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Research Article

Developing a Randomised Efficacy PREcision medicine Platform trial design for Cavernomas: the CARE PREP study

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Plain language summary

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What was the aim?

A cerebral cavernous malformation, also known as 'cavernoma', is a cluster of blood vessels that form blood-filled caverns in the brain. Cerebral cavernous malformations can bleed to cause a stroke or cause epileptic seizures. In 2015, a partnership between patients and doctors ranked finding drugs to treat cerebral cavernous malformations as the fourth research priority for cerebral cavernous malformations. There are no specific drugs for cerebral cavernous malformations, but human and animal studies have identified several promising drugs. We obtained funding from the National Institute for Health and Care Research to plan for a future study that would test promising drugs.

What did we do?

Between September 2022 and August 2023, we set up a large international collaboration of doctors, researchers, and patients. An international patient and public involvement advisory group advised the collaboration. We identified promising drugs that are already available. We also approached companies that were developing new drugs for cerebral cavernous malformations. We grew our international collaboration. We agreed on the details and design of an innovative study, called a 'platform trial', that could compare several promising drugs with standard care to treat cerebral cavernous malformations. We met face-to-face once and online nine times. The patient and public involvement advisory group met five times. Fourteen countries seemed suitable for an international study. One pharmaceutical company was willing to collaborate. With the patient and public involvement advisory group, we agreed on a design for a platform trial to compare aspirin and propranolol with standard care. We submitted a funding proposal to the National Institute for Health and Care Research Efficacy and Mechanism Evaluation Programme in May 2023. The National Institute for Health and Care Research Efficacy and Mechanism Evaluation Funding Committee encouraged us to resubmit the application and provided suggestions to improve the design. We addressed the feedback and re-applied. We sent monthly newsletters to collaborators. We presented our work at the 2022 Alliance to Cure Cavernous Malformation International Scientific Meeting. We worked with the patient and public involvement advisory group to produce plain English newsletters, social media posts and a project summary.