



**Australian Paediatric Surveillance Unit**  
**20 Years of Research into Rare Diseases**

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We thank the Cross family for the photo (front cover) of Molly, who has Angelman Syndrome. Lisa Footner, Photography

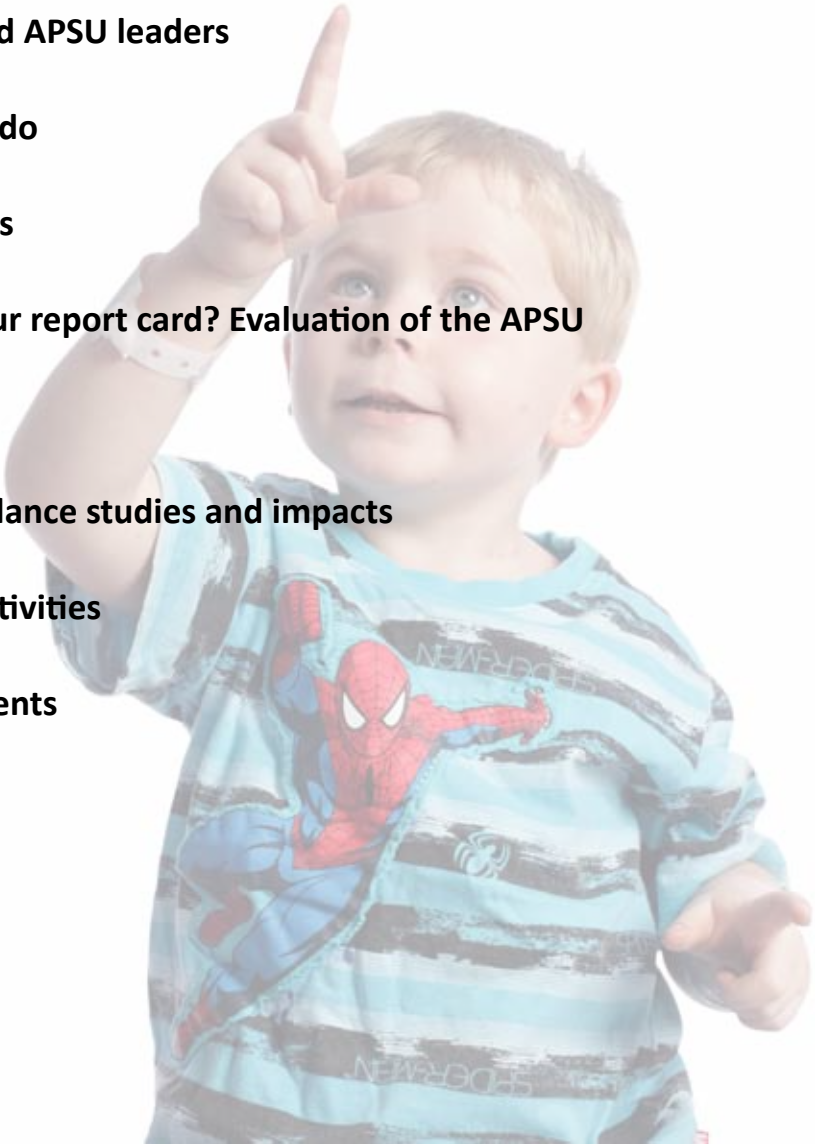
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## From the Director

Professor Elizabeth Elliott, AM, Director, Australian Paediatric Surveillance Unit

In 1993 I established The Australian Paediatric Surveillance Unit (APSU) to address gaps in knowledge about rare conditions of childhood. The unique national data APSU has provided since are invaluable. As I reflect on the past two decades, I feel a sense of great pride in the collective achievement of Australian paediatricians, who have contributed so willingly to APSU research and have, in turn, improved child health outcomes.

Whether working in a remote desert community clinic or a modern tertiary hospital, paediatricians throughout Australia participate in APSU surveillance and benefit from its scientific and educational outputs. The high, sustained return of monthly report cards over 20 years attests to the value with which clinicians regard the APSU. Formal evaluations confirm that paediatricians believe the APSU is important for generating knowledge and identifying future research directions and that the data collected by the APSU both help guide their clinical practice and inform public health policy.

Our remit has evolved to extend beyond national rare disease surveillance, to include a wide range of related activities. In collaboration with the National Centre for Immunisation Research and Surveillance, the APSU initiated and manages Paediatric Active Enhanced Disease Surveillance (PAEDS), a novel inpatient surveillance system in five paediatric hospitals in five States. The value of PAEDS was highlighted in our ability to respond rapidly to provide data about children hospitalised with influenza during the 2009 H1N1 pandemic. As outlined in this report, APSU data have widely influenced clinical practice and informed advocacy, services, treatments and policy. In a project funded by the Australian Research Council we are partnering with parent support groups (SMILE, the Steve Waugh Foundation and the Association of Genetic Supports of Australasia), the Sydney Children's Hospitals Network, and the Royal Australasian College of Physicians (RACP), to evaluate the impacts of rare diseases on families and providers. Through this we will determine what skills, knowledge and supports are required to improve child health and wellbeing.

The APSU has had leadership roles in the development and ongoing activities of the International Network of Paediatric Surveillance Units and has supported development of other national surveillance systems for monitoring rare outcomes of pregnancy and rare eye diseases. In 2009, the APSU initiated a national discourse on the need for a national plan for rare diseases. This work, highlighted in a *Lancet* World Report, is being progressed through the National Rare Diseases Committee. APSU is also represented on the Orphanet Australia National Advisory Group, to better support Australians with rare diseases. APSU is conducting a national audit of transition services from paediatric to adult care. Despite many successes and high productivity, long term infrastructure funding remains elusive.

