Health Policy xxx (2017) xxx–xxx

Contents lists available at ScienceDirect

Health Policy

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

Medicines access programs to cancer medicines in Australia and New Zealand: An exploratory study

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ARTICLE INFO

Article history:
Received 8 December 2016
Received in revised form 4 December 2017
Accepted 8 December 2017

Keywords:
Pharmaceutical policy
Compassionate use
Cancer medicines
Medicines access programs

ABSTRACT

Medicines Access Programs (MAP) offer access to publicly unfunded medicines at the discretion of pharmaceutical companies. Limited literature is available on their extent and scope in Australia and New Zealand. This study aims to identify MAPs for cancer medicines that were operational in 2014-15 in Australia and New Zealand and describe their characteristics. A preliminary list of MAPs was sent to hospital pharmacists in Australia and New Zealand to validate and collect further information. Pharmaceutical companies were contacted directly to provide information regarding MAPs offered. Key stakeholders were interviewed to identify issues with MAPs. Fifty-one MAPs were identified covering a range of indications. The majority of MAPs were provided free of charge to the patient for medicines that were registered or in the process of being registered but were not funded. Variability in the number of MAPs across institutions and characteristics was observed. Australia offered more MAPs than New Zealand. Only two of 17 pharmaceutical companies contacted agreed to provide information on their MAPs. Eight stakeholder interviews were conducted. This identified that while MAPs are widely operational there is lack of clinical monitoring, inequity to access, operational issues and lack of transparency. Our results suggest a need for a standardised and mandated policy to mitigate issues with MAPs.

1. Introduction

Granting access to new cancer medicines is a growing challenge for pharmaceutical insurance institutions because of the high cost of these medicines [1]. Both Australia and New Zealand have implemented national medicines policies that aim for equitable and sustainable access to medicines [2]. Funding decisions are based on a rigorous value for money assessment that ensures subsidised access to medicines that have been estimated to be cost-effective. However, concerns have been raised on the delays in the regulatory approval and funding of new cancer medicines in these two countries compared to similar countries in Europe and the United States of America [3–5]. At the same time, the rise of targeted, individualised cancer medicine, promoted by mass media and social media campaigns has increased the demand for access to new cancer medicines.

Outside clinical trials, compassionate use programs may be the only way for cancer patients to access expensive, new cancer medicines that are not yet approved by regulatory authorities or funded by a government. Internationally, there is no agreed terminology or nomenclature for compassionate use programs. In Australia, Medicine Access Programs (MAP) is an umbrella term for programs made available at the discretion of pharmaceutical companies that supply new and publicly unfunded medicines to patients either free of charge or at a reduced cost (Fig. 1) [6]. These programs facilitate access in various situations such as the use of unapproved medicines, off-label use (use in a non-approved indication), lack of access to clinical trials (no active clinical trials are available or the patient does not meet the eligibility criteria), or 'bridging' time between the end of the clinical trial development, regulatory approval, funding recommendation and listing. They may also allow a pharmaceutical company to supply an approved but publicly unfunded medicine to prescribers within certain parameters. In Australia, pharmaceutical companies can offer free medicines following registration in a Product Familiari-

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https://doi.org/10.1016/j.healthpol.2017.12.004
0168-8510/

