



---

# CAR T therapy receives orphan drug designation from the FDA

 Iqra Farooq | Jul 26, 2019

The FDA has granted an investigational chimeric antigen receptor (CAR) T-cell therapy, called MB-102, orphan drug designation. This means that the drug now has 'orphan status'; those intended for effective and safe treatment of rare diseases affecting fewer than 200,000 people in the US, or affecting more than this, but is not expected to recover costs of developing and manufacturing the drug.

This CAR T therapy targets the CD123 antigen and was previously granted orphan drug designation in December 2018 for the treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN). CD123 is widely expressed in the bone marrow cells of patients with myelodysplastic syndromes and hematological malignancies.

It is expected that a phase I/II multicenter trial will begin in the next few months, with a phase I first-in-human trial (NCT02159495) of MB-102 identifying that low doses of the experimental drug have resulted in complete responses in patients with AML. This dose-escalation trial currently being conducted will determine the safety and efficacy of the drug in patients with relapsed or refractory AML or BPDCN.

So far, no patients have experienced higher than grade III cytokine release syndrome or neurotoxicity, with both being reversible and well-managed.

The orphan drug designation allows the manufacturers to qualify for various incentives, such as credits for clinical trials, and if approved, seven years of market exclusivity.

## Reference

Mustang Bio Receives Orphan Drug Designation for MB-102 (CD123 CAR T) for the Treatment of Acute Myeloid Leukemia, MustangBio press release, <http://ir.mustangbio.com/file/Index?KeyFile=398860454> [accessed 26.07.2019]

---

© 2019 Scientific Education Support Ltd. This PDF is provided for personal use only. For wider or commercial use, please seek permission from [secretariat@scientificeducationsupport.com](mailto:secretariat@scientificeducationsupport.com) and attribute the source as: <<https://amlglobalportal.com/medical-information/car-t-odd-fda>>