

# Chapter 6

## PROPHYLAXIS IN HEMOPHILIA

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### Prophylaxis: WHAT YOU NEED TO KNOW



Prophylaxis is the **Standard of Care** for all people with severe hemophilia everywhere around the world

#### What is Prophylaxis ?

**Prophylaxis** is regular and ongoing preventive therapy with a hemostatic agent.

This is in contrast to **episodic replacement therapy** (also known as on-demand therapy), which is treatment with clotting factor infusions only at the time of a bleed.

Prophylaxis is recommended over episodic therapy in all countries, but recognizes that **less intensive prophylaxis** may be used in resource constrained countries.

#### What are the goals of Prophylaxis ?

People with hemophilia should begin prophylaxis early in life, ideally by age 3 years, but it can be started at any age.

The **goal of prophylaxis** is to prevent bleeding in people with hemophilia, while allowing them to lead active lives and achieve quality of life comparable to non-hemophilic individuals.

#### What are the benefits of Prophylaxis over Episodic replacement therapy

##### Benefits of Prophylaxis

- ✓ People with hemophilia who are on regular prophylaxis have significantly fewer bleeds and a reduction in joint disease
- ✓ Prophylaxis can prevent or reduce the risk of intracranial hemorrhage
- ✓ The longer-term benefits include less chronic pain, reduced need for orthopedic surgery, hospitalization and emergency room visits
- ✓ All of this leads to a **better quality of life** and greater participation (i.e., regular attendance) in educational, recreational, and professional activities

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### WHAT ARE THE DIFFERENT TYPES OF PROPHYLAXIS TREATMENT?

Prophylaxis can be given using either clotting factor concentrate (CFC) replacement therapy (standard half-life (SHL) or extended half life (EHL)) or non-factor replacement therapy (e.g. emicizumab).

#### SHL replacement therapy

- SHL CFC are used as a prophylactic therapy for people with Hemophilia A and Hemophilia B
- SHL CFCs need to be infused frequently:
  - 3-4 times per week for Hemophilia A (FVIII)
  - 2-3 times per week for Hemophilia B (FIX)
- If prophylaxis is taken according to the schedule provided by your doctor, you will have fewer bleeds and less joint problems over time
- The frequent infusions may lead to difficulties with veins especially in young children

#### EHL replacement therapy

- EHL CFC are used as a prophylactic therapy for people with Hemophilia A and Hemophilia B
- EHL CFCs require less frequent infusions compared to SHL CFCs:
  - Can be as low as once every 1-2 weeks (for some EHL FIX products)
- Prophylaxis using EHL means you may have:
  - Less frequent infusions
  - Fewer trips to the clinic or hospital if you get infused there
  - Less problems with your veins

#### Non-factor replacement therapy

- Prophylaxis using non-factor replacement therapies now exists
- Emicizumab is the only non-factor replacement therapy approved at the time of this publication
- Emicizumab is used as a prophylactic treatment for Hemophilia A only
- It is administered subcutaneously (under the skin, not in the vein like clotting factor) once weekly or less
- Prophylaxis using non-factor products may lead to:
  - Less burdensome prophylaxis regime
  - Improved adherence
  - Increased participation in social and sports activities