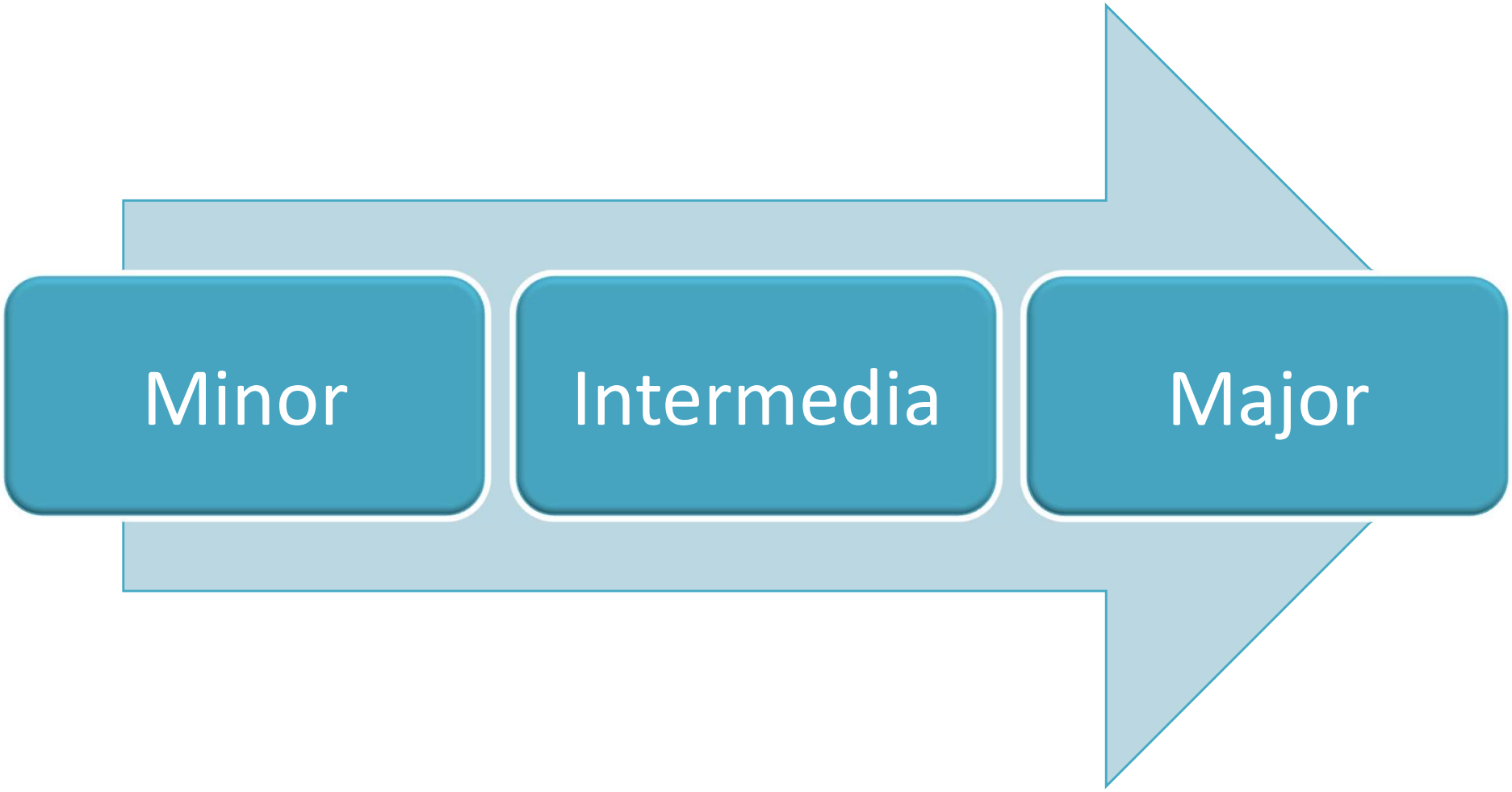

بسم الله الرحمن الرحيم

- $\beta - thalassemia$

At a glance

- Type of Thalassemia
- Transfusion
- Iron chelation
- Splenectomy
- BMT

- Different types of β – thalassemia



Thalassemia minor

- Usually asymptomatic $(\pm Pallor)$
- Diagnosis: CBC, Hb, RBC count,MCV,MCH,HbA٢,HbF,HbA١

