Aim: Since 2002 imatinib mesylate (IM) is registered for patients with advanced irresectable/metastatic GIST. A subset of patients has a (very) long-term response to IM therapy.

Methods: Clinical data were retrospectively collected from 4 international databases (Poland, France, Czech Republic, the Netherlands). Eligible patients had advanced GIST and started with IM between November 2000 and January 2009. We assessed biological and clinical factors associated with the duration of IM treatment and overall survival (OS).

Results: A total of 643 patients were identified with a median follow-up of 7.9 years, of whom 24 were still on IM at last follow-up. Median OS was 7 years (95% CI 6.1-7.7), median IM treatment time was 35 months (95% CI 31-41), with 226 and 40 patients being treated for more than 5 and 10 years, respectively. Factors associated with a response duration >5 years, tested by univariate analysis, were complete surgical resection of the primary tumour (p<0.05), good WHO performance status (PS 0) (p<0.0001), KIT exon 11 mutation (p<0.05) and normal baseline haemoglobin (p<0.05), leukocytes (p<0.05), neutrophils (p<0.05) and albumin levels (p<0.0001). Patients with local recurrence or metastases in the liver only were on IM for a longer period compared to patients with peritoneal metastases (p<0.05). No additional factors could be identified in the (small) cohort of patients responding more then 10 years. Remarkably, in our analysis no statistically significant influence of the primary tumour size, location of the primary tumour and mitotic index (at the time of diagnosis) on both overall survival and duration of IM treatment could be demonstrated.

Conclusions: GIST patients with a complete resection of the primary tumour, good WHO performance status, KIT exon 11 mutation and normal haemoglobin, leucocytes, neutrophils and albumin levels at baseline are more likely to respond to IM therapy for a longer period. Since data on patients responding over 10 years remain limited, a case-control study will be initiated to further elucidate factors influencing very long term responses.

Disclosure: H. Gelderblom: research grants from Novartis en Pfizer; A. Le Cesne: Honoraria: Novartis, Pfizer, Pharmamar, GSK for advisory boards; P. Rutkowski: honoraria and Advisory Board for Novartis, Advisory Board for Bayer, travel grants from Pfizer; N. Steeghs: Grants for Netherlands GIST registry (Novartis, Pfizer en Bayer). All other authors have declared no conflicts of interest.