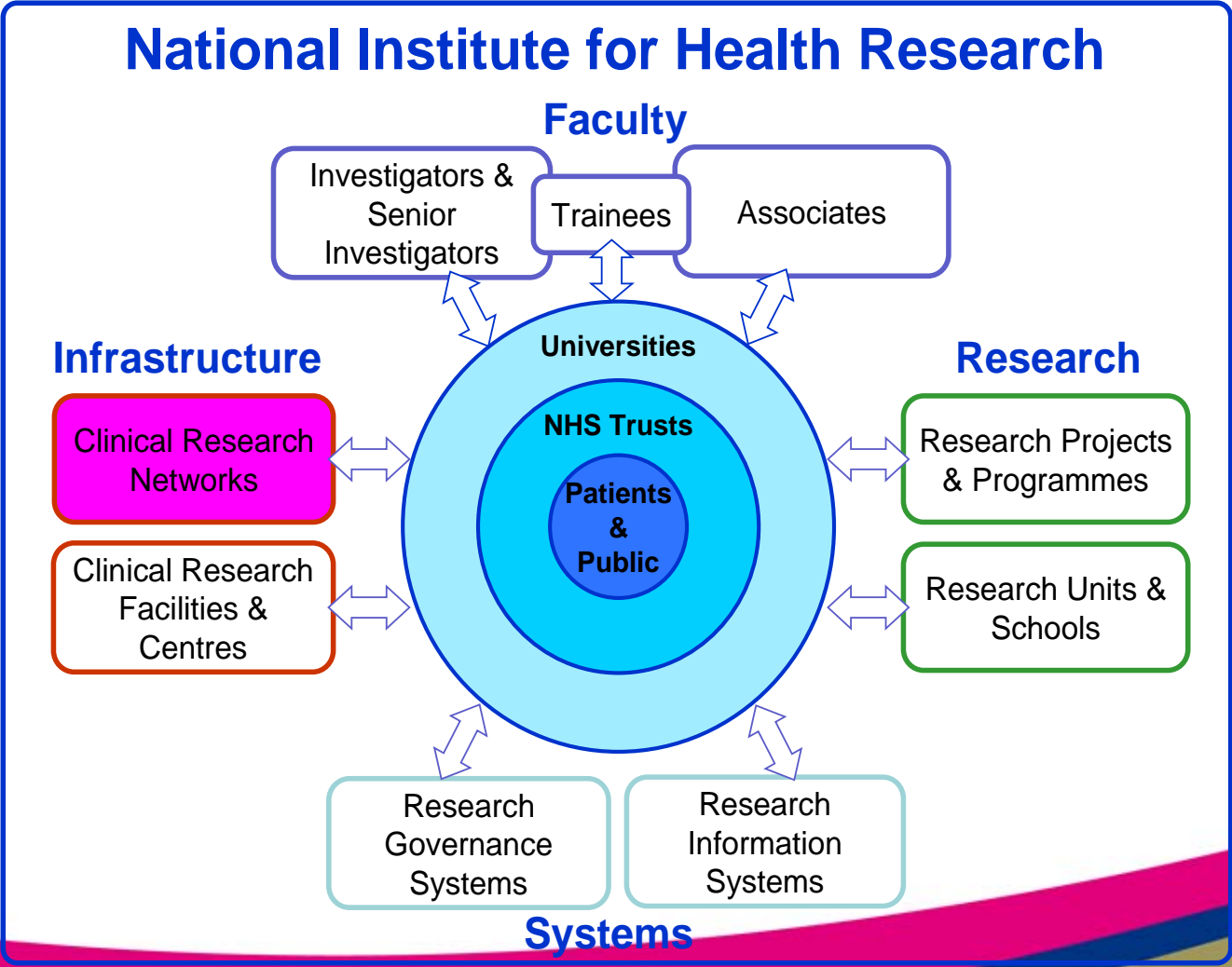




Medicines for  
**Children Local Research  
Network**

## Best Research for Best Health 2006

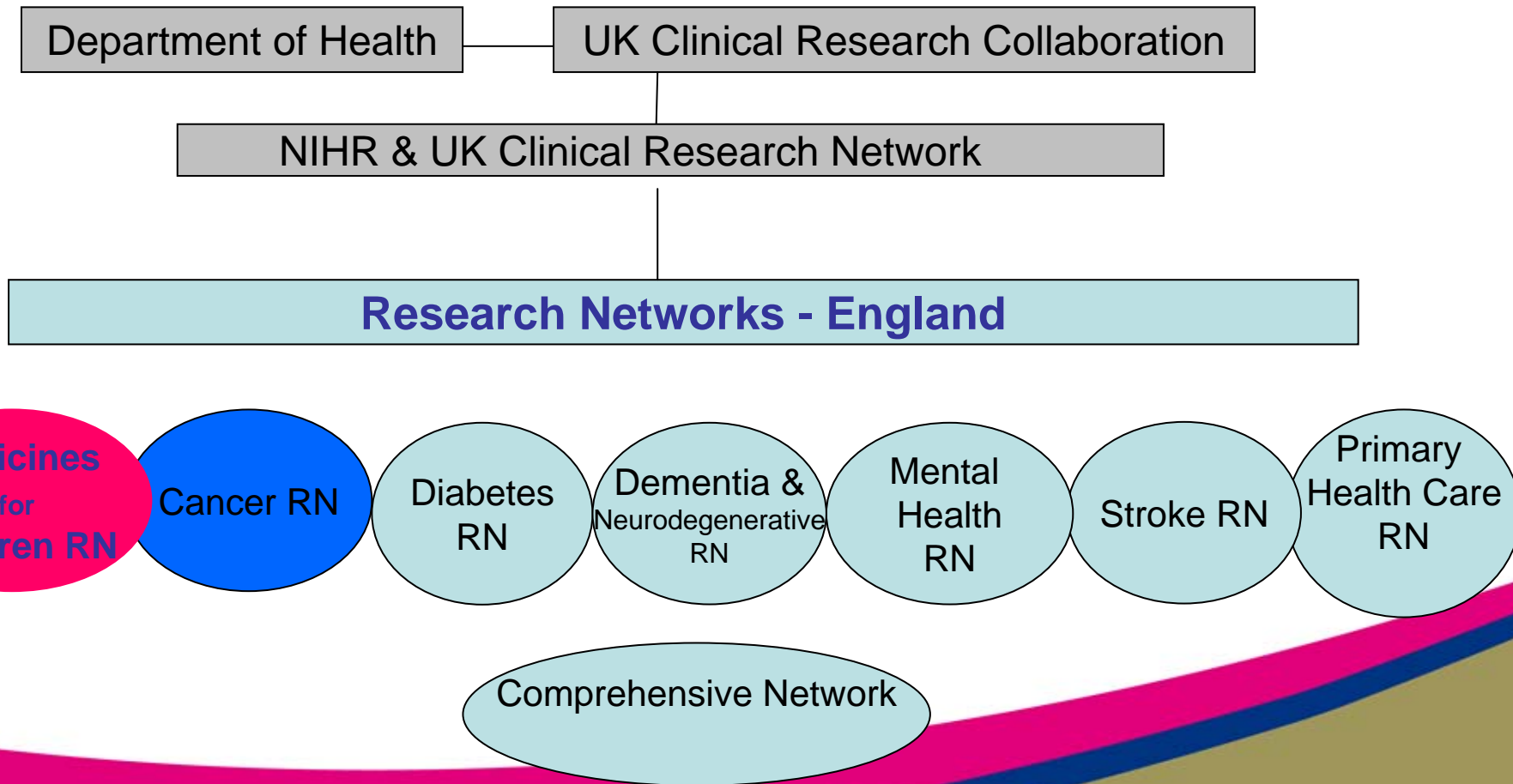
- To establish the NHS as an internationally recognised centre of research excellence
- To attract, develop and retain the best research professionals
- To commission research focused on improving health and care
- To strengthen and streamline systems for research management and governance



# UK Clinical Research Networks

- Most radical change to research in the NHS since Culyer Report (1996) which documented research activity for the first time
- Incentives exist for the pharmaceutical industry to run trials through the networks in collaboration with the NHS
- The number of clinical trials conducted in the NHS is expected to increase enormously!

