Personalized medicine leads to better outcomes for patients with cancer

6 June 2016

In a meta-analysis of hundreds of clinical trials involving thousands of patients, researchers at University of California San Diego School of Medicine report that therapeutic approaches using precision medicine, which emphasizes the use of individual genetics to refine cancer treatment, showed improved response and longer periods of disease remission, even in phase I trials.

The findings are published in the June 6, 2016 issue of *JAMA Oncology*.

After reviewing 346 phase I clinical trials involving 13,203 patients, the authors found that in precision medicine treatment arms more than 30 percent of patients responded to treatment compared to 4.9 percent of patients enrolled in non-personalized arms. Patients treated with precision medicine also benefited from longer progression-free survival with a median of 5.7 months before their disease worsened compared to 2.95 months for the others.

"Our analysis shows that in the era of precision medicine, phase I clinical trials using personalized therapy with a biomarker-based approach can do more than assessing the toxicity and side effects," said Maria Schwaederlé, PharmD, lead author and UC San Diego Moores Cancer Center researcher at the Center for Personalized Cancer Therapy. "These early trials can result in improved outcomes for patients, even among people whose disease is resistant to standard treatments, by selecting patients who will respond best using a personalized approach from the start."

The study involved 58 precision medicine treatment arms and 293 that did not. Personalized arms led to improved outcomes across tumor types. The use of a personalized approach was associated with higher response rate of 24.5 percent in patients with solid tumors compared to 4.5 percent in non-personalized strategies. Similarly, blood cancers had a 24.5 percent response rate compared to 13.5 percent. In both tumor types, using precision medicine gave patients a longer progression-free survival (4.1 versus 2.8 months in solid tumors and 13.6 versus 4 months in hematologic tumors).

In a sub-analysis of 234 arms testing targeted drugs, the authors found that using biomarkers to assign patients to treatments led to response rates of 31.1 percent compared to 5.1 percent for those that did not. This shows the importance of pairing targeted therapy with a biomarker.

Another sub-analysis of the precision medicine trials showed that while the use of both genomic and protein biomarkers improved outcomes, genomic biomarkers performed better. Targeting genomic alterations resulted in a 42 percent response rate compared to a 22.4 percent response if the biomarker was directed at a protein overexpression.

"What we observed is that phase I trials can serve both to inform us on the effectiveness of new therapies as well as identify patients likely to benefit most if a personalized approach is employed," said Razelle Kurzrock, MD, director of the Center for Personalized Therapy and Clinical Trials Office at Moores Cancer Center and senior author of the paper. "Another important point is that targeted drugs in and of themselves are often quite useless if not combined with a patient's individual tumor biomarkers to determine whether they are likely to benefit from a particular therapy."

More information: Maria Schwaederle et al, Association of Biomarker-Based Treatment Strategies With Response Rates and Progression-Free Survival in Refractory Malignant Neoplasms, *JAMA Oncology* (2016). DOI: 10.1001/jamaoncol.2016.2129

Provided by University of California - San Diego