EE266: COST-EFFECTIVENESS OF NEWBORN SCREENING FOR CONGENITAL ADRENAL HYPERPLASIA IN JAPAN

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Objective:

Congenital adrenal hyperplasia (CAH) is a group of autosomal recessive disorders resulting from a congenital deficiency of an enzyme necessary for glucocorticoid biosynthesis in the adrenal glands. In Japan, population-based newborn screening (NBS) for CAH has been conducted since 1989, aiming for preventing lifethreatening salt-wasting crisis, incorrect sex assignments, and mortality. The objective was to assess the cost-effectiveness of **NBS for CAH in the Japanese newborn population.**

Table 2) Parameter

	Base case
Incidence	5.6E-05
salt-wasting %	78%
Sensitivity	1.0000
Specificity	0.9999
Retest rate %	0.38%
Mortality in the first year (Early detection)	0.70%
Mortality in the first year (Clinically identified)	1.50%
Neurological complication (Early detection)	0.70%
Neurological complication (SW Clinically identified)	18.50%
Neurological complication (SV Clinically identified)	9.40%
Screening test cost	833
Management costs per year	131,547
Treatment cost for adrenal crisis	475,184
Utility: Congenital adrenal hyperplasia	0.98
Utility: Neurological complication	0.84

Methods:

We compared the NBS strategy for CAH with no screening in the Japanese newborn cohort. We performed a model-based economic evaluation with the decision tree and Markov models to estimate lifetime costs and outcomes from the healthcare payer's perspective in Japan (Table 1). Model structure and major parameter are shown in Figure 1 and Table 2, respectively. The mortality rate of each health state was calculated using the age-specific mortality rate in the general population. The incremental cost-effectiveness ratio (ICER) was calculated by quality-adjusted life year (QALY) and screening and healthcare costs. To assess the uncertainty and the robustness of the results, we conducted one way sensitivity analysis and illustrated tornado diagram.

Table 1) Settings of the analysis

Population	Newborns for fiscal year 2023 in Japan
Perspective	Healthcare payer

SW: salt wasting form, SV: simple virilizing form

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Results:

The number of detected CAH in the cohort of 770,759 was 47.1 (incident rate 1:16,364). 111.3 participants were retested as false positives. Compared with no screening, the incremental cost of the NBS strategy was 854 Japanese yen, and incremental effects were 0.0002 QALY gained. The ICER for NBS versus no screening strategy was 3,958,224 JPY per QALY gained (the willingness-to-pay threshold is typically used 5 or 6 million JPY in Japan). Incidence rate, NBS test cost, and salt-wasting proportion affected ICER by one-way sensitivity analysis.

Lifetime
2%
Newborn screening for CAH
No screening
Quality-adjusted life year
Treatment costs and screening costs
Decision-tree model and Markov model

Decision tree model



Table 2) Base case result

	QALYs	ΔQALYs	Cost (JPY)	ΔCost	ICER
NBS	35.42698	0.000216	1,098	854	3,958,224
No screening	35.42676	_	244	_	



Markov model



Figure 1) Model structure

Figure 2) One way sensitivity analysis for ICER

Discussions:

The NBS strategy for CAH was more cost-effective than the strategy without screening. However, uncertainty remains in the analysis because of the limited parameters available regarding the effectiveness of screening, transition probabilities and utility.

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