



European Group for Blood and Marrow Transplantation

Dietger Niederwieser
President of the EBMT
President of WBMT
Leipzig, Germany

Problems associated with international academic clinical studies

- Scientific evaluation
- Ethical Committee for each country
- National approval from competent authorities
- Radiation commission
- Translation of the protocol in different languages
- Different requirements in each country and different definitions
- Different type of studies (treatment optimizing studies; ONIS studies)
- Insurance for each country billed to the Sponsor
- Directive specific issues:
 - Labelling
 - SUSAR reporting
- Funding
- Stem cells are considered drugs
- Reporting should be part of the transplant

Ideal world for academic international studies

- Scientific evaluation
- Ethical Committee for each country
- National approval from competent authorities (CA)
- Radiation commission (Germany)
- Translation of the protocol in different languages

Approach: One stop shop or at least combination of national authorities, scientific evaluation and Radiation commission (if applicable). Translation not necessary anymore.

Solution: 1) one country processes the protocol for all others
(+ easy; - politically not immediately feasible, language, a.o.)
or
2) Central institution composed by competent authorities representative of each country. Model EMEA (CAT; "clinical trials facilitation group").
Allocation: DG Sanco, DG Research, EMEA but independent with a board representing scientific, authorities and ethics (+ all CA involved; one language; - expenses)

Ideal world for academic international studies

- Different type of studies (treatment optimizing studies; ONIS studies).

Approach: Distinguish between studies, but not on quality

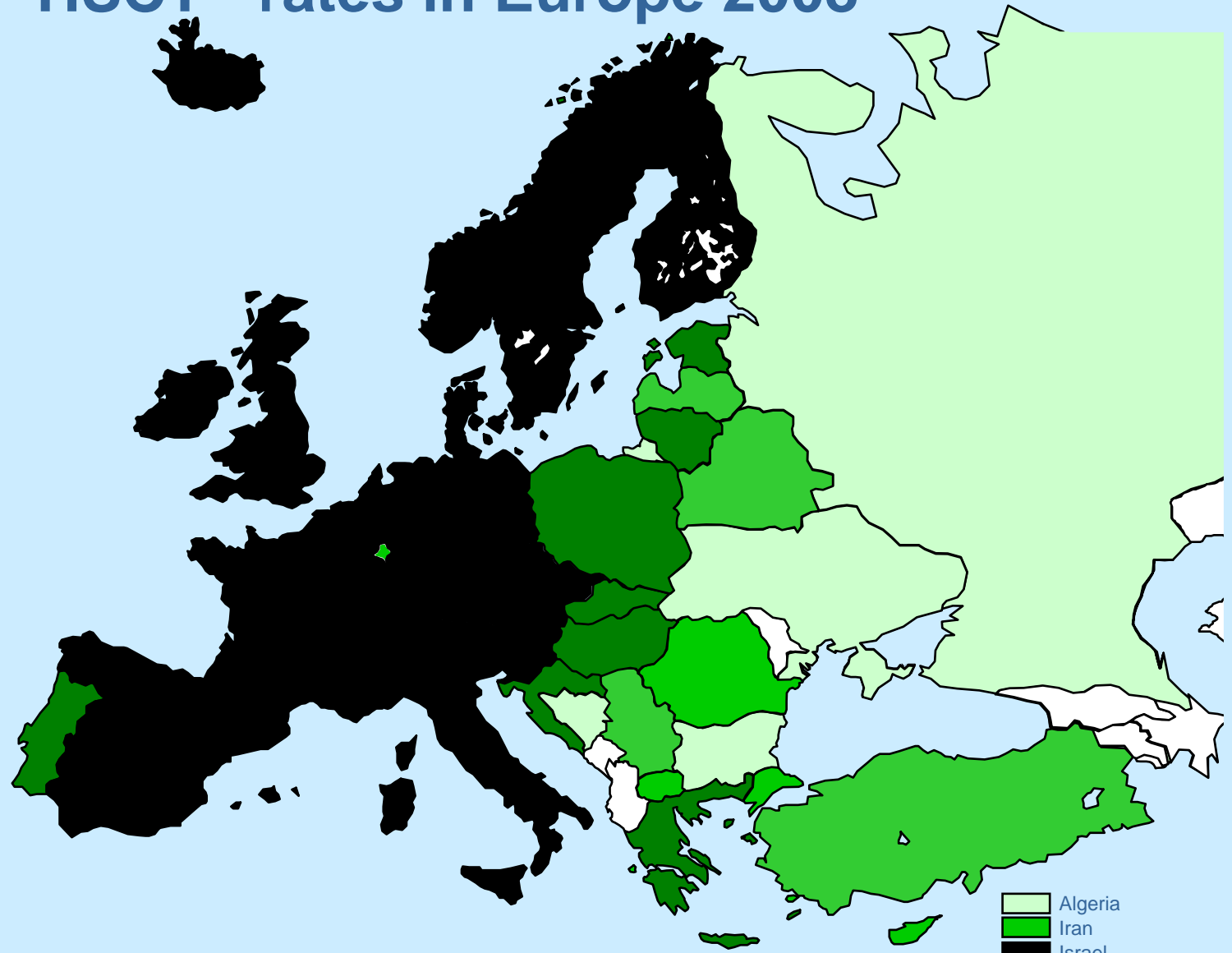
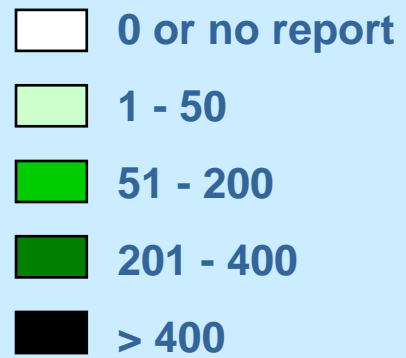
Solution: Treatment optimizing studies (reduced intensity conditioning vs. conventional stem cell transplantation).

