Hamilton Health Sciences

Hemophilia Case Study

GHEST- 2022

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Case Scenario:

Patient with severe Factor VII deficiency required emergency heart surgery.

What is the best treatment option for this patient?

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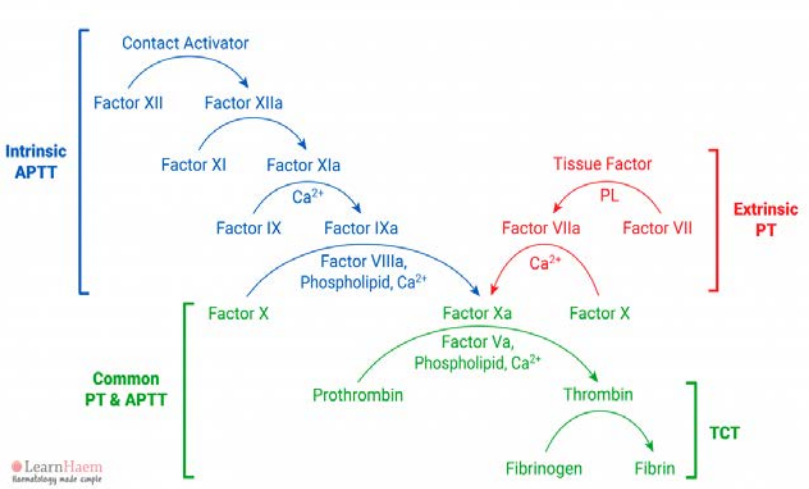
Objectives

- What is FVII deficiency?
- Treatment options
- What is SAP?
- Result of the case

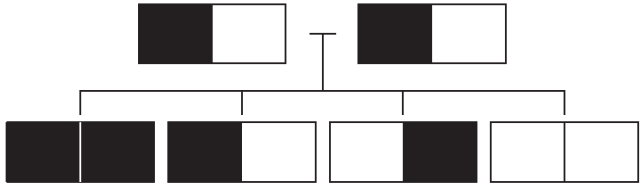
Factor VII Deficiency:

- Factor VII deficiency is a rare disorder causing reduced or deficient activity of the clotting factor VII
- Factor VII is produced in the liver and interacts with other clotting factors and substances to ultimately help form a clot and stop bleeding
- Patients with this disorder have difficulty stopping bleeding



Factor VII Deficiency:

- Factor VII deficiency can be either acquired or inherited.
- Unlike other hemophilia diseases, Inherited factor VII deficiency is an autosomal recessive disorder not a sex linked disorder
  - Can affect both males and females equally



- Patients can also acquire Factor VII deficiency through severe liver disease, sepsis or vitamin K deficiency

Factor VII Deficiency:

- The severity of the disease is dependant on the level of Factor VII in the blood stream
  - The less factor present the more severe the condition

- Our patient is going for emergency major surgery and needs something to help assist with potential bleed.

Case Study:

This patient had an inherited Factor VII deficiency.

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Case Study:

What treatment options are available for this patient?

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Treatment Options in Transfusion Medicine:

- Factor replacement therapy to temporarily raise the levels of Factor VII in the blood stream to stop the bleeding

- Treatments available in Transfusion Medicine:
  - Prothrombin Concentrate complex (PCC)
  - Fresh Frozen Plasma
  - Recombinant Factor VIIa

Treatment Options in Transfusion Medicine:

- Prothrombin Concentrate Complex (PCC)
  - Composed of all the vitamin K dependent factors (II, VII, IX, X)
  - Various quantities of each factor
  - Difficult to dose

  - Not the best choice for this patient

Treatment Options in Transfusion Medicine:

- Fresh Frozen Plasma
  - FVII has a short half life
  - Requires large volumes to make an impact
  - Not the best choice for this patient

Treatment Options in Transfusion Medicine:

- Recombinant Factor VIIa
  - Made in the laboratory using recombinant technology
  - Commonly known as Niastase

  - Good option for this patient but there are some associated risks

Case Study:

