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The cost-effectiveness of including screening for severe combined immunodeficiency (SCID) in the UK NHS Newborn Blood Spot Screening Programme

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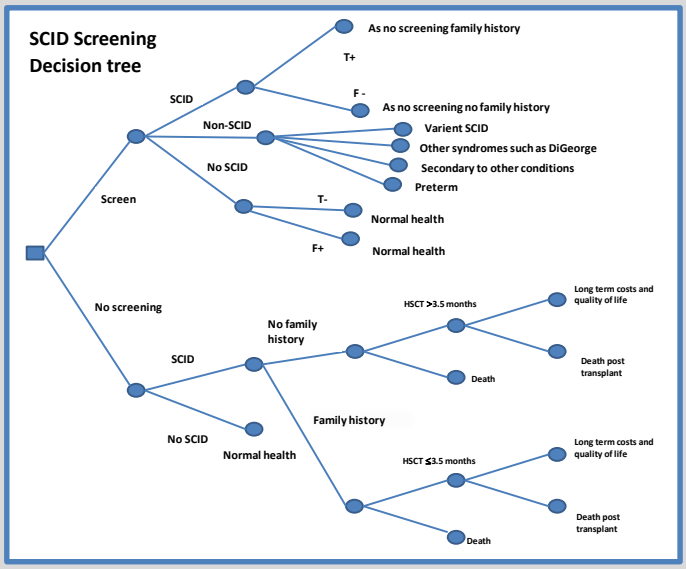
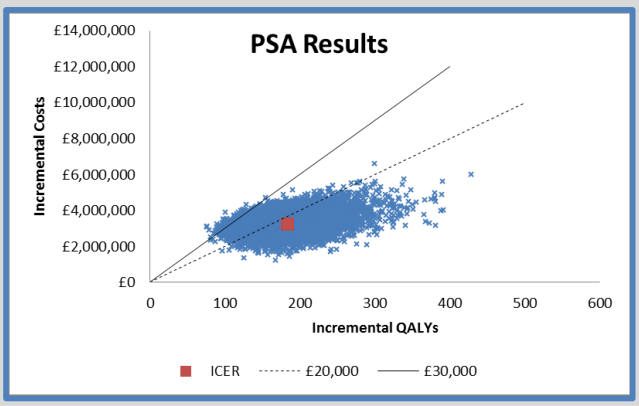
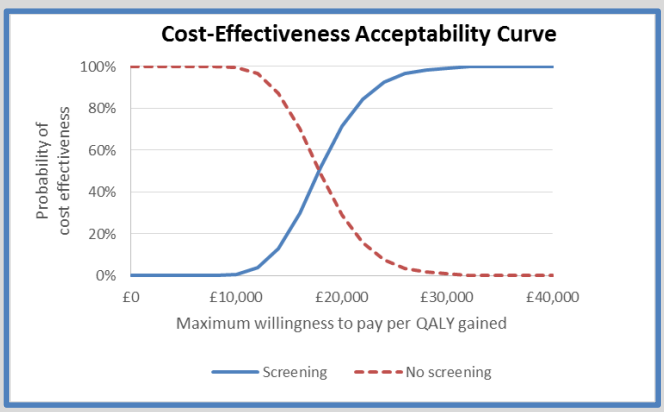
Objective - To assess the cost-effectiveness of including screening for severe combined immunodeficiency (SCID) in the NHS Newborn Blood Spot Screening Programme.

Methods - A decision tree model with life-table estimates of outcomes was built. Model structure and parameterisation were informed by systematic review and expert clinical judgment. A public service perspective was used and lifetime costs and quality adjusted life years (QALYs) were discounted at 3.5%. Standard treatment following screening was hematopoietic stem cell transplantation with additional treatment options for adenosine deaminase deficiency SCID. The model estimated the number of non-SCID cases identified incidentally. Probabilistic sensitivity analysis (PSA) was undertaken. A threshold analysis was used to investigate the impact of false positives and those diagnosed with non-SCID T-cell lymphopenia (TCL) by screening who would have presented as healthy at birth.







Results - Screening for SCID was estimated to identify 310 (72-811) newborns with positive results per year including 260 (25-760) false positives, 7 (1-21) pre-terms, 26 (9-50) newborns with non-SCID TCL and 17 (14-22) SCID cases. Screening would increase overall QALYs and costs with an incremental cost-effectiveness ratio (ICER) of £17,642. The increase in QALYs was driven by improved survival in the screened cohort with SCID mortality reducing from 8 (5.3-12) deaths to 1.7 (0.6-4.1) per year. Results were sensitive to a number of parameters including the cost of the screening test, the incidence of SCID, and quality of life estimates. The threshold analysis estimated that to push the cost-effectiveness over £20,000 per QALY the 6.5 (1.5-16) healthy at birth cases would each need a disbenefit of 2 QALYs and the false positive cases a disbenefit of over 12 quality adjusted days each.

Results Table

Outcome	No Screening Mean	No Screening 95% CI	Screening Mean	Screening 95% CI
Total costs (discounted)	**£3.98 m**	£2.91 m £5.26 m	**£7.22 m**	£5.88 m £8.97 m
Total QALYs (discounted)	**228**	167 298	**412**	309 531
SCID mortality	**8.1**	5.3 12.0	**1.7**	0.6 4.1
SCID detected (number of cases):				
Via screening	-	- -	**17.3**	13.5 21.9
Symptomatically	**11.1**	8.4 14.2	**0.2**	0.1 0.3
Via family history	**4.9**	3.2 7.0	-	- -
SCID not diagnosed	**1.5**	0.1 4.6	**0.0**	0.0 0.0
Non SCID TCL identified*	-	- -	**25.6**	9.3 50.0
Number of pre-terms identified	-	- -	**6.8**	0.5 21.2
Total presumptive positives	-	- -	**309.8**	72.0 811.4

*Some patients would be identified without SCID screening but are not included directly in the no screen arm of the model

Conclusion - Screening for SCID is potentially cost-effective at £20,000 per QALY, key uncertainties relate to the quality of life and behavioural impacts of identifying false positives and children with non-SCID TCL.

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