General AML

PRGN-3006 is granted Orphan Drug Designation for relapsed or refractory acute myeloid leukemia by the U.S. FDA

Jan 09, 2020

The U.S. Food & Drug Administration (FDA) has granted Orphan Drug Designation to PRGN-3006 for the treatment of patients with relapsed or refractory (RR) acute myeloid leukemia (AML). PRGN-3006 is an investigational therapy which uses a proprietary UltraCAR-T™ therapeutic platform to reduce the time to manufacture chimeric antigen receptor (CAR) T cells, enabling the administration of CAR T cells one day after non-viral gene transfer. PRGN-3006 UltraCAR-T is a multigenic CAR T-cell product, co-expressing a CD33 CAR and membrane-bound interleukin-15 (mbIL15), together with a ‘kill switch’. CD33 CAR precisely targets malignant cells expressing CD33, while expression of mbIL15 allows the elimination of ex vivo expansion of T cells prior to cell administration. The kill switch provides a mechanism for selective depletion of PRGN-3006 post-administration in order to improve therapeutic control. There is an ongoing trial of PRGN-3006 (NCT03927261), in patients with RR AML and high-risk myelodysplastic syndromes, which has completed enrolment of the first cohort and initial data is expected later this year. Preclinical data on PRGN-3006 were presented at the 61st American Society for Hematology Annual Meeting & Exposition, in Orlando, US.

Orphan drug status is granted to medicines aimed at treating, detecting or preventing rare diseases or disorders affecting < 200,000 people in the US. This status grants certain incentives such as a seven-year exclusivity marketing period, applications for funding for phase I and II clinical trials, reduced market application filing fees and rapid regulatory processing later on.

References

1. Healio. FDA grants orphan drug designation to PRGN-3006 for AML. https://www.healio.com/hematology-oncology/leukemia/news/online/%7B3c7aeba4-ac5c-4c00-95f0-ee4148a44f31%7D/fda-grants-orphan-drug-designation-to-prgn-3006-for-aml Published, January 6 2020, [Accessed 2020 Jan 08]


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