Cost-effectiveness of neonatal screening for medium chain acyl-CoA dehydrogenase deficiency: the homogeneous population of the Netherlands

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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
The aim of this study was to determine the cost-effectiveness of population-wide neonatal screening for medium-chain acyl-CoA dehydrogenase (MCAD) deficiency in comparison with no screening. It was concluded that screening for MCAD deficiency was cost-effective from the perspective of society. On the whole, the study was well conducted and the results were clearly presented, although some data used in the model were not reported in full.

Type of economic evaluation
Cost-effectiveness analysis

Study objective
The objective of the study was to determine the cost-effectiveness of population-wide neonatal screening for medium-chain acyl-CoA dehydrogenase (MCAD) deficiency in comparison with no screening.

Interventions
The addition of screening for MCAD deficiency to existing neonatal screening performed by tandem mass spectrometry was compared with no routine screening for this specific deficiency.

Location/setting
Netherlands/hospital.

Methods
Analytical approach:
A decision analytic model (probably based on a Markov chain) was developed in order to assess the economic and clinical impact of the two strategies in a hypothetical cohort of 200,000 newborns. The model was populated with published evidence and data obtained from a study performed in the authors’ setting. A lifetime horizon was used. The authors stated that a societal perspective was adopted.

Effectiveness data:
The clinical and epidemiological data were derived from a selection of known studies that were considered relevant to the authors’ setting. The scenario without screening relied on all clinically ascertained Dutch individuals with MCAD deficiency born before July 2003 and documented in case report forms. Data for the scenario with screening were based on estimates from a neonatal screening programme undertaken in 5 screening centres in the northern region of the Netherlands. An assumption was made about life expectancy for patients with identified MCAD. The key clinical estimate was the accuracy of screening.

Monetary benefit and utility valuations:
None.

Measure of benefit:
The summary benefit measure was the number of life-years (LYs) associated with the two strategies. LYs were derived using the decision model approach and were discounted at an annual rate of 4%.

Cost data:
The analysis of the costs included the costs of screening (personnel, equipment and materials) and MCAD deficiency (outpatient and inpatient visits, laboratory tests, medical consultations and treatments, travel expenses, special education, institutionalisation and productivity losses). Only costs that were directly attributable to MCAD deficiency were included in the analysis. The resource use data were derived from Dutch sources and included data obtained from case report forms and data from 5 Dutch screening centres. The costs were determined using multiple time studies. Productivity losses were estimated using the friction cost method. Discounting was relevant given the long-term time horizon of the analysis, and an annual rate of 4% was applied. The costs were in US dollars ($) and the price year was 2004.

Analysis of uncertainty:
The issue of uncertainty was addressed by means of second-order Monte Carlo simulations and was presented using cost-effectiveness acceptability curves. A univariate sensitivity analysis was undertaken in order to identify the role played by some individual model inputs.

Results
The cost per newborn was $4.22 without screening and $6.10 with screening. Overall, if screening was applied to a Dutch cohort of newborns, the costs amounted to $22,911 for 13.86 LYs gained. Thus, the incremental cost per LY gained with screening was $1,653 over no screening.

The cost-effectiveness acceptability curve showed that, at a monetary valuation of $5,000 per LY gained, screening was almost certainly cost-effective.

The Monte Carlo simulation showed that the incremental cost-effectiveness ratio ranged from -$4,345 to $14,839.

Authors' conclusions
The authors concluded that population-wide screening for MCAD deficiency was cost-effective in the Netherlands. These findings supported the introduction of nationwide neonatal screening for 15 diseases, including MCAD deficiency in the Dutch setting in 2007.

CRD commentary
Interventions:
The selection of no screening as the basic comparator was appropriate as it reflected the patterns of care in several areas of the Netherlands at the time the analysis was conducted.

Effectiveness/benefits:
The clinical data were based mainly on studies conducted in the authors’ setting, which appears appropriate given the study objective. In particular, data on the accuracy of screening were obtained from local estimates from 5 screening centres; this represents a positive feature of the analysis. Some assumptions were made around life expectancy, but the impact of these on the cost-effectiveness results was not fully investigated.

Costs:
The selection of the societal perspective was appropriate since all costs were included, irrespective of the payer. The measurement of costs was based on local data, which were relevant for the analysis. There was little information on the unit costs and resource quantities, and this will hinder the generalisability of the study results to other settings. However, the authors did report a breakdown of cost categories and the impact of each category on the total costs. The price year was reported, which enhances the possibility of performing refiation exercises in other time periods.

Analysis and results:
The costs and benefits were synthesised and were well reported, although the authors referred to an online appendix for some details. The issue of uncertainty was appropriately addressed by means of a probabilistic analysis. The results of the cost-effectiveness acceptability curves were presented graphically. However, the issue of the generalisability to other settings was not explicitly addressed. Comparisons with the findings from other studies were made and discussed. The authors acknowledged some limitations of the analysis, mainly related to the use of assumptions and data from multiple sources, as is commonly the case in modelling studies.
Concluding remarks:
The quality of the study methodology was satisfactory but model inputs were only partially reported, especially with respect to economic data. Overall, the authors’ conclusions appear appropriate and robust.

Funding
ZonMw.

Bibliographic details

Other publications of related interest


Indexing Status
Subject indexing assigned by NLM

MeSH
Acyl-CoA Dehydrogenase /deficiency; Case-Control Studies; Cost of Illness; Cost-Benefit Analysis; Female; Health Care Costs; Humans; Incidence; Infant, Newborn; Lipid Metabolism, Inborn Errors /diagnosis /economics /epidemiology; Male; Neonatal Screening /economics /standards; Netherlands; Reference Values; Retrospective Studies; Risk Assessment

AccessionNumber
22007001554

Date bibliographic record published
13/08/2007

Date abstract record published
09/08/2008