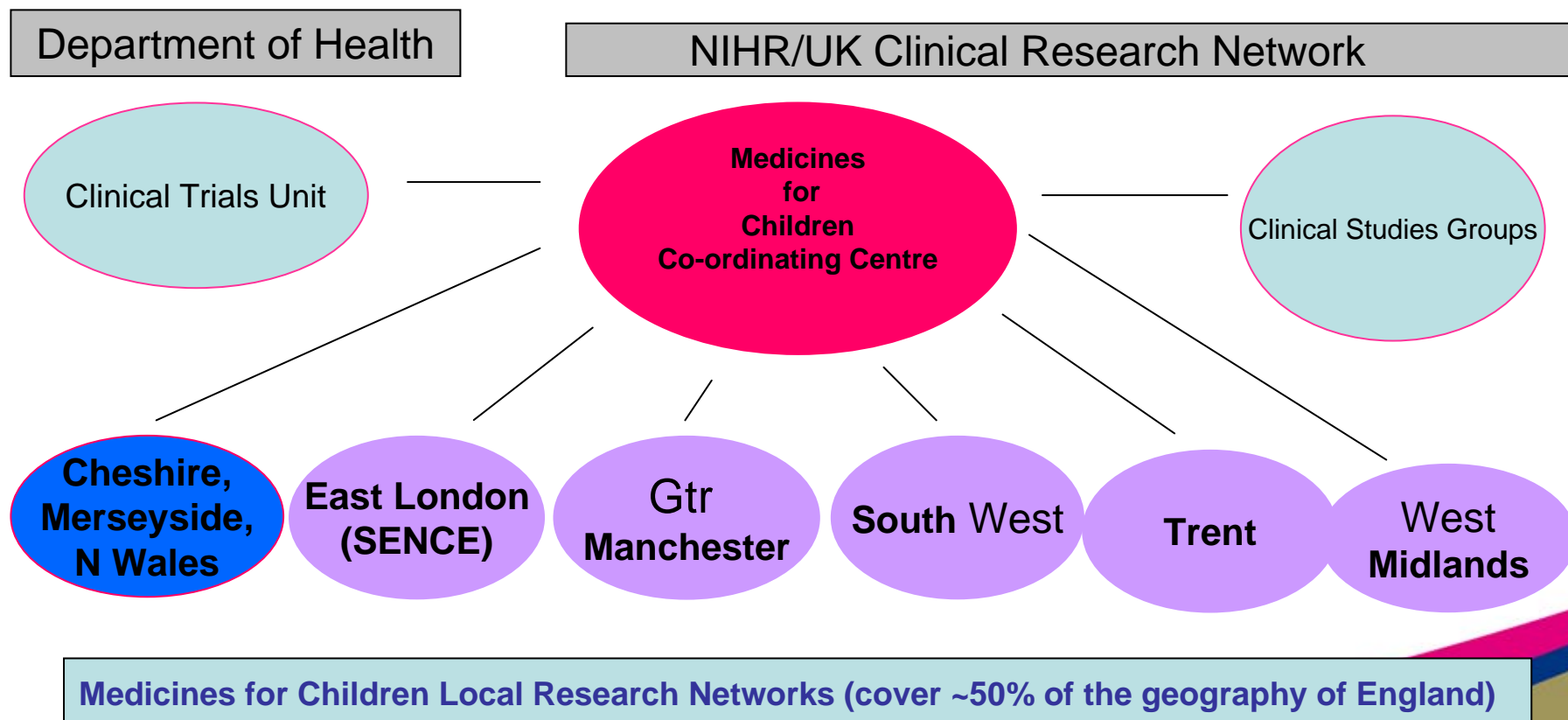
# Topic Specific Research Networks



## Inequity of R&D Funding provided as an annual levy by DoH 2007

• Birmingham Children's	£1,425,349
• Liverpool Children's	£780,641
• Royal Liverpool /Broadgreen	£3475,672
• Manchester Children's	£6,913,711
• Great Ormond Street	£28,494,166
• Guy's St Thomas's	£15,716,618
• Hammersmith	£48,926,516
• Liverpool Women's	£527,676

# Medicines for Children Research Network



# Clinical Studies Groups

Prioritise topics to study, develop protocols, advise on formulation issues and methodology.

