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# Overall survival benefit with masitinib mesylate in imatinib-naive, locally advanced, or metastatic gastrointestinal stromal tumor (GIST): 4-years follow-up of the French Sarcoma Group phase II trial.

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**Abstract:**

**Background**: Masitinib is a novel tyrosine kinase inhibitor which, in vitro, has greater activity and selectivity than imatinib (IM) against both wild-type KIT receptor and its mutated form in the juxtamembrane region (IC 50=100 nM versus 200 nM for IM, 3 nM versus 27nM and 40 nM versus 120nM, respectively, for exons 9,11, and 13). This multicenter phase II study evaluated efficacy and safety of masitinib as a first-line treatment of advanced GIST. **Methods**: IM-naïve patients with inoperable, locally advanced or metastatic GIST received oral masitinib (7.5 mg/kg/day) until progression, refusal or toxicity. Efficacy variables included response rate, best response (RECIST), progression-free survival (PFS) and overall survival (OS). Initial results were previously reported in EJC 2010. We present here the same series with updated PFS and OS (median follow up of 48 months). **Results**: 30 patients with a median age of 58 years (60% of males) were included from June 2005 to April 2007 in five French institutions. At the cut-off date (31 august 2010), 9 patients are still under treatment with a median treatment duration of 41 months (min=33, max=52). Two additional progressions have been reported for a total of 14 events (13 progressions and 1 death). Updated median PFS is 41 months (95% CI: [17.5; NR]) with PFS rates of 60% [39; 77], 56% [35; 73] and 45% [24; 64] respectively at 2, 3 and 4 years. With 8 patients dead, median OS is not yet reached with OS rates of 90% [72; 97], 87% [68; 95] and 74% [52; 87], respectively, at 2, 3, and 4 years. The main frequent relevant grade 3 toxicities were: rash (10%), neutropenia (7%) and abdominal pain (7%) with one patient presented a grade 4 skin exfoliation. No other relevant long-term toxicities were reported and no more patients discontinued treatment due to suspected toxicity. **Conclusions:** The long term results observed with masitinib confirm a very interesting activity with prolonged PFS and OS. These results support the head to head comparison with imatinib in the currently ongoing phase III randomized clinical trial in first line locally advanced or metastatic GIST patients.