APSU success reflects the commitment of Australian paediatricians, APSU staff and students, our partners and our funders, and I thank them sincerely for their support. I give particular thanks to the APSU's Deputy Director Associate Professor Yvonne Zurynski and the Board Chair, Professor Carol Bower. I am grateful for long term support from the Division of Paediatrics and Child Health (RACP), Commonwealth Department of Health and Ageing, and National Health and Medical Research Council of Australia. It is with pleasure and gratitude that I acknowledge 20 productive years of the APSU.

*"I feel a sense of great pride in the collective achievement of Australian paediatricians, who have contributed so willingly to APSU research studies which, in turn, have improved child health outcomes".*

## Our Team

The APSU relies on highly dedicated people who bring exceptional skills in research, administration and data management. Our achievements would not have been possible without our excellent team members past and present. For a complete list of APSU staff please see page 28.

Staff: (L-R): Yvonne Zurynski, Kirrilee Drew, Jade Mangan, Oluyemisi Ijamakinwa, Neil Hime, Ingrid Charters, Elizabeth Elliott, Marie Deverell.



# Key partners and APSU leaders



**The Hon. Tanya Plibersek MP, Minister for Health and Ageing**  
Through 20 years of national surveillance, the Australian Paediatric Surveillance Unit (APSU) has built an impressive evidence base of 53 rare childhood diseases, including genetic disorders, child mental health problems, rare injuries, rare infections and vaccine-preventable diseases. With 1,400 Australian clinicians reporting to and receiving aggregated clinical data from APSU, earlier diagnoses and treatment of rare chronic conditions in children becomes possible. With this information, APSU's work enables continuous improvements to our research, clinical and policy responses. APSU surveillance and information is especially important during times of epidemiological emergencies, when emerging diseases and outbreaks may demand a rapid national response. The Australian Government is proud to have supported your important work to improve the detection, prevention and awareness of communicable and chronic diseases.

I congratulate the APSU on achieving 20 years of continuous service to public health.



**Professor Warwick Anderson, AM, CEO, National Health and Medical Research Council of Australia**  
The APSU has provided an important platform for national research into rare childhood conditions over the last 20 years. The NHMRC is proud to have supported the APSU through an Enabling Grant which resulted in a reliable, accessible and unique mechanism for conduct and dissemination of national research that better informs policy and practice.



**Associate Professor Susan Moloney, President, Paediatrics and Child Health Division, Royal Australasian College of Physicians**  
The Paediatrics and Child Health Division of the Royal Australasian College of Physicians is proud to have an ongoing partnership with APSU. The work done by APSU over the last 20 years has resulted in a greater understanding, in the Australian context, of many rare conditions and contributed to the dissemination of this information to Fellows of the Division. The APSU have also supported and contributed to the College's Annual Congress over the last decade. I have greatly enjoyed the resources and education provided about many and varied diseases and conditions.



**Professor Carol Bower, Board Chair, Australian Paediatric Surveillance Unit**  
Since its inception 20 years ago, the APSU has grown, developed and been enormously successful in the surveillance of a wide variety of paediatric conditions in Australia, as evidenced by this Report. Importantly, the results of APSU research and advocacy have led to a better understanding of and improvements in child health. It has been my great pleasure to have been an investigator on several APSU projects, a member of the APSU Scientific Review Panel since 1998 and Board Chair since 2003. I am confident that the new APSU Advisory Council will champion the APSU and its ongoing important work.



**Professor Fiona Stanley, AC, Patron, Australian Paediatric Surveillance Unit**  
I'm very proud to be the APSU Patron! The idea of the APSU is especially important, because whilst we had data on some paediatric conditions, we did not have data on the rare paediatric outcomes that might only be seen in paediatric hospitals, outpatient clinics or surgery. The APSU has been incredibly successful in studying many important paediatric conditions in Australia and these studies have resulted in improved child health.  
Happy 20th birthday and long may you prosper!

# APSU: what we do

Associate Professor Yvonne Zurynski, Deputy Director, Australian Paediatric Surveillance Unit

Established in 1993 by Elizabeth Elliott, the APSU is a unique national resource which has provided vital national data on 53 rare childhood diseases - an often neglected area of research. The APSU provides infrastructure for collaboration by supporting the establishment of national research teams for each rare condition studied. Up to 16 different rare conditions can be studied simultaneously and data on demographics, diagnosis, clinical management and outcomes are collected.

The APSU provides national coverage by involving an estimated 90% of all paediatricians around Australia who each receive an APSU report card every month. The success of the system is a credit to Australian paediatricians, some of whom have been reporting to the APSU every month for 20 years! We thank each and every one of them!

