- Press Release -

LFB announces the extension of the indications of WILFACTIN® / WILLFACT®[1] to the entire paediatric population in the European Union

Les Ulis (France), June 6, 2024. LFB announced today the extension of the indications of WILFACTIN® / WILLFACT® to the entire paediatric population of the European Union and United Kingdom. This human coagulation Von Willebrand factor is indicated for the prevention and treatment of haemorrhages or for surgical bleeding in patients with Von Willebrand disease when desmopressin treatment alone is ineffective or contraindicated.

Dr Patrick Delavault, Executive Vice President in charge of scientific, medical and regulatory affairs at LFB, underlines: *"Thanks to this extension, our Von Willebrand factor, which contains low residual levels of human factor VIII (≤10 IU/100 IU VWF:RCo), can be used to treat all age groups of patients suffering from Von Willebrand disease. Our teams can be proud of the work done to serve patients affected by this rare coagulation disorder."*

A treatment for Von Willebrand disease in all age groups

Von Willebrand disease is a bleeding pathology caused by a genetic defect affecting the concentration, structure or function of Von Willebrand factor (VWF), an essential protein in the mechanisms of primary haemostasis (interaction with platelets) and coagulation (transport and protection of factor VIII). There are several different types of Von Willebrand factor deficiency: partial quantitative (type 1), which is the most common, or complete (type 3) and qualitative (type 2). The disease affects both men and women, its clinical expression is highly variable.

This extension of the product indications to the entire paediatric population without age restrictions was approved on 11 January 2024 within the European Union, in a procedure having involved Austria, Belgium, the Czech Republic, Denmark, Finland, France, Germany, Greece, Hungary, Italy, Lithuania, Luxembourg, the Netherlands, Norway, Poland, Slovakia, Spain, Sweden and the United Kingdom.

Clinical studies in the paediatric population

The efficacy and safety of LFB's Von Willebrand factor in the paediatric population (< 18 years) was evaluated in four prospective open-label phase 2/3 studies (42-73-305, 32-73-803, 32-73- 805, 42-73-406) and in one prospective real-life observational study (42-99-302). One of these studies[2], published in January 2023, evaluated the efficacy and safety of WILFACTIN® in children under 6 years of age.
In all, these studies evaluated the treatment in a total of 56 paediatric patients: 23 children were under 6 years of age, 21 were between the ages of 6 and 11 years, and 12 were between 12 and 17 years. Most of the patients had a severe form of Von Willebrand disease, including 25 patients with type 3. They received at least one injection of Von Willebrand factor for the treatment of bleeding episodes,

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[1] WILFACTIN®: LFB's Von Willebrand factor available on the market in France, Belgium, Finland, Latvia, Italy, Lithuania and the Netherlands.
WILLFACT®: LFB's Von Willebrand factor marketed in Germany, Austria, the Czech Republic, Denmark, Spain, Hungary, Norway, Poland, Slovakia, Sweden and the United Kingdom.

[2] Gouider E, Klukowska A, Maes P, Platokouki H, Pujol S, Henriet C, Bridey F, Goudemand J. Efficacy and safety of von Willebrand factor concentrate almost devoid of factor VIII (Wilfactin®) in paediatric patients under 6 years of age with severe von Willebrand disease. Blood Transfus. 2023 Jan;21(1):83-92. doi: 10.2450/2022.0329-21. Epub 2022 Apr 19. PMID: 35543677; PMCID: PMC9918383.

to prevent bleeding during surgical procedures, or for the long-term prevention of recurrent bleeding episodes.

The treatment was evaluated in 122 bleeding episodes including 21 severe haemorrhages and 29 surgeries. The haemostatic efficacy of the product was judged by the physician to be “excellent” or “good” in 89.3% of the bleeding episodes and in 100% of the surgeries.

Seventeen patients received long-term prophylaxis administrations at a frequency of approximately two per week, at a median dose between 42 IU/kg (≥ 12 years group) and 52 IU/kg (other age groups).

No serious adverse effects, thromboembolic events or appearance of inhibitors were observed during these clinical studies.
The data confirm the efficacy and safety of this plasma-derived Von Willebrand factor concentrate in paediatric patients with Von Willebrand disease. The dose and duration of treatment must be adapted to the patient’s clinical status and plasma Von Willebrand factor and factor VIII levels during treatment.

About LFB
LFB is a bio-pharmaceutical group that develops, manufactures and markets plasma-derived medicinal products and recombinant proteins for the treatment of patients with serious and often rare diseases. LFB was founded in 1994 in France and is among the leading European suppliers of plasma-derived medicinal products to healthcare professionals. Its vision is to provide patients with new treatment options in three major therapeutic areas: immunology, haemostasis, and intensive care.
LFB currently markets 15 medicinal products in more than 30 countries.
For more information about LFB, go to www.groupe-lfb.com*.*

LFB contact person:
Didier Véron – Executive Vice President of Corporate Affairs
Phone: +33 (0)1 69 82 72 97 or +33 (0)6 08 56 76 54
verondidier@lfb.fr