Aim

To review the evidence on clinical effectiveness of combination therapy for idiopathic pulmonary arterial hypertension (IPAH) compared to monotherapy; to identify and review guidelines for using drugs for IPAH; and to determine the potential for expanded use of drugs for IPAH.

Conclusions and results

From the limited number of published randomized controlled trials (RCTs), it appears that some additional benefit may be derived from certain combinations of agents in IPAH. However, more studies in this area are needed to provide sufficient evidence. Guidelines recommend caution with respect to combination therapy for IPAH until more evidence is available, and treatment guidelines for IPAH reserve combination therapy for more severe cases that fail to respond to monotherapy. Combination therapy should be supervised by experienced PAH-specialty practitioners, or within the context of clinical trials. Additional evidence from clinical trials currently under way is needed to determine if there is potential for expanded use of combination therapy.

Recommendations

Not applicable.

Methods

Published literature was obtained by cross-searching BIOSIS Previews, EMBASE, and MEDLINE on the OVID search system between January 2003 and December 2008. Parallel searches were performed in PubMed and the Cochrane Library (Issue 4, 2008) databases. Websites of regulatory agencies and health technology assessment and related agencies were also searched, as were specialized databases, eg, University of York Centre for Reviews and Dissemination. These searches were supplemented by hand searching the bibliographies of selected papers. Two individuals screened and selected articles for inclusion in the report. Results were reported and discussed in this report.