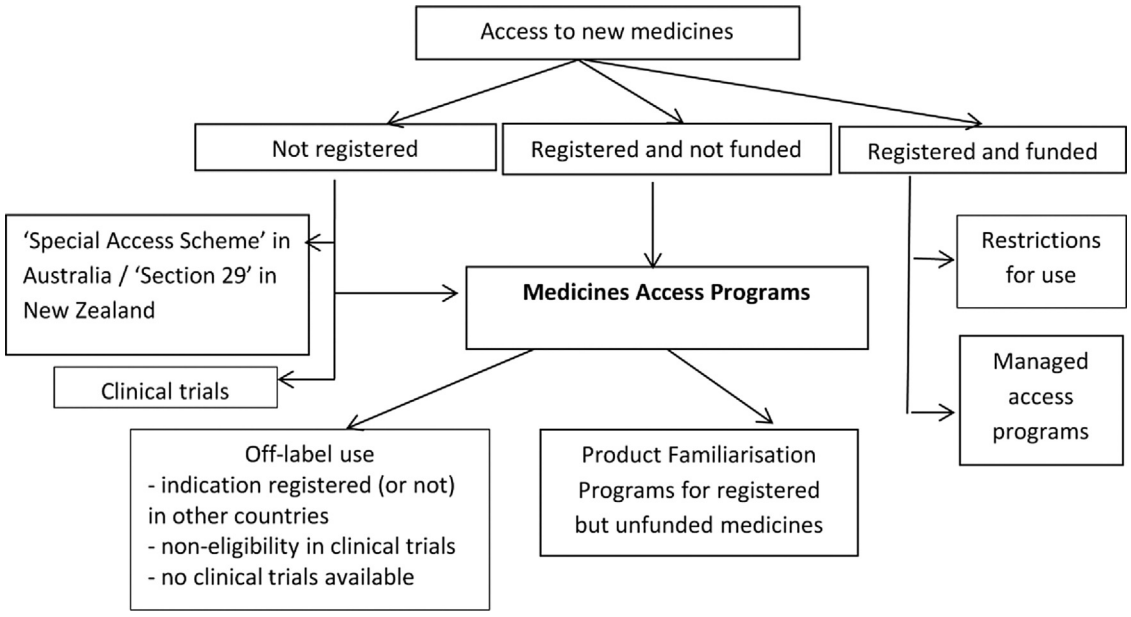

Fig. 1. Access to new medicines in Australia and New Zealand.
Note: the Special Access Scheme in Australia and Section 29 in New Zealand regulate access to unapproved medicines.

sation Program which allows a health care professional to enroll a maximum of ten patients in the program [7].

MAPs may have both benefits and risks. The patient may benefit from an otherwise inaccessible medicine and the pharmaceutical company may get to promote its medicine, develop a future market share and lobby to obtain a successful recommendation for funding. However, critics argue that MAPs are usually not sustainable, create equity issues for those unable to get access to the program or cannot afford the costs that may be incurred, and there is the potential for inadequate enrolment of patients [6]. New medicines do not have a well-established safety profile, may have uncertain clinical benefit and carry a risk of toxicity [8]. However, since MAPs in Australia and New Zealand are not required to be formally monitored, unlike clinical trials, there may be limited or no systematic recording of health outcomes and adverse drug events.

Concerns have also been raised that some of these programs may be accessed by cancer patients who are desperate and vulnerable [9]. These programs may have a significant financial cost for the patients, provide questionable tangible benefits and place the patient at risk of severe toxicity. In 2015, Australian oncologists reported discussing unfunded cancer medicines with an average of 2.5 patients per month and prescribed them to an average of 0.9 patients per month [10]. Furthermore, it was observed that oncologists were prepared to recommend a cancer medicine that would cost their patients an average of AUD$ 9395 per each additional month of survival [11].

There is limited literature on the extent and scope of MAPs in Australia and New Zealand. A report funded by Medicines Australia (the peak body representing pharmaceutical companies in Australia) suggested that the MAPs for cancer medicines were widespread. It reported this from a sample of nine pharmaceutical companies in Australia that provided 28 cancer medicines via MAPs to more than 4700 patients in 2011–2012 [12]. However, it did not list the names of these medicines or the characteristics of those programs; nor did it gauge stakeholders' perceptions and experiences of these programs. The objectives of the current exploratory study were to establish a list of MAPs and their characteristics (types of cancer/patient population covered, patient co-payment and the number of patients enrolled) in Australia and New Zealand in the 2014-15 period. The stakeholders' perceptions of MAPs were also investigated by surveying key stakeholders in the provision of MAPs in these countries.

This study focused on formal MAPs organised by pharmaceutical companies which catered to more than one patient at a time. Information on one-off compassionate supply requests was not sought.

2. Methods

2.1. List of MAPs for cancer medicines

A list of MAPs to cancer medicines and their respective characteristics in Australia and New Zealand was established through a survey of hospital pharmacists working in the area of oncology and a survey of pharmaceutical companies that market cancer medicines.

2.1.1. Development of a preliminary list of MAPs

A preliminary list of MAPs available in Australia and New Zealand was developed using a literature review of published and unpublished reports, approaching key informants likely to have information on these programs and reviewing applications for new cancer medicines made to the Pharmaceutical Benefits Advisory Committee (PBAC) (which examines applications for funding in Australia) for subsidy between July 2013 and June 2015. The informants (oncologists, oncology pharmacists, members of drug and therapeutics advisory committees) were identified through the research team's professional contacts. They were subsequently sent an email to explain the project and invited to provide information on MAPs. The objective of this step was to identify a preliminary list of cancer medicines that were offered through MAPs rather than focus on their characteristics.

2.1.2. Survey of hospital pharmacists

The preliminary list of MAPs was formulated into an online questionnaire using SurveyMonkey®. The preamble explained that the focal point of the survey was on formal programs offered to more than one patient at a time rather than a one-off supply to a particular patient. The questionnaire sought information on

Please cite this article in press as: Grover P, et al. Medicines access programs to cancer medicines in Australia and New Zealand: An exploratory study. Health Policy (2017), https://doi.org/10.1016/j.healthpol.2017.12.004