



UCSF Benioff Children's Hospitals Become the First US Qualified Treatment Center Contracted to Administer Individualized Gene Therapy WASKYRA®

Descrizione

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WASKYRA® , the first gene therapy approved in the US to treat Wiskott-Aldrich Syndrome, is now commercially available, with more centers expected to be added in the coming months.

CAMBRIDGE, Mass. and ROME, Sept. 28, 2026 /PRNewswire/ - Fondazione Telethon (FT), an Italian biomedical charity with more than 35 years of advancing research on rare and complex genetic diseases, and Orphan Therapies (OT), the commercial subsidiary of Orphan Therapeutics Accelerator, a non-profit biotech committed to advancing and expanding access to promising therapies for ultra-rare conditions, today announced the designation of UCSF Benioff Children's Hospitals as the first US Qualified Treatment Center (QTC) designated to administer WASKYRA® (etuvetidigene autotemcel) to eligible pediatric patients aged six months and older and adults with Wiskott-Aldrich Syndrome (WAS) who have a mutation in the WAS gene and for whom hematopoietic stem cell transplantation (HSCT) is appropriate and no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available.

Orphan Therapies is the exclusive U.S. commercialization partner for WASKYRA® , which was developed and submitted to the U.S. Food and Drug Administration (FDA) for regulatory approval by Fondazione Telethon. WASKYRA was approved by the FDA in December 2025 and is the first gene therapy available to treat Wiskott-Aldrich Syndrome, and also the first advanced therapy to be commercialized entirely through a non-profit collaboration.

Wiskott-Aldrich Syndrome (WAS) is an ultra-rare, life-threatening immunodeficiency caused by mutations in the WAS gene. The disease is characterized by thrombocytopenia and bleeding complications, recurrent infections, eczema, immune dysregulation, and an increased risk of autoimmune disease and malignancy.

UCSF Benioff Children's Hospitals are among the nation's foremost pediatric hospitals and have extensive expertise in caring for patients with rare genetic and immunologic disorders. The activation of

UCSF Health as a QTC establishes the commercial availability of WASKYRA® to eligible patients in the U.S. and represents the first step toward building a U.S. clinical network of highly specialized clinical centers to support patient access.

“This milestone and partnership with Orphan Therapies reflect the ongoing and successful evolution of Fondazione Telethon’s model, which was initially designed to translate scientific research into tangible therapeutic opportunities for patients,” said Ilaria Villa, CEO of Fondazione Telethon. “After decades of groundbreaking research, we recognized the need for novel, collaborative solutions to market-based challenges that keep urgently needed treatments from reaching people affected by rare genetic diseases.”

“Our purpose is to provide reliable and sustainable commercial access to vital treatments for very rare conditions,” said Beth White, Chief Commercial Officer of Orphan Therapies. “To do so, we find new ways to work within existing systems to deliver for patients. Our partnership with FT on WASKYRA® is an initial proof point for an approach that we intend to apply across a range of therapies for ultra-rare conditions.”

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