

# Pediatric medicinal clinical trials: How do we carry them out in the UK?

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Mandy Wan

Lead Paediatric Research Pharmacist

NIHR Clinical Research Network: Children / Evelina London Children's Hospital

CRN : Networking Locally, Delivering Globally



*Delivering research to make patients,  
and the NHS, better*

# Disclaimer

The opinions expressed are my own and not  
the National Institute for Health Research  
Clinical Research Network.

# Why?

- **In pursuit of evidence-based medicine**
- **Critically assessing the validity of the data available to support therapeutic decisions for children**
- **Enactment of the EU Paediatric Regulation**
- **Is it too early to assess its impact?**
- **Many unanswered questions.....**



# Funding

**Funding for investigator led research come from a variety of sources:**

- **Commercial funders**
- **Non-commercial funders**
- **Government (e.g. NIHR)**
- **Research councils (e.g. Medical Research Council, Wellcome Trust)**
- **Charities**
- **European Commission**

**“Association of Medical Research Charities (AMRC) members support over one-third of all UK publicly-funded medical research in the UK, with members investing over £1.2 billion in health research in the UK in 2012.”**

# Funding

- It is important that the various types of costs of research are appropriately attributed in order to ensure that the appropriate funding arrangements are put in place.
- Guidance for non-commercial research is set out in the guidance 'Attributing the costs of health and social care Research and Development'.

**Research Cost**

**NHS Treatment Cost**

**NHS Support Cost**

# Patient Public Involvement

- We believe that high quality research, good recruitment and improved health outcomes for children, depend on listening to the voices of children and young people, as well as their families and carers.
- As a result, we want to ensure the design and direction of clinical research is not simply based on the views of research professionals and clinicians. So we put children, their families and carers at the centre of what we do.



<http://generationr.org.uk/>

# Patient Public Involvement



*National Institute for  
Health Research*

Clinical Research Network

# Pharmacy & Formulation Input

No drug **PRODUCT**  
No clinical trial





# Pharmacy & Formulation Input

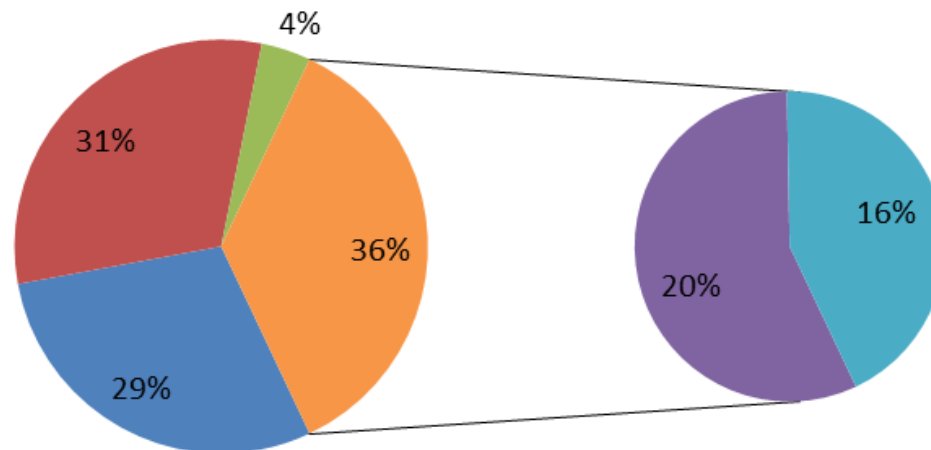
- Reviewed a consecutive series of 45 investigator led clinical trials proposals between April 2012 and March 2015
- 103 products were required to support these studies

Clinical Specialities, n	Clinical trials (n=45)
Allergy, Infection and Immunity	7
Anaesthesia, Intensive Care	2
Cardiology	1
Diabetes and Endocrine	2
Gastroenterology, Hepatology and Nutrition	2
General Paediatric	2
Haematology	2
Inherited Metabolic Disorders	1
Neonatal	7
Nephrology	4
Neurosciences	4
Pain	1
Psychiatry	1
Respiratory and Cystic Fibrosis	6
Rheumatology	3

