MultipleMyelomaHub

General MM

Regulatory Updates

Emily Smith | Sep 10, 2019

The beginning of September 2019 has already seen two orphan drug designations (ODD) for drugs designed to treat multiple myeloma (MM).

CT053

On the 2nd September, 2019, the United States (US) Food & Drug Administration (FDA) announced it was granting ODD to CT053. CT053 is a fully human, investigational, anti-B-cell maturation antigen (BCMA) chimeric antigen receptor (CAR) T-cell product for the treatment of relapsed/refractory MM (RRMM). The FDA had previously awarded Investigational New Drug (IND) clearance to CT053 in June 2019.[1,2]

This award is based on results from a phase I clinical trial conducted in China. The results of this trial were presented at the 5th Annual Immunotherapy In Myeloma Scientific Workshop in Denver, US. As of the 28th February 2019, 24 patients with R/R MM were treated, with 70.8% of patients achieving a complete response (CR) and 87.5% achieving an overall response. No events of grade ≥3 cytokine release syndrome (CRS) were reported.[1,2]

Isatuximab

The Korean Ministry of Food and Drug Safety has granted ODD to isatuximab (Isa) for use in combination with pomalidomide (P) and dexamethasone (d, Isa-Pd) in patients who have previously received two or more treatments for R/R MM. This approval is based on the results of the ICARIA-MM trial, presented at the American Society of Clinical Oncology Meeting (ASCO) in June, 2019.[3]

In the ICARIA-MM phase III study, patients with R/R MM were randomized to either Isa-Pd (n= 154) or Pd alone (n= 153). At a median follow-up of 11.6 months the progression-free survival, as determined by independent review committee assessment, was 11.53 months for patients receiving Isa-Pd compared to 6.47 months for those receiving Pd (P = 0.001). The hazard ratio was 0.596, indicating Isa-Pd significantly improves PFS. Median overall survival was not reached in either arm. The triplet also had a manageable safety profile.[4]

References

1. European Pharmaceutical Review (EPR): Multiple myeloma treatment receives Orphan Drug Designation. https://www.europeanpharmaceuticalreview.com/news/98371/multiple-myeloma-treatment-receives-orphan-drug-designation/ [Accessed 2019 Sep 03]
2. CARSGEN therapeutics. CARsgen Therapeutics Receives No Objection to Clinical Trial Application from Health Canada for CT053-MM-02, BCMA-CAR-T Cell Therapy for rrMM. http://www.carsgen.com/media/news?id=cascRxXK7e [Accessed 2019 Sep 03]
3. Korea Biomedical Review. Multiple myeloma treatment isatuximab named orphan drug. http://www.koreabiomed.com/news/articleView.html?idxno=6378 [Accessed 2019 Sep 03]

4. Richardson P.G. et al. A phase III randomized, open label, multicenter study comparing isatuximab, pomalidomide, and low-dose dexamethasone versus pomalidomide and low-dose dexamethasone in patients with relapsed/refractory multiple myeloma (RRMM). Abstract #8004. American Society of Clinical Oncology meeting, Chicago, US. 2019 Jun 02