



Health Indicators for Rare Diseases

Framework

Rare Diseases Task Force

12 March 2008 / Paris / France



Commission Communication
and
Health Indicators
for Rare Diseases

Problem: diagnosis

Given the large number of tests and the need to design and validate a specific set of diagnostic assays for each, no single country can be self-sufficient in the provision of testing. This results in exchange of patient

Indicators of special interest to rare diseases include mortality age, survival rate from diagnosis, duration from first symptoms to diagnosis, related morbidity, and health expectancies.

there is little agreement on which diseases justify a systematic screening approach according to WHO criteria. The organisation of population or targeted screening is conditioned by many issues such as the quality and reliability of the test, the availability of an effective treatment/intervention for those screened, the prevalence of the disease and its severity and the choice/value that society attributes to the screening.

Indicators to monitor the situation...

Development of health indicators in the field of RD: The development of health indicators is needed to monitor the situation of affected persons in the EU and its evolution. Compilation of existing sources of data should be encouraged, especially those already funded at EU level. A set of realistic and meaningful indicators should be defined in the area of orphan drugs availability and accessibility, in the area of centres of expertise/reference, in the policy field at MS and EU levels.

Definition of health indicators

- Parameters used to evaluate
 - Health status
 - Impact of health policies
- Indicators have to be
 - Relevant to the question that is being posed
 - Reliable: same value if measures again
 - Useful to decision-makers
 - Valid: measure what they are meant to measure
 - Applicable
 - Feasible on a large scale



Main objectives of health Indicators

- Measure RD as public health issue
 - For visibility / advocacy
 - To identify targets of interventions
 - To allocate appropriate resources
- Enable surveillance of status and trends
 - Measure the impact of prevention / diagnosis / screening / treatment
 - Identify etiological and modifying factors
 - Analyse geographical differences and changes over time
 - Document influence of health policy measures
 - Guide new research initiatives
- Provide efficient and consistent reporting mechanisms



Legal basis for health indicators

- EC aim is to produce comparable information of the health status of populations and health systems
- Legal basis
 - in the health strategy plan 2008-2013
 - Eurostat
 - Contract agreement with OECD
 - ECHI: System of Community Health Indicators
 - Project of Communication (2009) in the European Health Information, Knowledge and eHealth System

Past and ongoing projects



- ECHI: comprehensive list of 400 indicators with short list of 80
- ECHIM
- ICHI: International Compendium of Health Indicators
- ISARE: Health Indicators in the European Regions
- EUPHIX: European Public Health Information, Knowledge and Data management System
- EUROTHINE: Tackling Health Inequalities in Europe
- EHEMU: European Health Expectancy Monitoring Unit



Potential sources of data

- Registries
- Death certificates
- Hospital discharge charts
- Surveillance systems
- Patients' associations
- Ad Hoc surveys
- Litterature



Criteria for selection

- Must be a tool for health policy
- Must be related to a priority health problem
- Must allow comparisons across regions/
over time
- Data should be available
- Must be integrated into a more global perspective of the health information system



Health Indicators for RD

- The « classical » indicators hardly apply to the RD area, due to coding difficulties
- There is a need to define appropriate goals and to select potential indicators



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Selection process

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Goals

The header features a yellow background with several white stars of varying sizes scattered across it. On the right side, there is a partial view of the European Union flag, showing its characteristic blue field with twelve yellow stars arranged in a circle.

- Document the contribution of RD to morbidity and mortality
- Measure their socio-economic impact
- Document the availability of appropriate Health Services
- Document the state of art of R&D
- Monitor geographical differences in Europe
- Enable surveillance of status and trends over time

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Document contribution to morbidity and mortality

- Prevalence (globally and by disease)
- Incidence
- Mortality rate (specific age groups)
- Survival rate
- Health expectancy (DALY, PYLL)
- Contribution to Hospital admissions
- Contribution to mental/physical / neurosensory disabilities
- Contribution to transplantation / dialysis....
- Perceived health (QoL)
- Functional health

Measure their socio-economic impact

- Impact on families (economic, social, psychological)
- Annual budget to cover orphan drugs
- Contribution of consanguinity

Availability of appropriate health Services

- Availability of genetic tests for RD
 - by certified laboratories
 - by accredited laboratories
 - by laboratories participating in EQA schemes
- Number of diseases for which there is a biological test
- Number of diseases for which neonatal screening is in place
- Impact of prenatal diagnosis on birth prevalence
- Identified expert clinics

Availability of appropriate health Services

- Age at diagnosis
- Proportion of patients at home or in institutions
- Availability of orphan drugs among those with EMEA approval
- Number of Patients' organizations and of diseases covered
- Availability of Help-lines for RD



Information, research, technology development

- Number of RD with a specific code in ICD
- New Orphan products approved by the EMEA
- Call for proposals for research into RD
- RD for which good practice guidelines are available
- RD for which there is a registry, geographical coverage
- RD for which there are on-going clinical trials

Equity, regional differences

EU initiatives

- Countries with specific funding processes and Plans for RD
 - In the field of research
 - In the field of information
 - In the field of clinical care
 - Centres of expertise
 - Orphan drugs
 - In the field of testing
- European reference networks for RD
- European registries
- EU Funding programs for research and public health in RD
- Courses, congresses and seminars in RD



Surveillance of status and trends

- RD for which a diagnostic test exists (genetic, biochemical, other)
- Laboratories accredited for genetic testing
- Neonatal screenings
- Prenatal diagnosis
- Diagnosis delay
- Perceived health (QoL)
- People at home or in institutions
- New Orphan products approved by EMEA
- % of marketed drugs among those with EMEA approval

Next step



- To be defined together
 - What should be done
 - Who should do it
 - How it should be done
 - What are the available resources