



To: BLA STN 125846/0

From: Jared Greenleaf, CMC/Facility Reviewer, OCBQ/DMPQ/MRB1

Through: Kathleen Jones, Ph.D., Branch Chief OCBQ/DMPQ/MRB1

CC: Rabia Ballica, Ph.D., Lead Consumer Safety Officer, OCBQ/DMPQ/MRB1
Antonia Panthiruvellil, Regulatory Project Manager, OCBQ/DMPQ/MRB1
Iryna Zubkova, Regulatory Project Manager, OCBQ/DMPQ/ARB

Applicant: Fondazione Telethon ETS, U.S. License Number 2378

Product: WASKYRA – etuvetidigene autotemcel

Indication: Treatment of pediatric patients aged 6 months and older and adults with Wiskott-Aldrich Syndrome (WAS) who have a mutation in the WAS gene for whom hematopoietic stem cell transplantation (HSCT) is appropriate and no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available.

Due Date: December 10, 2025

RECOMMENDATION

Based on the information submitted to BLA 125846/0 and amendments, approval is recommended. For Post-Marketing Commitments (PMCs) and inspectional follow-up recommendations, see the primary DMPQ committee review memo (signed November 19, 2025).

ADDENDUM TO DMPQ REVIEW MEMO:

The applicant confirmed (amendment 125846/0.31) a change to the indication. Previously, the indication was for the “treatment of Wiskott-Aldrich Syndrome (WAS) in patients aged 6 months and older.” The indication is now for the “treatment of pediatric patients aged 6 months and older and adults with Wiskott-Aldrich Syndrome (WAS) who have a mutation in the WAS gene for whom hematopoietic stem cell transplantation (HSCT) is appropriate and no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available.”