Over the last 20 years the APSU has provided a unique platform for the study of rare childhood diseases and supports the following activities:

- National disease surveillance
- Research into rare diseases and their impacts on families, clinicians and health services
- Education for clinicians, families and students
- Policy and advocacy
- International collaboration

## APSU facts: Did you know...?

- 53 rare conditions studied
- Approximately 300 individual researchers involved as collaborators
- Grants totalling over \$12 million
- Monthly response rates >90% for 20 years
- 300,000 report cards sent; 282,000 returned
- 990 clinicians reported in 1993
- 1,400 clinicians report in 2013
- 6,010 rare disease cases reported
- 190 original journal articles
- 350 scientific presentations
- 270 media (TV, radio, newspaper) items



*“APSU does much more than surveillance. We inform policy and practice and we lead important research into the psychosocial and economic impacts of rare diseases on children, families and health services. APSU is truly unique!”*

## National disease surveillance

The APSU provides a national surveillance system accessible to researchers and clinicians wishing to study rare childhood diseases or conditions. The Department of Health and Ageing (DoHA) has supported surveillance for rare infectious diseases since 1997. In 2012 the APSU also received funding from DoHA Chronic Diseases Fund to support the study of rare chronic conditions such as interstitial lung disease, obesity hypoventilation and bronchiectasis. Annual surveillance for complications of influenza has also been funded by DoHA since 2007. In 2013, the APSU tendered successfully for DoHA funding for the study of Female Genital Mutilation in young girls. The NHMRC supported APSU surveillance infrastructure through an Enabling Grant from 2006-2012.

### *The APSU has supported the establishment of other national surveillance systems:*

#### ***Paediatric Active Enhanced Disease Surveillance System (PAEDS)***

PAEDS is an inpatient surveillance network of 5 tertiary paediatric hospitals in 5 Australian states established in 2007. Jointly developed and managed by the APSU and the National Centre for Immunisation Research and Surveillance (NCIRS), PAEDS provides detailed linked clinical and laboratory data on children hospitalised with rare infections, vaccine preventable diseases or potential adverse events following immunisation. The ability of PAEDS to respond to other epidemiological emergencies, emerging diseases and disease outbreaks was demonstrated in our rapid response to the 2009 H1N1 influenza pandemic. This was funded by an emergency NHMRC Influenza grant and for this project the team has been named by NHMRC as one of “10 of the Best” projects for 2013.

#### ***Australian Maternity Outcomes Surveillance System (AMOSS)***

The APSU supported the development of AMOSS which is modelled on APSU methodology and informed by the UK Obstetric Surveillance System (UKOSS) and is represented on the Advisory Board by the APSU Director and Deputy Director. AMOSS provides data on serious, rare outcomes related to birth and pregnancy.

#### ***The Australian and New Zealand Ophthalmic Surveillance Unit (ANZOSU)***

The APSU provided information and support during the development of ANZOSU which provides information on rare eye diseases.



## Research into rare diseases and impacts on families, clinicians and health services

The psychosocial and economic impacts of rare disease on families, clinicians and health services have rarely been studied. The award of a highly competitive Australian Research Council (ARC) Linkage Grant will enable the APSU to address these gaps. The project aims to inform new health service delivery models and improvements to economic and emotional supports for families. This grant brings together many partners including:

- The Royal Australasian College of Physicians
- The University of Sydney
- The University of Western Australia
- Sydney Children’s Hospitals Network
- SMILE Foundation
- The Steve Waugh Foundation
- The Association of Genetic Support of Australasia

An honours project by Matilda Anderson in the Sydney Medical School Postgraduate Programme produced important pilot data on the experiences of families living with a child with rare disease and will inform the ARC Linkage Grant project.

### *Transition to adult services*

The APSU is researching the transition from paediatrics to adult health services in young people affected by rare chronic conditions by undertaking a review of transition services nationally and internationally. In a Forum organised by the APSU and supported by Rare Voices Australia and the SMILE Foundation, young people called for adequate preparation for transition, better linking between paediatric and adult health services, and a “One Stop Shop” where they could access a case manager or “transition coach” to help navigate the health system.

### ***Families living with rare disease: Did you know...?***

- 43% feel diagnosis was delayed
- Psychosocial impacts are moderate to high for 90% of families
- Less than half received information about peer-support groups
- In the last 12 months, on average each child had 9 visits per child to specialists, and 6 to GPs
- 70% had admissions to hospital

Zurynski Y, Elliott EJ. Challenges of transition to adult health services for patients with rare diseases. *Medical Journal of Australia* 2013; 198 (11): 575-576.

Anderson M, Elliott EJ, Zurynski YA. Australian families living with rare disease: Experiences of diagnosis, health services use and needs for psychosocial support. *Orphanet Journal of Rare Diseases* 2013:22.



# Education for clinicians, families and students

## Clinicians

The APSU provides educational resources for Australian paediatricians by:

- Distributing surveillance protocols to ~1400 paediatricians, providing information about diagnosis, treatment and outcomes for each of the rare conditions studied
- Developing educational modules, e.g. the RACP Indigenous Medicine Module
- Holding educational workshops on rare diseases e.g. FASD, Rett syndrome
- Presenting a dedicated APSU session at the annual RACP Congress each year
- Publishing APSU results in reports and journals

APSU studies have informed: the development of new clinical resources, e.g. a diagnostic tool for fetal alcohol spectrum disorders; a review of diagnostic criteria for early-onset eating disorders; development of screening policy for Vitamin D deficiency; and a protocol for Vitamin K prophylaxis.

## Families and the public

Easily accessible information sheets are made available on the APSU website. These are written in lay language for ease of understanding and often include links to other resources including support groups. Eight rare disease educational workshops for families with children with rare diseases have been held, mostly to coincide with international Rare Disease Day.

