

# Investigating clinical trial samples to predict how atypical teratoid rhabdoid tumours will respond to treatment

**Project title:** Molecular Biological Profiling for the SIOP-ATRT-01 Trial

**Lead researcher:** Dr Daniel Williamson, Newcastle University

**Project Stage:** Ongoing (started March 2023, planned end February 2026)

**Funded by:** Joel Prince Starlight Fund and Our Buoy Hugo's Fund

## ABOUT THE PROJECT

Atypical teratoid rhabdoid tumours (ATRT) are a rare but aggressive type of childhood cancer that starts in the brain or spinal cord. Patients are usually very young and current treatments are often ineffective or have a high risk of damaging long-term effects. For many other childhood cancers, biological profiling is used to find out which patients are likely to respond to well to treatment and those who won't. In this way, doctors can avoid using too strong treatments that have lots of side-effects on patients that are low-risk, and find which patients need different and newer treatments. Biological profiling has not yet been used for children with ATRT, in part because of the rarity of the disease and the lack of clinical trials.

Dr Daniel Williamson's lab team at Newcastle University, along with others, have shown that there might be a link between a tumour's genetics and its response to therapy by analysing samples from ATRT patients. Using state-of-the-art genetic testing that looks at tens of thousands of genes and their expression, the researchers found at least three subtypes of ATRT which appear to affect patient survival.

Using the genetic testing results, the team could then use artificial intelligence techniques to find biological patterns which, when a child is diagnosed, could predict how likely they are to respond to treatment. Whilst these techniques are promising, researchers have not been able to gather enough evidence and data yet because of the rarity of ATRT.

Alongside the new international SIOP-ATRT-01 clinical trial, Dr Williamson will use the state-of-the-art genetic testing to analyse the UK patients' samples. This will create a valuable set of data that includes differences in tumours' molecular biology and how the patients responded to standardised treatments. Teams in other countries will also be using the same techniques to create datasets, which will be pooled at the end of the trial. Dr Williamson hopes that the internationally pooled data will help show whether biology predicts response to therapy, and therefore can be used to direct future treatments for patients with ATRT.

## PROGRESS

Whilst waiting for the clinical trial to open fully, and to receive the new cancer samples from enrolled patients, Dr Williamson has recruited and trained a new team member. Rose Bailey has now been working on the project since October 2023, and has been trained to handle tissue samples, analyse genetic data, and manage databases.

The data gathered in this project will help inform a prognostic model that could tell doctors how a patient would respond to treatment. Dr Williamson's team has been working to increase the amount of data included in the model. The researchers have added more information about ATRT patients to the study, and collaborated with another scientist to increase the genetic data included in the model. Finally, they have been going through all of the ATRT samples collected in recent years. This means that both old and current patients will have access to high-end genetic analysis.

## WHAT'S NEXT?

In the coming year, the team will start receiving samples from the clinical trial, and can begin their analyses.

In the longer term, the researchers already have plans to build upon and continue this work. Dr Williamson has submitted two grant applications, one of which will undertake more work alongside the SIOPE-ATRT-01 clinical trial. The other project will improve the pre-clinical (lab based) testing of medicines for ATRT.



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