Developing new cancer endpoints to improve patient care

As survival rates for cancer increase, clinicians and patients are increasingly interested in finding ways to measure the performance of an intervention or the effectiveness of a medication. This is the driver behind a new partnership looking at meaningful endpoints in cancer care.

The Health Economics Unit (HEU) is collaborating with Janssen, The Association of the British Pharmaceutical Industry (ABPI) and DATA-CAN: The Health Data Research UK Hub for Cancer to understand not only if cancer treatments work to save patients' lives, but also the quality of life they leave them with.

The HEU is carrying out a feasibility study, engaging with stakeholders including leading clinicians on lung cancer and multiple myeloma, data teams and NICE. It is also conducting an in-depth literature search to combine with the stakeholder feedback, helping to identify and validate new endpoints that could be used for future studies.

Using meaningful endpoints to inform patient care

Endpoints – or markers in the data to say that something has been successful or not – allow more informed decisions to be made around patient care. HEU's Head of Data & Analytics, Julia Wilkins, explains that one obvious example of an endpoint in cancer treatment is whether patients die or not.

Julia said: "A traditional endpoint for cancer therapy is asking 'do patients survive or not?' But survival rates are higher now, so if a patient is seemingly well, how can you measure the performance of an intervention like a certain drug or type of surgery?"

Defining and analysing other, more complicated endpoints, leads to more helpful outcomes.

Julia said: "If there are five different treatment options and a patient will survive using all of them, we need to work out if there are other data points that we can use to show if one is better than another."

"People are interested not just in survival, but the quality of that survival. If someone needs to decide whether they use a certain drug or have a type of surgery, we want the data to be able to show whether it can help you to have a good quality of life. Patients want this kind of data to help them make these decisions."

Adding value through experience and insight

The feedback gathered from clinicians and other stakeholders has revealed a need to identify new 'surrogate' endpoints that can be put into the data to show how patients are recovering.

The project looks to understand more about those who have survived from lung cancer and multiple myeloma – they have survived, but are they doing well? And are there potential indicators for a patient not doing well that they could pick up?

Julia said: "We've got a very experienced team and as we go through the project we're coming up with our own ideas. One of these is using machine learning to look at the data that's available and letting it tell us what the endpoints could be."

For example, the team might look at a patient that is deteriorating. By analysing the data, they can look for markers – like increased visits to hospital – to indicate if this could have been predicted, which could then be applied in future for other patients. HEU analysts are able to apply their expertise and experience in data analysis and machine learning to create meaningful insights which can be used to inform future treatments.

Julia said: “In this way, we as a team are adding value to the original proposal. We’re not just answering a question we were given, we’re adding our own thoughts and suggesting other ways that it could be done. And the principles of our work can be applied to other cancers and to other diseases.”

Informing the discussion on cancer treatment

The project is now in its feasibility phase where the team has a set of emerging endpoints and can begin exploring the data sets. From here, they will decide which would be suitable for a validation exercise.

Julia said: “We’re aiming to publish a thought leadership piece that will contribute to the adoption of novel endpoints for lung cancer and multiple myeloma. This will help guide clinical trials, help NICE in their assessment of how well drugs are doing and contribute to patient groups using information to make decisions about their own care.”