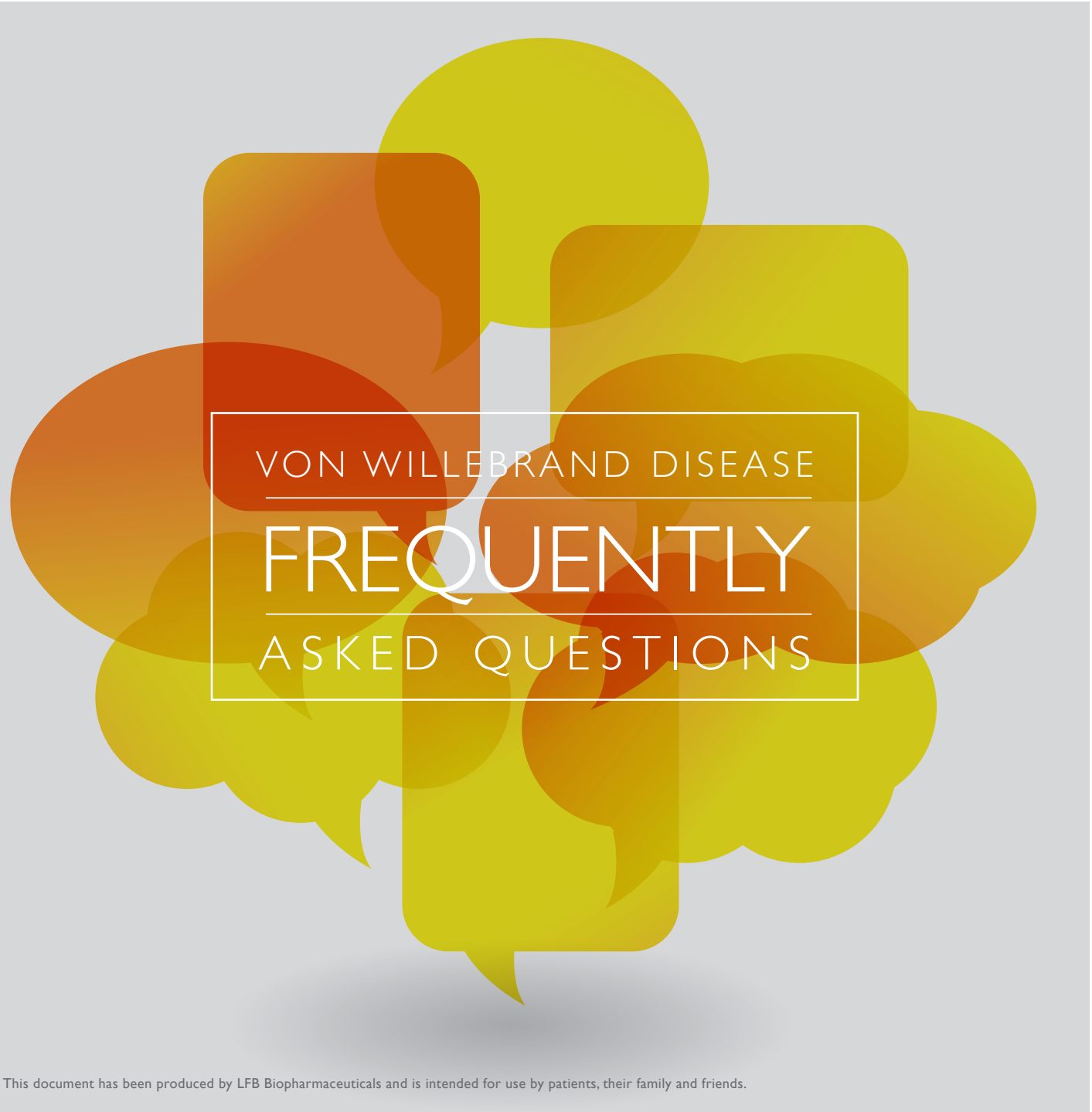

This document has been produced by LFB Biopharmaceuticals and is intended for use by patients, their family and friends.

Contents

What is von Willebrand disease (VWD)?

Von Willebrand disease (or VWD) is an inherited bleeding disorder. It is the most common inherited bleeding disorder, affecting up to 1 in every 100 people. VWD is caused by missing or defective von Willebrand factor (VWF), a clotting protein in the blood. It was named after Erik von Willebrand, the Finnish doctor who first described the disease in 1926.

Although VWD is a bleeding disorder, it is different to haemophilia – unlike haemophilia it affects both women and men, it is more common, and it has different bleeding symptoms.

What is von Willebrand factor (VWF)?

VWF is one of the clotting proteins in the blood. It acts like a glue which allows platelets (a type of blood cell) to stick together and plug up damaged blood vessels. It also carries and protects another clotting factor called FVIII (factor 8).

What are the symptoms of VWD?

The symptoms of VWD vary from person to person. They can include:

- Frequent nosebleeds
- Bleeding from the gums
- In women, heavy periods and bleeding after childbirth
- Bruising easily
- Prolonged bleeding from cuts
- Bleeding from the lining of the gut
- Excessive bleeding during and after surgery or a tooth extraction

People with VWD should always let their doctor, dentist or surgeon know, even if it is a mild form.

With treatment, people with more severe symptoms can lead normal and active lives.

What causes VWD?

VWD is a genetic disorder caused by a fault in the gene responsible for the production of VWF. This fault can be passed on to a child by one or both of their parents. People who have inherited one copy of the faulty gene may have mild symptoms; whereas if they have inherited two copies of the faulty gene symptoms may be more severe (type 3; see next page).