# Pharmacy & Formulation Input

- 8 of the 45 trials had support from pharmaceutical industry

- UK licensed product suitable for use
- UK licensed product requires further processing or unblinded personnel on site to perform blinding
- Suitable licensed product from EU was sourced
- Bespoke manufacturing required for IMP
- Bespoke manufacturing required for placebo



# Pharmacy & Formulation Input

- **Estimated cost (packaging, labelling, shipments +/- drug/placebo manufacturing cost)**
  - **Estimated cost for 19 trials was available.**
  - **Min cost per patient = £95**
  - **Max cost per patient = £6000**
  - **Median cost per patient = £342**

# Buy-in

- **Key challenge: What is standard of care?**
- **Current practice is often not evidence-based**
- **“Standard” in one hospital may be different to “Standard” in another**
- **Prove ample opportunity for full participation of interested investigators - greater sense of “ownership” and thus commitment**
- **Do we compromise?**
- **To what degree?**

# Managing the trial

- **Often challenging!**
- **Everyone is always very busy!**
- **Expect lots of unexpected issues**
- **Think about “marketing” your trial**
- **Providing frequent positive reinforcement**
- **Healthy competition is never a bad thing**
- **Maintain enthusiasm.....and keep smiling!!!**

# There is hope!

- **The number of registered clinical trials units in the UK has substantially increased to support new investigators**
- **The regulatory environment has eased a little**
- **Risk adapted approach (esp. for paediatric trials looking at off-label use)**
- **More streamlined approval processes**
- **Many tools available to support investigators**
- **More research in clinical trials methodology in paediatrics**

# Evelina London Children's Hospital

- Commercial clinical trials
- Investigator led clinical trials



# Evelina - our team

**NICU studies RN cover:**  
 Julia Wurie- KCL oversight,  
 Isabel Viac- Xe CTIMP  
 Helen Bromfield- Planet  
 Mary Ann Sharma- imaging

