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Cost-Utility Analysis of Including X-ALD in the Spanish Newborn Screening Program

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INTRODUCTION

X-linked adrenoleukodystrophy (X-ALD) is the most common peroxisomal disorder with a strikingly heterogeneous clinical spectrum, including childhood cerebral form (ccX-ALD), which is highly lethal in boys without hematopoietic stem cell transplantation before symptom onset. Therefore, early diagnosis of X-ALD is critical to administer pre-symptomatic treatment (haematopoietic stem cell transplantation (HSCT)) that reduces mortality and improves quality of life.

OBJECTIVE

To assess the cost-effectiveness of implementing universal newborn screening (NBS) for X-ALD in Spain

METHODS

- Cost-utility analysis using a lifetime decision-tree model (*Fig. 1*), parameterized through a systematic review
- Spanish National Health System (NHS) and social perspectives
- Deterministic and probabilistic sensitivity analyses

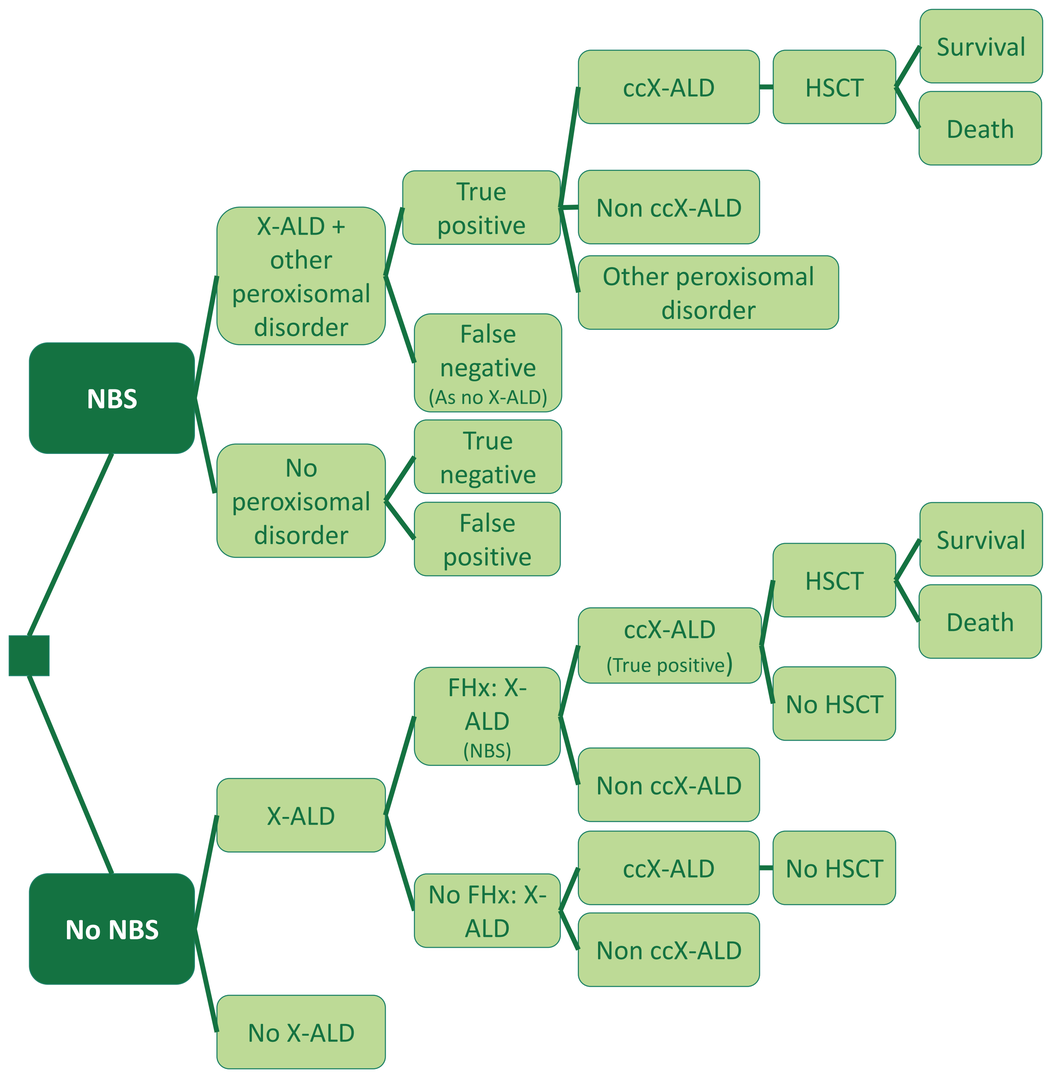

Figure 1: Simplified decision tree for X-ALD screening

RESULTS

Table 1: Model results – Hypothetical newborn population

			Population
Population (newborns in Spain 2022)			329,251
ccX-ALD Cases			3.11
No NBS	ccX-ALD cases detected by family history	Effective HSCT	0.36
		Ineffective HSCT	0.03
		Death due to HSCT	0.03
	ccX-ALD cases not detected by family history		2.69
NBS	ccX-ALD cases detected by NBS	Effective HSCT	2.62
		Ineffective HSCT	0.23
		Death due to HSCT	0.25
	ccX-ALD cases not detected by NBS		0.01

NHS Perspective

Table 2A: Cost-Effectiveness: NBS vs. No NBS (NHS)

Strategy	No NBS	NBS
Cost/newborn (€)	1.16	0.45
Δ Cost (€)	-	-0.71
Utility (QALYs)	6.783290	6.783300
Δ Utility (QALYs)	-	0.0000093
ICER (€/QALYs)	-	-76,133.95

