

SUPPLEMENTARY MATERIAL

Table 1S. Baseline Characteristics of the Patients included in the RATIFY study

| | All Patients (N = 717) | Midostaurin Group (N = 360) | Placebo Group (N = 357) |
|--|---------------------------|--------------------------------|-------------------------------|
| Age at trial entry — yr | | | |
| Median | 47.9 | 47.1 | 48.6 |
| Range | 18.0–60.9 | 19.0–59.8 | 18.0–60.9 |
| Female sex — no. (%) | 398 (55.5) | 186 (51.7) | 212 (59.4) |
| Race — no./total no. (%)† | | | |
| White | 275/309 (89.0) | 147/165 (89.1) | 128/144 (88.9) |
| Other | 34/309 (11.0) | 18/165 (10.9) | 16/144 (11.1) |
| Subtype of <i>FLT3</i> mutation — no. (%)‡ | | | |
| TKD 162 (22.6) 81 (22.5) 81 (22.7) | | | |
| ITD with low allelic ratio | 341 (47.6) | 171 (47.5) | 170 (47.6) |
| ITD with high allelic ratio | 214 (29.8) | 108 (30.0) | 106 (29.7) |
| Modified European LeukemiaNet classification — no./total no. (%)§ | | | |
| Favorable | 29/547 (5.3) | 16/269 (5.9) | 13/278 (4.7) |
| Normal | 375/547 (68.6) | 172/269 (63.9) | 203/278 (73.0) |
| Intermediate II | 104/547 (19.0) | 59/269 (21.9) | 45/278 (16.2) |
| Adverse | 39/547 (7.1) | 22/269 (8.2) | 17/278 (6.1) |
| White-cell count per µl¶ | | | |
| Median | 34,900 | 35,600 | 33,000 |
| Range | 600–421,800 | 600–421,800 | 800–329,800 |
| Platelet count per µl | | | |
| Median | 50,000 | 50,000 | 50,000 |
| Range | 2000–461,000 | 2000–461,000 | 8000–444,000 |
| Absolute neutrophil count per mm ³ ** | | | |
| Median | 2.2 | 2.2 | 2.3 |
| Range | 0–55.9 | 0–55.9 | 0–55.9 |

† Race was reported by the patients. Race was not reported for European patients (195 in the midostaurin group, and 213 in the placebo group).

‡ The subtypes of the *FLT3* mutation are point mutation in the tyrosine kinase domain (TKD) or internal tandem duplication (ITD) mutation with either a high ratio (>0.7) or a low ratio (0.05 to 0.7) of mutant to wild-type alleles.

§ Cytogenetic data according to a modified European LeukemiaNet classification were available for 547 patients (269 in the midostaurin group, and 278 in the placebo group). Data on mutations in the nucleophosmin gene (*NPM1*) or the CCAAT/enhancer binding protein alpha gene (*CEBPA*) are not included. A classification of favorable indicated the presence of t(8;21) and inv(16) or t(16;16), normal the presence of a normal karyotype, intermediate II the presence of cytogenetic abnormalities that were not classified as favorable or adverse, and adverse the presence of adverse-risk cytogenetic abnormalities.

¶ Data were available for 707 patients (355 in the midostaurin group, and 352 in the placebo group).

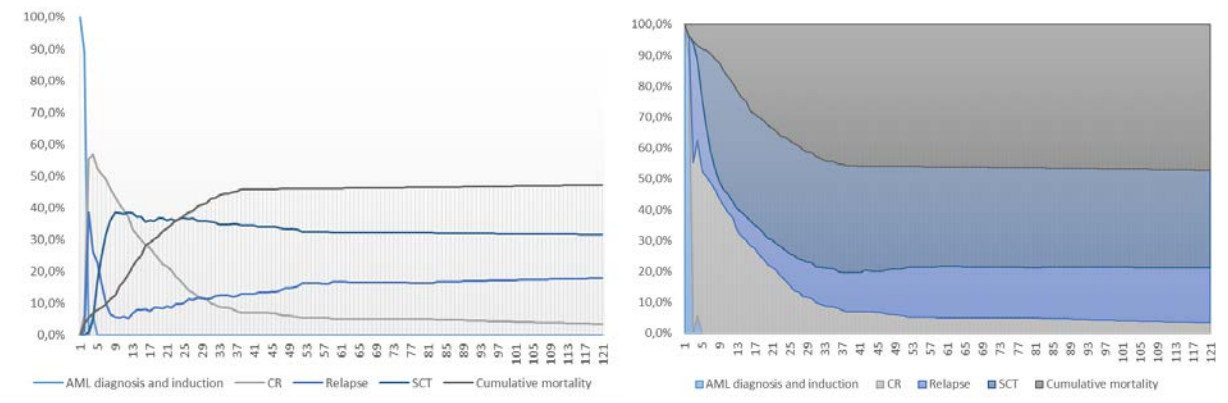
|| Data were available for 702 patients (351 in the midostaurin group, and 351 in the placebo group).

** Data were available for 673 patients (339 in the midostaurin group, and 334 in the placebo group).

This table was taken from: Stone RM, Mandrekar SJ, Sanford BL, et al. Midostaurin plus Chemotherapy for Acute Myeloid Leukemia with a *FLT3* Mutation. *N Engl J Med*. 2017;377(5):454-464. doi:10.1056/NEJMoa1614359

Figure 1S. Model outcomes and traces with midostaurin (a) or with standard chemotherapy (b)

a) Midostaurin



b) Standard chemotherapy

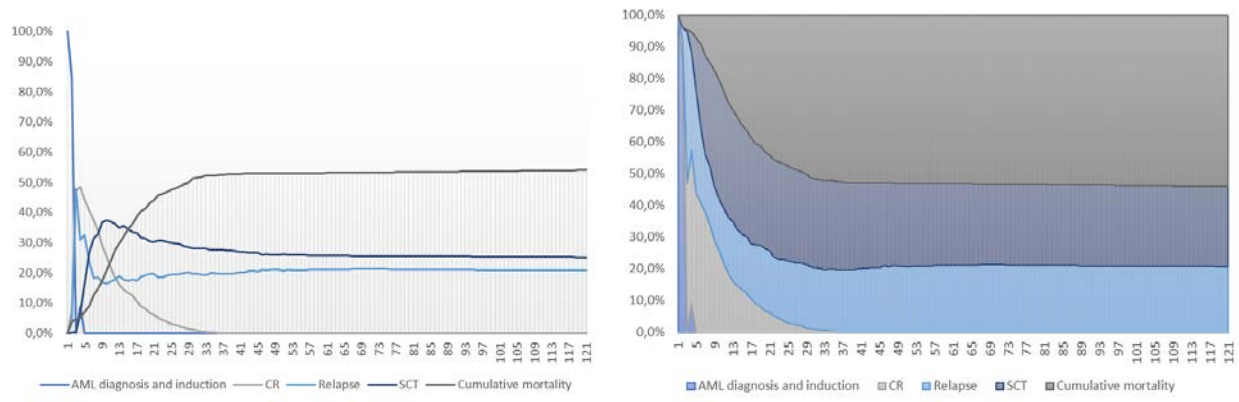


Figure 2S. Proportion of patients reaching each treatment line in the RATIFY study