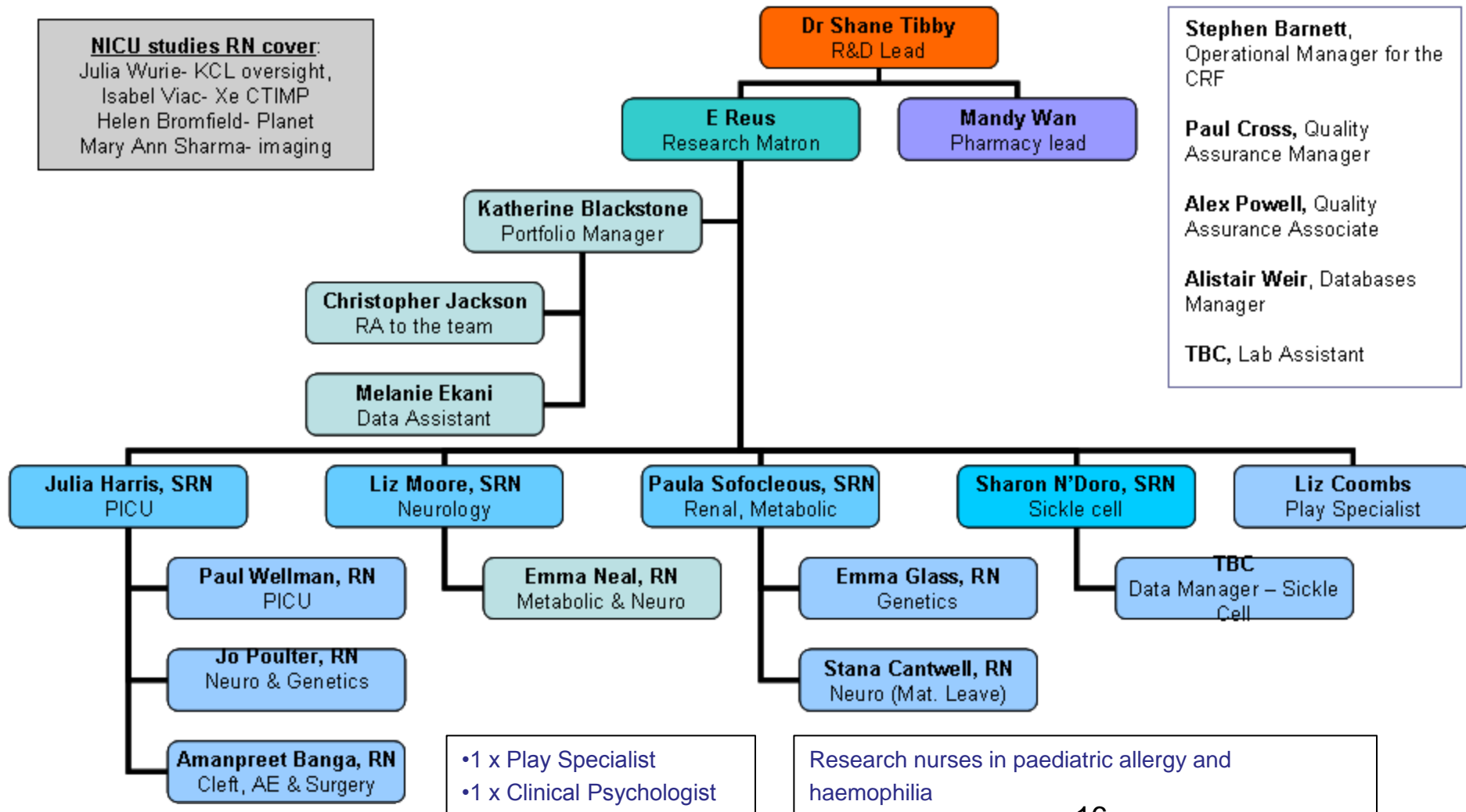
**Stephen Barnett,**  
 Operational Manager for the CRF

**Paul Cross,** Quality Assurance Manager

**Alex Powell,** Quality Assurance Associate

**Alistair Weir,** Databases Manager

**TBC,** Lab Assistant



•1 x Play Specialist  
 •1 x Clinical Psychologist

Research nurses in paediatric allergy and haemophilia



# Feasibility assessments

- **Co-ordinated by our Portfolio Manager (Oversight of the whole portfolio!)**
- **Express of interest request from NIHR Clinical Research Network**
- **Site Selection/ Qualification visits**
- **Ensure all those that need to be involved are involved at the earliest stage**
- **Do we have the capability?**
- **Do we have the capacity?**
- **What support is being requested by the investigator?**
- **Setting realistic targets**
- **Work collaboratively with our R&D contract team on contracts and costings**

# Research nurses

- **“There will be no clinical research without research nurses!”**
- **The engine for clinical research!**
- **“Manage” the investigators!** 😄
- **Advocate for the participants**
- **Coordinate study visits**
- **Carry out some of the study visits activities**
- **Complete case report forms.....and much more.....**

**Training is at the heart of our philosophy:**

- **Support from a senior research nurse**
- **Research nurses competency training**
- **Regular training sessions**

# Other team members

- **Non-clinical members (Research delivery professionals)**
- Administrative support
- Completion of case report forms
- **Play specialist**
- Use play interventions to support trial participants
- Provide distraction & Enhance engagement
- Improve participants' experience with painful procedures
- **Clinical Psychologist**
- Support children and families in a holistic way
- Social and psychological impact
- **QA team**

# Multi-disciplinary is key!



**and we talk to each other.....**



**National Institute for  
Health Research**

**Clinical Research Network**

**Thank you!**



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