INTERNAL MEDICINE JOURNAL

RACP Specialists. Together

doi:10.1111/imj.16536

BRIEF COMMUNICATION

Medicine Access Programmes: what do patients think – a patient-reported outcome study on ribociclib in metastatic breast cancer in Australia

Natasha Yap [1], Vanessa Wong [2,3], Catherine Morton,[2] Richard de Boer,[4,5] Sally Baron-Hay,[6] Robert Blum,[7] Benjamin Forster,[8] Susan Chua,[9] Kerrie Clarke,[10] Katharine Cuff,[11] Michael Green,[12] Elgene Lim,[13] Kelly Mok,[14] Louise Nott,[15] Michelle Nottage,[16] Ali Tafreshi,[17] Daphne Tsoi,[18] Anthony Uccellini,[19] Peter Gibbs[2,3] and Sheau Wen Lok [2,4]

[1]Department of General Medicine, Royal Melbourne Hospital, [2]Gibbs Labs, Personalised Oncology Division, Walter and Eliza Hall Institute of Medical Research, [3]Department of Medical Oncology, Western Health, [4]Department of Medical Oncology, Peter MacCallum Cancer Centre, [5]St Vincent's Private Hospital, [9]Department of Medical Oncology, Eastern Health, [12]Epworth Freemasons, and [19]Olivia Newton-John Cancer and Wellness Centre, Austin Health, Melbourne, [7]Department of Medical Oncology, Bendigo Health, Bendigo, Victoria, [6]Department of Medical Oncology, Northern Cancer Institute, [8]Department of Medical Oncology, Mater Hospital, [13]Department of Medical Oncology, Kinghorn Cancer Centre, and [14]Department of Medical Oncology, Liverpool Hospital, Sydney, [10]Department of Medical Oncology, Albury Wodonga Regional Cancer Centre, Albury, [17]Department of Medical Oncology, Wollongong Private Hospital, Wollongong, New South Wales, [11]Department of Medical Oncology, Princess Alexandra Hospital, and [16]Department of Medical Oncology, Royal Brisbane Hospital, Brisbane, Queensland, [15]Department of Medical Oncology, Royal Hobart Hospital, Hobart, Tasmania, and [18]Department of Medical Oncology, St John of God Hospital, Perth, Western Australia, Australia

Key words
Medication Access Programme, patient-reported outcome, quality of life.

Correspondence
Sheau Wen Lok, Peter MacCallum Cancer Centre, 305 Grattan Street, Melbourne, Vic. 3350, Australia.
Email: sheauwen.lok@petermac.org

Received 16 February 2024; accepted 6 September 2024.

Abstract

This study evaluated patient-reported outcomes (PROs) of Medicine Access Programmes (MAPs) for Australian metastatic breast cancer patients on ribociclib. Limited patient awareness of MAP enrolment was identified, emphasising the need for improved education and consent processes. Most patients expressed gratitude for accessing non-funded medications and perceived enhanced medication adherence as a key benefit. Integrating PRO data with real-world registry data provides comprehensive insight for future MAP development.

Medicine Access Programmes (MAPs) serve as a bridge between drug development and regulatory and funding approval, offering early access to promising therapies outside the clinical trial setting.[1] MAPs are available in most medical subspecialties but are relatively prevalent in the field of oncology. While MAPs offer early access to therapies for patients, there is minimal literature regarding patients' understanding and opinions about MAPs and whether they impact patients' perceptions of their treatment. Here we report a recent substudy conducted specifically to evaluate patient-reported outcomes (PROs) regarding a MAP that was available in metastatic hormone-receptor-positive breast cancer.

Funding: Novartis provided financial support to the study.
Conflict of interest: None.

Metastatic breast cancer is associated with an overall poor prognosis. Ribociclib is a cyclin-dependent kinase 4/6 inhibitor which has demonstrated significant clinical benefit when given in combination with an aromatase inhibitor in the first-line treatment of metastatic hormone-receptor-positive breast cancer.[2] In Australia, access to ribociclib was initially facilitated through a cost-free MAP, allowing eligible patients to receive the medication after Therapeutic Goods Administration approval and before it was included in the Pharmaceutical Benefits Scheme (PBS).[3] These patients were subsequently transitioned to the PBS programme once approval was granted.

Kisqali Access Registry for Metastatic breast cancer in Australia (KARMA) was established in August 2019 as a non-interventional study of Australian patients who received first-line treatment with ribociclib and an

aromatase inhibitor via the ribociclib MAP from May 2017 to June 2018. The main results of this study have been published.[2]

A substudy of KARMA was conducted to evaluate PROs in patients with metastatic breast cancer enrolled in the ribociclib MAP in Australia to assess their awareness and understanding of the MAP. A PRO questionnaire (Appendix I) was developed and administered to eligible patients by telephone interview, and a descriptive analysis was conducted to summarise the responses. Verbal informed consent was obtained from patients prior to administering the interview. The questionnaire covered topics such as patients' understanding of the MAP, medication delivery methods, overall experience with ribociclib and impact on quality of life. This study and its reporting have been approved by Melbourne Health HREC, an institutional ethics committee.

