

Autologous stem cell transplantation for soft tissue sarcoma: insufficient research into therapy

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Due to a lack of suitable studies, it is unclear whether patients with soft tissue sarcoma can benefit from autologous haematopoietic stem cell transplantation. With this type of therapy, some of the patient's own (autologous) stem cells are removed at a convenient time and generally re-implanted after a course of high-dose chemotherapy. At present, there is neither indication nor proof of additional benefit compared to conventional chemotherapy. Owing to current gaps in knowledge, autologous stem cell transplantation for this disease should only be used within controlled trials at present. This is the conclusion of the final report of the Institute for Quality and Efficiency in Health Care (IQWiG), published on 25 November 2009.

Relatively rare type of cancer: 2,500 cases in Germany annually

Soft tissue sarcomas are malignant tumours whose tissue structure is similar to the soft tissues of the human body, particularly muscle, fat or supporting tissue. They often originate from these soft tissues. These tumours can appear anywhere in the body, with the lower extremities, i.e. legs, being the most frequently affected. This is a relatively rare type of cancer: the annual number of newly diagnosed cases in Germany is estimated at 2,500. Studies of patients with a tumour from the Ewing family of tumours were not included in the evaluation, as here the differentiation between soft tissue and bone tumours is not always clear-

cut.

Often a soft tissue sarcoma first manifests itself as a painless ulcer. It may cause pain when, for instance, it presses on other organs, which in turn might restrict their functioning. The tumour becomes life-threatening when it spreads to other organs or forms metastases in other parts of the body ("high-risk patients").

Tumours frequently recur

Surgery is the standard treatment for patients with a localized soft tissue sarcoma, whereas patients with distant [metastases](#) generally undergo chemotherapy. The aim is to kill off the [cancer cells](#).

Even if the chemotherapy appears to be successful, the disease soon recurs in a high number of patients. In certain cases, therefore, it is recommended that high-dose chemotherapy and autologous [stem cell transplantation](#) is followed immediately. Autologous stem cell transplantation is a planned rescue therapy for high-dose chemotherapy-related severe haematological toxicity. The increased dose is intended to reach those hidden cancer cells that may have hitherto survived - at least this is the assumption of this experimental procedure. However, the cancer cells may be resistant to the chemotherapy, irrespective of the size of the dose.

Autologous stem cell transplantation: stem cells are donated by patients

This type of high-dose [chemotherapy](#) usually damages vital haematopoietic stem cells in addition to the tumour cells. Consequently, stem cells are removed from the patient before the treatment and re-implanted afterwards. These stem cells mostly colonize the bone marrow

and stimulate haematopoiesis. If the transferred [stem cells](#) originate from the patient, this is known as autologous stem cell transplantation. IQWiG did not investigate allogenic stem cell transplantation, where the cells are donated by a donor.

IQWiG investigates benefit for high-risk patients

The focus of this report published by IQWiG is on "high-risk patients": these are patients who have an advanced localized or metastasized soft tissue sarcoma. In order to assess the benefit of autologous stem cell transplantation, IQWiG and its external experts compared the treatment results of patients with and without stem cell transplantation as presented in the scientific literature currently available.

Studies without a control group were also included

As with the disease itself, stem cell transplantation for soft tissue sarcomas is relatively rare. In 2005, for example, the European Group for Blood and Marrow Transplantation (EBMT) reported a total of only 69 autologous stem cell transplants as a result of a [soft tissue sarcoma](#) diagnosis. Considering the low number of cases and the probability of a low number of clinical trials overall, IQWiG and its external experts did not limit their search to specific study types.

Although studies without a control group, such as case reports, are generally associated with a high uncertainty of results, they were included in this benefit assessment. Under certain conditions, they can help to identify dramatic effects or adverse events.

All studies were susceptible to bias

IQWiG and its external experts included a total of 105 studies in its

assessment. Only 5 studies had a comparative design, i.e. each included a group receiving treatment either with or without autologous stem cell transplantation. In none of these 5 studies were the patients randomly assigned to one of the 2 groups, i.e. these were non-randomized studies. The remaining 100 studies described case series or individual cases.

Due to their design and the quality of their conduct, all studies were highly susceptible to bias. There were some contradictory results, and for this reason no robust results could be derived from the comparative studies, either.

Patients must be informed of uncertainty in quality of data

Despite the wide-ranging search for suitable studies, the evidence currently available is not sufficient to derive a possible additional benefit or harm from autologous stem cell transplantation in soft tissue sarcomas.

In IQWiG's opinion, therefore, the use of this type of therapy cannot be justified outside of controlled clinical trials. These trials would be the best way to close the gaps in knowledge and thus increase patient safety. Not all these clinical comparisons would have to be randomized trials. However, it would have to be ensured that the therapy results were actually comparable.

IQWiG considers it essential that patients are fully informed of the currently uncertain quality of data before deciding for or against such a treatment.

Source: Institute for Quality and Efficiency in Health Care

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