Clinical research: Helping you to help your families

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Young Persons Advisory Group
Clinical research: what and why?

• A process to answer uncertainties or questions about health or illness in people
• To overcome our uncertainties or ignorance and so to improve care:
  – Further our understanding of causes of disorders
  – Improve our ability to diagnose disease
  – Identify and evaluate treatments
  – Assess preventative or risk-reducing strategies
  – Understand the impact of disorders and interventions on families
Some pros and cons of clinical research?

- Research by definition involves uncertainty, but that word itself causes anxiety
- It's costly, slow and bureaucratic — why? – to ensure safety, well-being and best practice
- Researchers and patients get frustrated
- Patients in clinical research studies have better health outcomes
- Hospitals that do research have better patient outcomes
- Research protects patients from wrong practices
Child drug errors 'too frequent'

Mistakes are being made in a high number of drug treatments given to children in hospital, experts warn.

A snap-shot study by the University of London of five hospitals in the city found 13% of the 3,000 prescriptions they examined had an error.

And a fifth of drugs given to children in these hospitals during 2004 and 2005 were administered incorrectly.

Most errors were harmless but a small number were potentially fatal, Archives of Diseases in Childhood reports.

On five occasions, one of the investigators intervened to prevent the patient suffering the consequences.

Too common

Over a period of two weeks, they watched how nurses gave drugs to children on 11 wards at the five hospitals.

They picked up 429 administration errors among the 1,554 doses of medicine given to 265 children, giving an overall error rate of 19%.

When pharmacists reviewed the...
Meager Dosage Data for Kids Makes for Uncertain Prescriptions

Clinical trials rarely include children; as a result, less than half of all drugs are approved for pediatric use. What can be done?

October 15, 2014 | By Dina Fine Maron

The meetings start, as gatherings at 7 A.M. so often do, with people wandering in clutching their coffees and searching for a seat. Fingers instinctively go to pagers affixed at the hip to check for messages, and then all eyes turn to the stacked folders strewn across the table in a generic conference room at Nationwide Children’s Hospital in Columbus, Ohio—the kind of room with long tables, chairs and a projector at the front.
Best advice 1940s-1980s

50 million copies sold up to 1988

Parents were advised to place infants to sleep on their front contrary to evidence from clinical research
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Better research 1995


• By 1970, there was a statistically significantly increased risk of death for front sleeping compared with back (3 times increased risk)
• Earlier recognition of the risks of sleeping on the front might have prevented over 10,000 infant deaths in the UK and at least 50,000 in Europe, the USA, and Australasia.
Effect of change in European Law to mandate research in Children's medicines 2007

**Table 7:**
Number of children to be enrolled in clinical trials

<table>
<thead>
<tr>
<th>Number of subjects</th>
<th>2006</th>
<th>2007</th>
<th>2008</th>
<th>2009</th>
<th>2010</th>
<th>2011</th>
<th>2012</th>
</tr>
</thead>
<tbody>
<tr>
<td>Preterm newborns</td>
<td>0</td>
<td>0</td>
<td>0</td>
<td>207</td>
<td>82</td>
<td>2,281</td>
<td>1,712</td>
</tr>
<tr>
<td>Newborns</td>
<td>0</td>
<td>0</td>
<td>5</td>
<td>64</td>
<td>169</td>
<td>1,105</td>
<td>1,172</td>
</tr>
<tr>
<td>Infants and toddlers</td>
<td>330</td>
<td>21</td>
<td>20</td>
<td>59</td>
<td>351</td>
<td>2,788</td>
<td>3,141</td>
</tr>
<tr>
<td>Children</td>
<td>2,142</td>
<td>181</td>
<td>200</td>
<td>2,230</td>
<td>2,055</td>
<td>10,325</td>
<td>20,677</td>
</tr>
<tr>
<td>Adolescents</td>
<td>368</td>
<td>111</td>
<td>205</td>
<td>1,577</td>
<td>2,861</td>
<td>9,054</td>
<td>13,193</td>
</tr>
<tr>
<td>Sum of above</td>
<td>2,840</td>
<td>313</td>
<td>430</td>
<td>4,137</td>
<td>5,517</td>
<td>25,553</td>
<td>39,895</td>
</tr>
<tr>
<td>Reference: number of paediatric trials</td>
<td>316</td>
<td>355</td>
<td>342</td>
<td>404</td>
<td>379</td>
<td>334</td>
<td>332</td>
</tr>
</tbody>
</table>

Source: EudraCT Data Warehouse using a pre-defined query on 6 March 2013, modified by excluding studies for “Immunological medicinal products”
NIHR Clinical Research Network: Aims