ONIS (observational non interventional studies).

HSCT - rates in Europe 2008

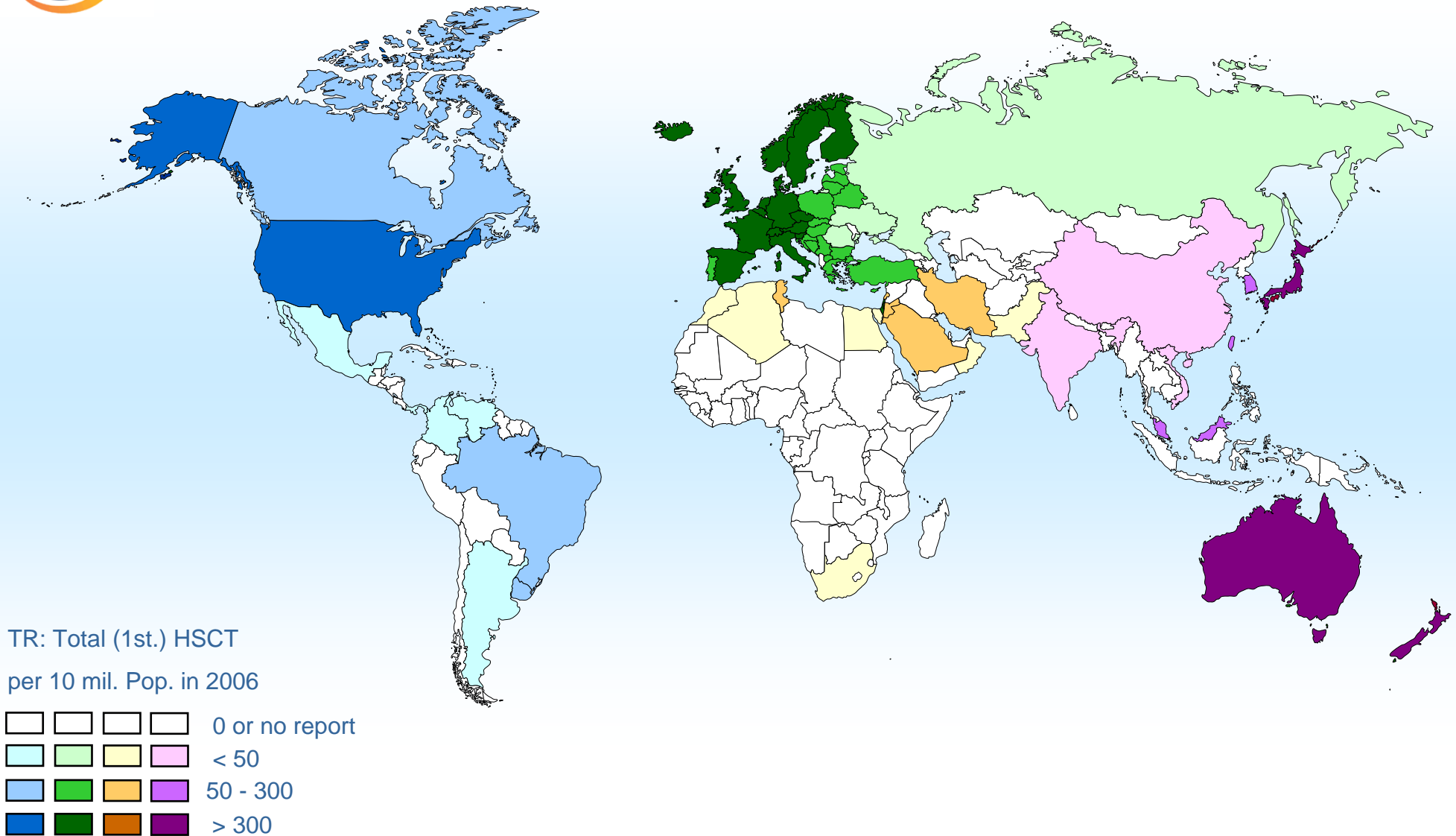
Total transplants (all)

per 10 million 2008





Transplant Rates in the 4 Continental regions: all HSCT



Ideal world for academic international studies

- Insurance

Approach: European Insurance.

Solution: Coverage should be provided by public health care system

Justification: Academic clinical studies are advancing the field of medicine and are part of patient treatment

Ideal world for academic international studies

■ Funding

- Approach:
- Italian approach (5% of marketing budget for academic clinical studies).
 - European approach (DG Research or/and DG Sanco)
 - Country of PI covers all countries

Solution: Sufficient funding

Ideal world for academic international studies

- EU-Directive specific problems

Approach: Define labelling
simplify SUSAR reporting

Solution:

- Use common sense
- Evaluate changes regularly
- Provide favourable environment for academic clinical studies

Ideal world for academic international studies

- Stem cells are considered drugs

Approach: Define grafts as transplants

Ideal world for academic international studies

- Reporting of expensive procedures are required
(55.000 x 75.000€= 4125 x 10⁶€)

Approach: Support or establish registry

Solution: Financial compensation of reporting