



# Clinical Practice Guidelines & Access to Highly Specialized Care

**New CPGs/CDTs produced by the  
ERN-EuroBloodNet**

**Example on Sickle Cell Disease**

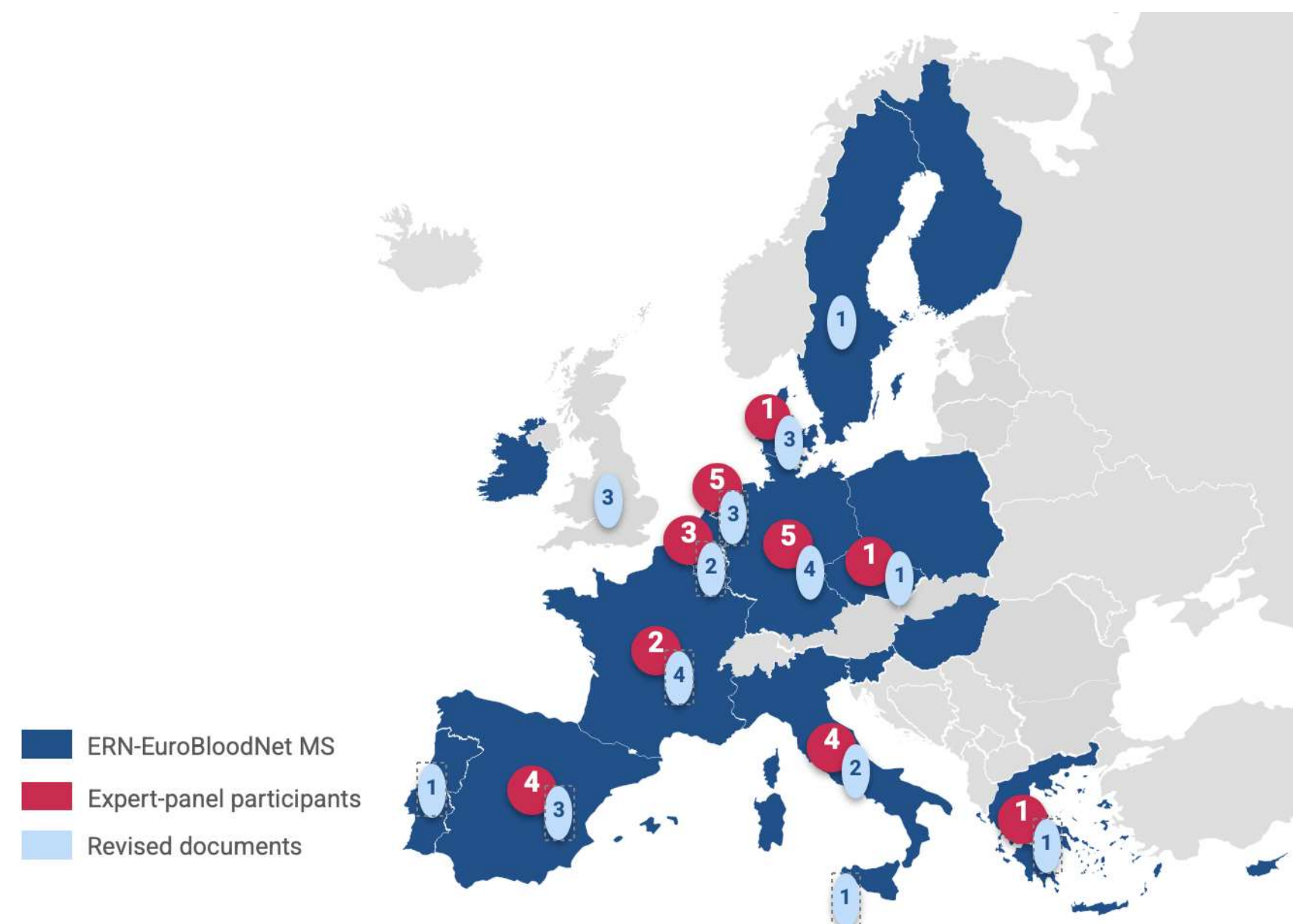
Béatrice GULBIS

Hôpital Universitaire de Bruxelles – ULB, Brussels

Co-coordinator ERN-EuroBloodNet



**Systematic comparative analysis of  
national SCD recommendations  
in Europe**





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**Systematic comparative analysis of national SCD recommendations in Europe**

