Thalassemia Research Center

MAHIDOL UNIVERSITY
Wisdom of the Land

Ideal Center for Hemoglobinopathies

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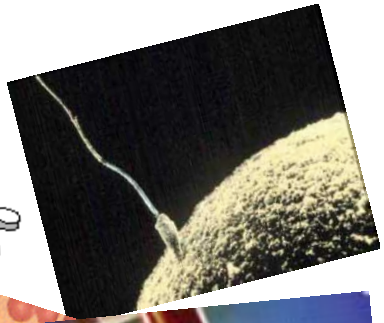
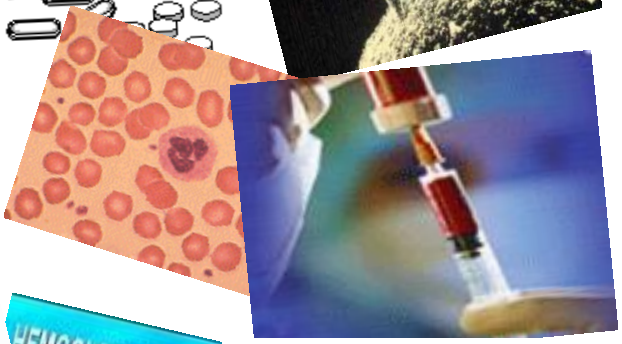
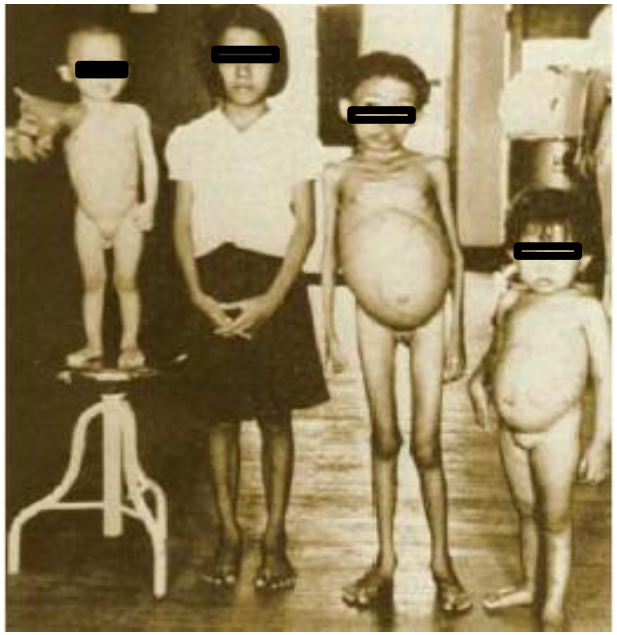
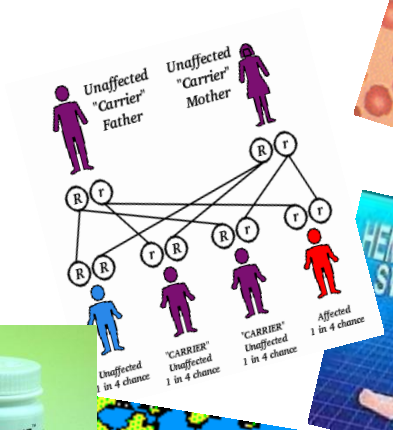
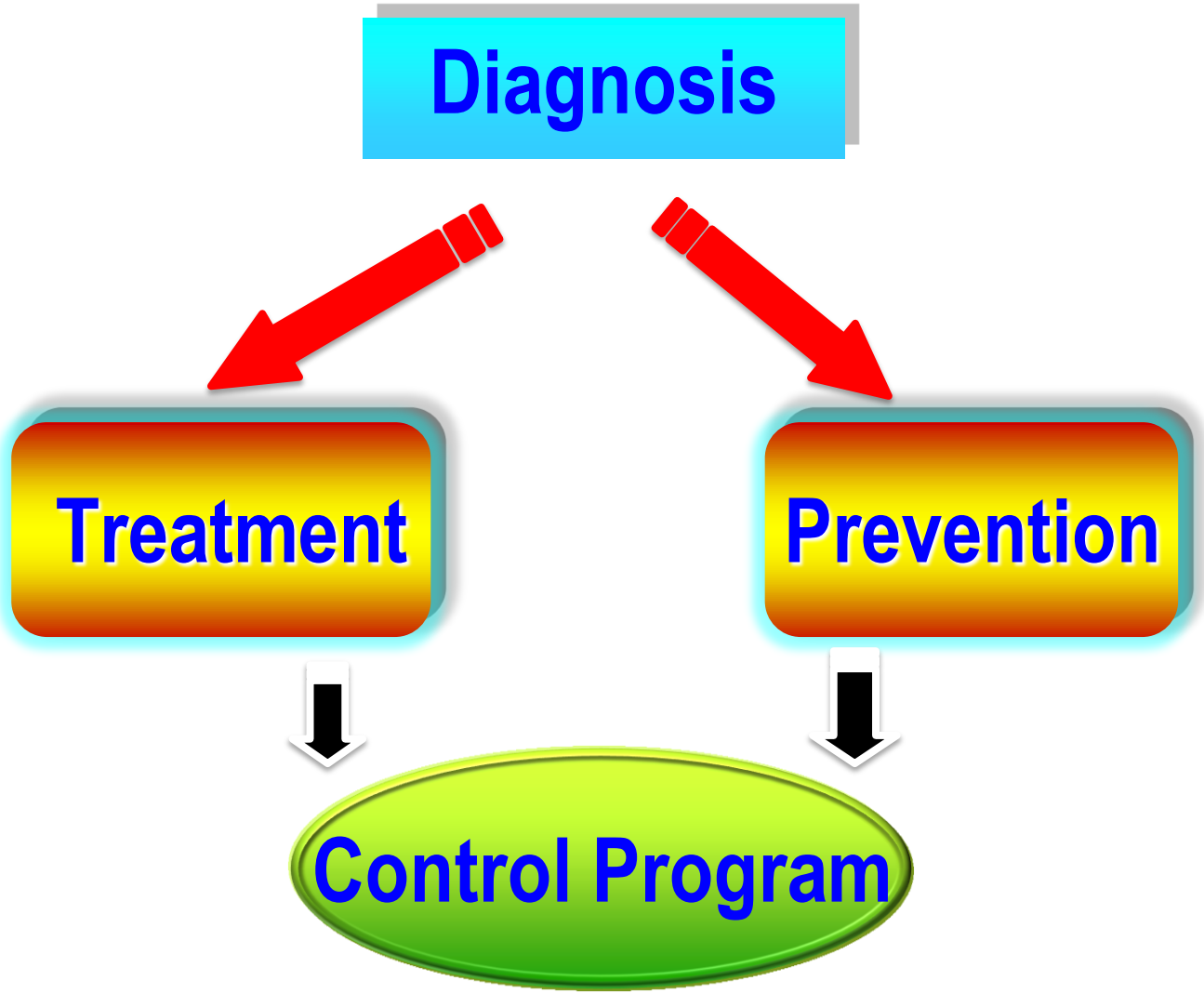