## Students

The APSU provides opportunities for students to undertake post-graduate research degrees at Masters or PhD level, honours research projects and research projects for advanced trainees in paediatrics for the Fellowship of the Royal Australasian College of Physicians. Students find the APSU environment supportive and stimulating as the APSU encourages student collaborations with researchers and clinicians. Our academic supervisors encourage the early dissemination of results from student projects through conference presentations and journal publications. Students at honours level have published in peer reviewed journals and Michael Smith won the Dean's prize for the best Summer Scholarship Project. We are very proud of our students and their achievements!

Anderson M, Elliott EJ, Zurynski YA. Australian families living with rare disease: experiences of diagnosis, health services use and needs for psychosocial support. *Orphanet Journal of Rare Diseases* 2013;8(1):22.

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Esterman EE, Lahra MM, Zurynski YA, Booy R, Elliott EJ. Influenza infection in infants aged <6 months during the H1N1-09 pandemic: a hospital-based cohort study. *Journal of Paediatrics and Child Health* 2012; June 18: DOI:1111/jpc.12266.

Fitzpatrick JP, Latimer J, Ferreira, M, Martiniuk ALC, Peadon E, Carter M, Oscar J, Carter E, Kefford M, Shandley R, Yungabun H, Elliott EJ. Development of a reliable questionnaire to assist in the diagnosis of fetal alcohol spectrum disorders (FASD). *BMC Pediatrics* 2013; 13(1): Article number 33.



Michael Smith receiving his award from Professor Bruce Robinson, Dean, Sydney Medical School



# Policy and advocacy

## Rare disease national plan

With other Australian rare disease organisations, the APSU has brought attention to rare diseases, and the need for a coordinated approach to health and social policy. In 2009 the APSU convened a National Rare Disease Working Group and, with support from the Australian Research Alliance for Children and Youth (ARACY), drafted 8 principles to be addressed by a National Plan for Rare Diseases.

To progress the notion of a rare disease plan the APSU, with colleagues from the Office of Population Health Genomics, Western Australian Department of Health convened a symposium, “Awakening Australia to Rare Diseases” in 2011. This confirmed support for a national plan and establishment of The National Rare Diseases Committee (on which the APSU is represented by E. Elliott and Y. Zurynski) to progress a plan.

## Advocacy

APSU advocates on behalf of children with rare diseases through participation in the Orphenet Australia National Advisory Group, the Steve Waugh Foundation Medical Advisory Committee and the Smile Foundation Board.

## Examples of clinical and public health policy impacts of APSU studies:

- Supported public health prevention strategies, e.g. informing or supporting vaccination programmes such as rubella, varicella, human papillomavirus; contributing to Polio-Free Certification by the World Health Organisation
- Data on injuries related to inappropriate use of child restraints and seatbelts informed new laws
- Data on fetal alcohol syndrome informed the 2009 review of NHMRC guidelines on alcohol use in pregnancy, and development of clinical diagnostic and management tools
- HIV and Hepatitis C studies informed prenatal screening policy
- The study on Haemolytic Uraemic Syndrome informed production code for fermented meat and requirements for laboratory diagnostic testing

“APSU has advocated for families living with rare disease during each International Rare Disease day since 2009”.

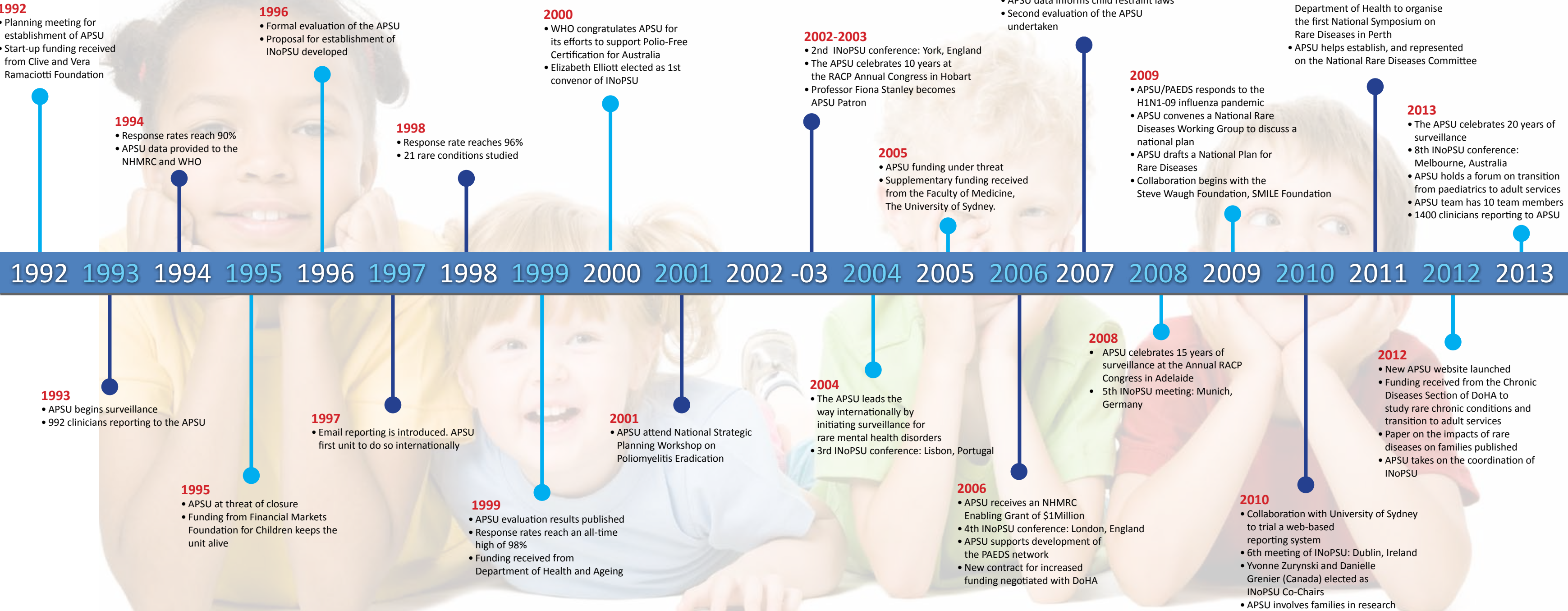
Jaffe A, Zurynski Y, Beville L, Elliott E. Call for a national plan for rare diseases. *Journal of Paediatrics and Child Health* 2010; 46(1-2):2-4.