Doctor requested plasma derived Factor VII

Problem: Transfusion Medicine does not stock this product and only available through the Special Access Program (SAP) of Health Canada

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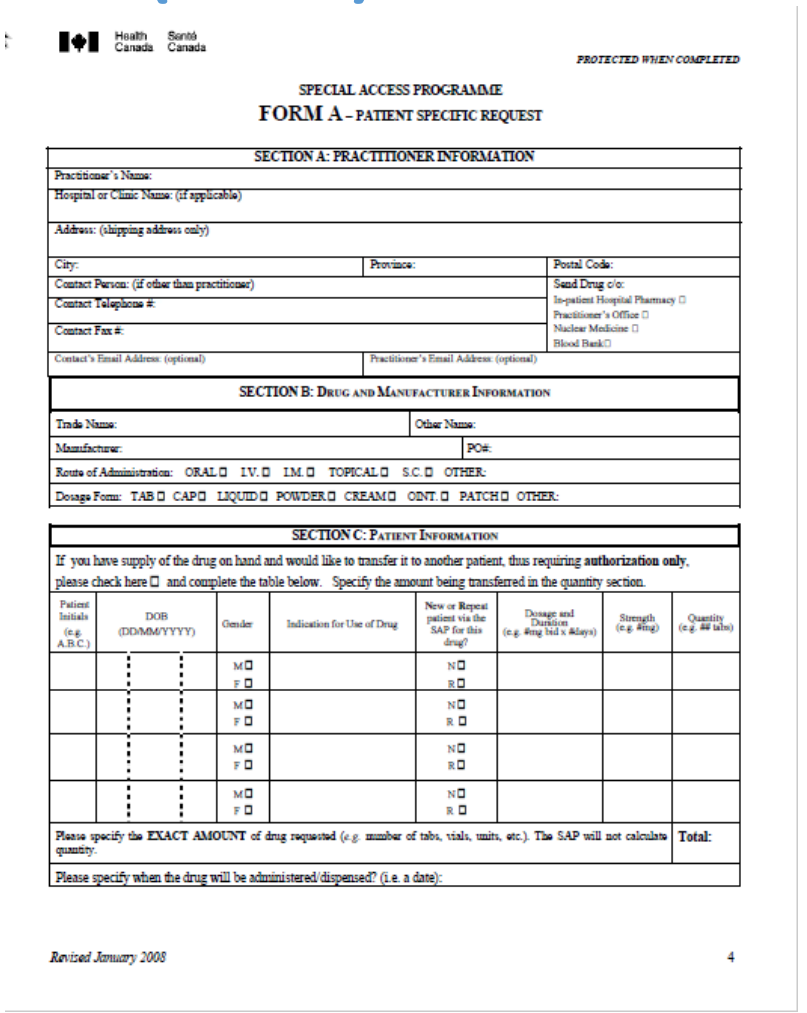
Special Access Program (SAP):

- SAP considers requests for access to non-marketed drugs when conventional treatment is not appropriate or failed
- SAP allows drugs that are not to be sold or distributed in Canada
- Physician needs to fax the two page form which can take up to 24h to process

Special Access Program (SAP):

- The form can be accessed on the Canadian Blood Services website
- https://www.blood.ca/sites/default/files/Health_Canada_Special_Access_Programme_Request_Form.pdf
- Transfusion Medicine can be of assistance
- But patient is going to surgery ASAP…



Health Canada / Santé Canada

PROTECTED WHEN COMPLETED

SPECIAL ACCESS PROGRAMME

FORM A – PATIENT SPECIFIC REQUEST

SECTION A: PRACTITIONER INFORMATION

Practitioner's Name:

Hospital or Clinic Name: (if applicable)

Address: (shipping address only)

City: | Province: | Postal Code:

Contact Person: (if other than practitioner)

Contact Telephone #:

Contact Fax #:

Send Drug c/o:
In-patient Hospital Pharmacy ☐
Practitioner's Office ☐
Nuclear Medicine ☐
Blood Bank ☐

Contact's Email Address: (optional) | Practitioner's Email Address: (optional)