Assist trial development and link to consumers and user groups

- Allergy, infection, immunity and nephrology
- Anaesthesia, pain control, intensive care and cardiology
- Diabetes, Endocrinology and Metabolic disease
- Gastroenterology, hepatology and nutrition
- General paediatrics
- Methodology
- Neonates
- Neurology
- Rheumatology
- Pharmacy and Pharmacology
- Respiratory and cystic fibrosis
- Children's Cancer and Leukaemia group

# Patient Public Involvement

- Stand up Speak up!
- Parent & Carer Council
- Children's Council
- Involve
- Children's Parliament Liverpool (includes a health minister)
- Patient Advisory Liaison service

# Geographical map of Local Research Network sites




# Medicines for Children Research Network Mission statement

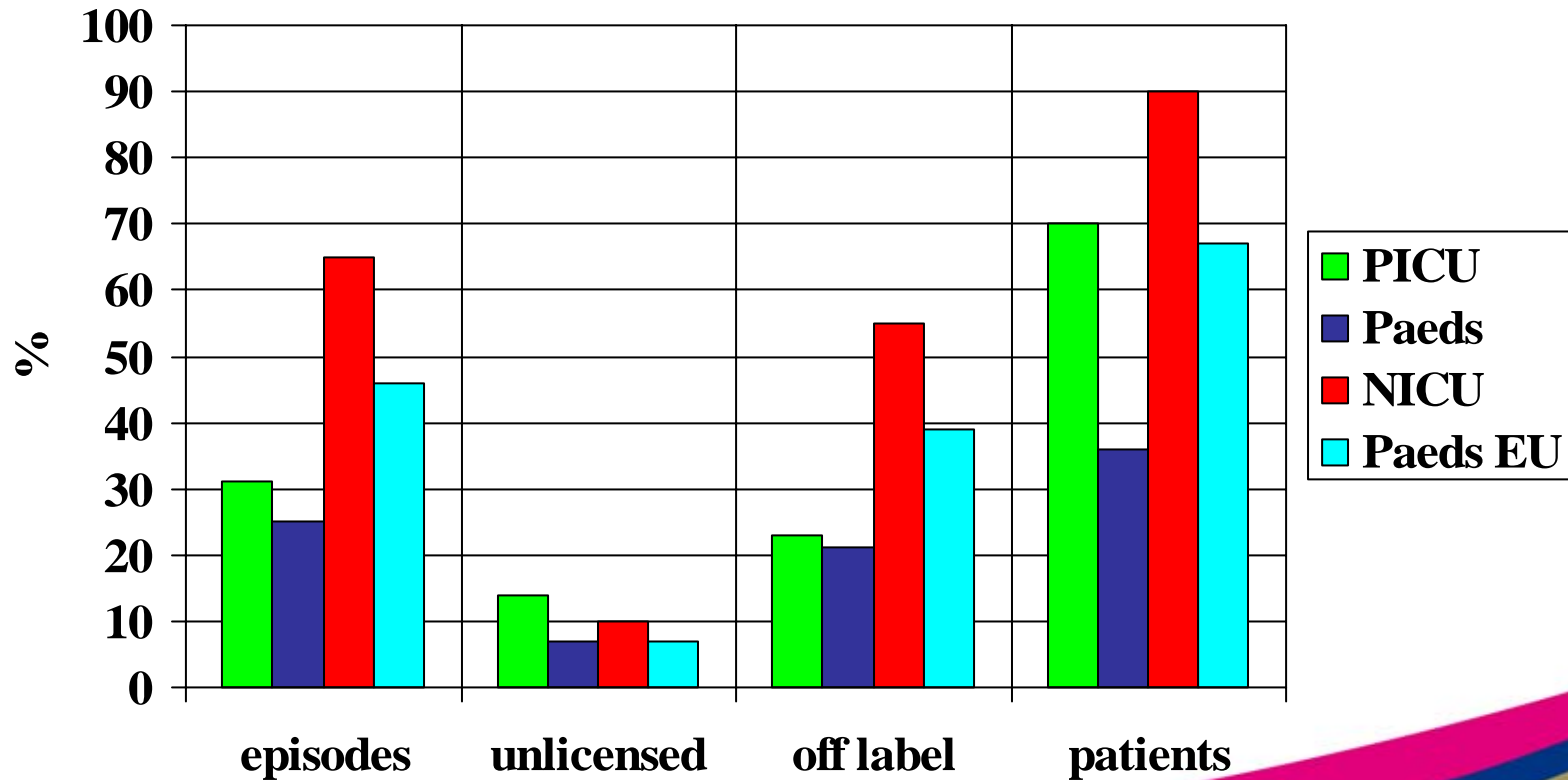
To provide a world-class infrastructure to support high quality clinical trials and other well designed studies in developing and providing drugs which are both safe and effective in the treatment of children

# Use of unlicensed medications in children

Paediatric doses and indications for clinical use  
are extrapolated from adult data

- reluctance to introduce new drugs
  - medication errors
  - inadequate dosing
  - unforeseen adverse effects
- 

