

**Department of Health and Human Services
Food and Drug Administration (FDA)
Center for Biologics Evaluation and Research (CBER)
Office of Biostatistics and Pharmacovigilance (OBPV)**

RWE EPIDEMIOLOGY CONSULT MEMORANDUM

Date: December 8th, 2025

From: Jane Namangolwa Mutanga MD, MPH, PhD
Weidong Gu MD, PhD
ARWEB/DABRA/OBPV/CBER/FDA

To: BLA 125846/0
Cecilia (CC) Crowley RPM-OMRR/OTP/CBER/FDA

Through: Yun Lu, PhD
Deputy Division Director, DABRA/OBPV/CBER/FDA

Sponsor: Fondazione Telethon ETS (Rome Italy)/US Agent eCTD LLC

Product: WASKYRA - etuvetidigene autotemcel

Application Number: BLA 125846

Indication: WASKYRA is an autologous hematopoietic stem cell-based gene therapy indicated 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.

Due Date: December 10, 2025

ADDENDUM TO RWE REVIEW MEMO:

The applicant confirmed (amendment 125846/0.31) a change to the indication. Previously, the indication was for "Treatment of patients aged 6 months and older with severe WAS who have a mutation in the WAS gene and for whom no suitable HLA-matched related HSCT donor is available." The indication is now "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."