

Pharmacy Focus:

Waskyra® for Wiskott-Aldrich Syndrome



Key Takeaways



Wiskott-Aldrich Syndrome (WAS) is a rare, X-linked genetic disorder that primarily affects males.



Symptoms of WAS include bleeding, eczema, and recurrent infections.



Waskyra® is the first gene therapy approved by the FDA for WAS.



The launch timing and pricing for Waskyra® are currently unknown, but this treatment is expected to have costs similar to other gene therapies on the market.

Disease Overview¹

Description: WAS is a rare X-linked genetic disorder involving immunodeficiency, bleeding episodes, eczema, recurrent infections, and increased susceptibility to autoimmune disorders.

Subtypes & Severity:

- WAS is caused by a mutation in the *WAS* gene, which plays a role in cell signaling and immunity.
- WAS is generally divided into the following subtypes:
 - Classic WAS (the most severe form), which may lead to autoimmune disorders and lymphoma
 - Milder forms that include X-linked thrombocytopenia and X-linked neutropenia, among others

Occurrence: WAS is estimated to occur in 1 of every 100,000 live births and is almost exclusively seen in males.



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Treatment²⁻³

Allogenic hematopoietic stem cell transplantation (ALLO HSCT) is the most effective treatment currently available. However, patients may not have a donor available. For these patients, Waskyra® offers a form of stem cell transplantation by which the patient's own cells are genetically modified in a lab and then infused back into the patient.

Waskyra®	
Generic Name	etuvetidigene autotemcel
Manufacturer	Fondazione Telethon ETS
Approval	December 6, 2025
Use	In patients six months and older and adults with WAS who have a mutation in the <i>WAS</i> gene and for whom ALLO SCT can be done, but no matched related stem cell donor is available.
How It Works	Waskyra is an autologous HSCT. A patient's own (autologous) stem cells are collected and then genetically modified in a lab to carry the <i>WAS</i> gene. Then the cells are administered back to the patient. The patient's own body is then able to make the missing protein for their blood cells.
Procedure Location	Hospital (inpatient)
Timeframe	One-time infusion
Efficacy	Waskyra was studied in 18 total patients with WAS. The major efficacy outcome was that the rate of severe infections during a period after Waskyra was given decreased from 2 infections (in the 12-month pre-treatment period) to 0.2 infections.
Estimated Cost Per Month	Unknown
Associated Benefit	Medical

HMConnects™

As part of the HMConnects cost containment program, HM Insurance Group (HM) works to support cost management opportunities around the use of gene and cell therapies and other high-cost pharmaceutical treatment options that can impact our clients' bottom line. The Pharmacy Operations (RxOps) team watches the market – and our book of business – to anticipate how current and future advancements will impact financial risk levels for HM's client base. Standard practices include reviewing, auditing and collaborating on the content of current policies, monitoring trends and implementing appropriate cost savings techniques. Additional practices include the prevention of stockpiling, working to ensure prescriptions are filled via in-network pharmacies and assessing to determine if patients are properly dosed based on weight and lab values when appropriate. All of these services are provided to HM's clients at no additional cost to them.

Pharmacy Focus provides valuable information about pharmaceutical industry developments and their associated costs that can impact the growing claims trend in the insurance market. Be aware of influences and gain insight into approaches that may help to contain costs. Please share topic suggestions or feedback with HMPHarmacyServices@hmig.com.



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References: ¹Malik MA, Masab M. Wiskott-Aldrich Syndrome. [Updated 2023 Jun 26]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2025 Jan-. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK539838/>. ²Waskyra [package insert]. Rome, Italy: Fondazione Telethon ETS; December 2025. ³Waskyra: Gene Therapy for WAS. Wiskott-Aldrich Foundation, Inc. Available at: <https://www.wiskott.org/About-WAS/treatment-of-was/waskyra>. Accessed December 12, 2025.

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