• Ensure patients and healthcare professionals from all parts of the country are able to participate in and benefit from clinical research
• Improve the quality, speed and co-ordination of clinical research
• Unify and streamline administrative procedures associated with regulation, governance, reporting, and approvals
• Increase collaboration with industry partners
• Integrate health research and patient care

http://www.crn.nihr.ac.uk/
Patient recruitment

This is the fourth consecutive year that the Network has surpassed its target to recruit 500,000 patients per year to clinical studies.
Children’s Research Activity

Cumulative number of studies

Annual number of children recruited into studies
Breadth of studies: 70% in rare diseases

- Neurosciences: 43 studies
- General/Other: 49 studies
- Rheumatology: 22 studies
- Gastroenterology, Hepatology and Nutrition: 29 studies
- Inherited Metabolic Disorders: 23 studies
- Neonatal: 34 studies
- Anaesthesia, Intensive Care and Cardiology: 11 studies
- Allergy, Infection and Immunity: 30 studies
- Cleft and Craniofacial: 1 study
- Pain and Palliative Care: 1 study
- Respiratory and Cystic Fibrosis: 18 studies
- Diabetes & Endocrinology: 17 studies
- Pharmacy and Pharmacology: 2 studies
- Dermatology: 4 studies
- Developmental/Behavioral: 13 studies
- Educational/Quality of Life: 2 studies
- Nephrology: 21 studies
- Haematology: 10 studies
- Reproductive: 1 study
- Orthopaedic: 6 studies
- Respiratory and Cystic Fibrosis: 18 studies
- Ophthalmology: 8 studies
- Pharmacy and Pharmacology: 2 studies
- Developmental/Behavioral: 13 studies
- Educational/Quality of Life: 2 studies
- Diagnostic: 1 study
- Pain and Palliative Care: 1 study
Somers Clinical Research Facility
Understanding a Barrier to Research: RECRUIT: Communication about clinical trials as observed and experienced: the views of parents and practitioners

Families and professionals participating in 4 different types of study interviewed:
- Families value the opportunity to hear and potentially participate in research
- Professionals have concerns about approaching families and exhibit a paternalistic approach to research
Patients as Partners
Members of the London Young Persons Advisory Group
Young Persons Advisory Groups

- Members aged 8 – 18
- Consultation and collaboration service for researchers
- Meet every 6-8 weeks
Role of the group

• **Learn** about health/clinical research in monthly meetings
• **support** and **work** in **partnership** with researchers to help them with their studies
• **provide input** and work with key organisations such as National Research Ethics Service, Royal College Paediatrics Child Health, NIHR Clinical Research Facilities and Hospitals, to stress importance of research and importantly the involvement and engagement of young people in their processes
How we can help researchers and companies

- Talk to them about research perhaps in our words – it helps them think about it differently
- Advise them on whether their study sounds good for children – tell them what’s important to us
- Help them with the information sheets they produce to explain the study to children
- Sometimes reassure them that it is a good study and not to be afraid of asking families to help
What’s important to us

• Getting the results of the research you participated in
• Knowing that future generations won’t have that problem because you’ve helped to deal with it
• Hearing whether our feedback helped the researcher
• Letting young people be more educated about things that are going on that might affect them personally
Feedback from researchers

‘Thank you to the group and you for letting me come along. For me personally it was a great experience. I have taken the feedback to the researchers and they are going to redo the forms and submit to ethics.’

‘Thank you for a most wonderful session with the Young persons Group at GOSH yesterday. Could you please let the group know that they were not only helpful, but also inspiring. We will definitely incorporate their suggestions into our revisions of the information leaflets, and their comments into in the rationale for our approach to involving young people in the project and I dare say others as well.’

‘Thank you for inviting me to come to the YPAG meeting… I've just read through all of the really useful feedback from the group and made some helpful changes to my info sheets. They're a great group - all very thorough and had some really good suggestions to make!’
Changes we would like to see

• More research is needed *with* children, not simply *on* children

• Researchers should find ways to demystify clinical trials and experimental medicine
  – Better images
  – Workshops in schools
  – Raise awareness about clinical trials research and its value
  – Publicise it better
You're invited to join us for
Generation R
young people improving research

11th September 2013, Science Museum, London, 10am - 4pm

The award winning NIHR Medicines for Children Research Network Young Person's Advisory Group invite you to an event to showcase how children, young people & families have improved the design, development and delivery of paediatric research.

Topics led by Young People

Benefits of Collaborating with Children & Families
Impact & Evidence in the Design & Delivery of Research
Disseminating Research Results
The Future of Research / Educating the Next Generation

Special Guests

Professor Dame Sally Davies Chief Medical Officer
Sir Iain Chalmers Editor, James Lind Library
Simon Denegri Chair of INVOLVE
Dr Jonathan Sheffield CEO, NIHR Clinical Research Network

To register your interest please log on to
Conclusions

• Clinical research including trials are important to improve our knowledge and treatments
• Actually in a rare, genetic condition they are even more important
• The NIHR has created great opportunities for researchers and for patients to take part
• We have shown that children and families want to know about research, want to get involved and can make research better