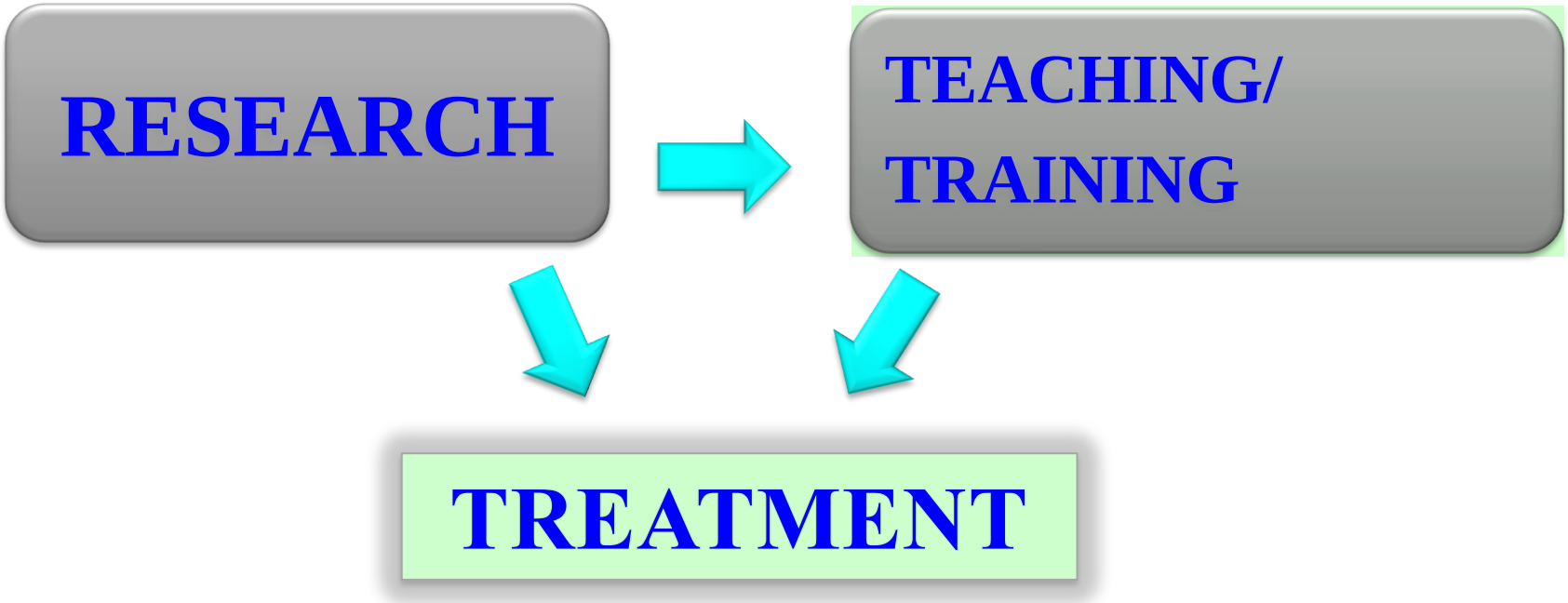




THALASSEMIA CENTER



THALASSEMIA CENTER



Outline of my talk:

1) TIF recommendation

2) Thalassemia Center, Torino, Italy (Antonio Piga)

3) Thalassemia prevention, Guagxi, China

"Nanning Declaration"

4) SEARO/WHO

Aims of Management

Thalassaemia International Federation

- Long Survival
- Good Quality of Life

Survival of patients

- Optimum treatment required for survival and quality of life.
- No treatment means early death
- Less treatment means poor quality of life and early death

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Factors affecting survival

- The treatment protocol
- Adherence to it
- Family support
- Psychosocial support
- Quality of follow-up
- Early recognition and intervention of complications

Quality of life

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- Good clinical condition
- Psychosocial support
- Endocrinological monitoring of growth, puberty, hypogonadism, amenorrhoea, reproduction
- Complications e.g. diabetes, heart
- Bone pains
- Employment, marriage

Patient care

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- Voluntary blood donation
- Safe blood
- Provision of essential drugs, chelating agents
- Free medical treatment
- Expert Reference Centers. Quality of care.
- Psychosocial support
- National registers

Thalassaemia International Federation

Patients must also cope with:

- The disease, its complications and chronicity
- Demanding treatment, especially chelation
- Family: supportive but may increase stress
- Caregivers (doctors/nurses) – overprotective, or figures of authority
- Society: peer relationships, stigmatisation, education, career, marriage

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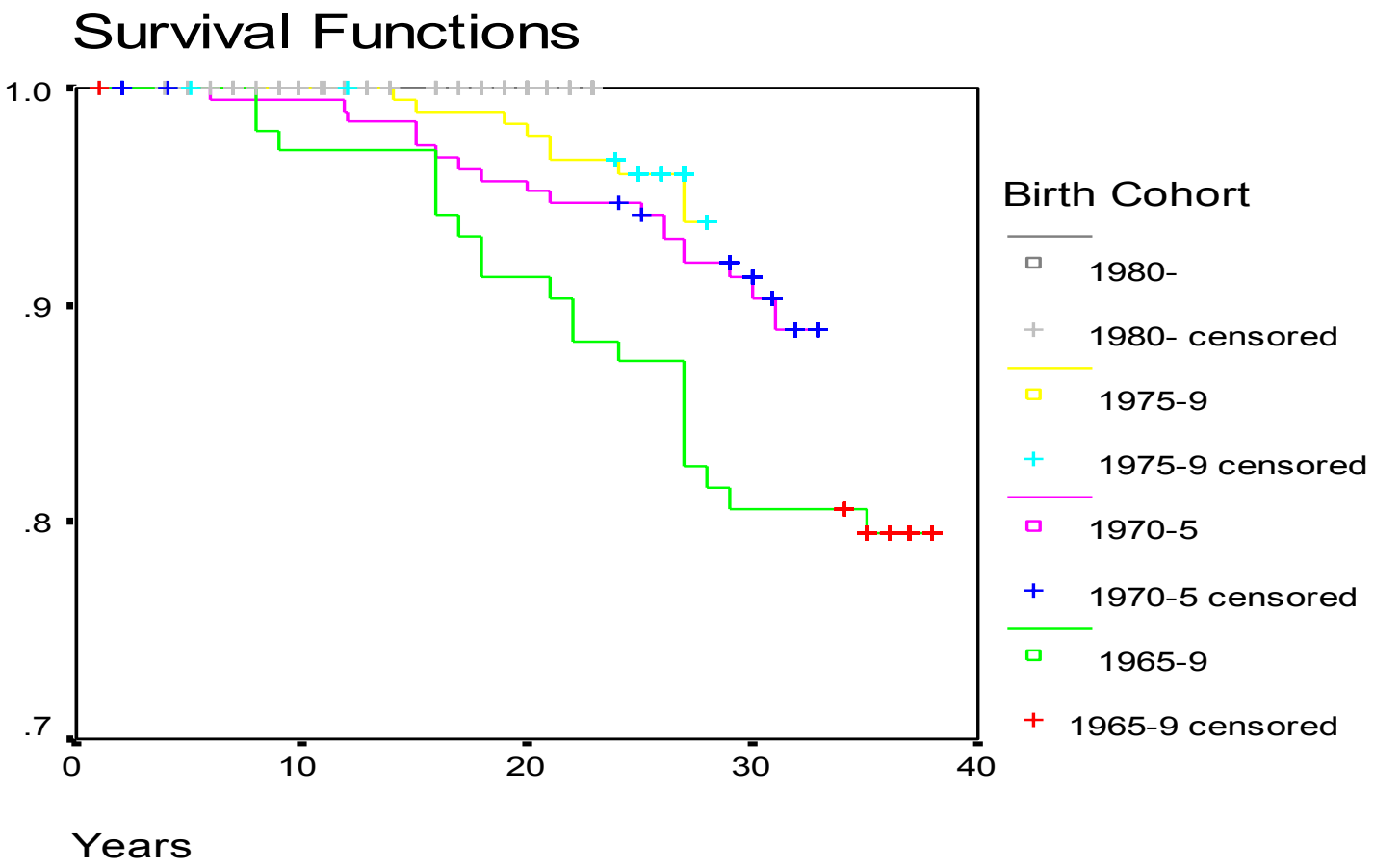
Survival of thalassemia patients in Cyprus by birth cohort



Comprehensive care

- Treating the whole person and the family, through continuous supervision of all the medical and psychosocial aspects…
- Every facet of the person – physical, emotional, psychological, educational, financial and vocational factors are addressed
- www.hemophilia.org

Comprehensive care?

- In a European survey (Enerca) – *20.5% of thalassaemia patients wait for more than 2 hours* for the blood transfusion.

