

## **A prospective, large real-life study shows trabectedin confers long-term efficacy in soft tissue sarcoma**

**Vienna and Madrid, September 26<sup>th</sup> 2015:** PharmaMar today announced real-world data from a large prospective post-commercialization study in patients with advanced soft tissue sarcoma (STS) in Europe confirming the long-term anticancer activity of YONDELIS<sup>®</sup> (trabectedin) in different subtypes of sarcoma.

"The results from this interim analysis are in line with the efficacy demonstrated in clinical trials and, more important, are consistent with what we find in clinical practice," says Dr. Nicolas Penel, MD, PhD, Department of Medical Oncology, Centre Oscar Lambret, France. "Patients with soft tissue sarcoma are certainly benefiting from this treatment, and studies such as this one continue adding value to the management of patients treated with trabectedin."

The non-interventional, multicenter, prospective Phase IV study has recruited 217 patients from 41 European centers. Among the patients, there were 27 different subtypes of soft tissue sarcoma with leiomyosarcoma and liposarcoma being the two most common ones (41.9% and 23.5%, respectively). Although most of the patients received chemotherapy before enrollment, 10% of the patients received trabectedin in first line therapy.

The median progression-free survival was 5.5 months at the time of this analysis measured by RECIST, Choi or clinical evaluation. The data also showed some durable response with 18 patients still on treatment when this analysis was performed. "It was interesting to see how more than half of the patients receive 6 cycles or more of this treatment and 16.1% of the patients received the drug for 1 year or more", pointed out Dr. Penel, who added that "given the manageable safety profile of trabectedin, the data underscore the clinical benefit for these patients."

Retrospective Choi criteria showed a different pattern of response compared to RECIST in 44 patients, in which median PFS was 15.3 months versus 8.1 months when measured by Choi and RECIST, respectively.

The lead author Nicolas Penel, MD, PhD, Department of Medical Oncology, Centre Oscar Lambret, France will present the full data today at the 18th ECCO – 40th ESMO European Cancer Congress in Vienna (ECC 2015) (Abstract#3435, Saturday, Sep 26 from 16:15 - 18:45 at Hall C Poster Board P300).

#### **About the Phase IV Y-IMAGE study with trabectedin:**

- ❖ Y-IMAGE is a non-interventional, multicenter, prospective Phase IV study of trabectedin in patients with advanced soft tissue sarcoma. The trial enrolled 217 patients with 27 sarcoma histotypes across 41 centers in Europe that were receiving trabectedin in the approved label (83.9% of patients previously treated mostly with anthracyclines and/or ifosfamide and 10.1% of patients were naïve). The aim of the study is to evaluate trabectedin in clinical practice to obtain real-world data, comparing radiological response assessment measured by RECIST Response Evaluation Criteria In Solid Tumors) or Choi criteria<sup>i</sup>.
- ❖ The primary objective is PFS and secondary endpoints include objective response rate, disease control rate, overall survival and safety. The data shown here are from an interim analysis of the primary endpoint.
- ❖ Median PFS was 5.5 months assessed by RECIST, Choi and clinical evaluation after 166 PFS events. The median treatment duration was 5.5 months and 16.1% received trabectedin for one year or more. At 3 and 6 months, 69.4% and 46.9% did not have disease progression.
- ❖ RECIST versus Choi evaluation showed a median PFS of 15.3 months and 8.1 months, respectively.
- ❖ The most frequent grade 3 and 4 were neutropenia (17.9%) and transaminase elevation (7.9%). Febrile neutropenia occurred in 2.7% of patients and most common grade 3 adverse events include fatigue (3.7%), nausea (1.4%), and vomiting (1.4%). A fatal case was recorded as well.

#### **About Soft Tissue Sarcoma**

Soft tissue sarcoma is a type of cancer originating in the soft tissues that connect, support and surround other body structures, such as muscle, fat, blood vessels, nerves, tendons and the lining of joints<sup>1,2</sup>. In the United States, nearly 12,000 people will be diagnosed and approximately 4,870 are expected to die of soft tissue sarcomas in 2015<sup>ii</sup>. Leiomyosarcoma is an aggressive type of soft tissue sarcoma that occurs in smooth muscles, such as those in the uterus, gastrointestinal tract or lining of blood vessels<sup>iii</sup>. Liposarcoma originates in fat cells and most commonly occurs in the thigh and abdominal cavity, though it can occur in fat cells in any part of the body<sup>iv,v</sup>.

**About YONDELIS® (trabectedin)**

YONDELIS® (trabectedin) is a novel, multimodal, synthetically produced antitumor agent, originally derived from the sea squirt, *Ecteinascidia turbinata*. The drug exerts its activity by targeting the transcriptional machinery and impairing DNA repair. It is approved in 78 countries in North America, Europe, South America and Asia for the treatment of advanced soft tissue sarcomas as a single-agent and for relapsed ovarian cancer in combination with DOXIL®/CAELYX® (doxorubicin HCl liposome injection). Under a licensing agreement with PharmaMar, Janssen Products, L.P. has the rights to develop and sell YONDELIS® globally except in Europe, where PharmaMar holds the rights, and in Japan, where PharmaMar has granted a license to Taiho Pharmaceuticals.

**About PharmaMar**

Headquartered in Madrid, PharmaMar is the world-leading biopharmaceutical company in advancing cancer care through the discovery and development of innovative marine-derived anticancer drugs. The company has a rich pipeline of drug candidates and a robust R&D oncology program. YONDELIS® is commercially available in 78 countries for the treatment of advanced soft tissue sarcomas and for relapsed platinum-sensitive ovarian cancer. PharmaMar develops and commercializes YONDELIS® in Europe and has three clinical-stage programs under development for several types of solid and hematological cancers, PM1183, plitidepsin, and PM60184. PharmaMar is a global biopharmaceutical company with subsidiaries in Germany, Italy, France, Switzerland and the United States. To learn more about PharmaMar, please visit us at [www.pharmamar.com](http://www.pharmamar.com).

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<sup>i</sup> [http://www.nature.com/bjc/journal/v102/n5/fig\\_tab/6605567t1.html#figure-title](http://www.nature.com/bjc/journal/v102/n5/fig_tab/6605567t1.html#figure-title)

<sup>ii</sup> American Cancer Society: Cancer Facts and Figures 2015. Atlanta, Ga: American Cancer Society, 2015 <http://www.cancer.org/acs/groups/content/@editorial/documents/document/acspc-044552.pdf>. Accessed: May 2015.

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iii The Liddy Shriver Sarcoma Initiative. An Introduction to Leiomyosarcoma of the Bone and Soft Tissue. Available at: <http://sarcomahelp.org/leiomyosarcoma.html>. Accessed: May 2015.

iv The Liddy Shriver Sarcoma Initiative. What is Liposarcoma? Available at: <http://sarcomahelp.org/liposarcoma.html>. Accessed: May 2015.

v University of Rochester Medical Center. Liposarcoma. Available at: <http://www.urmc.rochester.edu/encyclopedia/content.aspx?ContentTypeID=134&ContentID=221>. Accessed: May 2015.