Epidemiological Modelling to Estimate the Number of US Patients with Multiple Myeloma at Different Lines of Treatment

Methods

A compartmental model based on differential equations was developed, modelling population group moves between treatment phases over time.

Patients transition through four main compartments representing different LOT: first (LOT1), second (LOT2), third (LOT3), and after third (LOT4+).

- Transition to next LOT is defined as a change in therapy due to progression, toxicity or any other reason.
- Patients in LOT1-3 could include patients who could transition to the next LOT or die.
- In LOT4+, patients could change treatment type when they transitioned to the next LOT (Figure 1).

For SCT eligible patients, the first LOT is represented by induction, SCT, and maintenance therapy post-SCT.

The model estimated patient numbers in 4 strata based on combinations of age group (65 or older), cytogenetic risk (high, standard, or unknown) and whether patients received DAR at some point during their treatment course. For each stratum, the model output is derived from the inputs of the Please refer to the model for details.

Results

Overall distribution of patients with MM in the US

At steady-state conditions, the model projected an overall MM prevalence of 128,093 patients in the US in 2020. Of these, 20,182 were asymptomatic and 1,598 symptomatic (untreated), with 106,043 receiving treatment (of whom, LOT1: 54% [n=56,792]; LOT2: 27% [n=28,287]; LOT3: 10% [n=10,629]; LOT4+: 10% [n=10,389]).

The estimate of 128,093 patients compares well with an estimate derived from the Surveillance, Epidemiology, and End Results program (SEER) 2016 data of 131,392 (based on a population size of 327,459,041 and implied incidence of 9.81 per patient-year).6,7

Patients with prior exposure to a PI, an immunomodulatory agent, and/or an anti-CD38 antibody (represented by DAR)

An estimated 50% (516,10389) of patients in LOT4+ (standard deviation [SD] by probabilistic sensitivity analysis=1,561,26) had prior exposure to a PI, an immunomodulatory agent, and/or an anti-CD38 mAb as represented by DAR.

Sensitivity analysis

Model estimates of the number of US patients with MM at LOT4+ with prior PI, immunomodulatory agents, and DAR exposure were most sensitive to mortality, TNT, and the proportion of patients receiving DAR in LOT4+ (Figure 3).

Conclusions

This study is among the first to estimate the current number of patients with MM in the US by LOT, accounting for recent changes in the treatment landscape. Our up-to-date estimate can be used as a basis for understanding patient treatment experience and need in this difficult-to-treat population.

The epidemiological model allows for projections of the number of patients by LOT with certain treatment exposure characteristics (e.g., patients in LOT4+ whose prior treatments include PIs, immunomodulatory agents, and anti-CD38 mAbs). This modelling framework can be adapted to other settings outside the US.

Study limitations include: input derivation for TNT and OS was based on exponential distribution; target population prediction is based on steady-state while in the model, the target population evolves in response to changes; model inputs were based on multiple real-world effectiveness studies potentially biasing results due to heterogeneity across studies; DAR market shares are expected to evolve.

Disclosures

AM, MD, and JCR are employees of Evidera, CN, FW, and YG are employees of and hold stocks and shares in GlaxoSmithKline. NJ was employed by GlaxoSmithKline at the time of the study. Funding

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Figure 2. Model prediction of the US MM population size by LOT

Figure 3. OWSA for predicted prevalence of MM in the US

Figure 1. Modelled patient flow from diagnosis to LOT4

References

[Accessed December 4, 2016].

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