- *61.7% are transfused in the mornings* – to suit the hospital without considering the educational (50% attend school or university) or employment (13% work full time) commitments of the patients

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Patient needs – rarely satisfied

- 271 hours per month are spent on blood transfusions, chelation, other medical appointments, travel time, phoning'

  Compagno LM et al Ann NY Acad Sci, 2005

- Transfusion times to suite education and work
- Shortening waiting time
- Increasing communication time

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Time

- Average time doctor spends with a patient in the US: 18.7 minutes
- Average time doctor spends with a patient in Germany: 15 minutes
- What about patients with chronic disorders?
- *What about Thailand? Medical Center vs Provincial hospital*

The need for expert centres

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- A centre where the quality of comprehensive, patient centred, care is assured
- The capacity to provide expert diagnosis or confirmation of diagnosis, including genetic tests and genetic counselling
- Laboratory as well as clinical capacity
- Training and education of stakeholders and service providers

Expert Centre

Adapted from the EU Task Force Criteria

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- A centre where the quality of comprehensive care is assured
- Where there is the capacity to provide expert diagnosis
- Well qualified staff with experience
- Supporting- self management
- Networking with secondary centres but also with other expert centres

Expert thalassaemia centre

The capacity to provide expert case management:

- Good practice guidelines and protocols
- Expert advice & information to patients
- Multidisciplinary team- coordinated
- Psychosocial support
- Staff/patient ratio

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Patient-friendly services

- Staff willing to spend time and to listen
- Privacy and confidentiality
- Discuss sexuality, contraception, puberty, diet, risky behaviour, school problems, etc.
- Convenient appointment times – consider school and work
- Patients feel welcome and safe

Clinical information systems

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- Using technology to organise data
- Monitoring patient health status
- Electronic patient records
- Identifying patient sub-groups for pro-active interventions
- Rights of patients to the contents of their records
- Electronic patient registries
- Networking between centres nationally and internationally

Decision support in thalassaemia centres

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- Evidence-based guidelines (updated)
- National standards for optimal care
- Regular training of staff in using protocols
- Sharing information with patients
- Electronic infrastructure, telemedicine, videoconferencing between doctors, etc.

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Delivery system design

- Coordinating the multidisciplinary team
- Defining roles in the team
- Regular follow up of patients
- Communicating with the patient at home
- Collaboration with primary care services and supporting local physicians

Self-management support

- Patient empowerment- improving autonomy
- Patient / caregiver partnership in setting goals, action plans, presenting solutions
- Patient information – person to person – to acquire skills and confidence in self-care
- Health workers' time
- Changing physicians' attitudes: paternalistic to partnership

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Community resources

- Supporting patients needs
- Educational needs of young patients
- Patient support groups and assistance from without the reference centre
- Links between the Centre and community agencies – social assistance
- Health education and information to the public

Additional standards

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Links to primary/secondary services:

- Patients with poor access to the reference centre – distance, poverty
- Ethnic minorities/immigrant groups – scattered geographically
- Private/public sector relationship (does referral to the centre cancel the role of the private or primary care provider?)

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Other duties of the thalassaemia service

- Epidemiological surveillance
- Collaboration/links with other national and international centres
- Close links with patient organisations

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Conclusion: Expert centres

Sufficient activity and capacity to provide services, gain experience, and sustain quality:

What is the minimum throughput for each service?

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Survival – treatment in specialised centres

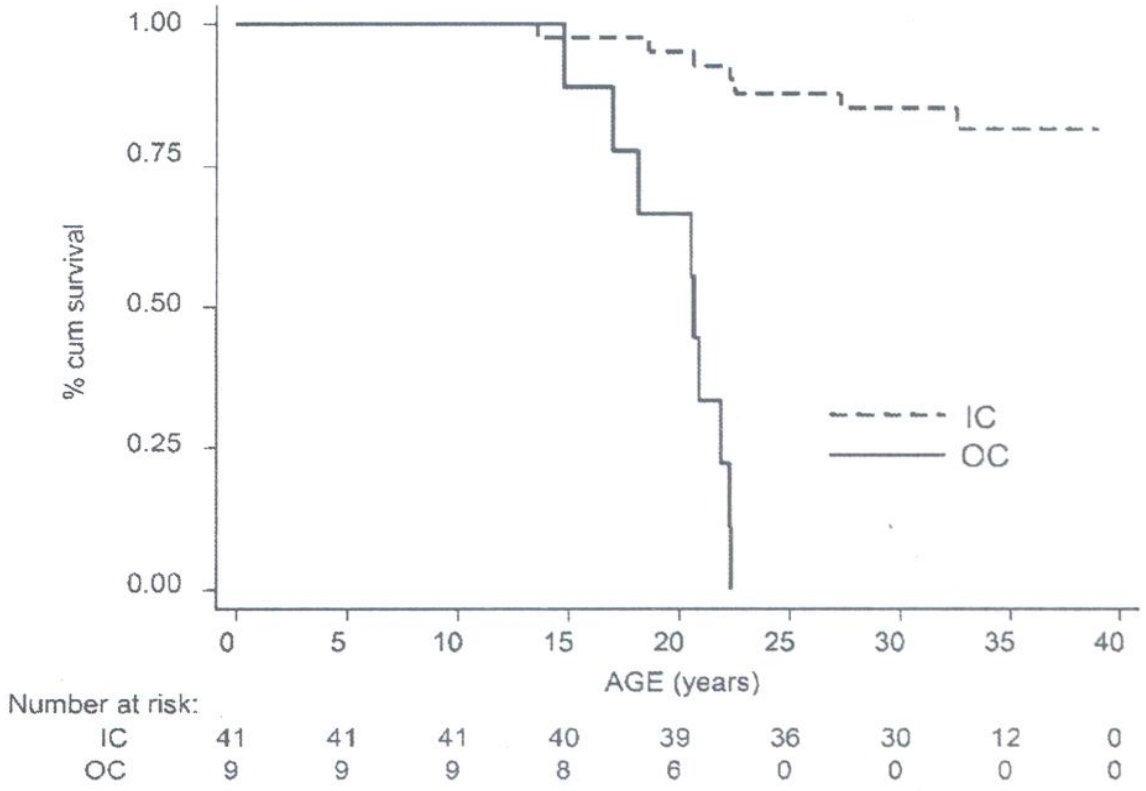

Fig. 1. Kaplan–Meier overall survival curves of patients referred to specialized centers (IC) versus patients referred to nonspecialized centers (OC). Log-rank *P*-value < 0.0001; hazard ratio of OC versus IC adjusted for sex (Cox model): 18.1, 95% confidence interval = 4.7–69.0; $P < 0.001$.

12TH INTERNATIONAL CONFERENCE ON THALASSAEMIA AND THE HAEMOGLOBINOPATHIES
14TH INTERNATIONAL TIF CONFERENCE FOR THALASSAEMIA PATIENTS AND PARENTS
ANTALYA, TURKEY, 11-14 MAY, 2011

The Ideal Centre for Haemoglobinopathies

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CENTER CHARACTERISTICS

- Responsibility with a single doctor, remaining the same as long as possible
- Dedicated Centre
- Dedicated staff
  - Staff stability
  - Institutional setting
  - Adequate staff/patients ratio

CENTRE CHARACTERISTICS

Adequate staff/patients ratio

- 1 nurse / 33 patients
- 1 doctor /50 patients
- 1 psychologist /100 patients
- 1 secretary /100 patients

from: WHO, Hereditary Diseases Program, 1994

CENTER CHARACTERISTICS

- Responsibility with a single doctor, remaining the same as long as possible
- Dedicated Centre
- Dedicated staff
    - Trained in Hbpaties
    - Career development
    - The quality as a target
    - Active care

"ACTIVE" CLINICAL CARE

- Set the appointment
- Plan tests
- Book tests
- Visit the patient
- Set case review
- Set patient-doctor conversation

ASSESSMENT OF IRON-RELATED COMPLICATIONS IN TM

1	2	3
COMPLICATIONS	**TESTS (diagnosis)**	**TESTS (risk)**
Heart Disease	EKG, Echocardio, Holter	Cardiac Iron (MR)
Liver Disease	↑ALT, AST, ↑fibroscan	Liver Iron (MR, SQUID)
Diabetes	OGTT	Pancreas MR[1-4]
Hypothyroidism	↑TSH, ↓fT3, ↓fT4	Liver Iron?
Hypoparathyroidism	↓PTH, ↓Ca, ↓1,25(OH)$_2$VitD3	Liver Iron?
Adrenal Insufficiency	↓ACTH test	Liver Iron?[5]
Hypogonadism, Infertility	↓LH, ↓FSH, ↓sex hormones	Pituitary Iron & Volume[6]
Growth Hormone Defic.	↓GH, ↓IGF-I	Liver Iron?
Osteoporosis	↓DEXA	Liver Iron?

