Spotlight on...

the International Network of Paediatric Surveillance Units (INoPSU)

Interview

Richard Lynn, INoPSU Liaison Officer and Scientific Coordinator for the British Paediatric Surveillance Unit

The British Paediatric Surveillance Unit (BPSU) has been facilitating epidemiological surveillance into rare paediatric conditions for over 20 years, including research into more than 70 rare paediatric diseases, the results of which have had a major impact on child health policy in the UK.

The methodology has been so successful that it has been adopted by other medical specialties within the UK and in paediatrics in a dozen countries around the world. In 1998 a network was formed, called the International Network of Paediatric Surveillance Units (INoPSU). This network covers a child population of over 50 million children and 10,000 paediatricians. Between the various members, epidemiological surveillance of nearly 200 rare paediatric conditions has been effectuated.

OrphaNews Europe spoke with Richard Lynn, INoPSU Liaison Officer and Scientific Coordinator for the British Paediatric Surveillance Unit:

OrphaNews Europe : How was the International Network of Paediatric Surveillance Units (INoPSU) formed and for what purpose?

Richard Lynn : The British Paediatric Surveillance Unit has existed since 1986. Quickly thereafter, other countries such as Germany, the Netherlands and Switzerland developed an interest in collecting cross national data on vitamin k deficiency bleeding and Haemophilus b
influenzae infections. The European initiative was the stimulus for the development of Australian, Malaysian and Canadian Paediatric Surveillance Units (PSUs). By the time of its launch in 1998, the International Network of Paediatric Surveillance Units INoPSU (www.inopsu.com) had 10 founding member countries covering a collective child population of over 48 million.

INoPSU's mission is to advance the knowledge of rare and uncommon childhood conditions that are high in disability, morbidity, mortality and economic cost to society, despite their low frequency, and to improve clinical practice and health policy by providing detailed epidemiological, clinical and outcomes data through surveillance on a national and international basis. INoPSU aims to enhance communication and collaboration between national PSUs and child health researchers worldwide by collecting simultaneous or sequential, standardised data to allow data comparison and to disseminate information on rare paediatric diseases to clinicians and the public alike. Our work on haemolytic uraemic syndrome and early onset eating disorders are two examples.

**OrphaNews Europe** : How many countries presently participate in the INoPSU? What is the relationship between national surveillance efforts and the INoPSU?

**Richard Lynn** : Currently there are 12 countries participating in the activities of INoPSU. Some countries (UK and Australia) have also extended active surveillance to several specialities other than paediatrics, such as ophthalmology, lung disease and psychiatry. The network communicates through its administrative centre based in the UK. The centre is kept informed of new studies under development. This allows for the sharing of study protocols and assists in connecting researchers in different countries working on simultaneous studies. In addition, the network shares publications of recent papers and other news and developments via email and through newsletters and the INoPSU website. Unit representatives and researchers come together every two years for a conference devoted to surveillance for rare paediatric conditions and to exchange information and discuss aspects of rare disease surveillance methodology and priority conditions for surveillance. The last such gathering was in Dublin Ireland last October where participants had the chance to hear Dr Ana Rath from Orphanet present on improving the quality of medical care for rare diseases.
OrphaNews Europe: How are diseases selected for inclusion in the INoPSU reports?

Richard Lynn: Conditions for surveillance are selected by the individual national PSUs according to national priorities and public health needs. Conditions are often chosen for surveillance when there are no other sources of information about the epidemiology and clinical management of rare conditions. INoPSU reports (www.inopsu.com/publications/index.html) often focus on conditions that have been studied in more than one country to highlight international comparisons in addition to reporting data that have been collected by individual PSUs to inform public health responses to disease management and prevention in that country over the past year.

OrphaNews Europe: How many rare diseases have been surveyed and why have the specific rare diseases included for monitoring been chosen?

