Cost-Benefit Analysis of the Detection and Treatment of Phenylketonuria (PKU) in Newly-Born Babies, in Belgium (*)

by

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SUMMARY on p. 468

INTRODUCTION

Since 1972, more than 100,000 newly-born babies are examined for PKU each year, at a cost, in 1981, of a little under 8,000,000 FB. This article aims to estimate whether or not this expense is justified, by means of a cost-benefit analysis.

As the most important category of benefits concerns the "saving" of a number of human lives each year, estimates of the economic value of human life will be used to render the benefits comparable with the costs.

Each annual investment in PKU screening entails both costs (the treatment) and benefits (costs and losses avoided) that extend many years into the future and which must therefore be discounted. Two discount rates will be used.

(*) The author wishes to thank professor E.S. Kirsch (DULBEA, ULB) for his help and guidance. This article is to a large extent based on earlier work at the ULB by Dekock (1976) and Methens (1981).
The first, 4.18% (Kirschchen et al., 1975) is based on the idea that as public investment diverts funds from private investment, the returns of the former should at least equal those of the latter. The value 4.18% is derived from the average of the interest rates of four categories of private investment (housing, other investment, consumption on credit and other consumption), weighted according to the fraction of the public investment that diverts funds from each of these four categories of private investment.

In order to examine the sensitivity of the benefit:cost ratios to the choice of the discount rate, a second, often employed value of 7% will also be used.

I. MEDICAL ASPECTS

PKU is a congenital disorder of the amino-acid metabolism, inherited as an autosomal recessive. The PKU child lacks an enzyme allowing normal conversion of phenylalanine to tyrosine. Instead a by-product, phenylpyruvic acid, accumulates in the blood-stream and is the direct cause of an irreversible mental-retardation, most of the PKU children having an IQ of between 10 and 50. Smith (1975) puts the case for screening in the following terms. "It (PKU) is not outstandingly common - about once in every 6000 births in Western countries - but it is currently the cause of an enormous screening programme. The reason is that, unlike so many other congenital disabilities, something can be done about PKU. If it is detected early enough, preferably within a few weeks of birth, and if the infant then receives a diet free of phenylalanine for several years, the victim stands a good chance of escaping the form of idiocy that would otherwise prevail".

For these reasons nearly every baby born in Belgium is screened for PKU, only 5% escaping all screening. Of the 95% of screened
babies, 90% are screened by government approved centres and 5% privately. Table 1 gives the annual number of examinations and cases detected, and the cost of the screening in the government approved centres for the period 1978-1981. The incidence is lower than that given by Smith (1975), being closer to 1 in 10,000 births.

Whereas the form of the treatment, a phenylalanine-free diet, is well established, the recommended duration of the diet is a subject of controversy. Some authorities consider that 6 years of diet are sufficient while others, noting that at 6 years of age the brain is not yet fully developed, recommend that the diet be continued for 30 years or even for as long as the PKU child should live (~70 years). In this article it will be assumed that the diet will be followed for 30 years. The difference in cost between 30 years and 70 years' diet would in any case be small, due to the effect of the discounting.

TABLE 1. PKU screening in government-approved centres (*)

<table>
<thead>
<tr>
<th>Year</th>
<th>No. of screenings in G.-A. centres (Thousands)</th>
<th>No. of PKU children discovered</th>
<th>Cost of screening (FB 1981) (Thousands)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1978</td>
<td>109</td>
<td>11</td>
<td>7,410</td>
</tr>
<tr>
<td>1979</td>
<td>114</td>
<td>10</td>
<td>7,980</td>
</tr>
<tr>
<td>1980</td>
<td>114</td>
<td>6</td>
<td>7,797</td>
</tr>
<tr>
<td>1981</td>
<td>112</td>
<td>13</td>
<td>7,709</td>
</tr>
<tr>
<td>Average (1978-81)</td>
<td>112</td>
<td>10</td>
<td>7,724</td>
</tr>
</tbody>
</table>

(*) Statistics supplied by the Ministère de la Santé Publique.
2. SOCIAL COSTS

The social costs concern:

- the screening. The average cost per year for the period 1978-1981 was 7.7 millions FB 1981, with 10 PKU children per year detected (see table 1);

- the treatment, i.e. the phenylalanine-free diet, assumed to be followed for 30 years. The cost of the diet is met by the PKU child's family, with the exception of two products, Lofenalac and Phenyldon, which are partly reimbursed by the INAMI (29,400 and 39,000 FB 1981 per year respectively – Methens (1981)). The parents receive a supplementary family allowance (73,368 FB 1981 per year) which is destined to compensate for their contribution towards the cost of the diet and the extra family care.