[1]Au-2007; [2]Noetzli-2009; [3]Papakonstantinou-2009; [4]Noetzli-2010; [5]Scacchi-2010; [6]Wood-2010;

ASSESSMENT OF IRON-RELATED COMPLICATIONS IN TM

Recommended Monitoring

LIVER & HEART	At diagnosis	0-3 y	3–10 y	10-18 y	Adulthood	More often, if:
ALT, AST	Yes	At transf.	At transf.	At transf.	At transf.	
Serum Ferritin	Yes	1-3 months	1-3 months	1-3 months	1-3 months	
Liver/spleen US	Yes	yearly	yearly	yearly	yearly	High risk
Liver fibrosis (Fibroscan)	Yes		yearly	yearly	yearly	High risk
Electrocardiogram	Yes		yearly	yearly	yearly	Severe iron overload, palpitations
Ecocardiogram	Yes			yearly	yearly	Severe iron overload, cardiac failure
Holter	No		Specific indication	Specific indication	Specific indication	Arrhythmia
Liver Iron (MRI, SQUID)	Yes	yearly	yearly	yearly	yearly	Severe iron overload, Treatment changes
Heart Iron (MRI)	No			yearly	yearly	Severe iron overload, Treatment changes

CONTINUITY OF CARE

- Rapid access to specific care
- Centre doctor available for consultation or sharing the responsability of management in any condition like:
  - **Emergency**
  - **In-patient**
  - **Infections**
  - **Heart failure**
  - **Splenectomy**
  - **Intensive chelation**
  - **Bone marrow transplant**

Applicazione Web-WTTO - Netscape

File Modifica Visualizza Vai Communicator ?

Indietro Avanti Ricarica Home Ricerca Guida Stampa Sicurezza Interrompi

Segnalibri Indirizzo: http://130.192.110.2/WWW/MOSTDOC-THAL2.0/html?WTTO+auth+switch_protocol Arg. correlati

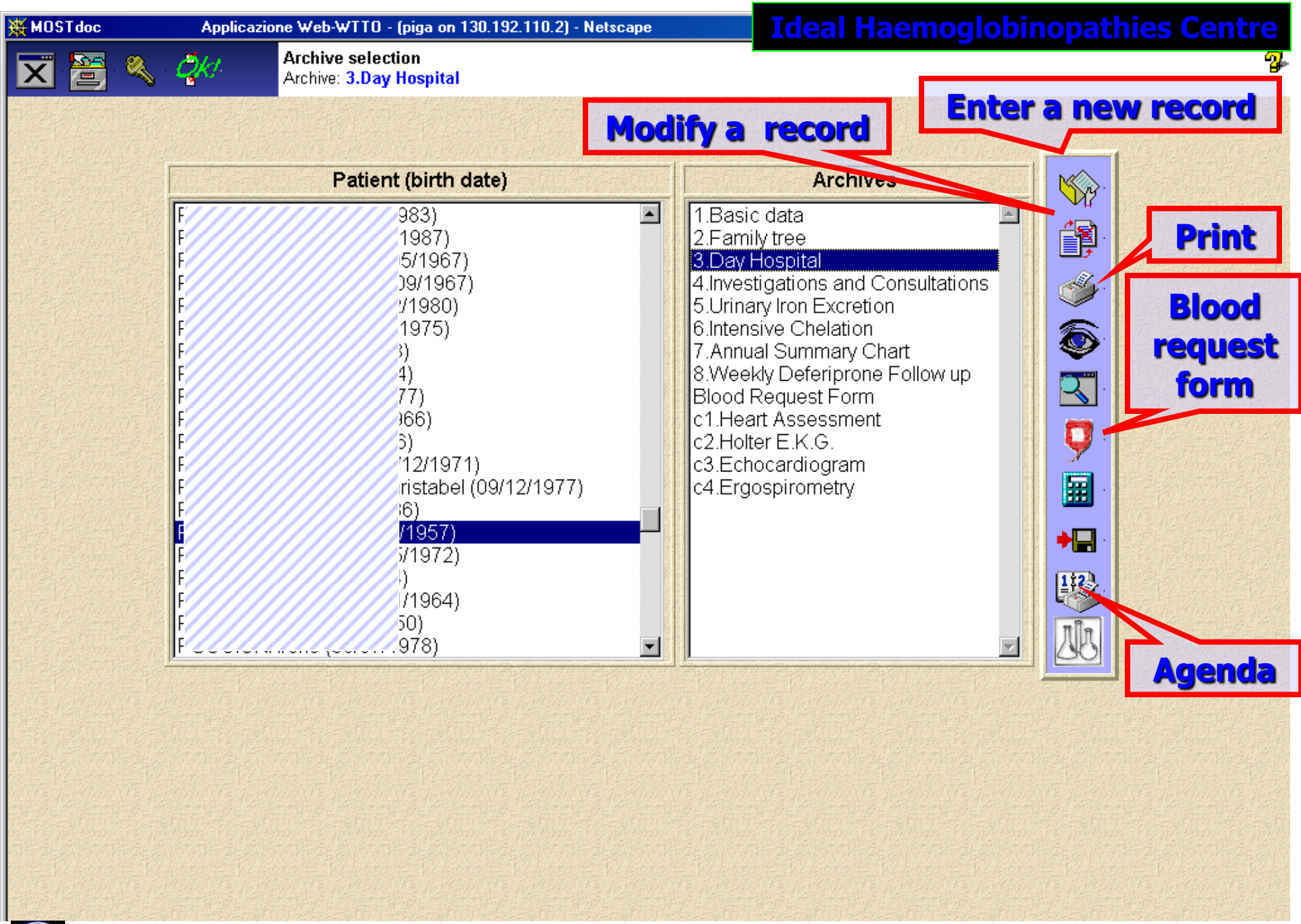
meetings thalassemia viaggiare immagini-med acquisti Google Motori di ricer iron guidelines EBM book&music metal detector journa

Web THAL

Cartella clinica computerizzata per la thalassemia

User: piga **Password:** ******

Avvio Start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Applicazione Web-WTTO - (piga on 130.192.110.2) - Netscape
Ideal Haemoglobinopathies Centre
Archive selection
Archive: 3.Day Hospital
Modify a record
Enter a new record
Print
Blood request form
Agenda
Patient (birth date)
Archives
1.Basic data
2.Family tree
3.Day Hospital
4.Investigations and Consultations
5.Urinary Iron Excretion
6.Intensive Chelation
7.Annual Summary Chart
8.Weekly Deferiprone Follow up
Blood Request Form
c1.Heart Assessment
c2.Holter E.K.G.
c3.Echocardiogram
c4.Ergospirometry