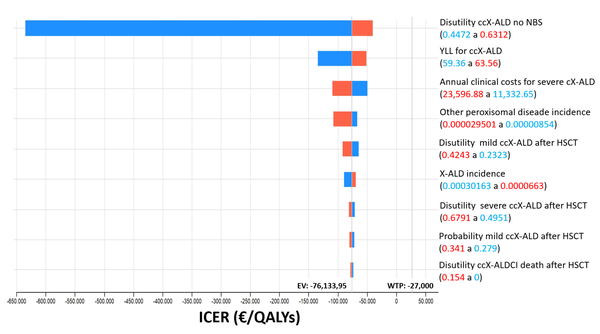

Figure 2A: Tornado diagram for deterministic sensitivity analyses (NHS)

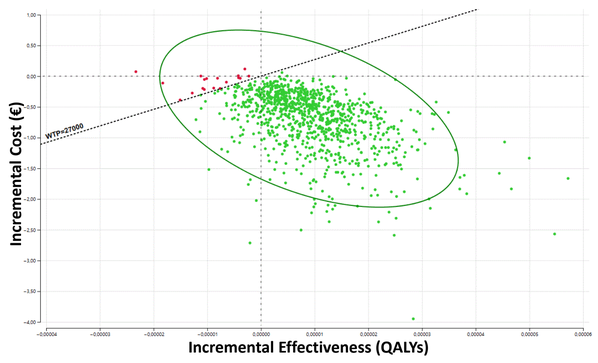

Figure 3A: Incremental cost-effectiveness, NBS vs. No NBS (NHS). Probabilistic sensitivity analyses based on 10,000 Monte Carlo simulations

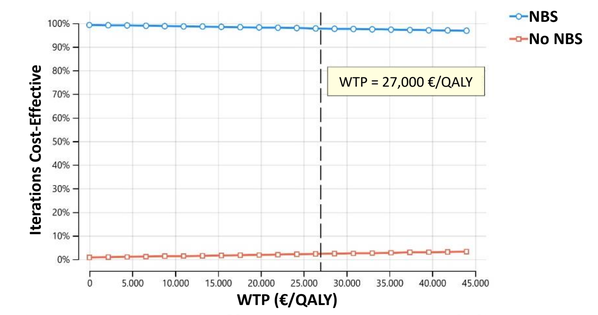

Figure 4A: Cost-effectiveness acceptability curve (NHS). NBS cost-effective at €27,000/QALY: 97.69%

Social Perspective

Table 2B: Cost-Effectiveness: NBS vs. No NBS (Social)

Strategy	No NBS	NBS
Cost/newborn (€)	8.64	3.28
Δ Cost (€)	-	-5.36
Utility (QALYs)	6.783290	6.783300
Δ Utility (QALYs)	-	0.0000093
ICER (€/QALYs)	-	-574,773.51

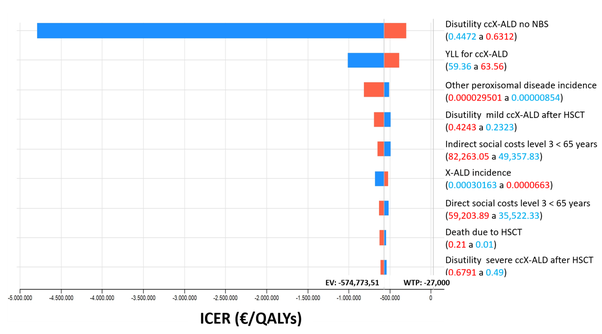

Figure 2B: Tornado diagram for deterministic sensitivity analyses (Social)

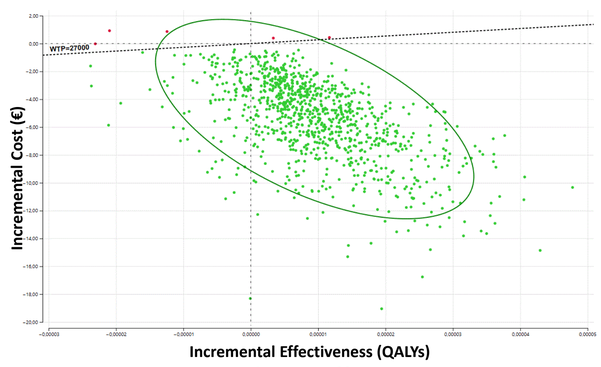

Figure 3B: Incremental cost-effectiveness, NBS vs. No NBS (Social). Probabilistic sensitivity analyses based on 10,000 Monte Carlo simulations

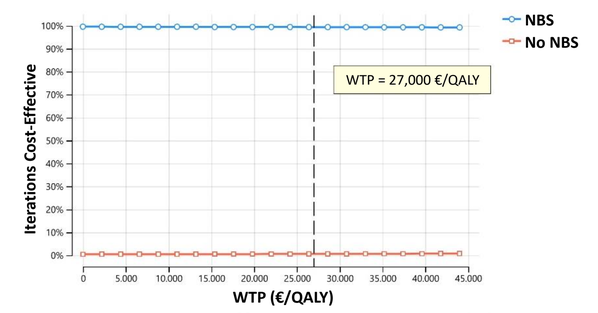

Figure 4B: Cost-effectiveness acceptability curve (Social). NBS cost-effective at €27,000/QALY: 99.38%

REFERENCES

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CONCLUSION

Including X-ALD in the Spanish NBS program is a cost-effective strategy from both NHS and social perspectives

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