C		MCV	MCH	Hb	HbA٢	HbF
Typical β – th minor		↓	↓	NL or ↓	↑ (3,5 – 7%)	↑ (2, –10%)
Atypical β – th minor	δ β thalassema minor	↓	↓	NL or ↓	NL	↑ (2, –10%)
	Semi silent β thalassema minor	↓	↓	NL or ↓	NL	NL
	Silent carrier	NL	NL	NL	NL	NL
		NL	NL	NL	↑	–

Diff: Dx - IDA

α thalassema Minor

- $\beta - thalassemia\ intermedia$

- Wide clinical variation

١. Mild to moderate anemia, normal to deformed face, …)

٢. One or both parents are atypical $\beta - th.\text{minor}$

٣. Splenomegaly

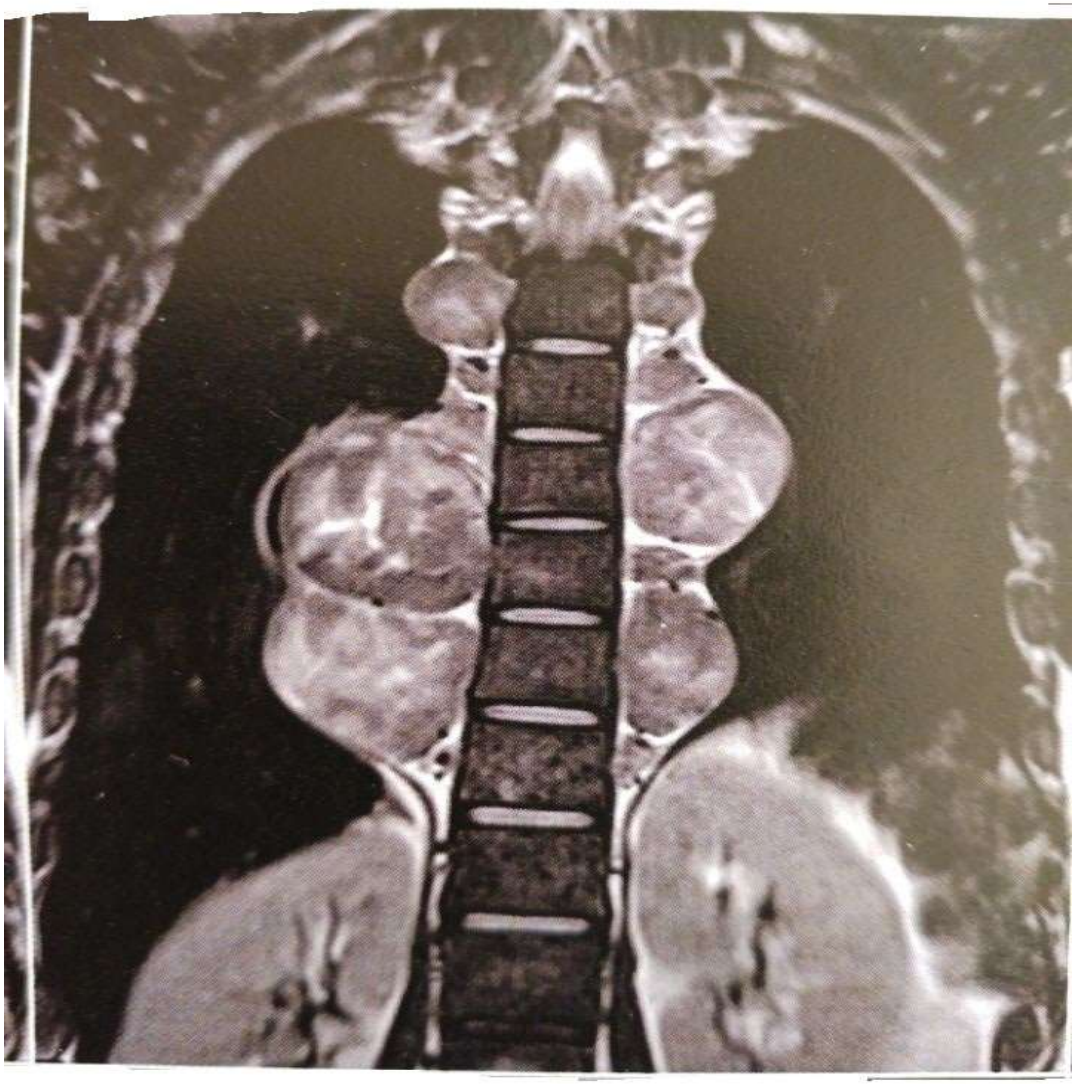
٤. Hb$\geq 7 - 8\ gr/dl$

٥. Age of diagnosis $> 2\ \ y/o$

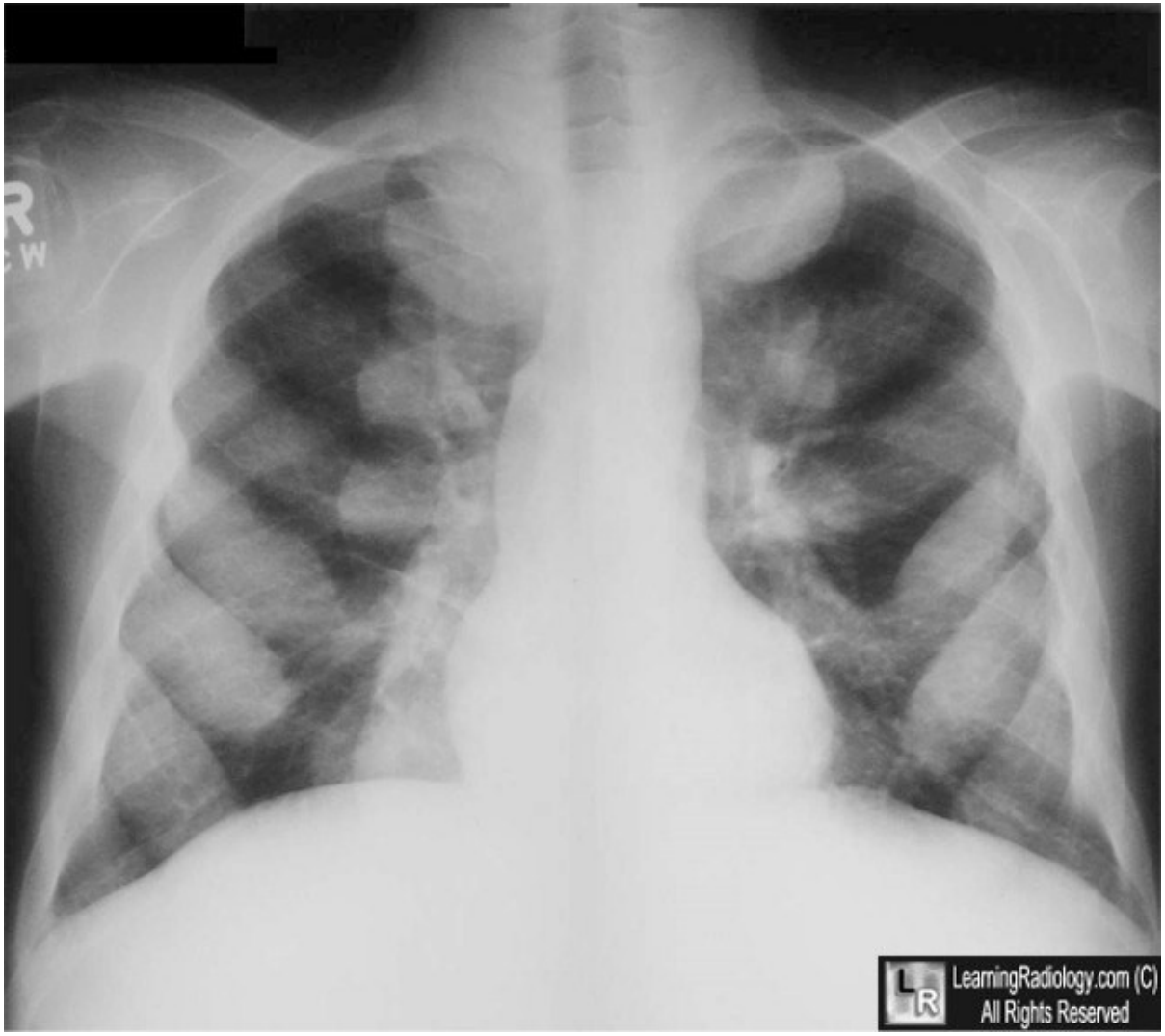
- β – *thalassemia intermedia*

- Acid folic : ۵ mg
- Hydroxyurea : ۱۰-۲۰ mg / kg/ day
- Blood transfusion
- Splenectomy