What are the different types of VWD?

There are 3 main types of VWD, depending on whether the VWF is missing, or not working properly.

Type 1: This is the most common type (around 8 in 10 people with VWD have type 1). People with type 1 VWD have a reduced level of VWF in their blood. The symptoms are usually mild which means that people with type 1 VWD don't usually bleed spontaneously, but can bleed if they have an operation, injure themselves, or have a tooth removed. In women with type 1 VWD, heavy periods (or menorrhagia), is usually the only bleeding symptom, although some women may have severe type 1 resulting in moderate to severe bleeding. Menorrhagia can also be a feature of other types of VWD.

Type 2: In type 2 the VWF does not work properly. There are several subtypes (including 2A, 2B, 2M and 2N) and bleeding is often more severe than type 1, but milder than type 3.

Type 3: Type 3 is rare (less than 1 in 10 people with VWD) and is the most serious. People with type 3 have little or no VWF and can have serious bleeding problems, including spontaneous joint and muscle bleeds or bleeding from the nose, gums and lining of the gut.

Acquired: Acquired VWD comes on suddenly, usually without the person having a personal or family history of bleeding. It can be caused by other medical problems or medicines. It is much rarer than the inherited forms of VWD.

How is it diagnosed?

The symptoms of VWD are usually mild and this means that it is sometimes difficult to diagnose.

The doctor will ask if there is any family history of bleeding disorders or bleeding symptoms, including heavy periods. A blood sample is taken, and tests carried out to diagnose the condition. These tests look at clotting time, the ability to form a clot, and the amount of VWF in the blood. After VWD is confirmed, a test to determine the exact type is performed.

The blood test may occasionally have to be repeated because levels of VWF can vary over time. For example, VWF can rise due to stress, exercise, the use of oral contraceptives, pregnancy and hyperthyroidism.

What are the treatments?

Treatment for VWD depends on the diagnosis and the severity. There are three main types of treatment to either prevent or treat bleeding. The doctor will explain which medication is most suitable, and when it should be taken.

Should sport be avoided?

Most people with VWD live normal and active lives and take part in most sports and activities.

In more severe cases, contact sports may need to be avoided. Protective equipment should also be worn for activities where there is a risk of bleeding or bruising.

Where can I find out more?

The following websites may be useful resources. You should also speak to your doctor or nurse about support in your local area.

www.haemophilia.org.uk

www.livingwithvwd.org

www.haemophilianetwork.org.uk

www.nhs.uk/conditions/von-willebrand-disease

www.hemophilia.org

Word index

Antifibrinolytic	a medicine that prevents the breakdown of blood clots
Defective	not working properly
Hormone	a chemical messenger produced in the body
Hyperthyroidism	an overactive thyroid (a gland in the neck), a condition where the thyroid produces too much thyroid hormone
Genes	they carry the information that determine the characteristics that are inherited from parents
Intravenous	given into a vein
Oral contraceptive	the 'pill'
Plasma	the fluid part of the blood. It contains a number of substances. It surrounds the blood cells and carries them around the body
Platelets	a type of blood cell that stick together to plug up damaged blood vessels
Protein	a type of material found in all living things. Proteins are made up of hundreds or thousands of small units called amino acids, which are attached to one another in long chains
Spontaneously	freely, without cause

Desmopressin or DDAVP

Desmopressin is the most common treatment and is a copy of a natural hormone that increases the levels of von Willebrand factor (and factor VIII). It works in most patients who are still able to produce some von Willebrand factor (type 1). It comes in two forms, either an injection or a nasal spray. A test dose is often given to check how well it works. Because it can cause fluid retention for up to 24 hours after it is taken, the amount of fluid drunk after treatment should be restricted.

VWF-containing concentrates

VWF is used to prevent or stop bleeding in more severe forms of VWD, including type 3, and where DDAVP doesn't work. It replaces the missing VWF and is purified from blood. Most types (but not all) also contain factor VIII. It is given directly into a vein.

Tranexamic acid

Tranexamic acid is an antifibrinolytic that prevents the breakdown of blood clots and may be used alongside other treatments or sometimes on its own to treat minor bleeds. It is available as tablets, a mouthwash or an injection. The doctor may recommend it to treat nose and mouth bleeds.

People with VWD should usually avoid taking aspirin and non-steroidal anti-inflammatory drugs (NSAIDs) such as ibuprofen, as these can further reduce the blood's ability to clot.

VWD and women

In girls and women with VWD who suffer from heavy periods, a doctor or gynaecologist can provide advice about ways to control heavy bleeding. This may include the oral contraceptive pill, tranexamic acid tablets, a desmopressin nasal spray (for type 1), or, in severe cases, VWF concentrate.

Although levels of VWF increase throughout pregnancy in most women with VWD, pregnant women with VWD are at increased risk of bleeding complications during labour and after delivery. Pregnant women are monitored carefully, and given treatment if needed to prevent bleeding. With precautions, most women with VWD can have successful pregnancies.

It is advised that pregnant women and their partners speak with their doctor or a genetic counsellor about the inheritance of VWD before becoming pregnant.

If the child is a baby boy, he should be tested for VWD before a choice is made regarding circumcision.

LFB Biopharmaceuticals Limited
PO Box 63587
London
N6 9BR

LFB Biomedicaments
3, Avenue des Tropiques
BP 40305
91958 Courtaboeuf Cedex
France

Date of preparation: March 2018
15/002 (1)

LFB
ETHICAL COMMITMENT