Of the 160 patients in the KARMA registry, 30 patients who remained on ribociclib were selected for this substudy. These patients were of English-speaking background.

When patients were asked if they understood how their ribociclib treatment was accessed (clinical trial vs. access programme vs. government funding), only 40% correctly identified it as part of an access programme funded by the pharmaceutical company. A total of 33% believed that they had received the medication through a clinical trial, and 17% thought it was government funded. For patients who knew that ribociclib was funded through an access programme, 33% indicated that they had a good understanding of the programme while 17% said they did not understand it.

When informed that ribociclib was prescribed through a MAP, the majority of patients (93%) expressed gratitude for having access to a medication they would otherwise not have received, as opposed to none who felt like a 'guinea pig in a research study'. Moreover, 67% of patients indicated that they would have improved their adherence to ribociclib if they had known that it was prescribed through a MAP.

Patient satisfaction with the medication and the MAP was high, with 97% rating their overall experience with ribociclib as good or excellent. A total of 57% of patients had to stop temporarily taking ribociclib due to side effects, while 20% temporarily stopped for reasons unrelated to toxicity, such as travel plans or while having radiotherapy. All patients had resumed ribociclib at the time of the interview.

In terms of interference with quality of life, family life and social activities, none of the patients reported any negative impact in the week leading up to the interview.

Discussion

The findings of this study shed light on several important aspects of MAPs in Australia. Limited patient awareness and understanding of the nature of access programmes indicate a need for improved education and consent processes from treating specialists regarding these programmes. At the very least, patients should be adequately informed about the source of drug prescriptions. Additional details of the access programmes can be tailored to each individual patient.

The limitations of this study include its small sample size, patients being from an only English-speaking background and the exclusive focus on the cost-free ribociclib access programme, which may limit generalisability to other types of access programmes not funded or require cost-sharing by patients. As the population of this PRO substudy was limited to patients who were still receiving ribociclib, there may be a bias towards patients with preferable outcomes and positive experiences with both the medication and the programme.

Although not specifically explored in our study, it is well recognised that ethical issues surround access programmes, including inequities in patient and clinician awareness of the availability of these programmes, lack of transparency and potential discontinuation of medicine supply after the cessation of a programme.[4] These issues raise concerns about equitable access to new medications and the need for greater transparency and accountability in the operation of access programmes. It is important to address these ethical challenges to ensure fair and equal access for all eligible patients.

Several recommendations can be made to improve the practice of access programmes for patients. First, improving the consent process for MAPs is essential to enhance patient awareness of the access programmes they are enrolled in. To achieve this, the consent process should involve a comprehensive discussion between healthcare providers and patients. Important elements to be discussed include a clearer explanation of the medication's source (pharmaceutical company or MAP), the purpose of the MAP, potential costs, treatment alternatives, benefits and risks.[5] Patients should be informed about the voluntary nature of their participation and the right to withdraw at any time. By enhancing the consent process, patients can make informed decisions about their treatment and participation in these programmes, which our research has demonstrated may enhance patient adherence with treatment.

In addition, engagement between pharmaceutical companies and stakeholders is crucial to address the ethical concerns surrounding MAPs.[4,6] Transparent and explicit communication of the values, goals and

eligibility criteria of these programmes can help build trust and ensure equitable access to medications.[4,6] Clear accountability mechanisms should be established to monitor and evaluate the outcomes of access programmes, ensuring that they align with their stated objectives.[4]

In recent times, promising initiatives have been developed to improve awareness and streamline clinician access to various MAPs. An example of this is the Medicines Access Portal, which was launched by Medicines Australia and Rare Cancers Australia. This is a secure online platform designed to facilitate communication between pharmaceutical companies and Australian medical practitioners regarding special-access programmes for patients. The Medicines Access Portal provides clinicians with streamlined access to information about available access programmes, including details about the type of cancer, treatment options and contact information for pharmaceutical companies. It represents a collaborative effort involving various stakeholders, including medical professionals, regulatory bodies and industry representatives, to enhance patient care and accessibility to critical medicines.[7,8]

Implementing the foregoing recommendations would contribute to the overall improvement of MAPs by enhancing transparency, patient safety and the generation of real-world data. Furthermore, these measures may address some of the ethical concerns surrounding MAPs and ensure equitable access to novel treatments for patients to whom they may be otherwise unavailable.

