to increase

2012 LAUNCH

The GMRN Registry was launched in 2012 and the London Regional Cancer Program the first site to register patients. The evidence generated from the platform has been leveraged by over 6 pharma companies for prospective and retrospective(longitudinal), HEOR, quality of life and epidemiological studies

PUBLICATIONS

Data from the GMRN Registry has been used in more than 6 publications to increase the understanding of the disease and treatment efficacy. Additionally, over two dozen abstract have been published

15 SITES

With 12 sites throughout Canada, the GMRN Registry is the largest registry for the disease in the region

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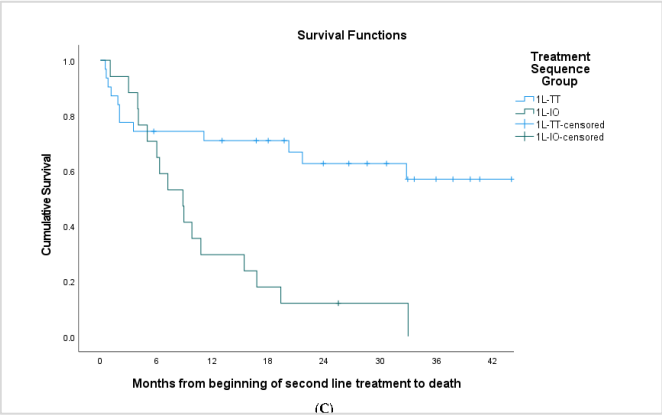
Real-World Evidence of Systemic Therapy Sequencing on Overall Survival for Patients with Metastatic BRAF-Mutated Cutaneous Melanoma

Kartolo A, Deluce J, Hopman WM, Liu L, Baetz T, Ernst S, Lenehan JG.

https://doi.org/10.3390/curroncol29030126

Aim: To evaluate optimal systemic therapy sequencing (first-line targeted therapy (1LTT) vs. first-line immunotherapy (1L-IO)) in patients with BRAF-mutated metastatic melanoma.
Methods: Nation-wide prospective data of patients with newly diagnosed BRAF-mutated metastatic melanoma were retrieved from the Canadian Melanoma Research Network (CMRN).




Current Oncology

Article

Real-World Evidence of Systemic Therapy Sequencing on Overall Survival for Patients with Metastatic BRAF-Mutated Cutaneous Melanoma

MDPI

Adi Kartolo [1], Jasna Deluce [2], Wilma M. Hopman [3], Linda Liu [4], Tara Baetz [1], Scott Ernst [2] and John G. Lenehan [5,*]

Abstract: Aim: To evaluate optimal systemic therapy sequencing (first-line targeted therapy (1L-TT) vs. first-line immunotherapy (1L-IO)) in patients with BRAF-mutated metastatic melanoma. Methods: Nation-wide prospective data of patients with newly diagnosed BRAF-mutated metastatic melanoma were retrieved from the Canadian Melanoma Research Network. Results: Our study included 79 and 107 patients in the 1L-IO and 1L-TT groups, respectively. There were more patients with ECOG 0–1 (91% vs. 72%, $p = 0.023$) in the 1L-IO group compared to the 1L-TT group. Multivariable Cox analysis suggested no OS differences between the two groups (HR 0.838, 95%CI 0.502–1.400, $p = 0.500$). However, patients who received 1L-TT then 2L-TT, and 1L-TT then 2L-IO had the longest OS compared to 1L-IO without 2L therapy, 1L-IO then 2L-TT, and 1L-IO then 2L-IO (38.3 vs. 32.2 vs. 16.9 vs. 6.3 months, $p < 0.001$). For patients who received 2L-TT, those who received 2L-IO had a trend towards OS improvement (8.9 vs. 6.3 months, $p = 0.053$). Conclusions: Our nation-wide prospective study failed to establish any optimal systemic therapy sequencing in advanced BRAF-mutant melanoma patients. Nevertheless, we provided evidence that immunotherapy has durable efficacy in advanced BRAF-mutant melanoma patients regardless of treatment line, and that Canadian medical oncologists were selecting the appropriate treatment sequences in a real-world setting, based on patients' clinical and tumour characteristics.

Keywords: BRAF mutant; melanoma; targeted therapy; immunotherapy; therapy sequencing

1. Introduction

It is well-established that effective systemic therapy options for advanced cutaneous melanoma have dramatically improved since the introduction of immunotherapy and, in the case of BRAF V600E/K-mutated melanoma, targeted therapy. For patients that have melanoma with an activating BRAF mutation, both treatment modalities are available, and typically the choice of which therapy is used first is at the discretion of the treating oncologist. Currently, there are limited data to guide selection of a first-line treatment. Guidelines from leading organizations offer little in the way of direction in this situation [1–3]. More recently, the 2019 ESMO Clinical Practice Guidelines suggested first-line therapy decisions need to be individualized according to patients' clinical status, comorbidities, treatment goals, and personal preferences, although immunotherapy should still be preferred as first-line therapy for its durable disease control even after treatment discontinuation [3].