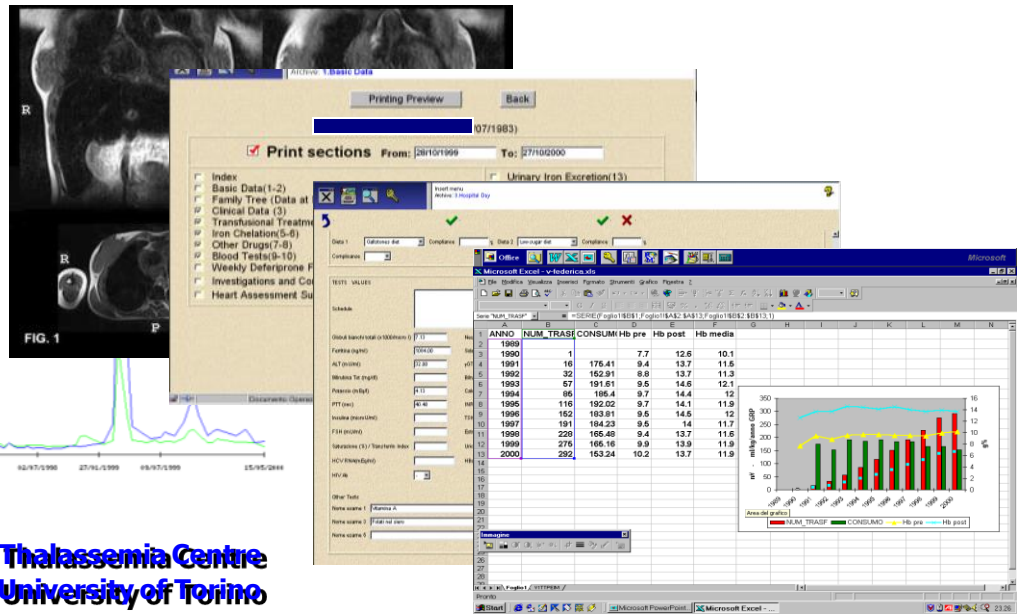
THALASSEMIA-ORIENTED COMPUTERIZED CLINICAL RECORD

Row data from
- transfusion
- chelation
- tests

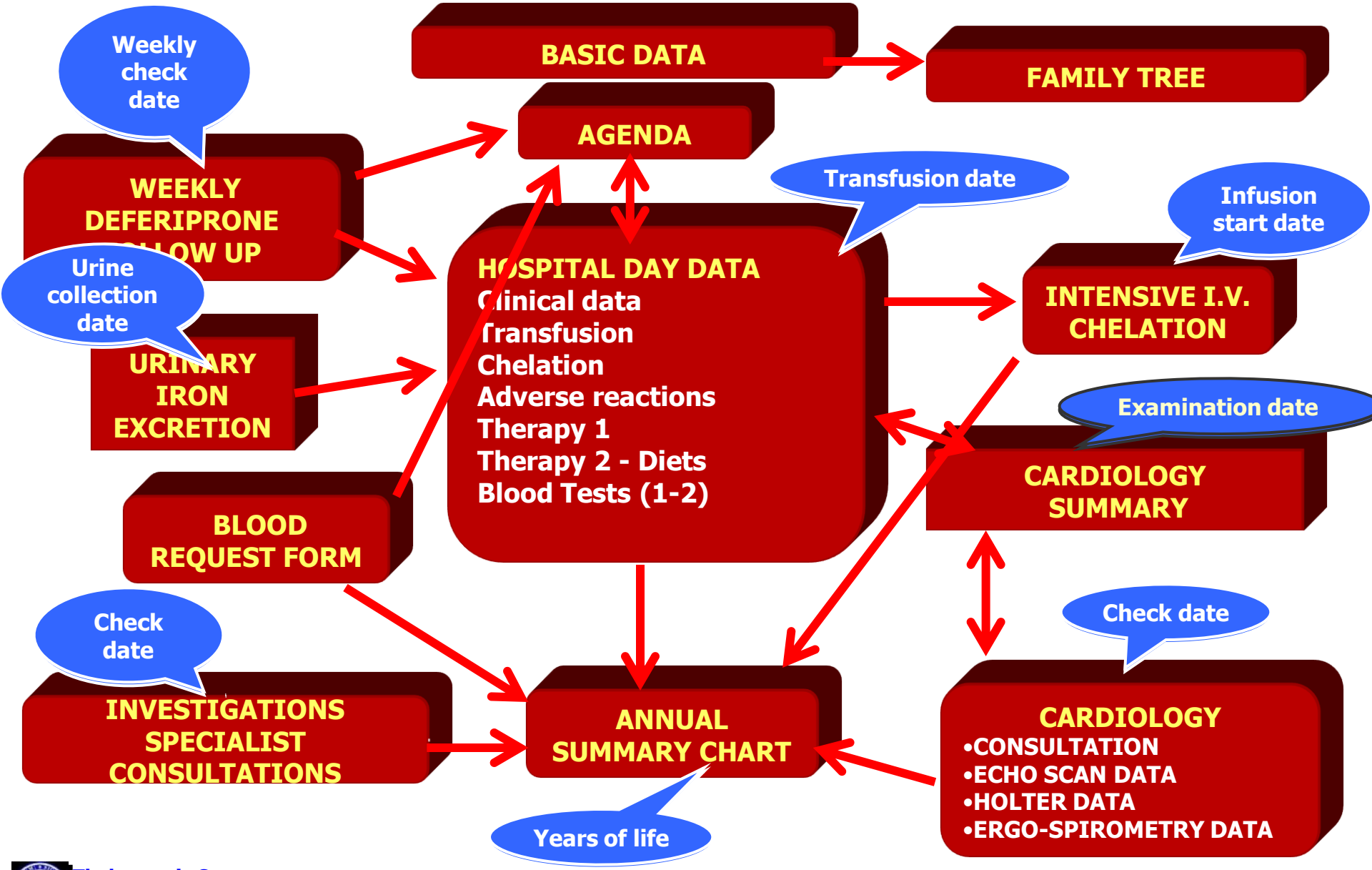
Complications

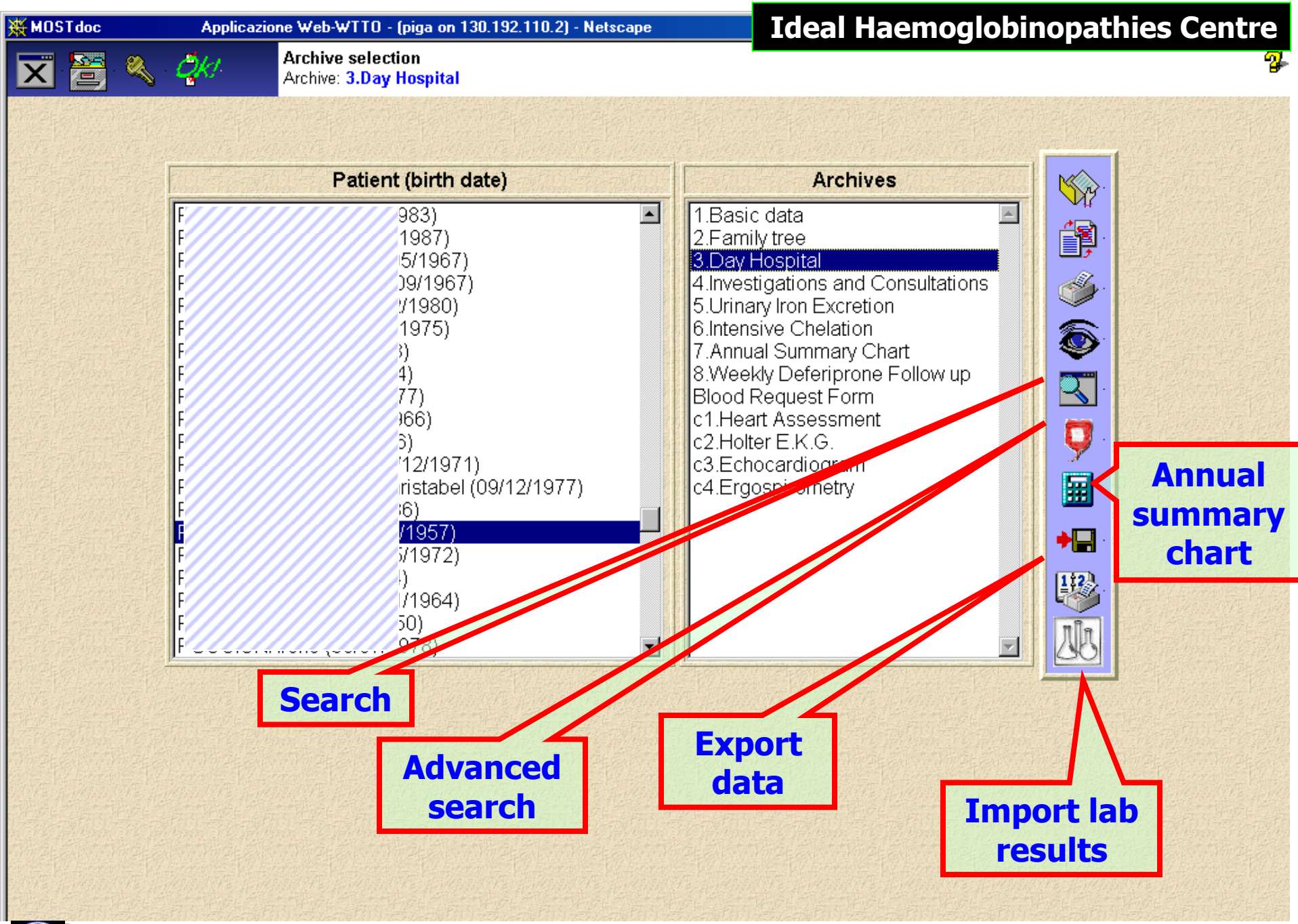
Therapies

Transfusional indexes
Iron balance
Appointments
Print-outs
Graphs
Multicentre studies



Archives structure