In conclusion, while patient awareness of the ribociclib MAP was limited, most patients expressed gratitude for access to non-government-funded medications and suggested improved medication adherence in the context of the access programme. Enhancing patient education and consent processes can empower patients and improve their understanding of what these programmes entail, leading to better treatment experiences and outcomes. The ongoing development of new applications such as the Medicines Access Portal is aimed at streamlining the process for prescribers and their patients and increasing awareness of and engagement in MAPs. Future research should focus on evaluating long-term PROs and exploring the impact of MAPs on health-related quality of life for all patients in Australia.

References

1 Vivot A, Jacot J, Zeitoun JD, Ravaud P, Créquit P, Porcher R. Clinical benefit, price and approval characteristics of FDA-approved new drugs for treating advanced solid cancer, 2000–2015. *Ann Oncol* 2017; **28**: 1111–6.

2 Wong V, de Boer R, Baron-Hay S, Blum R, Boyle F, Chua S *et al*. Real-world outcomes of ribociclib and aromatase inhibitor use in first line hormone receptor positive, HER2-negative metastatic breast cancer. *Clin Breast Cancer* 2022; **22**: 792–800.

3 Pharmaceutical Benefits Scheme. Ribociclib with an aromatase inhibitor for metastatic breast cancer – Pharmaceutical Benefits Scheme [Internet]. Pharmaceutical Benefits Scheme; 2020 [cited 2024 Jan 3]. Available from URL: https://www.pbs.gov.au/info/industry/listing/participants/public-release-docs/ribociclib

4 Grover P, Babar ZUD, Oehmen R, Vitry A. Medicines access programmes to cancer medicines in Australia and New Zealand: an exploratory study. *Health Policy* 2018; **122**: 243–9.

5 Lewis JRR, Lipworth W, Kerridge I, Doran E. Dilemmas in the compassionate supply of investigational cancer drugs. *Intern Med J* 2014; **44**: 841–5.

6 Council of Australian Therapeutic Advisory Groups. *Managing Medicines Access Programmes. Guiding principles for the governance of Medicines Access Programmes in Australian hospitals*. Version 2. Council of Australian Therapeutic Advisory Groups; 2018.

7 Australian Rare Cancer Portal. Introducing Medicines Access Portal [Internet]. ARC Portal; 2022 [cited 2024 Jan 3]. Available from URL: https://www.arcportal.org.au/news/58/introducing-medicines-access-portal

8 Medicines Australia. MAP offers new online pathway for accessing cancer treatments – Medicines Australia [Internet]. Medicines Australia; 2022 [cited 2024 Jan 3]. Available from URL: https://www.medicinesaustralia.com.au/media-release/map-offers-new-online-pathway-for-accessing-cancer-treatments

Appendix I

KARMA patient-reported outcomes questionnaire

The first part of the interview is to gauge your understanding of how you were prescribed this medication at the start.

1 When your doctor first prescribed ribociclib/Kisqali, did you understand that the drug was prescribed:
- **a** Through a clinical trial
- **b** Through an access programme funded by the drug company
- **c** By government funding
- **d** Not sure

2 Only if patient answers (b) to Q1:
How would you rate your understanding of the ribociclib access programme?
a I have a good understanding
b I know a bit about it
c I do not really understand it

3 How did you receive your medication in the beginning while you were on the programme?
a Hospital pharmacy
b Outside pharmacy
c It was posted to you
d Cannot recall

Ribociclib was approved by for government subsidy in patients with advanced hormone-sensitive breast cancer from June 2018. Prior to that, patients could access the drug through a drug company-funded access programme. This was provided at no cost to patients.
In light of what you have just been told (if you did not know before),

4 How do you feel about having been part of a medicine access programme in Australia?
a I feel fortunate to have access to a drug I would otherwise not have been able to receive
b I feel a bit like a guinea pig in a research study
c I feel guilty that not all patients in my shoes have access to this drug

5 Has being a part of an access programme like this changed the way you take your medication?
a I am more likely to be compliant with the medication
b I am less strict about taking the medication
c It does not affect the way I take the medication

The next part of the interview will ask questions about your experience with taking ribociclib/Kisqali.

6 How would you describe your overall experience with the medication ribociclib?
a Excellent
b Good
c Average
d Poor
e Very poor

7 Have you had to stop taking ribociclib for a period of time due to side effects?
a Yes
b No

If yes – please elaborate.

8 Have you had to stop taking ribociclib for any other reason?
a Yes
b No

If yes – please elaborate.

9 In the last week, has ribociclib interfered with your quality of life?
a Yes
b No

10 In the last week, has ribociclib interfered with you family life or social activities?
a Yes
b No