Global Initiatives in improving quality of healthcare in thalassaemia

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Ethics and Quality of Care

• Patients experience inequalities in their management across the world for many known reasons.

• At stake: medical errors, waiting lists, poor access to appropriate care, staff shortages, inadequate information and informed consent………………

  Breslin JM et al. BMC Med Ethics 2005

• These are experienced in the “developed world” as well in struggling economies

• Quality of care is an ethical principle

• Equity of care is a universal principle: The WHO Constitution (1946) envisages “…the highest attainable standard of health as a fundamental right of every human being.”

• Both quality and equity require planning at a central level.
Definition of quality of services

• The US Institute of Medicine (IOM) has defined the quality of health care as 'the degree to which health services for individuals and populations increase the likelihood of desired health outcomes and are consistent with current professional knowledge'.

• Indicators of quality:
  1. Safety – avoiding harm (Primum non nocere)
  2. Effectiveness – avoiding overuse and misuse of care
  3. Patient centred care, that is unique to each patient’s needs
  4. Timely – reducing waiting lists and harmful delays
  5. Efficient – avoiding waste of supplies and equipment
  6. Equitable – without variation according to characteristics (like income, race, refugee status etc)
The case of thalassaemia: disease specific standards

And other haemoglobin disorders
Aims of managing haemoglobin disorders

• Long survival and good quality of life: Optimum treatment is required
• No treatment means early death in childhood
• Less treatment means poor quality of life and premature death – this is the commonest picture on a global scale
Expertise is required in several areas

- **Epidemiology** – the often unknown burden of the disease, societal/professional ignorance, often leads to poor health planning
- **Diagnosis** – need for specialised laboratories: delays/inaccuracies
- **Life long and complex clinical care** – with psychosocial consequences
- **Multi-disciplinary collaborative care** – multi-organ involvement
- **Adequacy and quality of blood**
- **Uninterrupted supply of drugs and services**
**β-Thalassaemia major** (regularly transfused)

- Hypothyroidism
- Hypoparathyroidism
- Cardiac siderosis
- Left-sided heart failure
- Hepatic failure
- Viral hepatitis
- Diabetes mellitus
- Hypogonadism
- Osteoporosis

**Non-transfusion-dependent thalassaemias (NTDT)**

- Silent cerebral ischaemia
- Right-sided heart failure
- Extramedullary haemopoietic pseudotumours
- Hepatic fibrosis, cirrhosis, and cancer
- Gallstones
- Splenomegaly
- Osteoporosis
- Venous thrombosis
- Leg ulcers

Need for regular monitoring of patients
Prevention of complications

• Regular blood tests – haematology, biochemistry, serology
• Imaging – radiology, MRI (quantitating iron overload)
• Echocardiography - ventricular function, pulmonary hypertension
• Transcranial Doppler - in SCD from 2-16 years
• Ultrasonography – annual abdominal or as indicated
• Biopsies - as required
• Pulmonary function tests – mainly in SCD
• Ophthalmology and audiometry - annual
Patient expectations and concerns

Are these a primary issue in planning services?
Patient preferences – from a European survey

A coordinated team with an experienced doctor in charge?

- Not necessary: 21.82%
- Little use: 1.95%
- Useful: 3.59%
- Essential: 0%
- No answer: 72.64%

Enerca White Book 2012
Patient expectations – from a European survey

Enerca White Book 2012
Patient concerns – from a European survey
A patient’s view: what is essential?

- Patients with thalassaemia must feel that the unit is their own place,
- The medical staff have the patients’ best interests as top priority.
- Long-term management implies collaboration of the patient and the family
- A well-organised thalassaemia unit and its team must ensure continuous, appropriate treatment and a long and productive life for the patient with thalassaemia.
- Regular Peer review evaluation

George Constantinou – thalassaemia major (aged 60 years) 2010
A patient’s view: “a centre should have close links with patient support associations” (EU Task Force standard)

• To help the doctors understand their patients
• To help with educating the patients about their condition.
• To help the patients cope with their condition
• To support new families
• To be used as advocates to policy makers at local / national level

George Constantinou
Talking with Patients

- Improves coping and quality of life
- Helps to gain independence
- Friendly communication to patients on individual basis
- No interruptions, privacy, confidentiality
- Listen, pay attention and respond!
- Need to enhance clinician’s skills
Patient-friendly services/holistic care

- Staff willing to spend time and to listen
- Privacy and confidentiality to discuss sexuality, contraception, puberty, diet, risky behaviour, school problems, etc.
- Convenient appointment times – consider school and work
- Help the patient/parent to express feelings
- Maintain realistic hope
- Facilitate normal lifestyle and encourage self-esteem
Monitor Patient Reported Outcomes

• An outcome measure recognises and notes a change in the health of a person or a group… WHO
• “Shifting focus from volume of services to the value created for patients”
  Porter EM NEJM 2010; 363: 2477-81
• Patient experience and reported health status
• Adverse events
• Mortality
• Effectiveness of care
• Timeliness
Upgrading quality

This should be a national policy as well as the concern of each centre
Haemoglobinopathy centres across the world

• Many have this label – but are their services up to good clinical practice?
• They vary considerably: in their expertise, the number of patients that they serve, the support that they receive from health authorities
• Do they have accreditation and quality control – national or international?
• Are they centres of reference supporting peripheral centres in their area?
• The Thalassaemia International Federation proposes to address this issue
Standards of quality that can be followed

• Centres of excellence supporting peripheral centres: there is disparity between centres and so networking is recommended. This has found practical expression in haematology through the creation of the Eurobloodnet consortium.