When examining the randomized phase III data from all therapies, targeted therapy has a superior progression free survival (PFS) and overall survival (OS) compared to

Case Study:
Merkel Cell Carcinoma

Treatment Patterns and Outcomes in Merkel Cell Carcinoma: A 10-Year Institutional Review

Full Poster

Project Context

- Merkel cell carcinoma (MCC) is a rare, aggressive cutaneous malignancy with neuroendocrine features and a high risk of recurrence and metastasis.
- Limited real-world data is available on treatment outcomes across multimodal therapies.
- Aim: Describe disease presentation, progression, and evaluate treatment patterns and outcomes in patients with MCC over a 10-year period.
- Retrospective study of 51 MCC patients treated at the London Regional Cancer Program (LRCP) from 2010–2020.
- Data included demographics, staging, recurrence, survival, and treatment response to surgery, radiotherapy, chemotherapy, and immunotherapy.

The solution

- Conducted a retrospective chart review of MCC cases across a 10-year span.
- Evaluated treatment types and timing, including first- and second-line therapies.
- Captured outcomes such as disease progression, recurrence, survival, and cause of death.
- Provided insights into real-world response durability and unmet needs in advanced MCC.
- Informed future prospective registry planning to guide treatment strategies for rare cutaneous cancers.

The results

- 51 patients studied: 56.9% presented with Stage IIIA or higher.
- Recurrence or progression occurred in 37.3% of patients.
- First-line chemotherapy responses: 23.1% complete, 38.5% partial, 30.8% progressive disease.
- Median time to death (among deceased): 28.4 months from diagnosis.

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Case Study:
Advanced Basal Cell Carcinoma

Real-World Experience with Vismodegib at a Canadian Cancer Center

Full Article

Project Context

- Advanced BCC (aBCC), including locally advanced (laBCC) and metastatic (mBCC) cases, poses major treatment challenges when surgery or radiation is contraindicated.
- Vismodegib, a Hedgehog pathway inhibitor, is a first-line option for aBCC, but real-world data on outcomes, relapse, and long-term durability are limited.
- This retrospective study analyzed 46 patients with aBCC treated at the London Regional Cancer Program (LRCP) from 2012–2019, focusing on treatment response, progression, recurrence, and adverse effects.

The solution

- Patients received daily oral vismodegib (150 mg) and were followed through treatment response and beyond.
- Evaluated time to maximal response, durability of remission, treatment discontinuation due to adverse events, and outcomes after subsequent surgery or radiotherapy.
- Data informed clinical strategies around treatment length, timing of surgical intervention, and managing recurrence.

The results

- 91.3% overall response rate: 50% complete response (CR), 41.3% partial response (PR).
- Median time to maximal response: 5.3 months.
- 69.6% of patients experienced progression post-response; 82% of CR patients who stopped treatment relapsed.
- 23.9% went on to have surgery and 23.9% received radiotherapy, often for local recurrence or incomplete response.
- 43.5% discontinued treatment at least once due to adverse effects (most commonly muscle spasms and dysgeusia).
- Study supports early surgical integration and ongoing exploration of intermittent or shorter dosing strategies.

Case Study:
Squamous Cell Carcinoma

Evaluating Cemiplimab Effectiveness in Advanced Cutaneous SCC

Full Article

Project Context

- Cutaneous squamous cell carcinoma (cSCC) is the second most common nonmelanoma skin cancer in Canada
- Limited real-world data available on treatment with cemiplimab for refractory locally advanced (LA) and metastatic cSCC
- Aim: Characterize demographics and clinical outcomes for advanced cSCC patients receiving cemiplimab in a real-world setting
- Retrospective study of adult patients treated at the London Regional Cancer Program
- Data collected: treatment characteristics, Kaplan-Meier estimates of progression-free survival (PFS) and overall survival (OS)
- Focus on understanding real-world effectiveness and tolerability outside of clinical trials

The solution

- Leveraged patient registry data to conduct a retrospective analysis
- Assessed treatment duration, outcomes, and reasons for discontinuation
- Identified key clinical metrics including PFS, OS, and adverse event rates
- Provided insights into real-world effectiveness of cemiplimab for both LA and metastatic cSCC
- Supported evidence generation for decision-making in oncology care for rare, advanced cases

The results

- Study included 40 patients: 40% with LA disease, 60% with metastatic disease
- Median treatment duration: 3.5 months (range: 0.6–29.4 months)
- 25% of patients experienced at least one adverse event
- Main reasons for discontinuation: death (25%), progression (15%), AEs (5%), other (15%)
- Real-world survival estimates lower than clinical trials, but cemiplimab shown to be safe and effective in practice

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Thank You!

™