Research themes for the pharmaceutical sector

Macquarie University's Centre for the Health Economy (MUCHE) was established to undertake innovative research on health, ageing and human services. Our vision is to create a world where decision makers are empowered with applied, trusted and influential research into health and human services policy and systems.

MUCHE has partnered with five pharmaceutical companies to develop a unique and innovative Higher Degree Research (HDR) program (the Program) to evaluate questions relevant to the pharmaceutical sector. These partners include Amgen Australia, Janssen Australia, MSD Australia, Pfizer Australia, and Roche Australia.

The Program is one of a kind in Australia, aimed at stellar students willing to undertake research in three broad areas.

- Pharmaceutical policy research themes, such as access to medicines and reimbursement.
- Medicine management research themes, such as medication adherence and choice.
- Advancement of analytical method, such as using real world data to better inform government policy decisions.

While specific research questions are determined by the student and supervisor, both have the opportunity to draw on outcomes from an annual pharmaceutical sector research workshop organised and facilitated by MUCHE. This paper describes outcomes from the inaugural workshop on research themes for the pharmaceutical sector.

Workshop outcomes

MUCHE held a half day workshop in November 2017 at Macquarie University to discuss and debate potential relevant pharmaceutical sector research themes for HDR students to consider when undertaking a Masters of Research or a PhD within the Program.

Participants included representatives from Macquarie University, the pharmaceutical and self-medication industries, federal government, health non-government organisations and patient advocates.

The workshop was facilitated by Dr Henry Cutler (Director, MUCHE). Participants were asked to raise and discuss research ideas that would contribute to public debate, assist government and NGOs in decision-making, and aid in formulating strategy and policy within the pharmaceutical sector. Participants were also asked to consider the need for achievable research questions in terms of data and time constraints.

Seven main research themes emerged from the workshop.

1. Medicine choice among consumers and GPs
2. Post-market surveillance of medicine outcomes.

3. Outcomes based payments within the pharmaceutical sector.
4. Health equity, social justice and need.
5. Impact of medicines on broader economic outcomes (e.g., productivity and informal care).
6. International competitiveness of the Australian clinical trials sector.
7. Value of patient support programs delivered by pharmaceutical companies.

Each of these research themes are further discussed below, along with potential research questions that could be explored within a Masters of Research or PhD under the Program.

1. Medicine choice among consumers and GPs

Consumer choice has long been a rich vein of research in economics, and has made a significant contribution to our understanding of how individuals function within society.

Many consumer choices affect health outcomes. Some of these relate to medicines – decisions around when to seek advice, who to seek advice from, whether to follow that advice, and how to make choices within an often confusing environment. GPs offering advice must also make choices over treatment regimes, often within a complex and ever changing environment.

Given the shift towards consumer directed care within health and human services, better understanding choice is of growing importance in a connected, information-rich world.

Potential research questions include:

- How do consumers choose and use over-the-counter, complementary and prescription medicines?
- What factors influence a consumer's decision to fill a prescription?
- What factors influence a consumer's decision on whether to adhere and persist with medication?
- How does health literacy impact consumer and GP choice over medicines?
- How can public summary documents, drug labels and consumer medicine leaflets be better understood by consumers, and what are the implications for choice?
- Hoe does social media impact consumer choice?

2. Post-market surveillance of medicine outcomes

Pharmaceutical companies traditionally make large investments in post-market surveillance, which monitors the safety of medicines outside the closed arena of clinical trials, and which informs decisions around marketing. The Australian Government also undertakes Post Market Reviews of selected drugs to improve patient safety, better understand utilisation, and assess cost effectiveness, among other goals.

A wealth of post-market surveillance data is held by pharmaceutical companies. However, little published research has fully utilised this information to make better informed policy decisions. Such data could be analysed, and its value potentially extended upon, by linking it with other datasets held by other organisations, such as the Australian Government.

Potential research questions include:

- Are post-market reviews achieving their objectives, and could these be improved through better data

linkages with pharmaceutical company data sets?

- How can ongoing post-market surveillance be used to add value to patients, health care professionals, and the pharmaceutical sector?
- How can ongoing post-market surveillance be better used to optimise decisions around listing medicines on the Pharmaceutical Benefits Scheme (PBS)?
- How can post-market surveillance be better used to improve clinical outcomes?
  - Was the medicine clinically effective?
  - What clinician and patient behaviour impacted outcomes?
- What is the extent of off-label prescribing in Australia, and how does it benefit Australian society?

3. Outcomes based payments within the pharmaceutical sector

In the health care sector, payments are usually linked to activity under a fee for service regime. Examples include payments through the Medicare Benefits Schedule for GP and specialist services, and Activity Based Funding in the public hospital sector.

Recently, there has been growing interest by governments in linking payment to health outcomes, at least in part, to encourage value based care. It is argued that offering financial incentives for good outcomes encourages providers to improve their quality of care.

One important issue is appropriately attributing health outcomes to provider care in such a complex system as health production. Other important questions relate to designing an outcome based payments model, including whether to offer penalties or rewards, the size of incentive, and whether to use absolute or relative outcome measures.