Richard Lynn: Over 200 different rare paediatric conditions have been surveyed by the 12 countries within INoPSU. Many of the conditions have been studied by more than one national PSU, such as HIV, hyperbilirubinaemia, haemolytic uraemic syndrome and congenital rubella syndrome.

Each national PSU has a multidisciplinary Steering Committee that oversees the programme and reviews new study proposals. The conditions are chosen based on inclusion criteria such as, rarity, public health and scientific importance, and uniqueness. Some studies arise out of interest from individual paediatricians within a country, or are chosen because of national health policy need, for example, those relating to screening, hyperbilirubinaemia or a disease outbreak such pandemic influenza and variant-CJD (Creutzfeldt-Jakob disease). Some conditions are put forward by researchers and clinicians with support from patient groups.

OrphaNews Europe: How are data obtained for a specific condition? Is there a validation process for information?

Richard Lynn: Data are obtained through the process of active surveillance where paediatricians in each country contribute data on a number of rare conditions. At the moment, there are 10,000 paediatricians participating in INoPSU surveillance. Paediatricians receive a monthly report card, and are asked to indicate whether or not they cared for a child with any of the rare conditions listed on the report card. For each reported case, the paediatrician is asked to complete a peer-reviewed and ethically-approved detailed clinical questionnaire. The researcher then uses the data to validate whether the report meets the surveillance case definition criteria. The response rates for the monthly report card and questionnaire completion are very high, usually well over 80%. Alternate sources of data, e.g. laboratories or specialist clinicians, may also be used to validate data and to help maximise case ascertainment. In order to prevent inflating the incidence calculations, duplicate cases are excluded from the analysis. As the data are anonymous, obtained from the clinician and not from the family, and not used to develop long term registers, consent from individuals is not required in most instances.
**OrphaNews Europe**: Who do the data benefit and why? How are the data used?

**Richard Lynn**: The data collected have been used to benefit individuals who may develop rare diseases in the future, to inform clinicians and to improve clinical care of children with rare diseases, to inform public health policy and the public at large. Data from surveillance studies have been used to monitor incidence rates of vaccine-preventable diseases, effectiveness of vaccination programmes, and occurrence of vaccine-associated adverse effects. Data from INoPSU surveillance have resulted in the development of cohorts which enabled further study, e.g. identification of genetic mutations associated with Rett syndrome.

INoPSU is the ideal tool to gather timely data that impacts on health service planning, guides national legislation pertaining to injury prevention in children and young people, informs the development of screening programmes, and provides insights into genetic epidemiology. Surveillance units are able to respond quickly to recognition of emerging diseases of public health importance (for example v-CJD, pandemic influenza and melamine contamination of infant formula) by rapidly initiating studies to generate national data. For most conditions studied, PSUs have compiled the only national data, and in many cases PSU studies have generated hypotheses that have catalysed future research.

**OrphaNews Europe**: How is INoPSU funded?

**Richard Lynn**: PSUs in each country are responsible for securing their own funding: most rely on government funding and PSUs also charge researchers study fees to offset the costs of running the surveillance studies. The central INoPSU administration is funded by small donations from each national PSU. The costs of the INoPSU gatherings are borne by the unit volunteering the venue. INoPSU continues to apply to various funding agencies in the hope of maintaining and expanding the activities of the network. Given the impacts that have resulted from international study findings, the network remains positive of securing long term funding.

**OrphaNews Europe**: Can you comment on the future directions of the INoPSU?

**Richard Lynn**: Over the next few years, INoPSU will undertake activities to raise its profile by encouraging the publication of collaborative papers; by presenting study results at national and international conferences; and by establishing closer contacts with organisations such as Orphanet, EURORDIS (Rare Diseases Europe) and NORD (National Organization for Rare Disorders). Several of the units are inputting into the development of their national rare disease programmes and INoPSU is supporting Rare Disease Day. Importantly, the network will enhance the resource database and hopes to launch this feature along with a new website before INoPSU’s 15th anniversary conference in 2013.

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