In actual fact, the composition and cost of the diet will vary from child to child, from family to family and from year to year as the child grows up. For simplification it will be assumed here that the cost of the diet is constant and equal to the sum of the supplementary allowance and the INAMI contribution towards the Lofenalac and Phenyldon. The total cost of 30 years' diet, discounted at 4.18 and 7 %, is given in table 2.

The relatively minor costs of the medical surveillance of the treated PKU child (e.g. periodic blood tests) are not taken into account here.

3. SOCIAL BENEFITS (COSTS AND LOSSES AVOIDED)

The social benefits evaluated in this analysis concern the costs and losses avoided by discovering and treating the 10 PKU children per year.
Without detection and treatment, the PKU children are assumed to be brought up at home for their first five years of life, after which they are placed in an institute for mentally handicapped children for the rest of their life-spans (0-30 years). Their adult life-value is assumed to be zero, implying a loss of 10 adult life-values.

Thus, the costs and losses, without detection and treatment, concern:

- upbringing at home (0-5 years). Lacking Belgian statistics, the figures given in Willeke’s (1981) West-German study will be used (144,000 FB 1981 per year);

- upbringing in an institute (5-30 years), at 191,260 FB 1981 per year (note that this value excludes certain minor costs such as the cost of the specialised teaching given to mentally handicapped children - Methens (1981));

- the supplementary family allowance received by the parents and the institute (73,368 FB 1981 per year, for 25 years);

- the loss of 10 adult life-values. Goss (1982) has estimated the economic value of average women of different ages in Belgium. Briefly, his estimations are based on the following losses that are entailed by a woman’s premature death:

  \[
  \begin{align*}
  \text{the loss of her net production} & \quad - \quad 91.4 \% \\
  \text{the loss to the woman herself} & \quad - \quad 6.9 \% \\
  \text{the loss to those that know the woman} & \quad - \quad 1.7 \% \\
  \text{the loss to those that don’t know her} & \quad - \quad 100.0 \%
  \end{align*}
  \]

Discounting at 4.18 % and assuming a 2.8 % annual rate of growth of the GNP per person, the discounted life value at 21 years of age of a woman born in 1981 would be 10.6 million FB 1981.

Discounting at 7 %, this value becomes 3.7 million FB 1981 (note
that a discount rate of 7% together with a 2.8% annual rate of
growth of the GNP per person is approximately equivalent to a
discount rate of 4.18% together with a zero annual rate of growth
of the GNP per person). It should also be borne in mind that a PKU
child born in 1981 will be 21 in the year 2002. Thus the life-
values from Goss (1982), based on the year 1971, are being used
here to estimate life-values for the year 2002).

The equivalent values for men in Belgium have similarly been
estimated, being 15.2 and 9.5 million FB 1981, with 4.18% and
7% discount rates respectively.

With detection and treatment, the 10 PKU children are brought
up at home and will be assumed, for the moment, to have average
adult life-values. Thus the corresponding costs and losses concern
only:

- upbringing at home (0-21 years). Lacking Belgian statistics,
  the figures given in Willeke's (1980) West-German study will be
  used (144,000 FB 1981 per year, for the first six years of life,
  167,440 FB 1981 per year thereafter). This cost is assumed to
  be independent of the cost of the phenylalanine-free diet.

The social benefit is given by the difference between the costs
and losses incurred without detection and treatment and those
incurred with detection and treatment (see table 2).

Note that only the figures concerning the adult life-values
take account an annual mortality risk. Each treated PKU child is
assumed to survive at least until the age of 21, following the
average annual mortality rates thereafter. Each untreated PKU
child is assumed to live for 30 years.
4. BENEFIT : COST RATIOS

A summary of the discounted social costs and benefits and the resultant benefit:cost ratios, is given in table 2.

The benefit: cost ratio arrived at with a 4.18% discount rate is 6.1 and is highly favourable towards continuing mass-screening for PKU.

With a 7% discount rate, the benefit:cost ratio arrived at is significantly lower, 3.6, though still favourable towards continuing mass-screening for PKU.

