Evidence-based prevention requires evidence-based performance

The case of screening for congenital heart disease in child health care

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Study objective: To illustrate to what extent the cost-effectiveness of an evidence-based prevention programme may depend on evidence-based performance, by the example of screening for congenital heart disease in Dutch child health care. Methods: A patient follow-up study on 290 children with congenital heart disease, of which 83 with significant disorders, diagnosed over two years, and born in the south west of the Netherlands. Results: Adequate screening for congenital heart disease at Dutch child health centres, compared to inadequate screening, proves to be effective (OR: 0.18; 95% CI: 0.04–0.87). However only 15% of all patients with significant disorders in this study was adequately screened. Total health care costs involved over two years amount to over $3 million. Of these costs, 13% are to be attributed to screening tests; 8% to referrals resulting from screening. The costs for screening and referrals, as they were actually performed, are estimated at about $72,000 per patient benefiting from it. Were all children to be screened adequately this sum would be reduced to about $15,000. Conclusion: Not only should prevention programmes be evidence-based, but also outcome and quality, monitored by periodically establishing whether they are optimally performed.

Keywords: child health care, congenital heart disease, cost-effectiveness, screening

The need for preventive interventions to be evidence based is frequently stressed.1,2 Therefore the effectiveness of such interventions must be soundly substantiated. The relation between the costs of a preventive intervention and its actual effect, however, does not depend only on its potential effectiveness, but also on whether in practice it is optimally performed. Screening for congenital heart malformations, as applied in Dutch child health centres between the ages of 1 month and 4 years, and aimed at preventing haemodynamic complications by early treatment, provides a clear example of this proposition. We studied the cost-effectiveness of the actual screening programme and compared it with that which may be achieved after optimizing the screening performance. In this paper we will answer the five questions presented below. The main focus of the paper is on the cost-effectiveness of the screening (questions 3–5). The assessment of the effectiveness of the screening (questions 1 and 2) serves as a starting point. The methods sections on these first two questions are kept very concise. For the exact methods,3 their methodological justification4 and an extensive description of the screening methods used5 we refer the reader to former publications.

RESEARCH QUESTIONS

1 Is adequate screening for significant congenital heart malformations in Dutch child health centres effective, i.e. do adequately screened patients have less chance of being diagnosed ‘too late’ to prevent haemodynamic complications than inadequately screened ones?

2 How many children with congenital heart disease are presently benefiting from the Dutch screening programme? How many children would benefit if all children were adequately screened?

3 What health care costs are incurred for congenital heart malformations diagnosed between the ages of 1 month and 4 years? What expenditures are due to:
- screening tests in the whole population,
- referral procedures resulting from these screening tests,
- diagnosis and therapy for patients with clinically significant as well as clinically insignificant malformations?

4 What savings are made on diagnostic and therapeutic procedures for patients with a clinically significant congenital heart disease diagnosed ‘in time’ compared to such patients diagnosed ‘too late’? We will establish in particular how costs of hospitalization in paediatric intensive care units contribute to these savings.

5 What are presently the net costs due to the preventive programme per patient benefiting from it? What would they be if all patients were adequately screened?
The numbering of these questions correspond with the numbering used in the methods and results sections and with the numbering of the tables.

METHODS

1 Effectiveness

In a patient follow-up study all 290 consecutive patients, aged between 32 days and 4 years, presented at the Sophia Children’s Hospital Rotterdam from 11 April 1994 until 11 April 1996 with a congenital heart malformation were included. Of these, 83 had significant disorders, i.e. needing therapeutic interventions within 9 months after diagnosis. Paediatric cardiologists established whether or not these patients were diagnosed either ‘too late’ or ‘in time’ to prevent haemodynamic complications. Parents and child health centre physicians were interviewed in order to establish retrospectively whether or not they were adequately screened, i.e. at the right ages and in accordance with recently established guidelines, and by whom they were initially referred. Paediatric cardiologists were also asked to assess the severity of the disorder and classify it as ‘trivial’, ‘moderate’, ‘severe’, and ‘very severe’. Logistic regression was used to derive odds ratios, including 95% confidence intervals, for being diagnosed ‘too late’ depending on whether or not the patient had been screened adequately. Correction for severity was applied in order to adjust for length bias.

