

PATIENT GROUP WELCOMES NICE APPROVAL OF TRABECTEDIN 21st December 2009

Today's announcement that the National Institute for Health and Clinical Excellence (NICE) has approved the drug trabectedin (trade name Yondelis) for use by patients with advanced soft tissue sarcoma is very welcome.

There have been no new treatments for this heterogeneous group of rare cancers for more than a quarter of a century. Traditional chemotherapies get a poor response when used to treat advanced disease and although a small percentage of patients achieve remission survival rates are low.

Trabectedin was developed from the toxin of a Caribbean sea squirt by the Spanish company Pharmamar. It was licensed by the European Union in 2007, with the regulators recognising that it was an innovative treatment addressing an unmet need. Clinical trials across the world had refined knowledge of the drug and its activity so that clinicians were confident they knew how to use it, and which patients were most likely to respond well, but the levels of evidence of efficacy and benefit which are normally required for licensing were impossible to acquire because of the rarity of the disease.

This shortage of evidence for trabectedin has given the UK's health technology assessment processes, and NICE in particular, some new challenges. There have been times when it looked as though this significant new treatment might be refused because sufficient evidence did not exist, even though it would be impossible to gain that evidence.

"I welcome the decision and have a great sense of relief that this journey is now over," said Roger Wilson, Director of patient information and support group Sarcoma UK. "Patients who have had access to trabectedin through individual funding arrangements made by Primary Care Trusts have gained real benefits, with some having a high quality of life for long periods, well beyond their original prognosis."

"Pharmamar deserve to be congratulated for their commitment to this innovative treatment, and thanked for their support to UK patients shown through the patient access scheme agreed with the Department of Health."

The UK's busiest sarcoma treatment centre is the Royal Marsden Hospital in London. Head of the Sarcoma Unit, Professor Ian Judson, comments; "I welcome the news that this drug will now be made available for all patients who are likely to respond to it. We have observed prolonged benefit in some patients and as a result have pushed hard to obtain it from PCTs, some of whom have been reluctant, perhaps understandably, owing to the high cost of the drug. "

NOTES FOR EDITORS

Soft tissue sarcomas are cancers of connective tissue - muscles, nerves, tendons, fatty tissue etc. There are over 50 different histological sub-types, yet only about 2000 patients are diagnosed each year in the whole UK – less than 1% of all cancer diagnoses. The primary treatment is surgery, which can be a cure if the tumour is not too large and is diagnosed early enough. If the disease metastasises (spreads as a secondary cancer) it usually moves to the lungs, occasional to the liver, brain or bones, and becomes very difficult to treat.

The first line chemotherapy, doxorubicin, entered clinical use in the 1960s. It is a systemic cytotoxic chemotherapy used widely in the treatment of cancer. While some 30% of soft tissue sarcoma patients initially respond, the response is rarely long lasting. Second-line treatments include other older chemotherapies such as ifosfamide and dacarbazine. The struggle to find a better treatment has been the subject of international clinical trials for two decades, with no real success until now.

Trabectedin fills this gap. Its value has been clearly demonstrated in three of the commoner soft tissue sarcomas – leiomyosarcoma, myxoid liposarcoma and synovial sarcoma. It is anticipated that other sub-types will also respond but further studies are needed before clinical practice is extended.

SARCOMA UK

Sarcoma UK was set up in 2004 to provide sarcoma patients in the UK with information, support and advocacy. It is affiliated to The Sarcoma Trust, set up in 2007 as a charity supporting awareness of, and research into, sarcoma.

The UK's network of specialist treatment centres are all creating patient support groups with help from Sarcoma UK. All UK patients have access to printed information and an extensive website. Patients working as volunteers through Sarcoma UK represent sarcoma patient interests on a number of NHS bodies, including the National Cancer Research Institute.

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