Patient Decision Aid

McMaster University

Starting my child on prophylaxis (regular replacement therapy): when and what dose

This document aims to help parents of **new** patients with hemophilia to discuss prophylaxis options with their clinicians to make treatment decisions.

What is prophylaxis treatment?

- Prophylaxis is preventive treatment with regular clotting factor replacement to limit or prevent joint bleeding, progressive joint damage and other bleeds. The goal is to raise the factor VIII levels to within the range of mild or moderate hemophilia.

How is prophylaxis treatment given?

- Prophylaxis is given intraveneously, either into the veins with a needle OR with a venous access device (port-a-cath), which requires a small operation to put in place.

Who should consider this treatment?

- Children with severe hemophilia and tendencies to bleed, before joint damage has occured.

What is usually involved?

- The child is given injections by a family member at home. Sometimes, this can be done during regular visits to the hospital.

What are the two decisions and their options?

When to start prophylaxis treatment?

- **Early:** after the 1st joint bleed ***or*** during the 1st or 2nd year of age
- **Late:** after 2 or more joint bleeds ***or*** at 3 years of age or later

What *regimen* of prophylaxis treatment?

see reverse side for details of each

- **High** treatment dose
- **Intermediate** dose
- **Tailored** dose
- **Very low** dose

Explore the benefits and risks of each option

Use X's to show how much each benefit and risk **matters** to you: XX = a lot; X = somewhat; 0 = not at all

Start option	Benefits (reasons to choose this option)	how much it matters	Risks (reasons to avoid this option)	how much it matters
Early	Better joint health (i.e. keeping joints healthy and avoiding joint damage): *After 10 years, children on early prophylaxis had 1 joint bleed per year compared to 3 joint bleeds per year by children who started late* Patients with joint disease: Early / Late After 10 years: 0 / 15% After 17 years: 53% / 79%		Need for venous access device (because cannot infuse into peripheral veins) and complications: *Of 53 children with a venous access device, 16 (30%) had complications (e.g. infections) after 18 months*	
	Decreased anxiety about bleeding		Risk of missing treatment: *70% of families miss giving injections because of extra burden (time, injection schedule, uncooperative child)*	
Regimen option	**Benefits** (reasons to choose this option)	how much it matters	**Risks** (reasons to avoid this option)	how much it matters
High	Better joint health: *After 17 years, 36% of children on high dose had **no** bleeds compared to 7% on intermediate dose*		Need for venous access device and complications: *75% of children on high dose needed a venous access device compared to 29% on tailored dose*	
Intermediate	Less treatment burden		More joint bleeding	
Tailored	Better joint health: *After 5 years, 57% of children on tailored dose had **no** joint, brain or other serious bleeds compared to 44% on high dose*		Risk of bleeding before finding appropriate dose	
	Minimum needed treatment		Need for dose monitoring	
Very low	Less chance of getting an inhibitor (an antibody that destroys treatment before it can help): *4% of children on very low dose got an inhibitor compared to 47% on high dose*		Delay of other activities (e.g. vaccination, surgery, intense therapy for bleeding)	
			Risk of bleeding	
Note: the lower the dose, the lower the cost to society				

What do you want to achieve for your child?

Rank these objectives from 1 to 4, where 1 = matters most.

___ To have a fully active life with no worry about receiving intensive treatment

___ To have normal life activities and psychological development without risk of overprotection

___ To minimize his risk of getting an inhibitor

___ To have normal life activities (e.g. day care, school) with minimum limitations and minimum "medicalization"

What are the *regimen options* for prophylaxis treatment?

- **High** treatment dose: 1 or more vials of clotting factor replacement given 3 times per week
- **Intermediate** dose: 1 vial given 3 times per week
- **Tailored** dose: start with 1 or more vials once per week and increase to more times per week if bleeding occurs
- **Very low** dose: 1 small vial given once per week (starting before 1st joint bleed) for 1 year.