Kirby T. Australia makes up for lost time on rare diseases. *The Lancet* 2012; 379(9827):1689 – 1690.





# APSU Milestones







# Full marks on our report card?

## Evaluation of the APSU

The APSU is the only national paediatric surveillance unit to be evaluated twice (1999 and 2007) using the Centres for Disease Control and Prevention (CDC) criteria. The APSU system demonstrated: simplicity, acceptability and flexibility, data quality and representativeness, sensitivity, timeliness, stability and reliability.

The reporting mechanism was acceptable to clinicians who voluntarily return ~93% of monthly report cards. The majority (95%) of clinicians believed that the work of the APSU is valuable, specifically for:

- generating knowledge (89%)
- identifying research priorities (80%)
- guiding clinical practice (75%)
- informing public health policy (72%)

Below are just some examples of the many positive comments received from clinicians participating in APSU surveillance. They highlight the perceived value offered by the APSU to improve the knowledge base about rare paediatric diseases amongst clinicians.

*“Valuable role in the broad field of paediatrics. Keep up the fantastic work!”*  
*“The system is good and important for it to continue, and the added knowledge and publications gives benefits to all.”*

In addition, the APSU is reaching a wider audience of researchers, clinicians and policy-makers by increasing dissemination of results (> 190 journal articles, > 350 conference presentations and > 270 media items).

Limitations of the APSU include difficulties in confirming case ascertainment rates due to a lack of alternative national data sources and surveillance gaps persist for children in remote, rural and disadvantaged communities.

The evaluation results have informed continuous improvement projects. Despite the simplicity of the system and the excellent value for money it provides, APSU is reliant on short-term funding. Securing long term infrastructure funds continues to be a challenge.

He S, Zurynski YA, Elliott EJ. What do paediatricians think of the Australian Paediatric Surveillance Unit? *Journal of Paediatrics and Child Health* 2010;46(7-8):412-8.

He S, Zurynski YA, Elliott EJ. Evaluation of a national resource to identify and study rare diseases: The Australian Paediatric Surveillance Unit. *Journal of Paediatrics and Child Health* 2009;45(9):498-504.

# Messages from clinicians and stakeholders

*“There is much strength in the APSU which has amplified through maturity. Its simplicity in enabling thousands of clinicians to work together so easily and seemingly seamlessly is a credit to the central organising group. I salute Liz and Yvonne and the whole team for their endurance, patience, ingenuity, clarity, productivity and sheer bloody-mindedness to make things work. A very happy 20th Anniversary!”* **Professor Robert Booy**

*“The APSU provides great infrastructure, easy to set up a study with minimal fuss and great study data that I would not have been able to get without such an organisation. The staff is approachable and willing to assist whenever asked. I don’t believe I could have run our studies without APSU collaboration.”* **Dr Sam Mehr**

*“It has been fascinating to watch the APSU grow and develop from its tiny origins 20 years ago. So many great projects have benefited from the expertise and infrastructure that the APSU has provided. I have been involved in a couple of these - Adverse Effects of Complementary and Alternative Medicines in Children, and a current application for Chronic Fatigue Syndrome. These haven’t fitted into the traditional “rare diseases” model of the APSU but the adaptability of APSU’s framework plus the advice and guidance provided by the team has been really great. Here’s to the next 20 years!”* **Professor Mike South**

*“I remember an even more fresh-faced Liz Elliott than we see today, fired with enthusiasm about an Australian version of the British Paediatric Surveillance Unit more than 20 years ago. That vision has prospered beyond anyone’s expectations to an organisation of unparalleled achievement of many such units, which are now part of the International Network of Paediatric Surveillance Units. We look forward to even greater things in the next 20 years.”* **Professor Peter McIntyre**

*“The APSU has been a phenomenal success. I well remember the early discussions when it was being set up and have to admit that I didn’t envisage it having as great an impact as it now obviously has. It wasn’t easy in those early years and funds were very scarce for a long time. Despite these constraints, right from the start the APSU has provided a steady stream of rich data that has been of benefit not just to paediatricians but to many children and their families. Most lesser mortals would have given up, but not Elizabeth Elliott. It is a tribute to Liz’s vision, perseverance and ability to share that vision with others, that the APSU is now a valued aspect of Australian paediatrics.”* **Emeritus Professor Kim Oates**

*“The continued high rate of response from paediatricians over the 20 years of the APSU is testament to how highly the Unit is regarded and how valuable the surveillance system is. It is impressive how adaptable the APSU has been to changing concerns in paediatric practice and changes in methods of surveillance. Much of the research conducted through the APSU has led on to further study and, importantly, to influences on policy and practice. This represents the very best in research.”* **Professor Carol Bower**

*“The APSU has been an essential component of Australia’s surveillance for cases of polio-myelitis in children as part of the WHO global polio eradication initiative. The acute flaccid paralysis surveillance program was established in 1995 and has been the mainstay of clinical surveillance to monitor Australia’s polio-free status.”* **Associate Professor Bruce Thorley**





## Patient stories

### Sarah, *age 4, Interstitial Lung Disease*

Sarah was born with a rare lung disease. After lung biopsies at 3 weeks of age, tests for Cystic Fibrosis, many doses of artificial lung surfactant and multiple doses of steroids and antibiotics, Sarah was sent home on oxygen and requiring nasogastric tube feeds after three months in hospital. Children's Interstitial Lung Disease (ChILD) was diagnosed after extensive genetic testing when Sarah was 5 months old.