- β – *thalassemia intermedia*



- β – *thalassemia intermedia*



- $\beta - thalassemia\ major$

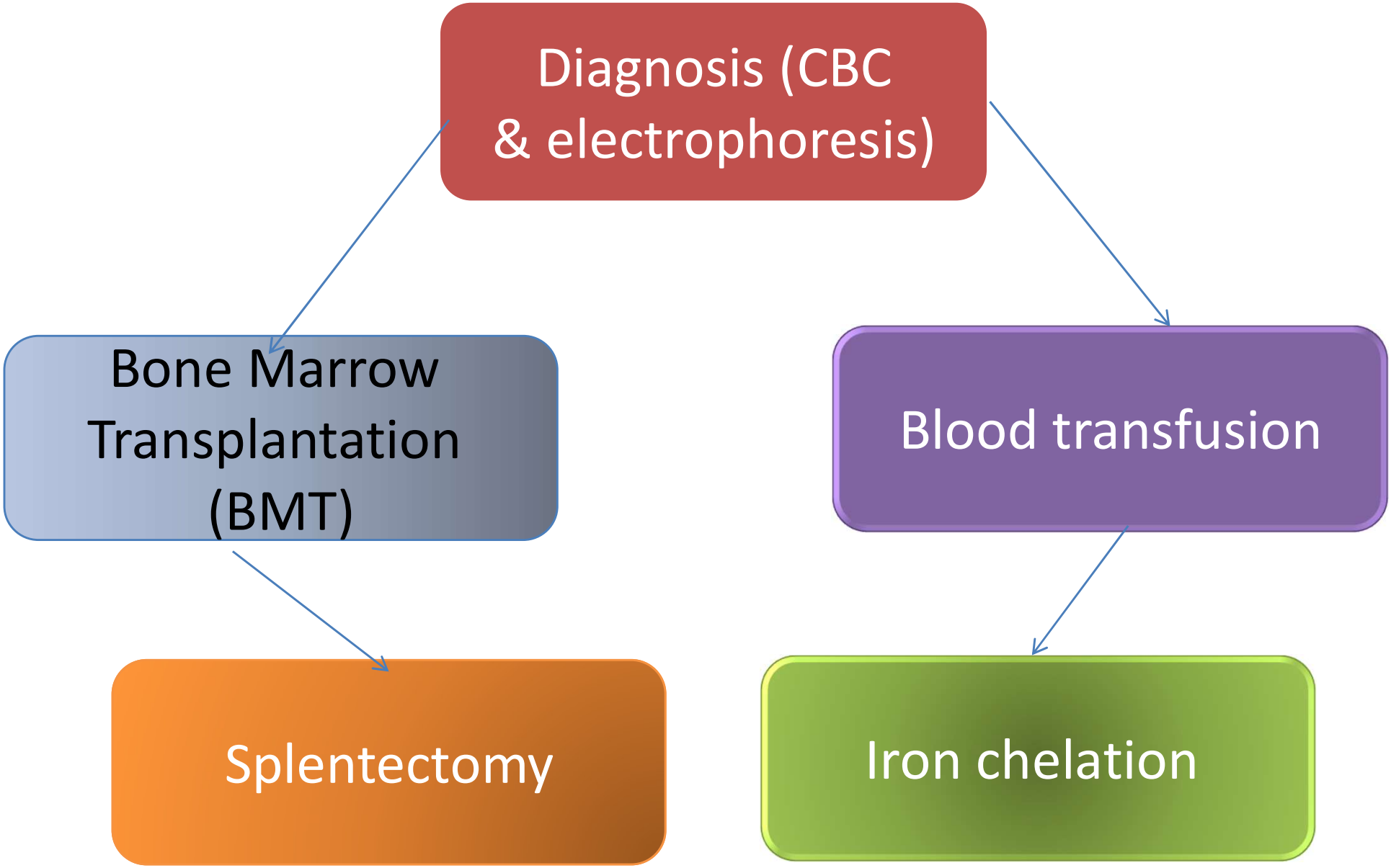
١. Age: < ٢ y/o

٢. Both parents are typical$\beta - th$. minor

٣. Hb < ٧ gr/dl

٤. Need blood transfusion

- ***Treatment β − thalassemia major***



Transfustion

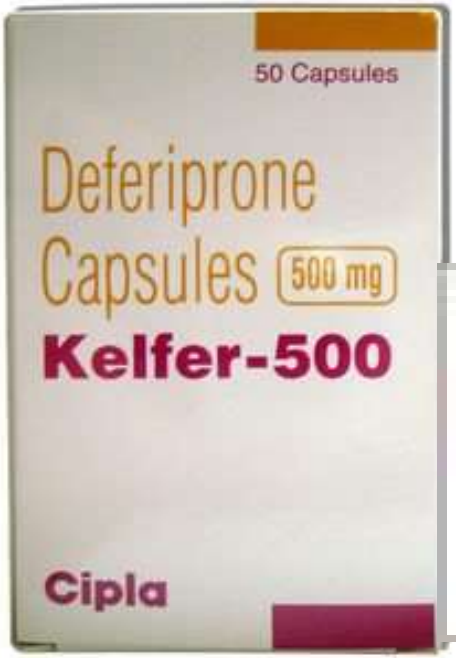
- Transfuse : ١٥-٢٠ ml/kg body weight over ٢-٣ hours (rate of infusion : ٥-٨ ml/kg/hr)
- In **heart failure**: ٥ ml/kg body weight over ٣-٤ hours (rate of infusion : ٢ CC/kg/h)
- LeukodepLeted red cell concentrates (filter)
- Keep pretransfusion on H b at ٩-٥- ١٠.٥ g/dl
