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Elicitation of expert prior opinion: application to the mypan trial in childhood polyarteritis nodosa

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Introduction

A major challenge in rare diseases is conducting clinical trials with sufficient power to inform best clinical practice when anticipated sample sizes are small. Historically, this has been a major barrier in rare paediatric autoimmune diseases. Bayesian methodology can be used to augment the sparse therapeutic data obtained from clinical trials in these circumstances.

Objectives

We elicited expert prior opinion for a future Bayesian randomised controlled trial for a rare inflammatory paediatric disease, polyarteritis nodosa (MYPAN, Mycophenolate mofetil for polyarteritis nodosa).

Methods

A Bayesian prior elicitation meeting was convened. Participating experts were drawn from across the EU and Turkey. Opinion was sought on the probability that a patient in the MYPAN trial treated with cyclophosphamide would achieve disease remission within 6-months, and on the relative efficacies of mycophenolate mofetil and cyclophosphamide. Expert opinion was combined with previously unseen data from a recently completed randomised controlled trial of mycophenolate mofetil versus cyclophosphamide in anti-neutrophil cytoplasmic antibody associated vasculitis.

Results

A pan-European group of fifteen experts participated in the elicitation meeting. Consensus expert prior opinion was that the most likely rates of disease remission within 6 months on cyclophosphamide or mycophenolate mofetil were 74% and 71% respectively. This prior opinion will now be taken in to account and will be modified to formulate a Bayesian posterior opinion when data from 40 patients completing the trial randomised at a 1:1 ratio to either receive cyclophosphamide or mycophenolate mofetil are available.

Conclusion

We suggest that this methodological template could be applied to trial design for other rare diseases, and is of particular relevance to rare autoimmune conditions that currently lack a good evidence base for treatment.

Disclosure of interest

None declared.

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