Diagnosis is often delayed resulting in feelings of uncertainty for families. Financial costs to families are also substantial. "In the first year of life we were \$18,000 out of pocket for medical expenses alone. We also had to install air conditioning and buy a new van to carry all of the equipment that Sarah constantly needs as well as our other 2 children."

"Our home has been transformed into a hospital ward, with oxygen cylinders, meters of tubing and an oxygen concentrator, a pulse oximeter, a VPap machine, air purifier and many different medications and specialised formula and equipment for gastrostomy feeds. Sarah has had 25 hospital admissions and requires a team of about 13 health professionals to care for her. Despite the struggles Sarah experienced in her first few years of life she now attends kindergarten in a main stream public school and is a happy, thriving little girl" Susanna Walker, Sarah's mum.

Families experience isolation, psychological and financial stress while they deal with delayed diagnosis, difficulties in accessing appropriate health services, lack of access to quality information about the disease, and lack of access to social support services. Often treatments are out of reach of families because they are not available or very costly.

Similarly, health professionals feel ill-equipped to deal with rare diseases due to lack of knowledge about diagnosis and management, and poor access to educational opportunities and specialised referral services.

### Siobhan, *age 18, Klippel-Trenaunay Syndrome*

Siobhan is currently transitioning from paediatric to adult health services. She attended the APSU Transition Forum with her mum and sister.

"I haven't been able to complete school properly. I've been attending school on and off for the last eight years with varying degrees of success. Most of this is due to surgery, pain and fatigue. I am at TAFE completing a certificate IV course so I can go to University.

I have trouble keeping friendships/relationships. Again, I didn't attend school properly so I have no long lasting relationships from that, and most people don't understand and seem confused by my illness. It also strains my family relationships and I feel bad for my family who have to listen to me when I'm feeling ill. They can't help me, and I feel terrible for it.

I've had an abundance of surgeries, some terribly serious and some only slightly so, some that weren't meant to be serious but somehow ended up costing me years of my life.

I'm still transitioning, but it's been a trial. As I have a rare condition it's almost impossible to find doctors who will take on a case like mine. Added to the fact that no one informed me I needed to transition until a month before I turned eighteen, essentially it was thrown at me and I've been in limbo ever since. I'm too old for paediatrics, but too difficult a case for adult services to treat in this country. It's tiresome really. I wish there was a simple way for this to go. I wish I had a simple illness to work with."



*"Our home has been transformed into a hospital ward, with oxygen cylinders, meters of tubing ....and many different medications and specialised formula and equipment for gastrostomy feeds. Sarah has had 25 hospital admissions and requires a team of about 13 health professionals to care for her." Susanna Walker*



# Selected studies and impacts

## Severe complications of influenza (2007 – current)

**Investigators** (APSU and PAEDS): Elizabeth Elliott, Yvonne Zurynski, David Lester-Smith, Robert Booy, Marino Festa, Peter McIntyre, Peter Richmond, Chris Blyth, Helen Marshall, Mike Gold, Jim Buttery, Jenny Royle

Child deaths due to influenza in 2007 prompted the Department of Health and Ageing to request surveillance for the severe complications of influenza. APSU responded within 2 weeks, demonstrating capacity to respond rapidly to emerging diseases and epidemics. Following the successful pilot in 2007, the APSU has conducted surveillance for children aged <15 years admitted to hospital with laboratory confirmed influenza and severe complications, during every influenza season from 2008 to 2013.

In 2009, during the H1N1 pandemic an NHMRC grant (633028) supported surveillance through PAEDS, using a modified APSU protocol, for all hospitalised cases of influenza.

### Key Findings:

Severe complications of influenza (APSU 2007 – 2012):

- 270 cases reported 2007-2012; 100 during the 2009 H1N1 pandemic
- 110 admitted to intensive care for severe complications e.g. pneumonia, encephalitis
- 18 deaths reported; 5 in children who were previously healthy
- Most children with predisposing chronic conditions unvaccinated
- Use of antivirals (Tamiflu) low: 7% in 2008; 65% in 2009; 35% in 2012

Influenza pandemic (PAEDS July-Sept 2009):

- High burden on health services: 601 children admitted to 6 paediatric hospitals
- Described serious complications such as serious neurological problems, including in previously healthy children
- Provided information on the use of Tamiflu in infants aged <12 months
- Highlighted need for improved vaccination of health professionals and prevention of nosocomial infection

### Study Impacts:

- Provided evidence showing that even previously healthy children may develop very serious complications of influenza, thereby supporting influenza vaccination for children
- Showed significant impacts of influenza on paediatric in-patient services
- Highlighted the need to improve the implementation of the current recommendation to vaccinate children with chronic conditions who are especially vulnerable
- NHMRC grant (633028) was featured in “NHMRC’s 10 of the best grants – 2013”

Khandaker G, Zurynski Y, Buttery J, Marshall H, Richmond PC, Dale RC, et al. Neurologic complications of influenza A(H1N1)pdm09: Surveillance in 6 pediatric hospitals. *Neurology* 2012;79(14):1474-81.

