The REFoRMS Project

Authors

Jess Morgan, Lucy Beresford, Connor Evans, Gemma Bryan, Helen Fulbright, Robert S Phillips

Affliations

Centre for Reviews and Dissemination, University of York, UK; University of Surrey, UK; Hull York Medical School, UK, Leeds Teaching Hospital, UK

REFoRMS is an overarching programme of work with multiple workstreams that aim to understand the decision-making process for families of children and young people with relapsed and refractory rhabdomyosarcoma.

Systematic Review

Aims

To conduct a systematic review of interventions evaluated in early phase trials for paediatric relapsed & refractory rhabdomyosarcoma.

Methods

Databases & trial registries were searched in June 2021. Records were screened in duplicate. Eligible studies explored interventions aimed at disease control **in patients <18 years old with R+R RMS**, and were performed after 2000. Quality assessment used the Downs and Black checklist. Narrative synthesis was performed.

Results

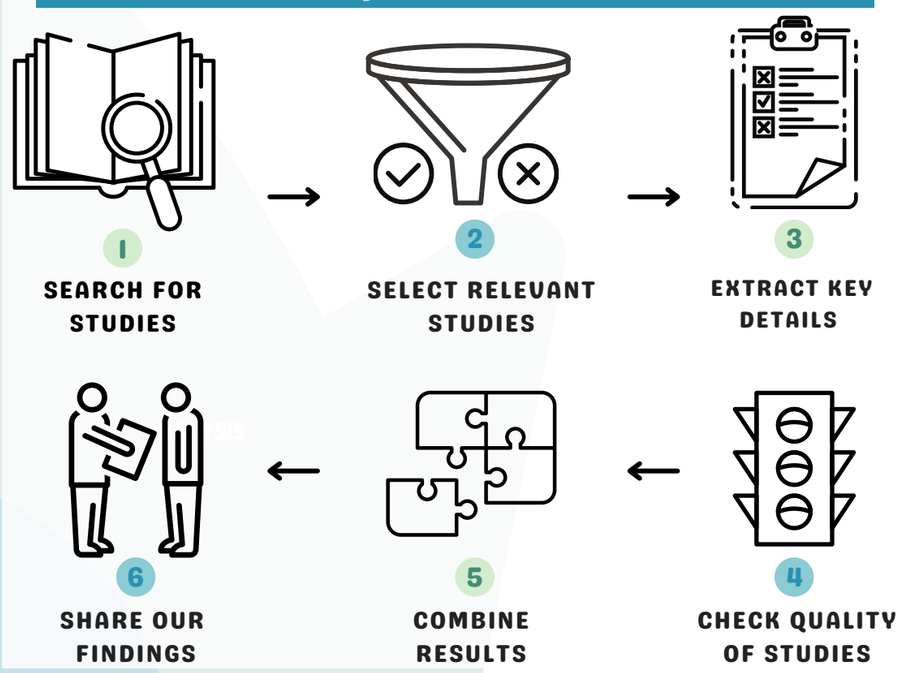
We screened 16,965 studies, and included X full text studies including over **1,100 children and young people with R+R RMS.** We included studies evaluating standard chemotherapies (n = X), novel agents (n = X, including X studies looking at targeted therapies), local therapies (n =), HSCT (n =), cellular therapies (n =) and vaccines (n =). 191 trial registry records of 169 unique studies were also available, including **62 currently active trials**. Overall, poor response rates were seen across the interventions evaluated. Adverse events vary dependent on the intervention.
Study quality was poor, and there were issues with inconsistent reporting. More granular data by tumour type would have aided further analysis.

Qualitative Study

Families need to make difficult decisions about which options to seek for their child with relapsed or refractory disease and how to prioritise their care.

This project will explore how parents or guardians make treatment decisions for children and young people with relapsed/refratory disease, what factors influence their decisions and how the patients themselves are involved in the decisions.

What is a systematic review?



Living REFoRMS

Living REFoRMS will be the **first living systematic review in childhood cancer** (starting Autumn 2022)

Living REFoRMS will continue the work conducted in the original review by updating the evidence regularly. This will help to create an up-to-date online resource on early phase studies for researchers, clinicians and families.

As researchers, we'll work on the best methods to efficiently screen, extract and synthesise data, so this project could be reproduced in other tumour types.

Local REFoRMS

We identified several **retrospective studies** evaluating the effectiveness of local therapies in children with rhabdomyosarcoma (including brachytherapy or AMORE).

Looking at the available evidence on local therapies was important to our parent advisory group; so we're in the process of collating the available evidence on local therapies for patients with R+R RMS.

The REFoRMS Parent Group

The REFoRMS parent advisory group have been invovled throughout this project, have helped prioritise outcomes, and will help determine the best ways to share our results with families of children and young people with rhabdomyosarcoma.

1. Downs, S.H. and Black, N., 1998. The feasibility of creating a checklist for the assessment of the methodological quality both of randomised and non-randomised studies of health care interventions. JECH, 52(6), pp.377-384.

@REFoRMS_Rhabdo

reforms-project@york.ac.uk