SECTION B: DRUG AND MANUFACTURER INFORMATION

Trade Name: | Other Name:

Manufacturer: | PO#:

Route of Administration: ORAL ☐ I.V. ☐ I.M. ☐ TOPICAL ☐ S.C. ☐ OTHER:

Dosage Form: TAB ☐ CAP ☐ LIQUID ☐ POWDER ☐ CREAM ☐ OINT. ☐ PATCH ☐ OTHER:

SECTION C: PATIENT INFORMATION

If you have supply of the drug on hand and would like to transfer it to another patient, thus requiring **authorization only**, please check here ☐ and complete the table below. Specify the amount being transferred in the quantity section.

Patient Initials (e.g. A.B.C.)	DOB (DD/MM/YYYY)	Gender	Indication for Use of Drug	New or Repeat patient via the SAP for this drug?	Dosage and Duration (e.g. #mg bid x #days)	Strength (e.g. #mg)	Quantity (e.g. ## tabs)
		M ☐ F ☐		N ☐ R ☐			
		M ☐ F ☐		N ☐ R ☐			
		M ☐ F ☐		N ☐ R ☐			
		M ☐ F ☐		N ☐ R ☐			

Please specify the **EXACT AMOUNT** of drug requested (*e.g.* number of tabs, vials, units, etc.). The SAP will not calculate quantity. **Total:**

Please specify when the drug will be administered/dispensed? (i.e. a date):

Revised January 2008 4

Recombinant vs. Plasma Derived Factor VII:

Recombinant Factor VII	Plasma Derived Factor VII
Made in the laboratory using recombinant technology	Made from human plasma from blood donors
Half life 2-4 hours	Half life is ~ 6 hours
Stored at room temperature	Stored in the refrigerator
Available readily	SAP
Niastase	Factor VII, Takeda

Recombinant vs. Plasma Derived Factor VII:

- A few things to consider before choosing the best treatment option for this patient:

Time

&

Circumstances

Recombinant vs. Plasma Derived Factor VII:

Recombinant Factor VII	Plasma Derived Factor VII
Available readily	SAP

- **Time**:
  - Patient going for <u>emergency</u> surgery
    - Need product available now
      - Recombinant Factor VII
  - Major procedure patient could be in the OR for a long time
    - Product with the longest half life
      - Plasma Derived Factor VII

Recombinant Factor VII	Plasma Derived Factor VII
Half life 2-4 hours	Half life is ~ 6 hours

Recombinant vs. Plasma Derived Factor VII:

Recombinant Factor VII	Plasma Derived Factor VII
Available readily	SAP

- Circumstances:
  - Major surgery, complications could cause major blood loss
    - Need product available to replace lost factor during bleed
      - Recombinant Factor VII
  - Inherited factor deficiency
    - Associated potential risks with Recombinant FVII
      - Plasma Derived Factor VII

Case Study:

Patient received Recombinant Factor VII

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Result of case study:

- There were some potential risks with choosing Recombinant Factor VII
  - Potential cause for thromboembolic events after use for those with pre-disposing risk
  - Potential to develop factor VII antibodies

Serious Warnings and Precautions

- Both arterial and venous thromboembolic adverse events have been reported after treatment with **rFVIIa**, mostly in patients with predisposing concurrent risk factors. (See *General* under WARNINGS AND PRECAUTIONS; Pharmacodynamics, under ACTION AND CLINICAL PHARMACOLOGY; ADVERSE REACTIONS).
- Reports of fatal and non-fatal outcomes, including those associated with thromboembolic events have been received during off-label use of **NiaStase RT®** (See *General* under WARNINGS and PRECAUTIONS).
- Patients with inherent Factor VII deficiency may have pre-existing or may develop anti-Factor VII antibodies during therapy with **NiaStase RT®**. The clinical significance of these antibodies is unknown. See ADVERSE REACTIONS section.

*Screen shot from Niastase package insert

Result of case study:

- The best treatment option for this patient was Plasma Derived Factor VII
- Due to the overall circumstances of the case the patient received Recombinant Factor VII

Thank you

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