4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2026-N-9547]

Issuance of Priority Review Voucher; Rare Pediatric Disease Product; GENGLYCOS (parisglasgene brecaparvovec-opnr)

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the issuance of a priority review voucher to the sponsor of a rare pediatric disease product application. The Federal Food, Drug, and Cosmetic Act (FD&C Act) authorizes FDA to award priority review vouchers to sponsors of approved rare pediatric disease product applications that meet certain criteria. FDA is required to publish notice of the award of the priority review voucher. FDA has determined that GENGLYCOS (parisglasgene brecaparvovec-opnr), approved on August 19, 2026, manufactured by Ultragenyx Pharmaceutical, Inc., meets the criteria for a priority review voucher.

FOR FURTHER INFORMATION CONTACT: Myrna Hanna, Center for Biologics Evaluation and Research, Food and Drug Administration, industry.biologics@fda.hhs.gov, 240-402-7911.

SUPPLEMENTARY INFORMATION: FDA is announcing the issuance of a priority review voucher to the sponsor of an approved rare pediatric disease product application. Under section 529 of the FD&C Act (21 U.S.C. 360ff), FDA will award priority review vouchers to sponsors of approved rare pediatric disease product applications that meet certain criteria. FDA has determined that GENGLYCOS (parisglasgene brecaparvovec-opnr), manufactured by Ultragenyx Pharmaceutical, Inc., meets the criteria for a priority review voucher. GENGLYCOS (parisglasgene brecaparvovec-opnr) is an adeno-associated virus (AAV) vector-

based gene therapy indicated to reduce daily cornstarch intake as an adjunct to nutritional management in adult and pediatric patients 8 years age and older with Glycogen Storage Disease Type la (GSDla).

For further information about the Rare Pediatric Disease Priority Review Voucher Program and for a link to the full text of section 529 of the FD&C Act, go to https://www.fda.gov/industry/developing-products-rare-diseases-conditions/rare-pediatric-disease-rpd-designation-and-voucher-programs. For further information about GENGLYCOS (parisglasgene brecaparvovec-opnr), go to the Center for Biologics Evaluation and Research's Approved Cellular and Gene Therapy Products website at https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products.

Grace R. Graham,

Deputy Commissioner for Policy, Legislation, and International Affairs.

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