- Transfuse every ٢-٤ weeks

- ***Iron chelation***

- Deferoxamine (desferal) (IV or SC)

- Deferipron, (L1),(Feriprox),(Po)

- Deferasirox (Exjade),(osveral),(Jadenu)



• *Iron chelation*

-Jadenu ٣٦٠ mg = exjade ٥٠٠ mg.

-Jadenu ١٨٠ mg = Exgade٢٥٠ mg

-Jadenu ٩٠ mg= exjade ١٢٥ mg

.Elijade

.Nanojade

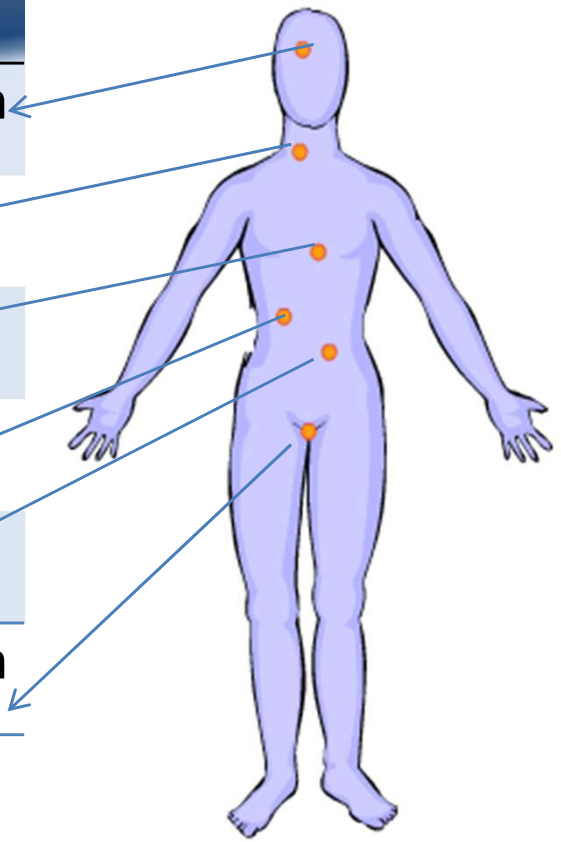
.Nusveral

.Defrazex

.Avisirox

- ***Clinical consequences of iron overload***

Organ	Consequences
Pituitary gland	Hypogonadotropic hypogonadism
Thyroid	Hypothyroidism
Heart	Cardiomyopathy
Liver	Cirrhosis;carcinoma
Pancreas	Diabetes
Gonads	Hypogonadotropic hypogonadism