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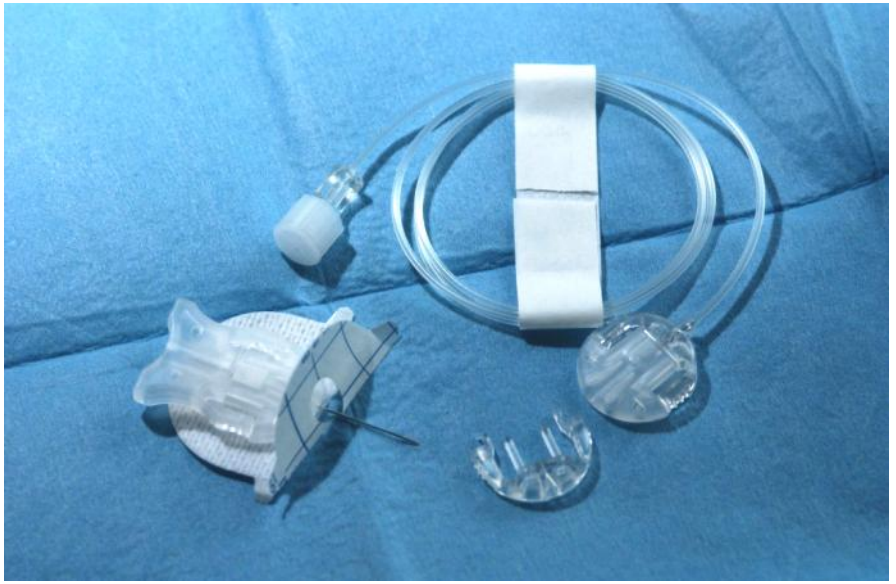
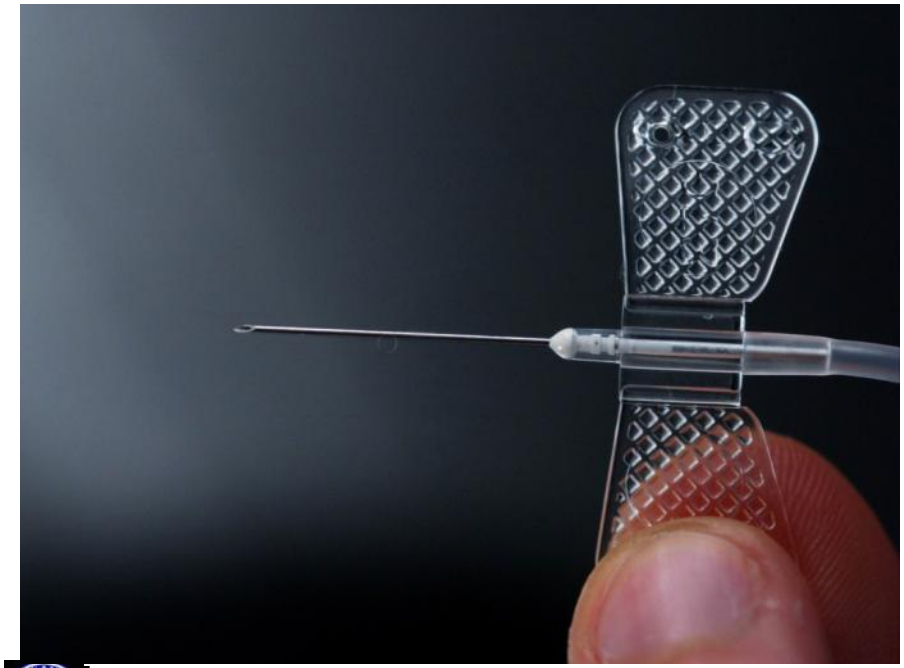
Blood Request Form
c1.Heart Assessment
c2.Holter E.K.G.
c3.Echocardiogram
c4.Ergospirometry
Search
Advanced search
Export data
Import lab results
Annual summary chart

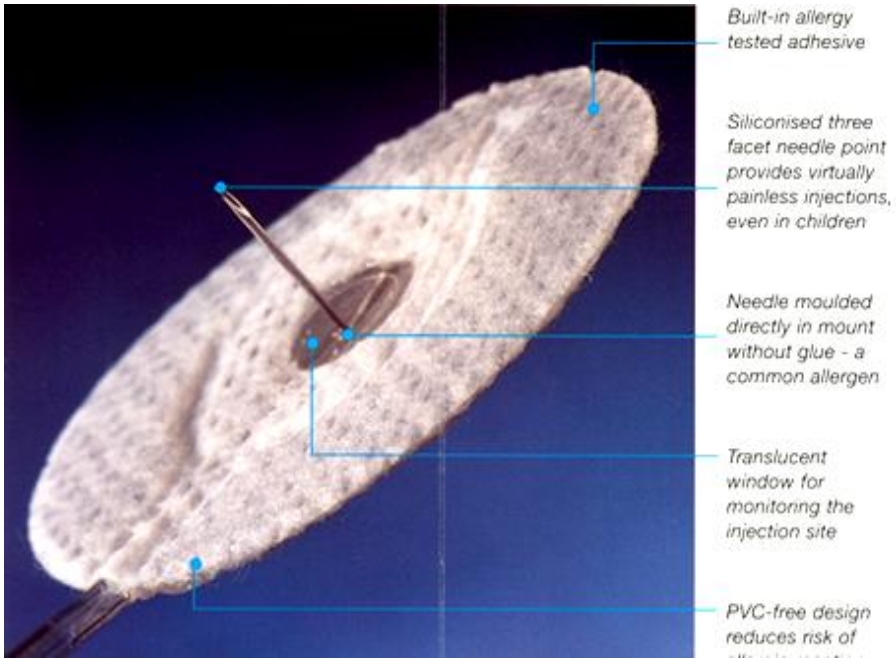
TRANSFUSION

- High quality blood
- Comfortable setting
- Patient-oriented facility (day, night, holiday)