2 Patients benefiting from screening, presently and if all children were adequately screened

The proportion of patients diagnosed ‘too late’ in a population exposed to screening as presently performed in the Netherlands and in a population with optimal screening, i.e. in which all children were adequately screened, are derived from the results of the effectiveness evaluation mentioned above. We estimated the proportion of patients diagnosed ‘too late’ in an imaginary situation without screening on the basis of this proportion among patients not referred by child health centre physicians after correction for severity, presuming that in such an imaginary situation a distribution of severity similar to that in the total study group is to be expected.

To estimate, for a time window of two years, the numbers of patients in the south-west of the Netherlands benefiting from screening presently, and if all children were adequately screened we proceeded as follows. We calculated correction for severity, presuming that in such an imaginary situation a distribution of severity similar to the total study group is to be expected. We estimated the proportion of patients diagnosed ‘too late’ depending on whether or not the patient had been screened adequately. Correction for severity was applied in order to adjust for length bias.

3 Health costs

All costs are calculated based on the price level of 1996.

Cost estimates are made for the population of the south-west of the Netherlands, more specifically the area from which all children with congenital heart disease are referred to the Sophia’s Children’s Hospital, for two years. In this population 75,441 children were born during 1995 and 1996.

In the Netherlands, six screening examinations are recommended in the first four years of life. In a window of two years, therefore, children undergo an average of three screenings. Screening is an integrated part of the periodical examination by the child health centre (CHC) physician. During these fifteen minute visits doctors assess health changes since the previous visit, perform a physical examination, evaluate findings with the parents and contribute their part to the health education programme. We estimate the cardiovascular examination to take 1 minute of each visit. The health care costs were calculated on the basis of CHC-physicians’ salaries and the costs of overhead and accommodation established by the Rotterdam Homecare Foundation. (In the Netherlands, preschool child health care is part of private organisations for home care.)

Costs for referrals

We considered the consequences of a referral by a CHC-physician for a potential congenital heart malformation to be on average:

- one visit to the general practitioner,
- one visit to a paediatrician,
- one electrocardiogram, and
- an ultra-sound investigation in one out of three cases.

The costs are calculated on the basis of fees. The number of referrals in the south-west of the Netherlands over two years was estimated by the registered referrals for congenital heart malformations by Rotterdam CHC-physicians during the first half of 1996.

Costs for diagnosis and therapy

Calculations are based on the study group of the patient follow-up study mentioned above. Estimates of the costs for the 83 patients with a clinically significant malformation were based on examination of their hospital files. Estimates of the costs for the remaining 207 patients with clinically insignificant malformations were based on examination of hospital files of a random sample of 40.

These data were gathered for the two years following the first visit to the paediatric cardiologist. The following information was registered:

i) Outpatient visits

ii) Hospitalization days at

- Medium care units;
- Intensive care units.

iii) Diagnostic procedures

- X-thorax;
- electrocardiogram;
- 24 hour electrocardiogram;
- ultra-sound investigation;
- transoesophagal ultrasound investigation;
- diagnostic catheterizations.

iv) Therapeutic procedures

- surgical operations;
- interventional catheterizations;
- diuretic medication days.
Costs of medical procedures are based on Dutch health care fees.9,10 In the Netherlands, an extensive fee system exists for the reimbursement of specialist costs and hospital costs in medical procedures. Costs of outpatient visits, intensive and medium care hospitalization days are based on departmental data from the Sophia Children’s Hospital, including employee salaries, consumables and supplies, housing and maintenance, and overhead costs. Since both direct and indirect costs are included, these are full unit costs. Costs of medication are based on the Dutch Pharmacotherapeutic Manual.11