Finally one must consider the possibility that treated PKU children will have a lower than average adult life-value. Firstly their life-expectation may be lower than average and secondly their productive value might be lower (for example treated PKU children tend to have difficulties in attention and concentration). Were one to assume, arbitrarily, that their adult life-value was only half the average value, then the benefit:cost ratios would become:

Benefit:cost ratio : 3.5 (4.18% discount rate)  
2.2 (7% discount rate)

These values are notably lower than those of 6.1 and 3.6 respectively given assuming that the treated PKU children have an average adult life-value. However they are still favourable towards continuing screening.
TABLE 2. The social benefits and costs for one year's PKU screening in the period 1978-1981 (in millions of FB 1981)(a)

The social benefits:

<table>
<thead>
<tr>
<th></th>
<th>Without detection and Treatment (1)</th>
<th>With detection and Treatment (2)</th>
<th>Social benefit = Difference (3)=(1)-(2)</th>
</tr>
</thead>
<tbody>
<tr>
<td>(Discount rate)</td>
<td>4.18% 7%</td>
<td>4.18% 7%</td>
<td>4.18% 7%</td>
</tr>
<tr>
<td>Upbringing at home</td>
<td>6.6 6.3</td>
<td>18.0 13.8</td>
<td>-11.4 -7.5</td>
</tr>
<tr>
<td>Upbringing in Institute</td>
<td>24.9 17.0</td>
<td>0.0 0.0</td>
<td>24.9 17.0</td>
</tr>
<tr>
<td>Supplementary allowance</td>
<td>11.4 9.0</td>
<td>0.0 0.0</td>
<td>11.4 9.0</td>
</tr>
<tr>
<td>Loss of life-value (b):</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>5 men</td>
<td>76.1 47.8</td>
<td>0.0 0.0</td>
<td>76.1 47.8</td>
</tr>
<tr>
<td>5 women</td>
<td>55.5 19.6</td>
<td>0.0 0.0</td>
<td>55.5 19.6</td>
</tr>
<tr>
<td>TOTAL :</td>
<td>174.5 99.7</td>
<td>18.0 13.8</td>
<td>156.5 85.9</td>
</tr>
</tbody>
</table>

The social costs:

<table>
<thead>
<tr>
<th></th>
<th>(Discount rate)</th>
<th>4.18% 7%</th>
</tr>
</thead>
<tbody>
<tr>
<td>Detection</td>
<td></td>
<td>7.7 7.7</td>
</tr>
<tr>
<td>Treatment: Lofenelac + Phenylidon (INAMI contribution)</td>
<td>9.5 7.2</td>
<td></td>
</tr>
<tr>
<td>Lofenelac + Phenylidon (Parental contribution) plus remaining costs of diet and extra family care</td>
<td>8.6 9.0</td>
<td></td>
</tr>
<tr>
<td>TOTAL :</td>
<td>25.8</td>
<td>23.9</td>
</tr>
</tbody>
</table>

Benefit:cost ratios:

<table>
<thead>
<tr>
<th></th>
<th>Discount rate</th>
<th>4.18% 7%</th>
</tr>
</thead>
<tbody>
<tr>
<td>- Average adult life-value</td>
<td>6.1</td>
<td>3.6</td>
</tr>
<tr>
<td>- 1/2 average adult life-value</td>
<td>3.5</td>
<td>2.2</td>
</tr>
</tbody>
</table>

(a) using 4.18% and 7% discount rates.
(b) assumes treated PKU children have average adult life-values.
CONCLUDING REMARKS

For an investment to be judged desirable, its projected benefit:cost ratio must not only be greater than unity, but also compare favourably with those of other projects competing for the same funds.

The 4 benefit cost ratios obtained, following the different hypotheses (see table 2), for screening for phenylketonuria all satisfy the basic economic criterion of being greater than unity. Most public-health projects however are authorised without any cost-benefit analysis, and therefore it is not at present possible to compare the benefit:cost ratios of screening for PKU with those of other projects competing for the same public funds. This is a regrettable state of affairs, considering that it is relatively simple to perform cost-benefit analyses for projects that avoid human deaths (e.g. motorway illumination, emergency re-animation, etc.) now that more objective estimations of the economic value of human life are available. For example, the benefit:cost ratio for mass-screening for breast and cervical cancer in Brussels (Goss, 1982) was 0.9, using a 4.18% discount rate. Of course much work remains to be done in this area, particularly as concerns the evaluation in monetary terms of more intangible benefits such as an improvement in the state of health, or the reduction of pain and suffering.

Nevertheless it is now possible to have a clearer idea of what returns one is getting for a given investment in different life-saving projects and thereby to maximise the overall social benefit of investment in public health.

Insofar as PKU screening is concerned, the benefit:cost ratios obtained are overall highly favourable.
BIBLIOGRAPHY


SUMMARY

The early detection and treatment of PKU by mass-screening avoids irreversible brain-damage in about 10 children per year. Comparing the costs (detection and treatment) with the benefits (avoided supplementary cost of upbringing in institutes for the mentally handicapped, avoided loss of 10 adult life-values) gives benefit:cost ratios of 6.1 and 3.6, with 4.18 and 7.0 % discount rates respectively. Assuming treated PKU children will have only half the average adult life-values would give benefit:cost ratios of 3.5 and 2.2 respectively.