**TRANSLATION IN ENGLISH - REPOSITORY**





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## Systematic comparative analysis of national SCD recommendations in Europe

1. Diagnosis at birth
2. Prevention and management of complications
  - a. Standard follow-up
  - b. ACS
  - c. VOC
  - d. Stroke + TCD/MRI
  - e. Priapism
  - f. Kidney
  - g. Splenectomy/Splenic sequestration
  - h. Cardiomyopathy
  - i. Pregnancy
  - j. Pre-surgery
3. Treatments including prevention
  - a. Vaccination/antibioprophylaxis
  - b. Analgesia
  - c. Fever treatment
  - d. Hydroxyurea
  - e. Blood transfusion
  - f. HSCT
4. Transition





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# National Recommendations on Acute Chest Syndrome (ACS)

A. Collado & M. Rab

Prioritized question: How is ACS diagnosed?	Level of evidence	1	2	3	5	6	9	16	10	11	12	13	25
Recommendation		BE	CZ	DE	DE	DK	ES	FR	GR	IT	NL	SE	UK
The diagnosis of ACS is made on the basis of new pulmonary infiltrate on the thorax image with clinical symptoms	C3	NA	NA	Yes	Yes	Yes	Yes	Yes	NA	Yes	Yes (C3)	Yes	Yes
Prioritized question: How is ACS treated?	Level of evidence												
Recommendation 1: Mild ACS should be treated with simple (top-up) transfusion when anemia is present	A3	NA	Yes	Yes	Yes	Yes	Yes	Yes	NA	Yes	Yes (A3)	Yes	Yes
Recommendation 2: Severe ACS should be treated with exchange transfusion	A3	NA	NA	Yes	Yes	Yes	Yes	Yes	NA	Yes	Yes (A3)	Yes	Yes
Recommendation 3: Oxygen should be provided in case of hypoxia	A3	NA	NA	Yes	Yes	Yes	Yes	Yes	NA	Yes	Yes (A3)	Yes	NA
Recommendation 4: Adequate hydration, but limit fluid administration to avoid overhydration	A3	NA	NA	Yes	Yes	Yes	Yes	Yes	NA	Yes	Yes (A3)	Yes	NA
Recommendation 5: Start broad spectrum antibiotics irrespective of culture results	A3	NA	NA	Yes	Yes	Yes	Yes	Yes	NA	Yes(C)	Yes (A3)	Yes	Yes
Recommendation 6: Start analgesics for adequate painrelief	C	NA	NA	NA	NA	Yes	Yes	Yes	NA	Yes (C)	Yes (A3)	Yes	NA
Recommendation 7: Start incentive spirometry	A	NA	NA	Yes	NA	NA	Yes	Yes	NA	Yes(A)	NA	Yes	Yes
Recommendation 8: Start thrombosis prophylaxis		NA	NA	NA	NA	NA	Yes	Yes	NA	NA	NA	NA	NA
Prioritized question: How can ACS be prevented?	Level of evidence												
Recommendation 1: initiate preventive incentive spirometry upon admission	A	NA	NA	NA	NA	NA	Yes	Yes	NA	Yes(A)	NA	NA	Yes
Recommendation 2: Hydroxyurea is recommended as prevention in case of severe or recurrent	A2	NA	NA	Yes	Yes	NA	Yes	Yes	NA	Yes(A)	Yes (A2)	Yes	Yes
Recommendation 3: Bone marrow transplant can be considered as prevention for ACS	C3	NA	NA	Yes	NA	NA	Yes	NA	NA	Yes	Yes (C3)	Yes	NA
Recommendation 4: Chronic transfusion therapy can be considered (if HU is not effective)		NA	NA	NA	NA	NA	Yes	Yes	NA	NA	Yes	NA	Yes

## Missing, Evidence limited or inconsistent

- National recommendation (BE, CZ, GR)
- Beta2-mimetics use during ACS (pediatric/adult)
- Optimal use of incentive spirometry during ACS in adults
- Thrombosis prophylaxis use for prevention