• International standards for centres such as the JCI Ambulatory care standards

• Clinical care guidelines: based on expert evaluation of evidence (grading evidence based quality), with review from other experts (consensus)

• Information to the patient and informed consent from the patient the partnership model

• Equal access for all patients: no substandard care. Access requires ‘universal health coverage’—especially in hereditary and chronic conditions.

Accreditation of centres – evaluating services
The Network model

Centre of excellence
Acting as a Reference Centre

Blood Bank and laboratory support

Peripheral centre in a local hospital
Day care centre
Primary care centre
GP based
So what is a centre of excellence?

- One that follows **standards** that are acknowledged by experts in the field as ensuring:
  
  - Safe and equitable care
    - Following evidence based guidelines
  
  - Effective care
    - Keeping in mind outcomes
  
  - Efficient care
    - Based on good governance
  
  - Timely care
    - Considering patient needs above service needs
  
  **Patient centred care**
Standards that are proposed by TIF to be adopted

1. General standards for ambulatory care:
   - **Governance** – hierarchy, manpower planning, advocacy to health authorities, policy decisions based on data, ethical practices in all aspects of administration, *patients’ rights*
   - **Patient safety** – patient identification, haemovigilance, pharmacovigilance, environmental planning, disposal of hazards, fire safety, water quality
   - **Access to care** – avoid all barriers e.g. distance, language, income, ethnicity, race
   - **Partnership** relationship with patients
   - **Information management** – patient records, confidentiality, protection against loss.
Standards that are proposed by TIF to be adopted

2. European Committee on rare disease (EUCERD) recommendation:
   - The capacity to provide expert diagnosis
   - Expert case management with a multidisciplinary approach
   - Maintain a patient registry
   - Auditing and control mechanisms
   - A sufficient number of patients to maintain staff experience (? At least 50 regularly followed in one centre)
   - Partnership model in patient relationship
   - Contribution to research
   - Networking with other centres
   - Close links with patient organisations
Clinical standards that are proposed by TIF to be adopted

3. Follow an acknowledged clinical guideline or standard
   • Guidelines for the management of non transfusion dependent thalassaemia (NTDT) TIF 2nd ed (2017)
   • Standards for the clinical care of children and adults with thalassaemia in the UK 3rd ed (2016)
   • Many other national standards
Standards to provide expert diagnosis

Expert diagnosis greatly depends on laboratory confirmation:

• Laboratory standards support and confirm any clinical diagnosis

• Quality of laboratory performance is governed by standards

• Quality management of laboratories depends on **Internal quality control** as well as **External quality control**.
External quality assessment of medical laboratories

- The International Organization for Standardization (ISO), has developed quality systems to assess specific aspects of health services.
- A majority of laboratories rely on International Quality Standards known as ISO/IEC/17025 for all types of testing and calibrating laboratories and more specifically ISO 15189 for medical laboratories.
External quality assessment (EQA)

- EQA measures the standard of results produced by each laboratory and its performance relative to others
- Allows inter-laboratory comparison of results
- Provides ‘state of the art’ assessment for laboratory practice, methods, instruments and reagents/kits
- Shares problems and best practice through education
Quality is of paramount importance in health laboratories.

• Laboratories practicing the principles of quality assurance generate relevant, reliable and cost-effective results.

• A quality system has the following five key elements:
  ❖ Organizational management and structure
  ❖ Documentation
  ❖ Monitoring and Evaluation
  ❖ Training
  ❖ Quality standards
The complexity of tests in haemoglobinopathy diagnosis

### Summary of screening techniques

<table>
<thead>
<tr>
<th>Standard laboratory investigations</th>
<th>Details</th>
</tr>
</thead>
<tbody>
<tr>
<td>Haematology Diagnostics</td>
<td>Red blood cell count or complete blood count. Reticulocyte count, MCV, MCH, RDW, which may be supported by microscopic description of red cell morphology. Low indices indicate a possible thalassaemia.</td>
</tr>
<tr>
<td>Hb quantification</td>
<td>Hb A₂ &amp; F: HPLC, capillary electrophoresis (CE). A raised Hb A₂ indicates a β-thalassaemia carrier. Hb F: Alkali denaturation, Hb F-cells: Kleihauer test</td>
</tr>
<tr>
<td>Hb pattern analysis</td>
<td>Electrophoretic methods: agarose gel, cellulose acetate, isoelectric focussing. HPLC, capillary electrophoresis (CE)</td>
</tr>
<tr>
<td>Confirmatory tests (e.g., Molecular analysis)</td>
<td>DNA analysis, Protein analysis - mass spectrometry</td>
</tr>
</tbody>
</table>
Screening Algorithm