MEM
h
min
0363
dose
0,2 ml
OFF
ON

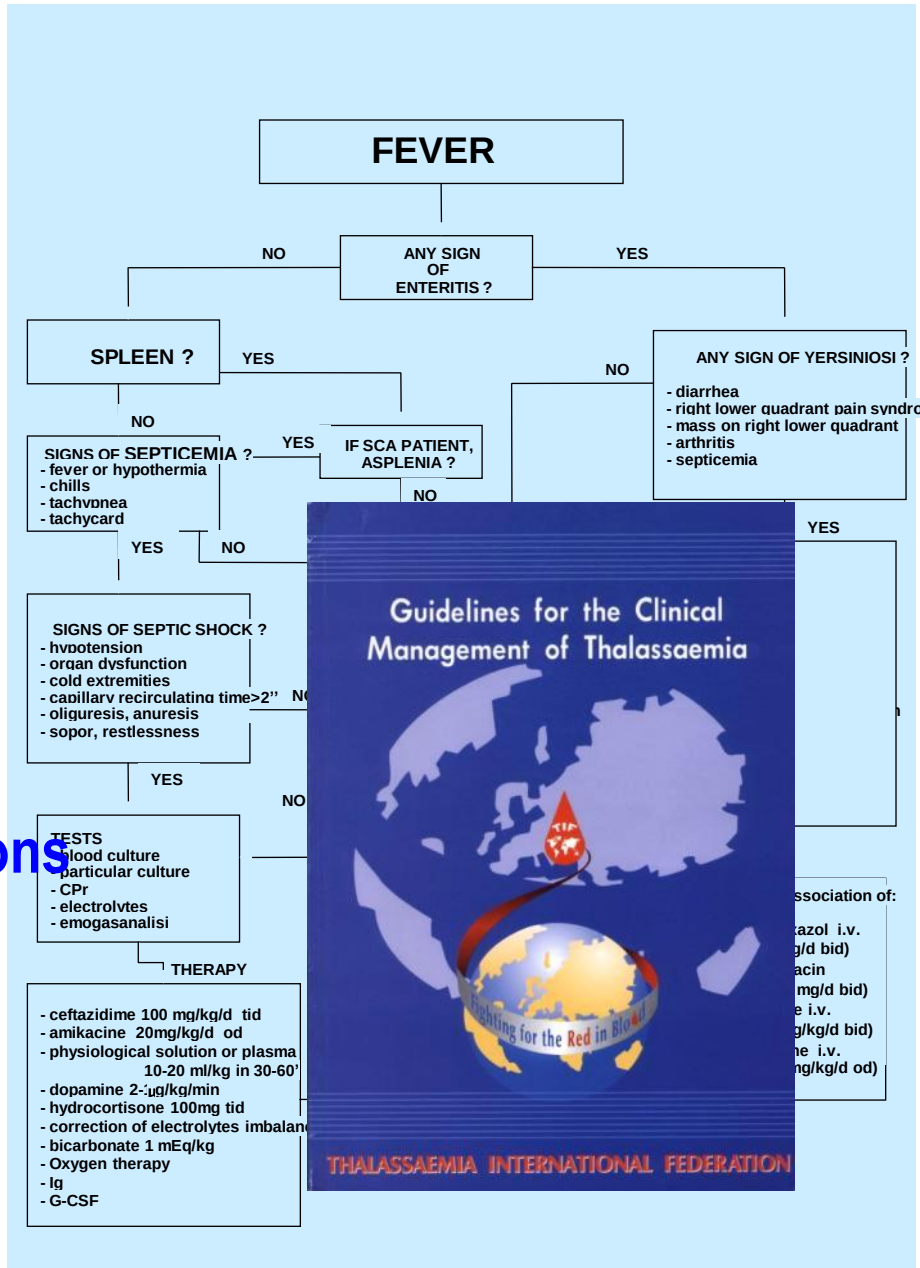





Built-in allergy tested adhesive
Siliconised three facet needle point provides virtually painless injections, even in children
Needle moulded directly in mount without glue - a common allergen
Translucent window for monitoring the injection site
PVC-free design reduces risk of

PROCEDURES

- Hemovigilance
- Splenectomy
- Fever
- Growth monitoring
- Chelation efficacy monitoring
- Chelation tolerability monitoring
- Intravenous Desferal
- Combination therapy
- Monitoring iron-related complications
- Family planning
- Pregnancy (follow up)
- BMT



CENTRE CHARACTERISTICS

- Responsibility with a single doctor, remaining the same as long as possible
- Dedicated Centre
- Dedicated staff
- Dedicated clinical record & procedures
- Availability of mental health professionals experienced in chronic diseases

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GUIDELINES FOR PSYCHOSOCIAL SUPPORT

2 - Methods

a - Containment model:

- Listening to
- Accepting
- Sharing
- Understanding
- Communicating

GUIDELINES FOR PSYCHOSOCIAL SUPPORT

3 - Methods - Tools

- Individual conversations
- Group meetings
- Summer camps
- Chelation clinic
- Staff meetings
- Interventions from mental health professionals and social worker

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- Multidisciplinary team

MULTIDISCIPLINARY TEAM

WHO

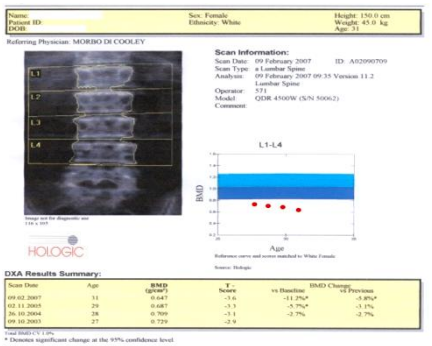
- 'Thalassemologist'
- Cardiologist
- Pneumologist
- Endocrinologist
  - Diabetologist
  - Reproduction gynecologist
  - Andrologist
- Hepatologist
- Radiologist
- Transplant specialist
- Psychiatrist/psychologist
- Social worker

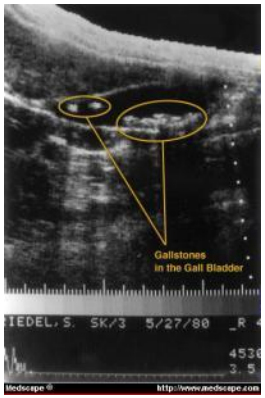
HOW

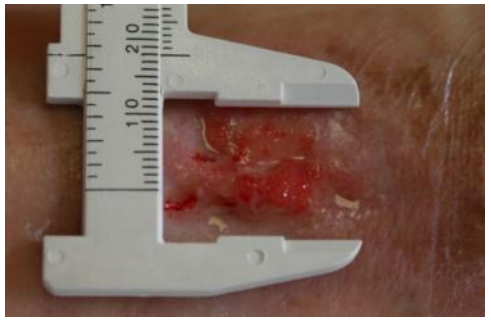
- Doctor in charge: coordinating the team
- Doctor: sensitizing each specialist
- Nurse: organizing

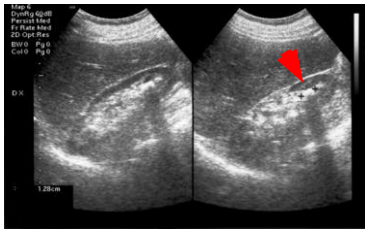
CLINICAL PROBLEMS THAT REQUIRE SPECIFIC CARE

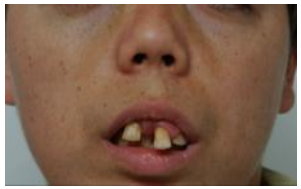
- Osteoporosis
- Hypoparathyroidism
- Infertility
- Biliary stones
- Kidney stones
- Leg ulcers
- Bone deaformities in adult patients









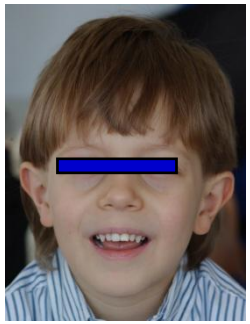


SPECIAL CARE AT SPECIAL MOMENTS

- Presentation
- Start of transfusion
- Start of chelation
- Non compliance
- Onset of a serious complications
- Conventional treatment versus SCT

