Managed Access Programmes: a powerful source of real-world insight

MAPs represent a unique opportunity to gather evidence from a cohort of patients receiving the same treatment, across multiple countries prospectively before launch

Managed Access Programmes' (MAP) primary objective is to provide treatment for patients with unmet medical needs who cannot get access via a clinical trial to an investigational product which is not commercially available. A MAP utilises country-specific regulatory mechanisms (compassionate use, expanded access, named patient) that exist to allow the legal supply of such medicines.

Frequently, the number of patients treated in a MAP is significantly higher than the number of overall patients treated in the clinical trials for that medicine. These programmes therefore offer a unique opportunity to collect real-world data before launch, providing evidence to support the product's effectiveness, safety and value.

Real-world evidence can be collected retrospectively or prospectively. The former involves interrogating databases containing patient data collected by health insurances, hospitals and national health services. These data sources present issues that are not easy to overcome, namely, the data is incomplete and may lack crucial information, patient data may be duplicated and patient treatment may vary too much to be able to draw conclusions. Prospective collection involves, for example, observational studies which are initiated after a product has been launched. At the time of the reimbursement decision, payers increasingly ask for more evidence of cost-effectiveness that applies to the real world but, usually, this evidence is not yet available as an observational study may be still in its planning stages.

This is why MAPs represent a unique opportunity to gather evidence from a cohort of patients all receiving the same treatment, across multiple countries prospectively before launch. In the months or years that it takes for a reimbursement decision to be made in a country, data can be collected ready to be provided when requested.

It is frequently argued[1] that the pharmaceutical industry is concerned about collecting data believing that new safety information could potentially derail the marketing authorisation. However, we have seen many examples where data has confirmed a positive safety profile in a larger population treated under a MAP[2,3,4], and in our experience, companies who have decided not to collect data usually regret that decision. Moreover, the FDA reviewed the Expanded Access Programs approved between 2005 and 2014 and only in two instances of the 1,033 programmes (ie 0.2%) was a clinical hold imposed for safety[5] reasons. Finally, many scholars have argued that the current situation of inconsistent and minimal reporting represents a missed opportunity[6].


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We have received reports of data from MAPs being used to assist in demonstrating product value as supportive data in health technology assessments. Even simple points such as duration of treatment, reason for discontinuation, dosage, safety and treatment adherence provide a valuable source of information on the usage of a product when the constraints imposed by a clinical trial protocol are lifted.

Commercial teams understand the value that real-world data provides and how it can bridge any gaps in data collected during clinical trials. We agree that closer collaboration of the commercial function with the scientific teams will increase the way biopharmaceutical companies leverage data resulting in more impactful outcomes and sustained value[7].

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6 See (2)

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