Khandaker G, Rashid H, Zurynski Y, Richmond PC, Buttery J, Marshall H, et al. Nosocomial vs community-acquired pandemic influenza A (H1N1) 2009: a nested case-control study. *Journal of Hospital Infection* 2012;82(2):94-100.

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**Early onset eating disorder (2002-2005)**

**Investigators:** Sloane Madden, Anne Morris, Ken Nunn, Michael Kohn, Bryan Lask, Susan Sawyer

**Key Findings**

- Young children aged 5 to 13 present with significant weight loss and associated psychological and medical complications
- 100 cases identified over 3 years; a quarter were boys
- Median weight loss was 6kg
- Weight loss was greatest among children aged 9 years or more and failure to gain weight was observed in younger children
- Twenty girls who had reached menarche had secondary amenorrhea
- Most (70%) of children expressed a fear of gaining weight, disturbed body image and a denial of the severity of their low weight
- Depression and anxiety most common co-morbidities
- Excessive exercise was described in 57% and self-induced vomiting in 12%
- Significant medical instability found in ~30% including:
  - bradycardia 42%,
  - hypothermia 32%
  - hypotension 21%

**Study Impacts:**

- These data will provide a very valuable contribution to the debate on definition and classification of eating disorders among young children and highlighted the need to review the DSM-IV criteria for eating disorders in children aged <13 years
- An international comparison of the incidence, diagnosis and outcomes of early onset eating disorders is being facilitated by INoPSU, with data available from the Canadian and British surveillance units

**Fetal alcohol syndrome (2001-2004)**

**Investigators:** Carol Bower, Anne Morris, Jan Payne, Eric Haan, Elizabeth Elliott

Prior to the APSU study, few data on FAS were published in Australia. The APSU study was an impetus for health professional education, policy, and new collaborative research.

**Key Findings:**

- FAS is still diagnosed in Australia; a wide range of medical, psychological and behavioural problems requiring health, education and community resources was identified; 24% children have significant birth defects
- 92 reported cases met criteria for FAS/pFAS with a median age at diagnosis of 2.8 years (birth to 12 y). 51% were male and 61% identified as Indigenous
- Only 42% of children lived with their biological parent(s), 17% lived with their grandparents or other relatives; and 60% were adopted or fostered; prevention opportunities missed
- Most (76%) mothers used one or more substances in addition to alcohol and ~50% children had siblings with FAS

**Study Impacts:**

- An Alcohol In Pregnancy Research Group was formed and conducted research on: attitudes and knowledge of women and health professionals regarding alcohol use in pregnancy and Fetal Alcohol Spectrum Disorders (FASD); the reasons Aboriginal women use alcohol in pregnancy; and communication of public health messages about alcohol and pregnancy
- APSU data informed development of educational materials for health professionals, adopted throughout Western Australia and available at: [http://alcoholpregnancy.childhealthresearch.org.au/media/68501/2011\\_booklet\\_for\\_health\\_professionals.pdf](http://alcoholpregnancy.childhealthresearch.org.au/media/68501/2011_booklet_for_health_professionals.pdf)
- APSU data informed 2009 NHMRC Guidelines on Alcohol Use in Pregnancy
- Formation by the Ministerial Council on Drugs of an Intergovernmental Committee on FASD, which resulted in a Monograph on FASD in Australia [www.nationaldrugstrategy.gov.au](http://www.nationaldrugstrategy.gov.au)
- Members of the investigator team (EE, CB) later successfully tendered to DoHA for development of screening and diagnostic tools for FASD
- Investigators (EE, CB) are involved in the Lililwan Project, which aims to determine FASD prevalence in Aboriginal communities in the Fitzroy Valley, Kimberley, WA. Collectively, these studies informed a House of Representatives Committee Inquiry into FASD submitted to government in 2011. This led to the announcement of a Commonwealth Action Plan (August 2013) with a \$20 million budget, with a focus on FASD research, prevention, and safety of woman and children.



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### ***Severe injuries related to seatbelts and child restraints (2006-2007)***

**Investigators:** Yvonne Zurynski, Elizabeth Elliott, Lynne Bliston, Mary McCaskill, Anthony Dilley, Fred Leditschke

#### ***Key Findings:***

- We collected data about 50 children aged <12 years seriously injured in road crashes while travelling restrained in a seatbelt or child restraint
- 80% of 4-8 year old children travelled in age-inappropriate restraints, usually in adult seatbelts rather than booster seats or approved restraints
- 94% of 6-8 year old children were misusing the seatbelt by placing the shoulder sash under the arm or behind the back
- Significant abdominal, spinal, head, neck and brain injuries were common
- Four children died; two children became paraplegic

**Study Impacts:** Results from this study attracted a great deal of media attention and informed the new National Transport Commission recommendations for child restraints under the 7th Amendment to Australian Road Rules and resulted in changes in child restraint law across Australia.

### ***Subdural haemorrhage and/or effusion (2010-2012)***

**Investigators:** Yvonne Zurynski, Susan Marks, Anna Stachurska, Ray Chaseling, Dimitra Tzioumi, Amanda Stephens, Cindy Molloy, Anne Piper, Judy Bragg, Glen Gole, Marianne Vonau, Peter Winterton, Anne Smith, Graham Vimpani

Subdural haemorrhage and/or effusion (SDH/E) is associated with significant morbidity and mortality among young children aged <2 years. There are no national data on SDH/E and its causes in Australia.

