Scottish Government Health Directorates Chief Scientist Office

A natural experiment using Scottish clinical data to estimate the real-world effectiveness of adjuvant chemotherapy in breast cancer patients (HIPS/16/26)

Researchers
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Aim
Over 4,600 women are diagnosed with breast cancer and around 1,000 people in Scotland die from the disease every year. More than 500 patients with early breast cancer are treated with chemotherapy each year in Scotland. Half of breast cancers are in women over the age of 65, but the average age in trials is closer to 50.

However, it is not known if chemotherapy benefits seen in clinical trial populations are the same in typical NHS real-world patients. Reliable methods for estimating the benefits and harms of chemotherapy in NHS patients are required to help inform clincans and patients and to help patients make difficult decisions about whether or not to undergo chemotherapy.

This project aimed to
- Measure the benefit from chemotherapy in a real-world early breast cancer population
- Validate a commonly used web-based benefit prediction decision tool showing how treatment might improve survival
- Determine the feasibility of using different statistical methods

Project Outline/Methodology
The very high quality and pre-existing linkage of Scottish healthcare and cancer registration datasets makes Scotland an ideal place to evaluate new methods to assess the benefits of chemotherapy and opther cancer tretments in patients.

De-identfiied data from around 70,000 women with a diganosis of breast cancer were analysed using different data science methods:
- Cox Regression analysis
- Propensity Score Matching
- Instrumental Variable analysis
- Regression Discontinuity Design analysis

Adjustment was made for clincial and molecular stage, comorbidity and socioeconomic status.

Key Results
1. The NHS Predict decision tool is based on a risk model that is valid in Scottish patients.
2. It is possible to estimate treatment effect using routine data
3. The Regression Discontinuity Design did not perform well in this example
4. Patients that are not typically included in clinical trials appear to benefit from chemotherapy to a similar extent to younger fitter patients.

Conclusions
We learned that high quality Scottish healthcare data, combined with leading data science methods make it possible to understand how treatments affect patients in a real-world NHS context.

What does this study add to the field?
Methods for natural experiments looking at drug effectiveness in the real-world (as opposed to in clincial trials) using routinely collected data are embryonic. However, data opportunities are now making it possible to test and further develop methods able to look at benefits of treatment in the real-world.

Implications for Practice or Policy
Age and other reasons for trial non-eligibilty should not be a barrier to patients benefitting from chemotherapy to reduce the risk of breast cancer recurrence after curative treatment. Current decision tools based on risk models are accurate as a basis for shared decidion making in this context.

Where to next?
We are increasingly adopting new cancer treatments into the NHS before robust evidence from long-term follow-up is available. We believe that data science methods using real world data can complement evidence from clinical trials during the process of early technology adoption. We have established a Real World Data Analysis service in an attempt to make such analyses available to ensure that new treatments represent good value for NHS patients.

Further details from:
https://edin.ac/cancer-data
or
https://edin.ac/health-economics