

CCLG: The Children & Young People's Cancer Association research: Learning to predict high-risk rhabdomyosarcoma at diagnosis to improve outcomes for children and young people

Project title: Defining molecular markers of high-risk in rhabdomyosarcoma cells and their tumour microenvironment to improve patient outcomes

Project stage: Complete (ended December 2025)

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Led by: Professor Janet Shipley, The Institute of Cancer Research



About the project

Doctors separate rhabdomyosarcoma patients into different risk groups so that they can give the right amount of treatment. This is based on where in the body the cancer is, its size, and whether the cancer cells have a fusion between genes, called PAX3 or PAX7, and FOXO. However, there is currently no way to determine how at risk most patients are if they do not have one of these fusion genes.

In this project, Professor Janet Shipley at The Institute of Cancer Research will look for a way to predict risk for PAX-FOXO1 fusion-negative rhabdomyosarcoma at diagnosis. Her research team has previously found evidence that tumours with low oxygen levels, few blood vessels, and high amounts of certain genes are linked to poorer outcomes. They have identified biological markers representing these features which can be simultaneously tested in samples from patients that were used for diagnosis and are available for study.

The researchers will map the markers onto over 100 rhabdomyosarcoma tumour samples. The data from this, such as how the markers are distributed in the tumour and the amount, will be compared to patients' responses to treatment. The team will be using state-of-the-art technology and AI to analyse this data and identify the best prognostic markers.

After validation, Professor Shipley plans to include the best markers in the current FaR-RMS clinical trial. She hopes that this work will go on to spare low-risk rhabdomyosarcoma patients unnecessary toxicity, and improve the treatment options for high-risk patients.

Results

The team analysed tumour samples from more than 100 rhabdomyosarcoma patients using advanced

imaging and computer-based analysis. They found that the way blood vessels are organised within the tumour is an important predictor of outcome. Tumours where blood vessels were more tightly grouped and less varied in structure were linked to poorer survival.

The researchers also developed a new AI-based tool to analyse these patterns in tumour tissue. This could make it easier to identify the features this project linked to patient risk. Overall, the study could help improve how patients with fusion-negative rhabdomyosarcoma are assessed at diagnosis.

What's next?

Professor Shipley's team will continue to analyse the large set of imaging data to look for additional markers that could further improve risk prediction. They will also investigate how the immune environment within tumours may influence patient outcomes.

These findings will now be further tested as part of a European sarcoma clinical trial to see if they can improve current risk stratification methods. If effective, this could lead to improvements to patient care and help doctors give the most appropriate treatments.



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