

A living review of new treatments for children and young people with relapsed and refractory rhabdomyosarcoma



Project title: Living-REFoRMS - A living systematic review of early phase studies for children and young people with relapse and refractory rhabdomyosarcoma

Lead researcher: Dr Jessica Morgan, University of York

Project Stage: Ongoing (started July 2022, planned end August 2025)

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ABOUT THE PROJECT

New treatments are continuously being developed for rhabdomyosarcoma that has not responded to initial therapy (refractory) or has come back after treatment (relapsed). Keeping track of new developments so that the most accurate information is used to make treatment choices is a particularly difficult for families and clinicians.

A recent CCLG-funded study called REFoRMS included a systematic review of treatments for children and young people with relapsed and refractory rhabdomyosarcoma. Systematic reviews are a form of research where all previous studies in an area are found and assessed before the results are combined to summarise the best evidence available at that time.

Dr Jessica Morgan's research team at the University of York plan to develop a living systematic review of studies into new treatments for relapsed and refractory rhabdomyosarcoma called Living-REFoRMS. Living systematic reviews are a new method where the evidence is regularly searched, assessed, and summarised so that the information is the most up-to-date that it can be. This living systematic review will look for completed research and currently open trials from all around the world. The researchers also want to develop a high-quality, regularly updated, online resource to share the findings from Living-REFoRMS that will be accessible to families and clinicians, and will help and support decision making in relapsed and refractory rhabdomyosarcoma. The online resource will be developed with and hosted by CCLG, so that families and clinicians only have to check one place instead of searching through many. The project will include a project steering group of families with experience of relapsed and refractory rhabdomyosarcoma, and professionals with expertise in this area, who will meet regularly to advise the researchers on this project.

PROGRESS

The team have now completed four updates of Living-REFoRMS, identifying 166 published reports of early phase studies involving over 1,300 children with relapsed or refractory rhabdomyosarcoma (RMS). You can read all of the reports here: tinyurl.com/REFoRMS-projects

Whilst most studies didn't include many RMS patients, therefore making it difficult to say if their intervention would work for this patient group, the third update identified a promising new treatment combination with an overall response rate of 63%, and a disease control rate of 91%.

The researchers also began work on the online resource for parents and professionals, holding a workshop in January to gather thoughts from around 30 parents plus clinicians and researchers. From this, the team have developed a detailed plan for the resource.

WHAT'S NEXT?

The key goal this year is to finish development of the online resource. The team will also be working on publishing scientific papers to share their methods and results, and plan two new updates within the next year. With one year left on their funding, they are also looking for funding to ensure the continuation of Living-REFoRMS.



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