PHASE II STUDY EVALUATING IMATINIB TO INDUCE PROGRESSION ARREST IN RECIST PROGRESSIVE DESMOID TUMORS NOT AMENABLE TO SURGICAL RESECTION WITH R0 INTENT OR ACCOMPANIED BY UNACCEPTABLE FUNCTION LOSS - A STUDY OF THE GERMAN INTERDISCIPLINARY SARCOMA GROUP (GISG)

Bernd Kasper[1], Viktor Gruenwald[2], Peter Reichardt[3], Sebastian Bauer[4], Geraldine Rauch[5], Michaela Sommer[1], Peter Hohenberger[1]

[1] University of Heidelberg, Mannheim University Medical Center, Interdisciplinary Tumor Center Mannheim, Sarcoma Unit, Mannheim, Germany; [2] Department of Hematology, Hemostasis, Oncology, and Stem Cell Transplantation, Hannover Medical School, Hannover, Germany; [3] HELIOS Klinikum Bad Saarow, Sarcoma Center Berlin-Brandenburg, Bad Saarow, Germany; [4] Sarcoma Center, West German Cancer Center, Essen, Germany; [5] University of Heidelberg, Institute of Medical Biometry and Informatics, Heidelberg, Germany.

Background: Desmoid tumors describe a rare monoclonal, fibroblastic proliferation characterized by a variable and often unpredictable clinical course. Surgery is the therapeutic mainstay for progressing patients, except if mutilating and associated with considerable function loss. For advanced disease different treatment approaches have been investigated and promising results could be demonstrated using imatinib. **Methods:** Therefore, we initiated a phase II trial within the GISG evaluating imatinib to induce progression arrest in desmoid tumor patients not amenable to surgical resection with R0 intent or accompanied by unacceptable function loss (NCT01137916). Major eligibility criteria were a histological confirmed desmoid tumor showing progressive disease according to RECIST 1.0 within six months. Patients were treated with a planned dose of 800 mg imatinib daily over two years. Primary endpoint was the non-progression rate after six months of treatment. Eleven out of 37 evaluable patients would be necessary to conclude a positive study result. Accrual started in July 2010 in five GISG centers and finalized in September 2013. **Results:** The final analysis for the primary endpoint showed that 24 out of 37 evaluable patients were progression-free at six months of imatinib treatment and reached the primary endpoint. Response assessment after six months revealed one partial response (3 %) and 23 stable diseases (62 %). Out of the 13 patients counted as non-successors, ten patients had documented disease progression (27 %). One patient terminated due to toxicity and there were two study withdrawals. **Conclusion:** With a 65 % progression arrest rate at six months after start of treatment, imatinib clearly exceeded the primary endpoint in this GISG trial encouraging further investigation of imatinib in this histology. Follow-up will continue until the end of the two years treatment duration.