If Hb level is low

If Hb level is normal

α-globin gene analysis for common mutation in the Mediterranean area:
-α3.8
-5nto
-αPolyA
-20.8
--MEDI

If no change or difference

Give Fe & repeat analysis + iron studies

Check Iron Status e.g., Zn Protoporphyrin

Silent β-thalassaemia trait

Δ + β thalassaemia trait

Characterisation of unidentified mutations by DGGE and direct sequencing of the β-globin gene

If no α-thalassaemia mutation is present

Further Investigations may be required – PCR or Mass Spectroscopy

Hb Variants:
Electrophoresis HPLC

Sickling positive

Other known variants

Hb S

Others

Hb C
Hb D
Hb E
Hb O Arab
Hb Lepore

Hb F %

MCV (fl)

MC3 (fl)

HbA2 %

β-thalassaemia trait

δ-thalassaemia trait

β-thalassaemia trait

Normal

< 78

> 78

< 27

> 27

< 3.5

> 3.5

< 1.5

< 1.5

< 3.0

< 1.6

Varies 0-5%

β-thalassaemia trait

δ-thalassaemia trait

MCV (fl)

MC3 (fl)

HbA2 %

Hb F %

> 78

< 78

< 27
Organising labs for screening

Reference laboratory
Red cell counter, HPLC, Cap electrophoresis, molecular

Peripheral Hospital lab: Indices, A2, gel electrophoresis
Field lab
OF, DCIP
Primary care lab
OF, DCIP, indices
Standards of care

Are they known? Are they applied? Are they audited?

Who is responsible to do all this?
Haemoglobinopathy Centre Staffing

Adequate staff/patients ratio

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

WHO, Hereditary Diseases Program, 1994
Decision support

- 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.
The Electronic Health Record – important for quality services and research
Survival of Thalassaemia patients in centres of excellence compared to peripheral centres

Forni et al 2009
Am J Hematol

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.
Notes form EC regulation 765/2008: accreditation

- Accreditation is part of an overall system, including conformity assessment.
- The value of accreditation lies in the fact that it provides an authoritative statement of the technical competence of bodies.
- It is necessary to develop a comprehensive framework for accreditation.....which functions by reference to binding rules. *This helps to strengthen confidence*
- May be compulsory or voluntary
- If a MS does not have a national accrediting body, *it should have recourse to the accrediting body of another MS*
- The accreditation body should have financial and personnel resources to fulfil its tasks
Joint Commission International JCI Ambulatory care

- Patient safety (clear identification, effective communication, medications, reducing the risks of infection, accidents in the centre). Patient pathway.

- Continuity of care and referral procedures, transfer, follow up, staff duties and qualifications

- Patient and family rights supported (e.g. Informed about rights, reducing language and other barriers, informed consent for blood transfusion)

- All procedures available for monitoring (e.g. MRI, lab quality, screening for pain)

- Pain management protocol

- Patient and family education

- Quality improvement
Annual peer review accreditation and quality improvement

Review Team: Professionals working in the clinical area, trained in peer review process

- Lead Reviewer
- Medical Consultants
- Specialist Nurses
- Managers and Commissioners
- User Representatives
**TIF Accreditation (certification) programme**

- Planned in 2017 for **haemoglobinopathy centres**
- The European Haemophilia Consortium already (2013) has a certification programme for haemophilia centres “in the context of great disparities” among centres. Based on EC Regulation 765/2008 and treatment standards.
- TIF is developing its disease specific certification based on:
  * TIF clinical guidelines,
  * the EUCERD rare disease recommendations
  * JCI Ambulatory Care model.
- TIF is seeking ISQUA recognition
- Expert help of international authorities, not only on thalassaemia but also accreditation experts, has been gained by TIF.
Health Care Organization

- Political will
- Financial support
- Leadership
- Promotion of safe & high quality care
- Chronic care needs recognized as a priority by the health care system & community.
- The support of patient driven organisations
Conclusion

Quality care assures quality life
The goal is quality centres across the world

- Multidisciplinary support, treatment protocols and guidelines
- Specialised laboratory support for diagnosis
- High level expertise/experience of staff
- Well maintained patient records
- Support to physicians and other health professionals
- Advice and guidance to policy makers

- Research and clinical trials
- Networking with national and international centres
- Networking with various patients’ associations
- Disease data surveillance
- Psychosocial support
Thank you for your attention