# Unlicensed Drug use in Paediatrics

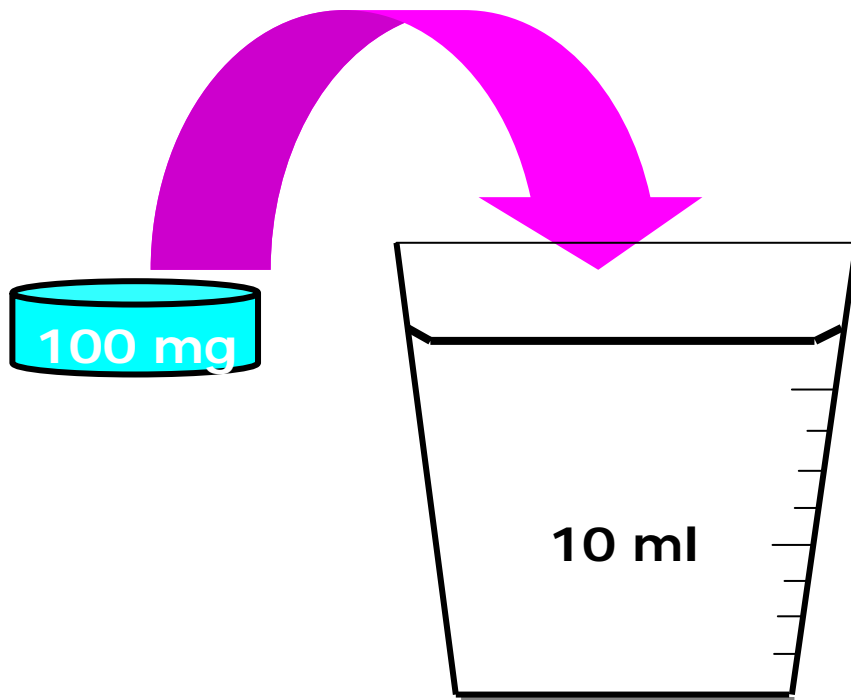


Survey of Unlicensed and Off Label Drug use in Paediatric Wards in European Countries  
Conroy et al 2000

## Industry disincentives due to:

- Patient numbers/disease burden is small
- Paediatric formulations are very expensive
- Recruiting paediatric patients is difficult
- Challenge of neonates -toxicity
- Outcome measures are difficult
- No 'safe and ethical' infrastructure and law to undertake such research until now

# Manipulation of dosage forms



5 ml = 50 mg ?

- **SOLUBILITY**
- **TIME**
- **STABILITY**

# Formulations appropriate for children

- Liquids
- Drops
- Buccal gels
- Transdermal patches
- Aerosols
- Tablets
- Capsules
- Chew tablets
- 'melt tablets'

