

Patient Registry Establishing and Maintenance **Issues at stake**



Tsveta Schyns, *PhD*

Founder and Secretary General
European Network for Research on
Alternating hemiplegia, ENRAH
Vienna, Austria

The point of view of a patient representative

- The experience with the European Alternating Hemiplegia of Childhood Patient Registry, funded by the FP6 grant *ENRAH for SMEs* 2005-2007
- The experience with the Registry of Children with Rare Neurological Paediatric Diseases with Paroxysmal episodes, co-funded by the PHP 2007 through the project nEUroped 2008- 2011
- Disease Registries, Rare Paediatric Chronic Long Life diseases

Establishing a Patient Registry

1: Network of expertise

- Involve:
 - Patients (Organisations) –disease burden, high expectations
 - Specialists with knowledge of the disease- expertise, dedication, ability to work in an international team
 - Researchers with interest of the disease- results sharing
 - Industry - low interest at an early stage of development
- Cover as many countries as possible
- One data collection centre per country - always an issue
- Set up a communication platform- ensure constant flow and exchange of information

Establishing a Patient Registry

2: Define the Purpose

- Set up a Committee
 - include representative of each data providing centre
 - include patient representatives
- Review the current stage of research and knowledge of the disease - external experts
- Translate into specific objectives or research questions to be addressed through the Registry

Establishing a Patient Registry

3: Registry Oversight

- Draw the Registry Charter
 - overall direction and operations
 - data access
 - publications
 - change of management
 - plan for the entire lifespan of a registry, including how and when the registry will end and any plans for transitioning the registry at that time

Establishing a Patient Registry

4: Data Content

- Questionnaire
 - accurate and complete
 - “too much or too little” dilemma
 - data cleaning

Design the data collection Form

- data structure
- data format

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5: Data Collection

- Comprehensive instructional materials
- Training of data collecting centres- limited clinical experience within individual centers
- Validation of diagnosis - at the centres
- Validation of Data entering the Registry –
Work Flow definition

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6: IT Product

- Use a ready product - customisation
- Develop the Product - quality
- Costs - a substantial part of the funding
- Quality - it can ruin your Registry

The need of a one free product easily customised for building up Web based disease Registries

