

## „Patient-focused clinical research is severely underfinanced“



*Prof. Stefan Bielack's, MD, statements on present state of the art research for bone and soft tissue tumours*

**WIR-Interview** (May 2009, Stuttgart). The paediatric oncology department at the Olga Hospital in Stuttgart is one of the largest special divisions for cancer-stricken children and adolescents in the German-speaking region, specialising in the diagnosis and treatment of soft tissue and bone sarcomas.

On the occasion of the bone and soft tissue tumour symposium held on 15 and 16 May 2009, a country-wide symposium for patients and family members organised in Stuttgart by the German Paediatric Cancer Foundation (DKS), Prof. Stefan Bielack explained the current state of research in an interview published in the German Childhood Cancer Foundation's magazine WIR.

**WIR: Sarcomas are relatively rare in children and adolescents. Why are they so dangerous all the same?**

*Bielack:* First of all, uncommonness and danger have little in common. In fact, rare illnesses may well have a tragic outcome for those affected by them. Sarcomas are dangerous because they can infiltrate and destroy healthy organs and tissue at the location where they grow. And what's even worse is that they have the potential of spreading throughout the body. The filiae, also known as metastases, can affect virtually any part of the body and wreak havoc there."

**WIR: What do we know about the development of sarcomas?**

*Bielack:* In the vast majority of affected children and adolescents, sarcomas develop spontaneously, i. e. without any obvious reason. An inherited disposition for

developing sarcomas or additionally other types of cancer is only seen relatively rarely, but in these cases, other occurrences have usually been reported in the immediate family. So-called radiation-induced sarcomas are unique; they are sarcomas induced by radiation conducted to treat a different type of cancer.

**WIR: Is the early detection of sarcomas possible?**

*Bielack:* Unfortunately, this is extremely difficult, because sarcomas usually do not stand out based on easily recognisable symptoms. We should consider them in case of prolonged, unexplained tenderness of the bones or new swelling of the soft tissue. Yet, on average two to three months and sometimes much more time elapses between the first signs of the illness and the proper diagnosis.

**WIR: How are sarcomas treated?**

*Bielack:* Sarcomas always require an adequate local therapy, meaning that the tumour needs to be surgically removed and sometimes treated with radiation. To prevent metastatisation, treatment of the vast majority of sarcomas additionally requires very intensive chemotherapy consisting of several drugs. Often, part of the chemotherapy is given prior to the surgery to reduce the size of the sarcoma and to allow the gentler and safer conduct of the surgery. In Germany, all children and adolescents afflicted with sarcoma are offered a therapy according to treatment protocols developed by a panel of experts of our association in cooperation with select partners in Germany and abroad based to the latest know-how. To resolve any arising problems, the panel of experts is helping the involved hospitals with words and deeds. This form of quality assurance helps achieve better outcomes in the therapy of paediatric sarcomas in our country compared to the majority of countries around the world.



**WIR: What main changes in the treatment have taken place in the past 20 years?**

*Bielack:* Unfortunately only very little has changed in terms of the healing rates: as early as in the mid 1980s, the same drugs were used and approximately identical treatment successes achieved as these days. However, today we are much better able to manage acute and chronic side

effects of chemotherapy and so the therapy is not as stressful any more as it used to be. As well, radiation can be used much more systematically and controlled these days. Major progress was made with respect to surgery, especially for the treatment of bone sarcomas in young patients. While the majority of patients were faced with amputation 20 years ago, this is usually preventable these days. Currently, the amputation rate is as low as 10 percent. For a few forms of sarcoma, drugs that specifically suppress the tumour's own growth signals and thus stop the progress of the illness have recently been launched. We are hoping that such drugs can be developed for as many types of sarcomas as possible.

**WIR: How do patients actually benefit from the improved diagnostic and treatment options?**

*Bielack:* Thanks to excellent imaging procedures, we are better able to determine the extent of the metastatisation and we are able to see how a sarcoma responds to the administered chemotherapy. This gives us the opportunity to treat the patient according to his/her personal risk. Drugs designed to reduce certain serious unwanted effects of chemotherapy such as nausea and alterations in the blood count are available. Surgeries can much more often be performed without disfigurement, and the radiation is more systematic and therefore associated with fewer accompanying impairments compared to just a few years ago.

**WIR: In your opinion, what are the main medical responsibilities in the near future in terms of diagnostics and cure?**

*Bielack:* Clearly the implementation of the now very extensive knowledge about the tumour biology of sarcomas into the

development and testing of new drugs with a specific effect. First, we need to find out for which sarcomas these kinds of drugs are feasible and effective and in a second step they need to be integrated into the standard of care. However, for the majority of sarcomas this will only be possible in addition to conventional chemotherapy. Unfortunately, only a few rare forms of sarcoma will be curable with novel substances alone.

**WIR: In your opinion, the limits and possibilities of medicine are far from having been reached?**

*Bielack:* For me, the possibilities and limits of medicine are only reached once all children and adolescents with sarcomas can be cured and then lead a completely normal life. Although this is a very ambitious goal, we must not be satisfied until it is achieved.

**WIR: Research is extremely expensive. Are the necessary funds available?**

*Bielack:* Unfortunately, the patient-focused clinical research is severely underfinanced throughout Europe. This is particularly true for therapy optimisation trials as they are conducted for the better care of cancer-stricken children and adolescents. On the one hand, the conduct of these trials is extremely complicated and expensive due to excessive bureaucratic conditions and rather unattractive for the pharmaceutical industry on the other hand: cancer in this age group is simply too uncommon and therefore, large scale-investments are not financially worthwhile for the companies. Luckily, supporters like the German Paediatric Cancer Foundation, the German Cancer Support and the German Research Association are sponsoring the sarcoma study groups of our association in their quest for better therapies. It is crucial that patients and their families also help voicing their concerns so that rare illnesses such as sarcomas are not forgotten among politicians and research sponsors and progress can continue to be made.

Prof. Stefan Bielack was interviewed by Klaus Riddering.

**Prof. Dr. Stefan Bielack**, scientific head of the nationwide conference for patients and relatives of sufferers of “bone and soft tissue cancers” organised by the German Childhood Cancer Foundation, is the medical director for paediatric oncology at the Olga **Hospital in Stuttgart** – one of the largest specialist departments for children and young people suffering from cancer in the German-speaking region, with a particular focus on the diagnosis and treatment of sarcomas of the soft tissue and bones. The clinic is the home of the **cooperative soft tissue sarcoma study group CWS** and the **cooperative osteosarcoma study group COSS**, two respected international research associations.

On the occasion of the 22<sup>nd</sup> annual conference of the European **Musculoskeletal Oncology Society (EMSOS)** in Stuttgart, within the framework of which the nationwide conference of the German Childhood Cancer Foundation for patients and relatives of sufferers of “bone and soft tissue cancers in children and young people” also took place, Bielack was elected to the post of EMSOS president for the next three years. EMSOS, the leading European association in the field of tumours of the musculoskeletal system, was **founded in 1987 by Europe-wide leading orthopaedic surgeons, oncologists and other specialists** involved in diagnosis and treatment, with the **aim of creating an international platform for scientific exchange**.