# Systematic comparative analysis of national SCD recommendations

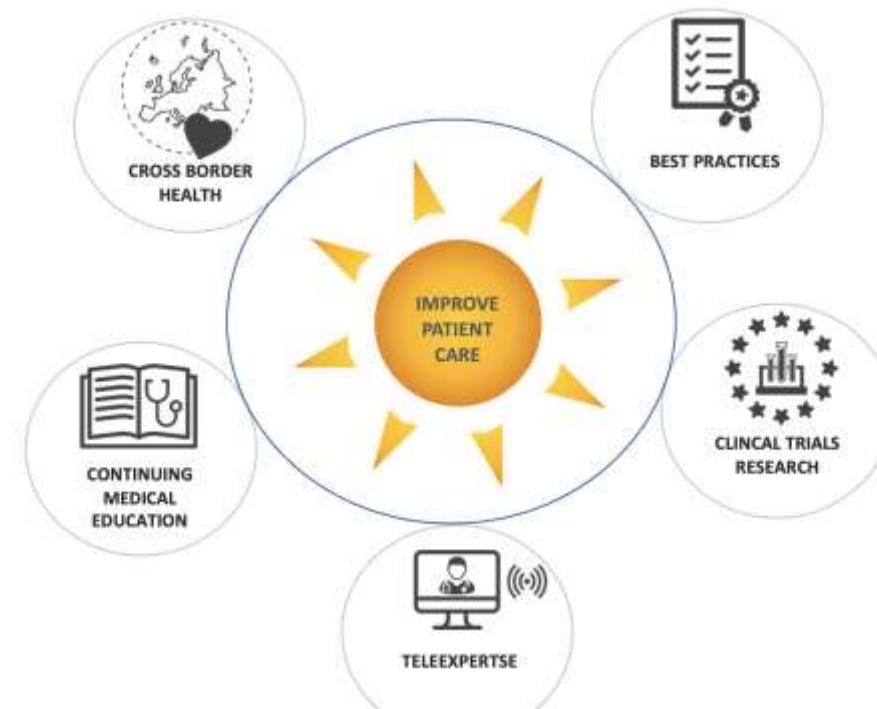
## Missing recommendations

- Improve inequity of care
- Integration of ERNs into national care systems



## Discrepancies, limited evidence

- **Research projects** : harmonized and evidence-based clinical policies
- **Place of registries** (16/40 session on registries & clinical outcome research)







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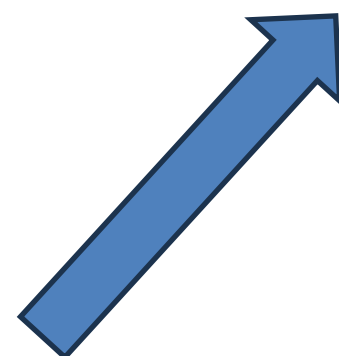
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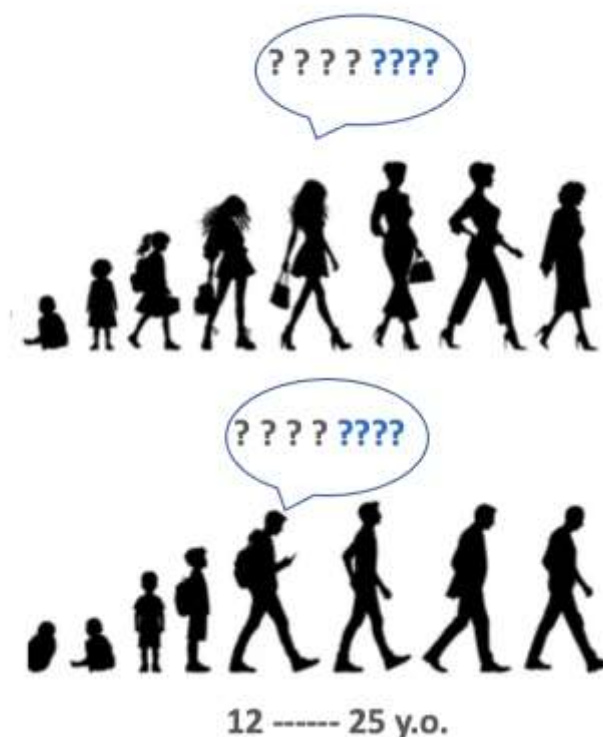
# National Recommendations on Transition (Pediatrics to Adulthood)

*H. Cario, R. Colombatti, M. de Montalembert*



All paediatricians ...

