Supplementary Online Content


eFigure. Natural History Kaplan-Meier Sensitivity Analyses for Untreated Cohort by Baseline Characteristics

This supplementary material has been provided by the authors to give readers additional information about their work.
eFigure. Natural History Kaplan-Meier Sensitivity Analyses for Untreated Cohort by Baseline Characteristics

The number of patients at risk are presented below the x-axis. The numbers in parentheses underneath a given time point on the x-axis are the number of deaths that occurred before or on that time point and after the previous time point on the axis. P-values are based on the log-rank test. Time 0 on the x-axis (i.e., beginning of patient being at risk) is date of birth.

A. **Untreated Male vs. Female**: Includes patients never treated plus treated patients that were censored at age of treatment initiation. There were 59 deaths in 123 untreated females; there were 65 deaths in 128 untreated males. Mean and median survival in untreated females were 13.7 and 14.5 years, respectively; mean and median survival in untreated males were 15.1 and 14.9 years, respectively. Median (quartiles) follow-up time is 9.5 years (6.0 – 13.9 years) for females and 10.0 years (5.0 years – 14.8 years) for males.
Untreated Born Before 1991 vs. Born 1991 or Later: Includes patients never treated plus treated patients that were censored at age of treatment initiation. There were 69 deaths in 90 untreated patients born before 1991; there were 53 deaths in 166 untreated patients born 1991 or later (note, birth year was missing for two patients, and both patients died). Mean and median survival in untreated patients born before 1991 were 14.9 and 14.8 years, respectively; mean and median survival in untreated patients born 1991 or later were 13.8 and 14.4 years, respectively. Median (quartiles) of overall follow-up time is 13.1 years (9.3 – 16.3 years) for patients born before 1991, and 8.3 years (4.0 years – 11.6 years) for patients born 1991 or later.
C. Untreated Known vs. Unknown Progerin-producing LMNA Mutation Status. Only includes patients never treated. Of the 195 patients not treated with lonafarnib, 79 had known genetic diagnosis (and 116 did not). However, 16 of these 79 patients eventually received trial treatment other than lonafarnib, yielding 63 never-treated patients with known genetic diagnosis for this figure. There were 42 deaths in 63 untreated patients with known status; there were 82 deaths in 116 untreated patients with unknown status. Mean and median survival in untreated patients with known status were 12.9 and 13.5 years, respectively; mean and median survival in untreated patients with unknown status were both equal to 14.5. Median (quartiles) of overall follow-up time is 10.7 years (6.1 – 14.6 years) for patients with known status, and 12.3 years (8.3 years – 15.8 years) for patients with unknown status.