Economic evaluation of tandem mass spectrometry newborn screening in Australia

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 evaluated the cost-effectiveness of tandem mass spectrometry to screen for metabolic disorders in infants. The authors concluded that screening was likely to be cost-effective, in Australia. The study was generally well reported, and appears to have been based on strong evidence. The authors’ conclusions were appropriate, but may not be generalisable outside Australia due to different population characteristics and health system costs.

Type of economic evaluation
Cost-effectiveness analysis

Study objective
This study evaluated the cost-effectiveness of tandem mass spectrometry to screen for metabolic disorders in infants.

Interventions
Tandem mass spectrometry was compared with no screening. Mass spectrometry was used to analyse the levels of amino acids and acylcarnitines, in a blood spot.

Location/setting
Australia/public health care.

Methods
Analytical approach:
The cost-effectiveness analysis used observational registry data, from screened and unscreened areas of Australia, between 1994 and 2002, and assigned costs to the outcomes. The analysis was restricted to four-year comparisons. The authors stated that they took a health service perspective.

Effectiveness data:
For screening, registry data were gathered for infants born, after the introduction of screening, in New South Wales and the Australian Capital Territory in April 1998 until March 2002, and in South Australia and Tasmania in February 1999 until March 2002. These data were combined to form a group of 460,000 screened newborns. For no screening, the registry data were from 1994 to 1998, and from 1998 to 2002 from areas that did not have screening. This group contained approximately 1,535,000 infants. The primary measures of effectiveness were the changes in mortality with screening, and life-years gained from these changes. Only the deaths of infants over seven days old were included.

Monetary benefit and utility valuations:
Not relevant.

Measure of benefit:
Two summary measures of benefit were used; life-years gained and deaths averted. The analysis of averted deaths was based on data collected for the first four years of life. Life-years after this were based on a life expectancy of 80.2 years, which it was assumed was reduced by 10 years for those with untreated metabolic disorders. Discounting was applied to future benefits at 1.5% annually.

Cost data:
The cost categories were screening and follow-up of positive results, treatment once conditions were identified, and health care, in the first four years of life. The costs of screening were from New South Wales, South Australia, and
Victoria. The unit costs were reported for those costs incurred after diagnosis. The costs of health care in the first four years, included emergency, out-patient, and in-patient care. The unit costs were from the various Australian states and territories. The long-term costs of the metabolic conditions were not included due to a lack of data. All costs were reported in Australian dollars (AUD). Where necessary, they were inflated using Australian indices. Future costs were discounted at 6% annually.

Analysis of uncertainty:
One-way sensitivity analyses were conducted by altering five key assumptions: the cost of the test, the life expectancy of affected infants beyond four years old, the inclusion of follow-up costs, the inclusion of deaths before seven days old, and the discount rates. A worst case was analysed.

Results
Screening resulted in 32,378 additional life-years, 0.738 deaths averted, and AUD 349,010 per 100,000 infants screened. The incremental cost-effectiveness ratio was AUD 10,779 per life-year saved, which was less than the published Australian threshold of AUD 61,000 per life-year saved. The incremental cost per death averted was AUD 472,913.

In the one-way sensitivity analyses, the incremental cost per life-year saved ranged from AUD 7,969 to AUD 21,676. The worst-case scenario generated a ratio of AUD 58,036 per life-year saved. All values were below the Australian threshold.

Authors’ conclusions
The authors concluded that tandem mass spectrometry screening, for rare metabolic disorders, was likely to be cost-effective, in Australia.

CRD commentary
Interventions:
The choice of interventions appears to have been appropriate, and they were sufficiently described.

Effectiveness/benefits:
The clinical study was observational, but it appears to have been appropriate for this analysis. The data on nearly two million births (all those recorded in the areas analysed) were included, making the outcomes accurate for the setting. The assumptions appear to have been either appropriate or conservative for the cost-effectiveness of screening. Sufficient justification and explanation of the assumptions and methods was provided.

Costs:
The costs were from appropriate Australian sources. The authors noted that the costs from one setting were unlikely to generalise to other settings. The reporting was sufficient, but few unit costs were provided, making it difficult to replicate the analysis for another setting, which could also have different treatment protocols affecting the costs.

Analysis and results:
The analysis was generally well conducted and appears to have been reasonable. A probabilistic sensitivity analysis could have assessed the overall uncertainty in the cost-effectiveness estimate, but the results appear to have been relatively insensitive to the variations in the parameters tested in the one-way sensitivity analyses. All the effectiveness data, from two different time periods, were pooled for no screening. A subgroup analysis of the two different time periods separately might have been informative.

Concluding remarks:
The study was generally well reported, and appears to have been based on strong evidence. The authors’ conclusions were appropriate; as they indicated, the conclusions may not be generalisable outside Australia.

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