

Chiesi Global Rare Diseases Receives FDA Approval for FILSUEZ® (birch triterpenes) topical gel for the Treatment of Epidermolysis Bullosa Date: 19/12/2023

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FILSUEZ topical gel is indicated for the treatment of partial thickness wounds in patients 6 months and older with Junctional and Dystrophic Epidermolysis Bullosa (EB)

FILSUEZ was well tolerated and met the primary endpoint with statistical significance, with 41.3% of patients achieving first complete target wound closure within 45 days, compared with 28.9% in the control gel arm, in the Phase 3 clinical trial[\[1\]](#)

BOSTON, December 19, 2023 - Chiesi Global Rare Diseases, a business unit of the Chiesi Group established to deliver innovative therapies and solutions for people affected by rare diseases, announced today that the U.S. Food and Drug Administration (FDA) approved FILSUEZ® (birch triterpenes) topical gel for the treatment of partial thickness wounds in patients 6 months and older with Junctional Epidermolysis Bullosa (JEB) and Dystrophic Epidermolysis Bullosa (DEB). FILSUEZ is the first approved treatment for wounds associated with JEB, a rare, moderate-to-severe form of EB with blisters beginning in infancy. FILSUEZ joined the Chiesi portfolio as part of the agreement reached during the acquisition of Amryt Pharma in January of this year.

EB is a debilitating inherited skin disease that causes a person's skin to be so fragile it can be injured just from touch. This rare, chronic, and distressing disorder affects infants, children and adults and is intensely painful; recurrent blistering and chronic wounds can result in intolerable pain with limited mobility. Living with EB entails daily challenges to navigate, including slow-healing wounds at risk of infection and painful dressing changes.

FILSUEZ is administered at home, allowing for integration into existing treatment routines. FILSUEZ is applied topically to the wound at each dressing change.

*"At Chiesi Global Rare Diseases we are driven by a need to alleviate the burdens faced in the rare disease community by providing innovative therapies and solutions that address debilitating unmet needs." **Giacomo Chiesi, Head of Chiesi Global Rare Diseases, said.** "We are grateful for the support of those living with EB and their dedicated caregivers which allowed us to reach this landmark FDA approval and proudly provide FILSUEZ as a solution for wound care management."*

Brett Kopelan, Executive Director, debra of America, added, *“The FDA’s decision to approve FILSUVEZ provides those living with EB a well-tolerated and effective treatment option for the most prominent and difficult symptom of EB, open wounds that may not heal.”* Kopelan also commented that, *“today marks an important milestone for those living with junctional EB, as FILSUVEZ is the first FDA approved treatment for this variant of the disease. I want to thank Chiesi for their years of close collaboration with debra of America and their dedication and commitment to bringing a treatment option forward for those with dystrophic and junctional EB. I also want to thank the patients who participated in the clinical trials to bring this therapeutic option to fruition.”*

FILSUVEZ was previously approved in June 2022 by the European Commission for the treatment of partial thickness wounds in adults and children, ages 6 months and older with both JEB and DEB.

About Epidermolysis Bullosa

Epidermolysis Bullosa (EB) is a rare and devastating group of hereditary disorders of the skin, mucous membranes, and internal epithelial linings characterized by extreme skin fragility and blister development. Patients with severe forms of EB suffer from severe, chronic blistering, ulceration, and scarring of the skin, mutilating scarring of the hands and feet, joint contractures, strictures of the esophagus and mucous membranes, a high risk of developing aggressive squamous cell carcinomas, infections, and risk of premature death.

About FILSUVEZ topical gel

FILSUVEZ® (birch triterpenes) topical gel, is a medicine that is used in adults and children aged 6 months or older with epidermolysis bullosa (EB). FILSUVEZ contains a dry extract from two species of birch bark consisting of naturally occurring substances known as triterpenes, including betulin, betulinic acid, erythrodiol, lupeol and oleanolic acid. FILSUVEZ is available as a gel that should be applied to the wound surface at a thickness of approximately 1 mm and covered by a wound dressing. The medicine can also be applied directly to the wound dressing.

About EASE Trial

The EASE trial ([NCT03068780](https://clinicaltrials.gov/ct2/show/study/NCT03068780)) is the largest ever global Phase 3 trial conducted in patients with EB, performed across 58 sites in 28 countries. It comprises a 3-month double-blind randomized controlled phase followed by a 24-month open-label, single-arm phase. Patients with EB target wounds of between 10 and 50cm² in size that were present for > 21 days and < 9 months were randomized in the double-blind phase to study treatment in a 1:1 ratio and wound dressings applied according to the standard of care. 223 patients were enrolled in the trial including 156 pediatric patients. Of those who completed the double-blind phase, 100% entered the open-label safety follow-up phase. The primary endpoint of the trial was to compare the efficacy of FILSUVEZ topical gel versus control gel according to the proportion of patients with complete closure of the target wound within 45 days of treatment. The primary endpoint was achieved with statistical significance (p-value = 0.013). While the key secondary endpoints did not achieve statistical significance, a number of favorable differences were observed.

About Chiesi Global Rare Diseases

Chiesi Global Rare Diseases is a business unit of the Chiesi Group established to deliver innovative therapies and solutions for people affected by rare diseases. As a family business, Chiesi Group strives to create a world where it is common to have a therapy for all diseases and acts as a force for good, for society and the planet. The goal of the Global Rare Diseases unit is to ensure equal access so as many people as possible can experience their most fulfilling life. The unit collaborates with the rare disease community around the globe to bring voice to underserved people in the health care system.

About Chiesi Group

Chiesi is an international, research-focused biopharmaceuticals group that develops and markets innovative therapeutic solutions in respiratory health, rare diseases, and specialty care. The company's mission is to improve people's quality of life and act responsibly towards both the community and the environment.

By changing its legal status to a Benefit Corporation in Italy, the US, and France, Chiesi's commitment to create shared value for society as a whole is legally binding and central to company-wide decision-making. As a certified B Corp since 2019, we're part of a global community of businesses that meet high standards of social and environmental impact. The company aims to reach Net-Zero greenhouse gases (GHG) emissions by 2035.

With over 85 years of experience, Chiesi is headquartered in Parma (Italy), operates in 31 countries, and counts more than 6,500 employees. The Group's research and development centre in Parma works alongside 6 other important R&D hubs in France, the US, Canada, China, the UK, and Sweden.

About debra

debra of America (debra) is a nonprofit organization dedicated to enhancing the lives of individuals and families impacted by Epidermolysis Bullosa (EB), a rare genetic disorder. The organization integrates direct services, advocacy, education and research to foster meaningful change for EB patients. Epidermolysis Bullosa is characterized by fragile skin prone to painful blisters from minor friction. debra provides vital resources, builds community and drives research to alleviate the challenges associated with EB, supporting patients and their loved ones. With a longstanding commitment to EB awareness and study, debra is a pillar of support. By uniting caregivers, advocates and supporters, the organization envisions a future where the burdens of EB are lifted, empowering those affected to lead lives of greater comfort. For more information about debra, visit www.debra.org.