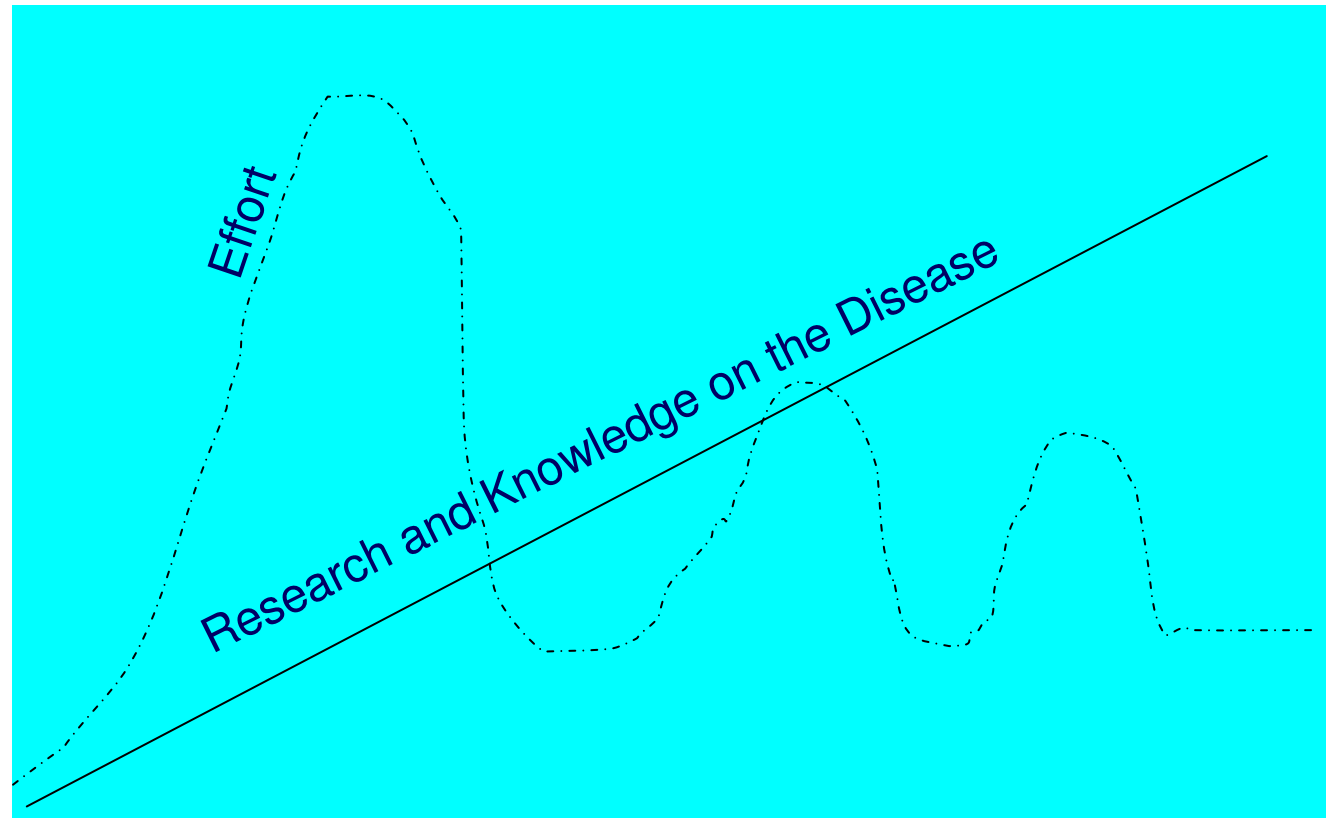
Establishing a Patient Registry

7: Funding

- The Framework Program: funding in a form of reimbursement of expenses- need a solid investment to start
- The Public Health Program : reimbursement of maximum 60% of costs- a challenge for non public organizations and for patient organisations to participate
- Other sources for an EU-based Registry of Patients?

Maintenance of the Registry

- Rare chronic diseases

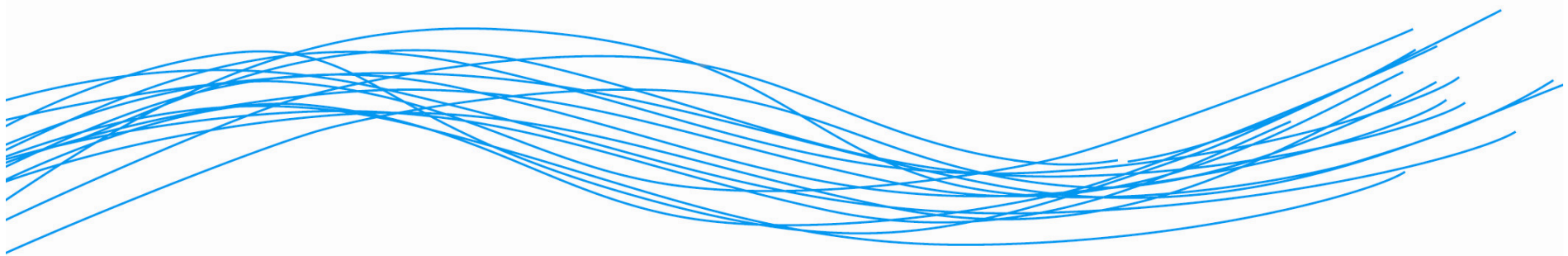


Patient Registry Maintenance

Main Issues:

- Usage - publish results of the data analysis; open the Registry for “outside users”
- Updating- patient & clinical information, research results
- Growth - include newly identified patients
- Adjust registry Design- project based
- Continuous Funding- project based; How to retain independency from the Sponsor/ Funding body ?

Patient Registry Establishing and Maintenance



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Thank you !