4 Savings in patients diagnosed ‘in time’
The mean difference in costs of patients diagnosed ‘too late’ and those diagnosed ‘in time’ is calculated, broken down into intensive care costs and all other costs. Whether a patient with a clinically significant congenital heart disease is diagnosed ‘too late’ or ‘in time’ is, to a large extent, determined by the severity of the disorder. Since severe disorders may generate more costs than moderate ones, this may lead to overestimation of the potential correlation between a delayed diagnosis and costs. To avoid this bias estimates are corrected for severity and 95% confidence intervals are calculated by means of linear regression analysis.

5 Costs per patient benefiting from screening
The costs of the screening programme are defined as the costs for all screening tests and for the referrals of patients with false positive test results. The latter number was the total number of referrals in two years minus the number of patients with true positive referrals. To calculate the mean outcome of costs and savings for one child benefiting from screening, savings per child (the difference between average costs for patients diagnosed ‘too late’ and ‘in time’) are subtracted from the costs per child. Finally, we estimated the results, for the case when intensification of the screening regime would double or quadruple the number of false-positives.

RESULTS
1 Results of the effect-evaluation of adequate screening compared to inadequate screening are summarized in table 1. (The screening history of one patient could not be traced, so calculations were made on 82 patients.) Adequate screening for congenital heart disease at child health centres, compared to inadequate screening, is effective (OR: 0.18). After correction for severity the odds ratio remains almost the same, although the confidence interval now just includes 1. However only 15% of all patients with significant congenital heart malformations in this study are adequately screened.

2 The results of the calculation of numbers of patients benefiting from screening are shown in table 2. For a time window of two years in the south-west of the Netherlands the number of patients benefiting from the present screening and screening after optimising the procedures are estimated at respectively 8 and 32.

3 The health care costs of the disorders under discussion over two years in the south-west of the Netherlands are summarized in table 3. These costs amount to over 3 million US dollars. Of the total costs, 13% are attributed to child health centre screening tests and 8% to referrals resulting from screening.

4 As demonstrated in table 4, the average costs for a patient diagnosed ‘too late’ exceed by about a quarter the average costs for a patient diagnosed ‘in time’. The difference is, to a large extent, attributed to the costs of hospitalization in intensive care units. The difference in intensive care costs is statistically significant. After correction for severity, however, the 95% confidence interval just exceeds 0. The difference in the remaining costs is statistically not significant and is almost entirely attributed to an overrepresentation of severe and very severe disorders among patients diagnosed ‘too late’. We conclude that after correction for severity, mean savings of $3343 may be expected by preventing a disorder from being diagnosed ‘too late’.

5 In table 5 the net costs due to the screening for each patient benefiting from this programme is calculated. Health care expenditures to be attributed to the

### Table 1 Classification of the 82 patients with clinically significant disorders diagnosed ‘too late’ and ‘in time’ in categories ‘adequately screened’ and ‘inadequately screened’

<table>
<thead>
<tr>
<th></th>
<th>‘Too late’</th>
<th>‘In time’</th>
<th>Total</th>
<th>%</th>
</tr>
</thead>
<tbody>
<tr>
<td>Adequately screened</td>
<td>2 (17%)</td>
<td>10 (83%)</td>
<td>12 (100%)</td>
<td>15</td>
</tr>
<tr>
<td>Inadequately screened</td>
<td>37 (53%)</td>
<td>33 (47%)</td>
<td>70 (100%)</td>
<td>85</td>
</tr>
<tr>
<td>Total</td>
<td>39 (48%)</td>
<td>43 (52%)</td>
<td>82 (100%)</td>
<td>100</td>
</tr>
</tbody>
</table>

