

APPENDIX

PubMed/MEDLINE, search through 12/5/12

("rare diseases"[MeSH Terms] OR "orphan drug production"[MeSH Terms] OR "orphan drug*" [All Fields]) AND ("comparative effectiveness research"[MeSH Terms] OR "comparative effectiveness"[All Fields] OR "evidence-based medicine"[MeSH Terms] OR "methods" [MeSH Terms] OR "outcome assessment (health care)"[MeSH Terms] OR "patient advocacy" [MeSH Terms] OR "patient-centered care"[MeSH Terms] OR "health technology assessment*" [All Fields] OR "registries"[MeSH Terms] OR "patient participation"[MeSH Terms] OR "epidemiology"[MeSH Terms] OR "patient preference"[MeSH Terms]) AND English[lang] AND "humans"[MeSH Terms]

Academic Search Premier, search through 12/10/12

(DE "rare disease") OR (DE "ORPHAN drugs") AND (DE "PATIENT participation") AND (DE "OUTCOME assessment (Medical care) OR DE "OUTCOME assessment (Social services)") OR (DE "DRUGS -- Effectiveness") OR (DE "PATIENT-centered care") AND (DE "methods"))

EMBASE, search through 12/12/12

"patient care" OR "outcome assessment" OR "comparative effectiveness" OR "patient participation" OR "treatment outcome" OR "evidence based medicine" OR "epidemiology" OR "register" OR "patient preference" OR "outcomes research" AND "rare disease" OR "orphan drug" AND ("clinical article"/de OR "clinical study"/de OR "clinical trial"/de OR "clinical trial (topic)"/de OR "cohort analysis"/de OR "controlled study"/de OR "drug

surveillance program"/de OR "evidence based medicine"/de OR "human"/de OR "major clinical study"/de OR "meta analysis"/de OR "methodology"/de OR "model"/de OR "outcomes research"/de OR "practice guideline"/de OR "prospective study"/de OR "questionnaire"/de OR "randomized controlled trial"/de OR "retrospective study"/de OR "systematic review"/de) AND "rare disease"/de AND [english]/lim