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Cost-Effectiveness Analysis of the newborn screening in Austria

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Objectives
Since more than 45 years, a preventive program for the detection of congenital metabolic and endocrine diseases is carried out successfully in Austria. The goal is to investigate every newborn baby a few days after birth to initiate a quality assured therapy as quickly as possible. Since 1966, this program is carried out by the Federal Ministry of Health at the University Clinic for Child and Adolescent Medicine, Medical University of Vienna. The aim of this study was to determine cost-effectiveness of the newborn screening.

Methods
We developed a decision-analytic model, which include specific Markov processes for the core disorders: cystic fibrosis (CF), phenylketonuria (PKU), medium-chain acyl-CoA dehydrogenase (MCAD), congenital hypothyroidism (CH), galactosemia (GL), and maple syrup urine disease (MSUD). Costs and health benefits were estimated for a cohort of newborns in Austria (77,761 births per year). The analysis focused on lifetime consequences. This encompassed direct costs (including screening costs and cost of illness), quality-adjusted life-years (QALYs) and reduced expectation of life. Costs were presented per child and for the Austrian birth cohort. Costs from published sources were used (2014 Euro) from the health care systems perspective. QALYs, life-years (LYs) and costs were projected over a life-time horizon and discounted at 3% per year.

Results
Without newborn screening, the total direct cost amounts to 201.04 € per child screened (calculations based on the 77,761 newborns). This strategy resulted in a discounted quality-adjusted life expectancy of 31.91 QALYs. The corresponding life expectancy without quality adjustment amounted to 31.93 life-years.

The screening strategy accounted for 20.11 € and quality-adjusted life expectancy increased by 32 QALYS over lifetime. Total lifetime expectation without quality adjustment are 32 life-years. From the health care systems perspective, a newborn screening program resulted in cost savings of 180.93 € per child. Screening results in 0.09 QALYs gained and 0.07 LYs saved per child. The incremental cost-effectiveness ratio (ICER) amounts to ~2,086.03 € from the health care systems perspective.

The incremental costs of screening ranged from 12.308 € (MCAD) to 291.332 € (PKU) in case of disease detection.

Fig. 1: Model Design

Clinical Data
Incidence rates of each disease were derived from the nationwide database of the reference center (Medical University of Vienna) and included data over 10 years. Probabilities of sequelae, mortality rates and quality adjusted survival rates were derived from literature.

Resource Use and Costs
Resource use and cost of screening were derived from the nationwide database of the reference center. Data on the resource use of the included diseases and sequelae were collected in two steps. First, the medical resources were derived by literature (e.g. disease specific guidelines). In a second step this literature review was verified by experts.

Direct medical costs derived from a number of publicly available sources like disease specific guidelines. In a second step this literature review was verified by experts. Indirect Cost represent outpatient clinic catalogue as well as the DRG catalogue (LKF) and official price lists for the Austrian Health insurances funds. Indirect Cost represent the great influence on the total costs. Variation of the NBS costs exhibit the great influence on the total costs. Variation of the NBS costs exhibit the great influence on the total costs.

Sensitivity Analysis
In the deterministic one-way sensitivity analyses (Figure 2), our results were robust to a wide range of plausible estimates of unit cost data. Cost savings persisted for the newborn screening strategy in all variations of the sensitivity analyses. Costs for moderate development delay and the NBS costs exhibit the great influence on total costs . Variation of the NBS costs are associated with a range of costs (16,1€-24,12€) per child in the NBS arm. The incremental cost-effectiveness advantage for the screening strategy ranged between -1,563,13€ to 2,039,80€.

Test results marked as “Dominates” save money and improve outcome relative to no testing. Table 1 demonstrates that the total panel is able to save money and improve outcome.

Tab. 1: Results, 2014

<table>
<thead>
<tr>
<th>Strategy</th>
<th>Lifetime costs per infant</th>
<th>QALYs per infant</th>
<th>ICER</th>
<th>LYs per infant</th>
<th>Lifetime discounted lifetime QALYs for birth</th>
<th>LYS for birth cohort</th>
</tr>
</thead>
<tbody>
<tr>
<td>No-screening</td>
<td>201.04</td>
<td>31.91</td>
<td>31.93</td>
<td>15.63</td>
<td>2,684,614</td>
<td>2,482,915</td>
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<tr>
<td>Screening</td>
<td>201.11</td>
<td>32.00</td>
<td>Dominant</td>
<td>32.00</td>
<td>1.56</td>
<td>2,488,358</td>
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<tr>
<td>Difference</td>
<td>0.93</td>
<td>-0.09</td>
<td>-0.07</td>
<td>14.07</td>
<td>-6,744</td>
<td>-3,482</td>
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</tbody>
</table>

Source: IPF own calculations

Fig. 2: Deterministic one-way sensitivity analysis

Clinical Data

References