- ***Deferoxamine***

-When to start :

- More than ١٠-٢٠ times blood transfusion
- Serum ferritin > ١٠٠٠ ng/ Ml
- Age > ٣ y/o (Not always)

- Dose :

- Children ٣٠-٤٠ mg/kg ٦ nights/wk
- Adult ٣٠-٥٠ mg/kg, ٦ nights/wk

- ***Deferoxmine***

- Rout of administration : IV infusion >S C > IM (choice : Sc by pump)
- Duration : ٨-١٢ hours by pump
- Patients **Vit C** status: **Vit C** depletion is common in iron over load

and reduce iron excretion in response to desferal (it is used٢ months after starting desferal with dose of ٣ mg/kg ١**houre** after initiation desferal pump infusion)

Complications:

١. Short stature and skeletal deformity if used too early
٢. Zinc deficiency
٣. ↓ed hearing and vision
٤. Urine discoloration
٥. Local reaction

- ***Deferiprone (L1)***
  - Dose : ٧٥ -٩٠ mg/kg/day divided to ٣ times/day(Q٨h)
    - ***Complications:***

١) The most severe adverse effect : **Agranulocytosis** <٥٠٠ (١%)

٢) Arthralgia & arthritis

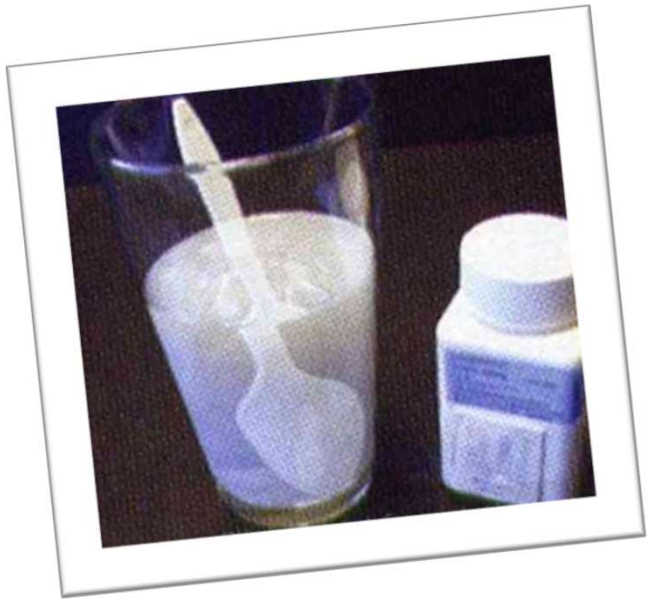
٣) Liver fibrosis ?

٤) Nausea vomiting , urine discoloration

٥) Zinc deficiency

- ***Deferasirox (Exjade)***

  - A once – daily oral chelator



- ***Deferasirox (Exjade)***

Dosage

- Exjade is a **Once Daily dose ٢٠-٤٠ mg/ kg** depend on the degree of Iron Overload

- ***Deferasirox (Exjade)***

  - Treatment – Related Adverse Events by Dose Level

		Exjade		
Preferred term	Severity	١٠ mg/kg (n=٥)	٢٠ mg/kg (n= ٦)	٤٠ mg / kg (n=٧)
Nausea	Mild	-	٢	١
Vomiting	Moderate	-	-	١
Diarrhea	Mild	-	١	٣
Abdominal pain	**Mild**	-	-	١

SPLENECTOMY IN BETATHALASSEMIA MAJOR

- ***Effects of splenectomy***

Disadvantages

- Infection
- Thrombosis
- ↑ Iron absorption

Advantages

- Reduces or remove
  Terns fusion need
- Improves anaemia
- Removes discomfort

- **Other conditions should be ruled out before splenectomy**

  - Acid folic deficiency
  - Infection (such as PVB ١٩ …)
  - All immunization (direct and indirect coombs test)
  - Blood volume