+ rare disease



Transition



Transfer



Follow-up





# National Recommendations on Transition Pediatric to Adult

*H. Cario, R. Colombatti, M. de Montalembert*

## Missing, Evidence limited

- Only few documents address the topic of transition and even less give detailed recommendations.
- Due to the complexity of the topic, all recommendations are expert consensus and not based on high-grade evidence

	Max level of evidence	17 DE	8 ES	15 FR	11 IT	25 UK
<b>Prioritized question 1: General prerequisites ?</b>						
<i>Recommendation 1:</i> a structured program for the transition exists	C	yes	NA	yes	yes [C]	yes
<i>Recommendation 2 :</i> An individual transition plan is recommended (clinical, developmental, and educational status).	NA	yes	yes	NA	NA	NA
<i>Recommendation 3:</i> Transition process involves patients, caregivers, pediatric and adult hematologists, and member of the psychosocial team.	C	yes	yes	NA	yes [C]	NA
<b>Prioritized question 2: When start transition planning and preparation ?</b>						
<i>Recommendation 1:</i> Transition planning and preparation start around 13 years/ old/ in early puberty.	C	yes	yes	yes	yes	yes [C]
<b>Prioritized question 3: How patient's readiness for transition is assessed ?</b>						
<i>Recommendation 1:</i> A suitable instrument is available		yes		yes	yes	yes [C]
<i>Recommendation 2:</i> Before transfer, at age 15-16 education and preparation is assessed.	C	NA	NA	NA	yes [C]	yes [C]
<b>Prioritized question 4: How is transfer of information from pediatric to adult departments provided ?</b>						
<i>Recommendation 1:</i> A structured transition report (medical, para-medical and socio-familial) is mandatory	NA	yes	NA	yes	yes	NA

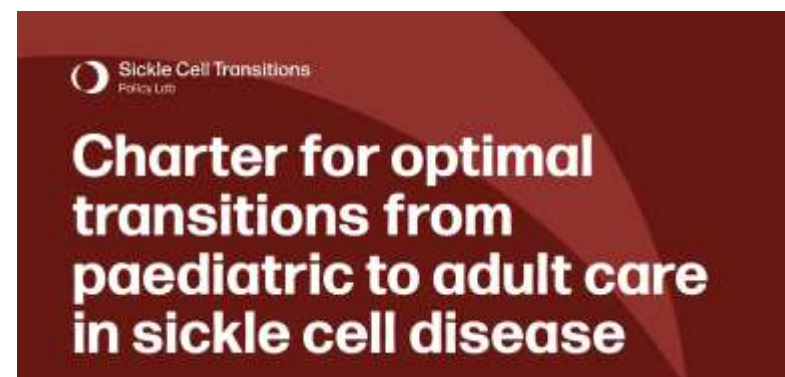


# Systematic comparative analysis of national SCD recommendations

Transition topic : integration part of other initiatives

- Conclusions highlight the necessity of a multidisciplinary team/stakeholders to address the question

- ERN-EuroBloodNet initiatives
  - ASCAT patients' education sessions



## EC/DG Santé/ERNs initiatives

- WG on transition – Survey

### Barriers to transition care

Barriers to provide transition care for AYA with rare diseases

1= no barrier, 5= huge barrier

	1	2	3	4	5
Lack of communication to patients/caregivers	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Lack of education to patients/caregivers	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Patient's attachment to the referring paediatrician	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Funding concerns for patients	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Lack of reimbursement for HCPs	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Lack of communication between HCPs	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Lack of education of HCPs	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Lack of continuity of care	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Over/under involvement of caregivers	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Access to specialised care	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Medical complexity and heterogeneity of patients	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Lack of expertise for some ultra rare diseases in adult departments	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Fragmentation of care services	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Fragmentation of social services	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Discrepancies between paediatric and adult care services	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Lack of local policy or guideline	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>



# Thank you!