Odds ratio for being ‘too late’ depending on whether or not adequately screened:

\[
\frac{2.10}{37.33} = 0.18 \text{ (95% CI: 0.04–0.87)}
\]

\[
= 0.20 \text{ (95% CI: 0.04–1.05)} \text{ corrected for severity.}
\]

### Table 2 Proportion of patients diagnosed ‘too late’ and numbers of patients benefiting from the screening in populations subjected to different screening exposures

<table>
<thead>
<tr>
<th>Patients diagnosed ‘too late’</th>
<th>Calculation of numbers of patients benefiting from screening</th>
</tr>
</thead>
<tbody>
<tr>
<td>Imaginary population not exposed to child health centre screening (patients not referred by child health care physicians)</td>
<td>58</td>
</tr>
<tr>
<td>Population exposed to child health centre screening as actually performed</td>
<td>48</td>
</tr>
<tr>
<td>Population exposed to adequate screening</td>
<td>17</td>
</tr>
</tbody>
</table>
programme as presently performed in the south-west of the Netherlands for each child benefiting from it are estimated at $71,657. If all children were to be screened according to the guidelines, this amount would reduce to $14,950. If, as a result of intensifying the screening strategy, the number of false positive referrals were to double, this amount would increase to $21,279, while a quadrupled number of false-positive referrals would cause this amount to rise to $33,937.

**DISCUSSION**

Since by national agreement between paediatric centres, all children with cardiovascular disorders in the south-west of the Netherlands are referred to the Sophia Children’s Hospital, the study group in our effectiveness study (question 1) fairly represents all children with a congenital heart disease which becomes manifest before the age of 4, within a birth cohort of two years. However, a small minority of congenital heart malformations, as for instance some kinds of Atrium Septum Defects and the Coarctatio Aortae, may be quite asymptomatic in early childhood. So the results of our study may not be entirely applicable to this specific group.

From a methodological point of view the most appropriate design to evaluate the effectiveness of screening is a Randomised Controlled Trial (RCT). Should practical and ethical grounds render a RCT not feasible for evaluating a screening programme, which is already established and running, observational designs must be used. The merits and pitfalls of a patient follow-up study for the effect evaluation of this particular screening are extensively clarified elsewhere.

Although costs per screening are modest, because of its mass character, the total costs of child health centre screening for congenital heart disease are substantial, exceeding 20% of all costs of relevant congenital cardiac disorders. (Of which 8% is due to diagnostic procedures for mostly false-positives.) Differences in costs between patients diagnosed ‘too late’ and ‘in time’ are nearly entirely attributable to differences in intensive care hospitalization days. Since in this calculation we are exclusively dealing with patients with clinically significant disorders this finding is not surprising. All these children had major malformations, the vast majority of which required surgery or intervention catheterization. Such measures give rise to medium care hospitalizations, extended outpatient

**Table 3** Costs of congenital heart malformations diagnosed between 1 month and 4 years of age throughout a period of two years in the south-west of the Netherlands (in US dollars)

<table>
<thead>
<tr>
<th></th>
<th>N</th>
<th>Costs per patient</th>
<th>Total costs</th>
<th>%</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cost of screening</td>
<td>75,441</td>
<td>5.40</td>
<td>407,114</td>
<td>13</td>
</tr>
<tr>
<td>Cost of referrals</td>
<td>1,247</td>
<td>199</td>
<td>247,998</td>
<td>8</td>
</tr>
<tr>
<td>Cost of diagnosis and therapy of clinically insignificant disorders</td>
<td>207</td>
<td>433</td>
<td>89,659</td>
<td>3</td>
</tr>
<tr>
<td>Cost of diagnosis and therapy of clinically significant disorders</td>
<td>83</td>
<td>28,443</td>
<td>2,360,736</td>
<td>76</td>
</tr>
<tr>
<td>Total</td>
<td></td>
<td></td>
<td>3,105,507</td>
<td>100</td>
</tr>
</tbody>
</table>