While the Australian Government can price medicines based on future clinical outcomes (through its Managed Entry Scheme), little research has been undertaken on whether other funding models are appropriate. For example, European countries are starting to explore funding models whereby drug manufacturers are required to reimburse government for observed non-responders. Yet these types of models can be complex, with outcomes dependent on the interrelationship between medicines, patient behaviour, and advice from doctors.

Potential questions include:

- How do physician behaviours impact persistence and compliance of medicines to deliver improved health outcomes?
- What factors should be considered when developing an outcomes based payment model for medicines in Australia?
- How could an outcomes based payment model for medicines be incorporated into the current reimbursement framework in Australia?
- What are the current hurdles to measuring the performance of a medicine in Australia, including regulatory, privacy, and access to data, and how can medicine performance be separated from other factors, such as physician behaviour?
- What metrics should be used to evaluate the performance of a medicine (e.g., patient outcomes), and what part do characteristics of medicines or conditions play in the choice of metrics?

4. Health equity, social justice and need

Equity and need are important concepts in the pursuit of social justice, and drive much discussion and decisions within health policy. For example, Medicare's strong public support is based largely on its provision of equal access to general practice and public hospitals.

Yet need is contentious, with debate over the definition of need in the context of health outcomes. Similarly, equity and need may not be well defined or well understood within Australian pharmaceutical policy, potentially leading to decisions with sub optimal social welfare outcomes.

Potential questions include:

- How is equity and need defined within pharmaceutical policy, and applied within pharmaceutical sector decisions?
- How well does pharmaceutical policy in Australia align with alternative definitions of equity and need?
- What is the distribution of co-payments for medicines in Australia across various patient characteristics and is this considered equitable?
- What role does government subsidies in promoting equitable access to medicines?
- Who has benefited from recent decisions by the Australian Government to fund medicines on the PBS, and are these decisions equitable?
- What is the impact of scheduling decisions on equity of access to medicines?

5. Impact of medicines on broader economic outcomes

Economic evaluations undertaken for PBS listing use a health care system perspective, focusing on the impact of medicines on health outcomes and costs to the health care system, with less regard for broader economic impacts, such as changes to productivity and informal care.[1] Yet these are important considerations, with social welfare impacted from changes to these economic resources.

There is debate over whether informal care and productivity should be incorporated into economic evaluations for PBS listing. Some contend that productivity is implicit within health outcome measures using Health Related Quality of Life (HRQoL) tools, while others disagree. There are also concerns around the reliability of measuring productivity and informal care.

Potential questions include:

- What reliable methods are available to measure changes in productivity and informal care associated with medicine use?
- How should productivity and informal care be incorporated into decisions around pricing and reimbursement of medicines?

[1] Changes in productivity may be included in supplementary analyses in PBAC submissions, but are not included in the base-case analysis.

6. International competitiveness of the Australian clinical trials sector

Clinical trials conducted in Australia are associated with around $1 billion in annual expenditure, with around 1,300 new clinical trials starting each year. The majority of this investment comes from international organisations, with Australia experiencing strong growth in demand for clinical trial sites since 2010.[2]

Yet there is tremendous competition for the right to participate in international multicentre clinical trials. There are several factors considered by organisations when deciding the location of their trials, including capability and capacity to undertake research, market factors such as cost, and government regulation.

Australian governments have recognised this competitive environment, and the challenges in attracting investment for clinical trials. While several initiatives are being pursued by governments, a better understanding of factors that affect Australia's attractiveness as a clinical trials destination, their relative importance, and actions to capitalise on those factors, is required.

Potential questions include:

- What is the current level of investment in clinical trials in Australia?
- How has investment in clinical trials benefited Australia?
- What characteristics impact an organisation's decision to locate a clinical trial, and what are their relative importance?
- Where does Australia's competitive advantage lie in attracting clinical trials?
- What changes in government policy and regulation could be undertaken to improve our competitive advantage in attracting more clinical trials?

7. Value of patient support programs

Many pharmaceutical companies invest in patient support programs to improve medication adherence. This often includes education and training programs, along with counselling.

However, improved access to technology and the shift towards greater consumer engagement and empowerment could provide pharmaceutical companies with an opportunity to develop and test 'next generation' patient support programs.

These programs could potentially better manage conditions across the patient journey, using an integrated framework with other service offerings, and help consumers make better choices to manage their condition.

Potential questions include:

- What patient support programs are currently being offered by pharmaceutical companies in Australia?
- Have patient support programs offered by pharmaceutical companies improved health outcomes, to what extent, and how?

---

[2] MTPConnect 2017, Clinical trials in Australia: The economic profile and competitive advantage of the sector, https://www.mtpconnect.org.au/Attachment?Action=Download&Attachment_id=54, accessed 2 January 2018

- How can data be better utilised to improve patient value within a support program?
- Are incentives strong enough for pharmaceutical companies to offer ‘next generation’ patient support programs in Australia?
- How could patient support programs be incorporated within the current medicines pricing and reimbursement framework in Australia?