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Adapting a trial design based on feasibility of recruitment where several treatment groups are possible and the outcome is long-term: pre-empt flexible-entry internal pilot study

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Background

Endometriosis occurs when the endometrium grows in abnormal locations outside of the womb, resulting in pain and reduced quality of life. See-and-treat surgery removes endometriotic lesions but the risk of recurrence is high. A HTA commissioning call asked to evaluate the long-term (three years) effectiveness of post-operative long-acting reversible contraceptives (LARCs) in preventing recurrence. A survey of gynaecologists indicated there was no consensus about which LARC (LNG-IUS or DMPA) or comparator (COCP or no treatment) should be evaluated. We designed a 'flexible-entry' internal pilot to assess whether a four-arm trial was feasible in light of possible strong patient preferences.

Methods

During the pilot, patients could be randomised to two, three or four treatment options provided one was a LARC and one was a non-LARC. An assessment of feasibility based on recruitment to these options and a substantive trial design was considered by an independent oversight committee. This design was fixed to ensure adequate power at the end of the study.

Results

The study ran for one year from April 2014 and 74 women were randomised. Only 5 (7%) women were happy to be randomised to all groups, with 60 (81%) having a LARC preference and 53 (72%) a non-LARC preference. Four-way and three-way designs were ruled out with a two-way (preferred LARC v COCP), stratified by LARC preference, considered feasible.

Conclusions

Where multiple treatment options are available a flexible approach to randomisation in a pilot phase can be used to assess feasibility and adapt a trial design.

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