**Table 4** Mean diagnostic and therapeutic costs of patients diagnosed 'too late' and 'in time' (in US dollars)

<table>
<thead>
<tr>
<th></th>
<th>'Too late'</th>
<th>'In time'</th>
<th>Difference</th>
<th>Difference corrected for severity</th>
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<tbody>
<tr>
<td></td>
<td>n=39</td>
<td>n=43</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Intensive care costs</td>
<td>7,967</td>
<td>3,122</td>
<td>4,845</td>
<td>(8,633 ; 1,057)</td>
</tr>
<tr>
<td>All other costs</td>
<td>24,025</td>
<td>22,126</td>
<td>1,880</td>
<td>(738 ; -3,628)</td>
</tr>
<tr>
<td>Total</td>
<td>31,993</td>
<td>25,249</td>
<td>6,725</td>
<td>(14,736 ; -1,286)</td>
</tr>
</tbody>
</table>

**Table 5** Costs and savings due to screening for each patient benefiting from screening in the present programme and if all patients were to be adequately screened in accordance with the guidelines (US dollars)

<table>
<thead>
<tr>
<th></th>
<th>Mean costs per each patient benefiting from screening</th>
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<tbody>
<tr>
<td></td>
<td>Present screening n=8 (see table 2)</td>
</tr>
<tr>
<td></td>
<td>Optimal screening n=34 (see table 2)</td>
</tr>
<tr>
<td>Cost of screening tests</td>
<td>407,114</td>
</tr>
<tr>
<td>Cost of false positive referrals</td>
<td>215,382</td>
</tr>
<tr>
<td>Savings, intensive care</td>
<td>-3,343</td>
</tr>
<tr>
<td>Total</td>
<td>71,657</td>
</tr>
<tr>
<td>Total if false positive referral = 200%</td>
<td>14,950 + 6,329 x 21,279</td>
</tr>
<tr>
<td>Total if false positive referral = 400%</td>
<td>14,950 + 3 x (6329 x 33,937)</td>
</tr>
</tbody>
</table>
surveillance and repeated diagnostic procedures in all cases. Children diagnosed 'too late', however, will have gone through periods of heart failure and hypoxemia more often. This will sometimes involve very serious symptoms. During such periods, these children will require treatment in intensive care units.

The mere fact that adequate screening for congenital heart disease appears to be quite effective and that it is a simple and inexpensive examination, does not prove that this preventive intervention is sufficiently cost-effective. This programme is an example of an intervention that would be sufficiently cost-effective if performed according to evidence-based criteria, but is actually cost-ineffective because of poor execution. This proves that evidence-based health policy not only concerns the introduction of efficient programmes, but also the monitoring of actual performance.

The fact that the present performance in the south-west of the Netherlands is rather poor, is as we explained elsewhere, mainly attributable to the quality of the actual examination by child health centre physicians. Improving this by upgrading (re)training programmes for this group, may increase the number of children adequately screened, and hence the number of patients timely diagnosed. This will finally bring down the expenditures per patient benefiting from screening. Even if such a policy substantially increases the number of referrals on the basis of false positive test results, these costs will still be less than the present expenditures.

CONCLUSION

Other cost-effectiveness evaluations on screening in child health care are extremely scarce and as far as congenital heart disease is concerned indeed non-existent. So comparisons are virtually impossible. However, the example in this paper supports the notion that not only child health care prevention programmes should be evidence-based but also their outcome and quality, monitored by periodically establishing whether they are adequately performed. We are convinced that this is not only true for expensive preventive programmes, such as screening for breast and cervical cancer, but also for relatively simple and inexpensive programmes such as in child health care. Otherwise we would be poundwise and pennyfoolish.

This study was supported by a grant from the Netherlands Heart Foundation.

The authors thank Ida Korfage MSc for calculating the costs attributable to homecare activities and Iet Juttmann-Punt for gathering data from the hospital files.

REFERENCES