

Background

Rare diseases affect a small number of people relatively to the general population and specific issues are raised in relation to their rarity. In Europe, a disease is considered to be rare when it affects 1 person per 2000. A disease can be rare in one region, but common in another. There are also many common diseases whose variants are rare.

The field of rare diseases suffers from a deficit of medical and scientific knowledge. For a long time, awareness of rare diseases was scarce and until very recently there was no real research or public health policy concerning issues related to the field.

Researchers and other stakeholders are increasingly working through networks in order to share the results of their research and to advance more efficiently. Networks of registries and surveillance systems are effective instruments to improve knowledge on rare and uncommon diseases and conditions. Surveillance units have been widely used in the field of paediatrics and child health, as well as in other settings.

INoPSU – International Network of Paediatric Surveillance Units, was founded in 1998. It currently has 13 member units, from 12 countries (Australia, Belgium, Great Britain, Canada, Germany, Greece, Latvia, The Netherlands, New Zealand, Portugal, Ireland, Switzerland, Wales).



Aims

This presentation describes the impact of data collected by paediatric surveillance units (PSUs), both individually and collectively as members of INoPSU, on public health outcomes, clinical care and research, thus contributing to disseminate this methodology of active surveillance.

Methods

All PSUs use a similar surveillance method, based on the systematic, active, individual and voluntary reporting of cases.

PSUs provide a structured but flexible system for researchers to communicate with a large nation-wide set of practising paediatricians and other child health specialists as active surveillance agents, in order to collect data.

Each month (sometimes, each week) a report card listing the conditions under surveillance is sent; both negative and positive reports are collected. Positive reports are followed by a specific data collection form. Either postal mail, e-mail or the Internet are used for case reporting and data collection. Anonymity and confidentiality of data are required. The use of additional sources of cases is strongly encouraged.

The research teams are responsible for data management and analysis, and for the dissemination of the study results. PSUs may provide assistance on those tasks.

PSUs are run and funded by paediatric scientific societies and/or Public Health Authorities and competitive research grants. The use of the PSUs services may be provided pro bono or subject to a fee.

INoPSU celebrates a biennial conference of PSUs and edits a biannual newsletter.

Results

Since 1998, the network has facilitated surveillance of over 250 rare paediatric conditions, including over 70 rare infections.

The surveillance projects have enabled researchers to:

- monitor public health interventions (e.g., anaphylaxis following immunisation),
- inform the development of new screening policy (e.g., invasive group B Streptococcal disease),
- elucidate geographical differences (e.g., haemolytic uraemic syndrome),
- describe the epidemiology and features of child mental health disorders and other childhood disabilities (e.g., eating disorders - anorexia nervosa),
- facilitate molecular epidemiological studies and establishment of international registries (e.g., Rett syndrome),
- guide paediatric clinical practice policy (e.g., Vitamin K deficiency bleeding),
- characterize the epidemiology of emerging/re-emerging diseases and conditions (e.g., progressive intellectual and neurological deterioration - PIND),
- identify and quantify product safety hazards (e.g., adverse effects from complementary and alternative medicine),
- respond rapidly during outbreaks/epidemics (H1N1 Influenza pandemic).

These projects had important impacts, such as:

- surveillance of acute flaccid paralysis (AFP) in Australia was central to the process of WHO certification of the Western Pacific region as polio-free,
- surveys on congenital rubella, subacute sclerosing panencephalitis, meningoencephalitis, AFP and other conditions have been crucial to monitoring the effectiveness and safety of immunisation programmes in different countries,
- childhood HIV surveillance led to changes in antenatal screening policy in Australia and UK,
- surveillance of neonatal herpes simplex virus suggests the need for an HSV-1/HSV-2 effective vaccine,
- surveillance of injuries related to the use of baby-walkers and to the use of seat-belt provided evidence for legislative action in Canada and Australia.

Dozens of papers have been published in peer-reviewed journals and many national and international guidelines and recommendations cite them.

Conclusions

Registries and research networks are effective instruments to improve knowledge on rare and uncommon diseases and conditions. International collaboration between PSUs sharing interests, definitions and methods further improves the effectiveness of research efforts.

What does this add to European paediatric research?

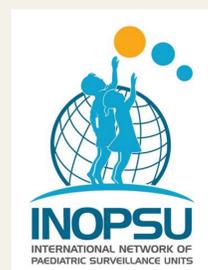
National PSUs and their International Network are widely available and have proven to be useful and effective partners for research on rare and uncommon diseases and conditions.

Selected Joint Papers

Grenier D, Lynn R, Zurynski Y on behalf of all national paediatric surveillance unit investigators. Public health impacts of the International Network of Paediatric Surveillance Units. *Paediatr Child Health*. 2009; 4(8):499-500.

Grenier D et al. Beyond Counting Numbers – Public Health Impact of Studies Conducted through National Paediatric Surveillance Units. *Arch Dis Child Jun* 2007; 92:527-533.

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