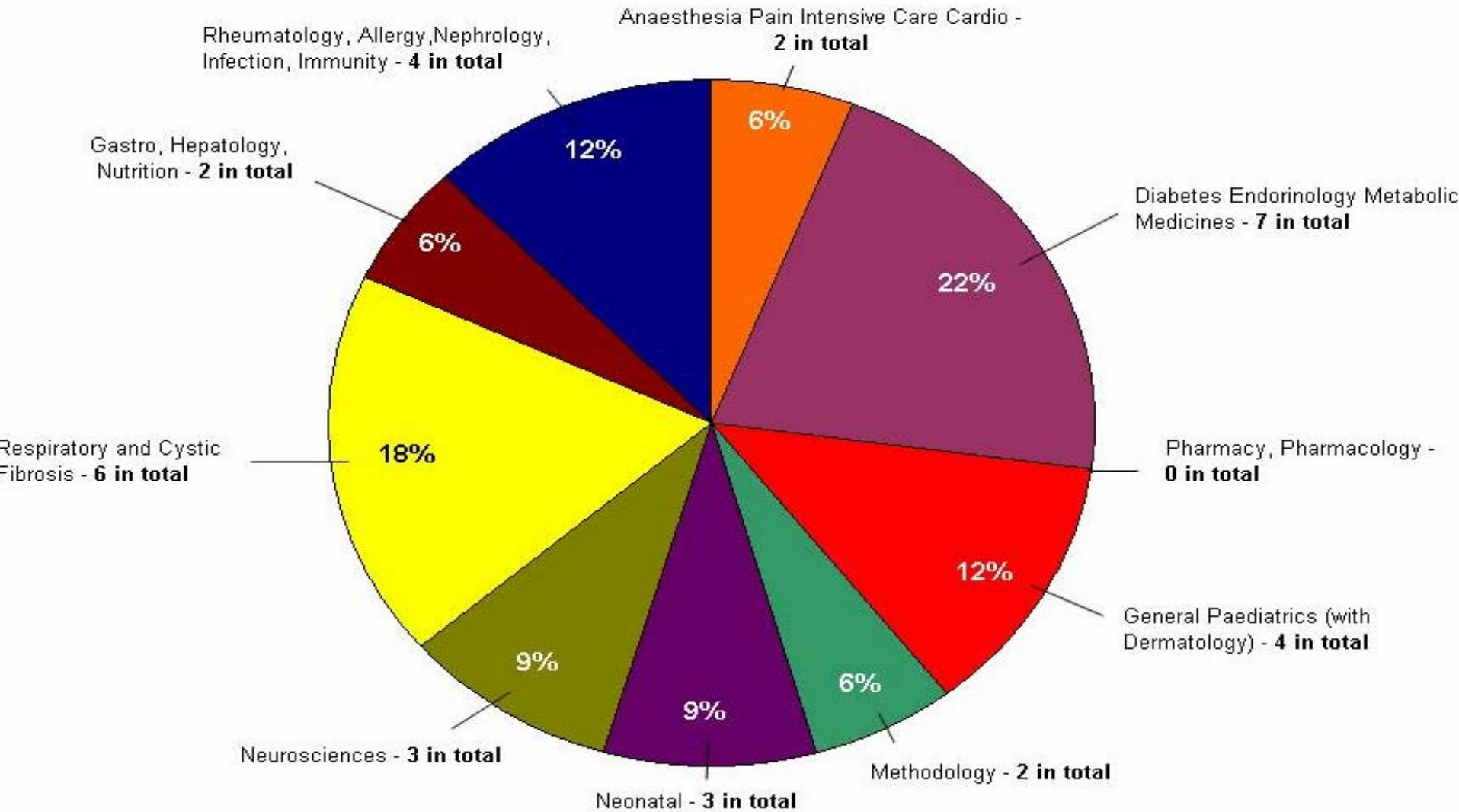


**Includes: appearance, taste, smell, texture & size**

# Chronology of Cheshire, Merseyside & N Wales LRN

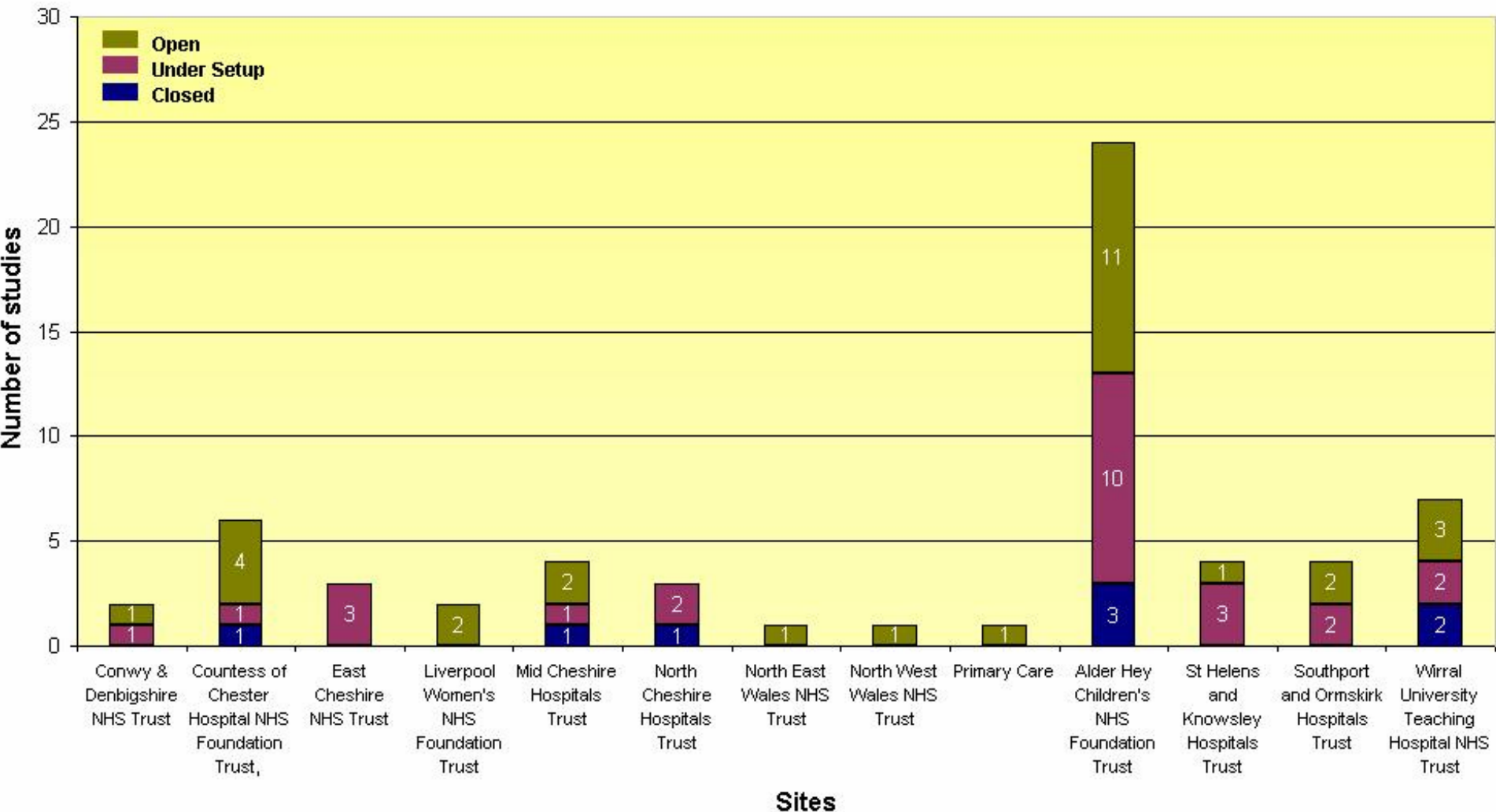
- June 2005: wrote proposal
- December 2005: awarded grant (£2m)
- April 2006: first staff in post
- September 2007: first child recruited to new trial
- December 2007: 50<sup>th</sup> child recruited
- October 2008: 32 studies adopted  
accrual 132

# Portfolio by Clinical Study Groups



# MCRN study portfolio at each LRN site

Number of studies per site that are Open/Under Setup/Closed



## Studies being run at Arrowe Park Hospital

### **BOOST II (RCT)**

Which Oxygen Saturation Level should we use for very premature infants (<28 weeks)

Principal Investigator:  
Donal Manning

### **INISS (RCT)**

International Neonatal immunotherapy study

PI: Oliver Rackam

**ICISS (RCT)** International collaborative infantile spasms study.

PI: Adrian Hughes

**TIPIT (RCT)** Study of thyroxine in pre term babies <32 wks, to determine effect on brain size & devt, pituitary adrenal axis and somatic growth. Pi: Donal Manning

**NEPHROTIC SYNDROME** long-term tapering vs standard prednisolone therapy for childhood nephrotic syndrome PI:Lil Breen

### **MASCOT (RCT)**

Management of Asthma in school age children on therapy

PI: David Lacy

**EMSC** Validation of early morning salivary cortisol (EMSC) as a non-invasive, patient friendly, reliable, cost effective measure of adrenal reserve in children receiving inhaled corticosteroids

PI:David Lacy

# Network Support for Investigators

- Research Infrastructure – research nurses, feasibility surveys, data management, unblocking barriers
- Trials agreement covers multiple sites
- Improved access to the NHS for trials
- Improved access for patients to trials
- Assistance with Governance Processes
- Standardise the cost of trials for industry
- Simplify ‘contracting’ between NHS and industry
- Clinical Trials unit – stats, methodology, trial documentation, randomisation, general advice

# Cheshire Merseyside and North Wales Local Research Network Team

## **Co-Directors:**

**Dr Matthew Peak Director of R & D**

**Dr Jo Blair Consultant Paediatric Endocrinologist**



**Network Manager: Dr Charlie Orton**

**Senior Pharmacist: Catrin Barker**

**Research Fellow in Formulation Science: Dr Utpal Shah**



## **Senior Research Nurses:**

**Lucy Bray Sarah Dyas Helen Hill**

**Team of Paediatric Research Nurses**

**Support staff : PA, Data Support Officer & Data Manager**



*Thank you*

Any questions?