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## Summary

Health Council of the Netherlands. Quality and quantity of allogenic stem cell transplants in children. The Hague: Health Council of the Netherlands, 2005; publication no. 2005/12.

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### Request for advice

Stem cell transplants are performed to restore or improve production of blood cells in bone marrow. The term allogenic stem cell transplant is used when the stem cells are taken from the blood or bone marrow of a donor. The intervention involves risks and is carried out on children who have certain blood diseases, such as leukaemia, and those with various metabolic diseases.

One of the quality criteria that the Health Council previously recommended for these interventions in children was that a minimum of 20 operations per year, per centre should be performed. The question arises of whether there are now reasons for reducing that number in the case of allogenic stem cell transplants for children.

### Link between quantity and quality

Research into treatment results has demonstrated that there is a link between quality and quantity in many cases. The results are on average poorer for centres that only perform a small number of a certain operation. The link has been found for all kinds of medical treatments but is most prominent for complicated interventions that involve high risks. For allogenic stem cell transplants it has also been demonstrated that the average mortality and morbidity rate is higher for small patient numbers.

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The link between quality and quantity was discovered by analysing large numbers of results. It is a statistical link rather than proof that a small number always leads to a poor result. The relationship between large numbers and a better result is probably explained by the fact that practitioners gain more experience through performing large numbers of operations and have a better opportunity to master the required knowledge. Furthermore, more space may be available for infrastructural facilities when patient numbers are large. However, it may also play a role that patients are referred to centres that achieve good results or that practitioners in large centres are more capable of selecting patients who would benefit from intervention.

### Recommended minimum number of stem cell transplants in the case of children

What does this link between quality and quantity mean for the required minimum number of stem cell transplants that should be performed per centre, per year on children? Whether the previously recommended minimum of 20 should be changed depends on whether there has been any change in the associated risks and the complexity of the interventions.

Allogenic stem cell transplants involve risks. The average mortality rate for interventions of this kind is 10 to 40 percent (depending on the nature and stage of the disease concerned and differences in the immune system of the donor and recipient). An important and severe complication is graft-versus-host disease (which arises because of immune reactions of the donor's stem cells to the patient's cells or tissue). The incidence of this complication has not decreased.

The complexity of an operation is also an important factor in stipulating a minimum number. After all, expertise can be gained more rapidly with less complex interventions. Allogenic stem cell transplants in children are complex because, among other reasons, they are performed for a wide range of rare disorders (immune deficiencies, inborn errors), which often require a specific approach. Interventions have also become more complex owing to the use of new treatment methods, such as donor-lymphocyte infusions and T-cell depletion.

The risks and complexity therefore do not provide grounds for revising previous advice to stipulate the minimum number of 20 operations per year, per centre. This number is slightly lower than the recommended minimum number of 25 allogenic stem cell transplants for adults.

Too small a number of patients is not only inadvisable in terms of the quality of treatment and patient safety; the efficiency of the relatively expensive operations is also important, because in many cases the costs per intervention are lower

with large numbers. Moreover, a small number makes it more difficult to pursue a quality policy, since statistical analysis of the results will produce less information. It is also not conducive to medical research, whereas this is precisely what is required with regard to allogenic stem cell transplants in children.