#### ***Key Findings:***

- Of 125 children reported over 2 years, in 65% the SDH/E was due to inflicted injury, 13% accidental falls, 7% due to birth injury, 6% medical causes and 16% undetermined
- Children with inflicted injury compared with those with SDH/E due to other causes, were more likely to present with retinal haemorrhages, seizures, “floppiness,” and body bruising
- Overall, 41% required neurosurgery and 45% were admitted to the intensive care unit
- Many were discharged to foster care

#### ***Study Impacts:***

- SDH/E has significant long term cost for the child, family and community. Our results have implications for improved community education and prevention policy







## International activities

The APSU has links with international organisations for rare diseases including Orphanet and the USA National Organisation for Rare Diseases. The APSU is represented by Elizabeth Elliott and Yvonne Zurynski on the Orphanet-Australia National Advisory Group. The group will contribute to Orphanet information about Australian rare diseases research, clinics and support organisations that will be easily accessible to Australian health professionals and families.

Importantly, the APSU was involved in establishing the International Network of Paediatric Surveillance Units (INoPSU) in 1998 and has been an active member ever since.

INoPSU has supported international collaboration to study rare childhood diseases. Collectively, INoPSU member units conduct surveillance in a population of over 46 million children. More than 8,000 clinicians contribute data on rare conditions each month and over 200 conditions have so far been studied to date.

### Examples of impacts on public health policy and clinical practice include:

- **Acute flaccid paralysis** surveillance confirms absence of wild or vaccine-related poliovirus; contributes to WHO eradication program.  
*Units:* Australia, Britain, Canada, New Zealand, Switzerland
- **Congenital rubella** surveillance supports childhood rubella vaccination programmes and the need for targeted vaccination for susceptible women including immigrants, non-immune women, either pre-conception or postpartum to prevent significant birth defects.  
*Units:* Australia, Britain, Canada, New Zealand, Switzerland, Netherlands
- **Early onset eating disorders** (<13 years) surveillance confirmed the need for pre-adolescent diagnostic criteria and early recognition, and evidence-based treatment, including amongst boys.  
*Units:* Australia, Britain, Canada, Netherlands
- **Haemolytic uraemic syndrome** surveillance described geographic variation in aetiology, highlighting the need for new diagnostic tests. Supported preventative measures, e.g. education; hygiene recommendations for kindy farms and new legislation on safe food production and monitoring of water supplies.  
*Units:* Australia, Britain, Canada, Latvia, New Zealand, Portugal, Switzerland
- **Vitamin K deficiency bleeding** surveillance confirmed that most cases are late onset and related to underlying liver disease; and that a high proportion of cases receive no prophylaxis none or an incomplete vitamin K prophylaxis at birth.  
*Units:* Australia, Britain, Canada, Germany, New Zealand, Switzerland, Netherlands



*“Congratulations to the APSU on reaching their 20th birthday. Their innovative ideas have pushed the boundaries of surveillance unit remit to become a force for rare disease advocacy across the whole of Australia.”*

*Richard Lynn, British Paediatric Surveillance Unit.*

*“Great leaders have a vision. Beyond gathering important national epidemiological data, the APSU established collaborative partnerships, and effectively brought information closer to families through innovative events like rare diseases day and workshops. Congratulations. The APSU is an inspiration for all.”*

*Danielle Grenier, Canadian Paediatric Surveillance Program.*

*“Congratulations to the APSU on 20 years of successful paediatric surveillance! The APSU has been innovative and extremely productive and has been admired all around the world. Keep up the great work- with all best wishes from the BPSU”*

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The APSU Chronic Disease Team (L-R): Marie Deverell, Oluyemisi Ijamakinwa, Neil Hime, Victor Wu.

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# Collaborating institutions and research groups

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- Australian Maternity Outcomes Surveillance System (AMOSS)
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- Association for the Welfare of Children in Hospitals (AWCH)
- Australia and New Zealand Paediatric Nephrology Association
- Australian CHARGE Association
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- Australian Polio Expert Committee
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- Commonwealth Department of Health and Ageing
- National Births Anomalies Steering Committee
- National Centre in HIV Epidemiology and Clinical Research
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- National Heart Foundation of Australia
- National Notifiable Diseases Surveillance System
- National Perinatal Epidemiology and Statistics Unit
- National Polio Reference Laboratory
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- British Paediatric Surveillance Unit
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- Netherlands Paediatric Surveillance Unit
- New Zealand Paediatric Surveillance Unit
- Portuguese Paediatric Surveillance Unit
- Swiss Paediatric Surveillance Unit
- Republic of Ireland Paediatric Surveillance Unit
- Welsh Paediatric Surveillance Unit





# *Support the APSU*

The APSU performs important research on rare childhood diseases and provides vital information to improve public health and prevention policy and clinical care. Ultimately, our research helps to improve the health and well-being of children living with rare infectious diseases, rare genetic conditions, rare allergies, rare mental health disorders, and rare injuries.

Although our work is important we have no ongoing infrastructure funding. We are reliant on competitive grants and, we welcome donations to support our research.

Your donations will go directly to support APSU research projects.

**Thank you!**



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Please contact the APSU on 02 9845 3005

Or send a cheque to:  
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