

# CCLG: The Children & Young People's Cancer Association research: New treatments for relapsed rhabdomyosarcoma

**Project title:** Novel models and new treatments for relapsed rhabdomyosarcoma patients

**Project stage:** Complete (ended May 2025)

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



## About the project

Of all the children diagnosed with rhabdomyosarcoma, a third will have their cancer spread or return after their initial treatment (otherwise known as a relapse). Unfortunately, these patients often have a low chance of survival and need more effective treatments for their relapsed rhabdomyosarcoma.

Scientists use disease models to assess whether new treatments work or to develop new treatments. For rhabdomyosarcoma, these models might be the same as the tumour genetically, physically or experimentally. This project aims to develop and test new models of rhabdomyosarcoma in order to find better treatments for relapsed patients. The research team at The Institute of Cancer Research, led by Professor Janet Shipley, will use tumour samples taken from relapsed patients in European countries enrolled onto the existing FaR-RMS international clinical trial for rhabdomyosarcoma. Cancer cells will then be grown flat in petri dishes, as well as in 3D and in zebrafish, as models of relapsed rhabdomyosarcoma.

These models will be used to screen and test available drugs, such as regorafenib, which is being introduced for relapsed patients into the FaR-RMS trial. The new drug will be used alongside traditional chemotherapy in the new models at the same time as they are being used to treat patients. This means that the models' reaction can be compared to patients' tumours' reactions so that the researchers can see which models are most like real tumours.

This study will try to identify potential treatments for further investigation in the models. Together with molecular characterisation of these models, this will provide better understanding of the biology of relapsed rhabdomyosarcoma that respond to particular drugs. From the models, tumour biomarkers can be found that show how a tumour is responding to treatment. Prof Shipley hopes treatments can be identified for children with relapsed rhabdomyosarcoma that could be introduced into the FaR-RMS trial.

## Results

The project has resulted in multiple new lab models for relapsed rhabdomyosarcoma, including ones developed from ascites (a fluid that can collect in some patients abdomens because of the cancer). Using different sources of rhabdomyosarcoma cells to develop the models helps researchers investigate

different stages of the cancer, and ensure that new treatments are able to fight rhabdomyosarcoma cells throughout the body.

One of the new models grew rhabdomyosarcoma cells in fish to show how things like blood vessels and immune cells are arranged inside tumours. The team tested treatments on this model and saw that different components of the tumour reacted differently to treatment. This showed how some rhabdomyosarcoma tumours can adapt to treatment, acquiring new characteristics that help them survive.

### What's next?

The team are currently investigating their models at the molecular level to ensure that they are good representations of the patient samples used to develop them. They are also looking at genes that can be linked to relapse and treatment resistance.

They have already found a genetic marker and mechanism that can be linked to resistance and have already applied for funding to study this further. They hope that this finding could form the basis of a targeted treatment.

Researchers at the Institute of Cancer Research have also applied for funding to use the team's models to test how sensitive patients cancer cells are to treatment in real time. If successful, this approach could be used to highlight promising treatment options for individual patients within the next few years.



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