THALASSEMIA AT PRESENTATION

- Set an accurate diagnosis
  - thal. genotype
  - RBC genotype
  - HLA genotype



- Support the parents
  - information
  - serial conversations
  - genetic counselling

STEM CELLS TRANSPLANTATION

1 For each patient:

- Information
- HLA typing of siblings (+relatives if parents have the same ethnic background)
- Set level of risk (grouping)

2 For patient with HLA identical relative:

- Detailed information (patient, donor, parents)

2aInformed consent=yes

- Make low risk SCT available

2b Informed consent=no

- Wait and update

3 For patient with no HLA identical relative:

- Consider BMT in special cases

4 For perspective couples at risk of thalassemia:

- Information
- Save cord blood

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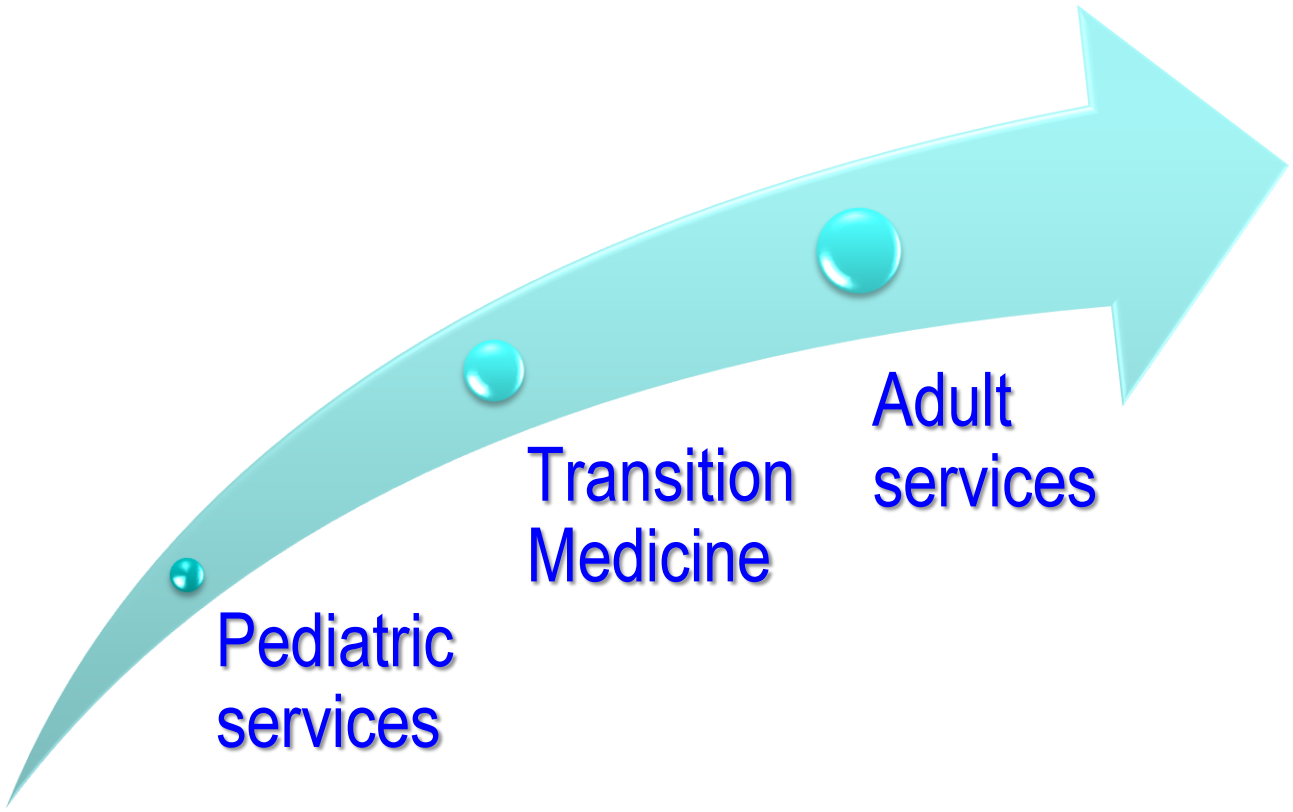
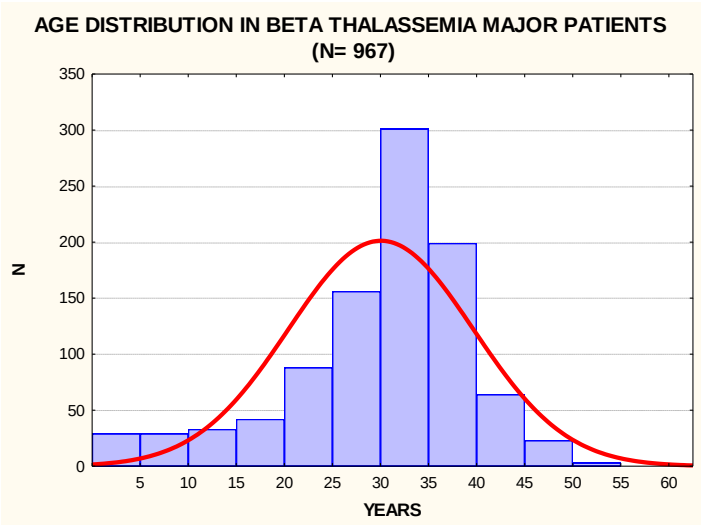
- Psychosocial support integrated in the global management
- Multidisciplinary team
- Non invasive diagnostic tecniques

Haemoglobinopathies Centre



AGE OF PATIENTS



CLINICAL PROBLEMS

- Pediatrics
- Adult Medicine

TYPE OF UNIT

- Blood Bank
- Pediatric Unit
- Hematology Unit
- General Medicine Unit

DEDICATED HAEMOGLOBINOPATHIES CENTRE


婚姻登记
MARRIAGE REGISTRATION


兴宁区
兴宁区
兴宁区

若夫妇双方均为同一类型的地中海贫血（以下简称
次怀孕，其子女有1/4的机会为重型地贫患者。婚检可以提前发现，怀孕
时要做产前筛查、产前诊断，才能有把握生下健康的孩子。

正常人和地贫基因携带者结婚，生育后代有两种结果：

- 1、后代是地贫基因携带者的几率为50%

- 2、后代正常的几率为50%

地贫基因携带者之间结婚，生育后代有三种结果：

- 1、后代正常的几率为25%

- 2、后代是地贫基因携带者的几率为50%

- 3、后代是重症地贫患儿的几率为25%
（红色显示就是需要重点干预的后代）

Thalassemia Research Center

MAHIDOL UNIVERSITY
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2nd International Conference on Thalassemia & Birth Defects

October 11, 2014, Nanning, China

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Nanning Declaration

Nanning Declaration

Prevention and Control of Thalassemia and Birth Defects

NANNING DECLARATION

The 2nd International Conference on Thalassemia & Birth Defects, jointly organized by the National Health and Family Planning Commission (NHFPC) of the People's Republic of China and the Thalassemia International Federation (TIF) has been taking place on 11 October 2014 in Nanning, China. 100 government officials and experts from China, Cyprus, Indonesia and Thailand, together with conference

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Nanning Declaration

October, 11, 2014, Nanning, China

1. Government-led and shared responsibilities
2. Raising awareness and encouraging social participation
3. Refocusing approaches to improve prevention
4. Extending research and health-promotion services
5. Collaborating actively and broadening exchange

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World Health Organization
Regional Office for South-East Asia

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Thematic Group Meeting on Management & Prevention of Thalassemia
7 to 8 August 2014, New Delhi, India

The objectives of the meeting are as follows:

- To learn from the experiences of the countries
- To review draft regional guidelines on management and prevention of Thalassemia

Thematic Group Meeting on Management & Prevention of Thalassemia

The Lalit Hotel
Barakhamba Avenue, Connaught Place,
New Delhi, India

August 7-8, 2014