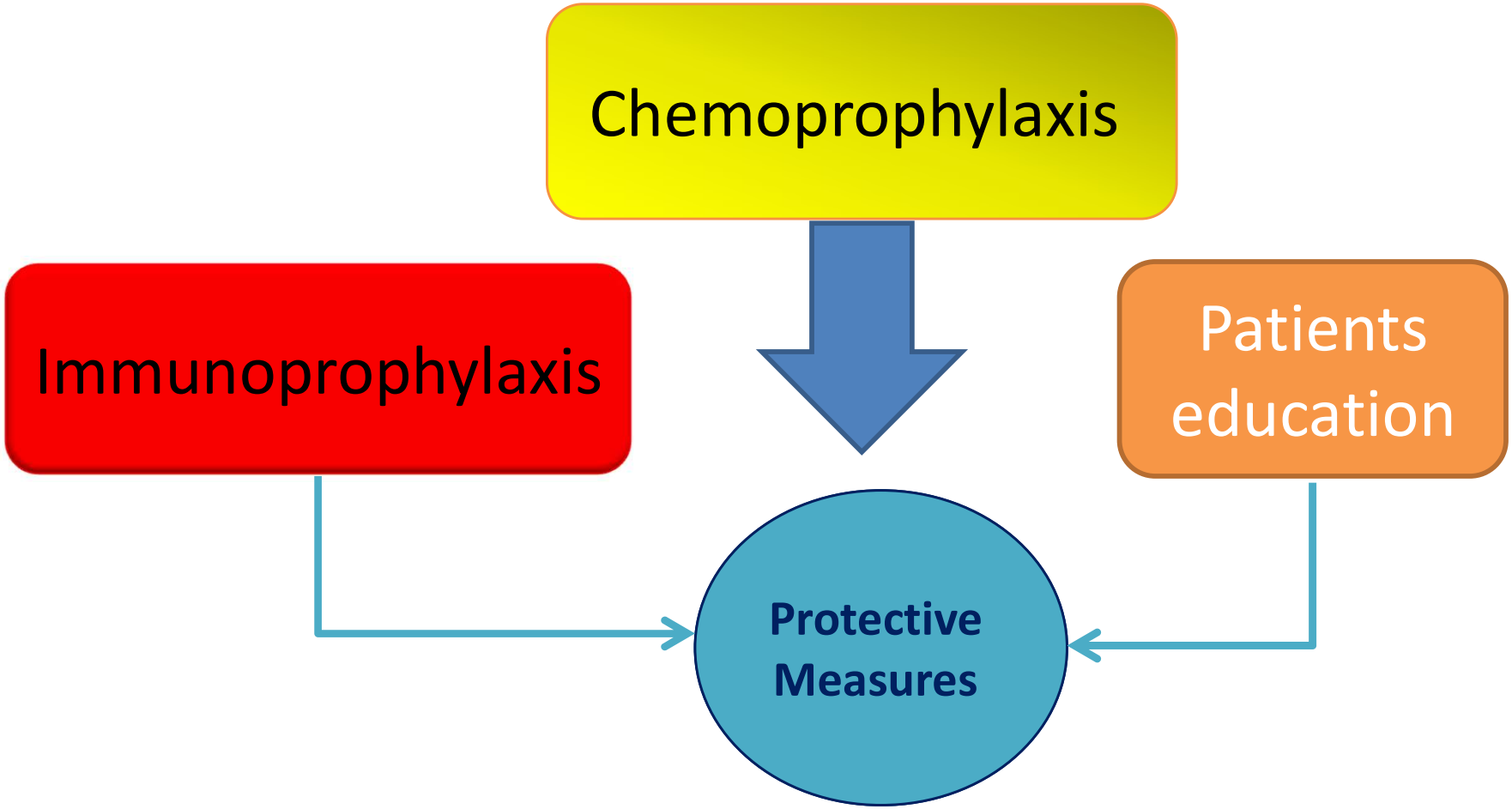
- ***Work up before splenectomy***

Abdominal songragraphy (rule out gall stone and accessory spleen)

Vaccination

Preventive measures

- There are three types of protective measures a physician can utilise after splenectomy



Bone Marrow Transplantation (BMT)

- All children who have an HLA-matched sibling should be offered the option of bone marrow transplantation.
- In low-risk HLA-matched sibling patients, there is at least a ٩٠% survival and an ٨٠% event-free survival.


Thank You For Your Attention
Any Question?