The cost effectiveness of screening newborns for congenital adrenal hyperplasia

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Record Status
This is a critical abstract of an economic evaluation that meets the criteria for inclusion on NHS EED. Each abstract contains a brief summary of the methods, the results and conclusions followed by a detailed critical assessment on the reliability of the study and the conclusions drawn.

CRD summary
This study examined the cost-effectiveness of newborn screening for congenital adrenal hyperplasia in comparison with no screening. The authors concluded that, using common benchmarks for cost-effectiveness in the USA, screening was unlikely to be cost-effective. The study was based on sound methodology, despite some limited reporting of the data sources. The authors’ conclusions appear to be valid, but will need to be corroborated by further analyses.

Type of economic evaluation
Cost-effectiveness analysis

Study objective
This study examined the cost-effectiveness of newborn screening for congenital adrenal hyperplasia (CAH) in comparison with no screening.

Interventions
Screening for CAH was compared with a strategy that relied on early clinical recognition from symptoms or family history. CAH screening consisted of the collection of one routine blood specimen from each child.

Location/setting
USA/hospital.

Methods
Analytical approach:
The analysis was based on a decision analytic model with a lifetime horizon. The authors stated that it was undertaken from the perspective of the health care system.

Effectiveness data:
The clinical evidence came from a selection of known, relevant studies. The data on mortality without screening were derived from a systematic review of the literature that was carried out for this economic evaluation. Sources from different countries and of different designs were used. The authors discussed the approach they used to select the best estimate from among those available, especially for the most uncertain inputs. The key clinical input was the efficacy of screening in reducing mortality.

Monetary benefit and utility valuations:
Not included.

Measure of benefit:
Life-years (LYs) were the summary benefit measure and were discounted at an annual rate of 3%.

Cost data:
The analysis included the cost of specimen screening and follow-up of children who tested positive (including hospitalisations, when required). A second screening test was performed for all positive tests. The costs and resource quantities were derived from a survey of six screening laboratories across the USA and a study carried out in Texas. All costs were in US dollars ($) and the price year was 2005. No discounting was applied as only the short-term costs were considered.
Analysis of uncertainty:
The issue of uncertainty was investigated using two approaches. A deterministic methodology was used in one-way sensitivity analyses and best- and worst-case scenarios, with published ranges of values for the model inputs. A probabilistic approach was used by assigning triangular distributions to all the parameters.

Results
In the base case, the incremental cost per LY saved with screening over no screening was $292,000 and it ranged from $30,900 in the best case to $2,866,200 in the worst case.

The probabilistic analysis showed that the incremental cost-effectiveness ratio was $255,700.

The results of the deterministic sensitivity analysis indicated that the cost-effectiveness ratios varied depending on the assumptions on mortality and costs, but even at a mortality of 9%, the incremental cost per LY gained was $136,300. Only in the best-case scenario when all variables were set at the most favourable level for the screening programme did the incremental cost per QALY fall to $30,900.

Authors' conclusions
The authors concluded that using common benchmarks for cost-effectiveness in the USA, newborn screening for CAH was unlikely to be cost-effective. They noted that further research should provide reliable data on the mortality associated with CAH in both screened and unscreened cohorts.

CRD commentary
Interventions:
The rationale for the selection of the comparators was clear as the proposed strategy was compared against a strategy of no intervention, which was the usual care.

Effectiveness/benefits:
The authors selected some sources of evidence and performed a literature review, but did not report the details of this, for those inputs that were deemed to be the most uncertain. A valid justification for the selection of the clinical estimates was generally provided. The key clinical estimates were also assessed in the sensitivity analysis, but the authors acknowledged that the clinical data were generally poor. LYs were an appropriate benefit measure, given the impact of the disease on survival. Quality-adjustment would have been useful, but was not possible due to the lack of reliable evidence. LYs are a generalisable measure and they allow comparisons to be made with the benefits of other health care interventions.

Costs:
The categories of costs appear to have been consistent with the perspective stated. This assumed that the long-term costs were equivalent between screened and non-screened children and a short-term framework was considered. The authors justified their exclusion of those costs not directly related to CAH. The unit costs, some details of resource quantities, and the price year were reported, but the sources of costs were not extensively described. The costs were treated deterministically in the base case, but the sensitivity analysis considered the issue of variability around these estimates.

Analysis and results:
The analysis used a valid approach for synthesising the costs and benefits, but these were not reported separately and only incremental cost-effectiveness ratios were presented. The issue of uncertainty was satisfactorily investigated and the impact of variations in the key inputs of the model was explicitly considered. The results of all the sensitivity analyses were clearly presented and discussed. The authors noted that the main study limitation was the scarcity of clinical data, which was in part due to the relatively low prevalence of the disease.

Concluding remarks:
The study was based on sound methodology, despite some limited reporting of the data sources. The authors’ conclusions appear to be valid, but will need to be corroborated by further analyses.
Funding
Not stated.

Bibliographic details

PubMedID
19039250

DOI
10.1159/000156115

Original Paper URL

Other publications of related interest


Indexing Status
Subject indexing assigned by NLM

MeSH
Adrenal Hyperplasia, Congenital /diagnosis; Cost-Benefit Analysis; Humans; Incidence; Infant, Newborn; Neonatal Screening /economics; Outcome Assessment (Health Care); Predictive Value of Tests; Probability; Public Health; Quality-Adjusted Life Years; Sensitivity and Specificity; United States

AccessionNumber
22009100798

Date bibliographic record published
02/09/2009

Date abstract record published
24/03/2010